SUMMARY

VHL ALLIANCE FDA PATIENT LISTENING SESSION

June 11, 2020

VHL Alliance
FDA Listening Session
June 11, 2020

Contents

Overview

A FDA Listening Session with the von Hippel Lindau Alliance (VHLA) was held on June 11, 2020 via video conference. Patient Listening Sessions are intended to be a resource for the medical product Centers to expeditiously engage with patients and their advocates with the goal of hearing directly from patients regarding their disease experience and to gain further appreciation of the patient perspective. This Session, to focus on von Hippel Lindau (VHL) was attended by staff from the FDA Centers for Drug, Biologics, and Devices, Office of the Commissioner as well as staff from the Reagan-Udall Foundation for the FDA and NIH, National Center for Advancing Translational Sciences (see page 13).

Discussion in FDA Listening Sessions are informal and not meant to replace, but rather compliment, existing patient engagement opportunities in the Agency. All opinions, recommendations, and proposals are unofficial and nonbinding on FDA and all other participants. This report summarizes the input provided by patients and patient manifestations of VHL, and the health effects and impacts reflect those of the participants. This report is not meant to be representative of the views and experience of the entire VHL patient population as the disease course is so heterogeneous.

Welcome and Opening Remarks

ANDREA FURIA-HELMS, MPH
Director of Patient Affairs Staff, Office of the Commissioner, FDA

Welcomed the group and expressed the FDA's appreciation for VHLA's Patient Listening Session. Ms. Furia-Helms highlighted the honor of Deputy Commissioner.

ANAND SHAH, MD
Deputy Commissioner for Medical and Scientific Affairs, Office of the Commissioner

Dr. Shah welcomed the group and noted the appreciation of the Medical Affairs group and VHLA for holding this session noting the criticality of these times with the COVID-19 pandemic. He expressed dedication to both COVID-19 and the simultaneous on-going work with critical diseases. Dr. Shah also encouraged VHLA to continue advocacy.

Dr. Shah highlighted that the FDA Listening Sessions are one of the many avenues that allow patients and advocates to communicate with the FDA, thus continuing a 40-year history of engagement begun during the AIDS Crisis. The FDA wants seeks the invaluable feedback from patients so that direct input can inform the advancement of treatments and cures, noting that "the more we can understand the needs of patients, we can develop most effective treatments."

SUSAN CHITTOORAN, MSW
Patient Affairs Staff, Office of the Commissioner, FDA

Ms Chittooran welcomed the group and thanked everyone for participating. She noted that meetings are non-regulating and non-binding, and that the FDA may not be able to address all questions during the session.

Von Hippel Lindau (VHL) Overview

GENETIC RARE DISEASE AND MANIFESTATION

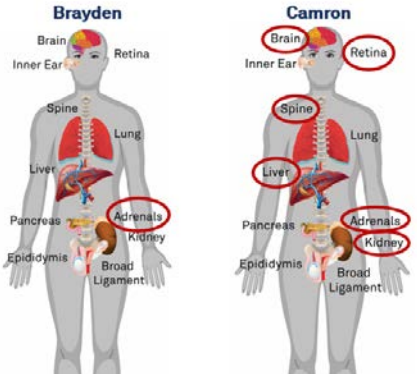
- VHL is by in large an inherited rare disorder. It is an autosomal dominant disease and about 20% of people with VHL have a de novo mutation. Estimates are that 1 in 36,000 people have VHL, with approximately 10,000 individuals living with VHL disease in the United States.
- The von Hippel-Lindau (VHL) gene, commonly referred to as a tumor suppressor gene, is located on chromosome 3p25, spans approximately 12.6kb of genomic DNA, and has three exons. The ubiquitous presence in species as small as Drosophila demonstrates the importance of the gene. Despite its small size, more than 15,000 gene mutations have been described, causing its 213 amino acid long protein product to be either dysfunctional or absent.
- Manifestation of the disease is in malignant and non-malignant tumors throughout the body including presentation in the: brain, spine, kidney, pancreas, adrenals, retina, endolymphatic sac, broad ligament, epididymis, liver and lung. Multiple lesions in any given organ are common.
- To date, surgery has been the primary intervention. Treatment through a HIF2 inhibitor is currently in clinical trials for which we are cautiously optimistic.

IMPACT OF VHL:

- Until an effective treatment is available, regular surveillance for tumor growth is the patient's strongest defense to prevent severe VHL complications.
- VHL manifestations occur throughout a lifespan requiring continual medical surveillance and imaging. The ritual of scans and tests is often followed by the news that yet another surgery is needed given the proliferation of the disease.
- Tumor growth as well as surgical interventions take a great toll on the human body which can lead circumstances including though not limited to partial or full loss of organs, need for dialysis, neurological damage, limited mobility and in some cases loss of independence, pancreatic insufficiency to the extent of nutritional impairment, loss of vision including blindness, hormonal insufficiency, loss of hearing to the point of deafness, infertility, chronic pain.
- The life-long repeated scans with frequent surgeries cause a "wait-and-see" and "what next" scenario that is played out regularly for patients that often has an emotional impact via chronic stress, anxiety, and depression.
- Early mortality is anticipated for people with VHL.

Patient Stories

BRAYDEN AND CAMRON KING



My father, sister and I have VHL. My sister is 12, I am 15, and my dad is 45. Each of us have a different story with VHL and it is my pleasure to have the opportunity to share a little bit of that with you today.

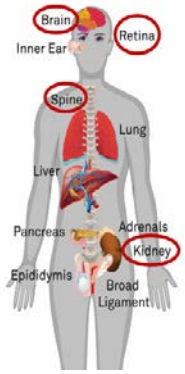
While my sister has not yet had any tumors, I have had four already. Three years ago, I had two tumors removed, one from each of my adrenal glands. I am currently waiting for the National Institutes of Health to open again so that I can have another surgery to remove a new tumor from my right adrenal gland and a paraganglioma from my neck. My dad has had several surgeries including having both of his adrenal glands removed, a portion of his right kidney, part of his liver, a tumor removed from his spine, tumors in his right eye, and he has had a treatment for a brain tumor.

We are extremely fortunate to have the opportunity to be able to be screened early and often for VHL manifestations. My dad's first tumor was when he was 10 and my first tumors showed up when I was 11 - we really know about early onset. We also understand what it is like to have family members with VHL and what it means to have screening, surgeries and how to live and support each other through the challenges with this disease. My mom is an ICU nurse and we are so lucky to have her to help us navigate through the medical system and to manage all our tests, medications, and living life. She is awesome! VHL does not just affect the people who get tumors it affects everyone around you. My family and my friends as well as the people my parents work with and their friends all know and support us which is truly a blessing.

We want to make sure that we continue to work to increase awareness of VHL, but, most importantly, we need to continue to look to the future and really look forward to finding therapies that can increase the quality of my life. Most importantly, though, we need to find a cure. I don't know what my future holds - whether or not I'm going to have tumors in certain parts of my body or when, but what I do know is that I will have tumors and I will have to have more surgeries. I know that my family and friends will continue to support me through that challenge.

VHL is not something that I think about every single moment, but it is something that I live with, that I think about, that I am conscious of. In our family we do not let VHL define us as people, but rather it is an aspect of who we are. I just finished my freshman year in high school, and I look forward to a bright future. I know that the work that is being done to find treatments and a cure will help us to better live a happy and bright life, even with VHL.

DEANNA WICKIZER



I am 49 years old. I am first in family and was diagnosed with VHL when I was a senior in college. Being first in the family has its challenges, but I am so grateful that my brother sisters and nieces and nephews do not have this disease. To date, I have undergone seven brain surgeries, two partial nephrectomies and more than 50 eye surgeries, and currently live with at least one inoperable brain tumor. Since 2018 alone, I have had three brain surgeries and the placement of two brain shunts. The aftermath is a loss of mobility forcing me to use a walker. In addition, I have severe visual impairment and a lot of increasing difficulty in using my hands for writing, typing, etc. Adding insult to injury, because three of my brain surgeries were performed using the same point of entrance, the wound has not yet completely healed, and it's nearing a year now. This has resulted in a great deal of stress, as well as numerous doctor's visits and trips to the ER.

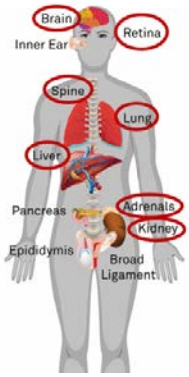
Each time I have to go for routine scans or scans due to current problems, I have extreme anxiety. I have obviously had a lot of bad news from scans! One of my biggest fears is completely losing my eyesight.

Because of VHL, I have lost my independence and career. I no longer have the capacity to do some of the things that have been my strengths all my life. The side effects and impacts of VHL have forced me to go on disability leaving behind a very successful and fulfilling career. I worked hard to build a successful career, earning my master's degree, and giving it up has been very difficult for me and has been accompanied with a grieving process. I have decided several times that I would not have any more surgeries, though so far I've obviously changed my mind when forced to do so.

Because of my diagnosis I am intentionally single and have chosen not to have children. I am a strong, independent woman and having to rely on friends and family for help with housework, getting to appointments, and many mundane tasks is very demoralizing.

It is no surprise that after having had to go through so many surgeries and stereotactic radiation treatment which have been so detrimental to my quality of life, I hope for the day that there is another option. It would be life-changing!

MAUREEN NIETFELD



My journey with VHL began at the age of four. That's when my mother took me to the eye doctor for what she thought was a lazy eye; something that she thought could be easily corrected with glasses. She was shocked when the doctor told her that my right eye was filled with tumors so the point that I had no vision. The doctor also told my mother I had cancer.

An appointment at Will's Eye Institute in Philadelphia was quickly arranged. Thankfully, I was seen by two wonderful surgeons. After three surgical attempts to save my eye. On the fourth surgery, they removed me eye because it was impossible to regulate the pressure which was causing non-relenting pain. It was

at this point that my mother was told that I likely had VHL. Of course, my mother had never heard of this strange disease but dutifully had my entire family was tested. None of them were diagnosed with VHL.

As recommended by the surgeons at Wills, I began a life of at least annual surveillance for VHL manifestations.

At the age of 10, I was officially given a diagnosis of VHL, thanks to the detection of my first brain tumor. By the age of 17 and 14 eye surgeries later, I had lost all vision on my left eye vision and was then totally blind.

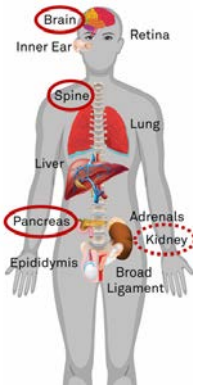
At the age of 16, I developed renal cell carcinoma and by the age of 26 had both kidneys removed forcing me to go on dialysis. Dialysis caused major hemorrhaging of the retinal hemangioblastomas in my left eye forcing the doctors to ultimately remove that I as well. During this time, I was also faced surgery to remove a pheochromocytoma removed in my bladder which was originally assumed to metastatic RCC. Fortunately for me, after 9 months on dialysis, my non-VHL brother was able to be my kidney donor.

For me, VHL has been relentless. I am now 38 years old and have had a total of 35 surgeries. Five of these surgeries have been brain surgeries, leaving me with balance issues, constant headaches, and loss of use of my right hand.

Years after my clinical VHL diagnosis, at the age of 40, my mother suffered a severe migraine and was rushed to the hospital. That is when they found a hemangiobolastoma closing off the top of her spine. This time her VHL genetic test came back positive as did one of my brother's. My grandmother died at the age of 38 from renal cell carcinoma; we suspect that she had also had VHL.

Today I have Riley, my seeing eye dog who not only serves as my sight, but also assists me in my issues with balance, parting mementos of my spine and brain surgeries. Today I also don't just survive with VHL but I work every day to thrive. I am a professional, a wife, and a mother. I have a little boy named Logan who is almost 2 years old. He, too, has VHL. My worry every day is not just for my mother, my brother, and myself but for my precious little Logan. I always want to remain strong for Logan and set a good example, but I can't help but feel a broken heart for him.

SETH HORWITZ



I am a 41-year-old VHL patient and I am the first in my family with VHL and will be the last.

Looking back, my first VHL manifestations began when I was around the age of ten. Minor headaches, nausea, and occasional breathing issues were all dismissed, by multiple doctors, as stress or a light case of asthma. In the late 80's few doctors knew of, or could diagnose, VHL. But, after four more years, it was clear that I was deteriorating rapidly. Instead of playing sports, as I had always done, I was laying on benches reading books. I felt a complete malaise and headaches had reached a crescendo. As school began, I didn't eat, I was taking a lot of naps, and had dry heaves in attempts to vomit.

After seeking treatment from yet more doctors, Dr. Sachs, likely the eighth in my growing chain, looked in my eyes during her exam, starting the path to emergency surgery to remove a golf ball-sized hemangioblastoma with cyst from my brain stem. After three weeks in the hospital, and

months of home rehab to relearn how to walk, I returned to school at the end of the second quarter. This sounds rough, but was close to a best case scenario given my situation. By my sophomore year, I was back to playing ice hockey goalie. Odd as that sounds, my mask covered the back of my skull, where bone was missing, so facing slap shots was deemed safe. Things were back to relative normal.

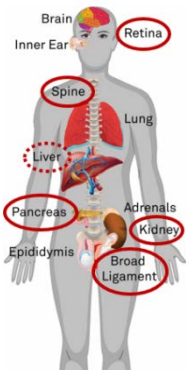
In the summer of 2000, after almost a decade of "good" MRIs, following my graduation from The Wharton School, but before a year in London with my new job at Morgan Stanley, I had my annual tests and found that a small cervical tumor had further grown. I was asymptomatic but given the possibility of more growth in the tumor while being so far from doctors who knew my case, surgery was recommended as the only option. I went to the hospital at 4 AM on a Monday, joked with the nurses, walked into the operating room, and jumped up onto the operating table. Roughly ten hours later, I woke up in recovery with no sensation or the ability to move below my shoulders.

A month later, it was clear that this deficiency was not going to heal itself soon. From the hospital, I was transferred to an inpatient physical therapy facility. After a few weeks, I was able to operate a powered wheel chair using the fingers on my left hand, and still wore a diaper and required a catheter. Seven weeks after that, I was a grown man discharged to live with my parents. I could stand up and use a specialized walker with a platform to brace my right forearm, but of course I could only do this if someone helped me up and then put me down. I had no feeling in the left leg that was supporting me and little movement in the right. Functionally, my left arm was now good, but lacked feeling to the point where I would burn my skin and not know. After another year of daily physical therapy, I was able to progress from a manual wheelchair to walk with two forearm crutches, and a leg brace, ready to take the next step.

I knew that there was no possibility to assume the job on Wall Street for which I had spent so many years preparing, as I could only work part time with needed physical therapy. My former peers were all making six-figure salaries plus handsome bonuses in challenging jobs, and I was doing menial work that I could have done while in high school. Over the next five years, I was able to both advance to walking with a cane, and working a full workweek in a position like I'd already had six years prior. Combined with future hiccups, unexpected complications from a VHL surgery have been devastating for my career, and life in general.
As with most, my VHL manifestations continue to this day. I have had two craniotomies in just the last four years, and more will come. I have innumerable lesions in my pancreas and kidneys that get poked and prodded at. To date, the kidney lesions are cystic and are not RCC, nor treated. Like others, I move on until the next test and inevitable surgery.

JACQUELINE ZARRO

My VHL presentation is multi-modal, with the most significant impact being inoperable metastatic pancreatic neuroendocrine cancer mediatized to the liver. My personal background and disease course dovetail in ways that have led to joining this important discussion today. From a professional perspective, after receiving my doctorate years ago in phycology, I spent the majority of my career in clinical research leading global staff in the management of studies that included rare disease, actually, as well as oncology and CNS. In fact, I have had the esteemed pleasure of participating in FDA meetings in this past role, and have a special reverence for the work that you do every day at the Agency.



Now, living on the other side, so to speak from a disease perspective, VHL is at the heart of the worst experience of my life, which was the death of my mother, and the best news of my life, which was that my sister and therefore her children do not carry the VHL gene. In the past on my commute to work I would regularly think of my mother a physician who could not carry out her life's work. I would hypothesize about whether it could have been an environmental lab exposure or perhaps an unknown pre-disposition that caused her wide-spread cancer. When I incidentally felt a lump in my abdomen when I was 44, I would come to learn that she likely had VHL as did I.

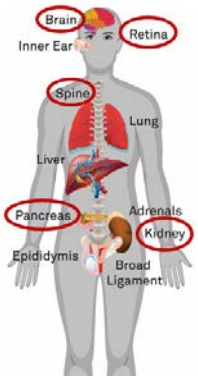
Since then VHL drastically altered my life. With much regret I eventually had to go on disability and start to view that my job needed to be management of medical interventions. Such a change in life was difficult as was realizing the front line challenges of self-advocacy. Even though I had discovered the initial kidney tumors, several years later I sat with medical staff who denied I could detect newly developing abdominal pain. This was despite my report of feeling so poorly that I could move nor call for assistance at times – and despite MRI images indicating continually increasing growth of pancreatic and liver masses. Six months later I was in surgery where neuroendocrine cancer was indeed confirmed.

As a summary, VHL manifestation in my case include: Stage IV Grade 2 Neuroendocrine Cancer, Bi-lateral Renal Cell Carcinoma, and cysts of the reproductive track, retina, and spine. Major medical interventions to date have included three abdominal surgeries, Somatostatin monthly injections, Peptide Receptor Radionuclide Therapy (PRRT), multiple rounds of liver directed radiation, and currently, two concurrent oral chemotherapy medications for a second time. Most impactful symptoms: extremely limiting fatigue, abdominal pain, and heart palpitations, flu-like symptoms including fever, as well impact on digestion and nausea. The psychosocial impact of symptom management crosses all functions of life: limited social and familial interaction, and ability to provide care for family, disability, and on-going medical care burden to name a few.

While I am immensely grateful for the treatments that currently exist for the sequala of VHL such as neuroendocrine tumors, they are time-limited, complex, and taxing. Knowing the commitment within the research community, I am confident that intervention to prevent the manifestations of VHL is possible. This would positively affect generations. My mother died at 58 not knowing she had VHL, and most likely did her brother at 22 years old. As discussed during this session, the impact on individuals and families is significant.

In conclusion, I speak on behalf of my fellow VHL patients including those who cannot voice their experience due to critically advanced illness. Together we look forward to a day where surgery is not the primary approach for managing VHL, and we thank you for your time and efforts.

MICHAELA THORNE



I am a 30-year old and am from Massachusetts. I was pregnant with my first son when I was diagnosed with VHL. That was in 2012, I was 22 years old. It was a huge shock not just to me, but for my family as well. Up until then, we all knew I was clear from the disease. When I was young, my mother took me regularly for my annual scans. Everything had always checked out. As the years went by and my mom became sicker and sicker from VHL my scans were less frequent, eventually stopping all together.

My mother was diagnosed with VHL when she was 9 years old. She got her yearly scans and every time she met with bad news. She was blind in her left eye and had multiple kidney and brain tumors. Eventually she loss all kidney function and was put on dialysis.

It was the brain surgeries that had the greatest toll on her body. I was in elementary school when excision of a tumor on her cerebellum left with such unstable balance that she could no longer drive. Years and a couple more surgeries later, she became wheelchair bound.

Before my mother started to get really sick, she did everything and anything she could to provide me with a happy childhood, but as she got sicker vacations slowly disappeared. Eventually, I stopped going to school; I almost didn't graduate high school. I feel like most of my life consisted of being in and out of the hospital visiting my mother. It was really tough.

After my own VHL diagnosis, I began getting my annual scans; scans of the eyes, brain, spine pancreas, kidneys etc. Over the years, they have found five brain tumors, multiple tumors in my kidneys, a tumor in my spine, a tumor in my left eye and multiple cysts in my pancreas. As a result, I live life in fear and anxiety. What if I need brain surgery or what if they have to remove my kidneys and I have to go on dialysis. My life is a life of "What If". For the sake of my children and husband, I push through my thoughts pushing through with a smile on my face. After all, I have been one of the lucky ones with VHL in that I have not yet had any major surgery.

In 2016, my husband and were I married. We were both aware of VHL and what it could bring, but that wasn't something that was going to hold us back. To backtrack a little, we had our first son in 2013. He was tested for VHL 8 months later, unfortunately he tested positive. Our second son came in 2014. I was too afraid of the outcome that I waited to have him tested. In 2018, our third son was born and I finally got the courage to get them both tested. Needless to say, I answered that call as a nervous wreck. Miraculously, both tested negative, I cried so many happy tears.

During our first pregnancy we had no idea that VHL could potentially affect our family and if we did I am not sure that I would have tried to have children. Yet, he brought so much joy to our life that we decided whatever happens is meant to be. Now we are blessed with three amazing boys!

Some days it can be hard living life with VHL. I can say it honestly takes a village to raise these tiny humans. If I didn't have the support system, I am not sure how I would manage all of these appointments.

In 2018, I stumbled upon a clinical trial for with VHL patients with renal cell carcinoma. While not wanting to get my hopes up, I jumped on board and gave it a try. My only hope was that this drug could potentially slow down, or even shrink, my 5 kidney tumors. Who knows, could it also impact

the lesions in other parts of my body such as in my brain, spine, pancreas? By participating in this trial, could I be helping my son and others with VHL?

Today, just over 1.5 years on the drug, I am ecstatic to report that my kidney tumors have all shrunk and my brain tumors have disappeared! I sat with my doctor and cried when I was given such incredible news. I never thought I would see the day where tumors would shrink, let alone disappear. It is life changing, I look back to the childhood I had with a mother who was always so sick and in the hospital. I can honestly say I was and still am scared that, that could be me someday. I do my best to put my big girl pants on and think positive for my family and I. It can be challenging some days but with this new medication I see hope for VHL patients like myself.

Summary

- Lifelong impact of VHL as well as need for non-surgical intervention and possible cure was highlighted.
- Dedication to VHL patients who have recently lost their lives to VHL was made noting their positive impact to the VHL Alliance, families, and communities.
- Patient Survey conducted by the VHL Alliance revealed that patients have a burden of concerns spanning: constant uncertainty; impact on finances, work, school; constant fear/dread; number of surgeries a body can realistically survive, chronic pain, to name a few.
- The survey also revealed that a non-surgical treatment for VHL would have a significantly positive impact including: more confidence to make long-term plans and goals in life; less fear regarding annual imaging/scans and next surgery; reduced chronic pain and recovery time from surgery; greater financial security; and promotion of ability to engage in school/career.

Dedication

On behalf of VHL patients everywhere, this information is dedicated to those VHL patients we have lost far before their time.


Shelby

Weeks after her college graduation, Shelby passed away following surgery to remove a hemangioblastoma on her brain stem. She never made it to the graduate school where she was to matriculate in the fall semester. A retinal hemangioblastoma led to Shelby's VHL diagnosis and subsequent genetic testing of the entire family. It was then that they learned that her mother, Lisa, is a VHL mosaic. Shelby's diagnosis saved Lisa's life.


Karen

Karen, past VHLA Board chair and a personal close friend and mentor. Unfortunately, her ophthalmologist never followed up with a possible VHL diagnosis when he discovered a hemangioblastoma in her retina. Years later, Karen's RCC was discovered through a relatively routine MRI on her pelvic region. The tumor was already greater than 4 cm in size. It had probably already metastasized before Karen was diagnosed with VHL. Karen died in April 2019.

A week before her first CNS scanning was scheduled, Alyssa passed away from a hemorrhaged brain hemangioblastoma. She was only 14 and had been suffering from severe headaches about which she never told her parents. Her mother Kim, who also had VHL, passed away just two years later.

Tom's life with VHL and dealing with the ramifications resulted in a life of depression and anxiety, so severe that he ultimately took his own life in his 40's.

Julie was a school counselor dedicated to helping the lives of her students. VHL left her an orphan at a young age. RCC and retinal hemangioblastomas, which left her legally blind, were among her VHL manifestations. It was the ultimately metastatic pancreatic neuroendocrine cancer that was the cause of her death 2 years ago. Julie's medicine was a daily dosage of hope to hundreds of students and families in their community, who remember her each year with a town "Festival of Hope."

Questions and Answers by FDA

What has been the experience with clinical trials?

- The criticality of the role of doctors as Principle Investigators and site staff who oversee the conduct at the site level was noted.
- Michaela Thorne, a participant in a current clinical trial, shared that her experience was extremely smooth; while anemia developed, all else has gone well with being fully accommodated for visits and regular scans.
- It was noted that this specific study was the fasted known enrolling trial; partially contributing was word of mouth amongst patients and ultimately met enrollment in approximately 2 months, truly demonstrating an unmet need.

How much outreach exists internationally?

- There are VHL Alliance affiliates throughout the world including Europe, Russia, and Asia. The U.S. VHL Alliance is considered the global patient organization since it is the only one with paid staff. Thus the materials that are developed at VHLA are shared with affiliates. An example of the interaction collaboration is the biennial International VHL Medical/Research Symposium. This year it was supposed to take place in Amsterdam, but will be held virtually due to the pandemic.
- Dr. Eric Jonasch added that the VHL research community is extremely small thus organizations are not wide spread globally as one sees with other diseases.

Closing Remarks

A number of FDA participants thanked the VHL Alliance for the Listening Session expressing that it was it was invaluable hearing from the patients. Dr. Furia-Helm also thanked everyone for attending, especially the VHL Alliance as they helped the Agency to understand how the FDA can best support patients.

Attendees

FDA ATTENDEES

- CBER - Center for Biologics Evaluation & Research
  - Office of the Center Director
  - Office of Tissues and Advanced Therapies
  - Office of Tissues and Advanced Therapies, Division of Clinical Evaluation and Pharmacology/Toxicology
- CDRH - Center for Devices & Radiological Health
  - Office of Product Evaluation and Quality
- CDER - Center for Drug Evaluation & Research
  - Office of Oncologic Diseases, Divisions of Oncology 1 (DO1) and Oncology 2 (DO2)
  - Office of Oncologic Diseases, Division of Hematology Oncology Toxicology
  - Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine, Division of Rare Diseases and Medical Genetics
  - Office of Neuroscience, Division of Anesthesiology, Addiction Medicine, and Pain Medicine
  - Office of the Center Director, Professional Affairs and Stakeholder Engagement
  - Office of Translational Sciences, Office of Biostatistics, Division of Biometrics V
  - Office of Regulatory Operations
- OC - Office of the Commissioner
  - Immediate Office of the Commissioner
  - Patient Affairs Staff
  - Office of Clinical Policy & Programs
  - Office of Orphan Products Development
  - Office of the Chief Scientist
  - Oncology Center of Excellence

ADDITIONAL ATTENDEES

- The Reagan-Udall Foundation for the FDA
- NIH, National Center for Advancing Translational Sciences

Financial Interest

One physician and the VHLA receive funding for research and programs, respectively, and one participant's spouse formerly worked for a pharmaceutical company, though no conflict of interests existed for this session nor did any participant received compensation for the meeting.

Disclaimer

Discussions in FDA Patient Listening Sessions are informal. All opinions, recommendations, and proposals are unofficial and nonbinding on FDA and all other participants. This report reflects VHL Alliance's account of the perspectives of patients and caregivers who participated in the session. To the extent possible, the terms used in this summary to describe specific manifestations of VHL disease, health effects and impacts, and treatment experiences, reflect those of the participants. This report is not meant to be representative of the views and experiences of the entire VHL disease patient population or any specific group of individuals or entities. There may be experiences that are not mentioned in this document.

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