

Alopecia Areata Patients and Caregivers Share Their Stories at FDA Patient-Focused Drug Development Public Meeting



Patients ready to share their experiences with alopecia areata at the FDA Patient-Focused Drug Development Public Meeting, Sept. 11, 2017 in Silver Spring, MD

September 11, 2017 – The sky was clear and blue as more than 200 people living with alopecia areata, caregivers and parents, members of the research community and other interested parties gathered at the headquarters of the U.S. Food and Drug Administration (FDA) in Silver Spring, Maryland to share their experiences and insights about living with the disease, with another 200 participating via webinar online. The FDA selected alopecia areata as one of 24 diseases to explore with patients through its Patient-Focused Drug Development (PFDD) program, aimed at gathering insights on living with the disease, reactions to currently available treatment options and views on potential treatments.

The meeting room included a panel of FDA representatives at the front along with a table with five seats for patient panelists to speak, with the largest section of the room specified for patients and patient representatives. The back portion of the room included a press section and seats for industry representatives and the public.

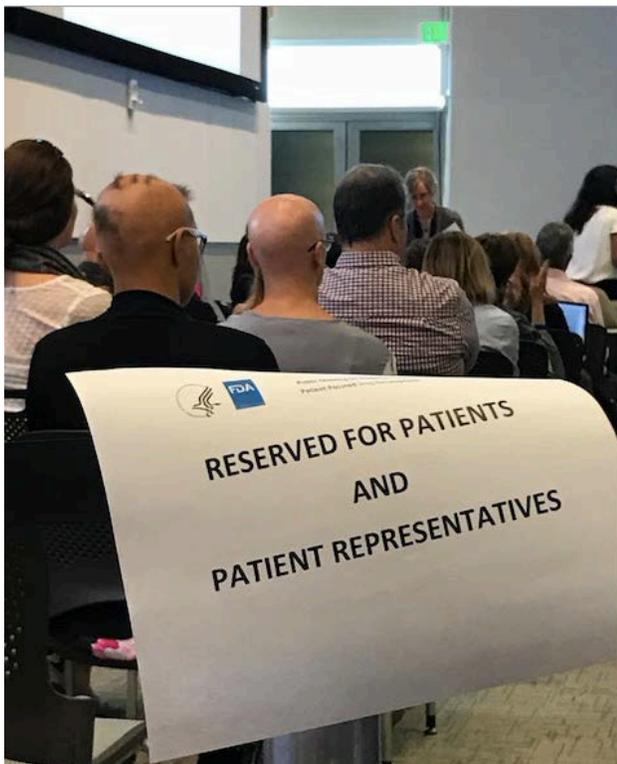
Theresa Mullin, PhD, Director of FDA's Office of Strategic Programs (OSP), gave an overview of the Patient-Focused Drug Development Initiative, explaining that the FDA is developing a more systematic way of gathering patient perspective on their condition and available treatment options through a series of meetings with patients. Dr. Mullin described the purpose of the sessions as giving the Agency patient perspective on benefit vs. risk tradeoffs that FDA will take into account during their assessment of potential new drugs. 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.

With this context in place, Melissa Reyes, MD, with the Division of Dermatology and Dental Products, (DDDP), provided an overview of alopecia areata and current treatment options. The disease usually appears by age 40 and affects males and females equally. In children, the mean age of onset is between 5-10 years of age, and onset in first two decades is associated with more severe disease.

She further described how hair loss from alopecia areata presents in three patterns: focal (patches of hair missing), totalis (no hair on the head) and universalis (no hair anywhere on the body). It can also affect the nails. Alopecia totalis and universalis almost always manifests before 30 years of age.

There are no FDA-approved treatment options for alopecia areata, leaving patients to try an array of treatments including injected corticosteroids, topical treatments and systemic treatments, such as immunosuppressants and immunomodulators, along with other more obscure approaches.

Dr. Ryes assessed the quality of life impact of the disease, including social, psychological and economic impacts, citing a study that showed alopecia areata patients have lower Health-Related Quality of Life than control patients and comparable to other chronic autoimmune skin conditions such as psoriasis and atopic dermatitis.¹ She also referenced the Cochrane review “Interventions for Alopecia Areata (Review)” – 2008, which describes in alopecia areata the “desperate need for large well conducted studies that evaluate long-term effects of therapies on quality of life.”



Listening intently at the FDA Patient-Focused Drug Development Public Meeting, Sept. 11, 2017 in Silver Spring, MD

Michelle Campbell, PhD, part of the Clinical Outcomes Assessment Staff, provided an overview of the pathway from PFDD patient meetings to clinical trials, describing PFDD meetings as a “starting point” for developing patient-focused outcome measures and endpoints to support and guide FDA risk-benefit assessments in drug reviews.

The patient and public input part of the meeting began after the FDA presentations. An FDA facilitator introduced two panels of 5 alopecia areata patients and caregivers to address two sequential topics: health effects and daily impacts of alopecia areata, and current approaches to treatment. The panel members each had three minutes to speak, providing their thoughts on the topic, and then the facilitator opened the floor for participants to answer questions from FDA panel members and share their experiences around that topic.

FDA also provided several multiple-choice questions to the patients, enabling them to provide answers via handheld devices provided on each seat. Results were shown in real-time on the screen to further facilitate discussions around specific topics, including aspects of the disease that are most bothersome, impacts on daily life, different types of drugs and devices they had tried, and the downsides to current treatments.

Several key themes emerged through these discussions:

- **The emotional impact of alopecia areata**, especially for patients whose disease appeared in childhood, is significant. Many patients described bullying and social stigma. Patients mentioned jobs lost and marriages ended. Parents struggled to maintain their composure while describing the suffering of their children, both with the disease and with the often painful and dangerous treatments available. Two patients mentioned attempting suicide. These stories were emotional for all attendees.



Instant polls of participants and patient statements revealed insights at the FDA Patient-Focused Drug Development Public Meeting, Sept. 11, 2017 in Silver Spring, MD

- **Other concerns beyond hair loss** – A number of patients spoke about other related conditions associated with their alopecia areata, including other autoimmune diseases, frequent colds, asthma, and thyroid disease.
- **Desperate need for treatments** – Many patients brought lists of the many treatments they have tried, ranging from harsh black tar that burned their skin to hundreds of injections into their scalp and eyebrows. All who spoke said they were attending the meeting to encourage research and development of new treatments.
- **Differing opinions on successful treatment** – Interestingly, while some patients were anxious for complete regrowth of all of their hair, others said a treatment that enabled them to grow back their body hair, even if not the hair on their head, would be a desirable treatment for them. Several patients described their experiences with an experimental treatment being used off-label that has provided this result and other promising examples of hair growth. Lightening the mood, one young patients said she hoped a future treatment would be “safe for kids to take and able to be coated in chocolate.”

After the panel discussion, FDA opened the floor for public comments, which enabled more patients and caregivers to share their experiences, along with comments from alopecia areata patient groups.

In her closing remarks, Dr. Oussova thanked all patients and caregivers for their candor and for taking the time to travel to the meeting to make their voices heard. She explained that alopecia areata patients across the country can continue to share their experiences with the disease until November 13, 2017 on FDA’s public docket for this topic: <https://www.regulations.gov/document?D=FDA-2017-N-3067-0001>. All comments submitted will be added to those from the meeting and published in a forthcoming Voice of the Patient report on alopecia areata.

¹ Health-related quality of life among patients with alopecia areata: A systemic review. *J Am Acad Dermatol* 2016;75:806-12.