

The Voice of the Patient

A series of reports from the U.S. Food and Drug Administration's (FDA's)
Patient-Focused Drug Development Initiative

Vitiligo

Public Meeting: March 8, 2021

Report Date: December 2021

Center for Drug Evaluation and Research (CDER)
U.S. Food and Drug Administration (FDA)

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Introduction

On March 8, 2021, FDA held a public meeting to hear perspectives from patients with vitiligo, caregivers, and other patient representatives regarding the symptoms of vitiligo that matter most to patients and current approaches to treating this condition. In 2012, FDA established the Patient-Focused Drug Development (PFDD) initiative to more systematically obtain the patient perspective on specific diseases and their currently available treatments. The vitiligo public meeting was the Agency's 30th Patient-Focused Drug Development meeting.

More information about Patient-Focused Drug Development can be found at <https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development>.

Overview of Vitiligo

Vitiligo is an autoimmune condition that causes the loss of skin color when the body attacks its own melanocytes—the cells that give skin, hair, and other areas of the body color leading to depigmentation (a loss of color/pigment for the skin). Some people also experience intense itching at the sites of depigmentation when vitiligo is in an active stage. Some people with vitiligo also have patches of pigment loss affecting the hair on their scalp or body. It is unclear what specific circumstances trigger the immune system to attack melanocytes in the skin, but research suggests roles for genetic and environmental factors. Vitiligo sometimes runs in families, but the inheritance pattern is complex since many causative factors are involved. Individuals living with vitiligo may be more likely to develop other autoimmune conditions, such as hypothyroidism, diabetes, pernicious anemia, and alopecia areata. Vitiligo is a common condition, affecting between 0.5 and 1 percent of the population worldwide. The average age of onset of vitiligo is in the mid-twenties, but it can appear at any age. ¹

There is no cure for vitiligo, and there are no FDA-approved treatments for repigmentation of vitiligo lesions. Treatments to attempt repigmentation include topical corticosteroids and psoralen and ultraviolet A (PUVA) and other light therapies such as excimer laser therapy.² Complete depigmentation therapies are also available for individuals who have not responded to other medical therapies and have extensive depigmented areas.³ Surgeries, such as skin grafting, melanocyte transplants, and micropigmentation are other treatment methods.⁴ Supportive or ancillary care includes psychotherapy, diet modifications, and corrective cosmetics.

Meeting overview

This virtual (online only) meeting provided FDA the opportunity to hear directly from patients, caregivers, and other patient representatives about their experiences with vitiligo and its treatments. Patients, caregivers, and other patient representatives were encouraged to participate in the facilitated

¹ <https://ghr.nlm.nih.gov/condition/vitiligo>

² <https://www.healthline.com/health/skin-disorders/vitiligo-pictures#support>

³ <https://pubmed.ncbi.nlm.nih.gov/22199060/>

⁴ <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3408993/>

discussion by submitting comments online during the meeting or by calling in via phone to share their experiences. The discussion focused on two key topics: 1) health effects and daily impacts of vitiligo and 2) patients' perspectives on current approaches to treating vitiligo.

For each topic, a panel of patients and caregivers (Appendix 2) shared comments to begin the dialogue. Panel comments were followed by large-group facilitated discussions inviting other patients, caregivers, and patient representatives to call in or submit written comments through the webcast platform. An FDA facilitator led the discussions, and members of a panel of FDA staff (Appendix 2) asked follow-up questions. Patient and caregiver participants were periodically invited to respond to polling questions (Appendix 3), which provided a sense of the demographic makeup of participants and how many participants shared a particular perspective on a given topic.

Approximately 1,000 people attended the virtual meeting through the live webcast, with around 650 identifying themselves as individuals with vitiligo or patient representatives. According to responses to polling questions, participants ranged in age from younger than 8 years old to over 60 years of age, with more than half of participants over the age of 40. Almost 65% of participants identified as female. Participants represented a range of experiences with vitiligo, with approximately 81% of respondents indicating the most bothersome aspects of vitiligo were the location and amount or extent of depigmentation. Others reported skin sensitivity and loss of hair color as bothersome aspects of their vitiligo. Although participants in this meeting may not fully represent the diverse population living with vitiligo, the perspectives they shared reflected a diverse set of experiences with vitiligo symptoms and treatments.

To supplement the input gathered at the meeting, individuals with vitiligo and others were encouraged to submit comments on the topic to a public docket,⁵ which was open until May 10, 2021. In total, 106 comments were submitted to the public docket, the majority by individual patients and caregivers. In addition to comments collected through the public docket, FDA launched a crowdsourcing campaign as another way for the vitiligo community to engage and interact with each other and the FDA. The comments received through crowdsourcing were collated and included in the public docket.

More information, including the archived webcast and meeting transcript, is available on the meeting website: <https://www.fda.gov/industry/prescription-drug-user-fee-amendments/fda-led-patient-focused-drug-development-pfdd-public-meetings#vitiligo>.

⁵ A *docket* is a repository through which the public can submit electronic and written comments on specific topics to U.S. federal agencies such as FDA. More information can be found at www.regulations.gov.

Report overview and key themes

This report summarizes the input shared by patients and patient representatives during the meeting. It also includes a summary of comments submitted to the public docket. To the extent possible, the terms used in this report to describe specific vitiligo symptoms, impacts, and treatment experiences reflect the words used by the patient and caregiver participants and commenters to the docket. It is recognized that the views and experiences shared in this report may not be representative of the broader population of patients with vitiligo, and that there may be other experiences with symptoms, impacts, treatments, or other aspects of vitiligo that are not included in this report.

The input from the meeting and docket comments provided rich detail on the impact of vitiligo on patients. The physical, emotional, and social toll vitiligo takes on daily life, along with the need for new treatment options, emerged within the key themes:

- Participants described depigmentation and skin sensitivity as the most burdensome aspects of their vitiligo. They emphasized that vitiligo is more than a cosmetic condition; it has debilitating physical and emotional impacts.
- Participants stressed the severe emotional impact of their vitiligo on daily life. They explained how their depigmentation has caused anxiety, depression, and suicidal ideation. They also attributed a loss of identity and a sense of decreased self-confidence to their vitiligo. They discussed the impact of vitiligo on maintaining friendships and relationships, working and attending school, and participating in social events and gatherings.
- Participants shared their experiences with prescription and non-prescription medicines. They discussed topical corticosteroids, PUVA and other light therapies, and other therapeutic options such as dietary modifications and corrective cosmetics. Their ability to manage their vitiligo was generally limited, with varying levels of success, and they stressed the need for a long-lasting treatment option that could offer permanent repigmentation. They also expressed the need for cost- and time-effective treatment options that could be conveniently administered at home.

The patient input generated through this Patient-Focused Drug Development meeting and the public docket comments strengthens FDA's understanding of both the burden of vitiligo on patients, and the challenges with treatments currently used to treat vitiligo and its symptoms. FDA staff will carefully consider this input during the drug development process, including when advising sponsors on their drug development programs and when assessing products under review for marketing approval.

This input may also be of value to drug developers. For example, it may help drug developers understand how to develop better endpoints for clinical trials to measure those aspects of vitiligo that are important to patients. It may help drug developers select or develop questionnaires that measure important concepts and engage with the FDA as they develop treatments. The information from these meetings can also help support the FDA review of clinical trial questionnaires to confirm that they are adequately capturing the individuals' and caregivers' perspective on health outcomes.

Topic 1: Health Effects and Daily Impacts That Matter Most to Patients

The first discussion topic focused on patients' experiences with vitiligo and its impact on their daily lives. FDA was particularly interested in hearing participants describe specific health effects and impacts in their own words. FDA was also interested in learning about how vitiligo affects people's ability to live normally and perform activities as fully as they would like.

Five panelists (Appendix 2) provided comments to start the dialogue. Panelists included:

- An African American and Hispanic woman, diagnosed with vitiligo at age 12, explained the changes she experienced in facial pigmentation, including the emotional impact of vitiligo and the importance of her support system and positive environment.
- A 51-year-old African American woman, diagnosed with vitiligo at age 43, described the depigmentation that occurred over 85% of her body within a short period of time. The emotional effects of her vitiligo included depression and suicidal ideation.
- An African American man in his 40s, explained that his vitiligo started in his 20s as a small spot on his hand before spreading all over his body in a matter of several years. His experience was also marked by severe emotional distress.
- A 47-year-old African American and Hispanic man, diagnosed with vitiligo 29 years ago, discussed the emotional and social impacts of his vitiligo, characterizing his experience with vitiligo as similar as a loss of identity and describing the stages of grief he experienced in the process.
- A White woman, living with vitiligo for over 30 years, described the heat intolerance, burning, and itching that accompanied her depigmentation. She does not leave the house or see anyone without applying corrective cosmetics, which she described as tedious and time-consuming.

The panelists' statements provided a detailed account of the physical and emotional burden of living with vitiligo daily. Their perspectives were further reflected in the subsequent large-group discussion, where meeting participants shared comments via phone and web commentary.

Perspectives on most significant symptoms

Participants shared their perspectives on the aspects of vitiligo that impact their daily life. In polling questions (Appendix 3, Q7), participants were asked to identify which aspects of their vitiligo were most bothersome. The top three bothersome aspects of vitiligo, based on the number of responses received, were 1) location of depigmentation, 2) amount or extent of depigmentation, and 3) skin sensitivity. Some participants experienced rapid depigmentation over many areas of their body, while others described small, depigmented spots that did not change for several years. Some participants felt their vitiligo was well-managed, while others chose to no longer pursue treatments, either because of acceptance or lost hope for effective management of their vitiligo.

Depigmentation

All participants experienced some amount of depigmentation, although the location and extent of depigmentation varied. For most participants, depigmentation was present on their skin, but some also

had depigmentation of their hair. Several participants described that their depigmentation started as a small spot on one area that then spread to other areas. One participant described an experience with vitiligo that remained constant for several years, but then spread suddenly: "In my 40s, I had a very small spot. Once I got over 60, it exploded. And it exploded literally overnight. One morning I woke up, [and] my forehead was gone. One morning I woke up, [and] I had spots all over my arms." Another participant stated, "Age 48, I saw white spots on my hands in September 2019. Not long ago... Vitiligo is spreading fast all over my body. Now I look like I almost lost more than 75 percent of my color." One expressed a gradual progression of depigmentation, "It started as a small spot on my hand, and it was really tiny. But then, it started to spread all over my body over the years: my hands, arms, arm pits, torso, back, chest, legs, feet, thighs, around my mouth, and genital area."

Many participants stressed the unpredictability of their depigmentation and repigmentation. One explained, "I have literally gone from having vitiligo on 70 percent of my face to not having any at all... it's happened about three or four times. It does still scare me that at any time my skin can decide... my pigment can go." Another participant, who was diagnosed in 1989, described how their depigmentation has changed over time, "It's really snowballed over the past 12 to 18 months." One noted the uncertain nature of their depigmentation, "I have had pigment come back in about 80 percent of my body and then go again, which is something that I really was not expecting." Another participant described their fear associated with the unpredictability of progression, "You never know what areas of skin are going to depigment next, or how widely."

Several participants emphasized the role that stress played when it came to their depigmentation. In many cases, they expressed how stress was correlated with progression of their depigmentation. One participant reported, "[The] stress of COVID-19 "caused my spots to become wider and new spots to appear on my legs." Another expressed a similar sentiment regarding the stress of COVID-19, saying, "I noticed small spots appearing on my legs and some on my hands. And then they started to merge together. So now those small spots are large spots on my body." Another participant added, "As a registered nurse, my vitiligo has increased significantly with the stress of caring for my COVID-19 patients."

Skin Sensitivity

Several participants referenced skin sensitivity and sunburn as burdensome physical aspects of their vitiligo. Many discussed how the depigmented areas of their bodies would turn red, become sensitive, and sometimes itchy, when exposed to the sun. They shared that sunscreen often failed to protect their skin from sunburn, and they avoided outdoor activities or took special precautions to reduce the risk of sunburn. One participant noted, "[I] have to constantly avoid the sun to avoid burning the vitiligo patches, and I feel like my face has been constantly red for years from the sun itself." Another participant stressed the impact of outdoor exposure on skin sensitivity, saying, "My skin burns very quickly in the sun affecting my ability to do yard work, exercise, and participate in outdoor activities." One emphasized the social impacts, stating, "It's embarrassing when I hang out with friends outside and have to constantly stop to reapply sunscreen or ask them to change plans or locations so I can stay out of the sun... Even driving the car, I try to avoid driving when the sun is at extreme angles so I can avoid burning on my face through the window."

Itch

Some participants reported that their depigmented areas cause them to itch. For some participants, the itchiness could come at any time and last for several minutes or hours. One participant described their itch as “tiny bee stings.” Another shared they experience intense burning and itching that causes them to awaken in the middle of the night, “It just feels like cactus needles stuck in my skin. And many times, the only thing that will help is just to get up and get ice packs. After that, it will usually resolve within an hour or so.” For others, itch was associated with the spread of depigmentation, one noted, “When my spots are spreading, they start to itch first.”

Other Symptoms

Participants described other symptoms that they attributed to their vitiligo:

- Burning of the skin, which for many is often accompanied by itching.
- Heat intolerance, which makes it difficult for some participants to participate in outdoor activities during the warmer months.

Comorbid Conditions

Several participants shared that they had been diagnosed with a number of comorbid conditions, including Hashimoto thyroiditis, metabolic syndrome, adrenal insufficiency, lupus, arthritis, Type I diabetes, multiple sclerosis, systemic mastocytosis, Graves’ Disease, alopecia areata, Raynaud phenomenon, narcolepsy, and other autoimmune conditions.

Overall impact of vitiligo on daily life

Throughout the meeting, participants described the impact that vitiligo has on daily life, including:

- **Severe emotional impacts.** Nearly all participants emphasized the severe emotional distress of vitiligo on daily life, placing limitations on participating in work, school, and social events. For many participants, their experience with vitiligo was “emotionally debilitating” and led to depression, anxiety, and suicidal ideation. One participant said, “The impact of losing so much color so quickly really bothered me for a while and caused me to go into deep depression and seriously contemplate suicide. I literally wanted to die so I wouldn’t have to deal with this awful disease that had taken over my beautiful black skin.” Another shared, “[I] had to cope with the daily anxiety attacks when moving out into society or into social settings outside of work.” One commented how their vitiligo made them feel “embarrassed and ashamed. I felt anxious and depressed.” Nearly all participants emphasized that vitiligo is more than a cosmetic condition. One stated, “The mental and emotional toll is devastating and will continue for the rest of my life.” The emotional impact of vitiligo on another participant had reportedly played a part in their substance abuse.

Almost all participants shared how their vitiligo negatively impacted their self-esteem and self-confidence. One stated, “Vitiligo made me extremely insecure about my body. I battled with low self-esteem, depression, low self-image.” Another participant said, “At a time in my life when I was ready to explore the world, learn new things, and have fun, vitiligo chipped away at my morale, self-esteem, confidence, and quality of life.” Another shared, that after 49 years of living with vitiligo, “I don’t want to go outside. I don’t want to see my face in the mirror.” Many participants also shared concerns about family members inheriting vitiligo and

experiencing the same emotional impacts.

Several participants disclosed that they had experienced a lack of sympathy and medical understanding for their vitiligo. Many emphasized that vitiligo is more than a cosmetic condition, that it impacts individuals both physically and emotionally. One participant talked about a “painful” clinical experience, saying, “[A] dermatologist told me, ‘Oh, you have vitiligo. It’s just nothing. It’s a cosmetic problem.’ He just dismissed all my concerns.” Another participant shared the emotional impacts of their vitiligo universalis, “Once you have universal vitiligo, I feel like a lot of dermatologists think that you’re fine. And I would like to say that it’s not over when you’re completely depigmented. Like you still have the [emotional] effects.”

- **Stigma and social isolation.** Many participants talked about the stigma of vitiligo, often leading to shame and social isolation. Several had experienced racist comments, one shared how they “had a dermatologist tell me he didn’t want to give me monobenzone because he wasn’t sure if I could handle being White.” Another participant admitted to carrying “a doctor’s statement in my car because of the political climate today with the police... I don’t want to be stopped, and somebody look at my license and look at my picture on my license, which does not look like my face anymore, and get arrested because they think I’m trying to be somebody that I’m not.” Another shared how “in the East Indian community, vitiligo is often thought of as a curse... I am afraid to see old friends because they may think I chose to depigment just to be White.” One stated that “people assumed I was contagious.” Another referenced a time they were on a lazy river with their eyes closed, “I listened to comments and behavior from people going by me not realizing I could hear. The fear of contagion is real.” Another participant had a young child ask her, “Why is your face so dirty?”

Other comments through which participants expressed their experience with stigma and social isolation were:

- “Vitiligo forced me into self-isolation, extreme introversion, and at times fully closed me off to the world.”
- “I felt ashamed as a kid, taunted and ... I dread[ed] social gatherings.”
- “I isolated myself in my teens and lost many friends because I was not confident... I felt unworthy of friends or attention for a long time.”
- “I used to swim, bike, skateboard, play the violin at concerts, and manage track and field at my school. But due to my vitiligo and the painful side effects of the treatments, I cannot practice the violin or participate in performances, meetings, or social gatherings as much as I would like.”
- “Even though I’m almost 34 and I’ve lived with vitiligo for over two decades, I still think about my skin condition every day while teleworking full-time and do not feel comfortable using video on meetings because I feel it is something someone will notice right away.”
- “I was fired due to visible vitiligo.”
- “[As] CFO of a clinical trials company, ... hiding my vitiligo in meetings with others is difficult.”

- “[While searching for a job and managing] to get interviews, ...I would do great... but everyone wanted to focus on my vitiligo... sometimes my friend would be like, ‘You’re a shoo-in for the job. You should get it. They need people desperately.’ And then after the interview, one of my friends told me, ‘The reason you didn’t get hired was because they were scared that you would run off customers. They were scared that customers would not come back because they think you have this disease that will be given to them.’”
- **Impact on physical appearance and identity.** Many participants expressed the physical effects of vitiligo on their physical appearance as a sense of loss of racial or ethnic identity, which caused emotional distress. One participant said, “I often feel like I’m losing the very thing that identifies me as a Black woman... Unfortunately, many may say it’s just skin, but for me, it goes much deeper than just skin color.” One detailed their transformation with vitiligo universalis over four years, “I’ve lost my genetic properties... I was proud of my Indonesian color. And I changed from a skin type five into a skin type one.” Another participant who opted for complete depigmentation expressed, “I miss my beautiful brown skin and clear Indian identity. I am not White, I am not Indian. I’m uncertain.” One shared how they no longer take any treatments, “I’m still dealing with the emotional stress of the loss of my color. My identity. And I do not want to go through this process again unless there is a cure and not a treatment.”

Participants also discussed how those with a naturally darker skin tone showed more obvious depigmentation in many cases. One participant described it as a “disparity... it’s more visible on colored people.” Another shared, “As I have a darker skin tone, my noticeable vitiligo draws the most attention from others with unwanted stares, disgusted looks, and unspoken questions.” However, participants with lighter skin tones still experienced similar impacts. One participant reported, “Although my son is not dark in skin color, it is still noticeable, *especially to him.*”

- **Relationship impacts.** Some participants with vitiligo described the difficulties of having intimate relationships. One participant shared, “[Vitiligo] created challenges when engaging in intimate settings because I wasn’t secure with my body, and I was afraid for others to see what I looked like.” Another discussed the frustration of “having to explain to every new romantic partner that ‘my skin looks different under my clothes.’” One participant expressed how they had difficulty getting married because of their vitiligo.

Participants also shared the impact of their vitiligo on their relationships and interactions with others. One participant described her son’s experience with friends at school asking him about his mom’s vitiligo and if she had been burned. She said, “I had to explain it to him and explain what vitiligo was. And eventually, with that education, he became an advocate. He was able to put together the words and to really articulate how it makes him feel when people make fun of his mom’s skin.” Another commented on attending her son’s sports games, “kids would stare. He would be afraid to even walk with me, hold hands with me because of so many stares.”

Topic 2: Patient Perspectives on Treatments for Vitiligo

The second discussion topic focused on patients' experiences with therapies used to treat their vitiligo. Five panelists (Appendix 2) provided comments to start the dialogue:

- An 18-year-old Asian American woman, diagnosed with vitiligo at age eight, discussed her history of treatment with a wide range of prescription drug and non-drug therapies. She no longer uses any treatments due to the time commitment associated with visiting the clinic on a regular basis.
- A 15-year-old Asian American young woman, joined by her mother, diagnosed with vitiligo at age 12, shared her experience with a variety of surgical and non-surgical therapies. Her current treatments are aimed at repigmentation.
- An African American man, diagnosed with vitiligo about 30 years ago, who has experienced treatment with a range of prescription drug and non-drug therapies. He no longer uses any treatments because of the time commitment associated with therapy.
- A 16-year-old White young woman, joined by her mother, diagnosed with vitiligo at age 12, presented her experience with topical medications and phototherapy. She noted that use of her in-home phototherapy unit compared to the in-clinic phototherapy treatment has made her treatment more convenient.
- A 61-year-old African American man, diagnosed with vitiligo nearly 40 years ago, spoke of a wide range of prescription drug and non-drug therapies that he experienced in the past.

In the large-group facilitated discussion that followed, patients and patient representatives discussed their experiences with prescription drugs, light therapy, and non-drug therapies. Many participants emphasized the tedious and time-consuming nature of existing treatment options and cited this as a main reason for choosing to discontinue treatment altogether. Almost all participants reported difficulty in convincing insurance providers that vitiligo is more than just a cosmetic condition and getting coverage. Some also described existing treatment options as ineffective or not long-lasting. Perspectives on treatments and therapies, as well as what participants would look for in an ideal treatment, are summarized below.

Perspectives on current treatments

Participants described a range of treatment approaches they used when looking for a way to manage their vitiligo. A large majority of participants reported (Appendix 3, Q9) use of topical medications for repigmentation and light treatment, including excimer laser, and psoralen and ultraviolet A (PUVA) light therapy. Many also reported use of temporary cosmetic measures, dietary and herbal supplements, and diet modifications as part of their treatment regimen. A large number of participants were not currently using any medical products nor therapies to treat their vitiligo.

Prescription and nonprescription medication

Participants reported using several prescription and nonprescription drug treatment options, including:

- **Topical creams** – The most commonly used type of prescription medication reported by participants was topical creams. Participants described their experiences using topical creams such as triamcinolone, tofacitinib, tacrolimus, and ruxolitinib. Several participants detailed their experience trying several topical creams but felt they were either ineffective or caused unpleasant side effects. One participant described how their topical creams “burned my face pretty badly. It didn’t work very well.” Another reported “the ointment was very greasy, and it often made me break out a little bit, which I wasn’t expecting. And there were like red bumps, and it really wasn’t a nice ointment to use.” Others discussed how topical creams prevented their depigmentation from progressing and some even resulted in repigmentation.
- **PUVA and other light therapy** – Many participants discussed their experiences using PUVA and other light therapy treatments, such as UVB light therapy and excimer laser therapy. Some participants stated how the PUVA and other light therapy treatments were effective for repigmentation, but the repigmentation did not last if they discontinued treatment. One participant stated, “75 percent of my body was depigmented. And then it all [pigment] came back with the PUVA treatment. But then I ran into a problem with my insurance company... then I lost it all [pigment] again.” One participant discussed the challenge of the treatments not being long-lasting, “I think it’s hard for people with vitiligo to go through treatment knowing that it’s very possible it [vitiligo] will come back.” Many participants expressed how tedious and time-consuming the PUVA and other light therapy administration was, which was a major factor behind some participants’ decisions to discontinue treatment. One participant reflected on her experience with light therapy as a child, “I think with the risks of being a child, what was hard for me when we looked into doing treatments was the frequency that we would have to do them in the doctor’s office. So light therapy is something that is seen as probably the most effective treatment along with creams, and we had to go to the doctor’s office three times a week to do that. And not having it in town, the cost associated with it, and the time spent, I think is what a lot of people just don’t do.” A few participants mentioned that they opted to purchase in-home phototherapy units because they thought “it would be more efficient than having to drive to the dermatologist three times per week for like two minutes in a light box.”
- **Other interventions** – Other interventions participants mentioned include punch graft surgery and suction therapy.

Perspectives on non-drug therapies

In response to a polling question (Appendix 3, Q10), many participants indicated they used one or more non-drug therapies to manage their vitiligo. Many participants discussed their experiences with psychotherapy and support groups and stressed the need for more psychological resources for individuals living with vitiligo. One participant stated, “I’ve been in psychotherapy for years. And that has definitely helped a great deal... I’ve been lucky to have a great therapist and people to support me in my vitiligo journey.” Another reflected on her experience with the support group she had founded, saying, “I am passionately empowering individuals to focus on body image, building their self-esteem, and loving the skin that they’re in. I share my journey today because I now stand in the strength of family, friends, and even strangers who offer their love, support, and encouragement. I live a happy, beautiful, and exciting life with vitiligo, and I wear it with great poise and grace.” Other participants stressed the importance of support groups for adolescents and young adults living with vitiligo and talked about the importance of being able to communicate and share stories with other adolescents and young adults living with vitiligo. Some participants talked about experiences with herbal medicine and dietary and

lifestyle changes. One participant noted very fast repigmentation after removing cereals and dairy products from the diet and increasing exposure to the sun.

Many participants shared their experiences using makeup or wearing certain types of clothing to camouflage their vitiligo. Several had relied on long sleeves and pants when going out in public, even in the summer. One participant stated, "I am embarrassed to be with a short sleeve shirt." A few participants discussed the importance of makeup for their jobs in the entertainment industry and challenges with the makeup rubbing off easily or wearing off over the course of the day. One participant remarked on the benefits and downsides of corrective cosmetics, saying, "Corrective cosmetics have been both my saving grace and the bane of my existence. The daily process is very tedious and time-consuming. It can take anywhere from 30 to 45 minutes in the morning. There are multiple layers of products that take various times to dry and set. And the base coat is always a DHA sunless tanner product that takes anywhere from eight to twelve hours to develop, which of course takes advanced planning. And the flipside is removing it all at the end of the day."

Perspectives on ideal treatments for vitiligo

Participants shared their perspectives on attributes they would look for in an ideal treatment for vitiligo. The attribute that participants mentioned the most was that the ideal treatment be effective and long-lasting with a guarantee that depigmentation will not come back. One participant shared, "Most of the treatments do bring improvement, I mean, not every treatment can work with everybody. But there are some treatments that will work with some people. But even in these cases, when we stop the treatment, it will all come back somehow slower or quicker... the efficacy question for me is also the lasting effect of the treatment if possible." Others described the "emotional rollercoaster" associated with depigmentation coming back after discontinuation of an effective treatment. One participant detailed, "We can manage wearing sunblock for the rest of our lives but going through multi-pigmented in all different areas of your life, it's just emotionally exhausting." One participant expressed the desire for a treatment for complete depigmentation so they would not have to deal with covering depigmented areas of their body.

Many participants expressed that the ideal treatment would be cost-effective and readily administered at home. One participant spoke to the desire for this, "If it's something that we can have at home that we can do ourselves, yes... if you're going to see results, I think a lot of people would be willing to do that. But I think for most of us in the vitiligo community, having it not take so much time and so much money is key." Some participants mentioned they would be willing to accept some side effects if the results of the treatment were promising. Some participants also expressed a desire for an ideal treatment with less side effects. One participant shared, "I would not consider any treatment with potential negative side effects. I would rather take more significant dietary and lifestyle measures before a treatment that could impact me negatively in other ways." Regarding makeup and corrective cosmetics, one participant, who worked in the entertainment industry, commented, "There should be a makeup line for makeup artists who work in TV and films... where there's a makeup line that the ingredients are specifically for entertainment people with vitiligo."

As part of the discussion, participants were asked to imagine a hypothetical scenario in which a new topical cream indicated to treat vitiligo had recently been approved by FDA (see full text in Appendix 3). The topical cream needs to be applied once a day. Research in animals and people show that this treatment may improve pigmentation by up to 50% in some people. More common side effects of this therapy may include redness and irritation at the application site, mild acne, and burning. Rarer but

more serious side effects may include fever, pain during swallowing, insomnia, nausea, and increased heart rate. When asked whether they would take the hypothetical medication, given the benefits and risks, 40% of participants answered “maybe,” 36% answered “yes,” and 24% answered “no.” Others also talked about the duration of lasting effects and their preference for the treatment to be longer-lasting. Participants also discussed the need for further research into the root causes of vitiligo and treating the underlying pathology and not just treating the symptoms.

Participants were also asked which area(s) of their body they would prioritize for treatment and what percentage of repigmentation they were willing to accept. Most participants expressed that their face was the most important area when considering treatment. One participant shared, “I would much rather prefer that results rise on my face as opposed to other parts of my body just because those are the most visual areas and those are the areas that people tend to judge.” Another added, “If there were some treatments that could bring the pigment back in our face areas, that would be very helpful because that’s where people see you. And they can hear you... if they can see a normal face when you’re trying to advocate or talk about something important.” Many participants indicated that 50% repigmentation was too low of a threshold for them to consider taking the medication and that they wanted a higher percentage of repigmentation. One participant said, “We definitely want a high percentage [of repigmentation], So typically, in the 80 to 100 percent, mainly because there’s also a pattern of progression of the vitiligo. So, your baseline assessment is constantly changing, which is why you want a very high degree of repigmentation and permanent repigmentation at very high numbers far greater than 50 percent just because the spread and the rate of spread is much higher than the rate of repigmentation.” Another mentioned, “For somebody who’s lost a lot of pigment already, regaining 50 percent [pigmentation] might make them actually look perhaps worse in terms of their own opinion versus being more uniform in color.”

Summary of Comments Submitted to the Public Docket

Over 100 comments were submitted to the public docket that supplemented the Patient-Focused Drug Development public meeting for vitiligo. Most comments were submitted by patients and caregivers; some comments were submitted by health care providers, professional organizations, patient groups, and expert researchers. In addition to comments collected through the public docket, FDA launched a crowdsourcing campaign as another way for the vitiligo community to engage and interact with each other and the FDA. The comments received through crowdsourcing were included as part of the public docket.

Overall, the comments received in the docket and crowdsourcing campaign reflected experiences and perspectives similar to those shared during the public meeting via live webcast.

Submitted comments on symptoms of vitiligo

Comments submitted to the public docket highlighted the physical, emotional, and economic impacts of vitiligo. Docket commenters mentioned all the symptoms of vitiligo that were discussed during the public meeting. A few examples are presented below:

- **Depigmentation.** Commenters shared their perspectives on the spread of depigmentation over time. A few noted that the start and/or spread of their depigmentation was usually associated

with a stressful event or time in their lives. One commenter stated, “With the coronavirus I’ve spent the last three months stressing and worrying about my job and my future. All that stress exacerbated my condition and new depigmented spots appeared literally overnight.”

- **Skin sensitivity.** Commenters experienced skin sensitivity as part of their vitiligo. Some shared how they needed to wear sunscreen or be mindful of their time in the sun to avoid burning. One commenter talked about tanning in the sun, saying, “The unpigmented skin will turn bright red, blister and then turn white again.” Others commented on itchiness and heat sensitivity that sometimes happened along with their depigmentation. One said, “When my vitiligo is active or increasing, I am unbearably itchy. I stopped treatment at one point, but the itching became so bad that I was forced to begin it again.”
- **Comorbid conditions and other symptoms.** Several commenters discussed comorbid conditions they have in addition to vitiligo. Comorbid conditions included psoriatic arthritis, Crohns Disease, lupus, chronic hives, and Hashimoto thyroiditis.

Submitted comments on the overall impact of vitiligo on daily life

Comments submitted to the public docket reflected the input received during the public meeting on the impacts of vitiligo on daily life and the severe emotional impacts on individuals living with vitiligo and their loved ones.

- **Severe emotional impact.** Many commenters talked about the severe emotional impact of their vitiligo. Many referenced struggling with anxiety and depression, and for some, suicidal ideation. One commenter shared, “When I was younger, I often turned down opportunities to speak publicly or be on television because I was so ashamed of my skin. I did not engage my life in the ways I wanted to because I was crippled by anxiety. I couldn’t fathom being laughed at just for existing. I didn’t try new things because I was afraid someone would say something about my skin... When I was pregnant, I was almost hospitalized due to a panic attack brought on by the thought of my son having vitiligo and experiencing what I went through. It can be crippling.” One commenter, who faced life-long bullying, shared the following: “I left my company because I just couldn’t tolerate it anymore. I don’t have any self-esteem. Panic attacks. Self-loathing. No job. Depressed. Social anxiety disorder. No love life. Vitiligo ruined my whole life.” Another commenter shared, “At my lowest points with vitiligo, I thought obsessively about my skin, I hated myself and I thought I didn’t deserve friends. I would cry myself to sleep at night. Going outside was sometimes impossible without first having a complete nervous breakdown.”
- **Stigma and social isolation.** Several commenters noted the stigma and social isolation they face as a result of their vitiligo. One commenter stated, “It is painful for my daughter to put on a bathing suit and go to the pool with her friends because she knows that she will get stares and she might get questions. It is painful for me as a mother to think of people teasing my beautiful daughter, to feel like I have to email teachers before school starts to make them aware of her condition and be alert to potential bullying, and that at times I need to reassure other parents that her condition is not contagious.” Another commented on the impact it has on time with their family, “It’s hard for me to play out in the sun with my kids as I’m constantly worried about burning. It limits what we are able to do as a family and is emotionally exhausting.”

- **Impact on physical appearance and identity.** Many commenters described low self-esteem and the loss of identity as major impacts of their experiences with vitiligo. One commenter said, “I find myself just staring at my spots and hating how my spotted body looks.” Another shared, “I was an outgoing, exuberant, vivacious, productive individual who worked and did numerous volunteer work in my church and community. Now I only think how long I should live. I’m afraid to tell friends and family. I will be seeing a psychiatrist and a therapist next week to maintain my sanity.” One commenter described the impact on identity for their loved one, saying, “Today he experiences depigmentation to 90% of his body which he describes as bittersweet. While he no longer experiences the gawking of strangers at his mottled, white and olive complexion, he suffers with feelings of imposter syndrome as he no longer is accepted into the Hispanic/Latinx community as he once was.”
- **Relationship impacts.** Commenters talked about the impact of vitiligo on their relationships. One commenter wrote, “Was just about to propose to a girl who eventually stop talking to me as soon as my face began to get lighter. All of this is just devastating to feel that I am not socially accepted by anyone anymore.” Another shared, “It affects us who have it by making us feel like we are not part of this world. It’s hard to participate in school or a job if people are just going to stare at you the whole time and even some people try to not touch you because they think it’s nasty. It’s sad for us to go to a dermatologist and for them to say there is no cure.”

Submitted comments on current treatments for vitiligo

The comments submitted to the public docket about experiences with treatments were similar to those expressed at the public meeting. Many cited their experiences with treatments being ineffective or not long-lasting in which their depigmentation would come back after discontinuing the treatment. One commenter shared, “Sadly, none of these treatments are permanent in getting my skin color back. It takes forever to come back and then it can be gone in a few days.” Others also stressed the unsustainable aspect of the time and cost commitments of treatments, one stating, “It is completely unrealistic to think any person, of any age, can drop their life (school, job, parenting etc.) during office hours, to drive to an office, remove make-up and clothing, for an in-office light treatment, re-dress, apply make-up (if applicable) then return to their life, 3 days a week, for months at a time.” Commenters also described undesirable side effects of treatments, such as itching, burning, blisters, and pain on areas of depigmentation.

- **Topical creams.** Several commenters reported using topical creams as part of their treatment regimen, with varying degrees of efficacy. Some noted that the feasibility of using topical creams depended on the area of the body that was depigmented. One commenter noted, “Creams and topical treatments are not useful on the hands, they wash off too easily.” Another stated, “Based on the location of vitiligo, some creams are not safe to use. Others cause other unwanted reactions.” And another commenter observed that their topical cream treatment “is very greasy and results in a multitude of acne.”
- **PUVA and other light therapy.** Many commenters reported using PUVA and other light therapy. Some cited discontinuations of PUVA and other light therapy due to the time and cost associated with the treatments. One commenter discussed challenges with getting insurance coverage for their therapy, “I did the light treatment, and it was working but my insurance company at the time decided to discontinue my treatment. I spent lots of money and time on this treatment for them to discontinue my treatments.”

- **Other therapies.** Commenters discussed using makeup to cover areas of depigmentation. Others had used diet modification to manage their vitiligo.

Submitted comments on ideal treatments

Commenters were generally in agreement on the ideal treatment effect—to permanently regain pigment and stop the spread of any further depigmentation. Others expressed desire for long-lasting makeup products tailored to individuals living with vitiligo. Many also emphasized the importance of the ideal treatment being time- and cost-effective, preferably administered at home. One stated, “I do not mind a daily treatment as long as it can be completed in 10 minutes or less.” Others also expressed the need for further research into the root causes of vitiligo and a treatment that would target the root cause instead of only treating the symptoms. One commenter shared, “Of course a cure is what we all want. I think we at least need a medication that will put us in remission for a longer amount of time than our current treatments. Right now, all treatments are not long lasting, expensive, and time-consuming.” Several commenters shared that they would prioritize their face and hands for treatment since they are the most visible parts of the body. One commenter wrote, “I want the FDA to know that although there are many people comfortable in their skin, there are MANY MANY more who are not and we need medical choices, clinical choices to move science forward with regards to vitiligo.”

Conclusion

This Patient-Focused Drug Development meeting for vitiligo provided FDA the opportunity to hear directly from individuals living with vitiligo and their loved ones about the health effects that matter most, the impact that vitiligo has on daily life, and what factors individuals consider when selecting a treatment. Vitiligo is more than just a cosmetic condition, with serious physical, emotional, and social impacts for individuals living with vitiligo. FDA recognizes the unique perspective patients have to contribute to our understanding of their condition and treatment management, and patient perspectives can play a critical role in informing both drug development and regulatory decision making.

Participants shared perspectives at this public meeting that illustrated the daily challenges and burdens of living with vitiligo. These perspectives emphasized the severe emotional impacts of vitiligo and the need for more effective and convenient therapeutic options to prevent depigmentation or promote repigmentation across all age groups and racial/ethnic groups.

FDA is grateful to the individuals living with vitiligo, as well as their loved ones and representatives, for courageously sharing their personal experiences and perspectives. This public meeting allowed FDA to learn more about what matters most to individuals living with vitiligo and the impacts it has on daily life. As Dr. Kendall Marcus shared during her closing remarks, “This kind of dialogue is important, not just for us, but also for the drug companies, the researchers, and other medical product developers who are also here today... I hope that the information you provided to us today helps to advance the science and development of new treatments for those who would like to have them.” FDA shares the vitiligo community’s desire and commitment to advancing the development of safe and effective treatment options.

Appendix 1: Meeting Agenda

Public Meeting on Patient-Focused Drug Development for Vitiligo

March 8, 2021

10:00 – 10:05 am	Welcome Robyn Bent, RN, MS, CAPT, US Public Health Service <i>Office of the Center Director, Center for Drug Evaluation and Research, FDA</i>
10:05 – 10:10 am	Opening Remarks Kendall Marcus, MD <i>Division of Dermatology and Dentistry, Center for Drug Evaluation and Research, FDA</i>
10:10 – 10:20 am	Overview of FDA’s Patient-Focused Drug Development Initiative Theresa Mullin, PhD <i>Office of the Center Director, Center for Drug Evaluation and Research, FDA</i>
10:20 – 10:30 am	Background on Vitiligo Brenda Carr, MD <i>Division of Dermatology and Dentistry, Center for Drug Evaluation and Research, FDA</i>
10:30 – 10:40 am	Overview of Discussion Format Robyn Bent, RN, MS, CAPT, US Public Health Service <i>Office of the Center Director, Center for Drug Evaluation and Research, FDA</i>
10:40 – 11:10 am	Topic 1: Health Effects and Daily Impacts A panel of patients and patient representatives will provide comments to start the discussion on health effects and daily impacts of vitiligo.

11:10 am – 12:15 pm **Large-Group Facilitated Discussion on Topic 1**

Patients and patient representatives in the audience are invited to add to the dialogue.

12:15 – 12:45 pm **Break**

12:45 – 1:15 pm **Topic 2: Current Approaches to Treatment**

Patient perspectives on current approaches to treatment for vitiligo. A panel of patients and patient representatives will provide comments to start the discussion.

1:15 – 2:20 pm **Large-Group Facilitated Discussion on Topic 2**

Patients or patient representatives in the audience are invited to add to the dialogue.

2:20 – 2:25 pm **Closing Remarks**

Kendall Marcus, MD

Division of Dermatology and Dentistry, Center for Drug Evaluation and Research, FDA

DISCUSSION QUESTIONS

Topic 1: Health effects and daily impacts that matter most to patients

1. Which **aspects** of vitiligo have the most significant impact on your life? (Examples may include depigmentation, itching, sensitivity to sunlight, etc.)
2. 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 vitiligo? (Examples of activities may include participating in social events, sports, being outside in the sunlight, etc.)
 - a. How does your vitiligo and its impacts affect your daily life on the **best days**? On the **worst days**?
3. How has your vitiligo **changed over time**?

- a. How has your vitiligo changed from childhood to adulthood (such as vitiligo severity, disease acceptance)?
 - b. Would you define your vitiligo today as being well-managed?
4. What **worries you** most about your vitiligo?
 - a. Is there a particular body area affected by vitiligo (such as face, body) that is of most concern to you?

Topic 2: Patients' perspectives on current approaches to treatment

1. **What are you currently doing** to help treat your vitiligo? (Examples may include prescription medicines, over-the-counter products, and other therapies including non-drug therapies such as diet modification.)
 - a. How has your treatment regimen changed over time, and why?
2. **How well** does your current treatment regimen treat the most significant aspects of your vitiligo? For example, how well do your treatments improve your ability to do specific activities?
3. What are the most significant **downsides to your current treatments**, and how do they affect your daily life? (Examples of downsides may include bothersome side effects, depigmentation of affected area is more noticeable, going to the hospital for treatment, etc.)
4. Assuming there is no complete cure for your vitiligo, what specific things would you look for in an **ideal treatment** for your vitiligo?
 - a. Is there a particular body area affected by vitiligo (such as face, body) that you would prioritize for treatment?
5. What factors do you consider when making decisions about selecting a course of treatment?

Appendix 2: Patient and FDA Panel Participants

Patient Panel and Discussion Starters, Topic 1

- Sharon M. King
- Tonja Johnson
- Mark Braxton
- Andre
- Jackie Gardner
- Olle Winst
- Alicia Roufs
- Paul

Patient Panel and Discussion Starters, Topic 2

- Jamila Chowdhury
- Priyanka and Vimla
- Lee Thomas
- Carrie and Bella Guarino
- Stephen Taylor
- Jay Hampton
- Beverly Smith
- Meri Izrail Kohen

FDA Panel

- Kendall Marcus (Division of Dermatology and Dentistry (DDD), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER))
- Shari Targum (DDD, OND, CDER)
- Snezana Trajkovic (DDD, OND, CDER)
- Brenda Carr (DDD, OND, CDER)
- Melissa Reyes (DDD, Office of Surveillance and Epidemiology, CDER)

- Laura Marquart (Division of Health Technology 4B (DHTIVB), Office of Health Technology 4 (OHTIV), Office of Product Evaluation and Quality (OPEQ), Center for Devices and Radiological Health (CDRH))
- Yujin Chung (Division of Clinical Outcome Assessments (DCOA), OND, CDER)
- Gavin Imperato (Division of Clinical Evaluation and Pharmacology/Toxicology (DCEPT), Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER))
- Theresa Mullin (Office of the Center Director (OCD), CDER)

Appendix 3: Meeting Polling and Scenario Questions

The following questions were posed to participants at various points throughout the March 8, 2021 public meeting on Patient-Focused Drug Development for Vitiligo. Participation in the polling questions was voluntary. The results were used as a discussion aid only and should not be considered scientific data.

Demographic Questions

1. Where do you live?
 - a. Within Washington, D.C. metropolitan area (including the Virginia and Maryland suburbs)
 - b. Outside of the Washington, D.C. metropolitan area

2. Have you ever been diagnosed as having vitiligo?
 - a. Yes
 - b. No

We will ask that the remainder of the questions be answered by people who responded “yes” to Question 2.

3. What is your age?
 - a. Younger than 8 years old
 - b. 8 – 17 years old
 - c. 18 – 29 years old
 - d. 30 – 39 years old
 - e. 40 – 49 years old
 - f. 50 – 59 years old
 - g. 60 years old or older

4. Do you identify as:
 - a. Female
 - b. Male
 - c. Other

5. Where is your vitiligo located? **Select all that apply.**
 - a. Scalp
 - b. Face and/or neck

- c. Hands and/or feet
 - d. Trunk, arms and/or legs
 - e. Skin folds (such as armpit)
 - f. Genital area (such as groin)
 - g. Other
6. In what pattern does your vitiligo appear?
- a. Focal
 - b. Segmental
 - c. Generalized
 - d. Other
 - e. I'm not sure

Questions for Topic 1: Health Effects and Daily Impacts

7. What aspects of your vitiligo are most bothersome to you? **Please choose up to three answers.**
- a. Pattern of depigmentation
 - b. Amount or extent of depigmentation
 - c. Location of depigmentation
 - d. Loss of hair color
 - e. Skin sensitivity
 - f. Other
8. What do you find to be the most bothersome impacts of vitiligo on your daily life? **Please choose up to three answers.**
- a. Time or cost of daily maintenance
 - b. Refraining from activities
 - c. Self-consciousness or embarrassment
 - d. Bullying or discrimination
 - e. Impact on relationships with family and friends
 - f. Impact on sexual intimacy
 - g. Emotional or psychological impacts
 - h. Other

Questions for Topic 2: Current Treatment Approaches

9. Have you ever used any of the following medical products (drug therapies or medical devices) or interventions to treat your vitiligo? **Check all that apply.**
- a. Topical medications for repigmentation (such as Protopic, Elidel)
 - b. Oral medications (such as prednisone, Xeljanz)

- c. Light treatment (laser, phototherapy, PUVA therapy)
 - d. Surgery (grafting, melanocyte transplants, micropigmentation)
 - e. Topical medications for depigmentation (such as Benzoquin)
 - f. Other
 - g. I'm not using any medical products or interventions
10. Besides the medical products or interventions mentioned previously, what else are you doing to manage your vitiligo? **Check all that apply.**
- a. Temporary cosmetic measures
 - b. Dietary and herbal supplements
 - c. Diet modifications
 - d. Over the counter products
 - e. Complementary or alternative therapies
 - f. Counseling or psychological treatment
 - g. Other
 - h. I am not doing or taking any therapies to treat my vitiligo
11. For the medical products or interventions you use, what do you consider to be the most burdensome impacts? **Please choose up to three answers.**
- a. How the medication is administered
 - b. Change in the pigmentation of my skin
 - c. The treatment only provides minimal benefit
 - d. The treatment is effective only for a short-term
 - e. Bothersome side effects of the treatment
 - f. Concern about serious risks of the treatment
 - g. Uncertainty about long-terms effects of treatment
 - h. Difficulty in accessing treatment
 - i. Other

HYPOTHETICAL SCENARIO 1: NEW SKIN CREAM

Imagine that a new topical cream (not a steroid) indicated to treat vitiligo has recently been approved by FDA. Your doctor believes that you/your loved one may be a good candidate for this medication.

The topical cream needs to be applied once a day. Research in animals and people show that this treatment may improve pigmentation by up to 50% in some people. More common side effects of this therapy may include redness and irritation at the application site, mild acne, and burning. Rarer but more serious side effects may include fever, pain during swallowing, insomnia, nausea, and increased heart rate.

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

12. Given the risks and benefits, would you take this medication?

- a. Yes
- b. No
- c. Maybe

HYPOTHETICAL SCENARIO 2: CLINICAL TRIAL FOR A NEW DRUG

Imagine that you have been invited to participate in a clinical trial to study an experimental treatment for vitiligo. Your doctor believes that you may be a good candidate for this clinical trial.

This experimental treatment is an oral immunosuppressant. Early research in animals and people show that this treatment may improve pigmentation by up to 50% in some people after 1 year. The purpose of this study is to better understand how well this treatment works and its safety.

More common side effects of this therapy may include headache, diarrhea, and pain in the sinuses (such as nose, forehead, cheeks). Rarer but more serious side effects may include stomach/abdominal pain, liver problems, or cancer. This clinical study will last 18 months and clinic visits will occur every 3 months. Visits will involve routine blood work.

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

13. Based only on the information presented in the scenario, would you consider participating in this clinical trial? Please choose one response.

- a. Yes, I would consider participating in this study
- b. No, I would not consider participating in this study
- c. I'm not sure