

Externally-led Juvenile Idiopathic Arthritis Patient-Focused Drug Development Meeting Report

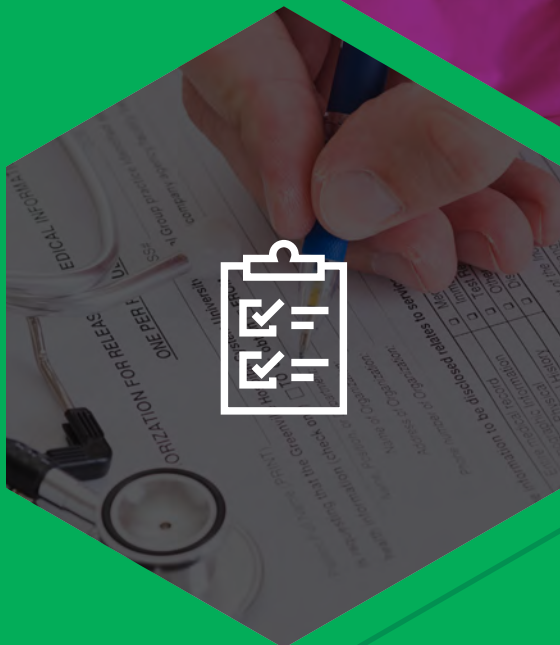


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The Food and Drug Administration (FDA) seeks to understand and reflect the perspectives of patients and caregivers in the drug development process. Over the past six years, the FDA has conducted 25 internally-led patient-focused drug development (PFDD) meetings on various conditions to systematically gather critical input regarding the issues and challenges that affect patients' treatment needs, choices, compliance and quality of life. Recognizing that a significant number of diseases still needed to be addressed, the FDA issued a call for partners to conduct externally-led PFDD meetings to collaborate on these critical information-gathering initiatives. The Arthritis Foundation answered that call in March 2017 hosting an Osteoarthritis externally-led PFDD meeting. Then again in August 2018, the Arthritis Foundation partnered with the Childhood Arthritis and Rheumatology Research Alliance (CARRA) to co-host the externally-led PFDD meeting about Juvenile Idiopathic Arthritis. The Arthritis Foundation and CARRA are proud to engage with the FDA in this initiative to provide patient input regarding juvenile idiopathic arthritis (JIA).

The Arthritis Foundation and CARRA

For more than 70 years, the Arthritis Foundation has been the leading patient advocacy group for adults and children living with arthritis, the nation's leading cause of disability. A core tenet of the Foundation's mission is accelerating groundbreaking research through financial support and strategic partnerships. This mission served as the catalyst for the Foundation's collaboration with the Childhood Arthritis and Rheumatology Research Alliance (CARRA).

“

Participation in research is the path to a cure and the Arthritis Foundation is committed to bringing new opportunities for researchers, clinicians, patients and families to actively pursue better treatments with us.”

– Ann Palmer,
Arthritis Foundation

Established in 2002, CARRA is a pediatric rheumatology research network whose mission is to conduct collaborative research to prevent, treat and cure pediatric rheumatic diseases. CARRA's research network includes almost all the pediatric rheumatology centers across the U.S. and Canada. Members include clinicians, researchers and research coordinators. The Arthritis Foundation has funded CARRA's infrastructure and operations since its inception and the organizations have been collaborative partners for more than a decade. In 2015, the partnership evolved to a more strategic one that aligns scientific agendas; expands research about the impact, unmet needs and treatment of pediatric rheumatic diseases; and prioritizes patients' and families' participation in meaningful and high-quality clinical and translational research.

Among recent collaborations has been an effort with the FDA to advance understanding of patient perceptions around treatment, risk/benefit analysis, and clinical trial design. A manuscript entitled “Toward Improvement in the Timely Authorization and Availability of New Medicines for Juvenile Idiopathic Arthritis: Multi-Stakeholder Perspectives” has been submitted for publication summarizing an April 2018 CARRA- and AF-sponsored meeting of FDA, European Medicines Agency (EMA), major pharmaceutical companies, clinicians, researchers, patients and parents.

Purpose of the PFDD Project

Through its Division of Pulmonary, Allergy and Rheumatology Products, the FDA oversees drug development for rheumatic diseases including juvenile idiopathic arthritis (JIA). With the enactment of the Prescription Drug User Fee Act (PDUFA) in 1992, Congress created a mechanism for the FDA to accelerate the review and approval of innovative medicines and products based on the best available science.

As part of this initiative, the FDA recognizes the need to broaden patient and caregiver input to identify the critical variables, endpoints and outcomes that more accurately represent the burden of disease and the impact of available treatments. To help meet these needs, the PFDD initiative was launched in 2012.

The goals of the PFDD initiative are to

- Develop treatments that meaningfully address the aspects of disease most important to patients.
- Tailor clinical trials to reflect patient’s perspective on the benefits and harms of treatment in drug evaluation.
- Ensure the information and data accurately represent self-reported benefits and harms and is directly relevant to patient’s treatment needs and decisions.

Nikolay Nikolov, MD, Associate Director for Rheumatology, FDA Division of Pulmonary, Allergy, and Rheumatology Products, summed up the importance of PFDD meetings during his introductory comments at the Externally-led JIA PFDD Meeting.

“

The information will help to better inform the benefits-risk assessment of new therapies, identify areas of unmet need and help drug developers and regulatory authorities, such as the FDA, design programs that are more relevant to addressing how patients feel and function.”

– Nikolay Nikolov, MD,
Food and Drug Administration

In December 2016, the 21st Century Cures Act was enacted to accelerate the translation of scientific discovery into FDA-approved treatments, foster interdisciplinary and cross-functional collaboration, and integrate patient insights into the regulatory review process. The law builds on efforts of the FDA to include patient perspectives into the drug and medical product development process.

Through their PFDD and 21st Century Cures Act efforts, one goal of the FDA is to bring safe and innovative therapies to arthritis patients faster. This will help minimize

- Joint damage, disability and other barriers to optimal health-related quality of life.
- Disruptions to educational and career experiences and opportunities.
- The negative impact on social, psychosocial and family dynamics.

History of JIA Treatment and Current State of the Art

More than 100 years ago when rheumatic diseases were first diagnosed in children, significant joint pain, loss of function, and deformity were the norm. Aspirin and glucocorticoids were the only drugs regularly used. However, the side effects of extended steroid use included increased osteoporosis risk, abnormal skeletal growth, obesity and increased infection risk. By the 1970s, nonsteroidal anti-inflammatories became available. Older treatments, such as gold and penicillamine, were sometimes used despite a high toxicity rates with little benefit. It wasn't until the 1980s and the availability of the disease-modifying antirheumatic drug (DMARD) methotrexate that an era of disease suppression with significant improvements in outcomes commenced. This paved the way for more aggressive use of DMARDs in JIA.

Biologic response modifiers, or biologics, became available in the late 1990s. These powerful and game-changing therapies for inflammatory arthritis specifically target specific components of the immune system. With small-molecule targeted therapies now available in adults, the JIA community is awaiting the first small-molecule indications for children.

Table 1. Biologics and Small-Molecule Targeted Therapies for Arthritis



TNF Inhibitors <ul style="list-style-type: none">• Infliximab (Remicade)• Etanercept (Enbrel) *• Adalimumab (Humira) *• Golimumab (Simponi) * *• Certolizumab (Cimzia) * *	IL-1 Inhibitors <ul style="list-style-type: none">• Anakinra (Kineret) * *• Canakinumab (Ilaris) *• Rilonacept (Arcalyst)
Inhibitor of CD28-B7 (T cell co-stimulation signal) <ul style="list-style-type: none">• Abatacept (Orencia) *	IL-17 Inhibitors <ul style="list-style-type: none">• Secukinumab (Cosentyx) * *• Ixekizumab (Taltz)
IL-6 Inhibitors <ul style="list-style-type: none">• Tocilizumab (Actemra) *• Sarilumab (Kevzara) * *	IL-12/23 Inhibitor <ul style="list-style-type: none">• Ustekinumab (Stelara)
B cell Inhibitor <ul style="list-style-type: none">• Rituximab (Rituxan)	JAK Inhibitors [Small Molecule] <ul style="list-style-type: none">• Tofacitinib (Xeljanz) * *• Baricitinib (Olumiant) * *
	Phosphodiesterase Inhibitor [Small Molecule] <ul style="list-style-type: none">• Apremilast (Otezla)

* FDA Approved for JIA.
* * Currently in clinical trial for FDA approval in JIA.



Table 2. JIA Disease Subtypes

JIA Subtype	Characteristics
Oligoarthritis-persistent	<ul style="list-style-type: none">• Fewer than five affected joints with no additional joint involvement after the first six months• Highest risk for uveitis that may progress to glaucoma, cataracts and possibly blindness• Usually affects young girls
Oligoarthritis-extended	<ul style="list-style-type: none">• Initially involves fewer than five joints for the first six months and then disease progresses to additional joints• High risk for uveitis• Usually affects young girls
Polyarthritis RF negative	<ul style="list-style-type: none">• Involves five or more joints (small and large) within the first six months• Symmetric joint involvement of many joints is common• Rheumatoid factor blood test is negative• Occurs early in childhood• More common in girls
Polyarthritis RF positive	<ul style="list-style-type: none">• Involves five or more joints (small and large) within the first six months• Rheumatoid factor blood test is positive• Usually occurs after age 10• More likely to occur in girls• Similar features as rheumatoid arthritis in adults
Enthesitis-related arthritis	<ul style="list-style-type: none">• Involves inflammation where tendons and ligaments insert into the bone (entheses), very commonly around the heel but can occur in other places• Most commonly affected joints include the sacroiliac, knees, ankles and hips. The small joints of the feet and toes may also be affected .• Usually affects pre-teens and teenagers, although it can start earlier• More common in boys

JIA Subtype	Characteristics
Psoriatic arthritis	<ul style="list-style-type: none"> • Commonly occurs with the skin disease psoriasis • Other psoriatic features that can be seen include dactylitis (inflammation of individual toes and fingers) and nail changes • Axial disease (inflammation of the spine) can occur
Systemic arthritis	<ul style="list-style-type: none"> • Characterized by high spiking fevers • Salmon-colored rash that comes and goes with fever • Body-wide inflammation may affect many vital organs • Life-threatening complication called macrophage activation syndrome (MAS) can occur • Joint involvement may not occur initially, and can involve any number of joints • Affects boys and girls equally • Can occur at any age, even in adults
Undifferentiated Arthritis	<ul style="list-style-type: none"> • Doesn't fit into any one of the above categories



The trend toward earlier, more aggressive JIA therapy put methotrexate in the forefront as first-line therapy. It is also often combined with a biologic, especially if methotrexate alone has not been effective. Biologics are used alone generally when methotrexate is not tolerated. The biologic selected by clinicians and patients often depends on the subtype of JIA the patient has and other factors, but it often is impacted by the patient's health insurance coverage.

JIA Disease Subtypes

Juvenile idiopathic arthritis is an umbrella term used to describe a heterogeneous group of childhood-onset chronic arthritides that includes eight categories. Current classification is based on clinical features and thus is imperfect: it is not unusual for a child to have one initial presentation and then later be re-categorized as having a different subtype of JIA due to changes in the course of disease. A focus of current research is to assign a JIA subtype based on specific genetic, molecular, or other biomarkers rather than relying on variable clinical features such as the number of joints involved. This improvement will allow doctors to more accurately match the biology of a child with a specific evidence-based treatment.

Unmet Treatment Needs

The objective of the JIA PFDD initiative is to broaden awareness of unmet disease management needs and challenges of JIA patients and their families. These insights can help inform drug development, set priorities, and improve the pace of the FDA's approval processes.

The 2018 Outcome Measures in Rheumatology (OMERACT) JIA core set workgroup identified five disease impact domains and several related impact areas important to patients and parents. The domains included activity limitation/physical function, joint inflammation, patient perception of disease (overall well-being), adverse events, fatigue, stiffness, joint damage, extra-articular inflammation (including eye disease), emotional function, participation restriction and others.

Similar findings were reflected in the JIA PFDD pre-meeting insight gathering process (an online survey and five focus groups conducted by the JIA PFDD organizers). The most common themes among patients and families included the following:

- Need for safer, more effective, easier and less-painful-to-administer medications.
- Need for medications less likely to cause potential long-term side effects.
- Solutions to the challenges of dealing with comorbidities and multiple medication changes.

The intent of the JIA PFDD meeting was to establish a common understanding of the real-world impact of unmet treatment needs and priorities for patients and families. This information will in turn be used help researchers and health care leaders better understand where to focus time and resources. Fostering a responsive and collaborative process that operates at the intersection of innovation and patient insight will help transform how care is delivered to children with all types of arthritis.

Overview/Introduction of the Meeting and Pre-Meeting Content

“

The JIA PFDD initiative is critical to shaping the future of drug discovery for people with arthritis and advancing collaboration between the Arthritis Foundation, the FDA, patients, doctors and industry leaders.”

– Ann Palmer,
Arthritis Foundation

Similar to the Arthritis Foundation, leadership at CARRA saw the JIA PFDD as an opportunity to increase understanding of patients’ unmet needs and how JIA and its treatment impact their daily lives, realizing that this must drive medication development, and provide the underpinning for the efforts of providers, researchers, industry and regulators. In February 2018, the Arthritis Foundation and CARRA received FDA approval to host an externally-led PFDD meeting focused on JIA. To develop a comprehensive plan for the meeting and the resultant report, a project team was recruited. Project team members included pediatric rheumatologists, patient representatives, Arthritis Foundation staff, CARRA leaders and staff, Pediatric Rheumatology Care and Outcomes Improvement Network (PR-COIN) representatives and European Network for Children with Arthritis (ENCA) representatives (see Appendix 1). Planning meetings began March 30, 2018.

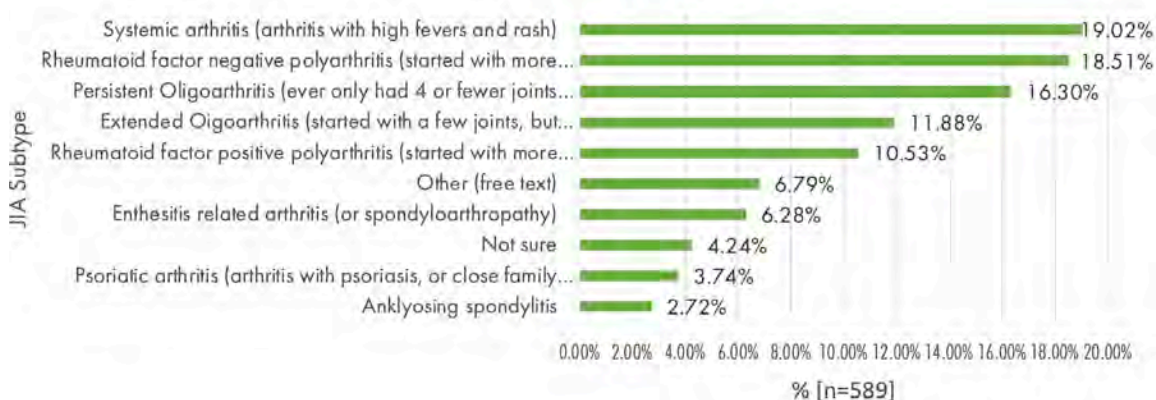
To inform content development for the JIA PFDD meeting and provide background data, the project team deployed an online survey to JIA families throughout the country between June 25 and July 13, 2018 and conducted five focus groups in Seattle, Washington on June 29 and 30, 2018. The preparation and planning process culminated in the Arthritis Foundation–CARRA JIA PFDD meeting in Washington, D.C. on August 2, 2018. While the individuals who participated in the survey, focus groups, and in-person meeting are not a perfectly representative sample of the broader JIA community, the information yielded was nonetheless incredibly valuable and touched on issues of importance to the whole community.

Pre-Meeting JIA Survey

There were 590 survey respondents with approximately an 8 to 1 ratio of caregivers to young adult participants representing 49 states and the District of Columbia (see Appendix 2). Due to Institutional Review Board concerns, only individuals over the age of 18 were permitted to answer the survey. Therefore, the vast majority (88%) of respondents were parents. The patients represented by their parents in the survey ranged in age, but 36 percent were between the ages of six and 15 years.

While all types of JIA included in Table 2 were represented, most participants reported either oligoarthritis (persistent plus extended) or polyarthritis (RF positive plus negative) at 28 percent each; 19 percent had systemic disease. Table 2 presents the argument that most subtypes of JIA were represented in the survey. That said, relative representation is different from published prevalence data. The most common age at diagnosis was between two and five years (35 percent). For 73 percent of respondents, the time since JIA diagnosis was less than 10 years, representing relatively recently diagnosed patients and families.

% Surveyed with JIA Subtype



The majority (~85 percent) of patients were seeing a pediatric rheumatologist, while the remainder were principally under the care of adult rheumatologists. Other published reports show that only about 50 percent of children with JIA are seen by pediatric rheumatologists, indicating a possible selection bias in our survey results. There is a well-documented shortage of pediatric rheumatologists, but that was not the focus of this PFDD meeting.

Pre-Meeting JIA Focus Groups

Five focus groups were conducted in Seattle, Washington on June 29 and 30, 2018. The 39 participants represented young adults with JIA and parents of children and adolescents living with JIA (see Appendix 3).

All disease subtypes were represented, with the largest percentage being those diagnosed with polyarticular JIA. The age of diagnosis varied, with the majority diagnosed between the ages of six and 10.

Participants shared unique perspectives on disease burden; impact on daily living; treatment concerns and challenges; psychosocial and family dynamics; as well as clinical trial concerns and recommendations.

PFDD Meeting Panelists

Twelve panelists were selected to participate in the PFDD meeting based on diagnosis, age of onset, geographic location, and other factors to assemble a diverse and as representative a panel as possible. Three panelists were young adults with JIA. Nine panelists were parents of children or young adults with JIA.

“

I was so young [when I was diagnosed] that I don't know what it feels like to live without pain.”

– Andrew Curtis, panelist

Each family living with JIA has its own experiences and each patient has a unique set of disease manifestations. While many people with JIA have a relatively limited disease course, the panelists and audience participants who gathered for the PFDD meeting tended to deal with the most challenging issues.

Panelists received onboarding materials to explain the purpose and scope of the PFDD meeting. They also worked with members of the PFDD project team to refine their messages and prepare for the day.

A key goal of the panelist testimonies was to deliver clearly enunciated calls to action. This is a key shift in the patient engagement paradigm -- moving from a largely emotive discourse to the lived experience as a patient or parent, and now to one that truly informs the conversation and compels action.

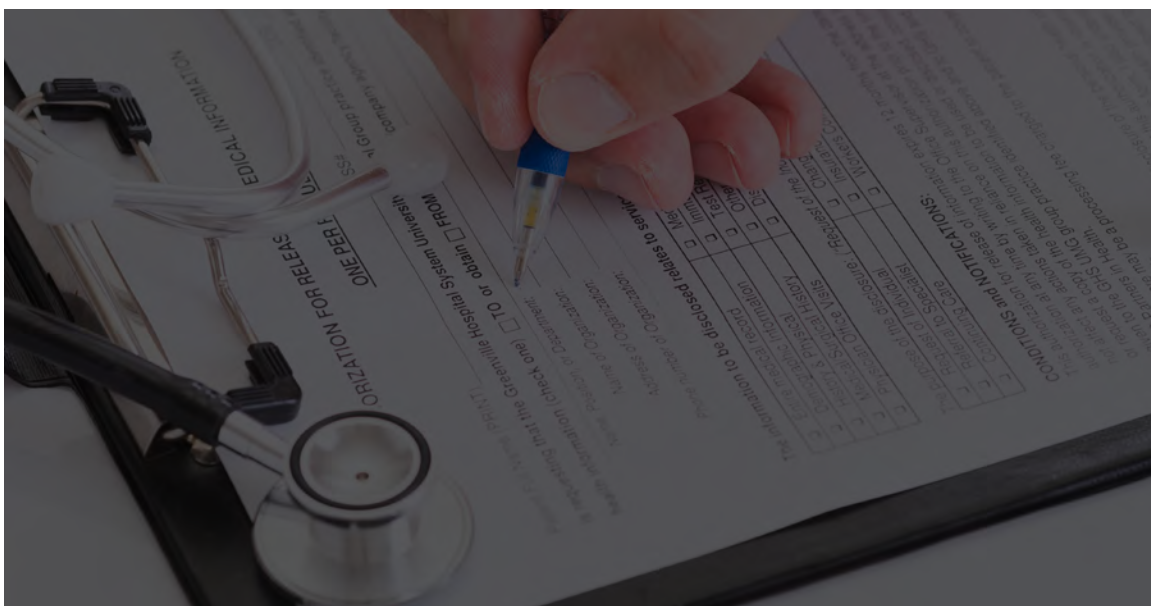
PFDD Meeting

The August 2 meeting provided patients, caregivers and patient advocates the opportunity to discuss the impact of JIA on daily life with FDA staff, researchers, clinicians, academic partners, drug manufacturers and other healthcare industry leaders (see Appendix 4). They shared information about the most challenging symptoms; issues that impact treatment choice and adherence; experiences with available treatments; and the need for new, safer and easier/less-painful-to-administer treatments.

The meeting attendees included close to 150 patients and their families, FDA staff, industry leaders, and researchers who attended in-person or via webinar.

The PFDD meeting was split into three topic discussions, with four panelists speaking during each discussion. A cellphone application was used to allow participants to answer polling questions (see Appendix 5). The in-person attendees and those participating via webinar were invited to speak and ask questions during Question & Answer sessions. Discussion topics included the following:

- Symptoms and daily impacts.
- Current approaches to treatment.
- Clinical trial design.



Topic I: JIA Symptoms and Daily Impacts That Matter Most to Patients

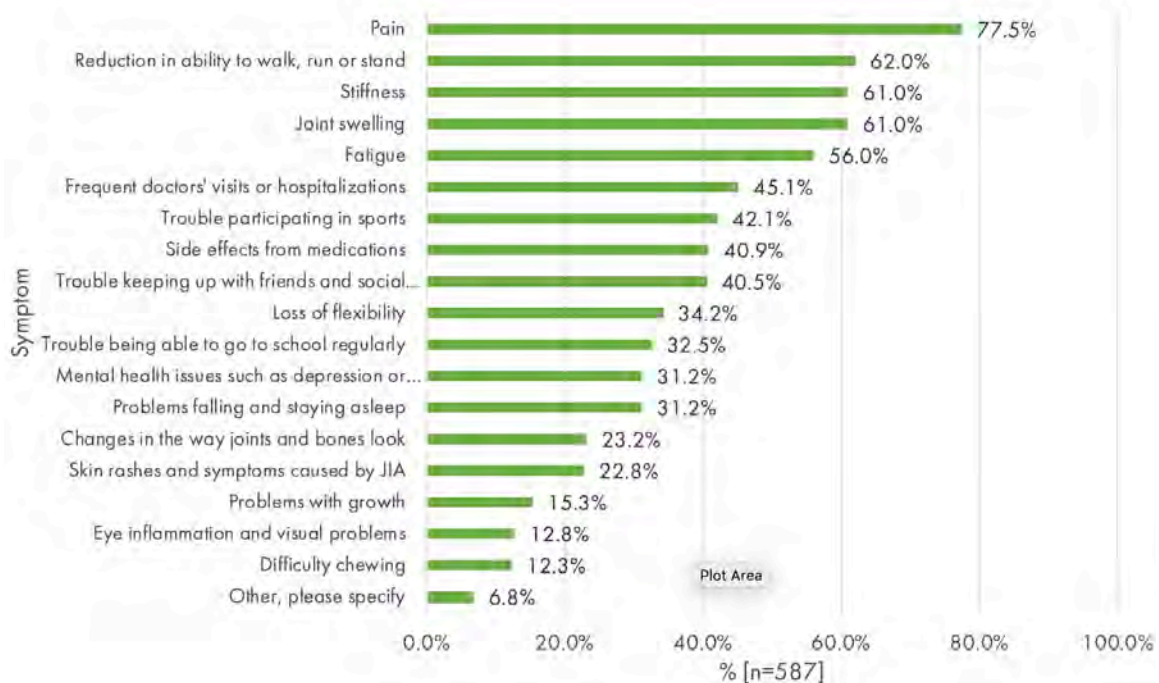
Panelists, audience members, web participants, survey respondents, and focus group members all gave input on how the disease affects their lives. Their responses fell into the following five main themes:

- Disease symptoms.
- Comorbidities and medication side effects.
- Financial and family impacts.
- Social, school, and recreational impacts.
- Loss of mobility.

Disease Symptoms: Pain and Fatigue

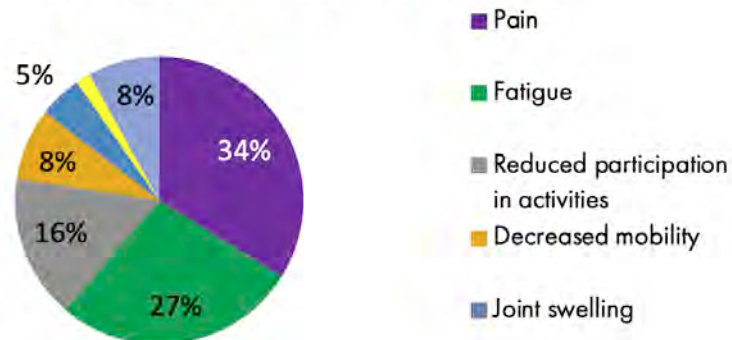
More than three quarters of pre-meeting survey respondents reported that pain was the symptom that affected them the most. Pain was also the symptom most frequently cited by focus group participants, was a common theme among panelists, and was the most impactful symptom for 34 percent of the in-meeting polling respondents.

The pre-meeting survey asked “Of all the signs and symptoms you/your child have ever experienced because of JIA, which ones have had the biggest impact on your/your child’s daily life? (Select all that apply).”



The in-meeting poll of 62 people asked “Which of the following symptoms has had the greatest impact on you?”

Symptoms of Greatest Impact



While fatigue was the second-most common response for focus group and meeting attendees, it was fifth among pre-meeting survey respondents, behind limited mobility, stiffness, and joint swelling. These symptoms were also described by panelists and audience members as part of the overall disease burden.

Panelist Kirsten Wilder is a mom of three children whose youngest daughter, Katherine, was diagnosed with JIA at age three. Ms. Wilder said that even with treatments that help manage other aspects of the disease, “the pain and fatigue never go away.”

When asked what changes they’ve had to make in their lives as a result of JIA, one audience member, whose four-year-old daughter was diagnosed at age three, said her daughter gave up ballet. “She wanted to go back and be with her friends, but she didn’t want to deal with the pain.”

Anjie Vago, a panelist with two daughters who have JIA, described years of pain and fatigue. Her daughters continue to struggle, even with treatment.



We’d be thrilled to be able to resurrect some of our old dreams and hopes without pain, without fatigue, with effective treatment for all aspects of juvenile arthritis”

– Anjie Vago, panelist

Comorbidities and Drug Side Effects

Comorbidities and drug side effects often increase the burden of living with JIA. This was a recurring theme among focus group members, panelists, and audience participants. Focus group members discussed complications associated with comorbidities, increased susceptibility to infections from their treatments, and how these factors all contribute to frequent doctors’ visits and hospitalizations. Although survey respondents weren’t asked about comorbidities, nearly half of them reported that frequent doctors’ visits or hospitalizations had the most impact on their daily lives.

Ms. Vago expressed concern about the impact of comorbidities and drug side effects. She noted that comorbidities are very often present in JIA patients of all ages. These can be other autoimmune diseases, such as a diabetes or inflammatory bowel disease. Gastrointestinal or other problems can also be secondary to the medications they take. For example, Ms. Vago's daughter Laura developed exogenous Cushing syndrome as a result of steroid treatment.

During the Q&A discussion, one commenter described how immune suppression due to medications leads to a greater risk of illness, compounding the worry her family feels when planning gatherings or outings.

“

If it's flu season and the cousin had a cough a week ago, we cancel Thanksgiving. I'm not willing to risk my daughter getting sick.”

– audience participant

Ms. Wilder, who lives in Maine, takes her daughter to a number of specialists due to drug-related susceptibility to infections and medication side effects. “Between the regular appointments with rheumatology, monthly infusions, PT, OT, continue, continue, continue, we know how to get to Boston and back very well.”

Financial and Family Impacts

A key theme shared by panelists, focus groups and audience participants was the impact that JIA has had on parents' and patients' ability to work.

Within the focus groups, several parents shared that they had quit their jobs or cut back to part-time to manage their children's care. Among young adults, challenges with work were widely cited. Many young adults talked about how much JIA had impacted career choices – some opted for a desk job rather than something requiring significant activity. Those with desk jobs also shared that they need to move around frequently to stretch and avoid stiffness.

Both young adult groups were vocal about the challenge of seeking and receiving accommodations at school or work. They cited the invisibility of the disease as a major barrier to others' understanding. One young adult group talked about the challenge of being able to access treatment with a full-time job – needing absences and allowances for doctor appointments, therapy sessions and infusions.

“

I should have been able to go back to work by now. I should be able to earn money to help pay for college. I should be able to quilt for hours, or audition for a play. My husband and I should have been able to take the trip we had planned last year to celebrate our 25th anniversary. Because of JIA and all that comes with it, these things are not our reality.”

– Anjie Vago, panelist

Ms. Vago described how the unpredictable nature of the disease limits how she spends her time and prevents her from making long-term plans, even though both of her daughters are now adults. She feels she needs to be available when her daughters are having a flare or otherwise need her.

Panelist Corinne Pinter, a mother of three whose 10-year-old daughter has JIA, also noted that being a full-time caretaker limits her ability to work a full-time job. She needs time off for doctor and therapy

“

We take care of their appointments, we take care of everything day-to-day. It's hard for us to get back into the workforce full time because you don't know what the day's going to bring. It's our constant burden.”

– audience participant

appointments, and when her child is having a bad day and misses school, she calls in sick to work.

An audience member whose daughter has JIA described how the need for medical coverage forced him to quit his job with a small business and seek employment with a company that offered better health insurance. In his new job, he travels 75 percent of the time and is away from his family “to pay for expensive treatments that work 50 percent of the time for my child.”

School, Social Activities, and Sports

A common theme among all patient groups was how JIA changed their ability to fully engage in life's activities, like school, sports, and social gatherings. The pre-meeting survey found that 42 percent had trouble participating in sports, 41 percent had trouble keeping up with friends, and 33 percent didn't attend school regularly. The in-meeting poll found that 16 percent cited difficulty participating in activities as the most burdensome aspect of their disease.

The parent focus groups spoke to their children limiting or quitting sporting activities. The young adults, however, described having the tenacity to push through, noting that adaptation had become so common they may not even realize they were making accommodations.

Parents offered that healthy classmates and friends didn't understand disease limitations and would leave their children behind. They also expressed fear that if their kids fully joined in a social activity, they would pay for it after the fact.

This concern was shared among panelists, as well. Ms. Wilder described how her daughter's disease limits her social activities. Before her daughter can go to a party, they have a lot to consider. “Is this worth it? How's this going to affect you the rest of the day?”

All three parent focus groups spoke to school challenges; although not necessarily reflective of every JIA family's experiences, kids represented in these groups missed school frequently or were home-schooled in extreme cases. Even when there was normal attendance, modifications were needed, such as additional time to move between classes and adapting physical education class.

Panelist Harley Powell, a 21-year-old with systemic JIA, recounted a painful middle school experience. Her aching and damaged hips caused her to use a mobility scooter at school. However, the elevator at school frequently broke. Rather than calling the elevator repair every day, the school

Focus Group Summary on Life Impacts



Young Adults	Parents
More focused on joint symptoms and related functional losses.	More focused on mental and emotional impact (manifested as depression or anxiety in older children; “meltdowns” and acting out in younger children).
Shared concern with parents regarding disease impact on participation in sports and other extra-curricular activities.	Shared concern with patients regarding disease impact on participation in sports and other extra-curricular activities.
“Invisibility of the disease” and lack of peer understanding affects school and social interactions and experiences and fuels guilt about “letting people down.”	“Invisibility of the disease” fuels misunderstanding about disease limitations among students and other parents. Children respond with self-imposed exclusion from activities or “pushing through.”
More focused on career prospects, on-the-job flexibility and accommodations, driving and use of assistive technology.	Concerned about “lost childhood” and the need to grow up quickly.
Concerned about comorbidities.	Concerned about reproductive health.

moved her class to the first floor. Ms. Powell described her horror, “Everyone in the class knew that they had to move it because of me. I can’t explain to you how embarrassed I felt. I wanted to melt to the floor.”

Panelist Nick Kim, a 17-year-old high school senior with enthesitis-related arthritis, had been an avid tennis player prior to his diagnosis a year prior to the meeting. He said that although giving up tennis was like giving up part of his identity, the social aspect of trying to fit in at school was even more difficult. He described having to drop out of Advanced Placement Calculus because his body couldn’t handle the rigorous work load. He even had to postpone taking the SAT because sitting in a chair and holding a pencil for four hours was too taxing.

“

Pain is pain, but what hurts even more for me is the emotional burden and the social aspects of adjusting to this disease.”

– Nick Kim, panelist

Loss of Mobility

Pre-meeting survey respondents listed “reduction in ability to walk, run or stand” as the symptom with the second biggest impact on daily life, behind pain. Among focus group members, swelling, stiffness and limited range of motion were among the most commonly cited symptoms with the most impact on daily life. Similarly, panelists and audience members frequently mentioned limitations in their daily lives due to loss of mobility.

Ms. Pinter described how her daughter struggles to get dressed because features such as buckles, shoe laces, zippers and buttons are difficult for her due to decreased mobility and flexibility in her fingers.

Audience member Tammy described their victories, “When he can ride his bike, when he can do full days of school, or mow the lawn – for us, those are victorious days. The same way we would cheer for an athlete – we’re like, ‘Yes, he had a normal day.’ And we’re gonna pay for it for two weeks, but ‘Yay!’”

Ms. Vago’s younger daughter, Erin, is a theater major in college. She recently got a job working in theater production, but accessibility is a concern. The technical booth is located at the top of three flights of stairs, with no elevator.

“

On days when I am going to be a stage manager, I have to plan very carefully how I spend my energy through the day. Right now, one of my greatest fears is that I won’t be able to get up those stairs and call the show.”

– Erin Vago,
audience participant

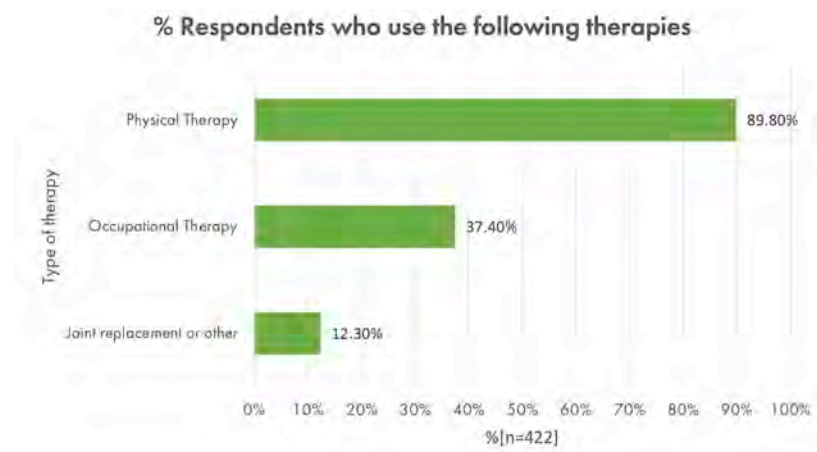


Topic 2: Patients' Perspectives on Current Approaches to Treating JIA

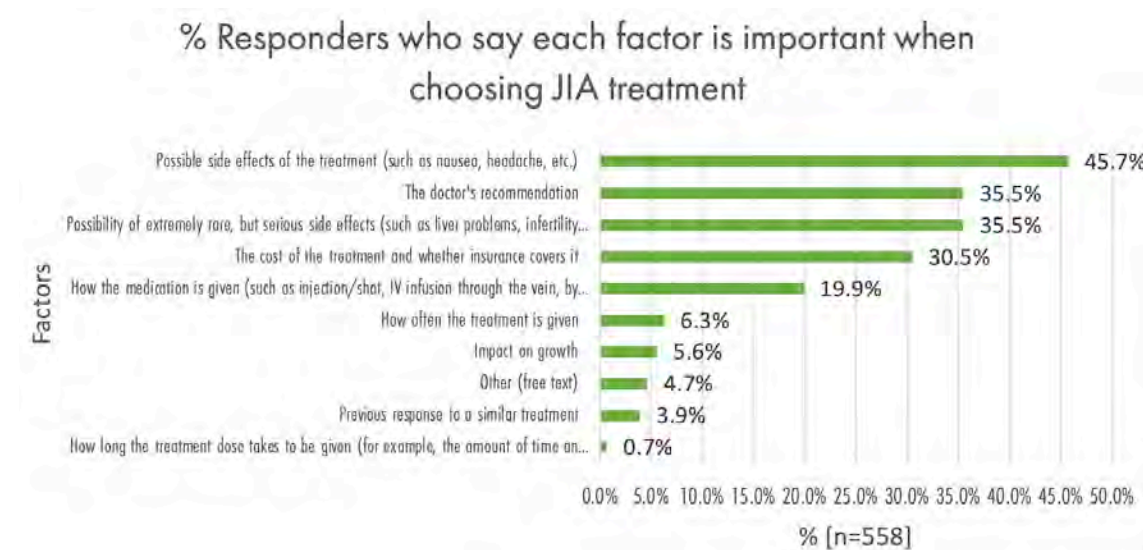
For a child diagnosed with JIA today, the prognosis is better than at any time in history. With the variety of treatments available and the aggressive use of other therapies, many children with JIA live healthy, active lives, and can avoid the joint damage and severe disability that was common a generation ago. Some people can even achieve remission, which is the ultimate goal of treatment.

Of the survey and poll respondents, everyone has taken at least one prescription medication for treatment of JIA. Thirty-one different medications were used at one time or another by survey respondents. Joint surgeries were needed for 52 (12 percent) of survey takers.

Three-fourths of pre-meeting survey respondents integrate non-medicinal therapies into their JIA disease management plans. Physical therapy is most commonly used among those who use non-drug therapies.



When choosing a treatment, the most important factors weighing on the decision for survey respondents were side effects, doctor recommendations and insurance coverage/medications costs.



Families are grateful for the improvements in JIA medications that have been made over the years. However, patients and families are still faced with unsolved issues regarding the availability and efficacy of treatment options. Survey respondents, panelists, focus group members, and audience

participants all noted limitations of current drug treatments. This came up about their ability to control symptoms, as well as issues with administration, side effects and long-term effectiveness.

Focus group members generally described an “ideal” drug as one with oral administration, reduced frequency of treatment, and few to no side effects. Panelists made similar comments.

Melanie Kohlheim, whose daughter was diagnosed at 20 months with polyarticular JIA, described the difficulties her daughter has faced not only with the disease, but also with finding a treatment that wasn’t scary or had many side effects.

“

An ideal medication for us would be something that doesn’t hurt and doesn’t require a Band-Aid. Something that isn’t an infusion or an injection; a medication that doesn’t cause painful oral ulcers, or daily tummy aches. A medication that doesn’t scare families fearing the future of health insurance and healthcare due to the high price tag.”

– Melanie Kohlheim, panelist

One audience participant described what he sees as a disconnect between what the medical companies view as a success versus what parents feel is a success.

“

’Good enough’ is not good enough for those of us who have to deal with this day in and day out.”

– Corey, audience participant

Uncontrolled Disease Symptoms

Although 87 percent of survey respondents said reducing pain is the most meaningful outcome of a drug therapy, pain was the symptom mostly commonly cited as not satisfactorily addressed by current therapies. One audience participant described seeing no relief from pain and inflammation despite taking multiple medications.

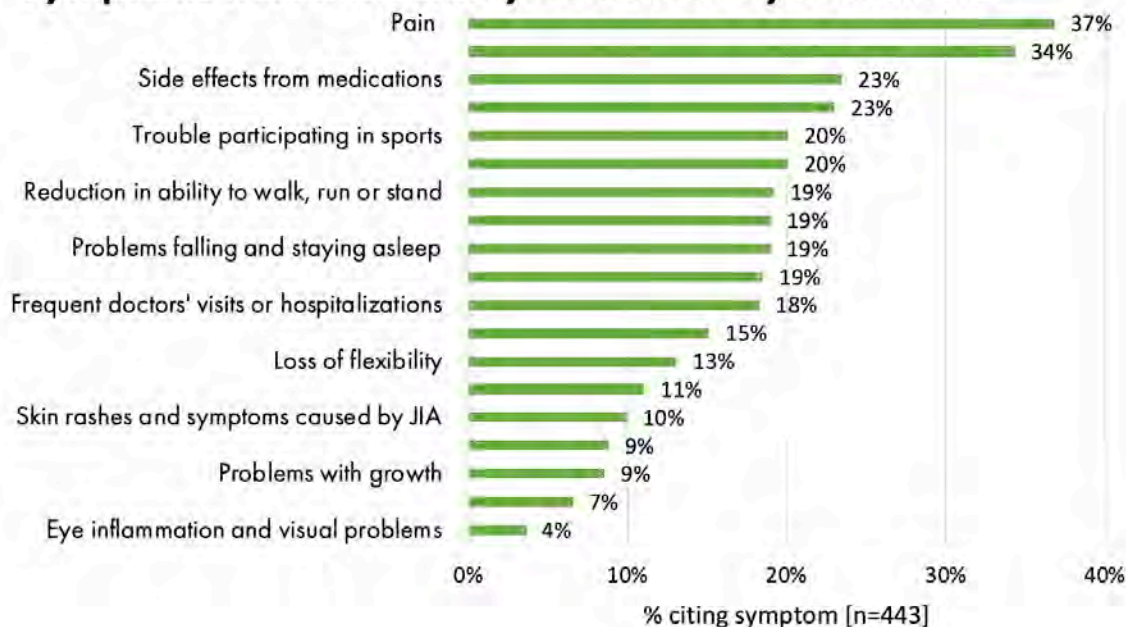
In general, very few people within the focus groups indicated that their current treatments had led to a medicated remission. Those who reported benefit from treatment noted there were still breakthrough symptoms.

Of 44 in-meeting poll respondents, 17 (39 percent) indicated they have not been able to gain control of their disease and 18 percent have tried more than 10 medications.

Another unmet need cited by participants are treatments targeted to specific JIA subtypes or symptoms.

Ms. Vago and her daughter Erin described frustration with the lack of treatment options for arthritis in the temporomandibular joint, which both her daughters have. Erin recently had jaw surgery with braces installed to hold her jaw in place. The braces do not allow for the doctors to monitor her jaw

Symptoms not satisfactorily addressed by treatments



arthritis using MRI. Erin explains, “So I’m going on a new drug but we have no idea if it’s gonna help the jaw. And not even knowing the status of my jaw right now, other than the fact that it’s ‘messed up,’ is absolutely terrifying.”



Not one of the drugs on the market today has been found to be effective in treating arthritis in the jaw. ... Compared to adult RA patients, JIA patients see jaw disease at a much higher rate.”

– Anjie Vago, panelist

This perspective was shared by participants during the Q&A session. One commenter who has encountered a lack of effective treatment options for both enthesitis-related arthritis and arthritis of the jaw, talked about struggling to find treatments that control all her symptoms. “I’m on my last biologic. My only hope after that is some kind of DMARD, or maybe a psoriatic drug.”

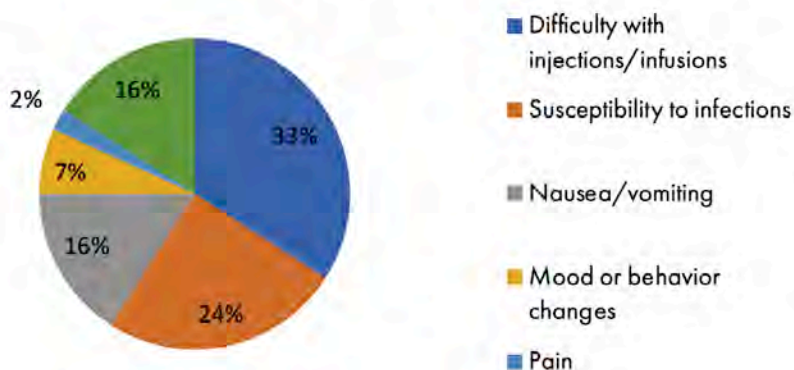
Panelist Rochelle Lentini’s son has been on several medications trying to chase multiple manifestations of his systemic JIA. She says, “He’s on his tenth biologic. And our biggest concern is that the biologic that’s he’s on only targets one area. And for him, we feel like he needs multiple biologics.”

Drug Administration Problems

Between needle phobias, issues related to infusions, and other injection-related traumas, problems with drug administration was a clear theme among survey respondents, focus groups, panelists, and audience participants.

One polling question asked the audience to list their most troublesome side effects from treatment. The top answer was needle phobia and issues with injections or infusions. Similarly, the top treatment downside among the parent focus groups was injection trauma.

Most Troubling Side Effect of Treatment (n=45)



Panelist Kate Kuhns, whose 15-year-old daughter Delaney was diagnosed at the age of six, described how years of infusions have been physically challenging for her daughter. For the past five years, her regimen has included monthly infusions with labs in between. Ms. Kuhns describes their appointments, “She was blowing veins every time, to the point she has a rule: you can do one arm one time, one arm the next, and after that the Life Flight team has to come down and put it in. We don’t do hands, we don’t go anywhere else. We were blowing veins too much.”

Although Ms. Kohlheim was able to find a treatment that works well for her daughter, she noted that because it’s an infusion, the treatment itself can be a burden. In addition to the pain her daughter experiences during these treatments, it also requires her daughter to take a half day off from school. Jacqueline Peña, whose 13-year-old son, Jacob, was diagnosed with polyarticular JIA when he was eight, described her son’s history with biologics. He is currently on his fourth one, which he receives intravenously every four weeks. The stress of infusion days can be overwhelming for both her and her son.

“

He has a needle phobia, so those days are stressful, they’re overwhelming, and you would think after three years they would get easier, but they don’t.”

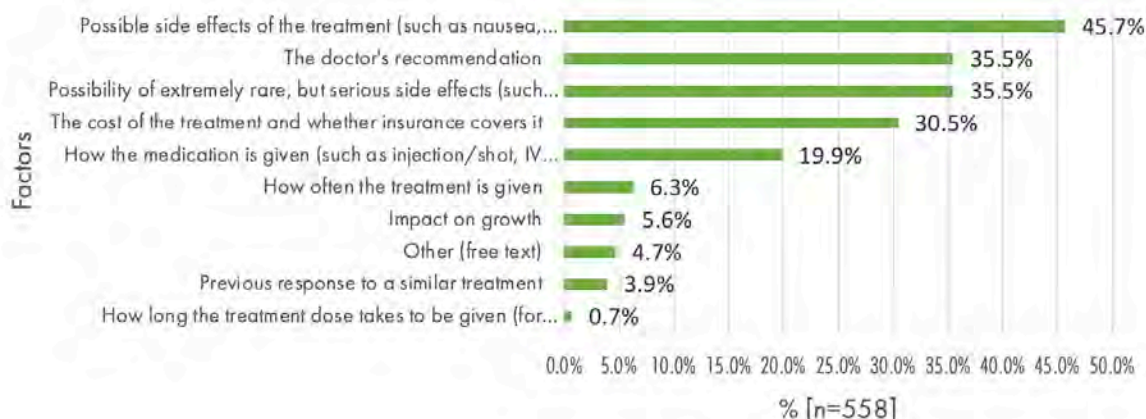
– Jacqueline Peña, panelist

Both of Ms. Vago’s daughters struggle with infusions, as well. “It’s hard to find a vein. You know, you go in and you have stick, after stick, after stick. And what kid wants to keep doing that? What adult wants to keep doing that?” While her daughter Laura now has a port that is making treatment a little more tolerable, she’s having trouble getting one approved for her other daughter, Erin.

Drug Side Effects and Vulnerability to Infections

Survey respondents were asked to choose which factors are important to consider when choosing a treatment for JIA (“Which of the following are most important to you/your child when choosing a treatment for JIA? Select up to two”). The majority of respondents cited side effects as an important consideration. Side effects were a commonly discussed problem among focus groups, panelists and audience participants as well.

% Responders who say each factor is important when choosing JIA treatment



One polling question asked what treatment side effect was most troubling for participants. Only two percent reported they had experienced no side effects. Nausea and vomiting were among the top responses. These side effects were also the most common among focus group members, especially those who had taken methotrexate.

Some people described side effects as being nearly as troublesome as the disease itself. Ms. Kohlheim shared how her daughter experienced oral ulcers as a result of DMARD treatment, causing her to stop treatment and try again—only to discover that even at a half dose, the side effect was unavoidable.



We deal with more side effects of medication than we do burden of disease.”

– Melanie Kohlheim, panelist

She also described having to constantly weigh risks against benefits to make decisions about her daughter’s treatment plan. “The other issue with these medications that are keeping her arthritis quiet is that we have immune suppression issues. We have challenges that you don’t normally think about. So if my daughter were to get strep throat, do we skip the biologic? Do we [postpone] the biologic, do we skip the DMARD? Do we push the DMARD out a day?”

Focus groups frequently discussed the increased susceptibility to infections as a major downside of immunosuppressive therapy. This was a commonly cited issue among panelists and audience participants, as well.

Ms. Peña noted that immune suppression issues affect her whole family. Her youngest son has to miss out on school and activities to prevent exposing his brother to infections. “Our youngest is also paying the price of this disease. He does not participate in any extra-curricular activities. He doesn’t go to school anymore either because the risk of him bringing home a virus, a bacteria, or some kind of illness is way too overwhelming. I kind of feel like we live in this bubble at home. And the fear of going out and being exposed to something because he’s so immune suppressed is terrifying.”

Broader Indications

Several people noted difficulties with getting insurance approval for drug treatments due to narrow sets of indications and age requirements. Medications that may work for and be approved for use in adults aren't approved for children; or drugs that work for one type of juvenile arthritis aren't approved for use in another type.

Ms. Kuhns shared difficulties she's faced with getting her insurance to cover drugs to treat her daughter's oligoarticular JIA, which is not an FDA-approved indication for biologics. Even though her daughter had been on a biologic for five years, her request to change the mode of administration was denied by her insurance company. She was eventually able to get it covered on her third request to the drug company's assistance program, but the experience demonstrates how difficult it can be to navigate treatment for various disease subtypes. Ms. Kuhns believes that "the indications and the usage need to be broader."

Ms. Kohlheim described similar difficulties with getting drugs approved by her insurance company when her daughter was still a baby. The first biologic she requested was declined, but they suggested another in its place. "And then they took four months to approve their own suggestion."

An audience member commented that despite finding a drug that is effective for her daughter, they will have to pull her off the medication once she reaches remission. Her doctor was reluctant to prescribe the drug at all, she said. "And because there's not enough research to suggest that it's effective and safe for children to take long term, he's adamant about ceasing the medication once remission is reached."

“

It's very, very scary as a parent. I just feel like there needs to be more research in how long our kids can be on the medicine. If it is effective, why do we need to stop the medication? Is it safety driven, or money driven, you know?"

– audience participant

Children often have even fewer treatment options. Ms. Kohlheim noted that she works with many families who run out of treatment options in patients as young as six years old. "Sometimes the FDA has only approved medications for patients over a certain age, or only in adults and not in kids."

Ms. Peña expressed frustration at seeing her son face stressful intravenous infusions every four weeks with no relief from his symptoms. And because many drugs are not yet approved for use in children, she fears he will soon run out of treatment options.

“

The worst part about it is that nothing is helping, and we are running out of options. We really need new treatments and new medications. Just at the age that he is, he's not old enough to try the adult drugs, so he can only do a certain number of biologics. It's frustrating and overwhelming to know that I can't do more for my son."

– Jacqueline Peña, panelist

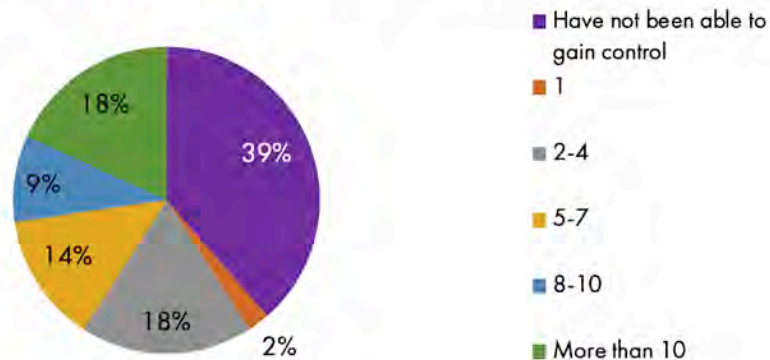
Running Out of Drug Options

Most survey respondents, focus group members, panelists, and discussion participants said that they had cycled through multiple drug therapies and still were not satisfied with treatment.

Pre-meeting survey response shows that over 40 percent have taken three or more medications for their JIA, and 18 percent have taken five or more. Considering the young ages of JIA patients and that in some cases, those who have cycled through multiple therapies have only been diagnosed a short time, this aspect of JIA management is daunting to many families. Also of note, the pre-meeting survey only covered numbers of DMARD and biologic therapies, not NSAIDs, steroids, or other widely-used medications.

The in-meeting survey question “How many treatments have you/your child had to try in order to get control of your JIA?” revealed that 17 of the 44 (39 percent) respondents had not been able to gain control of their disease, and 8 (18 percent) had to try more than 10 therapies to gain control.

Number of Treatments Tried (n = 44)



Methotrexate was the most frequently reported drug therapy among survey respondents and was also heavily discussed among focus group members. Although most people in the focus groups had been on at least one biologic, there was a wide range of treatment experience, from patients who had not yet tried biologics to those who had cycled through every biologic available to them. In fact, several people said that they had run out of options or feared they soon would.

Panelists and commenters described moving from one drug to the next because of newly developed comorbidities that weren't being addressed, unbearable side effects, or because the drugs lost their effectiveness over time.

The prospect of running out of medication options can be particularly troublesome for children diagnosed young. The pre-meeting survey revealed that for those diagnosed under 10 years of age (n = 373), 33 percent had taken three or more medications, 29 (8 percent) had taken six or more, and three had taken 10 or more medications. Considering again that the survey was specific to DMARDS and biologics, the total number of medications tried by these recently diagnosed patients is likely higher.

Audience member Nicole described her daughter's experience searching for the right medicine. “We tried a DMARD, and that went okay for about nine months. Then she developed an allergy to it so we

had to stop. We tried another DMARD, which did nothing. She got worse. And so they took her off that and put her on her first biologic, which we thought was our miracle drug. Her joints got better, she was doing really well for about a year or so. And we went to the eye doctor for a regular checkup and he said, 'Oh you have nothing to worry about. She's on this drug and she hasn't had anything in two years, so we're good.' Then he looked in her eyes and she had uveitis, both eyes. So that started the roller coaster. She was three."

Ms. Kohlheim described their experience with a biologic working, but then becoming ineffective.

“

Biologic number one worked like a magic wand. It was absolutely amazing. ... She could run, and she could play, and she could jump, and she was sleeping through the night. Unfortunately, that paradise only lasted for about two months, and she flared again. At that point we were ready to try biologic number two.”

– Melanie Kohlheim, panelist

One audience member shared an emotional account of how, after exhausting all her treatment options, she chose to go back to an infusion that had worked in the past but had given her severe respiratory side effects. After another rough start on that treatment, she eventually chose to push through the infusion despite potentially life-threatening side effects. Despite that, the treatments aren't helping her to be fully functional, and she's out of options. "And to have nothing left, you can't imagine what it's like."



Topic 3: Patients' Perspectives on Clinical Trial Design

“

My greatest wish is that we could change the way clinical trials are done to meet the needs of the patients.”

– Andrew Curtis, panelist

Panelists, audience participants, survey respondents, and focus group members laid out several considerations they felt would improve the clinical trial process for people with JIA to help achieve the goal of releasing innovative new treatments. Suggestions included better patient education about clinical trials, a quicker completion time, more diverse patient pools, outcome measures relevant to patients, trial designs that give the best chance for the patient to benefit, and patient inclusion in protocol design.

Across the five focus groups and the meeting audience, there was very limited experience with clinical trials. Of the 39 focus group members, five or six had clinical trial experience. Just three of 41 in-meeting poll respondents had participated in a clinical trial. For those who had not been in a trial, 65 percent said they were never given the opportunity.

“

It would make me more willing to do a clinical trial if I were walked through the process and know what would happen. Like, have a Plan A, a Plan B; know exactly what would happen before I get myself into it.”

– Ellie, audience participant

Out of Options

For those who had participated in a clinical trial, all cited running out of options as a key reason for joining.

Panelist Rochelle Lentini, a mother of two kids with JIA, described her younger son's experience with clinical trials. Now 19 years old, Parker first experienced symptoms at age eight, and enrolled in a clinical trial at age 12 after multiple hospitalizations and surgeries. Despite knowing there might be a risk her son could be chosen to receive a placebo, Ms. Lentini was willing to take that risk if it meant it might help her son.

“

When you have no options left because your kid is so sick, you're willing to try anything.”

– Rochelle Lentini, panelist

Andrew Curtis is an 18-year-old college student who was diagnosed with JIA at age two. When, at 13, a biologic didn't work for him, it was time for his family to look for new options. "At this point, it was 11 years into my disease, and we were running out of options due to the lack of medications." After being reassured he would not receive a placebo, he and his parents ultimately decided to try a clinical trial.

Vince Del Gaizo is a father of three whose son was diagnosed with JIA at a young age. After staying in remission for seven or eight years, his son experienced a systemic flare that affected him profoundly. In addition to mobility limitations in both wrists and an elbow, he also developed a debilitating rash. After struggling to find a medication that could treat JIA with systemic manifestations, he decided to try a drug trial.



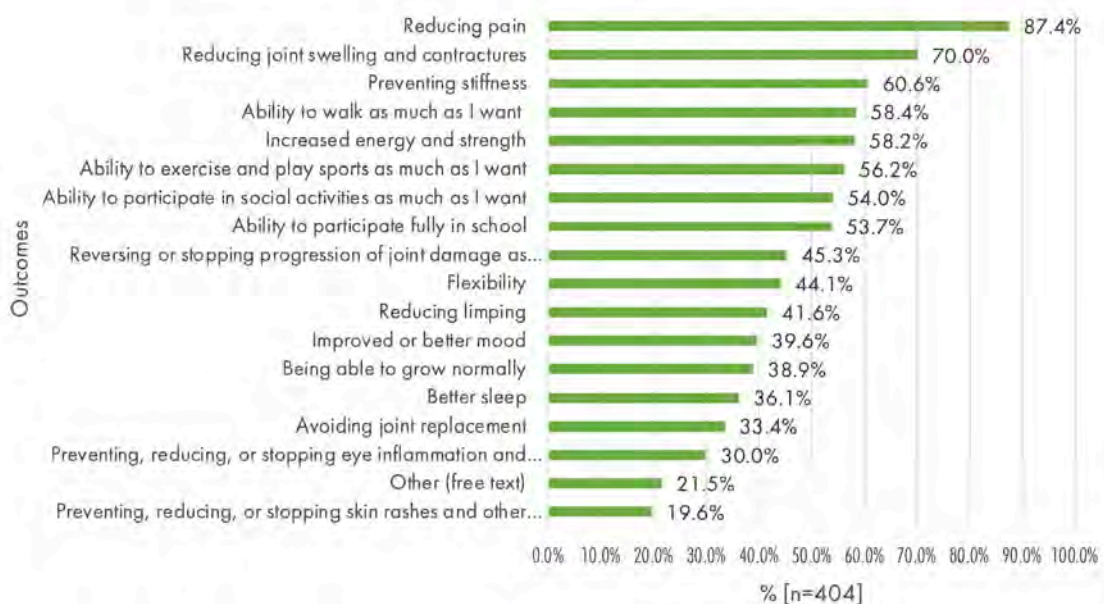
At the time in 2009, medications approved for JIA didn't really work well with systemic disease, and that was his most important outcome – to get rid of that rash. So we learned about this study that was going to help with his systemic manifestations of this disease, and his joints, and everything else.”

– Vince Del Gaizo, panelist

Across all five focus groups the sentiment was reticence to be involved in a clinical trial unless the patient is “out of options.” Young adults expressed little to no willingness to “gamble” on a trial if they were stable on current therapy but indicated that they would consider it if they had run out of options.

Outcome Measures

Survey respondents were asked to choose all the most meaningful outcomes for drug therapy for them. Pain reduction (87 percent), reducing joint swelling and contractures (70 percent) and preventing stiffness (61 percent) were the three most frequently selected outcomes patients are looking for from drug treatment.



Placebos

Panelists, audience members, and all five focus groups expressed strong feelings about placebo-controlled studies and withdrawal trials. Most said they'd only be willing to participate if they were guaranteed they wouldn't receive the placebo, while others said they'd be willing to try if the placebo periods were kept short. While the option of using an active comparator was seen as an improved scenario, it was still not considered a viable option for those patients who were stable on current therapy.

Mr. Curtis shared that even as he ran out of treatment options, his parents were reluctant to enroll him in a study. Eventually they agreed to let him participate, but only after they were assured he would not be receiving a placebo.

“

We were not willing to risk receiving a placebo and having my JIA continue to flare and lead to more joint damage than I already had.”

– Andrew Curtis, panelist

During the Q&A discussion, one commenter said that receiving a placebo could potentially put her daughter in a life-or-death situation, so she wouldn't be willing to try a drug trial unless they were guaranteed she would not receive a placebo.

“

The placebo is a really scary thing. Having the chance that you're on no medication, and maybe not even knowing that for a while, is a really scary thing to think about, especially as a kid.”

– Kate, audience participant

When Parker Lentini realized he was on placebo during the trial he participated in, the doctor gave him an out and said he could leave the trial. But Parker put the health of those who may come after him before his own health.

“

I know the cure is not here, possibly, in my lifetime, but if this can make a difference for children in the future, then I must do this.”

– Parker Lentini, audience participant

Parents in the focus groups said that in placebo-controlled trials and withdrawal studies, the potential length of the placebo arm was a major factor in the decision to participate. They also expressed concerns about the possibility that if their child participated in a withdrawal study in which their child responded initially to the drug and the drug were removed, it would ruin the chance to get a response from a drug again later.

Although Mr. Del Gaizo's son experienced a positive outcome from a drug trial, he acknowledges that withdrawal studies present an ethical challenge when the study removes access to a drug that's working for a child. He said that if it had been a withdrawal study, he'd have had a very difficult time removing the drug. He went on to say that he would have had a much harder time enrolling him in a placebo-controlled trial "unless the placebo period was really, really short."

Inclusion/Exclusion Criteria

Several panelists and focus group participants talked about inclusion and exclusion criteria for clinical trials. Panelists generally understood why some limitations exist but suggested that when trial participants don't have comorbidities, then patients with comorbidities can't make educated decisions about whether the drug is right for them. Parents also said that when drug trials aren't available for children, then treatment options for children won't increase.

Mr. Del Gaizo expressed concerns about the selection process for drug trials. Because his son's systemic disease sometimes includes bouts with psoriasis, and patients with psoriasis could not participate in the study, he had a lot of anxiety that his son would be ineligible for the study. Although he knew why it would be important to limit variables in the study, he suggested that limiting which patients could participate would also make the results unrealistic and not applicable for patients in the real world, who have comorbidities or who don't fit the exact inclusion criteria for a trial.

“

You're looking for a cohort of patients that doesn't exist. And then when you do prove the drug works in that select cohort, that patient is nothing like my son. So I still don't know if it will work in my son because my son is nothing like the people that you recruited for the trial.”

– Vince Del Gaizo, panelist

Liz Smith, a panelist with two daughters who have JIA, said that she would encourage her daughters to participate in a drug trial if they were eligible, but their comorbidities often disqualify them. "Emily has multiple, Sophie has two, so they're excluded from most trials before they can even get a foot in the door."

Ms. Powell expressed deep concern about the lack of drug treatment options available for children. Because of what she went through as a child with JIA, she is motivated to help find solutions so other children won't have to wait like she did to find a treatment that helped.

“

To the FDA and the pharmaceutical companies, I would ask for greater access to clinical trials for children. One way that this can happen is through more open inclusion/exclusion criteria so more kids are able to be included in trials.”

– Harley Powell, panelist

Expedited Process

In addition to requesting broader selection criteria for drug trials, several panelists commented on the amount of time it takes to be selected for a trial, and then how much longer it can take to complete a trial so the drug can reach the market.

Although he's grateful to have had a positive experience with his drug trial, Mr. Curtis shared his concerns about how long it took for the drug to be approved for the market. "I feel that five years is almost too long for other kids to wait to have this medicine."

Ms. Powell had similar concerns. She said she waited five years for the drug she's on now and loves. While she understands why drug developers are cautious about releasing a drug, she believes that five years was too long to have to wait while suffering. She wants drug developers to understand that there is an urgent need for children to have faster access to drugs that might help them.



We need more options available and we would like there to be an expedited review process for these kids, especially the children who have multiple issues. And then the multiple issues are also a concern, because then they get excluded from studies."

– Rochelle Lentini, panelist

Patient Input

From suggestions about how trials could be improved, to statements about the value of patient and parent input, focus groups and panelists expressed strong feelings about what they could offer if they were included in protocol design.

A topic of discussion among focus groups was the issue of pain tolerance and requests for adaptability in how medications can be administered. One panelist offered similar comments. Ms. Powell described a childhood spent enduring painful and invasive treatment methods and suggested that pharmaceutical companies should take age into consideration for delivery methods, as some methods can be tolerated differently by different age groups. She went on to suggest that drug developers should partner with patients and families to design clinical trials that are easier to participate in.

One audience participant explained that when her young child was put on a new pediatric formulation of a biologic, the packaging and needle looked the same as the original. The child was afraid it would hurt as much as the adult formulation she had been on. The mom tried to explain it to the child, but changing the look of the pediatric packaging could have made the experience easier for the family.

Mr. Curtis talked about the frequency of and distance to his follow-up appointments. "I was followed very closely to monitor me for adverse reactions. This involved regular appointments on a very specific schedule. We had to drive an hour each way to see our rheumatologist and couldn't miss an appointment because that's when I would receive my medicine. ... As I entered high school, many times, keeping my appointment made me miss exams and other school activities. ... There were times when I considered withdrawing from the trial because it became more and more challenging to go to the appointments on specific dates. But I stayed in the study because of how much the medicine was working."

the patient voice in drug trials to prevent children from developing aversions to specific aspects of treatment, such as color and smell.

Focus group members also had suggestions for making trials more appealing to parents and patients. Many expressed a desire to have more control in the process, such as the ability to select which arm their child would be in, as well as the ability to pull a child out of a study when they had concerns. Voiced most loudly by parents of children with sJIA – but endorsed by others – was the idea of a “liberal escape pathway.” They want intensely frequent monitoring during a trial with a very rapid response if it is determined a patient is not responding to the provided therapy. They also want assurance that they won’t somehow be “punished” or “looked down upon” for exiting the trial before its official end.

Meeting participants also expressed concerns about pulling their child out of a trial. Dr. Hermine Brunner from Cincinnati Children’s Hospital Medical Center was called upon to help clarify the

“

Any time you’re thinking about how to get children and parents interested, you’ve got to think about the patient voice and the fact that we have experiences that can be valuable in the process. We can help that clinical trial be more receptive, not just to the parent and to the doctor, but also to the patient.”

– Rochelle Lentini, panelist

situation for the meeting attendees. She explained that all clinical trial consent procedures allow for the patient to withdraw at any time for any reason. “It says in the consent form, if you don’t want to do it anymore, you are free to go away and stop with the whole trial.”

Mr. Del Gaizo suggested that patients could offer important insight regarding ethics requirements and the criteria that patients and parents consider when deciding whether to enroll in a study. “We need quick access to safe, effective treatments, and it needs to be done in an ethical way. I don’t have the answers. But what I do think is that a possible solution to this is to have some flexibility and to involve all stakeholders in designing the protocol.”

Focus group participants resoundingly endorsed participation in observational research and the release of that information to patients and families. One group spoke to the need for more patient-specific and patient-relevant data to be collected during the clinical trial process. They would like that information to be captured and shared with families trying a drug for the first time.

FDA Engagement

“

Patients are often experts in their conditions. The ability to hear what patients with JIA care about can help us understand what aspects of the disease are most important to patients and how patients view the benefits and risks of therapies and devices for JIA. This knowledge can then help facilitate the development of future treatments.”

– Rachel Glaser, MD, Food and Drug Administration

Through these PFDD meetings and other initiatives, The FDA seeks to strengthen and expand their links with patient groups to include them at earlier stages of discovery and the drug development process. PFDD meetings also shine an important spotlight on the impact of disease on personal, social, emotional and professional experiences. CARRA leadership felt that the meeting was an excellent demonstration of the importance the FDA places on giving patients and families a voice to ensure that all stakeholders better understand what it is like to live with these diseases, take these medicines, and have needs that are not being met. They outlined the next steps for all stakeholders, to synthesize these issues, continue these discussions and create an action plan.

Understanding patient perspectives helps the FDA teams approach their roles with unique insight into the complex needs of the JIA patient community. Through these efforts, the agency will advance the development of new treatments that not only address optimal disease management but recognize the burden of treatment. PFDD discussions assist in moving the science of patient input forward, providing rich and nuanced disease management and lived experience context that will help guide medical product development.

“

We are appreciative of the FDA’s leadership in the realm of patient engagement in drug development and look forward to even stronger collaboration between patients, clinicians, researchers, industry and the FDA as well as continued improvement in research.”

– Ann Palmer, Arthritis Foundation



Treatment Horizon: Challenges and Opportunities

The JIA PFDD survey, focus groups, and meeting uncovered many challenges faced by families living with JIA. They also revealed opportunities for drug developers and the FDA to improve treatments for people with JIA.

Targeted Treatments

Clinicians, parents and patients agree on the need to develop targeted treatments for specific JIA subtypes. JIA is a heterogeneous group of diseases. Within the different subtypes, disease features and progression can be strikingly different and medication response unpredictable. Knowing what treatment to give each patient based on disease pathology would help chart a course toward personalized pathways of care, predictive therapy responses, limited adverse effects, improved outcomes and decreased comorbidities. Mapping the next generation of treatments to the differences in the various forms of JIA will help to guide therapeutic selection to attain and sustain remission in people with JIA.

Clinical Trial Process

According to PFDD participants, the clinical trial process could be improved to be completed more quickly, operate with a smaller but more diverse patient pool and use clinical trial outcomes measures that are most important and have meaning to patients. The desired result: a rapid increase in the number and types of innovative and safe treatment options that are more effective, targeted to specific diseases, easier and less painful to give, and have fewer side effects.

Integrating Patients in Protocol Design

Patient participation in research has come a long way. It used to only mean being a research subject. The path forward now means leveraging patient expertise and experiences to guide clinical trial design and inform drug development. The new paradigm should reflect the patient's perspective on the benefits and harms of treatment; ensure drug development programs accurately represent those benefits and harms; and prioritize protocols that are directly relevant to patient's treatment needs, decisions, adherence, and outcomes.

“

We are jointly committed to rethinking the clinical trial process to better serve the needs of all stakeholders and explore how to bring the patient voice into drug development.”

– Laura Schanberg, MD, CARRA



Arthritis Foundation/CARRA Research and Patient Engagement Efforts



We encourage academic and professional researchers to incorporate patient level insights at all stages of study design.”

– Guy Eakin, PhD, Arthritis Foundation

The Arthritis Foundation and CARRA are committed to providing many opportunities for families living with JIA to inform and actively engage in research, recognizing the intrinsic value of patient perspectives and insights. Some of the opportunities include:

PARTNERS

PARTNERS is a patient-powered research network fueled by a collaborative partnership that includes the Arthritis Foundation, CARRA, the Lupus Foundation of America, CureJM, and PR-COIN. Funded by the Patient-Centered Outcomes Research Institute (PCORI), PARTNERS puts patients and their families at the center of research efforts integrating their input, insights and individualized experiences in every phase of the process. Anyone with a connection to JIA will have the power to influence the research conducted and supported by PARTNERS.

Arthritis Trial Finder

The Arthritis Foundation has developed a searchable portal that makes it easier to find arthritis-specific clinical trials based on disease type and location. Participating in a clinical trial helps to accelerate the discovery of new therapies and products for arthritis. The information is procured from the U.S. National Institutes of Health clinical trials database (clinicaltrials.gov).

Rheumatology Learning Health System

Modeled after the Swedish Rheumatology Quality Registry, the goal of the Rheumatology Learning Health System (RLHS) is to develop a comprehensive picture of an arthritis patient’s disease and the impact of treatment. The tool will leverage existing registries, electronic patient health records utilized in clinical settings, and patient-reported health status and behaviors. Tracking, combining and analyzing this data will provide key insights on health care delivery and experience and help to improve the quality of care for people with arthritis, resulting in better health outcomes. The Arthritis Foundation leads this initiative by partnering with CARRA, PR-COIN, Understanding Childhood Arthritis Network-Canadian/Dutch Collaboration, the American College of Rheumatology and the Dartmouth Institute for Health Policy and Clinical Practice. RLHS is designed to facilitate ongoing information sharing between doctors and patients to ease the development of dynamic, co-produced care plans. This will help to empower patients to better manage their disease between doctor visits, improve the patient care experience through improved doctor-patient communication and advance shared decision-making dialogue.

Live Yes

As part of the Arthritis Foundation’s Live Yes! Arthritis Network, it has created the Live Yes! INSIGHTS Patient Reported Outcome program. Data collected through these surveys provide insights about participating individuals and allows scientists and researchers to better understand what works and what’s needed in local communities. By providing data over time, participants can track their physical health, social connectedness with other arthritis patients, communication with healthcare providers, as well as emotional health, stress, and depression.

Externally-led
Juvenile Idiopathic Arthritis
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Appendix 1

Project Team

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Parent of Child with JIA
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Arthritis Foundation

Anjie Vago
Parent of two children with JIA

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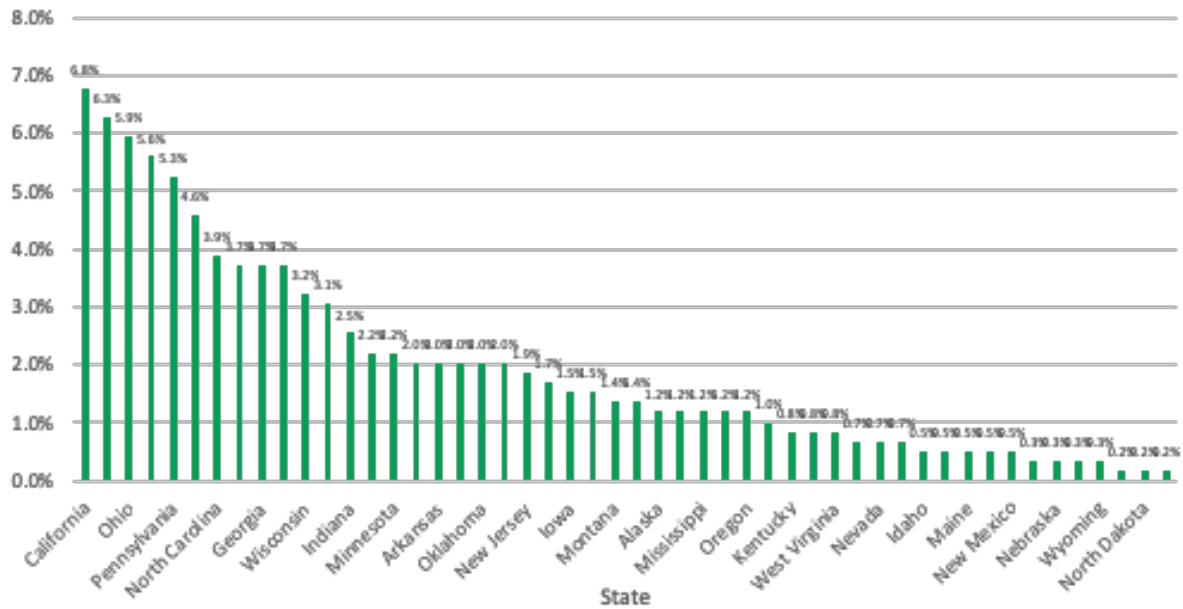
Appendix 2

Pre-Meeting Survey Results

Survey conducted June 25-July 13, 2018

Responses 589

States where respondents live

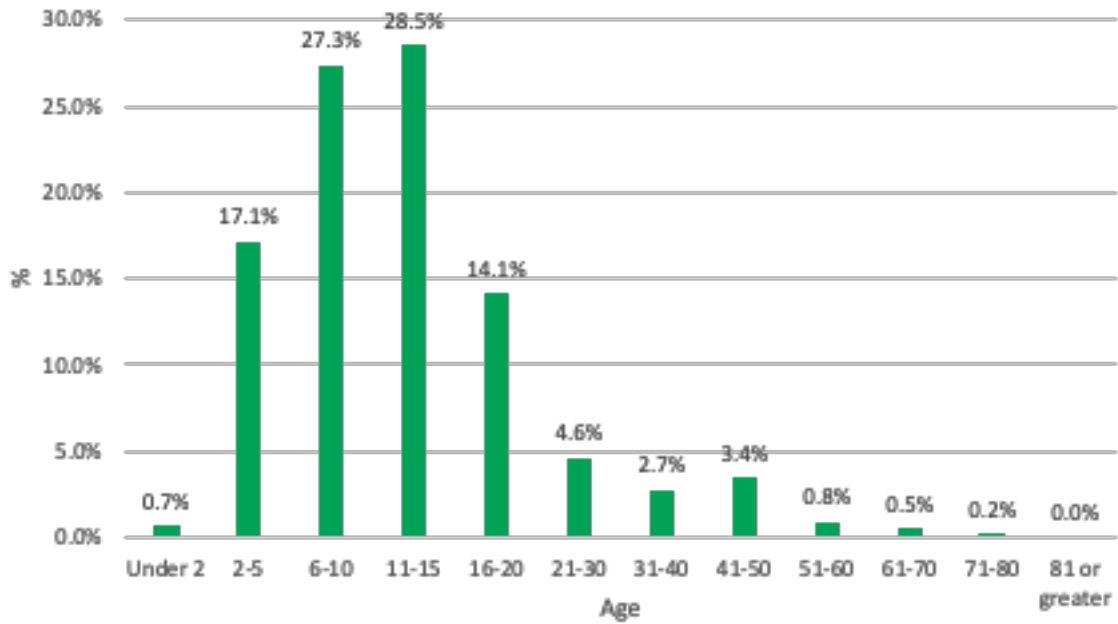


Demographics

Sex: 94.1% female, 5.1% male, .3% other

Age:

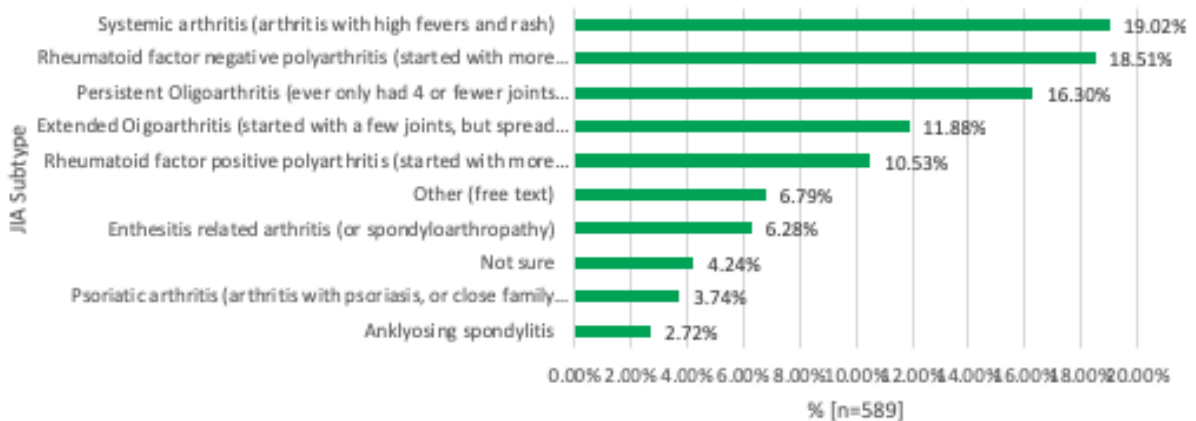
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Question - What type of JIA do you/your child have? n=589

- 88% of respondents are parents and/or caregivers
- 19% of those surveyed have systemic arthritis

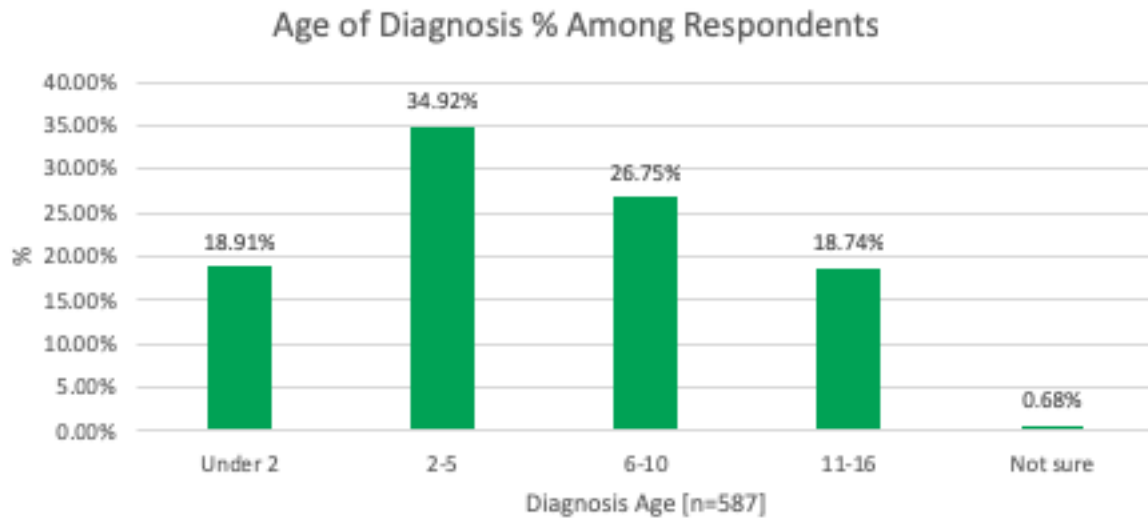
% Surveyed with JIA Subtype



Question - At what age did you/your child receive the diagnosis of JIA? N=587

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- 35% of those surveyed received a diagnosis between ages two and five

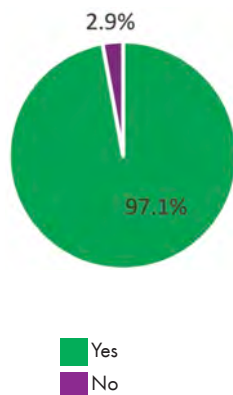


Question - Are you/your child currently seeing a rheumatologist? n = 586

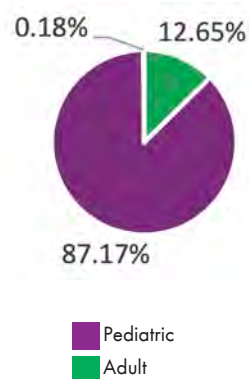
Question - What type of rheumatologist are you/your child seeing? n = 569

- 97% of respondents are seeing a rheumatologist; of those, 87% are seeing a pediatric rheumatologist

Currently seeing a Rheumatologist



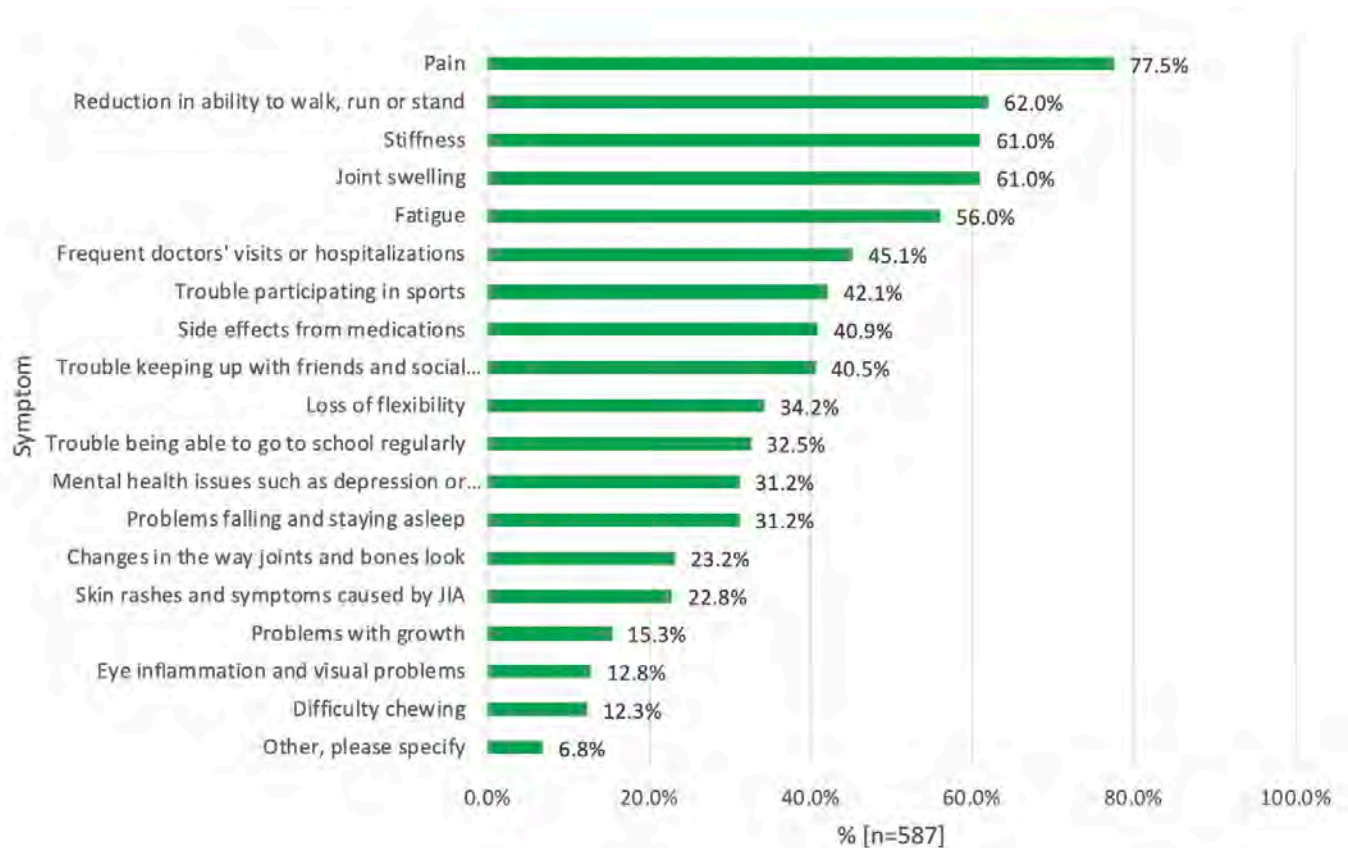
Type of Rheumatologist



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Question - Of all the signs and symptoms you/your child have ever experienced because of JIA, which ones have had the biggest impact on your/your child's daily life? (Select all that apply) n = 587

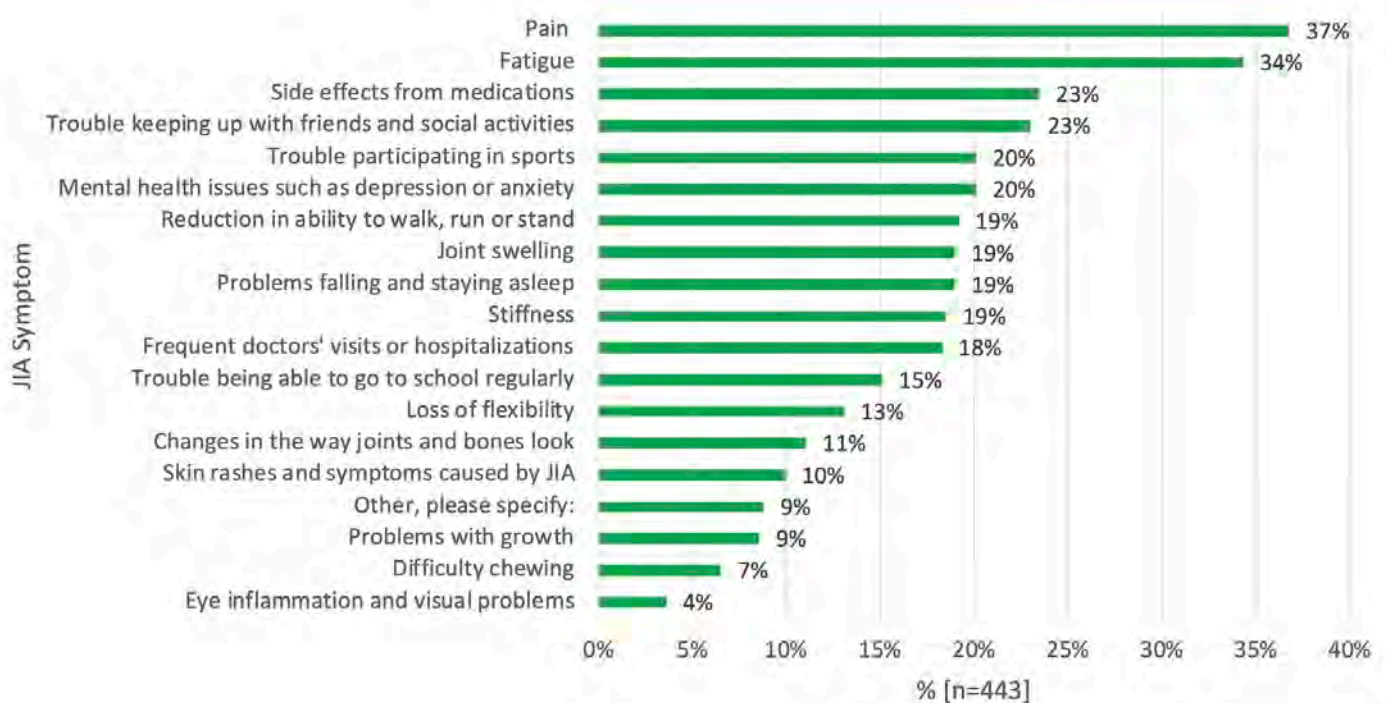
- 77% of respondents report pain as the symptom of JIA having the biggest impact on daily life



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Question - For the signs and symptoms you selected as ever having significantly impacted you/your child's daily life, which ones are not satisfactorily addressed by your/your child's current therapies? (Select all that apply) n=443

- 37% cite pain as a symptom not satisfactorily addressed by current therapies; 34% say fatigue

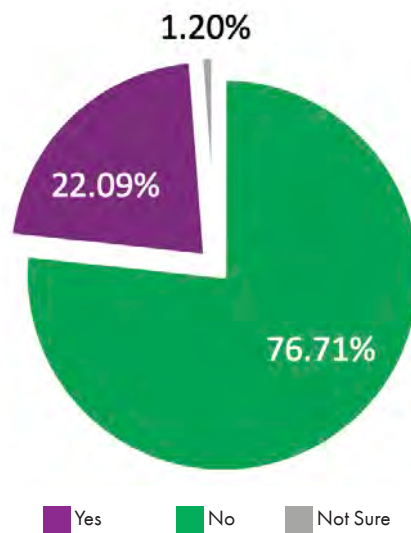


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Question - Have you/your child ever used anything other than prescribed medications (such as massage, acupuncture, herbal therapies, supplements, or hot or cold packs) to help reduce your/your child's signs or symptoms of JIA? n = 584

- 77% of those surveyed have used non-drug treatments to help reduce the signs and symptoms of JIA

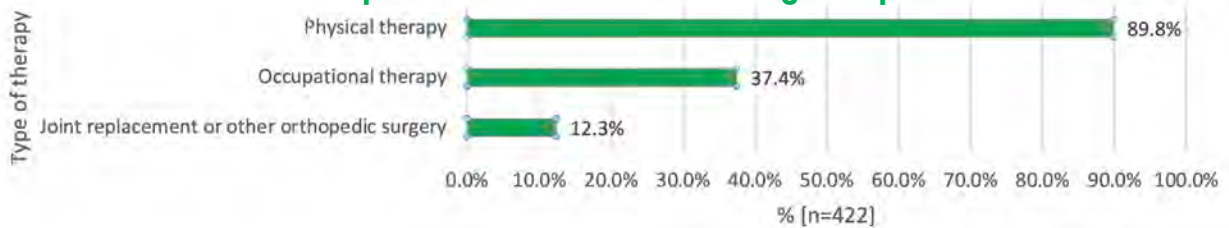
% Respondents who use non-medication therapy



Question - Have you/your child ever used any of the following therapies? n = 422

- 90% of respondents have used physical therapy

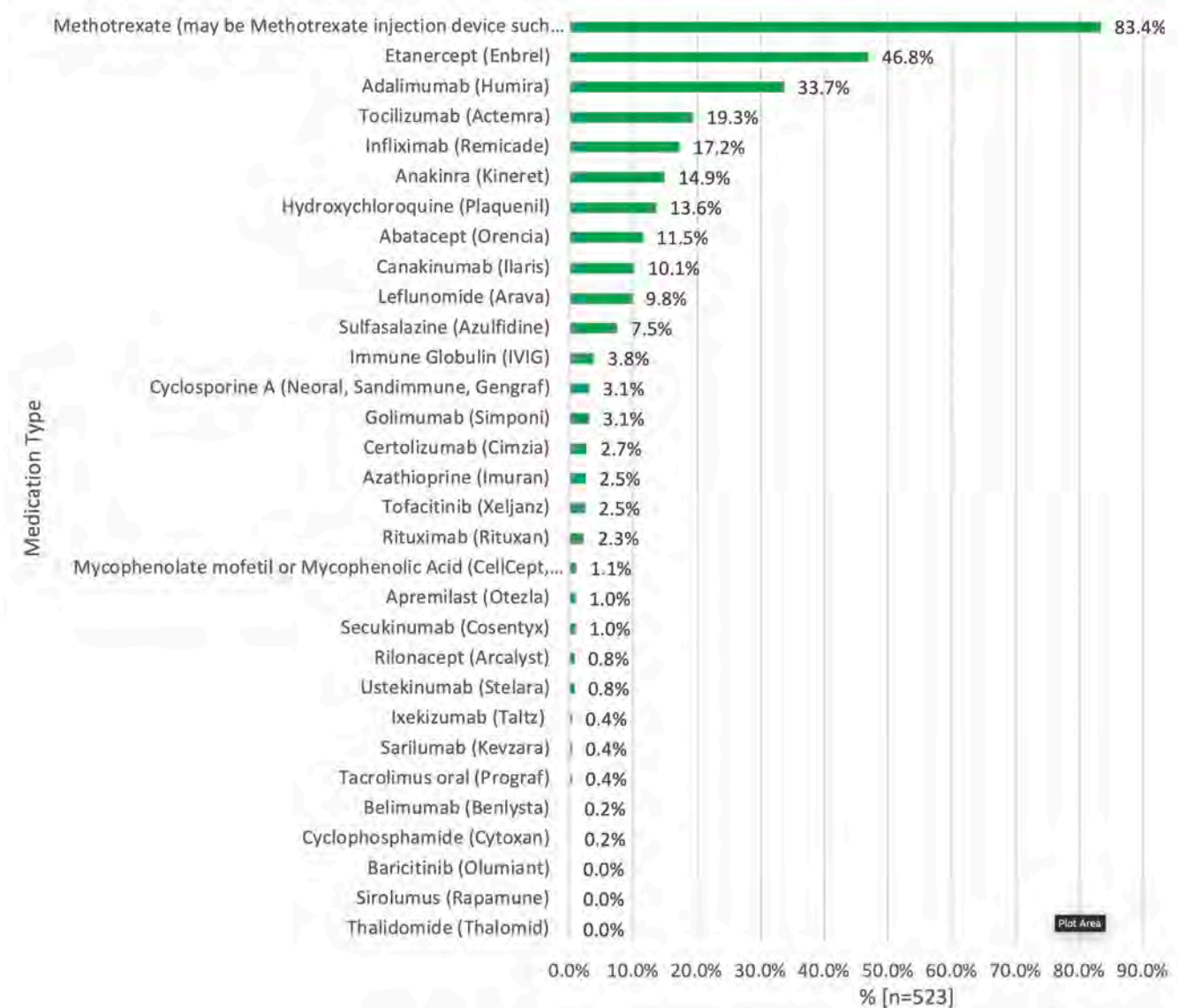
% Respondents who use the following therapies



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Question - Have you/your child ever used any of the following drug therapies to treat your JIA? (Select all that apply) n=523

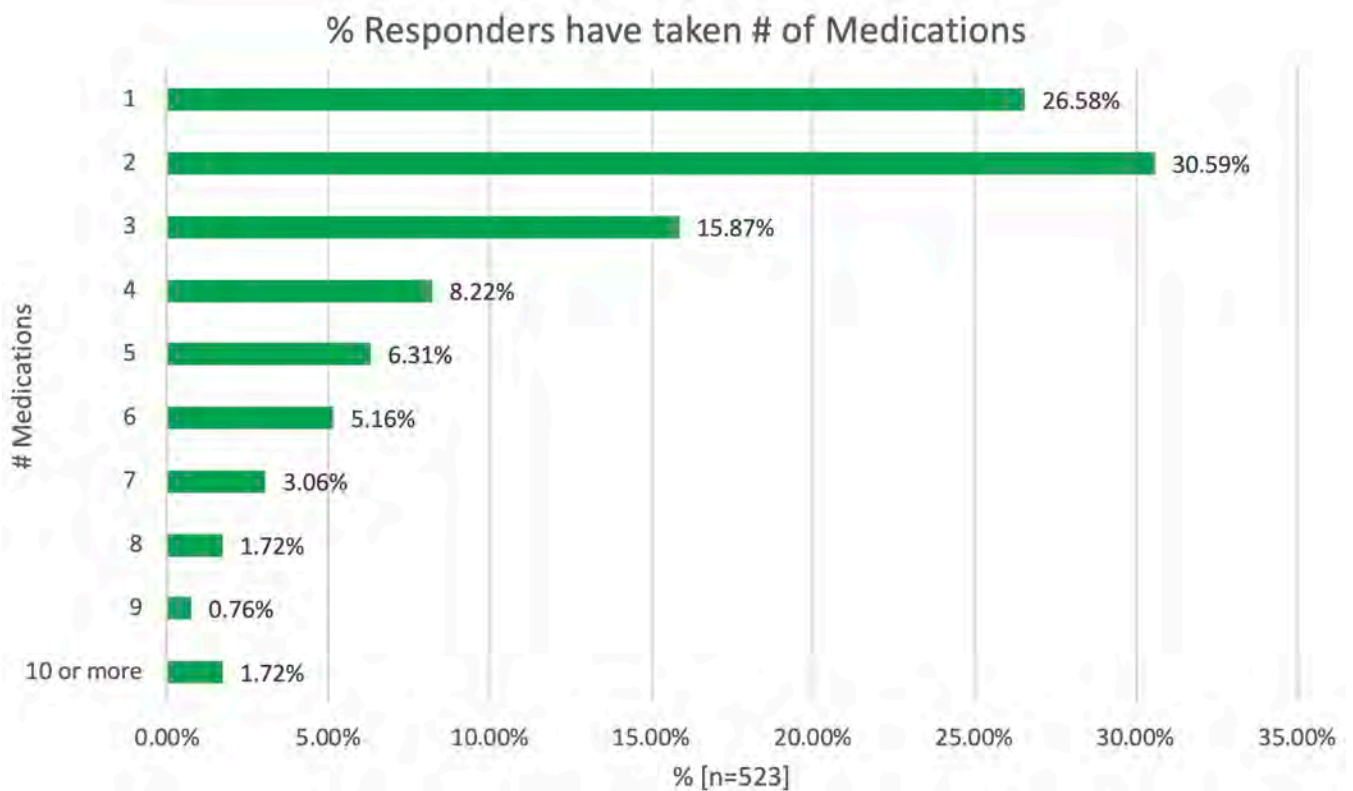
- 83% have used methotrexate to treat JIA



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Question - Have you/your child ever used any of the following drug therapies to treat your JIA? (Select all that apply) n = 523

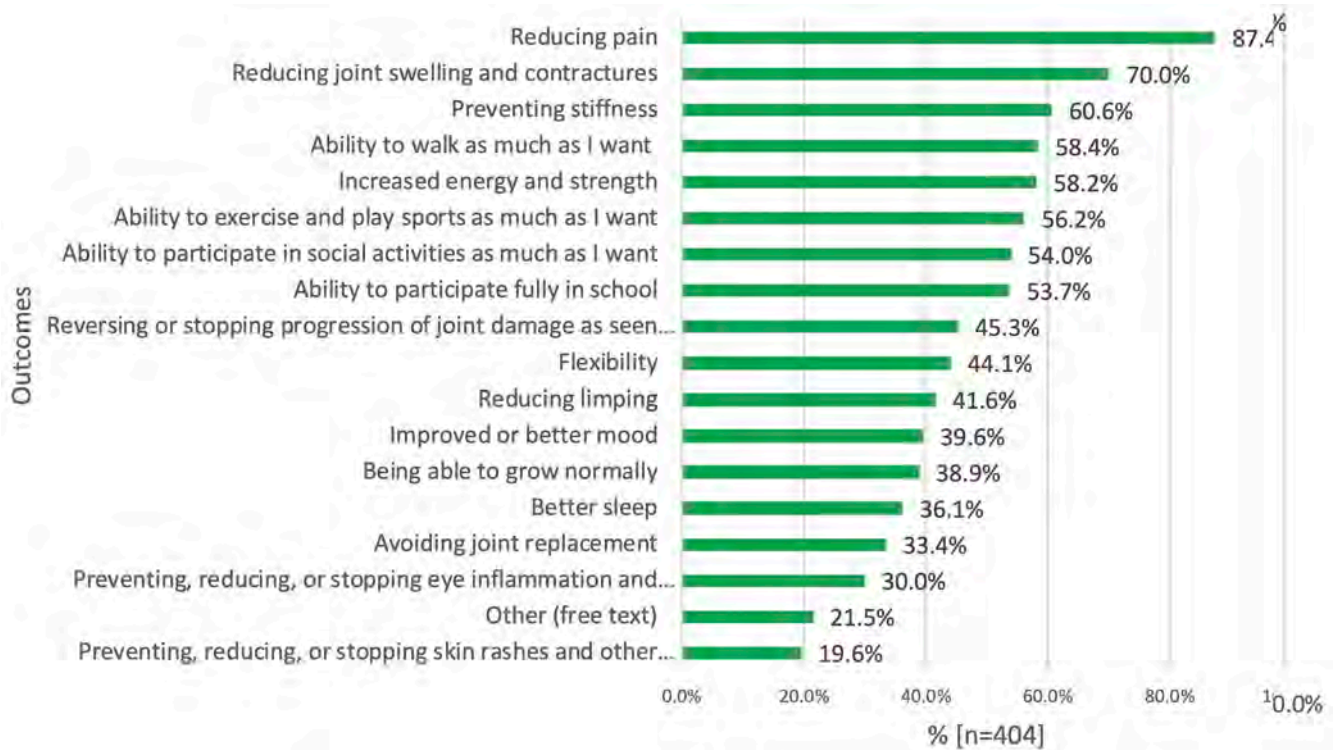
- Number of different medications patients have taken



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Question - To assess if a drug therapy is helpful for you/your child, which of the following outcomes are most meaningful for you/your child? (Select all that apply) n = 404

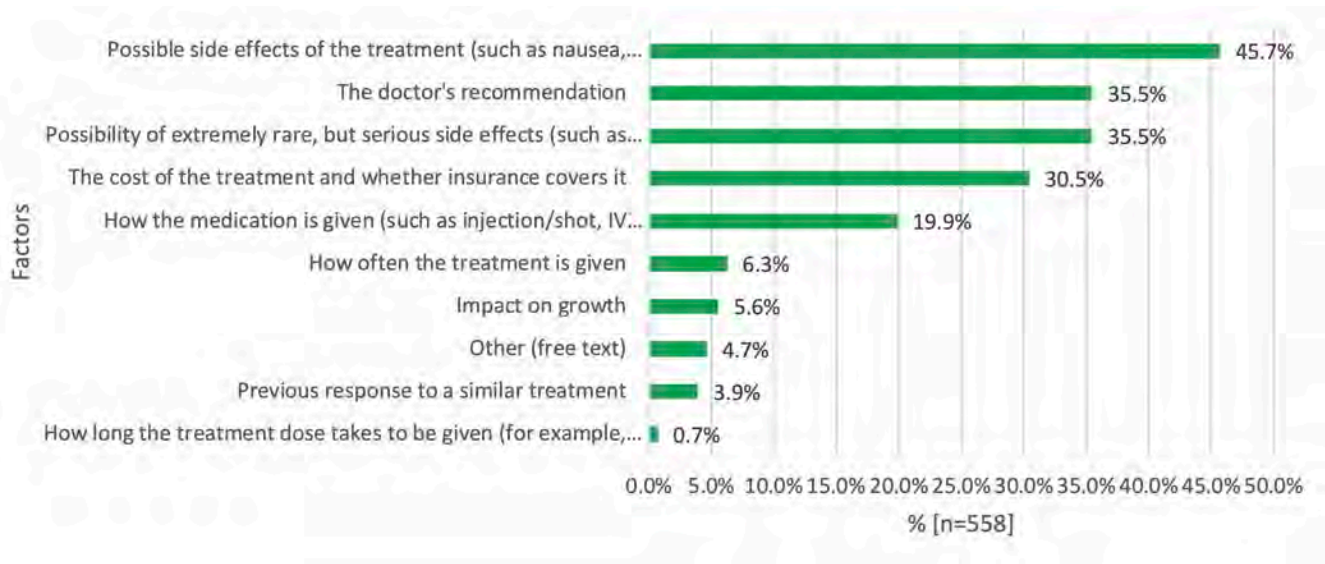
- 87% say reducing pain is the most meaningful outcome of a drug therapy



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Question - Which of the following are most important to you/your child when choosing a treatment for JIA? (Select up to two)? n = 558

- 46% say possible side effects are the most important consideration when choosing a treatment for JIA



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Appendix 3

JIA PFDD FOCUS GROUP SUMMARY

Background

On June 29 and 30, 2018, five focus groups were conducted in Seattle, Washington, with individuals and parents of people affected by juvenile idiopathic arthritis (JIA). Participants were from all over the country and were in Seattle to attend the Arthritis Foundation National Juvenile Arthritis Conference.

Key Findings

Burden of Disease

- The most common symptoms across all five groups were pain and fatigue, followed by swelling, stiffness, and limited range of motion. Among people with systemic JIA (sJIA), fevers and rashes were also noted.
- Parents frequently raised mental and emotional health issues; young adults were more focused on specific joint symptoms and related loss of function.
- All groups spoke to impact on ability to participate in sports. Parents noted impact on ability to participate in school and social activities; young adults were more focused on impacts on work, driving, and use of assistive technology.
- All five groups firmly expressed the profound impact of JIA on planning and budgeting (for time). Both short-term and long-term scheduling and planning are affected.

Treatment

- Participants described a wide array of treatment experiences, ranging from those who were biologic naïve to those who have cycled through every available biologic and are currently out of options. However, the majority of patients had been on at least one biologic.
- The dominant comment about treatment downside among the parent groups was injection “trauma.” This was followed closely by nausea and vomiting, and then mood and behavior changes.
- Young adults mentioned nausea and vomiting (secondary to MTX) but more notably raised susceptibility to and frequency of infections as a major downside.
- In benefit/risk determination, context was determinative and sJIA patients/families with more complicated disease courses saw shots and related side effects as necessary to control the disease. Families dealing with other diagnoses seemed to struggle with this decision process more.
- Concerns about reproductive health (due to side effects) came up in all three parent groups but did not in the young adult groups.

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- The three parent groups overall expressed an “ideal” drug as one with oral administration, and little to no side effects – again raising reproductive health as a concern. Reduced frequency of treatment was also desired.
- The young adult groups similarly discussed mode of administration. Although most preferred oral administration, they hoped for “adaptability” in how drugs are given citing it should work best for each person depending on their needs. Interestingly, young adults also noted that an ideal drug would last a very long time; this length of efficacy did not come up in the parent groups.

Clinical Trials

- Across the five groups there was very little experience with clinical trials.
- Across all five groups the overwhelming sentiment was reticence to be involved in a clinical trial unless the patient is “out of options.”
- This sentiment was even stronger in the context of placebo-controlled and withdrawal trials. Participants in all five groups had strong feelings about the use of placebo; use of an active comparator arm was seen as a positive step, but those stable on current therapy would still most likely not participate.
- Many parent participants had suggestions for making a trial more palatable (even placebo-controlled trials). Suggestions included 1) intense monitoring that would immediately alert patient/families and study teams of an issue; 2) a rapid response approach to get that patient on therapy right away. One group termed this the “rapid escape pathway.” Other parents expressed a desire to have more control in the process, potentially selecting which arm their child would be in, and having the ability to pull a child out of a study when they had concerns.
- Several findings from throughout the discussions lend themselves to the case for patient/parent involvement in clinical trial design. Two examples of issues to consider are pain tolerance and the inaccuracy of current pain measurement tools, and the young adult requests for “adaptability” in medication administration.

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Focus Group Participant Breakdown

Parent FG 1	Female with sJIA diagnosed at 5 (and IBS and CAPS) Female with sJIA diagnosed at 6 Female with sJIA diagnosed at 3 Female with sJIA diagnosed at 8 Female with sJIA diagnosed at 6
Parent FG 2	Male with ERA diagnosed at 6 Female with JIA and ERA diagnosed at 6 Male with JIA and ERA diagnosed at 10 Female with PsA diagnosed at 22 months Male with PsA diagnosed at 8 Female with poly diagnosed at 11 Male with ERA and MCTD diagnosed at 7 Female with ERA diagnosed at 13 Male with ERA diagnosed at 14 Female with PsA diagnosed at 3
Parent FG 3	Female with oligo, PsA, CRMO diagnosed at 2 Female with ERA and CRMO diagnosed at 8 Female with extended oligo and uveitis diagnosed at age 18 mos Male with ERA diagnosed at 8 Female with poly diagnosed at age 3
YA (ages 18 and up) FG 1	Female with poly diagnosed at 16 mos Female with poly diagnosed at 18 mos Female with poly diagnosed at 8 Female with ERA diagnosed at 14 Female with poly diagnosed at 13 Female with PsA diagnosed at 2 Female with MCTD and comorbidities diagnosed at 15 Female with JIA diagnosed at 13 Female with poly diagnosed at 11
YA (ages 18 and up) FG 2	Female with sJIA diagnosed at 15 Female with poly diagnosed at 9 Male with JIA diagnosed at 16 mos Male with JIA diagnosed as baby Female with poly and comorbidities diagnosed at 14 Female with JIA diagnosed at 15 Male with JIA diagnosed at 6 Male with poly JIA diagnosed at 9 Female with poly JIA diagnosed at 9

sJIA = systemic juvenile idiopathic arthritis; IBS = irritable bowel syndrome; CAPS = cryopyrin-associated autoinflammatory syndromes ; ERA = enthesitis-related arthritis; JIA = juvenile idiopathic arthritis; PsA = psoriatic arthritis; MCTD = mixed connective tissue disease; poly = polyarticular JIA; oligo = oligoarticular JIA; CRMO = chronic recurrent multifocal osteomyelitis.

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Appendix 4

In-Meeting Polling Questions

1) How old were you/your child at diagnosis? n = 55

Response	Count
a. Under 2	11
b. 2-5	22
c. 6-10	14
d. 11-16	8
e. Not Sure	0

2) How long have you/your child had JIA? n = 61

Response	Count
a. 1-2 years	9
b. 3-5 years	14
c. 5-9 years	17
d. More than 10 years	21

3) Which of the following symptoms has had the greatest impact on you? (choose one) n = 62

Response	Count
a. Pain	21
b. Fatigue	17
c. Joint swelling	3
d. Stiffness	1
e. Decreased mobility	5
f. Reduced participation in things such as sports, school and social activities	10
g. Other	5

4) How many treatments have you/your child had to try in order to get control of your JIA? n = 44

Response	Count
a. 1	1
b. 2-4	8
c. 5-7	6
d. 8-10	4
e. More than 10	8
f. Have not been able to gain control	17

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5) What has been your/your child's most troublesome side effect from treatment? (choose one) n = 45

Response	Count
a. Nausea/vomiting	7
b. Mood or behavior changes	3
c. Susceptibility to infections	11
d. Difficulty with injections/infusions (site reactions, pain and needle phobia)	15
e. Pain	1
f. Other	7
g. Have not experienced side effects from treatment	1

6) Have you/your child ever been involved in a clinical trial for a JIA treatment? n = 41

Response	Count
a. Yes	3
b. No	38
c. Not sure	0

7) If you/your child have been involved in a clinical trial for a JIA treatment, what made you want to participate? (choose one) n = 6

Response	Count
a. Ran out of currently approved treatments to try	1
b. Doctor recommended the trial	1
c. Wanted to help my community	3
d. Liked/felt comfortable with the way the study was designed	0
e. Because of the benefits of being in a clinical study, such as free medical care	0
f. Was hopeful that the experimental treatment would work	0
g. Other	1

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8) If you/your child have not been involved in a clinical trial for a JIA treatment, why not? n = 43

Response	Count
a. Never had the opportunity	28
b. Was stable on current treatment and did not want to change	6
c. Fear of being given a placebo	6
d. Fearful of clinical trials, in general	1
e. Other	2

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Appendix 5

SPEAKER AND PANELIST BIOGRAPHIES

ANDREW CURTIS

Andrew Curtis is an 18-year-old young adult from Highland Mills, New York. He will be starting his first year of college at Albany College of Pharmacy and Health Sciences where he is pursuing his Doctor of Pharmacy degree. He plans to get into clinical pharmaceutical research. Andrew was diagnosed with oligoarticular juvenile idiopathic arthritis (JIA) at age two.

VINCENT DEL GAIZO

Vincent Del Gaizo lives in New Jersey and is a father to 18-year old triplets. He has been active in the field of pediatric rheumatology since 2001, when one of his triplets was diagnosed with systemic JIA at 15 months old. Currently, Vincent serves as co-investigator of a National Patient-Centered Clinical Research Network (PCORnet) network called PARTNERS, and in several other research and quality improvement roles. In addition, he has, and continues to work very closely with the Arthritis Foundation in all activities related to pediatric rheumatology. Vincent recently made a major career change, going from a 30-year career as a small business owner to the Director of Strategic Partnerships and Patient Engagement for the Childhood Arthritis and Rheumatology Research Alliance (CARRA).

GUY EAKIN

Dr. Guy Eakin leads the Arthritis Foundation's science department. In this role he works to engage the scientific community in the Foundation's mission to conquer arthritis. Previously, he was at BrightFocus Foundation for almost 10 years where, as vice president of scientific affairs, he led nearly \$90 million of international biomedical research initiatives for Alzheimer's, macular degeneration and glaucoma, identifying projects that could change patient lives in the shortest timeline possible. Guy earned his doctorate from Baylor College of Medicine and pursued research at Memorial Sloan Kettering Cancer Center and the University of Texas M.D. Anderson Cancer Center.

RACHEL GLASER

Dr. Rachel Glaser is a Clinical Team Leader in the Division of Pulmonary, Allergy and Rheumatology Products, CDER, at the FDA. She is a board-certified Internist and Rheumatologist. She received her medical degree from Johns Hopkins University School of Medicine and completed her internal medicine training at New York-Presbyterian Hospital/Weill Cornell. She then joined the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) where she completed her rheumatology fellowship. Following fellowship training, she joined a multispecialty practice before joining the FDA as a Medical Officer in 2014. At the FDA, Dr. Glaser is involved in the regulatory review of therapies for rheumatic diseases, including JIA.

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NICK KIM

Nick Kim is a rising senior at the Bergen County Academies in Hackensack, New Jersey. He is also an intern at Hackensack University Medical Center in the Pediatric Rheumatology Division, working under Drs. Kimura & Janow. Because Dr. Janow is the physician who diagnosed him, he feels particularly honored to be shadowing her. He also is doing work related to Patient Engagement for the Childhood Arthritis and Rheumatology Research Alliance (CARRA) research. Nick was diagnosed with enthesitis-related JIA in October 2017. He enjoys tennis in his free time but is currently also working on college applications.

YUKIKO KIMURA

Dr. Yukiko Kimura is the Chief of the Division of Pediatric Rheumatology at the Joseph M. Sanzari Children's Hospital at Hackensack University Medical Center and Professor of Pediatrics at Rutgers New Jersey Medical School and of the Seton Hall University-Hackensack Meridian School of Medicine. Dr. Kimura has dedicated her career to improving the health, treatment and outcomes of children, adolescents and young adults with arthritis and rheumatic diseases. She has been a leader in research in these disorders, including being the Chair of the juvenile idiopathic arthritis (JIA) research committee for Patient Engagement for the Childhood Arthritis and Rheumatology Research Alliance (CARRA), and is currently the immediate Past President of CARRA.

MELANIE KOHLHEIM

Melanie Kohlheim is the parent of a seven-year-old child with polyarticular JIA and she herself has ankylosing spondylitis. Her daughter Megan was diagnosed at the age of 20 months with more than 10 joints affected. Melanie and her family live in Granville, Ohio near Columbus. Professionally, Melanie has a background in Animal Science Business, Sales, and Marketing. She worked at a therapeutic horseback riding facility for 11 years before dedicating her time to JIA. Currently, Melanie is engaged in numerous research and quality improvement initiatives through Pediatric Rheumatology Care and Outcomes Improvement Network (PR-COIN), Patient Engagement for the Childhood Arthritis and Rheumatology Research Alliance (CARRA) and the Patient-Centered Outcomes Research Institute (PCORI). She dedicates a large amount of her time to educating families on the value of quality improvement efforts in bettering the care of children with JIA.

KATE KUHNS

Kate Kuhns and her daughter Delaney live in Roanoke, Virginia. Kate formerly served in the military and is now employed in the private sector, but her most important role is as a mother and advocate for Delaney who was diagnosed with oligoarticular JIA in 2009 when she was six years old. The disease has expanded to extended oligo. Kate and Delaney are both very involved with the Arthritis Foundation. Delaney has been attending Juvenile Arthritis Conferences for several years, where she has formed friendships with other young people diagnosed with JIA.

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ROCHELLE LENTINI

Rochelle Lentini is from Florida and a proud mother of two sons with JIA, one in remission and the other with active, aggressive disease. She serves on her local Arthritis Foundation's board, is a past conference chair, and an active volunteer. She has a background in special education and recently retired from directing a project at the University of South Florida promoting social-emotional development in young children.

LAURA MARROW

Laura Marrow is Director, Partnership Liaison within the Arthritis Foundation's science team. In this role Laura fosters and facilitates critical relationships between the Foundation and its clinical, academic, and research partners including the key partnership between the Foundation and Patient Engagement for the Childhood Arthritis and Rheumatology Research Alliance (CARRA). Prior to joining the Foundation in 2015, Laura served as the Clinical Program Manager for the Cystic Fibrosis Foundation from 2002-2015. In that role, she managed the accreditation and oversight program of a robust collection of care-delivery centers focused on high-quality care for people living with Cystic Fibrosis. Laura began her career in non-profit health with the American Cancer Society.

NIKOLAY NIKOLOV

Dr. Nikolay P. Nikolov is a board-certified Internist and Rheumatologist who joined the FDA in 2009 as a Medical Officer and is currently Associate Director for Rheumatology in the Division of Pulmonary, Allergy and Rheumatology Products, CDER. He completed an Internal Medicine residency at Lincoln Medical Center, Bronx, NY in 2002. He then joined NIAMS where he completed his rheumatology fellowship in 2004, participated in clinical investigational protocols, and studied cellular and molecular mechanisms of autoimmunity in animal models in the Immunoregulatory Group, Autoimmunity Branch. In 2005, Dr. Nikolov joined the Sjögren's Syndrome Group at the National Institute of Dental and Craniofacial Research, NIH, as a clinical investigator where he conducted clinical and translational protocols in systemic lupus erythematosus (SLE) and Sjögren's syndrome. At the FDA Dr. Nikolov is involved in the regulatory review of immuno-modulatory therapies, including biologics and biosimilars.

JACQUELINE PEÑA

Jacqueline Peña lives in Mason, Ohio and is mother to Jacob, who has polyarticular JIA along with multiple other conditions. She used to be a business manager, but needing to focus on Jacob's health, left the field and now considers herself a "domestic engineer," though her family refers to her by the title "wonder woman." Jacqueline loves to spend time with her family and focuses on raising her children and raising awareness of Jacob's medical conditions. Her goal is to find a treatment that will help Jacob's JIA.

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CORINNE PINTER

Corinne Pinter is from Sugar Land, Texas where she has been a licensed cosmetologist for over 20 years, but a stay at home parent for the past 12 years. She has three kids, two girls ages 12 and 10 and a son who is seven. Corinne currently focuses her volunteer efforts on the Juvenile Arthritis Conference, her local Jingle Bell Run and other local events. She is finding her way into patient/parent engagement in research and finds it truly rewarding to give a parent perspective. Her younger daughter was diagnosed with polyarticular JIA on her second birthday.

HARLEY POWELL

Harley Powell lives in West Orange, New Jersey and will be a senior at Seton Hall University (South Orange Campus). She began horseback riding for physical therapy as a child, but now enjoys doing so competitively. Harley also enjoys playing video games, watching television, and reading -- especially Harry Potter. She is studying to take the LSAT in September and is hoping to attend law school in 2020. Harley was diagnosed with JIA at the age of 2.

ANN PALMER

Ann Palmer has more than 30 years of non-profit management experience. Since 2013, she has revolutionized the Arthritis Foundation's approach, structure and focus to deliver the greatest impact in the lives of those with arthritis. Ann's previous background includes extensive experience at large voluntary health organizations, including the American Cancer Society, American Diabetes Association and the Cystic Fibrosis Foundation. Her roles have spanned from leading national operational restructures to bringing in new business systems that allowed her teams to raise a net income of more than \$100 million annually. Ann sits on the board of directors as the vice chairperson for the National Health Council and is recognized as one of the 100 most influential leaders in the health care industry by the Atlanta Business Chronicle.

LAURA SCHANBERG

Dr. Laura Schanberg is a pediatric rheumatologist and active clinical researcher. She received her medical degree and completed her residency and fellowship at Duke University. Dr. Schanberg is a founding member and past chair of the Childhood Arthritis and Rheumatology Research Alliance (CARRA) and is principal investigator on several notable clinical trials and the CARRA Registry, funded by NIH, Patient-Centered Outcomes Research Institute (PCORI), the Arthritis Foundation, and the pharmaceutical industry. Her research addresses patient-reported outcomes, particularly pain and disease symptoms in JIA.

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SUZ SCHRANDT

Suz Schrandt is the Director of Patient Engagement for the Arthritis Foundation and previously served as Deputy Director of Patient Engagement for Patient-Centered Outcomes Research Institute (PCORI). Ms. Schrandt's patient engagement focus stems from her own rheumatic disease diagnosis at age 14. Her prior posts include roles in health and disability law and policy, genetic discrimination, and public health. She currently serves as one of nine voting members on the FDA's inaugural Patient Engagement Advisory Committee and in various other roles in patient engagement and patient and clinician education.

ELIZABETH SMITH

Elizabeth "Liz" Smith lives in Burke, Virginia. She is a mother of six and grandmother of "6.3." Liz works as a preschool administrator, serves as a volunteer with the Arthritis Foundation, and is a Patient/Caregiver Representative on various committees. Liz has two children affected by JIA; her first child was diagnosed at age two and another had symptoms for several years before receiving a diagnosis at age 19.

ANJIE VAGO

Anjie Vago is an English teacher and private tutor from Elizabethtown, Pennsylvania. Her most important job is being a "medical mama" to Laura and Erin, both of whom have forms of JIA and other comorbid conditions. She and her husband, Shawn, have just celebrated 26 years of marriage. Anjie is involved with the Arthritis Foundation, Pediatric Rheumatology Care and Outcomes Improvement Network (PR-COIN), Patient Engagement for the Childhood Arthritis and Rheumatology Research Alliance (CARRA), and PARTNERS, as well as the patient and family advisory council at Penn State Hershey Medical Center.

KIRSTEN WILDER

Kirsten Wilder lives in South Berwick, Maine. She works part-time as a customer service representative, but also as a full-time mom of three teens. Kirsten's daughter Katherine was diagnosed 11 years ago with JIA at age three. Kirsten is Boston Children's Hospital's Parent Representative to PR-COIN, is a volunteer and Platinum Ambassador for the Arthritis Foundation and serves on her local Juvenile Arthritis Committee.

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TRANSCRIPTS





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Appendix 6 – Transcript 1

File Name: cameraisorecordbeforelunch

Number of Speakers: >5

Length of file: 2:02:33

Audio Category: Verbatim

Unusual Words or Terms:

(e.g. abbreviations, Company Names, Names of people or places, technical jargon
Suz Schrandt, Ann Palmer, CARRA, PRCSG, Yukiko Kimura, PR-COIN, Nikolai
Nickoloff, PFDD, Penicillamine, NAIDS, Methotrexate, Naproxin, TnF, IO6, systemic JIA,
spondyloarthritis, Infliximab, Golemumab, uveitis, enthesitis, dactylitis, Macrophage
Activation Syndrome, Pascual, oligoarthritis, sacroiliac, OMERACT, exogenous Cushing
syndrome, Bergen County Academy

Transcriber VIN: VVTRAS JA25 US0010

[Audio Begins] [00:00:01]

[intro music] [00:00:01-00:03:50]

Suz Schrandt: All right, if everyone can take their seats, we're just about to get started. And I'd like to invite anyone who's in the back of the room please-, please come up. I think we're doing a pretty good job with not too many people in the far back of the room. That's great.

All right. Uh, before I introduce Ann Palmer to really get us kicked off for today, I wanna go through just a couple quick housekeeping items. Um, one is that there is a WiFi code on each table, um, and you're going to probably wanna access WiFi later in the day when we start getting into some polling questions, so just want you to be mindful that that's there. Um, actually, let me tell you who I am. I'm Suz Schrandt with the Arthritis Foundation. I'm, er, uh, Director of Patient Engagement.

Um, so the other thing I wanna pull your attention to is, uh, a document that's on your table. It should say "Informed Consent and Privacy" at the top. This meeting that you're involved in today is really part of a larger project, um, where we're really working to collaborate together and find out where do we need to focus our efforts on the best new treatments for, uh, kids and young adults with JIA? And because we're collecting information from parents about their children and collecting information from young adults, we're really treating this as research. So if you can just use that document, read through it at your-, at your own pace – you don't have to do it right now.



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I wanna call out just a couple quick items. And first is that by being here, by being a part of the meeting, you are consenting to be a part of this project and participate. So certainly, if at any time during the day you change your mind and you decide you don't want to be a part of the conversation, you are welcome to leave and there's no ramifications from doing that.

The second part is just to call your attention to the privacy and confidentiality of what we share here today. This is a public meeting. It's going to be recorded and available on our website, and everything that we will discuss will eventually land in, uh, something called The Voice of the Patient Report, which is a really important document that will also be publicly available.

So as you're sharing ideas and experiences and insights, if you want to use just a first name, um, or use a pseudonym or in some other way, um, not reveal your identity, that's fine, that's totally up to you. Just wanna make you aware, um, that there's that-, that privacy consideration. And certainly, please don't mention other people by their full names unless you've gotten their consent to do that.

So with that, I'm very excited to introduce and welcome to the stage Ann Palmer, our President and CEO at the Arthritis Foundation, to get us kicked off for this very exciting day.


[applause] [00:06:43]

Ann Palmer: Well, good morning. And this is a very exciting day. It's one that we certainly have, uh, anticipated for quite awhile. And it's just really great to see so many of you here. And I-, I wanna say that, um, uh, it's so important for you to be here and to raise your hands and your voice to participate. You know, this is, uh, a meeting that we're co-hosting with CARRA, but one that the Arthritis Foundation is truly passionate about, and we're just so excited to have so many of you here. And-, and I hope you understand the importance of being here and using your voice and making that voice work, not just for your family, but for so many other families.

You may be wondering why we're in such a big room. And, uh, later today we'll be hosting our second JA conference of the year with about 850 families here. We'll open this up and kick this off tonight. So for us, this is the beginning of a long weekend that we're very excited about, and focusing on our community of JA families.

You know, this meeting is critical, uh, to shaping the future of da-, drug discovery and, uh, what's really important today is that we have not just wonderful JA families here – Moms and Dads – but we have the FDA, uh, we have doctors, and we have other industry leaders. So, uh, I wanna just say, uh, thank you so much to the partners who have come in, as well. Uh, we're obviously honored to have the FDA here with us, but we're also honored to have industry leaders, researchers and so many people who are





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here and want to listen to the voice of people with JA, the parents, and to really learn from those insights and take that-, that-, those insights forward.

We're particularly pleased to have the PRCSG group here from Cincinnati, and I want to welcome them, as well. They're from Cincinnati Children's Hospital Medical Center and, uh, our special guests of ours, as well. You know, we have had the privilege of hosting, uh, osteoarthritis, uh, patient focus drug meeting last March. And, uh, we're continuing that work. And so privileged to be, uh, also hosting this meeting and-, and looking forward, as Suz said, to the great result that will come from this.


[cough] [00:09:18] You know, um, participation in research has come a long way. You-, many of you know that, um, uh, we used to talk about, uh, patients being subjects of research, and, uh, I'd like to believe that the Arthritis Foundation has had, uh, a real part in making this, uh, this change. And this meeting certainly represents that. This is, you know, really an opportunity for-, for us to change that – from just being a patient participant in research. Uh, but you-, you need to know that now we're in a position where you're going to be able to influence how research is designed, the kinds of research that we focus on, and this is just really a powerful thing, and I hope that, uh, you believe and understand that we've come a long way and we're all gonna be better for this collaboration. Not only were you-, will you as patients and family members be better, but, um, we're gonna be better as a community because we won't have wasted resources.

Um, the Arthritis Foundation is proud of its work in, uh, improving care. We've had a long partnership with-, with CARRA for many years. Um, we've invested in a registry and have expanded that to, uh, many others, uh, forms of disease, like lupus and JDM, and we've launched a program called Partners, which is specifically designed to engage and offer families the opportunity to get engaged in research. We'll be doing some of that at our JA Conference while we're here. But, you know, creating treatments for patients is about collaboration. And we can't do this without all of you in this room. And we appo-, truly appreciate your involvement today. We look forward to the outcomes of this meeting and the bright future that this represents in beginning here. And your participation in this is the path to a cure.

We're committed to bringing you as many opportunities as possible like this, for you to join with us in the Arthritis Foundation and CARRA in-, in being able to move this agenda forward. So, again, my heartfelt thanks for each of you for being here. It's a very exciting day. We can't wait to, uh, listen and learn, and, uh, take the findings and move them forward.

And now-, now I'd like to introduce you, Yukiko Kimura, our co-sponsor. Yuki is, um, the past chair of CARRA. We've had a wonderful opportunity to work with her, um, uh, and we're, uh, pleased to, uh, have her here. She's, uh, gonna talk a little bit about CARRA, and, um, she's a great partner to the Arthritis Foundation. Yuki.





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[applause] [00:12:12]


Dr. Yuki Kimura: Thank you very much, Ann, and I'm really pleased to welcome all of you who are today, and also, um, the other people – the hundreds of other patients and families that are participating by webinar – so, um, I wanna welcome everybody to this. Um, I think a historic, um, day in which we, for the first time, are, um, gonna be able to, um, really understand, some patients and families about what it is like to have JIA, and how we need to improve treatment. We-, I'm gonna tell you about how much treatment has improved, um, over the last few years for JIA. But, um, you know, we have, um, someplace to go, so that's what we're here to do. And we need to hear from you, um, and get your ideas and opinions in order to move things forward.

Am I doing something wrong here? Oh, there we go. So, um, I just want to tell you a little bit about CARRA. CARRA stands for the Childhood Arthritis and Rheumatology Research Alliance. And, uh, we began in 2002 as a pediatric rheumatologist researcher-led research network, um, and we've grown quite a bit, um, so that now we have 120 sites, uh, what you see on this map, uh, spread across the United States and Canada, and now we have over 500 members. And, um, we're really pleased that, um, over 90% of all the pediatric rheumatologists in the country are members of CARRA. This is a picture of the, um, annual, uh, scientific meeting which was held, uh, this April in Denver. Uh, and we welcomed, um, over 600 participants to this meeting. Um, and we have had a very strong relationship with the Arthritis Foundation for many years. Actually, the Arthritis Foundation has been, um, funding our infrastructure since CARRA began. And we, um, embarked on a-, on a very important strategic partnership, um, in 2015, um, that has really, um, uh, raised us to the next level.

CARRA's mission is to conduct collaborative research, to prevent, treat, and cure pediatric rheumatic diseases and our vision is a world free of limitations from pediatric rheumatic disease. Um, our values are inclusiveness, trust, impact, and innovation. Um, one of the most important and central research projects, um, of CARRA is the CARRA Registry. Um, the CARRA Registry, uh, the new, uh, CARRA Registry began in the middle of 2015 and since that time we've been able to enroll over 6000 patients, which is a long way towards our goal of enrolling at least 10,000 patients to be followed for at least 10 years.

And here is, uh, uh, a map that shows you all the CARRA Registry sites and the enrollment of patients. And we hope to learn a lot – we're already learning a lot from, uh, the registry, but what we, uh, the main aim for the registry are to understand, um, the long-term outcomes of our patients with JIA and other rheumatic diseases, um, so that we know what happens to the patients with JIA as they-, they, um, get older and grow into adulthood and beyond. Um, we also wanna be able to understand the safety, both the short-term and long-term safety, of the medications that we use to treat our patients, which is actually one of the number one concerns that have been expressed, um, by our patients and parents about medications.





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Um, and through, um, studies that are embedded within the registry, we're gonna be looking at the comparative effectiveness of treatments, um, that are standard in treating certain specific diseases such as systemic JIA and poly JIA. And through clinical trials we will be studying the-, the efficacy of new treatments to treat, um, JIA and other disease. Um, and this is, um, uh, a-, a goal and hope that we have and the registry gets us closer to this goal of, um, being able to give every patient the opportunity to participate in research.


So patient engagement, um, has been a very important, um, part of CARRA since its very beginning. Um, and we've involved patients and parents in many of our research projects over the years. But recently, over the last few years we've really stepped it up, um, and formalized, um, the engagement that we do in research with our patients and parents so that they are involved, that you are involved, um, in, uh, in the process every step of the way, side-by-side with our researchers.

So, as Ann referred to, uh, we have patient engagement initiatives, such as Partners, which is, uh, funded by the Patient Centered Outcomes Research Institute, or PCORI, which brings together CARAA, the Arthritis Foundation, PR-COIN, um, the Lupus Foundation, and Cure JM, to, really, um, engage patients, and families, and research. Um, and we've done-, we're doing things such as, um, you know, creating training programs, for example, uh, for patients and parents and also researchers, um, to be able to perform patient-centered research.

Um, the news-, the registry newsletter is another example of, um, a, uh, a co-, um, initiated, um, project between patients, and parents, and researchers. Patients and parents, um, have a bit role to play in developing, um, you know, the content for the CARRA Registry newsletter which is what we give to all of our participants who participate in the registry, and for anybody who's interested, to find out what's going on in the registry, you know, what have we learned from the registry, and what, um, other sub-studies are going on in the registry, as well as other helpful, uh, stories that patients can benefit from.

And we have really been embedding, uh, patients and families into the research process, um, from, uh, from the ground up, by having patients and families participate in the CARRA, uh, annual meeting. We had-, we welcomed over 60, uh, patient and parents attendees at our annual meeting, for example, and they participated in the CARRA workgroups, um, which are focused on specific diseases, and doing, uh, and understanding research in specific areas.

Um, we have patient partners who are involved in, uh, the nuts and bolts of, uh, CARRA-wide research projects such as Stop JIA, and FROST, and Promote. Um, and we also have patients and parents now involved in reviewing grants that we give out through the CAR-, CARRA AF Grant process, so that they can help us determine which grants are the most important to fund.



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And patients and parents have taught us also that we need to translate our publications and our research findings so that they're understandable for patients, and-, and families, and the public to, uh, understand what it is that we learned, and how they can benefit from it.

And of course, this FDA externally-led patient focus drug development meeting, um, that we're co-sponsoring with the Arthritis Foundation is a very-, gonna be a very important facet of understanding how we conduct our research going forward. So what we hope to learn today, at this meeting, is how JIA affects children, young people, and families, um, and how to identify the most, um, bothersome symptoms that we need to address for our patients, um, what the current burden of disease and its treatments are. I mean, we have, really, you'll see later that we've come a really, really long way, uh, from where we were, um, 25 years ago when I started my training. Um, but we still have a long way sto go. And we need, uh, more and better treatment for JIA and we need to learn how to get there as quickly as we can.

So thank you very much.

[applause] [00:21:10]


Suz: Great. Thank you, Yuki. And next I'd like to invite up, um, Dr. Nikolai Nickoloff. He's the Associate Director for Rheumatology at the FDA's Division of Pulmonary Allergy and Rheumatology Products.

Dr. Nikolai Nikolov: Do you hear me? Well, thank, Suz, for that very kind introduction, and for the opportunity to be here today. Uh, my colleagues from the FDA, and I, are honored to be a part of this landmark, um, event, that has brought the Juvenile Arthritis community together to advance the development of new treatments for the disease. For this opportunity, I would like to thank the Arthritis Foundation and the CARRA, or the Childhood Arthritis Rheumatology Research Alliance, and all the staff, uh, that was involved in planning this meeting. But more importantly, I would like to thank all of you who overcame the challenges and discomforts of travel, and those who couldn't attend in person, but participate remotely, and uh, the-, on the phone and on the Web, to share your powerful stories with us.

You are here to today to educate us about what are the things that are most important to you as parents, families, and caregivers, who deal with this chronic illness. And we're here to listen and try to better understand this very important perspective. The perspective of patients with Juvenile Arthritis and their families, and the struggles you face every day.

The FDA has recognized the needs to have all stakeholders involved, and particularly patients, in identifying the key elements of clinical development, such as endpoints and





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outcomes that would represent more accurately the burden of disease. In that respect, the FDA is committed to conducting, uh, to including patient's perspective in drug development.

Over the last 6 years FDA has conducted over 24 patient-focused drug development, uh, meetings on various conditions. As the PFDD initiative began with the aim of providing a systemic way of gathering patient perspective on their conditions and treatment options, we are very happy to see externally-led patient focused drug development meetings like this one, uh, we're attending today, to continue this important work of advance-, of advancing approaches together from patients and caregivers.

The goals of a patient-focused approach to drug development and drug evaluation are to develop treatments that meaningfully address the aspect of disease that are most important to patients, to tailor our clinical trials to the needs of patients, to reflect patient's perspective on the benefits and harms of treatment in drug evaluation, and to ensure that the information that comes out of a drug development program accurately represents those benefits and harms and is-, and is directly relevant to patient's treatment decisions and needs.

Information gathered or provided through this format can better inform the benefits risk assessment of new therapies, identify areas of unmet need and help drug developers and the regul-, regulatory authority such as the FDA, design programs that are more relevant to address how patients feel and function. This has resulted in patient groups having a significant impact on drug development in the recent years and the [indiscernible] [00:25:10] discontinues.


We also have representation from industry, academia, and all the medical product development stakeholders in the room and on the Web. And we're all here today to learn from you. Today's meeting is a very important dialogue and I hope this is just the beginning that will lay the foundation of future conversations. The Juvenile Arthritis community is clearly engaged and eager to participate in this effort, which is evident from the attendance in this, uh, meeting. And from I understand, there is even a bigger crowd coming later today.

This meeting is as important to us as it is to you, so, um, I-, I would like to thank you for having us here, having this meeting. We really appreciate the opportunity and we're looking forward to a very productive meeting and discussion today. Thank you.

[applause] [00:26:07]

Suz: Thank you so much Dr. Nikolov, and thank you to all of our FDA partners who are in the room. I'm going to invite Dr. Yuki Kimura back to the stage to give us an overview of JIA and the therapeutics used for JIA. So, Yuki?





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Yuki: Thank you, Suz. [clears throat] [00:26:33] Um, so I'm gonna try to go very quickly, um, so we make up for some lost time here. So, um, hopefully I don't go-, go too quickly for you. But I just wanted to tell you . . . uh, start with, how far we've come. So when this picture of this poor little boy was taken, um, over 100 years ago with JIA, there was not very much that could be done for this child. And you can see that, um, he is gonna be suffering, uh, for his entire lifetime with, um, disability, unable to even sit up, and certainly not stand up, or even care for himself.

And, uh, with the advent of treatments like aspirin and steroids, uh, there was some improvement, um, but still there was a lot of, uh, disability and deformity that accompanied, um, JIA. Um, and even in the early 2000s, it wasn't unusual to see, uh, children with JIA in wheelchairs, um, at JIA conferences, um, and also, you know, with deformed joints and needing assistive devices like you see on the boy in the background, um, and this is what we see today. This is a-, a picture taken at JA Camp a few years ago. And I challenge anybody to be able to tell that there's anything wrong with this bunch of kids. So we have really come a long way.

Um, and a-, a lot of the reason for the improvement in outcomes has to do with the improvement, um, and the treatment that we have for children with JIA. You can see, in the early part of this-, of this past century, actually up to about 1980 or so, there really was very little, uh, in the way of treatment, aside from aspirin, um, things like gold and Penicillamine, Cortisone, um, and NAIDS, which are medicines like Naproxin and ibuprofen. And with the advent of Methotrexate, we finally start to make some headway, um, in the treatment of JIA and improving outcomes. And it is a medicine that we use to this day.

But really, it was after about 2000 or so, when we started to see the explosion of medications that, um, have become available and are also very, very effective, um, that really, really changed, um, outcomes for our kids.

Um, and I just – whoops – wanted to point out to you – I'm gonna go through this again – that five of those treatments have been approved, uh, for use in JIA. I'll just show you again. Um, and these have been done through, um . . . the approvals, regulatory approvals through FDA and EMA, have been done through clinical trials that were, uh, done in JIA.

So, um, just to go through, um, some of the different categories of medicines that are available to treat in-, all forms of inflammatory arthritis, not just JIA – um, these include, um, the most familiar, which are the TnF inhibitors, and I've bolded and starred the ones that are, um, approved for use in JIA. Um, we have, uh, a T-cell activation, uh, inhibitors, we have IO6 inhibitors, uh, we have inhibitors of D-cells, um, of the [indiscernible] [00:30:03] IO1, which is primarily used in the treatment of Systemic JIA and auto inflammatory diseases. And then we have some new ones, um, inhibitors of IO17, for



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example, which primarily are being used in spondyloarthritis and psoriatic arthritis and IO12 and 23 inhibitors, and then we have, um, some newcomers, some oral small molecules, such as the JAK inhibitors and [inaudible] [00:30:29] inhibitor which, um, are being used for various forms of inflammatory arthritis. And this is just, um, what's out there now.

So there's a lot of many, new other medications that are, uh, being developed, some in the same classes as these, some in new classes, um, that are in the pipeline to be, uh, used in inflammatory arthritis.

So you see that these five medications have been approved for use, um, in JIA, uh, through clinical trials. Um, but actually there were two others, um, Infliximab and Golemumab that were, um, studied in clinical trials, um, hopefully to be approved for use in JIA, but unfortunately, for one reason or another, it did not, um, get approved, even though we know that they are effective. Um, in fact, Golemumab has recently been approved to be used in the injectable form in Europe.

Um, the good news, though, is that, um, the-, all the drugs in green, um, are being studied, including, um, the, uh, IV form of Golemumab, um, in clinical trials, so that hopefully, in the next few years, they will be available for use in JIA.

So I just want to take a few minutes to talk to you quickly about JIA, um, and to tell you that JIA is not just one disease, it's many different diseases. Starting with the little girl with one swollen knee who developed uveitis or inflammation in the eye, um, to the little girl who presents with many, multiple swollen joints and, um, in her body, um, to the older, uh, child that presents with, um, arthritis that looks just like rheumatoid arthritis in adults, um, to the older, um, patient, um, boy, who developed, um, enthesitis, or inflammation around the heel, um, and also around the, uh, spine in [indiscernible] [00:32:35] joints, the psoriasis, um, uh, that affects, um, and causes arthritis, as well as arthritis that's accompanied by fevers and rashes.

So these are the current, um, classification, um, of JIA, the categories that exist currently, and they're far from perfect, because they really just rely on clinical, uh, characteristics that just . . . you know, the number of joints that are involved, whether or not, uh, the blood test is negative or positive, whether there's [indiscernible] [00:33:08], whether there's psoriasis in the patient or in a family member, or whether there's systemic symptoms that accompany it. So, um, they're far from perfect, and you hear, uh, actually stories, um, you know, when the panels comes up, about, you know, many of these, uh, children, you know, hav-, going-, having one diagnosis and go-, and then being re-diagnosed as another disease because these are just clinical. And in-, hopefully, in the near future, we'll be able to identify and, uh, group patients, um, on a more biological basis so that we will know precisely what the patient has and which drug is the best one to treat, um, every patient.



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So just going through the different diseases quickly, so the persistent oligoarthritis patients are patients, uh, like these two little girls that, uh, start, um, in early childhood with one or two swollen joints, and very often the knee, um, and these are the patients that are really at high risk for developing, uh, the uveitis that's asymptomatic, so that's why the-, your-, your kids need to get screened frequently, um, for this complication, because if it's not caught, um, they can develop terrible, uh, Glaucoma, Cataracts, and even blindness.


The extended oligoarthritis patients, uh, start out looking like this, uh, first little girl, but then go on to develop, um, other joints so that they be-, they have five or more joints within six months of diagnosis. And these patients are also at risk for developing uveitis. The rheumatoid factor-negative polyarthritis is just that. These are patients with, um, many joints – five or more joints – but usually many, many more than that, that have, um, affect, um, multiple joints, and, um, their blood test is negative for rheumatoid factor. These are kids that usually present, again, er, relatively early in childhood, um, and have arthritis of the small joints, um, as well as, uh, the large.

Uh, rheumatoid factor-positive polyarthritis are patients, um, also that have small and large joints, symmetrical arthritis usually, uh, but are rheumatoid factor positive, and they usually start at a later age, um, age 10 and above, um, and also affect girls more often than boys, and have features, uh, like rheumatoid nodules that make them very similar to adults with rheumatoid arthritis.

And [indiscernible] [00:35:36]-related arthritis usually affects older kids, uh, pre-teens and teenagers. Although it can start earlier and, um, usually boys more often than girls, um, and it can affect, usually the large joints in an asymmetrical fashion as well as, um, the [indiscernible] [00:35:53], which is basically inflammation where tendons and ligaments insert into the bone, very commonly around the heel, but can occur in other places, and can, in fact, um, and cause inflammation of the spine as well as the sacroiliac joints.

And then psoriatic arthritis is arthritis that accompanies psoriasis in a patient, um, that can have, um, many different features including, um, arthritis patterns, such as dactylitis, which is, uh, inflammation like you see in the toe on the-, on the-, on the left, um, that-, uh, and it-, so it affects different, uh, you know, toes and fingers, as well as nail changes. And then Systemic Arthritis is the one that's very different looking from the other types of arthritis, um, because of the high fevers that accompany it, um, as well as, um, the rash – the distinctive rash – that often accompanies the fevers, um, and can also affect many vital organs with inflammation, as well.

And then Undifferentiated Arthritis is basically arthritis, JIA, that, um, doesn't fit into any one category.



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So, uh, we conducted a survey with the Arthritis Foundation, um, of JIA parents and patients, um, and we're really, um, delighted that we had, uh, an extremely robust response with almost 600 unique respondents. And we asked them what JIA category they had, and this is-, you can see them on display here. Um, I think that JIA category is represented, um, with a slight overrepresentation of the Systemic JIA category, numbering – I don't know if you can see it but it's about 19%. Usually they represent more like 10% to 15% of our JIA patient population, but nonetheless, you can see that every category is represented.

Um, and I just want to take a few minutes now just to talk about Systemic JIA because the story of Systemic JIA is very illustrative of, uh, the dramatic advances that we have made in the treatment of JIA, and also it demonstrates how far we have to go. So JIA, or Systemic JIA is, um, uh, basically a disease that causes arthritis. And like I said, high spiking fevers, as well as rash, um, but it can also involve many other, um, areas of the body and can cause swollen glands, swollen, uh, liver and spleen, uh, inflammation and fluid around the heart and other vital organs, as well as life-threatening complications, um, especially something called Macrophage Activation Syndrome or MAS.

So, um, you know, it is true that some patients with systemic JIA get all better after 6 or 12 months. They-, they don't need any more treatment and the disease never comes back. But unfortunately, for the vast majority of patients with systemic JIA, they continue with chronic disease, either systemic disease, which can be life threatening, or, uh, severe arthritis, um, in some unfortunate patients, both. Um, and, the problem with systemic JIA is that the usual arthritis treatments, um, such as Methotrexate, and even biologics, such as the TnF inhibitors, um, don't work very well.

So because of this, um, we were forced to treat these patients for years and years many times, um, with toxic medications such as systemic steroids which result in stunted growth and steroid side effects like you see in this little boy. And even with all that, we had very poor disease control, which also stunts growth, and which can cause destroyed joints, um, as you see in this little boy, um, who has, uh, terrible, uh, joint deformities, and who underwent bilateral hip replacement surgery, uh, a few years after this picture was taken.

So the discovery that these patients, um, respond to different biologics, to IO1 and IO6 inhibitions, uh, was really a tremendous one, and really changed the outlook for patients with systemic JIA. This is from a very early study in-, on 2005 by Virginia Pascual at Baylor, um, which showed that nine patients with systemic JIA who were refractory to other treatments. All other treatments responded rapidly to, um, IO1 inhibition. And you can see the top two, uh, graphs, of fever and arthritis, um, that they both disappeared, uh, for most of the patients within a matter of two weeks after starting this medication. And these are patients that hadn't responded to anything. And you can see that the blood test results also followed, um, this sort of pattern of excellent response.



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So, um, the biologics have meant a tremendous amount and there are a lot of pros that we can, um, be thankful for, um, in terms of biologic treatment for systemic JIA. So as I said, um, patients have an excellent response – most patients do – and they have a rapid response of their systemic features and of their arthritis. And the really wonderful news is that we hardly, um, have to use steroids in most of the patients, and in some cases, we don't even ever have to use steroids, which is almost a miracle. And then there's, um, also some, um, evidence, uh, in case, uh, theories that shows that possibly that if you treat that patient with systemic JIA very early, uh, with, uh, medications, um, that are effective, like the IO1 and-, or IO6 treatments that they-, uh, the chance of remission are much better.

But then there are cons. So some, you know, because we're treating these patients so early, there are patients who may never have needed to take biologics, um, who are treated with them. And, of course, there are unknown long-term side effects, and some of these patients have developed, uh, lung disease, uh, that seem to be more common now than they were years ago, so we're not sure. And of course the treatments are very expensive, and they're also painful because they almost always have to be given by a needle, they take time to give, especially if they're IV. And they're dis-, disruptive of lives. And then not everyone responds optimally.

Similarly for other types of JIA, there have been, uh, tremendous changes in long-term outcomes, but there's a "but" to it, too, that some patients don't respond as well, um, as they should. And you'll hear from our panel discussions that they've had to try a series of many different medications over time. And sometimes people run out of options. And-, and, of course, we know that the earlier the disease is controlled the better the outcome, so we wanna be able to treat these patients with effective treatments early on. And of course, the long-term side effects, as well as the other, um, issues with, uh, biologic treatment.

So it is true, biologics have totally changed the outlook for many cha-, patients with JIA, like you see this little girl with poly JIA that I showed you earlier. And a few months later, look how she-, she, ah, looks now . . . incredible difference. But not every patient responds.

So we need more new treatments. Uh, we did-, in our survey we showed that patients, um, have-, had to take a lot of medicines. Seventy-three percent have had to take two or more medications. Twenty percent have taken five or more medicines, and two percent have taken over ten. And patients and their caregivers want, uh, their treatments to be more effective and safe, and, uh, less painful.

And then we found that, um, there are currently still symptoms that are not addressed satisfactorily by the treatments. So 73% of the patients that we surveyed, um, continued to have a significant, uh, uh, symptoms, that needed to be, uh, that need addressing. And some of the most common ones are pain, fatigue, um, side effects, um, and trouble keep-, keeping up with friends and activities. And, um, and the, um, these



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results were corroborated by the OMERACT JIA Core Set workgroup, which has worked hard over the last few years to develop measures that can-, that should be studied in clinical trials, um, that are important to, uh, patients and parents. Um, they went through a very rigorous, uh, uh, process, and what they found was, uh, very similar in terms of the, uh, measurements of outcomes that need to be, uh, looked at in JIA. So, um, you know, we have a lot of good news, but we still have challenges. Uh, we need to increase the number and types of treatment choices that are available, um, make new of-, an innovative and safer treatments that are easier to give, treatments that target specific JIA types, and we know what treatment to give each person, um, when they start. And we need clinical trials to test new treatments in ways that, um, can be done more quickly – it doesn't take years and years to accumulate enough patients and include a wider range of patients that are representative of the patients that we treat, and that answer more questions that patients wanna know, and also measure the impact of the-, of the outcomes that are important to our patients. Thank you.


[applause] [00:45:48]

Suz: All right, thank you so much Yuki. I'd like to invite our first panelist up to take their places at the table. Um, I-, I know that, uh, we're running about 10 minutes behind, so for those of you who are food motivated, like me, I wanna-, I want you to rest assured we will still, um, break for lunch. We'll probably break for lunch about 10 minutes later than planned, and we'll make up some time during the lunch hour, but I wanna make sure we have plenty of time to hear from our panelists, and have a great robust conversation, um, with everyone here.

Um, as our panelists are taking their seats, I want to just give you a sense for how the rest of this is gonna go, because it might be different than other meetings you're involved in. It's really gonna be one large conversation, not just with people in the room, but with people who are joining via webinar. Um, we'll be doing polling questions, and on my next slide, I'm actually gonna walk you through how that works, and-, and how you'll respond to the polling questions. Um, then we're gonna hear from our wonderful panelists.

And then we're gonna do large group-facilitated discussion. We'll put the questions up here on the slide. We invite all of you in the room, including the panelists, to speak, and signal when you've got something to say. We've got two mic stands, uh, on either side of the room. If you could just form a cue as you're, um, as think of questions or comments you'd like to make. And we'll be alternating between the comments in the room and, again, the comments on the webinar.

To answer the polling questions – what you'll wanna do is I had mentioned the WiFi codes. So some of you might have cell phone service, if you don't, or if you prefer to use the Wifi, you want to log on to the WiFi using the code at your table. And you can actually cast your vote with your Smartphone. You're gonna go to that website you se



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there. <https://Slido>, enter the event code, arthritis, and then make sure you're on the polling tab. So let me take a moment and let everyone get where they need to be. Okay? M-kay. I'm looking for a visual cue. It looks like most people are good. Okay. Great.

So at this point, we're going to hope that technology gods are kind and that the question will generate, um, from the back. So the first question is, how old were you, or your child, at diagnosis? I'll give it another few seconds. It's like a race, watching one one is gonna win.

[laughter] [00:48:53]

Okay. We've got a lot of people diagnosed young at this point, but quite a range. Okay. All right, we'll call it good there. So I think the-, the winner here was ages two to five, um, but there's , uh, uh, a-, a big range represented. Let's go to the second question: How long have you or your child had JIA? Oh, wow. We've got a lot of established patients and families. Oh, but the others are catching up. Great. Okay.

And then the third question, and this is gonna be about, um, symptomology, and some of what Yuki showed on her slides. So we wanna know which of the following symptoms has had the greatest impact on you? And I know it can be awfully hard to pick, but those are your answer choices – "fatigue," "pain," "decreased mobility," "reduced participation," "stiffness," and then "other." M-kay, and that's definitely consistent with what we saw in our survey, with what came of the OMERACT results. We also conducted several focus groups before today, and certainly saw that pain, and fatigue, and reduced participation in sports was a major issue, but-, but so are so many other symptoms.

So we can go back to the slide deck now. Thank you. And that-, that exercise really tees us up for this panel discussion. We get a sense that we have a lot of commonality as a community, but we also have differences, and so now we're gonna hear from four people who can provide their unique stories and really help shape some of that data and give it that-, that human face. Um, so I'm just gonna turn it over to Anjie. Um, they'll all introduce themselves and so it'll be one after the other. And then as soon as they're finished, we will come back together for that large group discussion. Anjie?

Anjie: Good morning and, uh, thank you for the opportunity to speak to you today. My name is Anjie Vago and I'm from Elizabeth Town, Pennsylvania. I'm an English teacher and a private tutor. I also volunteer with many juvenile arthritis-related organizations, such as the Arthritis Foundation, PR-COIN, CARRA and Partners. My husband Sean and I just celebrated our 26th anniversary and we have two daughters. Laura is 22 and Erin is 19.



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I'm sure many of you are familiar musical, Les Miserables. Near the beginning of that show, there's a song called I Dream The Dream. Fantine, a destitute single mother, sings this song as she laments the loss of her husband, and to have [indiscernible] [00:52:32] in her life by that unfortunate event. When talking about the happy times she sings, And the World Was a Song, and the Song was Exciting. There was a time that it all went wrong. I know that this describes a very different situation than what we're here to discuss today, but the first couple of lines of this song epitomize the way my husband and I felt when our girls were born and when they were young children.

Later in the song, Fantine elaborates on how she felt after it all went wrong. "But the tigers come at night, with their voices soft as thunder, and they tear your hope apart." That tiger, for us, was juvenile arthritis.


Laura, our 22-year-old began telling us around age 7 that she was tired and that things hurt. When she was 9 the pain became much easier for her-, her to identify as she began to have pain in her heels, then her left knee, and then both hands. Finally, three days before Christmas, when she was 12 years old, we saw a pediatric rheumatologist who didn't hesitate to diagnose her with juvenile idiopathic arthritis. Today, her worst systems are centered around her hands and wrists, plus her feet and ankles.

Erin, who is now 19, began to experience pain around age 13. And next month we-, we'll-, we will mark her five-year anniversary of her own diagnosis. Erin's largest challenges include fatigue and struggling to have full use of her hands. She's a college student who has to work much harder than most kids her age to pull off attending classes, completing assignments, and being involved in college life.

And then there's the challenge of paying for college, uh, but you'll hear more about that as I continue our story. Let me tell you about some of the ways this disease affects everyone in our family. First of all, our life has become all about triaging and reorienting. Things can change on a dime for us, depending on how the girls are feeling. And the unpredictable nature of our days is like living in one big domino setup. Having two chronically ill kids, there are times when my care is divided, and I have to decide who is in more-, in need of my attention at any given moment.

Just over a month ago, Erin had jaw surgery on a Monday. That Saturday Laura needed to be taken to the emergency room for some disturbing new symptoms. Poor Erin, still very much recovering – suddenly the healthier one – had-, had to to give up my care because Laura was in more urgent need of attention. As I just mentioned, Erin had jaw surgery 5-1/2 weeks ago. Laura had a more extensive jaw surgery 3-1/2 years ago. These were necessary as a direct result of JIA destroying their TMJ, their temporal mandibular joints.

You see, not one of the drugs on the market today has been found to be effective in treating arthritis in the jaw. Some people have found success with certain drugs, but this is atypical. Compared to adult RA patients, JIA patients see jaw disease at a much



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higher rate. This, of course, has caused a cascade of dominoes in our lives. Erin is not able to work this summer and earn money for college because of the surgery and extensive recovery period. We now have to travel to a hospital two hours away for Laura to see a jaw specialist because she has surpassed the expertise of the doctors in our area, and medications have not been effective. Erin will follow her there in a year or so, most likely.


In addition to current drugs not effectively treating the TMJ arthritis, the types of juvenile arthritis my girls have, spondyloarthritis and psoriatic arthritis, do not respond well to what's currently available on the market. Fatigue is a common, incredibly limiting symptom in these particular types of patients, and as you saw on the poll, for many types of patients. Uh, it is not easily controlled with anything that's available. With these types there's a lot of trial and error, requiring frequent medication changes, and agreeing to settle for a drug that seems "good enough."

Uh, just an example, Laura has been through just everything that's available and we're hopin' that she hold onto what she's on right now. Uh, also, comorbidities are very often present in JIA patients of all ages. In a 2013 study, published in *Clinical and Experimental Rheumatology*, 62% of the adult JIA patients surveyed reported having at least one comorbidity. These can be other autoimmune diseases, such as a diabetes, or gastrointestinal disorders, or they can be problems caused by side effects of medication. A prime example is the fact that my daughter, Laura, has developed exogenous Cushing syndrome, ah, as a result of protracted use of steroids as a part of various, um, arthritis regimens. And that's very common.

The presence of comorbidity triggers the needs for more doctors, more appointments, more medication, and more dominoes fall. To this point I've talked mostly about my girls, but what about the effects on my husband me? At our ages and stage of life, um, with both of our kids now young adults, we should be able to do things like travel, and maybe devote more time to our hobbies.

Uh, I was a stay-at-home homeschooling for many years, and I loved that life, but I should have been able to go back to work by now. Uh, I should be able to earn money to help pay for college. I should be able to quilt for hours, or audition for a play. My husband and I should have been able to take the trip we had planned last year to celebrate our 25th anniversary. Because of JIA and all that comes with it, these things are not our reality.

I am only able to work very part-time because I need to be available to take the girls to appointments and manage the unpredictability of our days. My husband has worked two jobs for most of our marriage. We thought he would just have to do that to get us through those tough early years, but guess again. See, more dominoes. Here's what I'd like to leave you with today. Juvenile arthritis has stolen some of our hopes and dreams,



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but we've created new ones, and found new purpose in life. The fact that my girls and I are here today is proof of that.

That said, we'd be thrilled to be able to re-, resurrect some of our old dreams and hopes without pain, without fatigue, with effective treatment for all aspects of juvenile arthritis. Gaining control over this disease will be a game changer for my family, for every family represented in this room, and as well as those participating at home. It will allow us to live more predictable lives and re-, and reverse the domino effect. Everyone can hear-, everyone here can play their part in helping us to achieve this. And we would be so grateful for your help. Thank you.

[applause] [00:59:27]


Nick Kim: Hi. Um, my name is Nick Kim. I'm 17 years old and I am a rising senior at the Bergen County Academy, which is a high school back in New Jersey. Um, I work as an intern at, uh, Hackensack Hospital, um, in pediatric rheumatology. And I was actually diagnosed with enthesitis-related JIA last October, making it almost one year, which means that I'm about to celebrate my first, uh, birthday as a JIA infant this upcoming year, which is something I'm excited about. Um, but reflecting on how much progress I've made this year, um, I just can't help but think of how hard it was, um, at first for me initially, and how hard it still is for me now.

Um, so before I was diagnosed I used to play a lot of tennis. Um, I would play two to three hours every day, um, even in the school year and the summer. And it was something I really loved doing. Um, and even I wew-, when I went somewhere and they required me to write a bio, I would always write about tennis. And it was just something I really loved doing and talking about it. Um, and I felt like it was a part of me. Um, and that is until the elbow pain came. And at first I dismissed it as, um, tennis elbow. But when the pain started spreading to my knees, and then my ankles, and then my wrist, and practically every other joint I could think of, um, this is when I thought, "Something is definitely wrong."

And here we went to a pediatrician and he told us that he thought it could be mono. And I remember thinking, I was panicking, because I didn't know what to tell my mom because mono's transmitted through, um, kissing and . . .

[laughter] [01:01:19]

. . . how do I explain to my mom that . . . you know. Um, but as time passed, I realized that this was the least of my worries because with each passing day presented a struggle that I had no idea how to cope with. So I remember I woke up one day, and I was in my bed, um, and, it was like sleep paralysis. It was like I couldn't get up, but it was so stiff and I couldn't move. And so I had to call my mom and I was just lying there in



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bed and she had to sort of, um, put her arm underneath my back so she could sort of lift me up and . . .

Even on days where I could get up, um, I found that I couldn't dress myself, I couldn't, um, I couldn't chew, I couldn't swallow, I couldn't walk. And to see 17-year-old me . . . just having to see my mom dress me, it was just so hard to deal with that fact that I couldn't really dress myself and what was even worse was that one day I just woke up, because I heard something, and I just saw my mom, um, next to me, on my bedside, and she was just holding my hand and she was just crying. And I didn't really know how to deal with that, like, because, like, what am I supposed to do? So I just-, um, I was-, just felt so helpless and I just watched her cry. And I want to say that it's gotten better from there, but I really can't because it was just a decline from there until I got my diagnosis. And on my diagnosis date, which is October 13th, um, 2017, it was so traumatic for me because, um, I feel like I've had my fair share of medical trauma in the past. Um, I've broken an arm, um, I've ruptured my ear drum. Um, but even through that, I never-, my mom has never shed a tear, um, in front of the doctor. And when she heard my diagnosis, um, she just completely broke down. And me, as her son, when I saw that, it was just so hard for me to see her suffering because of me. Um, but as much as it hurt me physically, for me, what hurt even more, was the social aspect of adjusting, um, at school and in terms of, like, fitting in with my friends and surviving in school.

So as a teenager who can never leave his phone, and I'm, sort of, I always prioritize my social life above every other part of my life, um, knowing that I couldn't do the things I wanted to because of my disease. It just hurt me so much. And this was evident in scenarios where, um, in school, I had to drop out of AP Calculus PC. And I worked so hard to get in that class, but my body couldn't handle the workload that, um, this class required.

And other times where I couldn't go upstairs. So I could take the elevator, but I couldn't because my friends would ask me, "Why are you taking the elevator." And I didn't know what to say, um, because I didn't want to give off the impression that I was, I mean, lack of better words, I was crippled.

And even my SAT, which is so crucial to my college application process, which I'm starting now, um, I had to cancel it because I couldn't sit there and hold the pencil for four hours, and just in a chair for four. It was just so taxing on my body, so I had to cancel my SAT. But the worst part was that I couldn't even tell my friends about it, um, because I didn't want others to think I was weak. And even if I did, um, they wouldn't take me seriously because of the label, "arthritis." And this is why I hate, I hate the word arthritis because, um, yeah juvenile idiopathic arthritis, it's . . . arthritis undermines the, um, struggle that each one of us, as patients, have to go through and how much hardships we face as not just patients, but just as people.



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Um, so there are few points I really want to get across this morning. Uh, and the first is, yeah, um, pain is pain, but what hurts even more for me is the emotional burden and the social aspects of adjusting to this disease. So, yeah, you might not see, um, it physically present in a patient, but you have to automatically assume that the patient is hurting so much inside. Because, for me, it was way worse than the pain, the physical pain, that came with this disease.

My next point is that, um, as a community we have come so far, uh, in a sense, us, as patients, we were our own doctors. Uh, we experimented on our own bodies. We had to see what worked and what didn't, and by adding 1% here, 1% there, slowly that 15% rose to a 90%, and, um, progress was evident. It was sure, but it was-, it was slow but it was definite. Um, and I still remember what it was like to feel like not to be able to get up, and to see my mom crying, um, at my bedside like that. Um, but honestly, it's because of all the effort that we put in together, um, collectively, that we are where we are right now. So I encourage all of us to try even harder, um, to come up with new unprecedented ways to achieve the unthinkable.

Uh, my last point is to keep listening. I want to remind you all that this is a disease that I-, I will never, um, be free from. It's something that will never leave me. And as a fellow patient, I've gotten so much insight, um, and hope, from hearing about the stories of, um, each patient –listening to their resilience. And one of the most valuable thing is even giving-, even under the, um, thought that this is disease is so, um, hard on me, but something I've gained from this is the caregiver community that, um, JIA offers by enduring together, uh, being united, and replacing our shared sense of despair with hope. Um, it's provided me not just with reassurance, but maybe, in the end, that idea that, yeah, it'll be okay.


Um, so I ask you to continue to listen to panels like these, um, getting insight from individuals, uh, and not patients collectively. Um, this is just as important as the science behind the scenes. Um, and maybe if we do all of this, and maybe one day, um, in the future, upon hearing a JIA diagnosis, we'll sort of let out a sigh of relief, rather than being scared of a journey of uncertainty. Thank you.

[applause] [01:07:11]

Kirsten Wilder: Hello. It's hard to follow Nick.

[laughter] 07:22

My name's Kirsten Wilder and I'm from southern Maine. Grew up in this area, though, so it feels a little big like home. I work part-time customer service at a specialty foods company, but my primary job is being the mom to three teenagers. The youngest of which is my daughter Katherine, who's over there.



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Um, when Katherine was three, she was diagnosed with her own version of JIA. This coming fall she was going to be a freshman in high school. She's not quite ready for that yet. Um, her treatment, uh, we receive in Boston. Boston is about a two-hour drive for us. So I think my car knows the way and back by now; it's been 11 years. So Katherine's JA story started, um, just days after her third birthday. What started out as a little cold turned out to actually be a bad case of strep throat. She got the scarlet fever rash and everything. It was pretty impressive. High fevers – and the fever didn't go away.

She is . . . again, she's my third kid. Everybody's had strep; fever usually goes away. And then the rash changed and then about a week after she started the antibiotics she woke me up in the middle of the night screaming bloody murder. My little quiet three-year-old who was content to just smile and watch her big brothers play was screaming like I've never heard anybody scream before.


Um, by the end of the day we had spent the entire day at the hospital. Of course it was a Saturday, so her pediatrician came in, took us to the hospital from his office, spent the day with her running labs, running tests, trying to figure out what was going on. Labs, of course, were terrible. Um, he personally looked at them under the microscope multiple times and was very proud to come in and say, "Well, I've ruled out cancer." "Excellent. Okay." So that was the day one.

Um, after that it was two steps forward, three steps back. This girl who was very social, very chatty, knew everyone and everything, stopped talking, she stopped walking, she stopped feeding herself. She started actually hiding from people that would come to see her. She began just laying on me – literally like a newborn infant. Three-year-old who refused to walk. And it was interesting because, like I said, it was two steps forward, three steps back.

Many evenings, by the time Dad got home from work or the brothers from school, she would try to get up and go, and be super excited. She'd find a way to wobble over sometimes, sometimes not. So it was very confusing, very, very, um, very terrifying, very confusing.

Uh, over the next couple of months she had multiple misdiagnoses of random childhood things because every day was a new rash, every day was a fever. Then the next day it's all gone and she's okay.

Um, about . . . I guess about a month in, her pediatrician went, "You know what? I need to send you down to Children's," Boston Children's in our area. He said, "Let's just rule out arthritis." I'm like, "Arthritis? What are you talking about?" So, um, let's see, this was April when the symptoms started. In July, mid-July, she had her first appointment with who was to become her new best doctor. And her rheumatologist, her pediatric



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rheumatologist took one look at her and said, "Well, this is an obvious case of JIA." I went, "Obvious!"

She showed me that . . . my . . . what I learned to be of my hyper-mobile child – who could bend her knees backwards, her elbows backwards – now had one knee that was contracted, and stuck like . . . that. So they were opposite. One went back and one went forward. So then we immediately began what was, uh, protocol in our area at that time, which was six weeks of NSAIDs, and let's see what happens. And it was like a gift. Amazing! Immediately she felt so much better.


And then, within a week, we realized, "Well, she was really, really, really bad off." So much better wasn't very much better. So soon after it was DMRs, biologics, uh, everything that went along with that. I think, uh, Prednisone was in there week one. Um, just one med after another trying to get this girl back to functioning.

So over the next several years she's gone through three DMRs, five biologics, injections, infusions, pills, and the works. She has seen every specialist under the sun because something she likes to do is – what often happens with these things – is she picks up random, different illnesses, diseases, that need their own unique specialist. Um, so between the regular appointments with rheumatology, monthly infusions, PT, OC, continue, continue, continue, we know how to get to Boston and back very well. For Katherine the pain and the exhaustion has always been the primary issues. Once she was, um, on these medications, her doctor called her a "simmerer." Everything just kind of simmered. So she can manage with the joints, she can manage this, but the pain and fatigue never go away.

For us as her family, it is the management side of things. Like I said, the multiple appointments, the constant traveling, but the impact is, uh, it's just all pervasive. For school for this girl, extensive support, wheelchair, uh, everything you can name, and still she's usually on a partial day, often a partial week. If there's one thing – two things – that I could leave you with today, besides for my thanks for being here, is that this is all pervasive.

You are not looking at a kid who goes and gets their medicine, goes home, and goes back to school. This child has never been sports, this child does not stay after school, she does not . . . If she goes to a party, it's, "We really have to think about this. Is this worth it? How's this gonna affect you the rest of the day?" It is an all-pervasive thing, and as for the family as well.

I'm part-time because I'm fulltime caretaker. So the financial aspect, the location. We can't leave the job, we can't leave the area, we must have these securities in place for her.



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And the second thing that I just wanna reiterate a million times over is the appreciation for this great group of people that all have this one common goal, and the idea of actually having a stronger priority on our pediatrics and the processes, streamlining – whatever we can do to get it down, to get these meds that these kids need immediately, have the choices. She's been told multiple times with different points in her life that "this is it." "This is it." Whatever your side effects are, whatever the problem is, whatever it's not doing, this is it.

It's thrilling to see that big long list of things that are in the pipeline that may some day be options for her. And I appreciate each and every one of you for being here, for listening to us, keeping your open mind, and thank you.

[applause] [14:53]

Corinne Pinter: Hi everyone. Uh, my name is Corinne Pinter. I'm here from Sugarland, Texas, a suburb of Houston. I'm representing my 10-year-old daughter, Emily, as you see her in the pictures. I'm a mother of three. I have a 12-year-old, 10-year-old, and 7-year old. Um, I'm currently, um, a part time cosmetologist, but a fulltime parent and caregiver to my lovely little kiddos, especially Emily.

So our journey started eight years ago, um, on Emily's second birthday. She fell off our swing set in the back yard, playing around with her sister. She presented pretty well afterwards, nothing broken, nothing crazy. Made sure she was all right, checked out, she's great. Woke up the next morning for her second birthday party – exciting day. Um, however, going to change her, grabbing her little hand, she pulled her hand and winced, and I noticed she had two little swollen fingers . . . which was weird. Then we continued to change her, got her up, she walked through the house and she was limping. Again, not normal for a two-year-old on the day of her birthday party. So we decided to take her to the ER to get her checked out and after some examination, x-rays, blood work, they thought, "Well, she's got reactive arthritis so, maybe, a virus – she's two. What two-year-old isn't sick with something. Seemed strange, but we had some sort of an answer so we had to follow up with the rheumatologist and a pediatrician.

So we get to the pediatrician first the next week and run the blood tests. And they're like, "Oh, yes, it's juvenile arthritis." Well, that just isn't normal to hear. I have a two-year-old. Like it's no big deal. So again, with further, um, testing, you know, MRIs, scans, all the nine, the blood work, just to make sure our bases are covered. She, indeed, was diagnosed in April with, um, juvenile idiopathic arthritis.

So in all this, your mind's spinning, "What do we do?" Medicines . . . not medicines . . . is she going to be in a wheelchair . . . is she not going to be in a wheelchair . . . are there surgeries? All these questions that most mothers and parents of two-year-olds don't ask because it's not "normal."



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So little Emily gets this diagnosis and we talk about treatment, and I have a phone call with the doctor. And he said, "Well, we're going to test her for leukemia." I was like, "Leukemia, why?" He's like, "Well, we can treat that." "Okay." [chuckle] [17:43]. Right to the heart, right to the gut, last thing you want to here is that's what they're looking for to treat. Once we figure out she does not have leukemia . . .the juvenile arthritis. You know, we realize we have a long road ahead of us that is going to be, a possibly, a bumpy road, maybe a smooth road. We don't know. It's a big question mark.

So in the early days of this, um, it's the constant struggle of . . .people don't understand the gravity of what this is on us, that weighs almost every day – literally every day. Um, we do get some excellent suggestions from people of what we should do . . .


[laughter] [18:22]

. . . and how we should treat our child, because everybody, including, you know, Dr. Internet, knows all that goes along with it. Not knowing you have an excellent care team that actually is trained and can guide you where to go and what to do with this. And even though they are helpful suggestions, icing joints doesn't always fix arthritis, there's no "fix."

But in the light of that, there are very-, there are a lot of functional challenges she does have. Um, she has to have pencil grips at school, accommodations, you have to be careful of backpacks, shoes, clothes, you know. A little toddler . . .I mean, you see her in there. Certain shoes she has to put on because she can't buckle, you have to Velcro. She can't tie shoes because the mobility and flexibility in her fingers isn't the same. You know, zippers, buttons, so many things go into our day-to-day lives that we have to think this out. And a lot of times clothing companies, at the time, were not thinking what we are. Things weren't available for us, however they are.

The fact that this is constant is another thing families-, other family of "normal" kids don't understand. My-, one of my best friends, the other day, came up with an analogy. It's like, "It's like wearing glasses. When you need glasses, you have to wear them every day to see." Emily has this every single day. We, as a family, have this every single day, and it's something we have to constantly think of, day-to-day challenges.

One of the things I have . . . And let me grab it, I forgot it. This is Emily from day one diagnosis to today – eight years of appointments, eight years of illness, eight years of changes, this comes everywhere with me. A lot of times in the doctor's office they'll be looking up on the online charts and I flip through to the date, and I said, "Yeah, I remember. It's right here, all of the things." Healthy kid's parents, don't have this. We have this. We have this for ourselves, we have this, sometimes for our other kids, because when you have multiple kids, you have to make sure you know what's going on with everybody at one time. [chuckle] [20:38] This is where your brain lives a lot of the time.



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And another thing is it's an impact on the whole family. As you've heard of other moms up here, we don't always get to work full time because we full time take care of our children. We take care of their appointments, we take care of everything day-to-day. It's hard for us to get back into the workforce full time because you don't know what the day's going to bring – if we have to call in sick, if they're having a bad day. You have to leave, they have to miss school, they have to miss appointments, they have to miss activities. It's our constant burden.

So now that we are eight years later, the difference between 2 and 10 is at 2 I had to make all the decisions. That's tough on a mama. I had to make some very tough decisions on medications, course of treatment long-term for her. Now that she's 10, she can start to make some decisions on her own. However, we still have to guide her in the right direction.

Another thing to know is this is invisible to a lot of people, just as Nick said. It looks like she's normal, and smiling, and happy. Underneath . . .not the same. Behind closed doors . . .completely different kid – crying, tired, overwhelmed, mentally drained, all the things. But over the years what I've learned is how important it is to speak up to get involved. And we have to be part of the solution.

One of the things I'm trying to do is get myself involved in participating in the research, helping to provide the insight of parents, because we also have to speak on behalf of our children. And we can't make progress in finding new and better treatments if we don't work together as patients and parents, and also giving the younger kids a say in all of this.


So we, as the patients and families, are taking this on, as you see. We appreciate this, and we do look forward to working with all of you to make this future for our children just a bit better.

So again, thank you for allowing us to be here, and listening.

[applause] [22:53]

Suz: Well, first of all. Wow. And thank you all so much. And we heard so many-, so many important points. Like we heard about the unpredictability of the disease, we heard about how it's a thief – and it steals all kinds of things – um, we heard about the emotional impact. Not just the emotional impact on parents watching their kids hurt, but on kids watching their parents hurt. Um, but we also heard about resilience, and hope, and energy to work together to make things better.

Um, so I think our panelists really kind of, um, set us up for-, for a great conversation. So thank you all so much for-, for sharing all of that.



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We're gonna move right into the next part of-, of the conversation that they've begun. And here, again, we invite all of you to please join in, share your thoughts and insights. We've got our two mic stands. Uh, if you have challenges getting to a mic stand for mobility issues, let us know and we can run one to. But otherwise, you can kind of form lines.

And we also have our folks, um, on the webinar, you-, you've got instructions for how to submit your chat questions? And we have folks in the back that are moderating that intake, so as questions are submitted via chat function, we'll be able to take a moment and come to the back of the room to-, to voice those questions and-, and comments and thoughts.


So do we-, do we have any brave volunteers for our first question, which is how does JIA affect your daily life on the best days and the worst days? And I will share that I think that this really can speak to that unpredictability that you all mentioned. And we heard in the focus group that, um, there is a huge disparity, and sometimes, when you're talking about making accommodations and changes, that's actually on a good day. On a bad day, maybe you're not doing anything at all. So I think there's a great deal of variance. So I see some folks, um, by the mic. Great. And please introduce yourself with your first name when you give a comment.

Caitlin: Is it on? Okay. My name is Caitlin. I have a beautiful four-year-old little girl, Nora, who was diagnosed right after her third birthday with systemic JIA. And for us the daily life is hard. I have-, she's got two younger brothers, so as a mom I gave up my career to stay home and make sure that she got to all of her appointments for therapy, for infusions, all of that. Um, so that's a lot on us, but we do it because she's great.

But the hard part on our daily life is watching her and we-, you can see in her mind how things work. About six months ago she came home one day from ballet and said, "Mommy, I can't do it anymore. It hurts and I can't go back." And we spent two weeks with her in tears every day because she wanted to go back and be with her friends, but she didn't want to deal with the pain.

Um, and then about three weeks ago, she was like, "When I don't have arthritis, Mommy, I'll go back to ballet." And we just kind of sat there, and I didn't have much to say to her because we have failed three medications . . . or two medications, and we're continuing to try the third one, but she's not in control, and we still have really hard days.

And so our daily life, um, even today we FaceTimed her and I just tell on her face, and I said, "Honey, what's wrong?" And she's like, "I don't feel good today." So, um, we face a lot of it, and she's just starting to realize kinda how to control her, or how to verbalize her own feelings on how she feels. And we're really trying to push that. But our life has been completely changed from this disease, and we really need better things for her,



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because even in the past year we've kinda lost a lot of her childhood. She spends a lot of it at home and inside, so that's why we're here, and we're hopeful that it's gonna push for some of that.

Suz: Thank you so much for sharing that.

[applause] [27:18]

Tammy: Hi, I'm Tammy. And our son is 13. He has FGIA. And his onset was when he was 10. We have been through . . . we're on our third biologic. We have never gained good control for him. Um, daily, I would say, mostly, I think we live in fear because things can change so quickly. He does trend toward MAS very easily. We have some pharmacy problems and even 6 to 11 days can just ch-, flip our kid completely upside down. Um, when he can ride his bike, when he can do full days of school, those . . . or mow the lawn. I mean, for us those are victorious days. The same way we would cheer for an athlete – we're like, "Yes, he had a normal day," or, "He got to go to Grandma and Grandpa's and play really hard." And we're gonna pay for it for two weeks, but "Yay!"


Um, so I would just say here, and just already being here, I can see that no matter what category we're falling into, I did not know that we had so much in common as far as fears, and feeling misunderstood, and hating the name arthritis. However, this foundation is doing so much for us. I mean, my eye was just "ploop," popped open just from the commonalities and the common struggles that we all have, that I already feel so much better understood. And I think that already, um, as far as the systemic piece . . . you guys, you got it. So awesome, and thank you so much.

Suz: Thank you.

[applause] [29:16]

Stacy: Hi, my name's Stacy. Um, my daughter's six years old; she was diagnosed at three. Uh, she has extended with uveitis and hypermobility. Um, I'm a single mom, so the ability to work part time and be 100% fulltime caregiver is very difficult. I am blessed enough that my employer is very flexible about being able to work remotely when I've needed to, or take extra time off when I've needed to without penalty, but that is a fear. I am, um, the insurance carrier for her . . . I can-, I, you know, I've had friends tell, "Well," you know, "maybe," you know, "my employer's hiring and," you know, "it'd be an increase in pay." And while that sounds amazing, that fear of not having the medical insurance that I currently have, the established doctors. You know, having to change doctors, change-, go through all of that pre-approval again, to make sure that her treatment doesn't change.

Um, coming on this trip from Des Moines, Iowa, uh, we had to think through all the walking, and, you know, how is she gonna do? She's one of those little kids who . . . she



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doesn't people to know. If she decides to tell, then she's going to share, but then otherwise she doesn't want me bringin' it up, she doesn't want me tellin' people, she doesn't want people to know that she's hurting – she'll push through it.

Um, the most common comment I get from people is, "Well, you'd never guess. She looks like a normal kid." And then as soon as, like, somebody says, as soon as we get behind that closed door, it's meltdown, it's, "I'm hurting, Mommy. I can't move." Um, we've had two days . . . we got here on Sunday. Monday, Tuesday we did all of the tours, and we brought her chair with us just in case. And, um, did . . . I think we logged almost 30,000 steps on Monday and Tuesday alone. And she was so excited to see everything. And, kept askin' her, "Are you okay? Do you need to sit down?" "Nope, let's keep goin'." Yesterday, all day in the hotel, "Mommy, we need the chair."

Um, she is, um, six months medicated remission at this point, but we are also now looking at her collar bone that now has inflammation since we've gotten here. Um, we've done soaks every night. I've made I don't know how many trips to Walgreens already since we got here. And she brought her weighted blanket with her which is kinda helpin' a little bit, as well.


But that's something that we always . . . Even bein' in medication remission, I am constant-, I don't feel . . . I-, I remember when the doctor used that word and my mom and I cried, and she couldn't figure out why we were cryin' because, "Mom, you normally don't cry until you're on the way home.

[chuckle] [32:16]

"You're doin' it in this office." And, "Are these happy tears, are these sad tears? What's goin' on now?" And, you know, explaining to her what remission – what that word meant. But still, every day we know that that word can be taken away from us, and living in constant fear and still havin' to plan the hypermobility pain. Every eye appointment – we've had a terrible time keepin' her uveitis under control, and we know that's the wildcard to take us out of remission in a heartbeat.

Anytime-, and she's one of those kids – she's very rare – she has symptoms when the uveitis flares, so she gets sensitive to light and can't handle any kind of bright lights, and that kind of thing. So that's our trigger a lot of times.

Um, helping her understand that even though she's in medicated remission, she still has to take her shots, she still has to do her medication. It's very hard to explain to a six-year-old. So on that day-to-day, how do you plan . . . you al-, even-, you know, having our word in our life, we are still thinking every day, "How-, what do I need to plan for?" Because it can change any second, and I'm beyond blessed that we're here, and totally don't feel kinda "alone" anymore, so thank you for listening to our voices.



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Suz: Thank you.

[applause] [33:44]

Ellie: Hi, my name is Ellie, and this is my mom, Becky. Um, I'm 13 years old and I was diagnosed with psoriatic arthritis two years ago, or about two years ago. And when I was diagnosed, I was currently wearing two wrist braces every day. I couldn't write in school. I wasn't sure if I was gonna make it through a full day of school or not. We always had grandparents ready to come pick me up if I wasn't able to make it through the whole day. And those were pretty much some of the worst days that I've had when I first started.

And now, every now and then I get different flare-ups and we don't know when I'm going to have them. Um, I sometimes have- . . .uh, this last year I had to go into school for like a week in a wheelchair, and my friends had to take me to my different classes, and I was definitely depending on other people to help me get around and write for me in school, and if I wasn't in school, give me my work. And my parents had to write for me at home. And I couldn't play sports, I couldn't run around or go to different activities, and it was very hard to go to sleepovers because I had to remember to take my medication, eat before I had to take different pain meds. I was taking, uh, the "as needed" medication pretty much every day for awhile.

So it's really unpredictable. And even though I'm on a injection every week, I still have those bad flare-ups every now and then.


Suz: Wow. Thank you so much, Ellie. You're very brave. Thank you.

[applause] [35:47]

Yeah, thank you so much for sharing that. Uh, you can go . . .

Adam: Good morning everyone. My name is Adam and I'm from Southern California. And my wife, Gwen and I, currently have seven children. And it is our youngest daughter, Sage, who is systemic. And listening today to the panelists and some of the other JA parents and-, and, uh, survivor with JA, I come to realize that, to me, my daughter's arthritis is the eighth child in our family, because my wife and I could go down through all seven of the kids, and we get down to the end and we realize there's still one more child that we need to hold onto, grasp, and take care of.

And when we come home from work and we're tired, I can see the race that my wife and I have to grab four kids first because the other one's going to have to have three kids, and then that last eighth child. That can be so difficult, and so challenging, and so unusual, because you don't know when you wake up whether it's going to be an eight



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child that is in the "terrible twos," or an eighth child that is going to be that calm 13-year-old, or that eighth child that is that horrific, argumentative, 17-year-old daughter.

[laughter] [37:23]

And that is a daily task that we go through, because you don't know what it's going to be like. And you can set up all seven perfectly, and then there's that cloud of the eighth that sits over my daughter, Sage. There's a small cloud that blocks the sun, or a horrific thunderstorm that absolutely rains down on everything that we call a parade of having a family. And I think it's living with that eighth child that is hard to describe to a family that doesn't have a child that is suffering or surviving with juvenile arthritis. And it's even difficult to explain it to doctors and researchers because until you have that eighth child, you'll never know.

But I hope, in listening to us, you have an idea of what it is to have that eighth child.

[applause] [38:31]

Suz: Thank you. I think we-, we've got some really clear themes, both from the panel and-, and what you've all shared in the room. This unpredictability, just not knowing when it's gonna strike, or when it might be a good day that you can celebrate. Um, and-, and that's a real challenge. I think several people spoke to fear because you're fearful of those bad days, that loss of control. Those are all incredibly challenging. Um, so thank you all for sharing.


I wanna check in the back and see if we have any questions or comments from our online participants.

Vincent: Yes, Suz. Thanks. Actually, th-, there's one comment here, uh, in particular that kinda sums up exactly what you were just saying, in that the, um, this-, this parent commented that on the worst day their-, their son quits, uh, talking, walking, eating, and was in agonizing pain. And now the pain and symptoms are a roller coaster from day-to-day, which is kinda what you were just, um, talking about.

Suz: Okay. Thank you Vincent. Anyone else in the room wanna contribute kinda that-, that distinction between good days and bad days. Um, I saw a lot of nods. Maybe what has been said already was spot-on for what you experience. And we can certainly go to our second discussion question, and I think folks are gonna have a lot to say about this one, too.

Oh, oh, we do have a question. I'm sorry. Go ahead.

Nikki: It's not a question. It's just our story's a little different. Our JIA . . . Our daughter-, my name is Nikki. Our daughter is now eight, almost nine, was diagnosed at JA at 13



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months, but got stable within two years with the JA, and then she got uveitis. And for us, and I could very much relate . . .for us, uveitis has been our biggest roller coaster. Um, and, for-, for Becka, our daughter, it's silent. There is no symptoms.

So we'll go a year with nothing. We'll go to the eye doctor every two or three months, things will be great. And then all of a sudden everything drops out from under you. And you're like, "Oh, my God. This again." And for me, as much as my fear of her having JA, is my fear of her going blind, and having, you know, damage to her eyes, you know. And I know there's not one that's worse or better, but for me, having that fear of my daughter is huge. And-, and again, it's that constant, that fear that we all have. Um, but it's, you know, we have a lot of good days. You know, she also quit ballet because of pain. Um, but most of the time she lives a really great life. She's a good artist, she's a fun kid, but I can tell, emotionally, sometimes, when we have shot night every Sunday night – that's a tough night. Remicade's fun. She gets to get toys and it's exciting, and even though it may be a bad day for Mom and Dad, it's a fine day for her. She doesn't mind it as much.

Um, but when I go to the eye doctor and they tell me that her eye is covered in cells and they can't see in the back of her eye, it just breaks my heart. And it-, I wish . . . And-, and I was on a panel last October, and we talked about different studies about different treatments, and, um, medications, and I found that there's just a not a lot . . .not any studies of uveitis in kids. Um, or very little. And the ones that are have very small sample sizes. And I think it's a very important thing that we need to look into. You know, it's as scary as everything else that our JIA kids go through. So that's my two cents.

Suz: Thank you so much for sharing. I think . . .

[applause] [42:36]

. . .and I'll-, I'll be sure to sort of call on you later today when we talk about both treatments and clinical trials. Because we do wanna surface . . .we have a lot of commonality, but we also some specific needs and hopes among the community. Um, do you have a comment?

Male: I wanted to speak to question two. I didn't know if you were ready to move . . .you mentioned earlier, so . . . [laugh] [43:03]

Suz: Sure, we can . . .we'll-, we'll go to question two.

Male: So as far as change in our life. Um, you heard from my wife earlier. My daughter has systemic JIA and, um, because of all the medical care that requires, and the multiple specialists that we had to see throughout the week . . . When she was first diagnosed I was working for a very small business in town. Um, we had okay health



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coverage for my wife and I, but really nothing for the kids. They were on public aid, and we were very limited on where we could go.

And so, as a change, I took a job for a much larger company, um, that totally turned our life upside down, in addition to systemic JIA. I now travel 75% of the time, but this job affords me the ability to pay for these expensive treatments that work 50% of the time for my child. And also to make sure we have coverage at all the different specialists that we need to see. So that's change number one.

Um, and that, you know, the burden of that is if I'm traveling and my wife calls me . . . is she calling me because I put something somewhere where she can't find it, or because she has to go to the Emergency Room for her labs to make sure we're not on the cusp of MAS, which is a potentially fatal complication of systemic JIA.

And so the other major change that we made in our life when my daughter was diagnosed – we have two children – a daughter and a son. And we made the conscious choice that we didn't want our son, the non-JIA child to ever be alone, whether that was when he's being raised in a waiting room or if the MAS were ever to take her. We wanted him to have somebody else. So now our six-month-old is over in the childcare room. The other two kids are at home, but, you know, that was something that shook us very much to our core. And I don't know if we would have made that decision in the same manner if it weren't for systemic JIA.

Suz: Wow. Thank you so much for sharing.


[applause] [44:47]

That-, that is a significant life change, definitely. Um, what do other have? We have . . . Colleen?

Colleen: I wanted to just tag on a little bit . . . wh-, what he was saying about siblings . . . is that. And then everybody's talked about how this affects your whole family. My daughter was diagnosed at 3 and she's 20 now. But, um, I have a 15-year-old and a 9-year old, and when they were really-, when they were small, the things that they might have done, they didn't get to do either because of my daughter. Just because, you know, you can only do so many things.

And then also, I-, I-, I'm embarrassed to admit this, but I didn't let Jack play soccer because Caitlin couldn't. And then I felt bad, like, "Ooh, she's not gonna go and watch Jack play soccer." So I just, like, didn't let Jack play soccer. And it-, it-, you know, so it affects the whole family.

And the other thing, you know, a lot of moms have talked about working part time, and I-, and I did that, too. And now that Caitlin is 20 and I'm thinking, you know, now I'm, like,



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working full time, but this planning for, you know, how she may need me and her life doesn't stop because she's 20 now. I mean, it has affected where she went to college, and what she's studying, and what her potential careers might be.

But, you know, she had her hips replaced at 11 and 12, and she's in that 2% that have been on more than 10 of those medications. So what does that mean if she wants to have children, and what does that mean for me? Do I get to retire? Do I have to retire? Am I gonna need to retire so I can help her be a mother if she wants to be a mother? If I help her be a mother, do my other kids get to have kids? I mean, it's like this planning thing doesn't end now.

And not to freak out those of you with, like, little kids. I'm sorry, but it's just, you know, this-, how this affects what we choose to do, where we choose to live. It doesn't, uh, it doesn't end, even when the disease is quiet, it doesn't end. That's my important point. Thank you.


[applause] [47:02]

Suz: Go ahead.

Erin: Hi. I'm Erin. I'm Anjie's younger daughter. Hi Mom. Um, so she mentioned that I'm in college. Um, I am a rising sophomore theater major. Um, I go to school two blocks away from where I live, which is really, really nice. Um, but, you know, with college, it's a major change for people anyway, but I have arthritis and a couple comorbidities on top of that, so that just makes all the planning even longer.

Like I said, I'm a theater major. I, for this semester, have lined up a couple technical jobs, um, with our productions, which I am extremely excited about. Um, unfortunately, our technical booth, where the stage manager, and soundboard operator, and light board operator sit, is up three flights of stairs, and there's no elevator. And there's really no way to make that space ADA compliant with the structure of the building. So on, you know, days when I am going to be a stage manager, um, for our second production of the year, I'm going to have to be planning very carefully how I spend my energy so that at the end of the day for those performances, I can get up those stairs. Um, right now one of my greatest fears is that I won't be able to get up those stairs and call the show. And there's gonna be a major problem and I'm going to feel terrible cuz I, you know, gonna feel like I caused it, even though, you know, I can't really control my arthritis.

So yeah, so this-, so this is in November, so I'm definitely planning for this. Um, but, you know, I can't really study abroad either. That's something that's greatly emphasized at my school. There's big "study abroad" fairs. I'm like, "Look, I can't go cuz I won't have my mom there to help me around in the wheelchair and things like that. Can't really take an infusion on the road with me."



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And it's, you know, for those of you who, um, have tried traveling with injectable medication, you know that can be very, very difficult. So, you know, even, like, even a short two week trip is just too much for me. So there is so much planning day-to-day. Sometimes I'll be like, "Hey, Mom, I'm not coming home today because I can't do the walk back and forth three times today, so I'm just gonna stay on campus. I'll see you at two o'clock in the morning when I get home from rehearsal. So there are so many more things that we hafta plan for, um, that just normal people would never even thing about. Thank you.

[applause] [49:47]

Laura: Um, my name's Laura. I'm Anjie's older daughter. And I haven't really-, I don't have a story like, uh, Jean does, changes I've had to make in college or anything, cuz I haven't gone to college. Um, but one change that will be happening, uh, very shortly, um, is I'm getting a service dog. And it is partially for my arthritis, partially for my comorbidities., but it will-, the dog will be able to do things like open doors for me. And this is a big deal for us because Mom and Erin are allergic to dogs. So we're planning how we're going to be able to handle this so that I can have more independence and, um, access to a companion who can help me. So, yeah . . .


Suz: Great. Thank you. Good luck.

Melanie: Hello everyone. My name is Melanie and I will be speaking on the treatment panel later today, but, in, um, the essence of saving some time, I thought this might be a good opportunity to share some of the burden of disease type points. Um, otherwise I-, I would probably have a four-hour presentation up there.

Uh, my daughter was diagnosed at, uh, 20 months old. She started with symptoms that are on the 11-month-old, and she is now 7. Um, you know, as-, as far as changes that we've had to make . . . basically everything. You know, Corinne's point about, um, the clothing and the shoes. You know, they stopped making Velcro tennis shoes at a certain size and my daughter, um, you know, is getting there now, so she finally learned how to tie her shoes, but what if that's not an option, right.

There are days where we wear leggings and pull-on boots because I know that we're gonna need those. There are days that we can wear jeans, but they can't have a snap, they have to have a hook. Um, there are days where, you know, we-, we kind of have to plan. So even the good days, um, you know, require some planning just because of medication and things like that.

The bad days, you know, I send her to school and hope for the best that I don't hear from the nurse the minute she walks off the bus. We have tummy trouble almost daily. Um, you know, whether an-, an anxiety thing, a side effect of a medication, her actual disease, some sort of co-morbidity we're developing. Who knows? But if she feels that



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crummy, you know, I-, I am fulltime mom and so for that reason I've had to cancel attending some meetings before, you know, because I needed to be nearby to pick her up from school.

Um, every single ache, pain, scrape, tumble requires rheumatology mommy, not just regular mommy. So when I hear, "My leg hurts. My elbow hurts." She could have fallen and have a scratch on her elbow, but I immediately look at not that one, but this one, too. Is this one puffy? Is that one normal? You know, you default into thinking the worst that it could be – the disease. And to be quite frank with you, rheumatology daddy doesn't cut it.

[laughter] [52:57]


So if I get a text that says, "Meghan's elbow hurts," and I say, "Did you . . ." He goes, "Yes, I checked both of them." It doesn't matter. Send me a picture. I need left, right, I need all angles. I need to look at it because rheumatology daddy is not rheumatology mommy, right?

So even when I am traveling, um, you know . . . And everybody will meet rheumatology daddy. He's finally here for the first time at a meeting. Um, and so he'll be here later. Um, you know, just planning around, um, the medication . . . I mean, we are on an infused drug at this point. Um, rheumatology daddy has offered to take her to an infusion before – over my dead body.

[laughter] [53:34]

[laugh] [53:35] And so, and she's doing great. We don't have an issue with her infusions. She sits there by herself, I sit in the corner and hang out for 3-1/2 hours, but, um, you know, it-, it requires a lot of planning. We ended up with an infusion on a day that her entire class was doing a really fun Christmas activity, and I could not move it. There were no appointments available for another week. I didn't want to delay her medication, so she had to miss that, you know.

There-, there definitely is an impact even when your child is doing well. Um, you know, and I think that, um, you know, this impact hits the entire family. I'm with Colleen, my older daughter, doesn't get to play impact sports either. We just-, we don't do it, we don't do soccer, we don't do flag football, we don't do . . . You know, we don't do the things where one could get hurt. Quite frankly, sometimes I feel like it's-, if my older daughter gets hurt, is that going to trigger her underlying potential arthritis? Um, she injured her back doing something silly a few years ago and I immediately said, "Yup, this is it. Now both of them are diagnosed. Here we go," and luckily everything – knock on wood – um, has been okay with her. [chuckle] [54:38] But, you know, it-, it definitely affects how we plan absolutely positively everything.



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Um, we-, I don't either of my children to attend birthday parties at trampoline parks or bounce houses and things like that because I feel like it's not fair . . .so it's not fair to my older one all the time, but it's not fair, you know, to my younger one to have her sister do those things.

Um, and the immune suppression – um, that probably hits us the most. If it's flu season and the cousin had a cough a week ago, we cancel Thanksgiving. I-, I'm not willing to risk my daughter getting sick. Um, we've been in a lot of positions where people have not been super thoughtful, um, and have attended, and my child gets sick and we're down for a week as opposed to the 48 hours someone else is down. So we absolutely have made a bazillion changes and that's with a child who's under pretty decent control. So, um, we'll talk more about treatment stuff later in side effects, but those were some of my points, um, you know, that I wanted to make about-, about this disease and how it's affected all of us.

Suz: Super. Thank you Melanie.


[applause] [55:41]

I know we're-, [clears throat] [55:43] we're just about to our lunch break, but I want to make sure we hear from both of you, and then we'll ask for any, uh, comments from the back and then we'll all-, I'll close us out for lunch.

Brook: We're over here snapping because we don't want to be between you and lunch. Um, hi. I'm Brook. Our daughter, Kate, is with us. She's 13. She was diagnosed at 18 months old. Kate is a little different in that she-, her arthritis has been pretty well controlled. She's been on three medications. She's currently on an infusion. But even with a well-controlled, very active child who plays lacrosse and travel softball, we still have made a lot of changes in our life.

One of the biggest is we had to move her from public school to private school. She missed 30 days of school in kindergarten because there was only one nurse between five schools and she was there Thursday afternoon. So we had to find a private school that had a fulltime nurse. She missed six days once we moved her. So it's a huge difference, but it's a huge burden, too.

We've also had to-, my husband and I both have to work, and we enjoy that, but we have to work in very flexible jobs because we have to go to an infusion once a month. And if we had to take vacation every time we had to go to an infusion, guess what our vacation would be? Right? In the hospital infusion lab. So we've had to make some real changes. They've all been changes that we've embraced and we understand. I had a great therapist tell me one time that everybody has to deal with something in their lives, you just got to find yours out really early. And so we try to approach it that way all the time.



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But even then, she was out of PE for all of fourth grade. We were lucky she was in a school that found something else for her to do during that time. But out of PE for a whole year, even well medicated and well taken care of. And when she plays travel softball, the kids do notice she's a little different; she misses some things. When you're 13, it's not really fun to be different. Luckily, we have a very vocal child who speaks up and speaks out and will talk about it, but still, even in, uh, what is probably one of the best cases for this debilitating disease, we're still making all of these changes.

[applause] [57:52]

Karen: All right. Good morning everybody. My name's Karen. My-, my daughter, Laura, is six and she has SJIA. So when I think about some of the basic changes, um, her biologics didn't work with the ones for her IO1, the IO6 did work. Unfortunately it makes your white blood cell count go really low. And if that happens then you have to discontinue use until your body's immune system builds itself back up, which it has not done yet. So how does that change us?


She can only take, um, some of the approved medications that are currently out there. We wake up about 30 to 45 minutes earlier than the rest of the world so she can stretch, so she can lay in the bed and rotate her ankles and her wrists and things like. And then once she gets the energy to get out of the bed, we do have a two-floor home, so I kinda wait to see if-, does she gonna slide down on her butt today, or am I picking her up and walking her down the stairs? Um, she's about 50 pounds, I can handle it. I don't know how much longer I can handle it, but that's where we're at now.

Dad is retired military and he is my blessing. So while I hear a lot of these moms saying, "I'm fulltime. I'm the one that gets to stay home," he's fulltime. He's actually upstairs because we're not ready to leave her to daycare. So he's with her now while I get to come down here and just learn a little bit more with you guys.

He's on-call. You know, 504's are great in the school system, but that doesn't mean that she's not gonna fall that day and it's gonna hurt more than another day, so he is always there for that. And then when we think about all the extra curriculars that we're nervous about putting our kids into – we have made the decision to put them in it, knowing that we're probably just losing a lot of money because she's gonna sit out about half the time. But we do it anyway because we want her to be "the normal kid," we want her to feel like she's engaged. So that's just all food for thought. Thank you.

[applause] [59:42]

Suz: Thanks so much. And, um, let's have . . . you can be our final comment, unless . . . Vincent, do we have any comments online?



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Vincent: Before we get to our comments, we want to thank everybody who's been, uh, writing in comments. And we want to encourage more people to do so from the far side. So if you're attending on the web conference, go to your Slido questions section, and go ahead and participate. And this will also include the-, the next segments where we do this sort of activity.

Here you go. Here's our last comment.

Female: Hi. I'll keep this really short because I know I'm standing between you and lunch. So, um, I just wanted to point out one thing. I agree with all the comments and the changes we've made in our life. I can't even begin to go into that. It'll take hours to say how our life changed. I had a very sports-active kid. We were supposed to go to Spain for him to play soccer. Everything changed. So we'll just leave it at that. But one thing I wanna point out is it's not just my life that changed, and his life that changed, and our family's life that changed. My job's life has had to change. So not only is it impacting me . . .so I-, I think it's important for manufacturers, and FDA, and everyone, to realize this isn't just affecting me personally. This is affecting my company's econ-, economic forecast. We're constantly having to plan whether I'm gonna be there, not be there, because I am a fulltime mom, and I'm also a fulltime employee, and I'm the person carrying the insurance. So this is way bigger than just me and my little circle. My circle is rippling out to everyone else. And that's really all I wanted to add.

Suz: Thank you.

[applause] [01:25]

Are we good in the-, on online?


Vincent: Yes. The-, the online comments have supported what we've been . . .

Suz: Great.

Vincent: . . .what the people in the room have been saying.

Suz: Super. All right. Well I wanna thank all of you – the panelists and everyone in the room and online. Um, we've heard so much about just how profound the impacts of this disease are, all of the changes that you make, um, the unpredictability that you live with.

So we're gonna break for lunch. We're gonna come back and start at 12:25. But feel free to bring your food back in here and that way you'll be here when we're ready to begin.



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And next we're gonna move into treatment and talk about what does that look like right now. Um, and then later this afternoon we're gonna start doing a little forecasting and talk about clinical trials and how do we all work together into the future to treat this disease. So at this point you're welcome to go get lunch and I wanna remind the 12 panelists to please come up front for a quick photo. So thank you.

[Audio Ends] [02:02:33]

Transcript 2

File Name: cameraisorecordafterlunch

Number of Speakers: >5

Length of file: 02:46:55

Audio Category: Verbatim

Unusual Words or Terms: Must be completed

(e.g. abbreviations, Company Names, Names of people or places, technical jargon

DMARD, NSAID, polyarticular JIA, Aphthous, Crohn's, fibromyalgia, POTS, EDS, oligo, Methotrexate, polyarticular juvenile idiopathic arthritis, Actemra, ERA, Remicade, avascular necrosis, Dr. Laura Schanberg, PCORI, phosi articular enthesitis-related, hypogammaglobulinemia, macrophage-activation syndrome, Dr. Guy Eakin, DPARP


[Audio Begins] [00:00:00]

[intro music] [00:00:01]

Suz Schrandt: I wanna give everyone a one minute warning. I know it took a while to get through the line, but I just wanted you all to, um, find your places and we'll start again in just about a minute.

[indiscernible] [06:50], Melanie and Liz. All right. Um, welcome back everyone and welcome on the folks online. Uh, we're still finishing our lunch in the room but we're gonna go ahead and get started and move onto our next topic which is treatment. So just like we did last time, we're gonna start with some polling questions. And I wanna remind, uh, folks in the room and folks online, uh, in your Slido, uh, uh, picture on your phone, you wanna make sure you're in the polling questions tab and not in the questions tab for this. Okay.

And at this point I'll turn it over to-, to you all in the back to put up our first question. And I do wanna say, so that it's-, so that it's not confusing, you can see all of the answer choices on your screen on your phone. The only answer choices that show up here are the ones that get the most results. So if it looks confusing because it's not the same as what's on your screen, that's why. But you can go ahead and vote on how many treatments have you or your child had to try in order to get control of your JIA? And so this could be, you know, more than 10, fewer than 2, um, or maybe you haven't been



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able to get control, which is also an answer choice. We'll give everyone a minute to vote.

Wow, that's a lot of people not able to gain control. That gives us-, gives us our charge, for sure, that we know we have some work to do. Let's go ahead and go to the next question. And this, I know, is going to be a big topic in our discussion together today. What has been your child's most troublesome side effects, or your most troublesome side effects, from treatment. And you've got several options there, including an "other" option if-, if these are not the ones that speak to you. So everyone take minute to-, to cast their votes there.

Great. Okay. So it looks like the-, the clear top three are needle phobia, issues with the injections or infusions themselves, but certainly susceptibility to infections is a big problem. And we hear, frequently, about nausea and vomiting. Certainly other side effects, as well. So thank you from the back. You can put us back on our regular deck. And next we're gonna hear from our treatment panelists. And here again, um, we have these folks here to really share their own stories, um, to help kinda punctuate the data that we're seeing and talking about. Um, they will all introduce themselves, so I'm gonna turn it over to Liz for all four of our panelists, and then we'll have a-, a large group discussion, um, at the conclusion of the panel. So, Liz?


Liz Smith: Good afternoon. Uh, my name is Liz Smith. I live 20 miles down the road in Burke Virginia so I win the prize for the longest commute for this meeting.

[laughter] [00:10:48]

Um, I'm a former preschool teacher, currently a preschool administrator and interim director of a job I didn't want. [chuckle] [00:10:57] I've-, I've turned it down. And I've served on FDA advisory committees, I've served on the advisory council at the NIAMS, and I have just recently joined their clinical trials review committee. So that's, uh. . . my first meetings are coming up in October.

I have three kids with rheumatic disease. So you can see them up there. Um, My son was not diagnosed as a child; he was diagnosed as a young adult, but his picture's there because the side effects were pertinent. Um, most of my focus will be on Emily who is the one. . . the nurse on the left and then she's holding her baby sister in-, in the lower picture.

Emily was diagnosed when she was two years old with polyarticular, then we were told psoriatic, and we've been told since then that she probably has overlapping, and she has a couple of other issues, as well. Um, her diagnostic process was, in the grand scheme of things, pretty quick. We were in this area and so we were fortunate to have a pediatrician who's close friends from high school, college, and medical school, had gone into pediatric rheumatology here in D.C., so we were able to get in very quickly.



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Um, but it started with a fall in the swimming pool, swollen ankle, and, you know, they told us it was this, told us it was that, told it was the other. And then one day her brothers came to us and said, "Mommy, Mommy, Emily's giving us the finger."

[laughter] 00: [12:29]

Well my daughter was two years old. [laugh] [00:12:28] She didn't know what that meant. And I, of course, I looked at my boys and said, "Why do you know what that means?"

[laughter] [00:12:35]

Uh, so-, so that was when we realized that her finger didn't bend, and other things were happening. And we went back to the pediatrician and lo and behold, our two-year-old had arthritis.


Um, we-, we were asked to talk a little bit about the-, the worst times. For us, with Emily, that was when she was seven years old. We had had stability for four years, um, on just a DMARD and an NSAID. Life was really good. She was doing things again, functioning. So her doctor said, "Let's stop her meds." That was before, I think, um, there had been a lot of study into whether or not children should be weaned or should stop abruptly. So we stopped her meds and we had these three absolutely glorious weeks – no drugs, you know, nothing. We could just pick up, we could leave our house, we could do what we wanted to do. And then she woke up on New Year's Eve and couldn't get out of her bed. . . getting ready to turn seven years old. Um, she couldn't participate in activities anymore; she couldn't keep up with her friends. She was seven years old and I was thinking of this as Nick was talking, thinking, "Wow, but at least she was only 7, not 16 or 17 when I was dressing her, and helping her in the bathroom. And her teachers were taking her to the bathroom at school because they had to take her into the staff restroom in order for her to be able to use the toilet at school.

So we went through this roller coaster ride of getting her stable again. She's now, as you can see from the picture, a nurse. She works full time. She loves her job. She's doing beautifully. She's never been in remission but, you know, drugs are good. What can we say? We like drugs in our house.

[laughter] [00:14:33]

Sophie, the one on the right started having symptoms, uh, joint pain, when she was in late middle school, okay, and then ended up with the chronic disease that involves arthritis, so that was bad.

Um, one of the things that-, that I will say about Emily is that we've-, we've been through multiple drugs. She started on a DMARD, then she went to biologic A – severe side effects and then it's lost its efficacy – so we went to biologic B. Three and a half years on



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biologic B, life was wonderful, and then efficacy started to de-, to-, to decline. We went to biologic C and she had seven absolutely amazing years on that. But her skin was a mess. So they took her off that biologic and put her on drug D and her skin cleared up. No-, no signs of psoriasis at all, but her joints went to hell in a hand basket. So it was back to biologic C, which no longer works.

So she's now on the fifth drug and she's been doing very, very well on that. Um, but it-, it has been a problem. You know, with-, they-, they put her on that last one because as she was starting her nursing career, going in there for her HR appointment, I was driving her because she could not actually turn her head to be able to safely drive her vehicle. She wanted to go, I think, into the emergency room as a nurse. She did her practicum in the ER and loved it. But she couldn't do it because that's not really conducive when you have arthritis. She needed something where she could lift her patients, and as a post partum nurse most of her patients weigh less than 10 pounds, so it's very doable. Um, I think, wi-, with, uh, all of this, one of the things I really want to say is that we have to remember that this disease is not one size fits all. And sorry, I'm skipping things, cuz she gave me my three-minute warning. You know, it's a challenge, it's not a one-size-fits-all thing. It's a guessing game. Uh, Emily's life was very heavily impacted by arthritis, physically and emotionally, knowing that we couldn't do the things, we couldn't travel. When her dad was still on active duty we came back up here to D.C. when he was assigned to another duty station because he was gonna deploy and I needed to be somewhere with family and friends, and a good support network, cuz I had six kids and I couldn't, um, manage by myself when-, when things were bad. So we have to think about that.

Um, I have Dave's picture there because one of the side effects, when we think about it, is something that. . . he was undertreated for years. And I know as parents we worry about this with our kids. He refused all treatment for his RA until he and his wife had that precious baby that he's holding in the picture. I think we all worry about what happens with our kids when they need meds, but will they be able to have children with these medicines?

Um, the last thing – like I said, I'm skipping a lot – that I want to get across is that Emily is on her fifth drug. She averages three to four years per drug. What happens when we run out? She's 26 years old. She's been on meds for 24 years. What happens when we run out of medicines? We need more choices. We need, uh, more drugs approved for kids.

And, having served as a patient rep, I appreciate everything the FDA is doing. I appreciate all of the listening you do to patients now and the involvement that FDA encourages from patients, but I would love to see more patients, caregivers, on FDA advisory committees so that when you have six doctors talking, you might have at least two patients or caregivers instead of just one, so it's maybe a more rounded approach.



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Um, and that-, that's not a. . . I hope that doesn't come across as a negative. It's a very, um, heartfelt thought for me.

Um, but thank you for letting us be here. Thank you for listening to us, and I hope I didn't skip too much in my effort to keep it below my seven minutes. [laugh] [00:18:47]

[applause] [00:18:48]


Jacqueline Peña: Hi. My name is Jacqueline Peña. My family and I live in Mason, Ohio. It's very close to Cincinnati. My son Jacob is 13 and he has polyarticular JIA. He was diagnosed at age eight, but I truly believe that he has had it his whole life. As I'm listening to everybody's story this morning, um, I could relate in so many ways. From the time he was born we were in and out of hospitals, in and out of doctors, seeing a number of specialists, and we couldn't figure out what was going on. I knew something was wrong when he would wake up and not be able to move from the waist down. Um, he started missing school because he was in so much pain. He was always sad and-, and we just didn't know why.

He was seeing an immunologist at the time for, um, a different syndrome. They were very high fevers and it was called PFAPA (Periodic Fever, Aphthous Stomatitis, Pharyngitis, Adenitis). And I believe that it was actually really linked to his PJIA. When we got his diagnosis it was a relief in some way because we had an answer. It wasn't the answer that we wanted, but it was an answer. And I felt like maybe now we could do something for him. I remember feeling this overwhelming guilt of "How did I miss it? How did I not know?" But then I remember a conversation that I had with family and friends, and they would tell me when I would mention that I thought my son had RA, that he was too young, that I was probably being a hypochondriac, that I was crazy to think it.

I fought, for many years, to get this diagnosis, and since then we have a multiple list of medical conditions that he currently has. Not only do-, not only does he battle PJIA, but he has Crohn's, he has fibromyalgia, POTS, EDS, a heart murmur, heart arrhythmia, and he has a feeding tube. His Crohn's became so bad that his arthritis was flaring up all the time. And no matter what medication he was taking, it wasn't helping.

We finally decided that we had no other choice but for him to get the feeding tube, and for about a year that was his primary source of nutrition. That was in 2017. We just marked our year anniversary, but I have to tell you, it hasn't gotten any easier. The current beast that we fight every day is PJIA. He's on his fourth biologic. He receives IV infusion therapy treatments every four weeks but he has a needle phobia, so those days are stressful. They're overwhelming, and you would think after three years they would get easier, but they don't.

The worst part about it is that nothing is helping, and we are running out of options. We really need new treatments and new medications. Just at the age that he is, he's not



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old enough to try the adult drugs, so he can only do a certain number of biologics. It's frustrating and overwhelming to know that I can't do more for my son.

I want to talk about the challenges that our family has faced. We have moved three times to three different states. He was born in Florida and we chose to move to Arizona for the dry heat, thinking it would help his arthritis symptoms. And while it did for some time, he still flared up. We realized that that was probably because he also had severe Crohn's at the time. Then, we decided to move to Ohio to-, so that he could become a patient at Cincinnati Children's Hospital to get, hopefully, better treatment, a new team of doctors, and, unfortunately, we received the bad news recently that there's pretty much nothing more they can do.

So it's frustrating, but I have hope. Every day I pray and I hope that something new's gonna come out on the market. That somehow, some way, something is gonna work for him. That's what keeps me going. I'm grateful for the new therapies that are out. I'm grateful that we've come so far with these biologics and all the treatments and medications that are currently being offered, but it's just not enough. He no longer goes to school. He has to be home schooled.


Our youngest is also paying the price of this disease. He does not participate in any extra-curricular activities. He doesn't go to school anymore either because the risk of him bringing home a virus, a bacteria, or some kind of illness is way too overwhelming. I kind of feel like we live in this bubble at home. And the fear of going out and being exposed to something because he's so immune suppressed is terrifying. My son is a warrior, and he battles this each and every minute of each and every day. He has more strength than I could ever imagine.

My husband has had to work at night so that I could work during the day. And then in 2015 I left my career and decided that I had to primarily focus on Jacob and getting him better.

I, um, I hope this isn't it. I hope that there's more to this. I hope that there's gonna be a silver lining somewhere. This roller coaster that we're on, that's full of twists, and turns, and stops, and-, it-, it's gotta end. And, um, I-, I can't thank you enough for allowing me to speak, for hearing the stories that we all have to share. It's so important that we make our voices heard because our children need this. They need more. And I am not gonna give up.

I consider myself a strong woman, but this disease makes me feel weak. I have to continue fighting for Jacob, I have to fight for my family, and I am not gonna let this beast win. Thank you.

[applause] [00:26:25]



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Kate Kuhns: Good afternoon. My name is Kate Kuhns, and my daughter and I live concurrently live in Roanoke, Virginia. My daughter, Delaney, is currently 15 and she'll be a high school sophomore later this month.


Delaney was diagnosed at the age of six, uh, when we were living in-, outside of Des Moines, Iowa. It took approximat-, approximately three months for her diagnosis. That was quick. Um, at that time, um, in Iowa, we had two pediatric rheumatologists. She presented with, um, ol-, oligo arthritis, so it was just in her hips, so we were pretty fortunate at that time.

It was September 2009, she woke up, couldn't get-, couldn't move, get out of bed. By December we had a diagnosis, treatment plan, and we were rockin' pretty well. Come August, um, that changed to extended oligo and we started adding more medicines to our plan. And we were on Methotrexate from December of 2009 'til now. We have been on, um, three different biologics, and one was phenomenal. I call it-, I-, I loved it, Delaney loved it. Um, and then recently, um, we would still be on it – that form of it – um, we were-, we've done infusions, we've done, uh, shots. Delaney did not like shots. She likes shots now.

Because of oligo, it is not the, um, indication for biologics. I'm not sure how many oligo parents know that. I didn't know that until real recently when we were trying to get her biologic that she's on switched from the infusion to the auto injector because Delaney's veins have been on injections eight, or, I'm sorry. . . infusions. . . her last infusion, um, round was five years, every month. Labs every four weeks, or maybe if she was lucky we could go, um, every other month. So she was blowin' veins every time, to the point she has a rule, you can do one arm one time, one arm the next, after that transport – Life Flight has to come down and put in. We don't do hands, we don't go anywhere else. So we were blowin' veins too much.

So it was time to find a different method for the infusion that was working. It was gonna be easy, we thought. I mean, we-, I've had the same insurance. . . I switched jobs, we relocated from Iowa to Virginia for her, um. . . JA – warmer climate. I'm a single parent. Um, I was military. When I had Delaney had, um, wonderful insurance. Once you, um, are in the military, you might have complaints about TRICARE, but I will never care about TRICARE. I love it. . . loved it. Wish I still had it. Um, it's great. Commercial insurance – love or hate it – but you always be careful what you wish you didn't have until you don't have it anymore.

But with her biologic process of trying to keep her on the one she had, or currently has, but just to change the form of the way she went from the infusions to get the auto injectors – denied automatically from the insurance company. She doesn't have poly JIA. Okay, well, she's been on it for the last five years. "Well, it's not FDA approved for oligo." Okay, well, it's-, she's been on it for the last five years, you've continued to approve her for the last five years F-, FDA says it's not approved for. . .



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So that's my ask – the indications and the usage needs to be broader. It's the same medicine used to treat the different subtypes. Extended oligo for so many kids are being used for. . . I mean, she's been, and so many of our kids have been used the same biologics, mine especially – seven, or eight out of the last nine years – and then all of a sudden to have this shock. I was told I could pay the cash price. More than my salary.

So, but, fortunate, using the biologic company, the third appeal through their assistance program, we were able to get the click-jet program, but it's was amazing to realize that just because of a-, a simple sentence. . . and going back and looking at the other two biologics she had already been on, has the same usage and indication. So that's my ask, is just to, when those are getting approved, making sure that the kids with oligo also is being looked at. And I know that the clinical trials and everything – just broaden the clinical trials – because that's so important, cuz there's only so many medicines that can work for our kids.


So, I thank everyone for coming and listening to our stories. Um, Delaney's had to give up a lot. She's done an online school in middle school cuz of stuff was not just working. Um, she had some side effects from some other med-, medicines that she was on through her treatment plans, and so she gave some Irish dance. She was an Irish dancer for years. So, um, but yeah, that's us. We moved, relocated, and but now we are. . . She loves the auto injector. It's 15 seconds. It tickles. She laughs. . . the first-, the first time. It was like, "You're laughing? You're not screaming?" It's 15 seconds of fun she calls it. So, the best. . . I'm like, "Okay. It took three months to get it approved, but okay." So that's all I will say to that.

So I appreciate everyone's time and effort, but if the indications and usage could be widened so more kids can have the ability to be covered that would be much appreciative. Thank you.

[applause] [00:33:17]

Melanie: All right. Good afternoon everybody. Um, I'm Melanie. Again, I spoke, um, for a few minutes earlier. Um, I live near Columbus, Ohio, but I'm a Connecticut native, so, um, I'm not a Midwesterner, I'm more of a New Englander.

Um, my current job – I actually am lucky enough to be engaged on numerous, um, pediatric rheumatology projects. Um, I work with PR-COIN, Partners, CARRA, and have a few PCORI projects, as well. So, um, I like to tell people that I jumped in head first [laugh] [34:01], uh, not first first, not a toe. I jumped all the way in. And so this has become my life and I, uh, couldn't be more happy. It's not exactly the path I had created for myself, but, um, it is, been a phenomenal silver lining to my daughter's diagnosis.




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Um, I touched on this a little bit earlier, but my daughter was diagnosed at 20 months old with polyarticular juvenile idiopathic arthritis. She was not walking at 20 months old. We had, um, you know, had her normal pediatric checkups at 12 months, 15 months. And every time the doctor and I kind of discussed the fact that she wasn't walking yet. But she had an older sister and two parents who liked to cart her around, so, um, you know, it kind of helped to explain that a little bit.

At 18 months we finally succumbed to the PT thing. Um, she did not do well in PT. We couldn't figure out what was wrong. Um, I believe that she had active disease starting around 11 months when she had a febrile seizure that launched her immune system into attack mode. Uh, when she was diagnosed she had over seven-, uh, over ten joints affected. Um, we would notice that when we wiped her hands after dinner she would, you know, cry, or pull away. Um, we noticed that she stopped crawling, she would just walk upright on her knees. Um, she had zero range of motion in one wrist and only 30% in the other, so that explains the crawling thing.

Our diagnosis was fairly quick. Luckily, our pediatrician's MA has a family history of rheumatoid arthritis and brought it to our attention. We had a referral that afternoon and a diagnosis within a few weeks. I still didn't buy it, though. Um, you know, I wondered if there was something else going on. You know, arthritis, you know, kids don't get that. You know, you really-, I was only about 90% on whether or not I thought this was what was going on until, um, one morning about five days after our diagnosis, we had another joint swell up to the size of a softball. And that was my breaking point. That is when I absolutely locked myself in my closet and had a complete breakdown. I, uh, officially bought it at that point. Um, we got her under control fairly quickly. And what I would like to talk about today is kind of, um, despite the control we have over her disease, what we still kind of deal with as far as the treatment goes – we started with, you know, NSAIDs, DMARDs, um, joint injections, all at the same time. I am the type of parent who wants to eradicate this immediately. And so I was not the conservative parent terrified of the medication. I said, "Please give me everything. Everything you have, and I want it all right now," and I had a doctor who was willing to help me with that.

The only problem was Meghan was only 20 months old and so none of this was approved. We paid out of pocket for our DMARD, and then we failed that DMARD, or as I like to say, the DMARD failed her. Within 11 weeks we had 3 new joints affected on the DMARD. So we finally were approved for biologic number one. Biologic number one worked like a magic wand. It was absolutely amazing. Um, she got sick all the time. We couldn't really take her very many places, but she wasn't in school yet so we could deal with that. But she could run, and she could play, and she could jump, um, and she was sleeping through the night. Um, you know, that is, um, that was when I knew that things were really working.



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Unfortunately, that paradise only lasted for about two months, and she flared again. At that point we were ready to try biologic number two. We asked for it and we had a huge struggle with insurance. Um, they allowed-, they-, they declined the biologic we had asked for, suggested another biologic, and then they took four months to approve their own suggestion.

During that time, my daughter was maintained on, um, a DMARD and, um, an NSAID, until her labs came back a month later horrible, and so we had to decrease those. So my poly JIA kid with, at this point, about 14 joints affected, was functioning on Tylenol. That did not go well.


We started biologic C, which was our magic wand, in August of 2013, and it put all of her large joints into remission. Um, I carried her into the hospital the day of her first infusion and she was running by the weekend. It was absolutely amazing. Um, this treatment doesn't come with, um, it doesn't-, doesn't come without a burden. It is an infusion. We do have to take a half day off from school. She does have to have an IV placed. Um, my daughter and I are both numb resistant, so for her a numbing cream that would normally take about 20 minutes, takes over an hour to actually work. Uh, we know that now and that's great, but it was a struggle that we had.

Um, we have been able to drop, um, some of our medications and maintain her fairly well. Although, we recently, um, in March of this year, found some active arthritis in her TMJ. We added a DMARD and it did this to her. So we have these oral ulcers that will not go away. Uh, we stopped the DMARD, they resolved. We start the DMARD up again, even at a half dose and they start up again. So we deal with, um, more side effects of medication than what we do burden of disease.

The other issue with these medications that are keeping her arthritis quiet is that we have immune suppression issues, we have challenges that you don't normally think about. So if my daughter were to get strep throat, do we skip the biologic? Do we push the biologic, do we skip the DMARD? Do we push the DMARD out a day? Um, those are things that, you know, you don't necessarily think about to ensure arthritis is quiet. The risk of her to flare. . . She doesn't remember what a flare looked like. You know, she was so young at the time, um, that she doesn't remember what a flare was like, and I don't ever want her to experience that again if we can avoid it. So we have to weigh the pros and cons of that.

We found that we stick with the biologic and we make that happen, but we do kind push the DMARD a little bit, um, and that-, we found that that works for us. But it's just something that you don't necessarily think about.

Um, something that I would like you to take home with you today – an ideal medication for us would be something that doesn't hurt and doesn't require a band aid. Um, something that isn't an infusion or an injection, a medication that doesn't cause painful



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oral ulcers, or daily tummy aches, a medication that doesn't scare families fearing the future of health insurance and healthcare due to the high price tag.

What I wanna make sure I get across today is that my daughter has been extremely lucky in her journey. I know it sounds like we've been through a lot, but our primary medication is working. She tolerates it fairly well, we can afford it. Unfortunately, sometimes active disease is hiding and doing permanent damage to joints like the jaw. The treatment that was working now needs to be modified and we have to deal with the side effects of those modifications. We go back up the hill on the roller coaster again, ready to free-fall.

Many families I work with in my JIA activities are out of treatment options at the age of 8, and 10, sometimes even as young as 6 – out of treatment options – often due to the age. Sometimes the FDA has only approved medications for patients over a certain age, or only in adults and not in kids.


I can only imagine the conversation that happens between that parent and that child. I think it might be something like this, "I'm so sorry, honey, but there are no other treatments for us to try. We'll try to manage your pain as best as we can until something else is available. We'll probably have to do surgery in the future to fix the permanent damage that may be done, but we will wait, and we will try what comes next." I'm hopeful, based on our gathering today, that the time and energy put into this day and the impactful listening that's happening in this room, and sharing our stories, can be very helpful and help kids in the future. Thank you.

[applause] [00:41:45]

Suz: All right. Well thank you so much to our panel. I think we heard so many important take aways about treatment. Um, that it's certainly as simple as-, as finding a good treatment and accessing that treatment. There's all sorts of-, of challenges and barriers. Um, sometimes that's because there's multiple diseases happening and you're playing Wack-A-Mole to try to figure out what you're gonna try to-, to work on today. Uh, there's these administrative issues, not just with insurance, but is it approved for you, is it-, does it have an indication that you need? Um, there's a guessing game.

And then I think one of the-, the big thing we heard about was burden – burden of the treatment itself, how it's administered, what the side effects look like. And then certainly, um, that folks are either out of options or running out of options, and that's pretty scary, um, especially for really young kids. You have a whole life ahead of you and wanna figure out what's next.

Um, so I'm interested now to hear now from those of you in the room, and the folks joining us online. We've got two questions with ou-, we've got about 30 minutes, and we've got two questions to talk through. This first one is gonna be really about what-,



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what does your current treatment regimen look like? Does it work for you? How well does it work? And then we're gonna get into downsides of treatment – side effects, etc. I do wanna remind the folks online, um, you were in the poll tab before, for the polling questions, now you need to be in the question tab. But that's a misnomer, you can use it even if you don't have a question. . . it can be for a comment. So don't be fooled, you can just make comments.

And we expanded the character limit, so I think it's now a 300 character limit and if you need more than that, just send us multi-part comments, and our folks, um, who are monitoring the intake, can-, can piece your parts together. Um, so thanks for working with us online.

So we've got our mic stands. I think we might have someone ready to make a comment.


Carol: Hi, my name is Carol. My son, Ian, is 18. He was diagnosed at age 15, after 3 months of not having any telltale symptoms of his SJIA. He had no rash, he had no swollen joints. In the four years he's had this, he's only had one swollen joint in this whole time.

Um, after he was diagnosed, he started on the Actemra, and that worked really well with the infusions. After about a year and half, we tried to wean him off to see if he was in remission. And within a week past his regularly scheduled date, the flare-up happened, and it was worse than it was during that whole entire three months of no treatment.

He got back on the infusions, did really well. This past January, he got switched to subcutaneous injections. He goes to college next-, this month now – three weeks away – and he's done really well with the sub-q injections. It's twice a month instead of the once a month. Um, he did have a flare-up at the beginning of June. He messed up on one of the injections, and he might have lost a couple of the little drops of medicine, and it was a day late, and there was a couple of other factors in there. He had to go to the hospital because he had a fever, which is part of the systemic JIA. They wanted to do blood cultures.

We made the mistake of not going to Children's Hospital. And the hospital that we went to didn't put all the puzzle pieces of the SJIA together with the fever. They wanted to do blood cultures and things like that, and that was just a big nightmare.

Um, one of the things with the current treatment that we don't know if it is just happening because it was ready to happen is that he has always been a runner – he's been a cross-country runner. After he got on his treatment, he's participated in cross-country for the last three years. He ran in the fall, but starting in January, his-, whenever he did cardio, whenever he would go out to run, he would just feel a tremendous pressure on his lungs, and he would not be running. So he wasn't running so much. We went to see the rheumatologist, told her about it, and he-, she said, you know, go to a



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pulmonologist to check to see if it was related to either asthma or an SJIA-related lung disease. Which, I really had no idea about those until she mentioned that. And that was another whole other scary part of the picture and everything.

So the pulmonologist has cleared him. He's had CAT scans, he's had x-ray, he's clear on that, but then the pulmonologist says, "Let's see what the cardiologist says, because something's happening here." The cardiologist is clearin' him. And now they're like, "Well, we're gonna breath-, the breathing to the pulmonologist." But it's just not knowing. You know, we may not have an answer. We don't know if it is just a coincidence that it coincided with the subcutaneous injections or if it's just something. . . a different progression of his disease. It's very comforting knowing that his heart doesn't have any abnormalities. Um, so that's just one of the things that we don't know that is going on with his treatment.


And him leaving for college should be. . . is a very happy time, but it's also just not knowing that whenever he gets a fever, is this the new normal? Is he going to have to go inpatient to have all these blood tests done, which our doctors had said, no, just try to get him down to us so we can check him out, and everything. But I totally understand one of the panelists saying, "If you get the fever, do we do the biologic?" You know, "He's not supposed to do it because it's gonna suppress his immune system." Just all those things that go into the planning.

And also, he just had a meningitis B vaccination, which his pediatrician has never done for anybody before, but because it's new and it's recommended for immuno suppressed patients, and everything seems fine with that. So, thank you for listening. Appreciate it.

[Applause] [00:48:23]

Suz: Thank you. It looks like we have someone coming to the mic? Uh, no ru-, no rush. Woman: Again, I'm a systemic parent. Um, and our son is 13, and everything that she just said, we are experiencing that same fear of what is happening. And we're seeing pulmonology frequently. He is still unable to be very active, which is. . . I mean, seriously, let's talk about our own mental health where we are trying to accept that this isn't going away is really, really hard, um, because we just have still never made it back to that base level of normal activity, unless it was, like, maybe the first few weeks after biologic number three, which has been about a year and a half for us that's he's been on that.

And that's the best we have been able to do. But we still have the wheelchair, the walker, a boot, a wrist brace, uh, that we need frequently. And we have an amazing school district that will allow him to lay down at school and hydrate. And they're really great at communicating with me. But, it's just the treatment regimen. . . I mean, we always wish for better. And our child is 13 and he's 200 pounds. We call him "Big E," or



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the "Big E that Could." And so we are very curious as to what if we were able to double this therapy? But that's not approved. And I don't know how many 200-pound pediatric patients there are out there. And then-, he's not-, he's a big boy. So that's always-, we don't know what the outcome would be, but it's not approved, so far it hasn't been an option for us. But that's just. . . this is the best we've done. What if we added more? And that-, so we've had to add-, be creative, as our rheumatologist calls it. But we also really, really fear what is happening in the lungs. Is something happening in the heart? So that's how it's going for us, as well.

Suz: Thank you.

[applause] [00:50:39]

Woman: Um, I'm an ERA patient. And, uh, there isn't really good treatment for ERA. And then you add jaw on top of that, and there's no good treatment for that either. I've had one surgery. I'm basically doomed to have my joints replaced in, you know, 10, 20 years. Um, so, you know, with-, and with the rest of my arthritis, there's just not a good way to control it. I've run through the gambit. I'm on my last biologic. My only hope after that is some kind of DMARD, and-, or maybe a psoriatic drug. So, yeah, I'm not-, my current treatment regimen does not help much.

Suz: Thank you for sharing.


[applause] [00:51:42]

And I think that's definitely part of our-, part of our mission here today is we-, we don't want to be out of options, so thank you for sharing that.

Maria: My name is Maria. I am from New Jersey, and my parents started taking me to the doctors when I was around four years old, when they noticed something was wrong. Fast forward, four years later, doctors, imaging, you name it, I had it done. And finally, by the age of eight, I was officially diagnosed with RS-negative polyarticular juvenile rheumatoid arthritis. And I am now 35 years old.

Um, I reached a point towards the end of 2017 where I have exhausted all of my options – biologics, DMARDs, you name it, I've been on it. So I sat with my rheumatologist and we discussed what we can do moving forward. And the first option was going back on an infusion that worked for me, but because I was at the highest dose, I would get frequent upper respiratory infections, so my rheumatologist at that time decided to stop it.

Knowing that, with that particular infusion, I had very severe reactions, and I had to heavily pre-medicate, we moved forward, and seven hours into my infusion, um, I started to have a reaction. I had very hard-, trouble breathing, my throat felt like I-, it



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was closing, so they stopped it. And the doctor told my father, who came to pick me up, that I cannot be alone for 24 hours. So the rest of 2017 we took a break. I wasn't on anything. Um, and it was rough. And then, finally, sat with my rheumatologist again and we decided to go back on another infusion where I had four treatments of, and all four times my throat closed again. But because it was, you know, we just had to try it again. And it was a very, very hard decision to make because. . . do I wanna put myself through that again at this point in my life, um, because it's physically taxing, it's emotionally taxing, and it was an extremely hard decision that-, that I had to make. So I decided to go through it in March, and my first treatment was eight hours at the infusion center. Um, within the first two, three hours, again, I had trouble breathing. I was heavily pre-medicated again. Um, we had to stop it, administer more Benadryl, start it again. Finally, I got through it after eight hours, went home. Again, couldn't be left alone for 24 hours. Two weeks later went through it all again. Um, I was only there for maybe seven hours. Um, came home, again, couldn't really be left alone for 24 hours. And so-, but I'm at this point in my disease where yes, is my current treatment helping me? You know, I'm maybe functioning on a good day at 40%. Um, and just to be the age that I am – at 35 – and not to feel so helpless. Like, and not to have nothing left, it's. . . You can't imagine what it's like. And [sobs] [00:55:29] sorry. . .


Suz: I-, I-, I think so many of us feel what you're going through and-, and we really thank you for being brave enough to share, because it really is our call to action. That's not-, that's not acceptable. So I think collectively, we can move forward and have more options so that you're not making those-, those trade-offs. Um, so thank you so much for sharing your story. Thank you.

[applause] [00:56:08]

Jen: I'm standing, so I will go, and echo some of the same sort of feelings. I'm Jen. I have had JIA since I was 11 months old. I have had it for 33 years now – do the math.

So I have run into a different issue where I'm out of options because, um, unrelated to my arthritis, they found a benign tumor many years ago. And because – and I know we're gonna have a clinical trial conversation later – but because people are excluded from those trials, they didn't know how biologics would, then, behave with tumors. Therefore, once they found it, and once it started doing some funny things, they just didn't feel comfortable having me on those medications anymore. So therefore, I'm really left with different sub-types of medications and DMARDs, which of course have their own issues. And I am afraid of failing those.

And there have been times in my life that it looked like I was going in that direction, and then I thought, "Are we just going to go back to Tylenol?" Like, we're hearing that you're maintained for a month without-, without any other options. I think coming up with things, and-, and as we continue to develop new drugs, thinking about how we can



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be, uh, looking at more real-world evidence to make sure that people like myself aren't excluded from those opportunities from newer drugs that are developed.

Suz: Great foreshadowing for the clinical trial panel. Thank you for sharing.

[applause] [00:57:30]

Corey: Hi. My name's Corey. Uh, my wife's talked a couple times about our son. Um, mine comes from a different. . . I'm lookin' more at, like, the cost of treatments. Um, we live in central Minnesota. And my son has high-functioning autism, so any insurance – he's already high-risk. So we're limited on our options of insurance. So we have him on MA. So my wife can't work because she has to be home with him. Um, I can't really get a better job because if I make more money, he's off of MA. His treatments are almost \$1 million a year. And if we get different insurance, when we pay for it, I have to get a job that makes two to three times than what I make now cuz at \$30,000, \$40,000 a year out of pocket that we have to pay in order to have him, uh, covered for insurance. So I don't know anybody in this room that can afford \$1 million a year to pay for treatment. Thanks.

Suz: Thank you so much.

[applause] [00:58:30]


That's definitely a hard-, hard issue. Let me pause for a minute and see if we have anything coming in from the web folks, and then we'll take our-, our two questions in the room, and then we'll go to our next, uh, or, next question.

Vincent or Laura?

Vincent: Sure, Suz. Thank you. We're getting, again, a lot of, um, validation for what's being said in the room, and lots of comments coming in. Um, pointing out a few things that, you know, some folks are very thankful, um, that the-, their treatments have been life changing. Um, then some folks are saying the treatments did a great job on a majority of the things, but there's still, you know, small amounts of inflammation that-, that they're having a hard time controlling, um, all the way to taking multiple, multiple medications and still not seeing any relief from pain.

Um, the other thing that was pointed out is that a-, a parent wanted to point out is, um, consideration for the development stage – um, when-, when to give medications, because missed milestones has, like, that butterfly effect, and it's a long period of time, you know, to a child's life.

Suz: Okay. Thank you so much. So we'll take our last two in-room comments. Um, I don't know. . . who was first? Go ahead.



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Man: So, um, my daughter is on her third biologic. Um, the first one, which was a daily injection, which was effective initially, but, uh, terrible to administer. She was three. She didn't understand why she had to have shots every day. Eventually she just assumed that everyone did that and when she learned that wasn't the case. . . well, it didn't go over well. Um, the second one was a monthly injection and we're hoping that that was going to be the one. Least invasive, least frequent, but we may as well have just given her sugar water. It did nothing.

So we moved on. Um, unfortunately, the course of treatment required that we tried so many times of virtually no benefit from that treatment before we could move on to the bi-weekly infusion that she's on now. And she's now four. Um, her veins are terrible. Her mother and I have given her terrible veins. [chuckle] [00:00:50] So we opted to, and pushed for, a port placement for her. So at this point we've modified our daughter for easier administration of medication. That is working 7 out of the 14 days between dosages, at best. So 50% of her childhood is spent dealing with this medication that's not working well.


And at this point, due to FDA approval on some of the other options, this is our last choice. So we're just kinda treadin' water. It's worked the best out of the bunch, but, uh, we're not satisfied with good enough. I feel that there is sometimes a disconnect with the medical community, of what they view as a success, versus what we, as parents, view as success. And I think if I took a quick show of hands, a lot of you would probably agree with me. Is that true? So that's something that I wanted to draw attention to, as well.

It's very easy in the scope of an appointment, or a phone call, or, you know, viewing from the outside, it's be like, "Oh, well, doing better." But "good enough" is not good enough for those of us who have to deal with this day in and day out. And a lot of the panelists have made that point today, as well.

Suz: Thank you. Thank you for sharing that.

[applause] [01:02:00]

And I think that issue around how we define success is also gonna to play into the discussion we have on clinical trials. We need to be at the table helping define what success looks like for treatment and for clinical trials. And then our last. . . Oh. Let's-, can I do the end person comment and then we'll come to the back? So please go ahead. Woman: Okay. Um, so we-, we have been very successful with treatment. We've-, my daughter as been on a DMARD and a biologic, um, but with uveitis, and with any, I guess, JIA illness, at some point they want to take off medic-, medication after being in remission for a certain amount of time. And as a parent, you know, I'm the conservative one, and my husband's like, "Yes, let's take her off medicine one." And it's very, very



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scary. Um, we've gone off Remicade biologic twice now. Um, the first time she was on it for a year.

The second time she was on it for two years, and that's after I begged our rheumatologist saying, "Hey, I went to JA conference and the uveitis specialist said this. We don't have uveitis specialist in our area. We live in Saint Louis. Um, and he allowed it, but basically said, "There's not enough evidence that it works one year versus two years versus life-long." And-, and again, it goes back to that research. But it's very frustrating as a parent that there's no good treatment regimen for our diseases.

Um, because my daughter has poly JIA and has uveitis, both are treated as the same illness, but different illnesses, if that makes any sense. So her JIA is very stable and has been, although she always does better on Remicade, physically, than when she's off of it. But she's in remission. Um, but when she goes off of it I am scared to death every single time. And my doctor was very honest with me, and he said, "You know that black box warning, we were told we can't keep kids on it any longer. For-, once they hit that medicated remission," you know, [snaps fingers] [04:13] "gotta take it off." And it's very, very scary as a parent. I just feel like there needs to be more research in how long these treatments can, you know, our kids can be the medicine. If it is effective, why do we need to stop the medication? Is it insurance driven, um, money driven, you know? Those are a lot of the concerns I have. The treatment is great, but why do we have to stop it? Does that make any sense?

Suz: Absolutely. And I think it's a-, a perfect research, uh, question to be asking. So thank you for sharing that.


Um, I wanna, I know-, I was getting a wave from the back that there was another comment. Okay.

Vincent: Sure. Suz, thank you. Um, uh, very similar to our last in-person comment, uh, it-, it's just that concern for the reaction, um, to the medication, and-, and trying to figure out, um, do I take that chance? Do I gamble with the condition or the medication?

Suz: Right. Yeah. Okay. Thank you so much. And I think, yeah, that definitely resonates with what other folks have said.

So we've got just under 10 minutes for our second question, and I know we're gonna have a lot of-, of thoughts on this one. We've already heard, um, about balancing, you know. When is it-, when are the side effects worse than the disease, and vice versa? Um, so it looks like we can start right here with the most significant downsides of the treatments you've tried.

Anjie: Hi. I'm Anjie again, from the first panel. Um, I actually have two things I'd like to mention. The first one, um, is allergic reactions. Um, one of our daughters, Laura, has a condition called mass cell activation syndrome, which basically means her body is ready to react at anything, anytime, whether or not it was okay yesterday, or last hour.



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Um, she had a pretty significant reaction to one of the biologics – uh, hives. Um, we were very, very scared about that one. Um, ended up having to go off of that, so it's definitely a concern that our family has, and I think probably, we're not alone with that. Also, just like one of the dads over here mentioned, um, our girls have terrible veins – really bad. Unfortunately I have bad veins, my mom has bad veins. They had it coming. My husband has garden hoses instead of veins so it would have been nice if they had gotten it from him.

But that didn't happen. So, um, you know, a lot of these treatments are really difficult for us – infusions. Because, um, it-, it's hard to find a vein. You know, you go in and you have stick, after stick, after stick. And what kid wants to keep doing that? What adult wants to keep doing that?


Um, Laura has a port. When she was 17 we convinced her pediatric rheumatologist that we needed that and it's been a godsend, has not, though, without its problems, um, but it's working for her. Erin, we are currently in the process of switching off of an infusion because the nurses just are not finding veins. Our current rheumatologist is not willing to go down that road with us for a port, so we are now looking at a complete med switch because she cannot tolerate the infusions anymore. Um, so that, you know, that-, that's been one of the hugest issues that we have faced with treatment.

Suz: Thank you.

Erin: So something that, uh, people have already touched on today, but I'd like to emphasize even more is the jaw. We do not have research on how these medications affect the jaw. And it scary going onto a med, and it's like, "I don't know if this is actually gonna help where it counts." Um, like Mom said, I am now switching to an injectable drug. And I emailed my doctor about it, because it was like, "Well, this thing I'm on right now is kinda starting to not help as much, so let's try something else." And I said, "How 'bout this, how 'bout that?" And she goes, "Great! There is no TMJ research on either of them." And I went, "Oh crap." Well, I, you know, so I'm going blind into this.

And, um, as Mom also shared, I just had jaw surgery about a month ago, just over a month ago, and at one of my pre-op appointments, uh, my surgeon looked at me and said, "Your joints are messed up." Another "Oh crap" moment. Um, so with-, I have to have braces for my surgery and, you know, the way we monitor the jaws through MRIs, I can't have those right now because of my braces. So, you know, we can't really be monitoring it. So to be going on a new drug that we have no idea if it's gonna help the jaw, and not even knowing the status of my jaw right now, other than the fact that it's "messed up" is absolutely terrifying.

Um, something else I just wanted to touch on. Um, it's really been hard with treatments. You know, arthritis is an invisible disease a lot of the time. You know, you can't tell that person has arthritis all the time just by looking at them. Um, and injections – doing those, has made me feel even more invisible. No one sees my treatment. No one sees how scared I am to poke myself in the stomach, even though it's an insanely short needle.



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And, uh, it-, it makes me feel vulnerable and invulnerable at the same time. And it's absolutely terrifying. Thank you.

[applause] [01:09:47]


Suz: Thank you so much. We'll go to-, to this side now.

Daisy: Hi, uh, Daisy again. Um, as we went through our courses of treatment, um, one of the first things that they did was the injection straight into my daughter's knee. Within five weeks that knee was probably four times the size of what it's supposed to look like. Um, and we were on our way back, the two drive, to, um, her rheumatologist, where we started Methotrexate. Within, um. . . and confirmed her other knee was now involved – we were at two joints. Within a month and a half, they said in three months, month and a half we were back with, um, a total of four joints, and, um, determining whether Methotrexate had failed her.

Um, through the course of the evaluation, they did determine, through conversations with her. . . again, we're talkin' about a three-year-old who really cannot communicate everywhere that it's hurting. We had to go to OT to teach her how to communicate that this is hurting. Um, I truly feel. . . I think somebody else said that they should have been diagnosed sooner. Um, we just recently were just watching old videos of her when she was little – learning how to crawl and walk. And I can see it. It's there in the videos. Um, so we went on our first biologic. Did amazing for her joints. The swelling all went away. Um, she was running, she was jumping, she was doing great, and then the eye got involved. And because the eye got involved and that biologic doesn't treat the eye, we had to, then, switch to her second biologic. But we couldn't switch to her second biologic cuz she didn't have enough joints that were involved. So we had to stay the course and try and deal with the eye because insurance would not approve the second biologic just for treatment of the eye. She had to have the right number of joints involved in order to go on that biologic.

I don't know if it's a blessing or a curse. . . she did get enough joints involved that we were able to switch to that bi-, biologic. Um, that biologic was hell when we first started it. We would be on it for for two weeks, she would get ridiculously sick, we'd have to come off of all of her meds to let her immune system come out to fight whatever it was – we'd go back on it. We did this about two months. And every time we came out, the eye got flared again. Um, and the doctor-, her rheumatologist said, "This is our last chance. We've gotta-, she has not been on this long enough to even tell if it's helping her because she keeps getting' sick."

Um, at that point, infusions felt like a threat [chuckle] [01:12:37] wi-, that we were trying to avoid going to infusions. Um, we managed to get to spring in Iowa where it's extremely cold. Um, and she finally settled in with that biologic and we did start to see, "Okay, everything's kinda calming down. The eye is under control again." Um, made it through



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the summer, and then October hit. And, as a little girl going into preschool, um, from October to February – not this past winter, but it would have been 2016 going into 2017 – um, she missed at least a month and a half of school because she would get the respiratory issues. And we'd go off all meds.

We had to see pulmonology, and every single doctor, to get everybody to sign off after two weeks of bein' off meds. And then we'd start the meds back up again gradually, starting with Methotrexate first. Within two days of getting' that medication, she was worse than what she was before we took her off it right before that. And during this whole time the eye is flaring again to the point where she had adhesions, and we had to keep her eye dilated in order to break the adhesions loose, in order to get that eye to heal.

And when you reach that point and your child's in preschool and you get a phone call from the preschool, when you've talked to the director, and they tell you, "We are calling an ambulance because your eye-, your daughter's eye will not react to our tests. We think she's fallen and hit her head." And you're in a panic saying, "Please do not call the ambulance. We are doing this to the child," you know, "to fix her eye." You know, it's-, there isn't eno-, there's nothing that is-, that I know of other than eye drops, and steroid eye drops, and dilating the eye that is targeted for the uveitis.


And it's scary. We did, finally, um, through the course the everything last year, her tonsils and adenoids were swollen. Um, coming into school year this year, took the tonsils and adenoids out, and we actually made it all through last winter without anything major. Um, but she, too, has had the strep throat to the Scarlet fever, and the rash, you know, different rashes, and for all of last year we had a mystery rash that would literally. . . I had to take pictures of this rash because by the time we would get to the doctor, the rash would be gone. And they did not no-, believe me, and I'm like, "No, seriously. It was here when we came in the waiting room."

So I guess that's kind of our experience with the downsides of treatments, you know, because they could never actually see the rash. They couldn't actually see this, or that, other than pictures. How do you treat that? How do you know if that's a side effect of medication, or just part of the arthritis? And-, and then the uveitis, not having, you know, anything that, you know, we had to get up to that joint count in order to get on somethin' that would help with that, so. . . thank you.

Suz: Uh hum. Thank you. That's. . . yeah.

[applause] [01:15:44]

Lots of complicated issues. So you two are gonna be our final in-the-room-comments. And we're, um, uh, just about ready to start our third topic of the day, so, um, we'll hear from you two first.



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Woman: I'll keep it brief again. Um, I seem to be at the end a lot. Um, downsides – I'm thinking more not just side effects, at least from my perspective. I kinda wanna talk about what Katie mentioned when she did her talk earlier. Um, to me one of the downsides, in addition to the many side effects that there can be, is the inconsistency in the approval process, and even in the instructions on usage. So, I happen to have a child who's got multiple diagnosis. Um, some of the main treatments treat both conditions. However, the drug levels, the prescribing levels, the frequency, is not consistent. So we constantly run into things where we're told, "Well, you're at this level of medication, so you don't need to have it more frequently." Meanwhile, rheumatology's going, "Our dosage is monthly regardless. We don't look at the drug levels." So we have-, we-, we struggle constantly with that battle between different specialists. What's acceptable, what's not acceptable, and how can we get my child treated? And-, and then the bigger picture is when I feel like when it's treating multiple conditions, it's a constant wave. So, "Great! His joints are great right now, but there goes his GI. Oh, great, that's better now, but his joints are bad." So I feel like. . . I don't know if it's because it's trying to do the job of two, or that we're just haven't hit that perfect storm of medications and frequency that's the problem.


So that's one thing I wanted to point out. Um, the second thing I would like to, like, point out, that we struggle with, is what's a side effect, and what's disease progression. So right now – Sorry, Ben, I'm calling you out – my son is getting crazy rashes. We can't figure out why. GI thinks it's psoriasis, dermatology is sayin', "Maybe not." And everybody just keeps scratching their head. Psoriasis could be disease progression based on the diagnosis that he has, or it could be from the biologic that he happens to be on. No one seems to know the answer.

So this is another thing. Like, I-, I wish we had some kind of guide. I feel like sometimes I stand here and go. . . I-, I-, who do I call? Do I call my GI? Do I call my rheumatologist? Do I call the pediatrician? Is it a virus? I don't know what this is. I need some kind of guidance on how to do that and I think the drugs could help with that direction, as well. Like, better guidance. All they do is say, "call." Call who? Thank you.

Suz: Thank you.

[applause] [01:18:34]

Nicole: Hi. I'm Nicole. Um, my daughter's diagnosed poly JIA at 11-month-old, after about 4 months of "what the heck is going on?" Um, just to kind of preface that, though – she had not hit any of her milestones on time. She is adopted. She was drug exposed prenatally, so we thought that was the cause. Um, but by the time she was ten and a half months old, she wasn't crawling, had just started sitting up on her own. We knew it was really something more. Um, and diagnosis happened after a week in hospital,




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where they tested her from everything from a virus to HIV, and other things, cuz they did not know what she could have come with from her biological mother.

Uh, her knee was suck at a 30% angle, so they to straight-, do a straightening cast on my 11-month-old for over a month. And when it came off, she crawled for the first time, and I cried, because I had not seen her do anything prior to that. And it took until she was about 16, 17 months old for her to walk. They tried a DMARD, and that went okay for about 9 months, and then she developed an allergy to it and we had to stop. They tried another DMARD which did nothing, she got worse. And so they took her off that and put her on her first biologic, which we thought was our miracle drug. Her joints got better, she was doing really well, uh, for about a year or so. And we went to the eye doctor for a regular checkup and he's like, "Oh you have nothing to worry about. She's on this drug and she hasn't had anything in two years, so we're good." Then he looked in her eyes and she had uveitis, both eyes. So that started that roller coaster. She was three.

And then at four – just before her fourth birthday, she couldn't really walk very well. She was limping constantly. Took her to the rheumatologist. "Oh, I don't see any active signs of disease," you know, "I-, I don't know what's going on. Just-, we'll keep watching her, keep her on the drugs, she's got no signs." This went on for months. And finally, I said, "You know, can we do some x-rays or something?" So they did x-rays and they said, "Oh we see erosion in her ankle." Um, she was in Irish dance, gymnastics, regular three-year-old – four-year-old, excuse me – and so I said, "You know, can you send us to an orthopedic doctor? I just wanna make sure she's not doing anymore damage." So we did that.

And I was totally prepared for, "It's erosion. She might need a brace, maybe injections in her ankle," I don't know. And I was-, I felt like I was punched I the stomach when the orthopedist said, "This is not JIA, this is avascular necrosis – bone death." One of the, um, bones in her ankle had died and collapsed. She was, at this point, hardly able to walk at all. And he told me that the only thing that could have caused this was steroid use. So we have, suddenly, this side effect of the steroids that she had been given during her biologic infusion that she was getting every month – that we thought were working great. And MRI showed that they weren't. She was so inflamed – her bone marrow was inflamed in this leg. And we were, all of a sudden, not able to continue with this biologic and we had to switch to something else. And she can no longer have steroids of any kind for the rest of her life because it might cause it again. She was wheelchair-bound for the last year. Not completely non weight bearing for her kindergarten year. And the infusion we are on now is not rated to treat uveitis. But we're using it as if it is. Uh, it's keeping her joints in pretty good condition. Uh, but we have not gone more than seven weeks without a flare of her uveitus in three years. She just turned six last week.



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She is, now, out of the wheelchair, which we weren't told probably wouldn't happen. Um, when I-, she was first diagnosed we were told that her ankle would have to be fused when she stopped growing between the ages of 17 and 21. And I searched for an orthopedist who knew what to do with this, and it was really hard to find one, because it's incredibly rare. She's the one kid a year that this happens to that isn't a cancer patient.

So steroids, for us, are completely out, and we're running out of options for her drugs for her JIA, and we are out of options for her uveitis. So I don't really know where we go from here. And it's terrifying for me every day, um, because I also have three other children and I'm single.

So side effects for us are it really messes with family life, as well as, "Is she gonna be able to walk by the time she's an adult even with all of these treatments that we have?" So that's where we are.


Suz: [sigh] [01:23:06] Thank you so much for sharing.

[applause] [01:23:08]

And I'm sorry. I feel like we could go for several hours just on this topic, so I hate to-, to-, to move forward, but I think so much of what we heard leads us right into our next topic, which is how do we get more information about the drugs that we do have? How do we get more treatments and more therapies? So we're gonna do a-, a crew change. The panel is going to, um, leave the stage, and our third panel is going to come up and take their seats. And I'd also like to, uh, introduce Dr. Laura Schanberg. This panel is gonna be just a little bit different in that we are talking about clinical trials. And so it's gonna be slightly more technical than some of the other conversations, and so Dr. Schanberg is gonna walk you through, just very briefly, um, some key terms, uh, in clinical trials, so that everyone'll kind of understand what we're talking about. Um, and you also have glossaries at your-, at your seat. And so you can certainly refer to your glossaries.

Folks who are on the webinar, you had a glossary emailed to you, so please reference that. Thank you Dr. Schanberg.

Dr. Laura Schanberg: Hello. Um, I'm gonna try to be really quick because we are, um, lagging a little bit behind. Um, so far we've had a pretty amazing day, um, listening and learning about what it's like, uh, to live with and deal with the treatments of JIA. And I can say, um, as a provider, um, is this is pretty enlightening. Um, we, as we take care of ya'll, see you at ap-, in episodes, right? We s. . . But we don't really go through so much all of the day-to-day stuff, and so this has been eye-opening, uh, to me, um, as well as, I'm sure, other, um, uh, providers in-, in the room.




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But we're gonna shift gears now, um, as Suz, um, said, to talk about another part of clinical trial development, and that is the clinical trials themselves. Um, we've been doing trials in pediatric rheumatology for well over 20 years now. Um, and, you know, when I started as a pediatric rheumatologist, we really had very little in the way of drugs. We had only NSAIDs. Um, and as I was a Fellow, and in my training, Methotrexate came in. And so from that perspective, things have exploded and it's super. Right now we actually have drugs that work for many patients. They are not perfect. Um, and many patients. . . there are still patients that don't respond. But we have made a great deal of progress. Um, and now we're sort of into a second phase of doing trials in pediatric rheumatology. And that is doing trials in an environment where we do have drugs, um, because the first phase was, in fact, doing studies, when we didn't have other things to offer patients. And that leads-, um, puts a whole new complexity and extra issues on about exactly how to do these studies.

So, um, I think this is a good time for us, that be having something like this, uh, uh, group today together, because it's a time to reassess how we do clinical trials. And it's a time to think about, um, getting patients more engaged in the clinical trials and their development from the very beginning. And we are all here today because that's something, um, that I think we all share an interest in, uh, moving forward. So we're gonna hear now from patients who have partic-, and their parents, um, who have participated in some trials, and get some insights from them about it. Now as Suz said. . . how do I change to the next slide? This thing? Okay. Woop, we already did that. I forgot. Okay. So we're just gonna really, really quickly go through a few, um, terms. They are on your glossary and so that's sort of a cheat sheet. As we're movin' through you may have somethin' to add.

But a clinical trial – let's just start with that. A clinical trial is a study that's, um, designed to test an intervention. And the intervention can be a drug. Today we're only talking about drug clinical trials, but it actually can be any kind of intervention. It could be a psychological intervention, um, it could be a dietary intervention. Um, whenever we think about clinical trials, we think about placebo, right? And placebo is a compound that looks like and tastes like, uh, the study drug, um, the drug of interest, but actually has no biologic activity.

The next one is randomization. Uh, and that's show, um, a participant in a study is put into one treatment or another, um, by chance. Okay? And that could be a placebo, or it could be, um, a study group, or a comparator drug. Uh, next is the blinding, double-blinding. So if the trial is blinded, it means that the participant doesn't know what drug they've gotten, or whether they've gotten placebo or, um, what. Uh, double blind means that in addition to the, um, uh, patient not knowing, the provider also doesn't know. So nobody in the local study team knows, um, uh, whether you're getting drug or not.



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An open label trial is a trial where everybody knows what drug you're on. Okay? You know and the people taking care of you also know. Um, a withdrawal trial is a study where everybody gets study drug to begin with. And then the responders – so people who have had benefit from the drug – are then randomized to either get. . . continue on the study drug or to, um, uh, get placebo, to have drug withdrawn in a sense. Okay?

And the reason that we picked these particular definitions is because, um, having had a bit of preview about what's to come, um, I know that those, uh, um, terms are gonna come up. Thanks.

[applause] [01:30:05]


Suz: I think that ramp gets longer every time I walk up it. Um, all right, thank you so much Laura. And, uh, we are gonna very quickly do our polling questions, um, because I-, I do wanna make sure we have enough time for our panel and for our discussion, and our closing comments later. So, um, let me turn it over to the back of the room for our polling questions. And a reminder to the folks on the Web, switch to your polling tab. So have you or your child ever been involved in a clinical trial for a JIA treatment? Wow. Okay. I think that's a pretty decided answer, so not a-, not a whole lot of involvement in clinical trials.

Let's go to our second polling question. If you or your child have been involved in a clinical trial, what made you want to participate? What was it that prompted you to do that? Let's see what answers we get here. [chuckle] [01:31:15] So we'll. . . we don't know for sure, we just know it's not any of the other answers. Okay. Well, there we go. Ran out of current options, wanted to help, the doctor recommended the trial. Okay. Great.

And then the third polling question, please. If you haven't been involved in a trial, and we'll actually be kinda talking about this a little bit more in our large group discussion. So, so far lots of people haven't been, either haven't been asked, or weren't a candidate . . . fear of placebo, being stable on other treatments. Okay. Great. All right. Well thank you. That really gives us a sense for sort of where we-, who's represented in the room and online, so that's really helpful.

So let's go back to the slides, and now we're gonna hear from our third, um, uh, panel of patients and parents to really focus on clinical trials. And, um, they're experiences with clinical trials and there thoughts and insights. So first we're gonna hear from Rochelle. And again, just like the prior two panels, we'll go one-, one, two, three, four, and then we'll have our large group discussion.

So, Rochelle?




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Rochelle Lentini: So first I have to say thank you to everyone in this room for hanging in this long. This has been a very long day. Um, my name is Rochelle Lentini and I actually, um, have rheumatoid arthritis myself. I joke that I caught it from my children. But, um, really I'm here today to talk to you about my family. My husband I have, um, two children. One is 21 and the other one is, uh, gonna be 19 on Sunday. Um, our oldest son, he began with enthesitis-related at 16 and by 18, 19, the doctor said, "No, I think he's good. He's doing great, in remission. We're-, we're-, we're doin' good." Our youngest son, um, Parker, he actually began having symptoms at eight years old. And he's who I'm gonna to you about because he's been involved in clinical trials. Um, when he was eight, he began with some fevers, this rash that would move around on his body, incredible pain, and a little bit of joint swelling. But, to us, we never noticed it, and the doctors didn't notice it.

Our primary doctor kept tellin' us, "Go to the ER," over and over. Probably about 9 or 10 times, we went to the ER. By the time we get there, no rash, no fever, like we made the whole thing up. Um, it literally took us a year and seven months to get a diagnosis. Uh, from that point on, it took another five to six months to get the correct diagnosis. And he was diagnosed with systemic onset juvenile arthritis. Um, in addition to that, he has hypogammaglobulinemia, which is an immune deficiency, and he has overlapping syndrome, so he has a lot of bits and pieces of autoimmune diseases.

Uh, his current doctor says that we lost way too much time in the beginning in getting the appropriate diagnosis for him. And ever since then, uh, we've been chasing the disease. And it is mean. It is really mean. Um, in the beginning of his journey. . . it started in elementary school. And he had to be on very high doses of steroids for about four and a half years. Um, he was in and out of the hospital. The first year after diagnosis he was in the hospital 14 times. He's had heart swelling, um, four times, he's had lung swelling quite a bit, [indiscernible] [01:35:22] lung disease, and swollen lymph nodes. Um, several times they thought he had lymphoma because his lymph glands were so swollen – um, all of them actually. I didn't know there were lymphs in the stomach, but there are. . . or down there. Um, he ended up using a wheelchair for four and a half years, and by the age of fourteen he had to have his first reconstructive surgery on both of his ankles, tibia, and feet. And right after this constant. . . he's about to have his fifth surgery on his feet. And right after his . . . [crying] [01:36:05] Excuse me.

He has 18 medical specialists and, uh, I just feel like his body is broke up into pieces amongst all these doctors. For us, the internal organs is our most concern. However, um, right now they're under control and currently his SI joints just keep getting worse, and worse, and worse, and his spine is startin' to fuse. He has been on everything. He's on his tenth biologic. And our biggest concern is that the biologic that's he's on only target one area. And for him, we feel like he needs multiple biologics, and so one of my thoughts for you all to consider.



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This has had a huge impact on our life. I'm sure you can imagine. I just recently retired from the university to take care of our son. Um, we were involved in a clinical trial and he started that trial at 12. . . do I really only two-and-a-half minutes? [chuckle] [01:37:23]. Sorry. Um, there's a washout period to a trial and that is horrible. So when you have no options left because your kid is so sick, you're willing to try anything. So we knew there was a placebo risk.


They put him on 80 milligrams of steroids to try to get him through this period of time and he got placebo. He went into macrophage-activation syndrome, and he started having trouble with his immunoglobins – they were, like, nonexistent. Um, the good news is, then, he did get drug and that drug has been the best drug he ever had. It worked for-, for him for three years, where all the other biologics he had tried only worked for 3 to 10 months. And when I say "worked," we never got close to medicated remission. He still has not even be close, but he definitely had improved quality.

Um, one of the things that I-, I would like to have you all to consider is that when, you know, you have a child with a life-threatening disease, like systemic onset JA, we-, we need more options available and we would like there to be an expedited review process for these kids, especially the children who have multiple issues. And then the multiple issues are also a concern, because then they get excluded from, uh, studies. And I just have two final things I'd like to say. You know, um, this is not a one-size-fits-all disease, and so the drugs aren't either. With such limited options, my child is under constant medical care. And in a good month he's ha-, he has 4 medical appointments, but on an average month, it's more like 8 to 12. Much has been stolen from his childhood and his wish, and my wish, and probably every parent in this room, is that the next generation of children do not have to struggle like this. When he realized he was on placebo, the doctor gave him an out and said he could leave the trial. And Parker said no. He said, "I know the cure is not here, possibly, in my lifetime, but if this can make a difference for children in the future, then I must do this."

The biggest impact JIA has made on my life is in teaching me how very precious life is. When you've watched your children-, child's closed be cut off of him to administer life-saving measures, you know that a key is needed and that is out there. And as my son, Logan, says, "We just have to find it." Thanks.

[applause] [01:40:22]

Andrew Curtis: Good afternoon. My name is Andrew Curtis. I'm 18 years old and I live in Orange County, New York with my parents and older brother, Jimmy. In a few weeks I'll be starting my first year of college at Albany College of Pharmacy and Health Sciences where I plan to pursue my doctor of pharmacy degree and get into clinical pharmaceutical research.



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I was diagnosed at two years old with juvenile rheumatoid arthritis. I was an early walker and [cough] [41:04] sorry. . . I was an early walker and was always running around with my brother, climbing on anything we could find. But suddenly everything changed. All I wanted to do was be carried, I refused to walk up and down the stairs. When my mom tried to put my shoes on, I complained that it hurt. She was getting worried that something wasn't right.

My mom took me to a pediatrician and he listened to what she had to say. He looked at my knees and ankles and thought I had arthritis. My parents were shocked but agreed with our pediatrician to at least have some blood work done to hopefully rule out the possibility. Unfortunately, within a few weeks I was being seen by a pediatric rheumatologist and was given the diagnosis of phosi articular juvenile rheumatoid arthritis. That diagnosis was given in 2003 and is today known as ju-, oligo juvenile idiopathic arthritis.


JIA is an auto immune disease and it's made major impact on my life. I was so young that I don't know what it feels like to live without pain. My family always supported me and encouraged to try new things, and I never gave up. As a result, I graduated high school with high honors, I was a three-season varsity athlete for all four years, as well as the lead euphonium in my high school band.

My arthritis has caused my family to change a lot of things to help me. For example, when we go on vacations, they need to think of-, think ahead about where we'll go, how we'll travel, when I'll take breaks for me to rest, and how my medication will be transported and kept on the trip.

The photo in the middle was taken shortly after my diagnosis. It's a picture of me in Cape Cod on vacation, sitting in a red wagon. The picture alone is pretty insignificant – and just-, just a kid in a wagon – but that wagon was-, is a major change for my family. It was a birthday present from my grandparents and it allowed me to be-, be pulled around rather than carried everywhere. So I could still go to the beach like a normal kid. Then it would make life so much easier for my parents because they could pull me around rather than just having to carry me everywhere.

In the early years of my diagnosis, there were so many specialists to see, and so many therapies that I needed, that my mom had to stop working and take care of me. It wasn't possible for her to work and take care of me at the same time. She can now work, but only part time, so she can still take me to all my appointments.

While I was looking for colleges, it was very different from when we looked at my brother. For him it was about programs and how the campus looked, and how it felt at home. But for me, every visit involved meeting with the disabilities office, looking at the buildings to find the elevators, and noticing if the-, if the paths were hilly and far from classes. Every place that left me wondering, "Can I actually function in these



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surroundings?" I do believe I found my perfect school, but my selection process was different from that of a typical student.


When I was 13 years old I failed on a biologic and my parents needed to-, needed to decide on my next treatment plan. At this point, it was 11 years into my disease and we were running out of options due to the lack of medications. At this point, it was looking like I'd need to move to infusion therapy. And my parents were hesitant because I was about to start eighth grade, and I would have to miss a day of school every month for this treatment.

My rheumatologist mentioned that there was upcoming clinical trial for an infush-, for a drug that was formerly only used as an infusion, but now is available as a subcutaneous injection for children. They were worried about allowing to be in the trial. But they only agreed because they were told it was open label and I would not be receiving a placebo. We were not willing to risk receiving a placebo and having my JIA continue to flare and lead to more joint damage than I already had. We decided to take a chance and I was accepted into the study.

Initially, I was followed very closely to monitor me for adverse reactions. This involved regular appointments on a very specific schedule. We had to drive an hour each way to see our rheumatologist and couldn't miss an appointment because that's when I would receive my medicine. While I understand the need to be closely monitored during the trial, it became a burden from time to time. We had to work around a rigid schedule and as I entered high school, many times, keeping my appointment made me cal-, made me miss exams and other school activities because of my absence. I'm still in the study now and it's been an almost five years. There are times when I considered withdrawing from the trial because it became more and more-, or challenging to make it and to be able to go to the appointments on specific dates. I stayed in the study because of how much the medicine was working, and the drug was finally FDA approved last May.

They continue to follow me in the study, and I'm happy for you to know that my experience helped other kids get access to this medicine. But if you look at the photo – Uh, sorry. So your left, my right – it's a collage. But the big picture is me in my first day of eighth grade, one month before the trial started. That, and every picture since is a school photo. That's how long I've been in this trial and that's how long every other kid's had to wait for the availability of this medicine. It seemed like a very long time for approval and I wish that it could have come out sooner for the other kids that were running out of options and couldn't find anything.

I'm so grateful to have had the opportunity to be in this study, but I still feel bad for the kids who fail on medicines and ran out of options like I was. My greatest wish is that we could change the way clinical trials are done to meet the needs of the patients. It wasn't always easy for me to get to my appointments, and I was lucky that my



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medicine was helping, but I feel that five years is almost too long for other kids to wait to have this medicine. We need a process to allow medicines to get to the market faster because not every kid has the same type of arthritis, and not every medicine is gonna work for every kid.


We need more options, and we need them now. My hope is that future kids will have the best medicine available to them when they're diagnosed so that they can get into remission quickly and live the most productive lives they can. I'm almost 18 years old. . . I mean I'm only 18 years old, so I'm sorry. Just turned 18. [chuckle] [47:00] I'm 18 years and I have permanent joint damage in my ankle and jaw. Next summer, when my friends are looking for summer jobs or hanging out, I'll be undergoing reconstructive jaw surgery to correct the damage that my juvenile idiopathic arthritis caused to my jaw. At this point in my life I can't help but wonder, if we had better medicine in 2003 what my outcome could have been. Thank you for allowing me to share my story.

[applause] [01:47:24]

Harley Powell: Good afternoon everyone. Uh, my name is Harley Powell. I'm 21 years old. I'd like to thank you all for attending this extremely important panel event and I would only like to command just a moment more of your time. I was diagnosed with JIA when I was two years old. As a matter of fact, I was diagnosed with juvenile rheumatoid arthritis, and I remember that only recently did the terminology change to become JIA. We've been searching since I was first diagnosed for a treatment that would put my arthritis in remission. It's been difficult to find the combination of medicine that would both suppress my pain and that consisted of bearable side effects, and we haven't succeeded in remission, but I think we've all finally found a medical regime that works well after 19 years. But it's been really rough for everyone.

And I say this all to you because 19 years is a long time. And you might all forget that I was a child for most of it. Children are limited, both in their options for medical treatment and in the ways that they can focus their energy. They can either focus their energy on school, or on their diagnoses, and it is veric-, very difficult to balance both. That is why the gathered members of the FDA here, and the pharmaceutical representatives, and the patients and family living with JIA, must continue to participate in an open dialogue about next steps to take. Because I know the children don't have to be limited in their option for treatment, it's just up to all of us to find the answer. What needs to be understood is currently there's a dire and pressing need for more accessibility to new and developing drugs that are currently undergoing clinical trials, and that children can have access to. Treatments that children currently have access to, without being in a clinical trial, are still disruptive to their schedule, and are sometimes a burden to bear when consider that they are only children.

For many years, the only medications that were al-, at all successful in treating my JIA were administered intravenously. That meant that I had to spend an entire day in the



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hospital while qualified professional administered them. And it also meant that I had to regularly be out of school once a month, or once every three months that it eventually became.


When I was a child, I also had to go off a very responsive medication because it had to be administered through a shot. Both of my parents worked and they had to give it to me before they left for the day. So that was just the way it had to be, but I did go to school crying every day.

Without access to clinical trials and new medication, children and their families are forced to choose more extreme measures. With few options available to me, I underwent a bone marrow transplant in elementary school. I was later told that this was an unconventional, if not an extreme approach to take, but I remember at the time we were hoping that, like, any transplant, the clean tissue would take and start producing stem cells in my body that did not have arthritis in them, or did not recognize my immune system as a threat.

The transplant, unfortunately, didn't take. And prior to that surgery I had had a port surgically implanted at the beginning of the transplant process. But that port then became infected several months later. I was rushed to the hospital in the middle of the night because my immune. . . excuse me. . . and even after I was released from the hospital, I couldn't leave the house for several months because my immune system was so compromised. Missing months of school at a time, both for that and for my treatment, was not good for the way that I planned my school career, because I would preemptively plan to be absent for months at a time, even in the following years when I didn't need to be.

The middle school that I attended only had one working ele. . . excuse me. I will come back to the point. But first I need to say having arthritis changes the way that you interact with the world. Joint damage can occur frequently and often. And for a very long time my hips were so damaged that it caused me great pain to walk. I use a mobility scooter at school, but that posed it's own problems. The middle school that I attended had only one working elevator in the entire building. It was split between the seventh grade classes on the first floor and the eighth grade classes on the second floor. And every day, regular as clockwork, the elevator would break when I needed to go back upstairs after lunch for Spanish class.

Eventually the school got sick of calling the repairman in every day, so they moved Spanish class to the first floor. Everyone in the class knew that they had to move it because of me. I can't explain to you how embarrassed I felt. I wanted to melt to the floor. And it was completely conspicuous to everyone else that every day I was absent, was a day that Spanish was held on the second floor. I could see that school was different for my younger sister when she passed through the same middle school. And when I saw that, it became abundantly clear to me that a gap exists between children



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and teenagers who are diagnosed with JIA and those without such diagnoses, and that gap needs to be closed.

Personally, I waited upwards of five years for a medicine that's working fantastically for me now. And of course there's a reason for caution, and every medicine needs to be monitored and regulated, but I don't understand why I had to wait five years, and it seems to me I could have avoided a lot of pain if I didn't have to wait that long. As a patient, I know better than anyone how important it is that we make sure that drugs are safe and effective, especially concerning children, and that can take time. But it needs to be balanced against the pressing need of the ticking time clock as patients who have run out of options are anxiously awaiting access to these therapies. To the pharmaceutical companies, I would ask for consideration of delivery method in age groups, and how difficult some delivery methods can be depending on the age of the patient. To the FDA and the pharmaceutical companies, I would ask for greater access to clinical trials for children. One way that this can happen is through more open/exclusion criteria so that more kids are able to be included in trial. And another way is by partnering with us, the patients and the families, to design clinical trials that are easier for us to participate in. I don't mean to suggest that children don't already have access to clinical trials. You just heard the previous panelist suggest that. . . excuse me, he said affirmatively that he was at panel. So I'm really happy that that's happening. It's already happening, and that's fantastic. But I would love some more of it. Thank you very much.

[applause] [53:47]

Vince Del Gaizo: Boy. So, um, hello everyone. My name is Vince Del Gaizo. So I live in, um New Jersey, and I wanna thank everybody for organizing this meeting and givin' me the opportunity to talk to you a little bit about, um, clinical trials. Um, a lot of people know about my son and-, and-, and, um, what he has gone through. He has also experienced a clinical trial and I'll talk to a little bit about it today. So, um, the picture of the three children – those are my three children, and yes, they're triplets. And the person all the way on your left is my son who was diagnosed with systemic JIA. So this picture was taken probably about two or three weeks after his diagnosis. And just to go over his diagnosis very, very quickly – he was really, really, really sick – 106 fevers twice a day, uh, just about every joint in his body was affected, lymph nodes. You look up systemic in the dictionary, he had the symptom. And that went on for about a month in an intensive care unit, um, where they try-, poked him and prodded, and tried all different kinds of things to see what was wrong with him, and couldn't really figure it out.

So eventually, um, we got the diagnosis and at this point we had a very tolerance for risk. Um, really, his condition could not get any worse than it was right there and it just had to stop. Like, it was-, the way he was was not conducive to life. And so we didn't really care what the side effects would be. The only thing that mattered to us is the



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quicker it works, the better, and we don't care about anything else. Um, we stabilized him, um, and he was well for about seven or eight years. And then, uh, he had a systemic flare, and this is where the clinical trial comes in.


So this systemic flare affected him, um, both wrists could not move one degree in either direction, his right elbow was locked at 90 degrees. I mean, he literally couldn't straighten it if you had a vice. Um, his shoulder, his knees. . . he couldn't care for himself. Similar to what Nick was talking about earlier – literally, we had to cut his food. He would go to get a drink of water, reach for it, and he couldn't straighten his elbow so he couldn't get the water, so he would get it-, get up, go to the pantry, get a straw, put a straw in his drink, and then take his drink. So he's just kinda figure-, and these stories are not unique. Everybody here has these same stories. These kids are so resilient and just wanna be normal kids.

Um, never once during this time did he ever complain about pain. . . never complained about pain. Uh, In fact, during this flare, he participated in an ice hockey tournament and his biggest concern going into the tournament was he also had the rash, and the rash was very, very itchy, and he used to scratch himself until he bled with this rash. And he would cry about this rash. And his concern going to play in a hockey tournament was not that-, that he couldn't move, it was that if he got itchy and he had equipment on, he couldn't relieve his scratch. So he wound up playing, and it was really kind of a-, a display of courage, you know, that all these kids have, that I've never seen.

Um, so, you know, he just decided that he's not gonna let the condition dictate what he can do. So what do we do? At this time there's no approved treatments for his condition. Uh, medications that are approved for JIA don't really well-, work well with systemic, um, disease, and that was his most important, uh, outcome, was to get rid of that rash. So we learned about this study, um, um, for him to enroll in, that was going to help with his-, uh, with his systemic manifestations of this disease, and his joints, and everything else.

So, um, at enrollment we learned about psoriasis. If you have psoriasis you can't join the study and my son had been having on and off bouts of psoriasis and we're going in there, please don't let him have psoriasis today so he can participate in this study. Um, so, you know, that just brings me to. . . like, I understand a little bit about the science with inclusion criteria. You wanna-, you wanna control everything so you could see if the thing is working, um, but I think wh-, wh-, from my perspective, you're-, you're looking for a cohort of patients that doesn't exist. And then when you do prove that it works in that cohort of patient, that patient is nothing like my son, so I still don't know if it works in my son because my son is nothing like the people that you recruited for this trial.

So anyway, um, get your-, your sheets out, your glossary. So he was put into a randomized placebo-controlled double-blind study that also had an open label period after the placebo phase. Um, we did-, we enrolled him in this study and, unlike Parker,



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um, I did it-, I enrolled him because I thought this was the best course of action for him. And the greater good. . . schmater good.

[chuckle] [01:59:52]


My primary role as a parent is to protect my son and to do what's right for my son. The greater good part of it, that's the value you add. And my son can do something for the greater good for himself, but I can't make him do something for the greater good for others. I have to protect him. So we went in, he-, he wound up getting drug. He had an immediate response. He woke up the next day, literally, 24 hours later. He says, "Wow, I can move my arm again." And then he picks up his shirt and he says, "Wow, the rash is gone." So it was a really happy ending. Um, I look back on that and I say, wow, if that was a withdraw trial I would no way be able to pull that rug out from under him and take that medication away.

After he suffered the way he did. . . find something that works – almost like a tease and say, "No, can't have it anymore." So I would have a really hard time enrolling in a study like that. Now if that study happened today, most-, I-, I don't know. I would have a hard time enrolling in a placebo-controlled trial if there's a-, if there's an option available to him, unless the placebo period was really, really short. Um, you know, I guess what I'm tryin' to say is you guys have a really, really hard-, hard job to do. Uh, we need quick access to safe, effective treatments, and it needs to be done in an ethical way. I-, I-, I don't have the answers. Um, but what I do think is that a possible solution to this is to have some flexibility and to involve all the stakeholders in designing the protocol. Um, we can't help with everything, but, you know, we can help with things like ethics and would be enroll, and would we not enroll. And I think that's really valuable information to consider when designing the-, these trials. Because they're nuanced, and depending on the stage of the disease you're in, you have a different-, you-, you have a different feeling on wheth-, what is important to you. So in the beginning it was just about quickness, I didn't care about risk. Later it was about getting rid of the rash. And-, and it, you know, you have to have flexibility to design the study that'll be the best. So, uh, I thank you for listening to us. And, uh. . . I don't know. . . Suz?

[applause] [02:02:37]

Suz: Great. Well thank you again. Again, just another just amazing panel bringing to light so many, um, so many needs, but also so many tough issues. And so thank you so much to all of you.

Um, we have about. . . let's see. . . 25 minutes to do our large group discussion. For our folks online, I want to remind you to now switch from polling to questions. And again, it doesn't have to be a question. Just to use the question function . . . can be a comment. Uh, I actually think I might time prerogative and jump to the second question, because in our polling questions we asked how many folks have been in a trial. Um, why, why



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not. I think if we go to-, to question two, we can kinda get at the same thing. So would you be willing to participate in a clinical trial, whether that's you because you're the young adult who would be in it, or a parent who would enroll your child. And then why or why not? And I-, I think I see someone already ready to answer.

Woman: Hi. Um, so for us, this question has come up a lot lately, just between parents and between, um, just even my husband and I, as we know that there are few options for our daughter with SJIA. And for us it's very hard. Uh, when she was first diagnosed, she was in the hospital for two weeks and didn't move. Um, and watching your kids like that is very hard, especially when they're so little. She didn't move, she didn't talk, she couldn't feed herself. It was a fight to get her to eat one meal a day. She was three and lost, like, 17 pounds in the hospital. I mean, that's a lot for a little one. She was barely skin and bones when we got her home. And so for us, the hard part is would she receive a placebo and if she does, does that send us right back to the hospital, does that put us in a life or death situation? And that's not something I'm really willing to put my kid through. Um, so for us it would really be whether or not we could be guaranteed that she would not get a placebo.

[applause] [02:05:07]

Suz: Thank you for sharing that. And I'm wondering, maybe I-, I should have asked you while you were still up. As a follow-up, sort of what-, what Vincent alluded to, um, would the length of time matter? If it was five days of placebo versus a longer time? Okay. Okay. That's helpful. And I'm seeing some nods in the room as well. Another comment?

Becky: Hi. My name is Becky. You met my daughter, Ellie, this morning. Um, the-, the piece of the puzzle that she didn't share with you is that her older brother who's two years older was diagnosed with enthesitis-related arthritis when he was four and she was two. So for the vast major-, majority of her life, she's only known being the supportive little sister until about two years ago when things changed for her. So there was definitely a-, a big shift in the family dynamics for all of us, and obviously for her, most importantly.

Um, as far as the-, the clinical trials go, both of our kids were very blessed. We live close to, um, Hershey Medical, Penn State, um, Hershey Med Center. So it's a comparatively an easy drive. [chuckle] [02:06:13]. It's a half-day off of work and the kids out of school early. And we have it figured out – the day that they're-, the doctors are there later, that kinda thing. Um, so we're-, we're with doctors that we completely trust, um, and definitely hear us. Um, but this is never been something that's been brought to our-, to our attention or posed as a pot-, a-, a possibility. So I don't know whether some of that's because of the way my kids have presented or the medications they're currently on, or, um, you know, what plays into that. But it's definitely something that, um, I think as parents, my husband and I are open to.



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Um, making sure that our kids have an-, an open-minded view of what possibilities are out there, what, uh, might be a better option for them. Um, it is very daunting to know where you are in the hierarchy, and as you climb further up towards the "end" of what's available for your kids. Um, and other people said this today, too, versus their age, where we are in the whole life spectrum, and knowing that you're getting close to the end of what-, what people can do for them at this point, um. . . Yeah, we definitely would be open to it and it's something that I'll probably ask some more about. Um, I'd be curious to see what potential there is for our kids to find some hope for themselves and, obviously, ultimately, to help the greater cause, too.

Suz: Right. Thank you. Thank you.

[applause] [02:07:39]


I think that really aligns with the-, the answer to the polling question. Is it that you're not being asked, are you-, is it not being offered as an option, or is there some reason maybe you're not a fit, and so that's why you're not being asked? What is-, what is that lack of opportunity piece? So we have a-, another comment in the room.

Brook: Okay. So, again, Brook, this is Kate.

Kate: Hi.

Brook: And Kate has poly and ERA. And so one of the things I would just say, um, I'm gonna let Kate talk about whether she would be willing to be in a trial, but I think for the FDA and for the drug makers, I would say the patient voice is really crucial here because one of the things that happens to these kids that fail multiple medications is they start to. . . Their brain's really powerful, they're really smart, resilient kids, and so they start to develop some aversions to certain things, like the smell of alcohol, the color yellow, the numbing cream that comes out. So any time you're thinking about how to get children and parents interested, you've gotta think about that patient voice and the fact that we have experiences that can be valuable in that process, that can help that clinical trial be more receptive, not just to the parent and to the doctor, but also to the patient. So I'll let the patient talk.

Kate: So I feel like if I were guaranteed that I would, like, not get the placebo, than I would be more likely, definitely, to participate in a clinical trial because it would benefit, um, more kids like me, and maybe be closer to a cure. But another thing is that the placebo is a really scary thing. Um, having the chance that you're on no medication, and maybe not even knowing that for awhile is a really scary thing to think about, um, especially as a kid. You know, sometimes it's hard to make your friends understand that, um, I'm having a flare, but don't worry, it was planned for. [chuckle] [02:09:44] So, um, yeah. [chuckle] [02:09:47].



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Suz: Right. Thank you so much.

[applause] [02:09:51]

Man: I need to lead off with a question just to better help my understanding of clinical trials before I can answer this one. But it shouldn't take too long. When clinical trials are administered, are they done at specific centers, depending on who's doing the research? Is that my understanding?

Suz: I-, I'm looking to my research colleagues in the room so that I don't say something wrong, but generally speaking you're at a study site because that's where. . . and there could be multi site's studies, but it's a happening a study that's happening somewhere where you are or you've traveled to be a part of.

Man: Is it particularly limited to sites where there is a dedicated pediatric rheumatologist?

Suz: I think it would really depend on the study.

Hermine: Usually.

Suz: Okay.

Hermine: Us-, usually.

Man: So in this-, in this connected age of technology, how can those of us who live two-and-half-hours from our center participate in a way that is not totally disruptive to our way of life? Because if that question can be answered, we're onboard.


Hermine: So, I mean [indiscernible] [02:10:54]pediatric-, EMS, right?

Man: Yes, we have EMS.

Hermine: The clinical trials are all listed on ClinicalTrials.gov. That gives you the opportunity, even if your doctor's not educating you about a certain trial opportunity to go there, to see which trials are available. Now, which trials are available depends a little bit on what medications have been found to be successful in a dog. Going back 50 years. . .

Man: Okay.

Hermine: . . . children with arthritis got medication for 30 years and were given with confidence like gold[?], and it turned out that it only gave side effects to the kid and had no effects on the medica-, on the house of the children at all. For that reason we



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need to be careful when we just give medication, hoping that it works. I think we need to be cognizant of the short term, but especially long term side effects. Remember those first oral contraceptive pills? They caused cancer later on in life.

Man: Um hum.

Hermine: So we-, in the offspring of the-, of the ladies who took the medication, so we need to be careful and have a control group somehow.

Man: Sure.

Hermine: I fully agree that this control group, you know, has to be, you know, that whoever gets placebo, it needs to be very patient-centered. And at no, you know, at no time a patient can be at-, at major risk.

Man: Um hum.


Hermine: And preferences is always need to be considered. And, you know, whenever you, or any family or any patient participates in a clinical trial. . . It says in the consent form, if you don't wanna do it anymore, you are free to go away and stop with the whole trial. The-, many of the trials in juvenile arthritis at the current time, they are designed with a long-term extension study. That was mentioned a little bit earlier, the five years on the study.

And the reason for that is being done is because sometimes it takes a long time until one has enough evidence to say we're confident that medication is safe and effective in children with arthritis. And this long-term extension is done to make sure the children, who might have been exposed to placebo and benefit from the medication, have long-term access. You know, that's done for ethical reasons, not to keep patients going backwards and forwards through the study site. At any time a patient is allowed to stop. Here at Cincinnati Children's, where I work, we have patients who have been on a study for six years. But, you know, they get-, they come in every couple of months and make sure they are safe on the not-approved medication yet, but they have access free of charge.

Man: Sure.

Hermine: So it co-, we need to weigh the benefits. But knowing about a study is best done in [ClinicalTrial.gov](https://clinicaltrials.gov), and many of the pharmaceutical, um, uh, companies, they will pay for study patients to drive, or reimburse you for time and-, and expenses.

Man: But that doesn't necessarily cover time lost to your employer, other family considerations. You see what I mean?



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Hermine: Yes. Yes.

Man: We-, we live in a very, very connected age. It-, it's 2018, we should be able to make this work with so many hospitals across the country.

Hermine: Yes.

Man: I live-, I live in Peoria, Illinois. We have the Children's Hospital of Illinois, which is a fantastic facility, they just lack pediatric rheumatology. I feel if you have a center that's-, that's close to you that might be able to collaborate with an administering center of the trial.

Hermine: Mmm.

Man: We-, we already have a hard enough time with the people getting access to pediatric rheumatologist, let's not make the trials even more difficult.

Hermine: Yeah. Fully understood. But on the other hand, when you have a very severe disease, you also wanna make sure that if a child gets an experimental drug, that that child is safe. Imagine you have an infusion reaction – it's a bad one – you want the child to be in a safe place.

Man: Sure.

Hermine: So one needs to weigh. . . and. . .

Man: So-, so, perhaps, a vetting process then for the facility?


Hermine: Yes. And there needs to be a pediatric rheumatologist specialist who knows what she or he is doing to make sure that, you know, the benefits of the drugs are appropriately affect.

Man: Um hum.

Hermine: You know, you heard a couple of patients going to the emergency room for months and years, and the arthritis not being recognized. So by the same token, such a doctor may not be able to recognize the benefits or the detrimental effects, and that will be scary.

Man: True. It's a-, it's a complicated issue.

Hermine: Yes.



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Suz: I-, before, I wanna make two quick points, and I know we've got, um, some comments from the webinar. So I'll go to you first and then the-, the webinar folks. I wanna just kinda capitalize on something Hermine just said, or-, or reiterate it. . . is a point just to inform our conversation, that there is the option to drop out of the trial. So if something was not going well, you wanted to drop out.

Another important piece, and it would totally depend on the study, is that placebo doesn't always, or necessarily, mean, no drugs. It might mean what you're on what's called, "standard of care," like Methotrexate, or you're on NSAIDs or Prednisone, or something, you're just not on the study drug, you're not on the biologic that's being tested. So I don't know that changes what people would contemplate, whether that would make you more willing. But it doesn't mean there's no drug. It might just mean it's-, it's absence of that test drug. I just wanna make sure that's clear. Now we'll go to you, and then we'll go to our webinar comment.

Woman: Um, just kinda to piggy-back, I'm not as familiar with clinical trials. Um, our rheumatologist hasn't tal-, discussed that with us, or talked about what options were available. Um, I don't know where the closest uveitis specialist is to us in the Midwest, um, to know if there's currently clinical trials goin' on with that. One thing that I did wanna ask, I guess, and I don't know who can answer the question – once a drug, my curiosity is, once drugs are deemed effective, and being FDA approved, where-, where is the panel that discusses with the patients and the parents, um, kind of in related to how children react to color, smell, feel, um, that kind of thing.

We just recently went on the new, uh, pediatric formulation of our biologic and I had to spend 20 minutes convincing my child this medicine was different, that it wasn't gonna hurt anymore. Because the injection looked exactly the same as the one that she screamed through for 30 minutes before, during, and after, getting that biologic. So somethin'. . . I mean, when you-, when you are administering to children, you have-, somebody has to ask those questions on, "Okay, they're on the adult formulation. We're not making a pediatric one. Is it smart to make that needle to look exactly the same? That packaging?"

Um, red, you know, I don't know how many people have told me, you know, "Doctors don't wear red because child-" you know, "Pediatric doctors don't wear red," or that kinda thing, because children re-, respond to that as a bad thing. The injection she receives, the plunger is red. I mean, these are just things that, you know, are they taking these into consideration during the trials, or after trials? Are they-, who's having those conversations with parents and children to help minimize even that anxiety affect when children are getting these new medications, and on top of that. . . shots. Yes, our kids get used to shots. They get used to blood draws, but the only drug that has an oral actual pill is the Methotrexate, that I'm aware of. Everything else that I'm-, I've been exposed to has been only an injectable option. And when she was three years and



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couldn't swallow pills, the alternative was either the injection into her arm, or orally taking the injectable medicine, which they said tastes terrible.

I mean, these are the kinds of things that our kids, when they're really little getting these medications, somebody's gotta start thinkin' about this on how do we get the right medication into their bodies in the most effective way to guarantee they're getting' all of the medicine, and in a way that's not gonna cause them additional mental turmoil, and stress, and anxiety. . . I guess are my questions.

Suz: So yeah. So I promise we didn't plant her in the audience, because. . .


[laughter] [02:19:07]

. . . that's literally-, that is the-, you are at the essence of why we need to partner – so we need patients and parents at the table with drug makers and the FDA, not in the. . . kinda the way you described it, it was sort of at the end, once something is developed, we want it at the beginning. So what are the targets that you care about, what do you want out of a treatment, what should it look like, how can it be administered? I won't get into the science, but part of the challenge with oral meds right now is a lot of the drugs that are-, are very complicated and fancy, can't be oral yet. Um, I don't know if they will be, but that they kinda have to be a certain way. That doesn't mean we can't all get together and figure out what's the best way to deal with what we have. Um, so you just teed up that partnership beautifully. That's why we wanna all be in the same room on the same page right from the beginning. So let me take, uh, comments from the webinar, and then I'll come to you.

Woman: Okay. Uh, thank you Suz. So, um, most of the comments have definitely reflected what's being said her in the room. We had one parent says that, yes, they would do anything they could to help their child. Um, they've concerns about what happens if the child really goes downhill once they start the study, what's the exit plan, what would happen there? Um, also, um, looking at, uh, interruption to daily life – what are we going to have to do to participate in this study? Is it already going to-, is-, is it going to disrupt life more than this disease already does?

Suz: Thank you. Uh, definitely resonates with what we're hearing in the room. Thank you. So we'll go to you, and then I know we have a-, a question from the panel too.

Man: Sure. I think, wh-, when I sit back and I listen to the panel and then the questions that are brought up by the parents here, and the questions from the webinar, I see it resonating around lack of knowledge more than anything, on whether. . . I don't know when trials are happening was mentioned. I don't know where trials take place, was mentioned, and I don't know how trials are done, was mentioned. So it seems to me, like, we have an educational issue here. And the second item that comes to mind really plays off of what Vincent said. Vincent said I'm gonna put my son in this trial because we're at the end.



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And it's very easy, as a parent, to be motivated about jumping into a trial when you're at the end. So I think if we, maybe, entertain the idea as a partnership between pharma, the Arthritis Foundation, doctors, and those of us that are involved parents, together in a partnership. . . really introduce kind of a-, a shift, a paradigm shift in the way we look at clinical trials . . .

A statement was made earlier today that doctors sometimes don't get it that "good enough" isn't good enough. And so maybe that's the time that we start to talk to patients, and we talk as patients and as parents with doctors and big pharma, it's-, that's the time that we start to think about trials with our children, not when we're at the end of the rope like Vincent was. But when we're right at that point where "good enough" isn't good enough. Because, then, that margin of error, that possible placebo effect, or placebo result for one of our children, isn't from at the end to where we can't come back, but from not really "good enough" to where we can certainly catch them in-, in a net.

So maybe we need to change the way we look at trials when we do it, and a little bit on how we do it, but certainly a partnership between all of us that are in this room, to educate all of us about what the outcomes can be, what the safety nets are, the fact that we can be-, we can pull our child out of a trial at any given time, and possibly, get us all thinking about this differently.

Suz: Great. Thank you so much. Do you want. . . ?

Rochelle: Yeah. I just-, I just wanted to make the comment as the. . . if there-, if there's a drug that can be successful in a trial like we had, and the numbers aren't there, then what happens to the FDA approval. If the drug trial was successful. . .

Suz: Okay.


Rochelle: . . . but there' not enough children because it's a rare disease. So, if you could.
. .

Suz: I don't think-, I don't know if there's answer to that. But it's another issue when you have rare diseases and so, um, smaller numbers of kids, and so you can't. . . there's literally not enough people to get in the study to get a drug approved. Um, I don't know anyone has answers, but that's, yet, another thing we have to. . .

Rochelle: Consider.

Suz: . . . partner and collaborate on to tackle. But it's a-, it's a-, it's a challenge.
Absolutely.

And then you can be our last comment on this question, and then we'll go to our final question.



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Woman: I was just wondering, for somebody like myself who is kind of at the end of her road with medications, and being an adult with JRA, essentially, if a clinical trial for a new JIA drug would be something I could be able to consider. I know in the past, when I would be put back on drugs that I was on as a child, as an adult my body reacted differently. So I also think something to consider is, when developing these drugs for JIA, to consider that the more, you know, the older they get, the more their bodies change. They might react a little bit more differently as they go on into adulthood than they were as a child.

Suz: Hmm. Yeah, it's an interesting point. And-, and certainly we know that kids are not just small adults, so it-, it-, there's very different, um, things going on. So it's a-, an important issue. Um, I'm gonna go to our last question. . . maybe. There is goes. And this has come up already. I mean, we heard about. . . we've got the Internet, why can't we figure out how to be connected? How can it take less time out of our lives? Um, we heard about a little more comfort if there's a placebo – make sure that's a short period of time, or we could escape the trial if we needed to if something bad was happening. Or, um, maybe it's that you wanna know what you're getting and not be randomized, or-, or not be blinded. Um, so there's-, we've heard a couple of these things. We've got, gosh, about three or four minutes – I'm wondering if there's anything else that jumps out at you when you think, "What would make me really likely to participate in a trial?" Um, I think in the polling question we certainly saw, "If I trust my clinician and my clinician trusts this study, that's definitely gonna give me a vote." Oop, I see a hand up.

Woman: Scholarship money.


Suz: Scholarship money, is that what you said? You might have to explain. Money to be able to be-, to facilitate your involvement in the trial?

Woman: Just the possibility of scholarship money for college if they participate in the trial. If some scholarship money were available for something like that for an older patient.

Suz: That-, that gets a little complicated with how the ethics rules work, but I think. . . um, but incentives to be in a trial, um, or-, or reasons that make it compelling beyond just the trial itself. Again, we won't get into a lot of bio-ethics. But, Liz, do you have a point?

Liz: Well just to kinda go back to what Vince said, I think I would encourage my kids, who are young adults now, to participate, if they were permitted to participate with more than one disease. Um, you know, um, Emily has multiple, Sophie has two, so they're excluded from most trials before they can even get a foot in the door. So if we can look at that issue a little bit differently.

Suz: I think that has really come up loud and clear here today – the inclusion/exclusion criteria. How can we think about those differently? Is there a way we can include more



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people so that we generate more knowledge about. . . and I think Vincent said that, as well, about knowing our unique responses.

Um, we have a couple more comments over here. Okay.

Ellie: Um, it would make me more willing to do a clinic trial if I was able to be walked through the process and know what would happen. Like, have a Plan A, a Plan B, like, know exactly what would happen before I get myself into it.

Suz: That's great. Good idea.

Woman: Ellie stole my thunder.

[laughter] [02:27:57]

Woman: So I-, I was gonna say the same thing. So my daughter is six, and when we go to our rheumatologist and they explain what the different options are available, they-, even though they try to explain it at her level, a lot of times she just wants to click the little toy that's hanging around the stethoscope, she's not 100% there and focused. And then, of course, they come to me, and I'm tryin' to get her to listen while I'm listening. But, um, the control group, absolutely.


Considering the fact that something like SJA, which is what my daughter suffers from, is such a unique form, and is an auto-inflammatory or autoimmune, and there's all these different debates and negotiations. That scope of that control group, for that particular group, i-, you know, it can be so large. It's just making it broader. And then explaining to her and me and making sure that we have more than just Web M.D. to review. There's a lot of scholarly articles out there that some of us who are still in academics might have access to, but the average person doesn't. And getting that person to us, as parents, will help us make those informed decisions.

Suz: That's great. And I think, you know, the-, the. . . Yes, absolutely.

[applause] [02:29:00]

I think the Arthritis Foundation and CARRA both have, um, we-, we provide some of that information, it's certainly something we can do more of and make sure it's targeted and-, and get hand, you know, information into the hands of the folks who really need it and want it.

Um, let me do one quick check on the webinar folks. Do we have any other comments?



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Woman: Yeah, we just have one comment on this last, uh, question, um, about if you could be guaranteed that the placebo would also be something helpful that would be, uh, would make the decision easier.

Suz: So there is something called an active comparator arm, where you really don't use a placebo. You're testing drug versus drug. Um, and so I think we'll-, we definitely wanna kind of. . . We'll take note of that-, that comment and-, and and write it down. And it actually, um, reminds me to make sure I say today is just the beginning of this conversation, and so, um, we've started, we-, we-, we did the survey, we've had focus groups, now you've all participated. Please reach out of us. Email us. We're gonna have an email address at the very end of the slide deck. Um, you can find us all in numbers of ways – phone, email, however – and keep sharing these ideas and thoughts, because, um, this is how things will get better. This is how we will make progress.

Um, so I don't want to steal our final speaker's thunder, because we have a couple folks offering closing remarks. So I'm gonna go to Dr. Rachel Glaser from the FDA, the Division of Pulmonary and Rheumatology Products. And, actually, our wonderful panelists, if you wanna, um, find your seats. Um, we'll hear from Dr. Glaser.
[applause] [02:30:52]


Dr. Rachel Glaser: Good afternoon. Uh, thank you all for inviting me to share some closing remarks with you today. My name is Rachel Glaser, and I'm a clinical team leader in the Division of Pulmonary, Allergy, and Rheumatology Products at the FDA, and I'm also a practicing rheumatologist.

Uh, DPARP is the division that oversees drug development for rheumatic diseases, including JIA. My colleagues from the FDA and I are honored to have the opportunity to be a part of this patient-focused drug development meeting that has brought the JIA community together to advance the development of new treatments for the disease.

I would like to thank all of you who brave D.C. traffic and the challenges of travel, and thank those who couldn't attend in person but participated remotely on the phone and on the webcast to share your powerful stories with us. We recognize it take time, effort, and courage to talk about your experience with the disease. Thank you all for being part of this meeting and sharing your experiences with us.

This patient-focused drug development meeting is a unique opportunity for the FDA to hear from patients, caregivers, and family members, about the impact of JIA on their lives. The perspectives and insights you have shared allow us to approach our rolls with insight into the complex needs of the JIA patient community.

Patients are often experts in their conditions. The ability to hear what patients with JIA care about can help us understand what aspects of the disease are most important to



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patients, as well as understand how patients view the benefits and risks of therapies and devices for JIA. This knowledge can then help facilitate development of future treatments.

Today's meeting was a demonstration of the important perspective of the patient. You have all identified a lot of key issues today. Many of the themes I heard today were common to many of the stories, but I also appreciated that each person has brought their unique experience and perspective, which made the discussions even more powerful. The first key theme I heard today was about the loss of one's quality of daily life related to JIA. Some of this included even routine activities like dressing oneself, or your choice of clothing, difficulty making it through a school day, or being able to take the SAT, and difficulty driving.


JIA has had a significant impact on activities that people enjoy, such as the ability to participate in sports and other extracurricular activities, the ability to participate in social events with one's friends, and also has an impact on the activities of caregivers and, as we heard several times, of siblings.

Additionally, we heard about the impact on daily life and there were a lot of differences, but also commonalities in terms of the impact on personal life, professional life, social life, and emotional impacts, as well. We heard about feeling helpless, both from the patient's side, as well as the caregiver perspective, um, the impact of the unpredictability of the disease, the sense that JIA is an invisible disease, um, and the feeling different than one's peers.

In today's discussion, there was an emphasis on symptoms, and I thought it was very moving to hear how these symptoms impacted one's quality of life. The main symptoms that were discussed today were pain, functional limitations, and fatigue. Recognizing and understanding how these symptoms overlap and interact, and how they can be distinct, was very helpful for us to hear. One early panel member mentioned that her, uh, she used the term, "her child's own version of JIA." Um, and I thought the statement highlighted sort of the individual nature of the disease.

There was also some fear expressed about not knowing what tomorrow will bring. One panel member spoke of feeling like, uh, she was living in a domino setup, and several people spoke of the need to be careful about how they expend their energy, and the need to plan around their treatments in both their good days and their bad days. And that, even after good days, that they may pay for that later.

We heard about the importance of support from family and friends to help with some of these issues, and the impact on the whole family, and even extending beyond the family to employers who may need to consider, uh, a patient's JIA in their plans. We also heard about therapy considerations, including insurance and financial burdens. Uh, we heard about the need for infusions, which are associated with pain,



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difficulties with IV access, uh, for those infusions, and the need to plan for their appointments about school and work. We also heard about other side effects of treatments. And while there's some therapies available then tha-, that are helpful for patients living with JIA, there's a high unmet need for more safe and effective therapies. We heard several times about the limited time therapy-, therapeutic options for certain disease manifestations, particularly TMJ and uveitis today. Uh, at least one person mentioned, um, differences in the definition of success between the medical community and the patient, and that sometimes "improvement" is just not good enough. And we heard about the number of therapies that patients have tried, and the fear of running out of treatment options, and feeling that they're at the end of the line and their last option.


It was noted that treatments are needed to manage daily symptoms, functional impairment, fatigue, and preventing joint damage and disability. There were also discussions on the complexity of risk and benefit for such therapies, and also the risk of discontinuing treatment when patients are doing well. In summary, today's meeting was a very important dialogue. And, as Suz alluded to, this is-, we hope that this will lay the foundation for future conversations.

As FDA continues striving to incorporate the patient perspective more broadly into medical product development, meetings such as this help enhance our understanding of the patient's perspective and as-, assist in moving the science of patient input forward. This input is valuable for FDA's drug review because it helps provide the underlying clinical context about the severity of the disease, and the currently available treatments that is necessary to take into account when assessing whether a drug's benefits outweigh its risks.

This kind of dialogue is extremely valuable, not only for FDA, but also industry, academia, and other medical product developers in the room and on the Web. The perspectives shared today can help stakeholders identify areas of unmet need in the patient population, identify or develop tools that assess benefit of potential therapies, and also raise awareness and channel engagement within the patient community. With a growing appreciation for the benefits that patient-focused involvement can provide to patients, drug developers, and regulators, there's an increasing need to strengthen and expand the linkages with patient groups into the earlier stages of discovery and the drug development process. Patient involvement enables new opportunities as different partnerships begin to develop within an involving research and develop-, development enterprise.

Once again, thank you for your participation and for sharing with us today.

[applause] [02:38:18]



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Suz: Thank you again, Dr. Glaser. And thank you to all of our, uh, FDA and industry attendees, um, who joined us today. And finally, I'd like to invite, um, Dr. Guy Eakin and Dr. Laura Schanberg, uh, up to the stage to provide the closing remarks.


Dr. Guy Eakin: So it's the end of a-, end of a long, but really magnificent day. So I'm really pleased to be joined on the stage here by-, by our friend, Laura Schanberg, from, uh, from Duke University. We've had the opportunity to talk about, you know, things that we've heard today, and things that we've heard in the process of, uh, organizing this meeting.

And the first thing I want to say is that we just heard from the FDA, a really wonderful transparency that says that they have, you know, they have leaned in, they have heard what's been said today, and taken that into their own organization. So that-, that's a wonder-, that's a wonderful opportunity to note success in this meeting, so I very much appreciate the, um, the very active listening that's been, uh, that's been offered by the-, by the various, uh, professionals who have come to this meeting. So I do wanna thank the, uh, the-, the number of people who have joined us from, really, all walks of life. Uh, you know, on the patient side, those who have come from near and far – have participated in focus groups, have participated in surveys, have participated in the polling on the webinar, you know, here in this room, all have just an-, an incredible wealth of, um, a wealth of experience.

Certainly we appreciate the participation of the FDA, as well as other industry partners. And I think we can all walk away and say that if you-, if you've ever received, you know, the answer, uh, from someone that a-, that a patient is not an expert, you know, then I-, I-, I think we can all say right now that, you know, it's very apparent that, you know, that-, that person was working on the wrong question. And it's just been, um, tremendously insightful today to hear the number of, uh, the number of ways that a-, a question that we might think of as being, perhaps, obvious, is nuanced by, you know, by the people who are actually living with the disease.

So what happens? You know, now, this is, you know, this-, this meeting is not an end unto itself, this has to reflect an ongoing partnership between our organizations. So this-, the-, the, uh, the outcomes of this meeting will be-, will be written into a voice of the patient report, but, you know, we're-, we're in a place that we, you know, we feel that this-, this role is not finished and we encourage and expect that all of the academic and professional researchers will seek to incorporate patient level insights at all stages of study design. You know, certainly including the execution, design, and interpretation of the data that comes out of those studies.

Right now, you know, as we, you know, reflect on the day that we've had, you know, I think, you know, one of the things that-, that really strikes me is that, you know, we're, you know, what we've done here today is we've really checked in on an evolving concept of what benefit is and what acceptance of risk is for a patient community. And we're evolving that concept that-, that treatment, you know, is not just taking care



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of bodies, but supporting goals that are-, that are co-produced between patients and the healthcare community. And if we're supporting those goals, and not just fixing the mechanism, you know, that we're preventing those dominos that Anjie mentioned early on, you know.

And that we, you know, we really have to do this and support those goals, those patient-level goals, because, as we heard, you know, even when we get something as rigorous as a-, as a diagnosis seems, we heard today that that diagnosis may shift. That dia-, there may be multiple diseases that-, that are used to describe even a single patient. And those diagnoses may change, but that burden disease that, uh, that-, that Kirsten and Karin described as being pervasive or constant, you know, but paradoxically, unpredictable. That's still remains and that's-, that's where-, that's where our research needs to go.


So, you know, how do we continue this network? Everybody around a table, you know, is there; probably with people you didn't know at the beginning of the day. We hope you'll be continuing through the Juvenile Arthritis Conference, we hope that you'll take part in some of the, uh, some of the tools that the Arthritis Foundation, and CARRA, and other partners are creating. For instance, the Arthritis Foundation's Live Yes network, which offers a number of volunteer opportunities.

We talked, uh, there was a mention earlier about ClinicalTrials.gov. We're about to launch, in the next week or, uh, sorry, next month or so, a-, a rescanning[?] of the data from ClinicalTrials.gov that makes it a little easier to find rheumatic disease clinical studies. That'll be on the Arthritis Foundation website.

But, you know, more importantly than anything else, we just-, we're here to remember now after-, after the conversations today, the patients have so much to offer in terms of clear opportunities to engage in the recruitment and design of clinical trials, consideration of the ethics, the wealth of research that's necessary to understand the unpredictability of the, uh, of the disease. But we-, but if addressed, then that would translate so powerfully to the-, to the benefit side of that risk benefit equation. And on the acceptance of risk side of that equation we've had such a nuanced conversation today about the context under which risk might be acceptable to a patient population.

So I'd, um, wanna take moment, certainly, again to-, to thank everybody, but particularly call out a few people in the room – the planning committee that really made this day possible. You know, in particular our-, our inimitable, you know, irreplicable, emcee, uh, Suz Schrandt.

[applause] [02:44:38]



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So what I'd like to do is . . . I-, I've tried to summarize for both of us based on our conversations. Laura, would you have anything else you-, you'd care to add?

Dr. Schanberg: Mention something about partners.

Dr. Guy Eakin: Oh, [chuckle] [02:44:54]. Okay, so early, early in the day, you might have heard this, uh, this word, "the partners network." So this is a wonderful consortium of many of the different disease foundations and academic researchers – CARRA, PR-Coin, [indiscernible] [02:45:07], Lupus Foundation of American, all working together to associate patients with, uh, with research opportunities, and also developing, uh, uh, a network of, uh, of opportunities for creating for what's called "a learning health system." So if you're here for the rest of the JA conference, you'll hear more about that.

Um, but, you know, any other questions. And we're gonna run out of time here to describe it more fully, but certainly I wanna invite anyone who has lingering questions to never hesitate to reach out to us at, uh, at any, you know, any way you can get in touch with the arthritis foundation, but certainly in the science department at AFscience@arthritis.org.

So with that I just wanna say thank you, and say that we're on the cusp of what, you know, if you're staying, this is that wonderful moment where it's the calm before the storm. These soft walls are gonna disappear, this room is gonna get even bigger and there's gonna be, you know, 800-somethin' families here networking together, learning from each other, and really, most importantly, educating this whole network about how to ultimately cure, conquer, put a stake in the heart of this disease.

[applause] [02:46:20]

[exit music] [02:46:27]

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