

A background image showing several orange, round pills scattered on a white surface. One pill is in sharp focus in the lower right, while others are blurred in the background.

Public Meeting on Patient-Focused Drug Development for Psoriasis

March 17, 2016



Welcome

Sara Eggers, PhD

Office of Strategic Programs

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

March 17, 2016

Agenda

- **Setting the context**
 - Overview of FDA's Patient-Focused Drug Development
 - Background on Psoriasis and Therapeutic Options
 - Road from PFDD Meetings to Clinical Trial Endpoints
 - Overview of Discussion Format
- **Discussion Topic 1:** Disease symptoms and daily impacts that matter most to patients
- **Lunch**
- **Discussion Topic 2:** Patients' perspectives on current approaches to treatment
- **Open Public Comment**
- **Closing Remarks**

A background image showing a cluster of orange, round pills in the upper left corner, with a single pill in sharp focus in the lower right corner. The pills are set against a white background with a blue curved border at the top.

Opening Remarks

Kendall Marcus, MD

Director, Division of Dermatology and Dental Products (DDDP)
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

March 17, 2016



FDA's Patient-Focused Drug Development Initiative

Theresa Mullin, PhD

Director, Office of Strategic Programs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

March 17, 2016

Patient-Focused Drug Development under PDUFA V

- **FDA is developing a more systematic way of gathering patient perspective on their condition and available treatment options**
 - Patient perspective helps inform our understanding of the context for the assessment of benefit-risk and decision making for new drugs
 - Input can inform FDA's oversight both during drug development and during our review of a marketing application
- **Patient-Focused Drug Development is part of FDA commitments under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V)**
 - FDA will convene at least 20 meetings on specific disease areas in FY 2013-2017
 - Meetings will help develop a systematic approach to gathering patient input

Identifying Disease Areas for the Patient-Focused Meetings

- **FDA announced a preliminary set of diseases as potential meeting candidates**
 - Public input on these nominations was collected. FDA carefully considered these public comments and the perspectives of our drug review divisions at FDA
- **FDA identified a total of 24 diseases to be the focus of meetings for fiscal years 2013-2017**

Disease Areas to be the focus of meetings for FY 2013-2017

Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Year 2016-2017
<ul style="list-style-type: none"> • Chronic fatigue syndrome/ myalgic encephalomyelitis • HIV • Lung cancer • Narcolepsy 	<ul style="list-style-type: none"> • Sickle cell disease • Fibromyalgia • Pulmonary arterial hypertension • Inborn errors of metabolism • Hemophilia A, B, and other heritable bleeding disorders • Idiopathic pulmonary fibrosis 	<ul style="list-style-type: none"> • Female sexual dysfunction • Breast cancer • Chagas disease • Functional gastrointestinal disorders • Huntington’s disease and Parkinson’s disease • Alpha-1 antitrypsin deficiency 	<ul style="list-style-type: none"> • Non-tuberculous mycobacterial lung infections • Psoriasis • Neuropathic pain associated with peripheral neuropathy (June 10) <p><i>To be announced</i></p> <ul style="list-style-type: none"> • Alopecia areata • Autism • Hereditary angioedema • Patients who have received an organ transplant • Sarcopenia

Tailoring Each Patient-Focused Meeting

- **Each meeting focuses on a set of questions that aim to elicit patients' perspectives on their disease and on treatment approaches**
 - We start with a set of questions that could apply to any disease area; these questions are taken from FDA's benefit-risk framework and represent important considerations in our decision-making
 - We then further tailor the questions to the disease area of the meeting (e.g., current state of drug development, specific interests of the FDA review division, and the needs of the patient population)
- **Focus on relevant current topics in drug development for the disease at each meeting**
- **We've learned that active patient involvement and participation is key to the success of these meetings.**

“Voice of the Patient” Reports

- Following each meeting, FDA publishes a Voice of the Patient report that summarizes the patient testimony at the meeting, perspectives shared in written docket comments, as well as any unique views provided by those who joined the meeting webcast.
- These reports serve an important function in communicating to both FDA review staff and the regulated industry what improvements patients would most like to see in their daily life.
- FDA believes that the long run impact of this program will be a better, more informed understanding of how we might find ways to develop new treatments for these diseases.



An Overview of Psoriasis

Jane Liedtka, MD

Division of Dermatology and Dental Products (DDDP)

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

March 17, 2016

Epidemiology of Psoriasis

- Psoriasis is a common, chronic, inflammatory, multi-system disease
- Predominantly skin and joint manifestations
- Affects \approx 2-3 % of the U.S. population

Clinical Presentation



- Areas of red, thickened, scaling skin - itchy or sore
- Joint involvement with arthritis in $\approx 1/3$ of patients

Clinical Presentation



Clinical Presentation



Treatment Options

- **Topicals**
 - Corticosteroids
 - Retinoids
 - Vitamin D analogs
- **Phototherapy**
 - UVB – narrowband, broadband
 - PUVA

Treatment Options

Systemic Agents – Approved Products

Treatment	Target	Approval
<i>Traditional Drugs</i>		
Methotrexate	Folic acid inhibitor	1972
Acitretin	Retinoic acid receptor	1996
Cyclosporine	T cell inhibitor	1997
Apremilast	PDE4 inhibitor	2014
<i>Biologics</i>		
Etanercept	TNF-alpha inhibitor	2004
Infliximab	TNF-alpha inhibitor	2006
Adalimumab	TNF-alpha inhibitor	2008
Ustekinumab	IL-12/IL-23 inhibitor	2009
Secukinumab	IL-17 inhibitor	2015

Impact of Psoriasis on the Quality of Life

- Social, psychological and economic impacts
- Impact for patients with severe psoriasis is comparable with that observed in diabetes and depression¹

¹Sampogna F, et.al. Italian Multipurpose Psoriasis Research on Vital Experiences (Improve) Study Group. Age, gender, quality of life and psychological distress in patients hospitalized with psoriasis. Br J Dermatol. 2006;154(2):325-331.

National Psoriasis Foundation Survey

Conducted 2003-2011– 811 respondents²

- 60% say psoriasis impacts their self-esteem and emotional well-being
- More than two-thirds avoid social activities, including dating and intimacy
- 51% of patients state that they are un- or undertreated, the top two reasons being fear of side effects and cost or perceived cost of therapy.

²Armstrong, AW et.al. Under treatment, treatment trends, and treatment dissatisfaction among patients with psoriasis and psoriatic arthritis in the United States: findings from the National Psoriasis Foundation surveys, 2003-2011. JAMA Dermatol. 2013 Oct;149(10):1180-5.

- The FDA is aware of unmet medical needs experienced by patients who have psoriasis.
- FDA is conducting this public meeting to hear comments about the impact of psoriasis on patients, caregivers and family members.
- Thank you for taking the time, and making the trip to share your comments with us today.



The Road from PFDD Meetings to Clinical Trial Endpoints

Yasmin Choudhry, MD

Clinical Outcomes Assessment (COA), Office of New Drugs (OND)

Center for Drug Evaluation and Research

U.S. Food and Drug Administration

March 17, 2016

Disclaimer

The views expressed in this presentation are those of the speaker, and do not necessarily represent an official FDA position.

PATIENT-FOCUSED DRUG DEVELOPMENT (PFDD) MEETINGS



WHERE DO WE GO FROM HERE



PATIENTS' VOICE





Two Pathways for FDA Clinical Outcome Assessment Review & Advice

1

Within an individual drug development program

- Investigational New Drug (IND) submissions to FDA
- Potential to result in labeling claims

2

Within the Drug Development Tool (DDT) qualification program; outside of an individual drug development program

- Potential to result in qualification*

Key Takeaways

- PFDD meetings are a “starting point” for developing & using patient-focused outcome measures and endpoints
- The outcomes of PFDD meetings will support and guide FDA’s assessment of clinical benefit in drug reviews
- Patients’ input ultimately helps determine:
 - WHAT is measured to provide evidence of treatment benefit
 - HOW best to measure what matters most to patients
 - WHAT amount of change is meaningful to patients



Overview of Discussion Format

Sara Eggers

Office of Strategic Programs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

March 17, 2016

Discussion Overview

Topic 1: The symptoms that matter most to you

- Which symptoms have the most significant impact on your life?
- How do these symptoms affect your ability to do specific activities?
- How your symptoms affect you on the best days? Worst day?
- How have your symptoms changed?

Topic 2: Current approaches to treatment

- What are you doing to treat your psoriasis?
- How well is/are the treatment(s) treating your significant symptoms?
- What are the biggest downsides to your treatments?
- What would you look for in an “ideal” treatment?
- What factors do you consider when selecting a course of treatment?

Discussion Format

- **We will first hear from a panel of patients**
 - The purpose is to set a good foundation for our discussion
 - They reflect a range of experiences with psoriasis

- **We will then broaden the dialogue to include patients in the audience**
 - The purpose is to build on the experiences shared by the panel
 - We will ask questions and invite you to raise your hand to respond
 - Please state your name before answering

Discussion Format, continued

- **You'll have a chance to answer "polling" questions**
 - Their purpose is to aid our discussion
 - In-person participants, use the "clickers" to respond
 - Web participants, answer the questions through the webcast
 - Patients or parents of patients only, please
- **Web participants can add comments through the webcast**
 - Although they may not all be read or summarized today, your comments will be incorporated into our summary report
 - We'll occasionally go to the phones to give you another opportunity to contribute

Send us your comments!

- **You can send us comments through the “public docket”**
 - The docket will be open until May 17, 2016
 - Share your experience, or expand upon something discussed today
 - Comments will be incorporated into our summary report
 - Anyone is welcome to comment

Visit:

<http://www.regulations.gov/#!/docketDetail;rpp=100;so=DESC;sb=docId;po=0;D=FDA-2015-N-4166>

Click Comment Now!

The screenshot shows the 'regulations.gov' website interface. The main heading is 'Public Meeting on Patient-Focused Drug Development for Psoriasis'. Below this, there is a 'Docket Folder Summary' section with a 'View all documents and comments in this Docket' link. The docket ID is FDA-2015-N-4166, and the agency is the Food and Drug Administration (FDA). A 'Comment Now' button is prominently displayed next to the document title. A red arrow points from the text 'Click Comment Now!' to this button. On the right side, there is a 'Comments Received' counter showing '6' and a 'Sign up for Email Alerts' button. A footer note states: '*This count refers to the total comment/submissions received on this docket, as of 11:59 PM yesterday. Note: Agencies review all submissions, however some agencies may choose to redact, or withhold, certain submissions (or portions thereof) such as those containing private or proprietary information.'

Resources at FDA

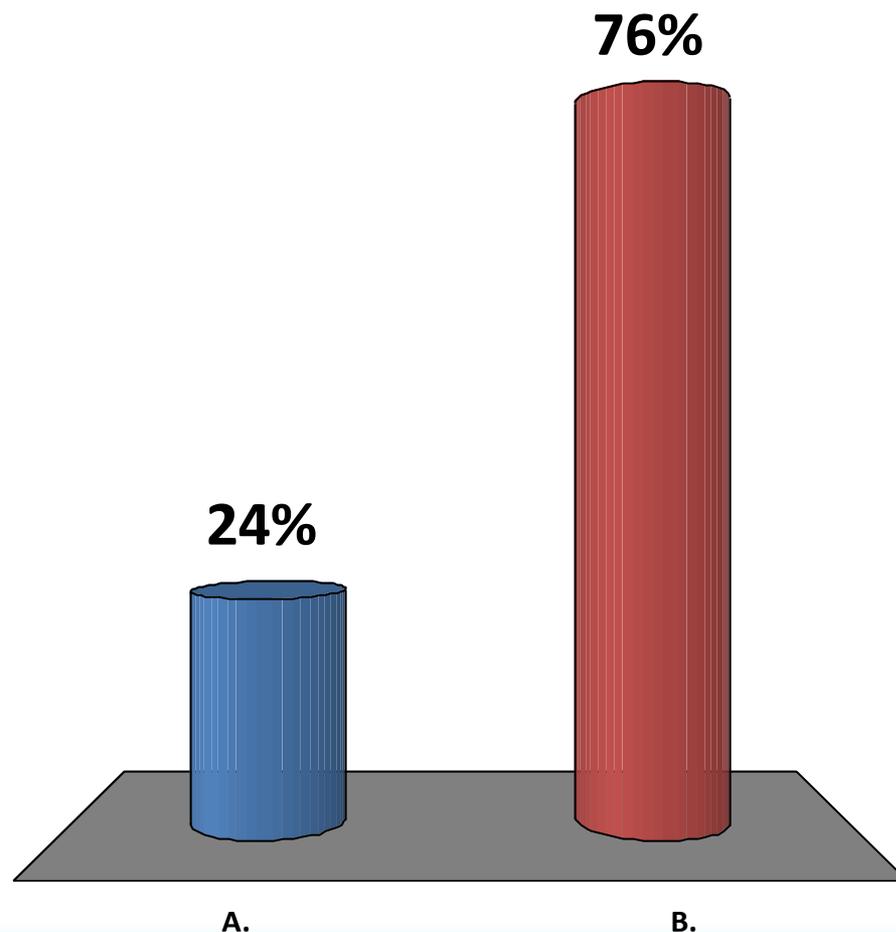
- FDA Office of Health and Constituent Affairs
 - Contact: PatientNetwork@fda.hhs.gov, (301) 796-8460
 - Liaison between FDA and stakeholder organizations
 - Runs the Patient Representative Program
 - Patient Representatives advise FDA at Advisory Committee meetings
- CDER Office of Center Director
 - Professional Affairs and Stakeholder Engagement (PASE)
 - Contact: Christopher Melton, christopher.melton@fda.hhs.gov
 - Facilitates communication and collaboration between CDER and patient and healthcare professional stakeholders and others on issues concerning drug development, drug review and drug safety.

Discussion Ground Rules

- We encourage patients to contribute to the dialogue—caregivers and advocates are welcome too
- FDA is here to listen
- Discussion will focus on symptoms and treatments
 - Open Public Comment Period is available to comment on other topics
- The views expressed today are personal opinions
- Respect for one another is paramount
- Let us know how the meeting went today; evaluation forms are available at the registration table

Where do you live?

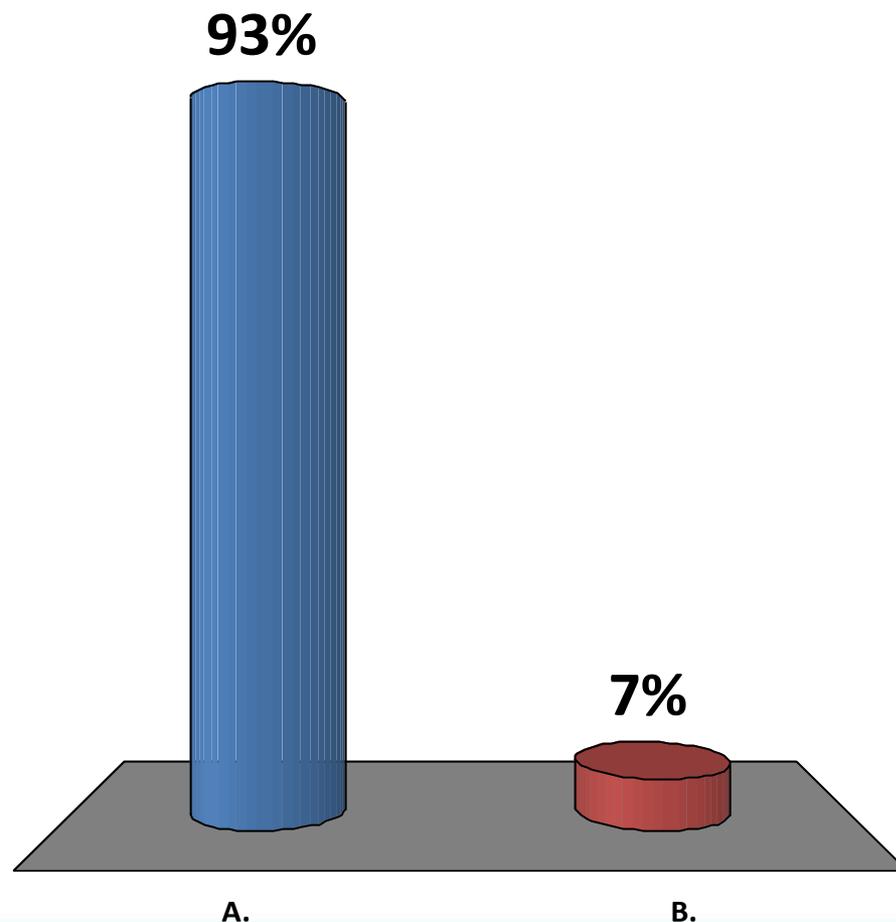
- A. Within Washington, DC metropolitan area (including the Virginia and Maryland suburbs)
- B. Outside of the Washington, D.C. metropolitan area



Have you ever been diagnosed as having psoriasis?

A. Yes

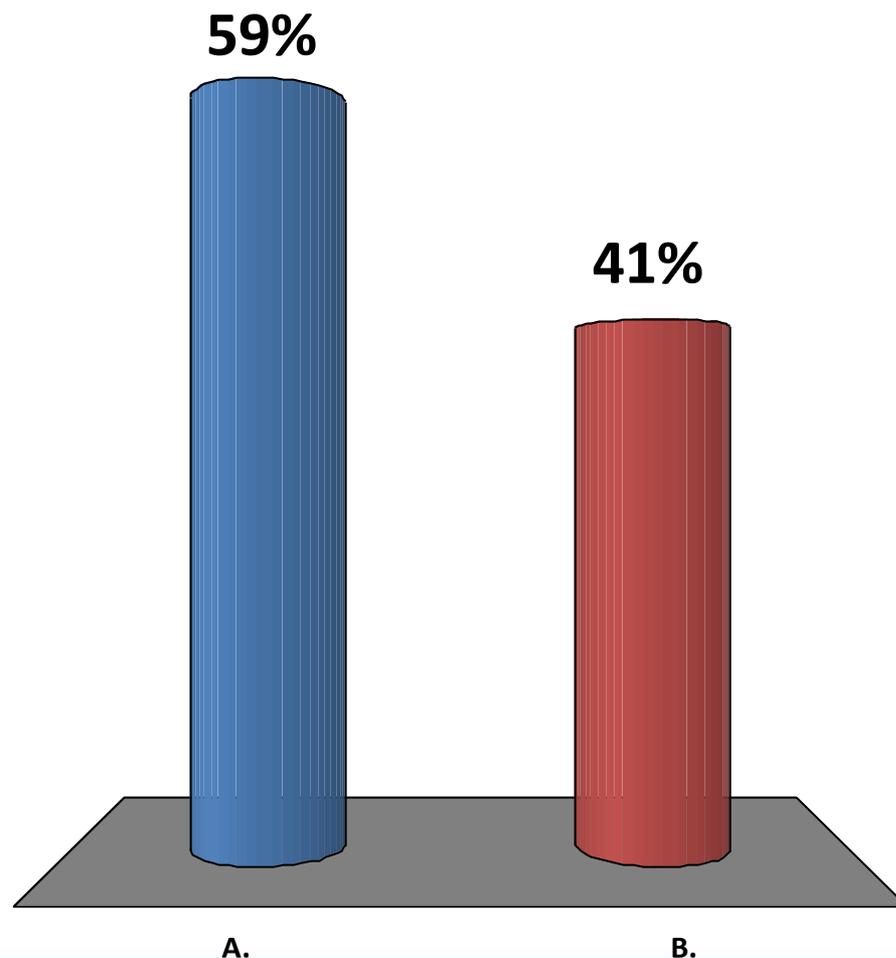
B. No



Have you ever been diagnosed with psoriatic arthritis?

A. Yes

B. No



Age?

A. Younger than 18

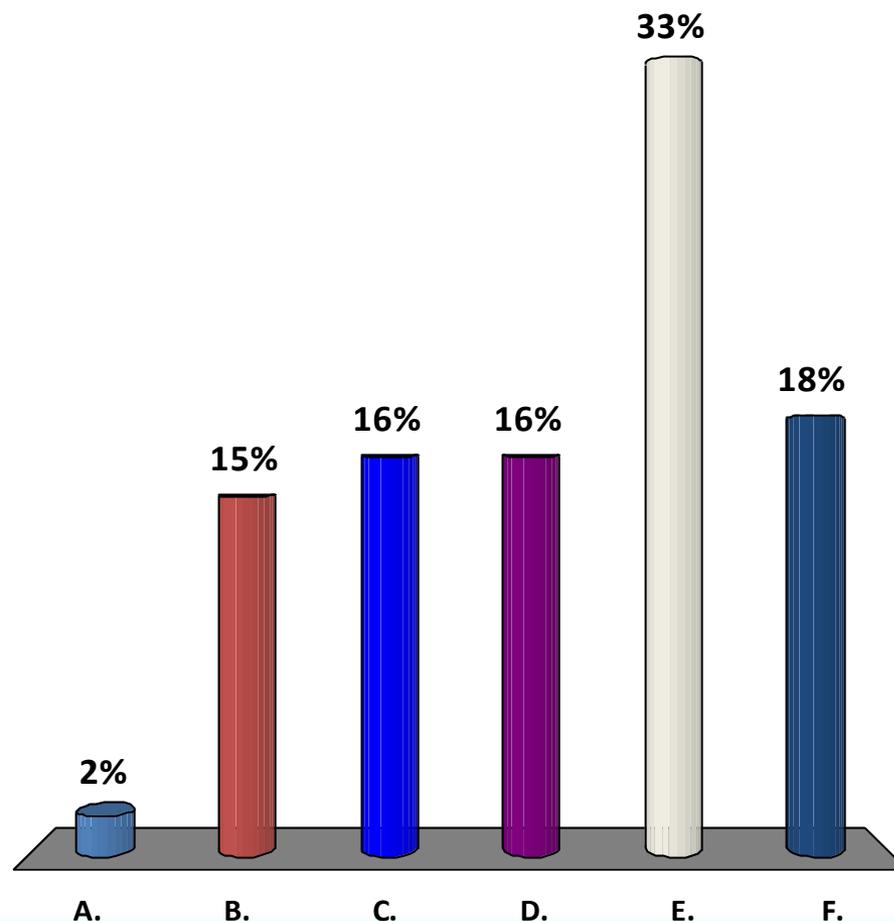
B. 18 – 29

C. 30 – 39

D. 40 – 49

E. 50 – 59

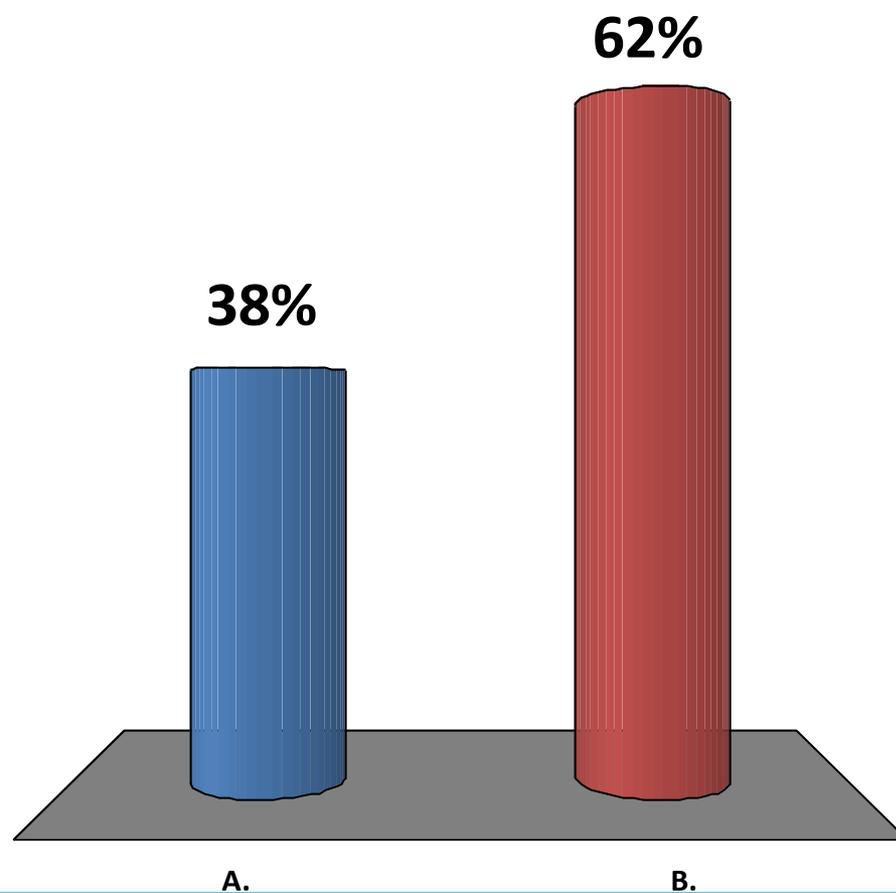
F. 60 or greater



Do you identify as:

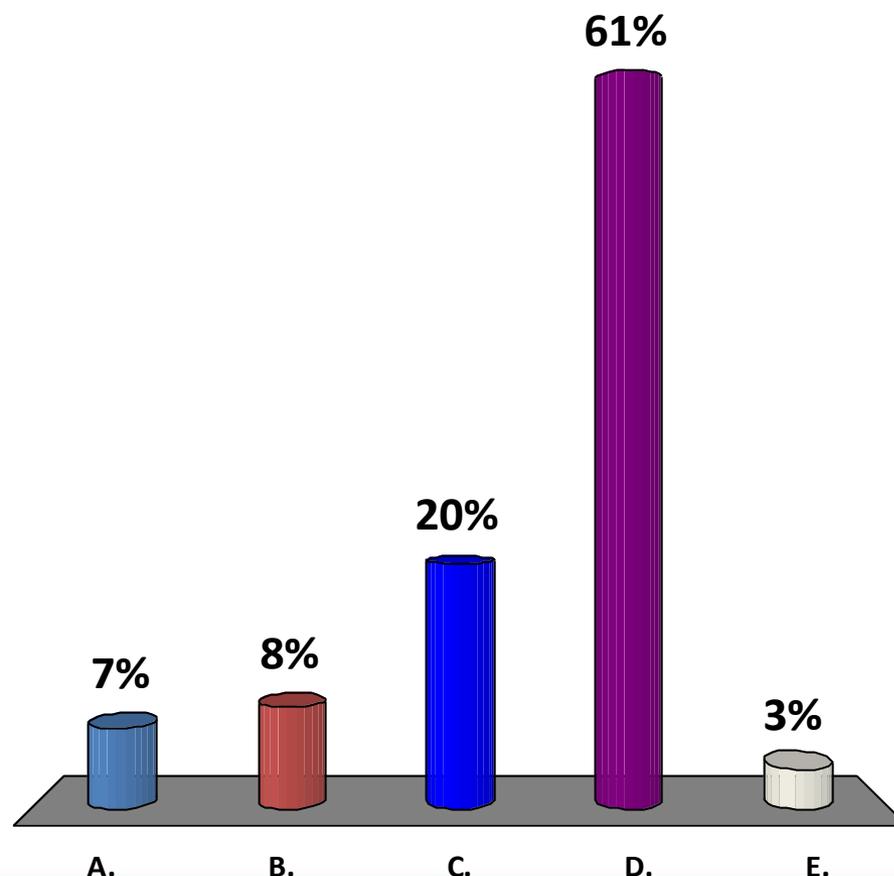
A. Male

B. Female



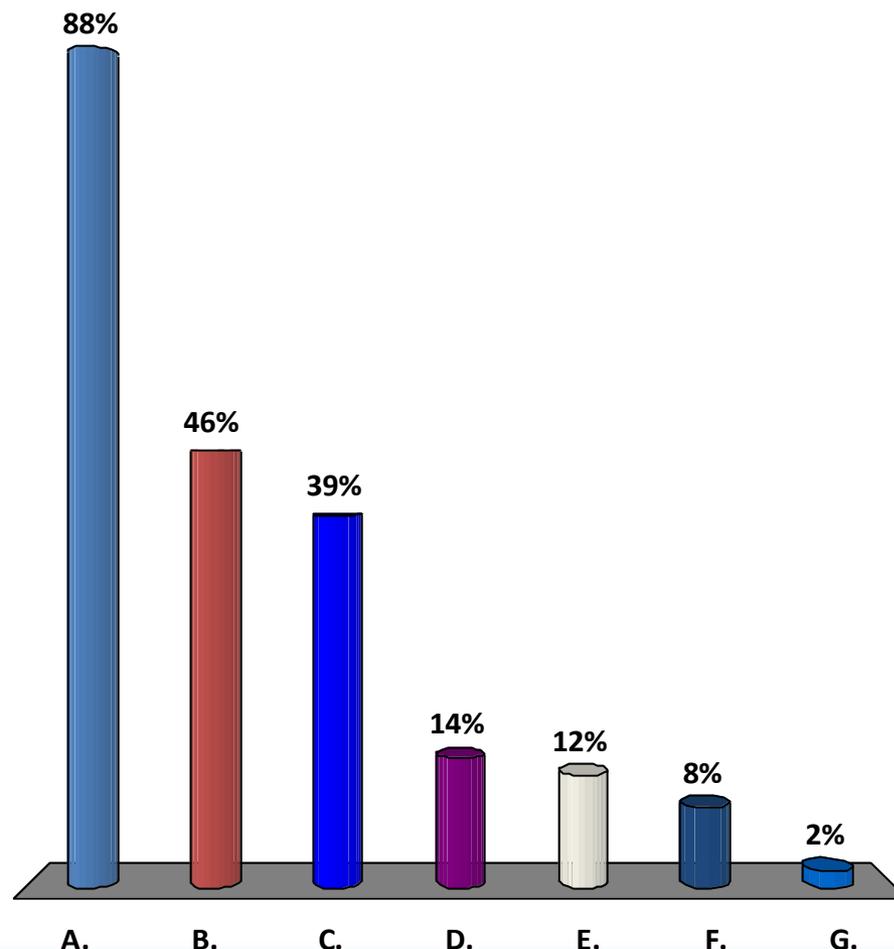
What is the length of time since your psoriasis diagnosis?

- A. Less than 5 years ago
- B. 5 years ago to 10 years ago
- C. 10 years ago to 20 years ago
- D. More than 20 years ago
- E. I'm not sure



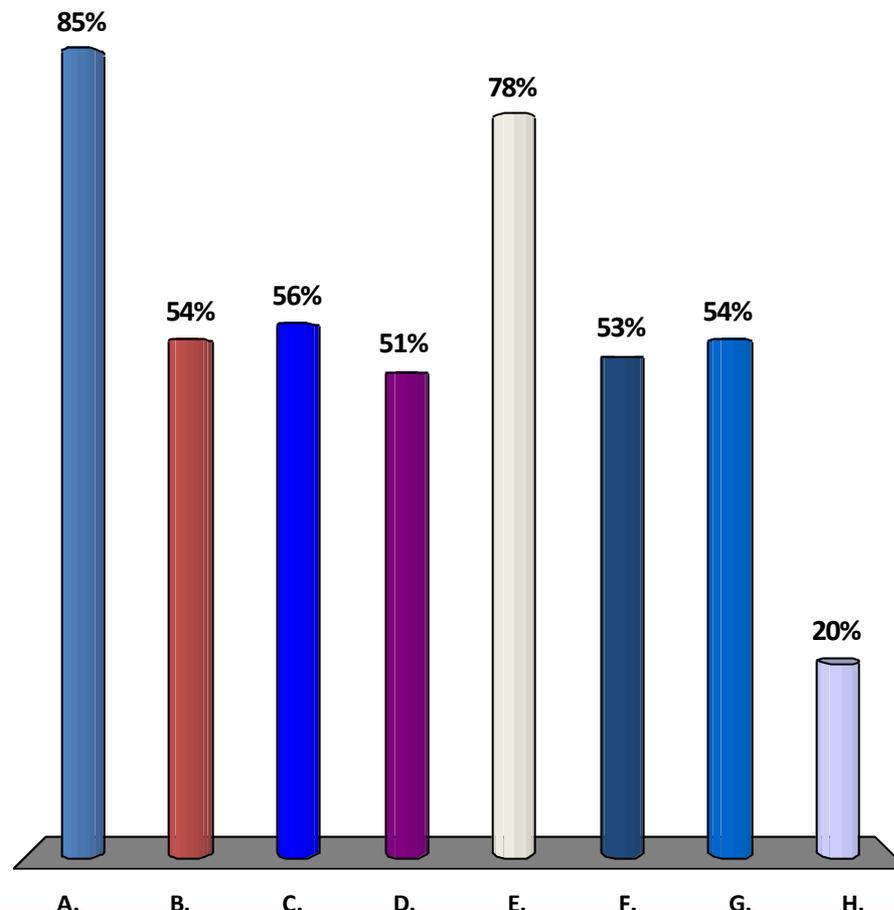
What type of psoriasis do you have? Check all that apply.

- A. Plaque (such as raised, red patches covered with silvery white flaky buildup)
- B. Guttate (such as small, dot-like lesions)
- C. Inverse (such as very red lesions in body folds)
- D. Pustular (such as white pustules surrounded by red skin)
- E. Erythrodermic (such as widespread, fiery redness and flaking over most of the body)
- F. Other
- G. I'm not sure



Where is your psoriasis located? Check all that apply.

- A. Scalp
- B. Face
- C. Hands or feet
- D. Nails
- E. Trunk, arms or legs
- F. Skin folds (such as armpit, fold between buttocks)
- G. Genital area
- H. Other skin areas not mentioned





Discussion Topic 1

Disease symptoms and daily impacts that matter most to patients

Sara Eggers

Facilitator

Topic 1 Panel Participants

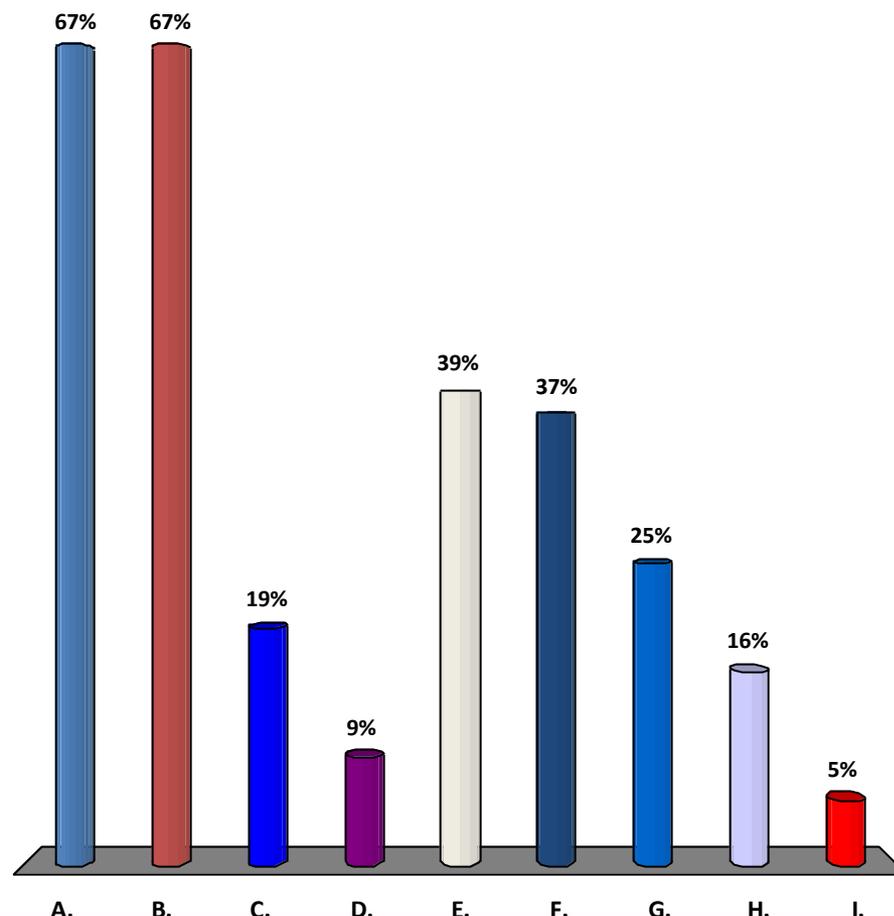
- Ellen Clements
- Marie Griffiths
- John Latella
- Diane Talbert
- Christina Tom

Topic 1 Discussion: Disease symptoms and daily impacts that matter most to patients

- Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
- Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition?
- How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days?
- How has your condition and its symptoms changed over time?
- What worries you most about your condition?

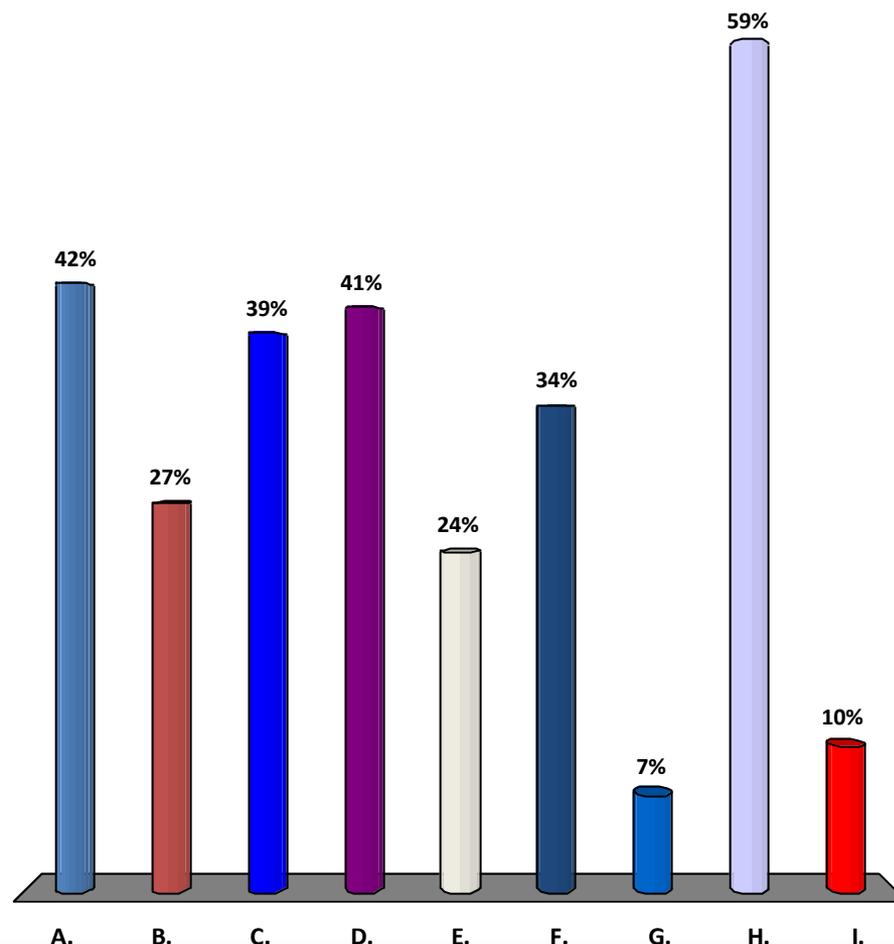
Of all the non-joint symptoms you have experienced because of psoriasis, which do you consider to have the most significant impact on your daily life? **Please choose up to three symptoms.**

- A. Flaking or scaling
- B. Itching
- C. Skin rash
- D. Skin tightness
- E. Dry, cracked skin that may bleed
- F. Pain or soreness
- G. Burning or stinging
- H. Thickened, spotted, pitted or ridged nails
- I. Other symptoms not mentioned



What do you find to be the most bothersome impacts of your psoriatic symptoms on your daily life? **Please choose up to three impacts.**

- A. Limitations on activities (such as work, school, sports, hobbies)
- B. Ability to concentrate or stay focused
- C. Ability to fall asleep or stay asleep
- D. Stigma or embarrassment
- E. Social discrimination
- F. Impact on sexual intimacy
- G. Impact on relationships
- H. Emotional impacts (such as self-esteem)
- I. Other impacts not mentioned





LUNCH



Discussion Topic 2

Patients' perspectives on current approaches to treatment

Sara Eggers

Facilitator

Topic 2 Panel Participants

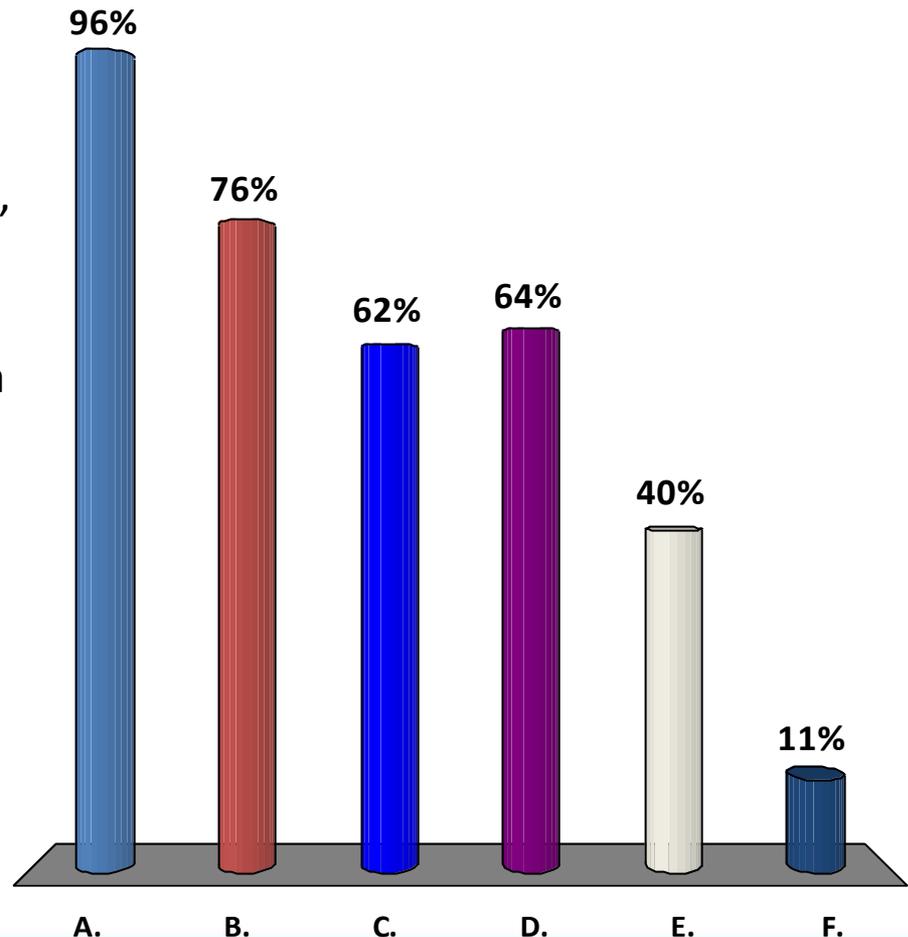
- John Earley
- Pantshia Gardner
- Caleb Sexton
- Sarah Truman
- Aimes Vasquez

Topic 2 Discussion: Patients' perspectives on current approaches to treatment

- What are you currently doing to help treat your condition or its symptoms?
- How well does your current treatment regimen control your condition?
- What are the most significant downsides to your current treatments, and how do they affect your daily life?
- Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?
- What factors do you take into account when making decisions about selecting a course of treatment?

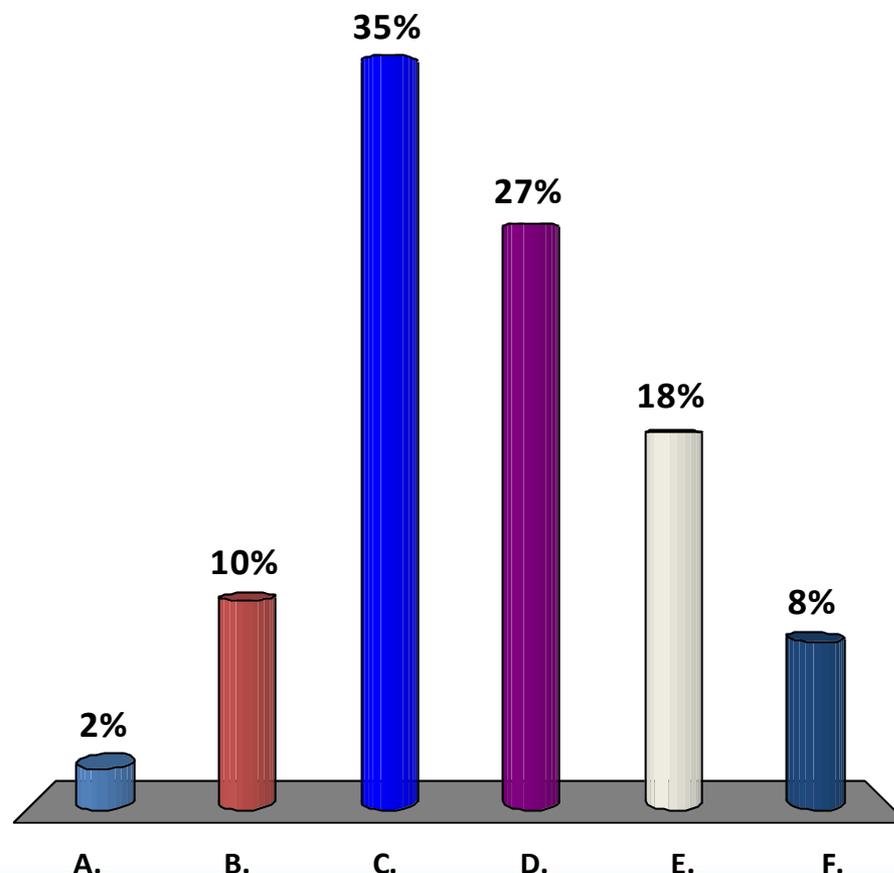
Have you ever used any of the following medical products (drug therapies or medical devices) to help treat your psoriasis? **Check all that apply.**

- A. Topical treatments (such as corticosteroids, retinoids, vitamin D analogue, anthralin, calcineurin inhibitors, salicylic acid)
- B. Oral or injected medications (such as Methotrexate, Enbrel (etanercept), Humira (adalimumab) Stelara (ustekinumab), Cosentyx (secukinumab), cyclosporine, acetretin, methoxypsoralen)
- C. Other prescription medicine (such as pain medication)
- D. Phototherapy
- E. Other medical products not mentioned
- F. I'm not using any medical products



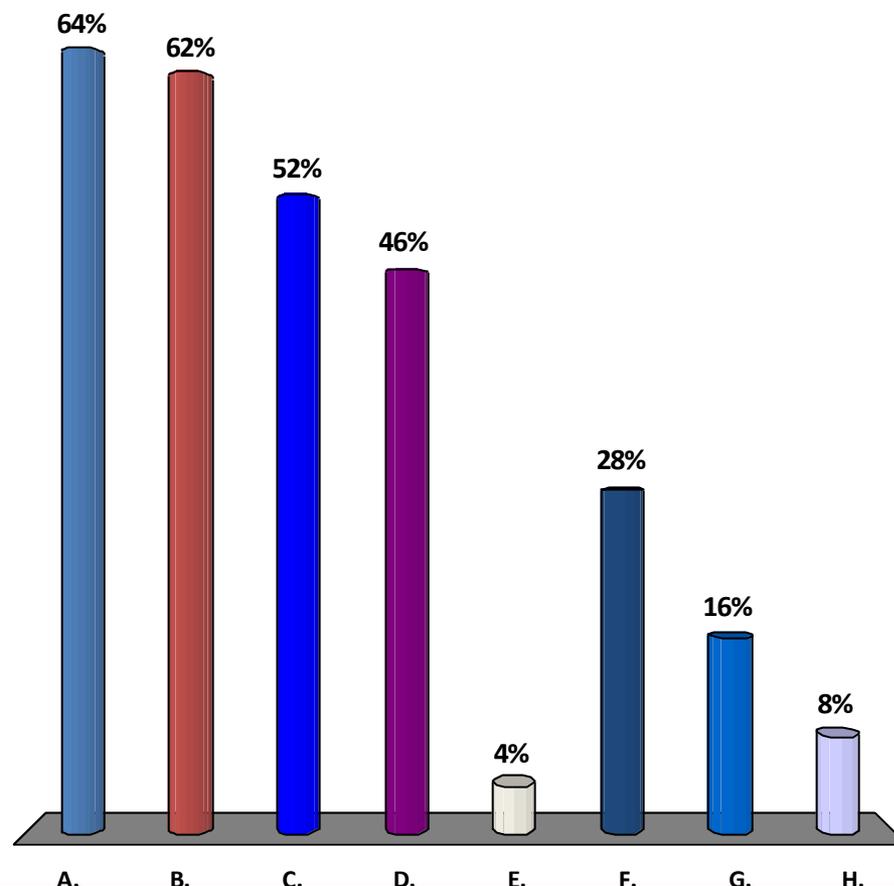
Besides your drug therapies, what else are you doing to manage any symptoms you have experienced because of your psoriasis? **Check all that apply.**

- A. Dietary and herbal supplements
- B. Diet modifications
- C. Over the counter products (such as coal tar, salicylic acid, epsom salt)
- D. Complementary or alternative therapies
- E. Other therapies not mentioned
- F. I am not doing or taking any therapies to treat symptoms



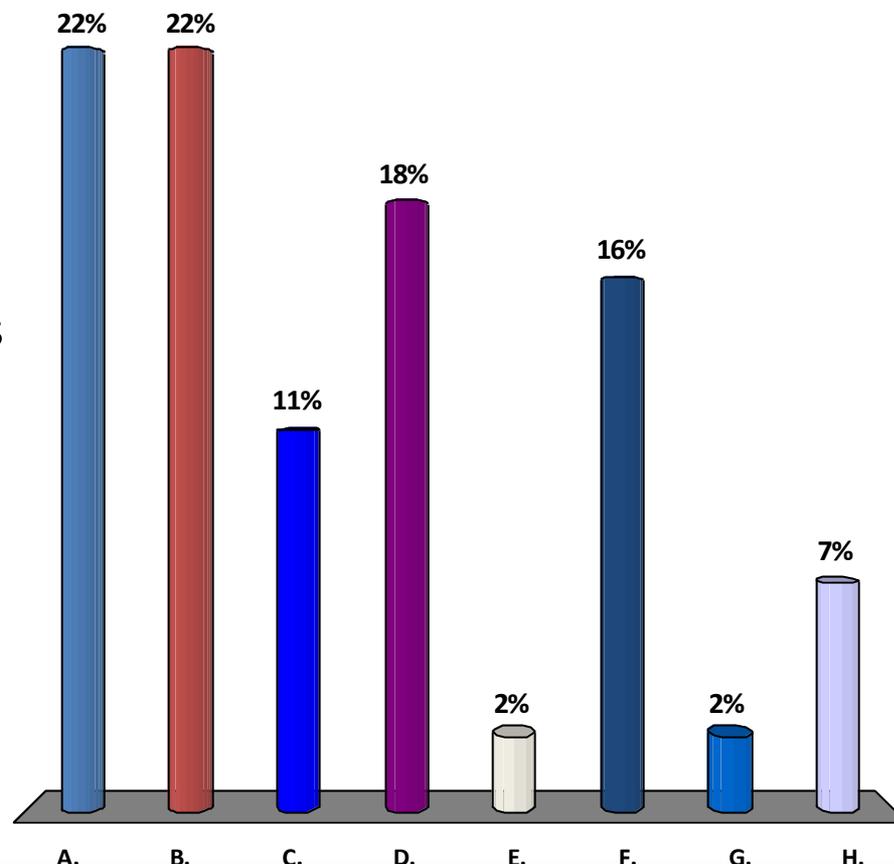
When considering a new treatment for psoriasis, which of the following benefits would you consider to be the most meaningful?
Please choose up to three.

- A. Reduced scaling or flaking
- B. Reduced itching
- C. Reduced redness and inflammation
- D. Reduction in the number of plaques
- E. Reduction in the number of pustules
- F. Reduced pain or soreness
- G. Reduced burning or stinging
- H. Other



When considering a new treatment for psoriasis, which ONE benefit would you consider to be the most meaningful? **Please choose one.**

- A. Reduced scaling or flaking
- B. Reduced itching
- C. Reduced redness and inflammation
- D. Reduction in the number of plaques
- E. Reduction in the number of pustules
- F. Reduced pain or soreness
- G. Reduced burning or stinging
- H. Other



Scenario

Imagine that a new injectable medication indicated to treat moderate to severe psoriasis has recently been approved by FDA. Your doctor believes that you may be a good candidate for this medication.

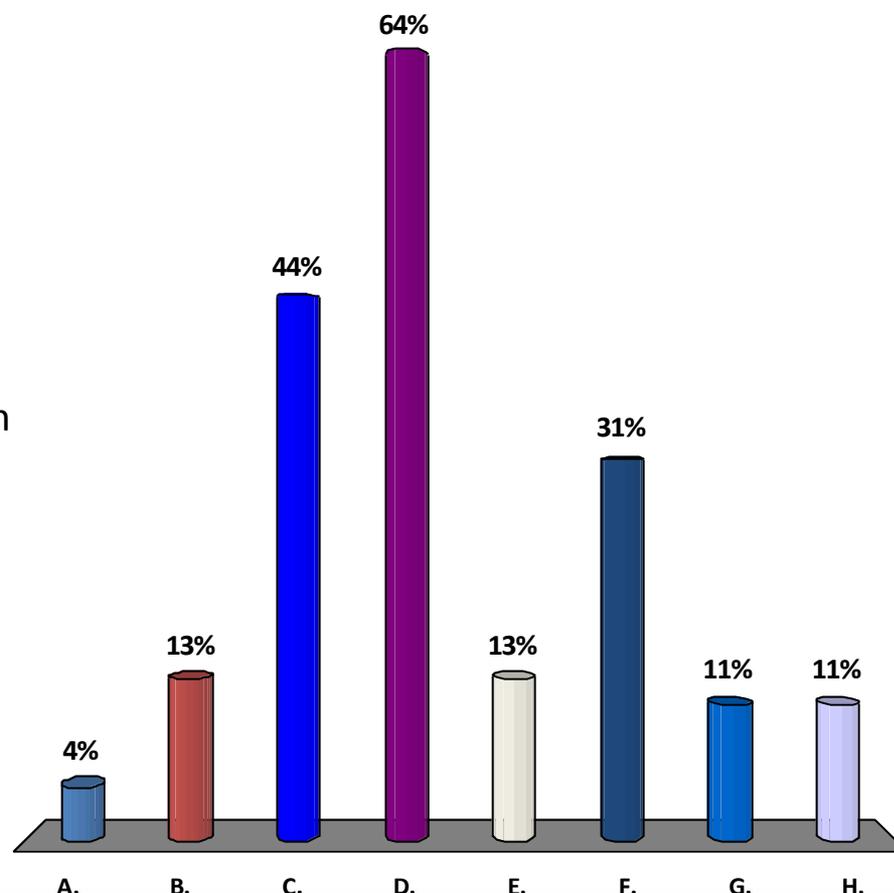
In the clinical trials that were conducted, the medication was shown to achieve at least 75% clearance of skin lesions in approximately one-half of adults treated for 3 months.

Common side effects of this medication include fatigue, headaches, weight gain, swelling, sore throat, diarrhea, and upper respiratory tract infection. The drug is also believed to cause rare, but serious side effects, such as liver problems, cancer, stroke, infertility, or birth defects.

What first thoughts come to mind as you hear this scenario? What questions would you ask your doctor about this treatment?

Of the following factors, which two would you rank as most important to your decisions about using treatments to help reduce or control the spread of your psoriasis? **Please choose two.**

- A. How the medication is administered (such as topical, injection, oral, etc.)
- B. The frequency and length of treatment
- C. Your access to treatment (such as insurance coverage)
- D. Whether the drug showed effectiveness for the specific benefit that is most meaningful to you
- E. The common side effects of the treatment (such as risk of infection, fatigue, etc.)
- F. The possibility of rare, but serious side effects (such as blood disorders or certain cancers)
- G. Your previous response to a similar treatment
- H. Whether there are other treatment options that you can still try



A background image showing a cluster of orange, round pills in the upper left corner, with a single orange pill in sharp focus on the right side of the slide. The pills are set against a white background with soft shadows.

Open Public Comment Period

Pujita Vaidya, MPH

Office of Strategic Programs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

March 17, 2016

A background image showing a cluster of orange, round pills in the upper left corner, with a single pill in sharp focus in the lower right corner. The pills are set against a white background with soft shadows.

Closing Remarks

Kendall Marcus, MD

Director, Division of Dermatology and Dental Products (DDDP)
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

March 17, 2016