THE VEDS MOVEMENT

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Vascular Ehlers-Danlos Syndrome (VEDS) - Patient-led Listening Session
10/07/2020

Objective of session

The objective of this session was to provide the FDA staff with an opportunity to hear and understand patient perspectives about living day-to-day with VEDS, needed treatment options, and what the community feels would be meaningful treatment outcomes. As a community, we wanted to express the physical and mental and toll this takes on patients and generations of families, as well as the frustration with the lack of medical knowledge and management.

Summary of topics discussed

Symptoms and life-threatening emergencies – Among the speakers, several have experienced life-threatening emergencies and shared their experiences living through these emergencies. They shared their anxieties about living with the threat of an emergency that can occur at any time, as well as symptoms such as bruising, injuries, and coughing up blood regularly.

Parenting a child with VEDS- Parents shared their experiences having children with VEDS, both from perspectives of those who do not have VEDS themselves and those that do. Overarching themes were of wanting to protect their children, while trying to balance the desire for their children with VEDS to have the opportunity for a fulfilling life. Frustrations with having to be the experts who direct their children's medical care rather than having physicians who are able to be the experts were also expressed.

Living as an adult with VEDS- The speakers expressed what it feels like living with a condition that can cause life-threatening emergencies, daily pain, gastrointestinal problems, bruising, scarring, and frequent injuries, as well as their experiences with the medical system, insurance, loss, challenges growing up with the condition and coping with child-bearing decision making.

Patient experiences with medical care and quality emergency care- The overarching theme here was frustration with medical care, including the feelings of being treated like a hypochondriac, confusion among medical professionals of VEDS with other non-life-threatening conditions, not being taken seriously in emergencies, and the fear of dying in the emergency department waiting room. Being passed from doctor to doctor, with resulting growing medical bills even if the physicians provide no help was found to be disheartening. But doctors who are willing to learn or help can make a difference can make a big difference.

Living with the threat of an untimely death - Speakers shared what it is like living with the threat of a spontaneous, life-threatening emergency that can happen at any time, including feelings of anticipation of these emergencies, impact on their loved ones, and what it means for their day-to-day lives and plans.

Impact of losing friends and family to this condition- The speaker for this section has lost five family members to VEDS, and everyone on the call has lost family and/or friends to VEDS as well. Losing friends and family to VEDS is devastating, and the feelings of loss are compounded by also knowing your own diagnosis or child's diagnosis. These losses can trigger feelings of sadness, fear or anxiety repeatedly and serve as a reminder of one's own mortality over and over again.

Moment of silence for those lost to VEDS – The community and listeners on the call shared a moment of silence and remembrance of those who have lost their lives to VEDS.

Treatment: Frustrations and Desires- Current recommendations on treatments, exercise, and monitoring for new aneurysms or dissections are inconsistent. Without sufficient evidence, management guidelines are based on expert opinions, which can differ. Managing relatively normal complications for most of the population- ie. cancer screenings, colds, injuries, and reproductive health- can be complicated and confusing, as many procedures and medications used for the general population are considered unsafe for those with VEDS, including, for instance, common antibiotics, cold medications, birth control, and pain medications. This leaves people with VEDS with limited options. The group here today considers a successful preventative treatment of life-threatening complications to be one that is proven effective and affordable. Development of treatments that are safe for those with VEDS for other struggles, such as gastrointestinal pain, dysmenorrhea, hormone therapy, menopause, musculoskeletal pain, are also considered vital to the people who spoke during this call to help improve quality of life. Finally, prevention of life-threatening events is paramount, but improvement in medical education and in the current emergency treatment standards to help those with VEDS survive when emergencies do arise are also vital.

Current state of research, support, and education- There have been studies overseas on the drug celiprolol, which is widely used in Europe and was recently denied approval by the FDA. The PCORI-funded VEDS Collaborative has been working on patient-centered research in VEDS, and recent mouse model studies have discovered a pathway for potential drug development to prevent life-threatening arterial and aortic events.

Partner organization
The VEDS Movement, division of The Marfan Foundation

FDA Offices Represented (17 offices/divisions):

Office of the Commissioner (OC):
- Office of Combination Products
- Office of Orphan Products Development
- Patient Affairs Staff
- Office of Pediatric Therapeutics

Center for Biologics Evaluation and Research (CBER)
- Office of the Director
- Office of Compliance and Biologics Quality/Division of Inspections and Surveillance/ Program Surveillance Branch

Center for Drug Evaluation and Research (CDER)
- Office of New Drugs/ Office of Immunology and Inflammation/Division of Dermatology and Dentistry
- Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine / Division of Rare Diseases and Medical Genetics (DRDMG)
- Office of New Drugs, Office of Cardiology, Hematology, Endocrinology and Nephrology, Division of Cardiology and Nephrology

Center for Devices and Radiological Health (CDRH)

- Office of Strategic Partnerships and Technology Innovation/ Division of All Hazards Response, Science and Strategic Partnerships
- Office of Product Evaluation and Quality
- Office of Product Evaluation and Quality/ Office of Health Technology/ Division of Health Technology
- Office of Product Evaluation and Quality/ Office of Health Technology 2/ Division of Health Technology 2 C
- Office of Product Evaluation and Quality/Office of Regulatory Programs
- Office of Product Evaluation and Quality/ Office of Health Technology 2/ Division of Health Technology 2 B
- Office of Product Evaluation and Quality/ Office of Health Technology 3/ Division of Health Technology 3 B
- Office of Product Evaluation and Quality/ Office of Health Technology 3

Non-FDA Organizations (2):

- Reagan Udall Foundation for the FDA
- European Medicines Agency

Patients and community members represented

- Sarah Jeffs, mother of a child with VEDS
- Aaron Sander, person living with VEDS
- Emma Borreggine, person living with VEDS
- Meg Boeglin, person living with VEDS and mother of two children with VEDS
- Jeremias Tays, person living with VEDS and father of two children with VEDS
- Kristi Posival, person living with VEDS
- Katie Wright, person living with VEDS and Director of The VEDS Movement
- Josephine Grima, Chief Scientific Officer of The Marfan Foundation

Disclaimer

Discussions in FDA Rare Disease Listening Sessions are informal. All opinions, recommendations, and proposals are unofficial and nonbinding on FDA and all other participants. This report reflects the [organization]'s account of the perspectives of patients and caregivers who participated in the Rare Disease Listening Session with the FDA. To the extent possible, the terms used in this summary to describe specific manifestations of [disease or condition], 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 [disease or condition] patient population or any specific group of individuals or entities. There may be experiences that are not mentioned in this report.