Sickle Cell Disease
Patient-led Listening Session

May 05, 2023
56 FDA attendees

OBJECTIVES

1. We hope to continue to engage with the FDA and amplify the Sickle Cell patient voice in future initiatives.
2. We would like to engage with supporting the development of guidance for stakeholders interested in supporting the needs of this underserved population.
3. We aim to reduce healthcare disparities in Sickle Cell by providing a space for the lived experiences of this population to support medical product advances, guidance for special considerations for enrolling patients or conducting trials and much more.

TOPICS DISCUSSED (SHARED IN ORDER OF AGENDA)

Mary Brown, President & CEO of Sickle Cell Disease Foundation (SCDF) opened our session sharing the history of SCDF, its purpose and initiatives to support people living with sickle cell such as:

- Health Education, Behavioral Services
- Teen and residential summer camps
- Projects such as Networking California for Sickle Cell Care (NCSCC) and YAAM Patient-Reported Outcomes service

Regina Hartfield President & CEO of Sickle Cell Disease Association of America (SCDAA) then shared the most critical challenges of people living with sickle cell disease (SCD) which include: discrimination/health disparities, access, sustained employment, minimal or no health insurance coverage, and mental health issues. She made clear that the SCD community can be supported by being informed and have access to cutting-edge therapies and more therapy options generally. SCDAA encouraged the FDA to:

- Fast track SCD treatments
- Educate the SCD community regarding FDA's Priority Review, Orphan drug, and Breakthrough Therapy programs
- Increase communications to the community
- Involve SCD patients early and often
- Partner with SCDAA and CBOs to increase awareness of FDA activities

Teonna Woolford, CEO of Sickle Cell Reproductive Health Education Directive (SC RED) then shared SC RED's efforts and the importance of addressing the issues affecting the SCD community relating to reproduction health. These include:

- Lack of contraceptive research
- No disease modifying therapy for pregnant women with SCD

She recommended that FDA approval for gene therapies should require the co-administration of fertility preserving interventions. And stressed that long term follow up for gene therapies is essential to establish cancer or infertility risks especially for young children undergoing these treatments.

Then people living with sickle cell and a caregiver shared their experiences with sickle cell treatment and care.

Living with sickle cell includes:

- Absence from school, work and daily life. A caregiver reported that her daughter has been unable to work for the last four years creating financial challenges.
- Treatments that don't always work or stop working after some time
- Monthly blood transfusions. A person living with Sickle Cell Disease shared that when he needs a transfusion, he has to have 2 large gauge needles in the veins in his arm, taking out red blood cells and replacing red blood cells. The total process takes 3 hours. Because of the that, he has to navigate taking time off of work and school.
- Mistreatment or under treatment. Two people living with sickle cell disease shared their experiences regarding long waits in the emergency room while the pain worsens and being over medicated. A person living with sickle cell disease shared her experience with a medical provider in which the doctor did not believe the person was experiencing medication side effects. This experience led to distrust of the medical professional.

Caring for someone living with sickle cell can also be a challenge as it is a genetic condition with no cure.

- Uncertainty of pain crisis creates challenges to plan for family events or outings resulting in emotional strain and can damage relationships
- Caregivers live with the constant fear that their loved ones may fall ill and are sometimes required to care for loved ones after major medical procedures– this can lead to absenteeism at work or asking for extended leaves with no job security
- Caregivers need to be included in drug trials and development as they play a critical role in disease management

People living with sickle cell are often trying to manage their condition at home to avoid the stigma and stress that ER visits create. They need innovative tools and devices that help them to translate their symptoms into actions that can be taken on their own to keep them out of the hospital and thrive in their daily lives.

Metrics for success of new treatments should include the patient voice according to those people who have tried multiple treatments to help manage the symptoms of sickle cell. A person living with sickle cell voiced the need for FDA to consider how to change quality of life measure that are important to patients and not just biomarker measures. Patients are measuring not only pain relief, but quality of life including physical effects such as hair loss or face rashes when considering new options for treatment.

Clinical trial experiences can vary, but patients want opportunities to learn more about clinical trials from trusted sources so that they can make a decision on whether to join. A person living with sickle cell encouraged FDA to require developers to include diverse

and underserved populations in clinical trials and support services for trial participants. She also mentioned that she has had opportunities to share experiences with pharma teams before trial development and involvement with the planning and design of trials which she feels are greatly valuable.

Sick Cells President & Co-Founder Ashley Valentine, highlighted the need to raise the voice of the sickle cell disease (SCD) community in research, policy, and advocacy. Ms. Valentine shared that the SCD pain experience is broad and must also be measured outside of the clinical experience and should include patient-important outcomes to ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. She listed the top five health effects of sickle cell disease with greatest impact on patient's life as chronic daily pain, fatigue, acute pain crises, cognitive impairment and iron overload.

Through previous research, Sick Cells collected SCD patients' top three areas of interest: (1) Reducing risks of organ damage; (2) Shortening the length of pain crises, and (3) Reducing stroke risk. Recommendations from Sick Cells for the FDA included:

- Increase clinical trial opportunities for older individuals with SCD;
- Recognize the need for increased linguistically diverse populations in clinical trials;
- Encourage use of community partnerships for collecting and communicating data; and
- Collaborate across federal agencies to improve patient access to FDA-approved therapies.

QUESTION & ANSWER SESSION

Q: What aspects of a clinical trial would make you more/less likely to participate?

A: There are multiple challenges with sickle cell patient involvement of clinical trials. Most often patients have to go to their provider to find information, but there is a breakdown in patient/provider communication. Patients are challenged to find information about clinical trials and are currently finding out only if they interact with Community Based Organizations (CBOs) or if they are talking to other people living with sickle cell. Also, trial information is often provided only in English and should be translated into Spanish as well.

Others discussed obstacles of sickle cell patient clinical trial involvement include:

- Providers not sharing information or not aware of trials
- Misconceptions and misperceptions about clinical trials and research (terminology clarification for example would be immensely helpful in increasing participation)
- Access to trial locations (some patients have to travel 4 hours to a sickle cell center for a trial)

It was also mentioned that listening to patients, incorporating their concerns makes a huge impact in their treatment. The pharmaceutical industry should be encouraged to include patients in the design of clinical trials as oftentimes the patient voice is incorporated too late in the process.

PARTNER ORGANIZATIONS

FDA DIVISIONS REPRESENTED

Office of the Commissioner (OC) – 6 *offices*

- OC/OCPP/PAS – Office of Clinical Policy and Programs/ Patient Affairs Staff *(organizer)*
- OC – Office of the Commissioner
- OC/OCPP/OOPD – Office of Clinical Policy and Programs/Office of Orphan Products Development
- OC/OCPP/OCP – Office of Clinical Policy and Programs/Office of Combination Products
- OC/OCPP/OPT – Office of Clinical Policy and Programs/Office of Pediatric Therapeutics
- OC/OCS/ACOMS – Office of the Chief Scientist/Advisory Committee Oversight and Management Staff

Center for Biologics Evaluation and Research (CBER) – *5 offices/divisions*

- CBER/OBPV/DABRA/BRAB – Office of Biostatistics and Pharmacovigilance/Division of Analytics and Benefit-Risk Assessment/Benefit-Risk Assessment Branch
- CBER/OCD – Office of the Center Director
- CBER/OCD/PS – Office of the Center Director/Policy Staff
- CBER/OTP/OCE/DCEH/BHB – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Clinical Evaluation Hematology/Benign Hematology Branch
- CBER/OTP/PSPS – Office of Therapeutic Products/Policy and Special Projects Staff

Center for Drug Evaluation and Research (CDER) – *7 offices/divisions*

- CDER/OCOMM/PASES – Office of Communications/Professional Affairs and Stakeholder Engagement
- CDER/OND/OCHEN/DNH – Office of New Drugs/Office of Cardiology Hematology Endocrinology and Nephrology/Division of Non-Malignant Hematology
- CDER/OND/ODES/DCOA – Office of New Drugs/Office of Drug Evaluation Science/Division of Clinical Outcome Assessment
- CDER/OND/ORDPURM/DRDMG – Office of New Drugs/ Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine/ Division of Rare Diseases and Medical Genetics
- CDER/OPQ/OLDP/DLBPI/LBB1—Office of Pharmaceutical Quality/Office of Lifecycle Drug Products/Division of Liquid-Based Products I/Liquid-Based Branch 1
- CDER/OTS/OB/DBII – Office of Translational Sciences/Office of Biostatistics/Division of Biometrics II
- CDER/OTS/OB/DBIX – Office of Translational Sciences/Office of Biostatistics/Division of Biometrics IX

Center for Devices and Radiological Health (CDRH) – *8 offices/divisions*

- CDRH/OPEQ/OHTI/DHTIA – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology I A
- CDRH/OPEQ/OHTI/DHTIB – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology I B
- CDRH/OPEQ/OHTI/DHTIC – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology I C
- CDRH/OPEQ/OHTIII - Office of Product Evaluation and Quality/Office of Health Technology III
- CDRH/OPEQ/OHTIII/DHTIIIA – Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology III A
- CDRH/OPEQ/OHTIII/DHTIIIB – Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology III B
- CDRH/OPEQ/OHTIII/DHTIIIC – Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology III C
- CDRH/OSPTI – Office of Strategic Partnerships and Technology Innovation

PATIENTS AND CAREGIVERS REPRESENTED

5 people living with sickle cell were represented and 1 mother of 2 daughters living with sickle cell was present to provide the caregiver perspective.