

COMPASS

A Publication Dedicated to Research Updates | **FALL 2017**

Cure SMA Launches Phase II of the SMA Industry Collaboration

The SMA Industry Collaboration is a multi-faceted partnership that brings together pharmaceutical companies, Cure SMA, and other nonprofit organizations, to share information, ideas, and data.

The SMA Industry Collaboration works together to address scientific, clinical and regulatory topics that are critical to advancing drug development in SMA and will benefit the broader SMA community. The members of the SMA Industry Collaboration, which are currently all engaged in drug development for SMA, are Astellas, AveXis, Biogen, Genentech/Roche, Cytokinetics, and Ionis.

Collaborative industry groups like this one are a rare opportunity to advance goals important to the SMA community. We thank our industry partners for their example of “the strong sense of collaboration that is helping to bring therapies for SMA to patients and families,” as Dr. Jonathan Goldsmith of the FDA recently remarked at the SMA Patient-Focused Drug Development Meeting.

In the first phase of the collaboration, the group coalesced around three major topics of importance to our community: engagement with the FDA and other regulatory agencies, increased education and awareness to parents and families and the medical community to reduce time to diagnosis, and increasing clinical trial site capacity. In phase II, we are actively implementing specific projects in each topic area.

The Industry Collaboration Joint Steering Committee works closely with Cure SMA to evaluate and prioritize activities that would advance the goals of these topic groups. The committee helps to prioritize and fund specific activities and projects to be undertaken by Cure SMA in partnership with each of three designated topic groups.

Education Topic Group Projects

The education topic group is working on expanding awareness of SMA among families and caregivers as well as pediatricians and other healthcare providers. This topic group will work with stakeholders, families, parents and caregivers and health care professionals, to improve recognition of SMA, to shorten the time to diagnosis and avoid unnecessary delays in beginning treatment.

More specifically, Cure SMA will be working closely with the American Academy of Pediatrics (AAP) to promote education and awareness of SMA. Additionally, Cure SMA is currently working on creating an online resource on our website targeting parents with concerns about their child’s motor development. The goal of this site will be inform and empower parents to identify key signs and symptoms of motor delays, prior to diagnosis, to equip them with the resources and language to effectively address their concerns with their pediatrician. The ultimate goal is to promote early recognition and diagnosis of SMA, enabling parents and doctors to target interventions that would lead to improved outcomes.

Clinical Trial Topic Group Projects

The clinical trials group is focused on identifying and pursuing opportunities to increase sites’ ability to conduct new trials and enroll and follow more patients, in light of anticipated increases in the number of trials being conducted within the

For more information on the current clinical trial’s news visit
www.cureSMA.org



United States and optimize readiness throughout the US. The goal is to improve our community’s access to clinical trial sites and increase capacity. In this phase, the topic group will focus both on assessing and improving existing clinical trial networks, as well as reaching out to potential new SMA clinical trial sites.

Regulatory Topic Group Projects

The signature accomplishment of this topic group has been the execution of a successful Patient Focused Drug Development Meeting with the FDA. Currently, the regulatory group is overseeing the development of the Voice of the Patient (VoP) Report, a follow-up from the PFDD meeting summarizing key learnings on the impact of SMA on individuals and families, and their expectations and priorities for current and future treatments. This report will be used to inform the decisions made regarding future therapies in SMA.

“I have heard of your commitment and love for your children; your courage and determination as adults, older children and teens; and how you maintain hope and unity.”

– Jonathan Goldsmith, MD
Associate Director for Rare Diseases, CDER, FDA

The topic group is also currently conducting a benefit-risk survey throughout the SMA community to better understand how individuals and families with SMA make risk assessments when coupled with specific benefits of a treatment. The learnings from this analysis will create a compelling case for the assessment and ultimate approval of new therapeutics. An analysis of this survey will be submitted along the VoP report to the FDA. This topic group will also work on plans for future engagement with the FDA.

Highlights and Results from Patient Focused Drug Development Meeting with FDA

On April 18, 2017, Cure SMA hosted an externally led Patient-Focused Drug Development (PFDD) meeting to share with the FDA and other stakeholders the perspectives of people living with SMA, its impact on their daily lives, and their expectations and priorities for current and future treatments for treating

SMA. This meeting with the SMA community was only the second externally led PFDD meeting to be granted.

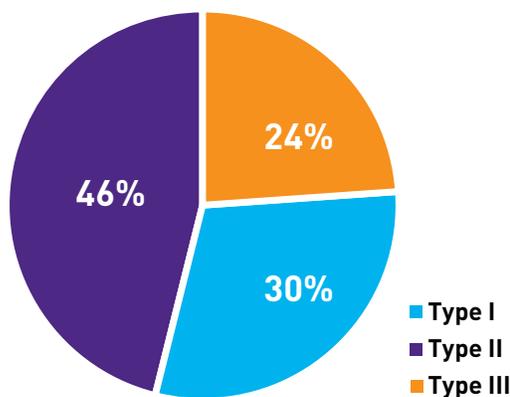
Meeting Overview

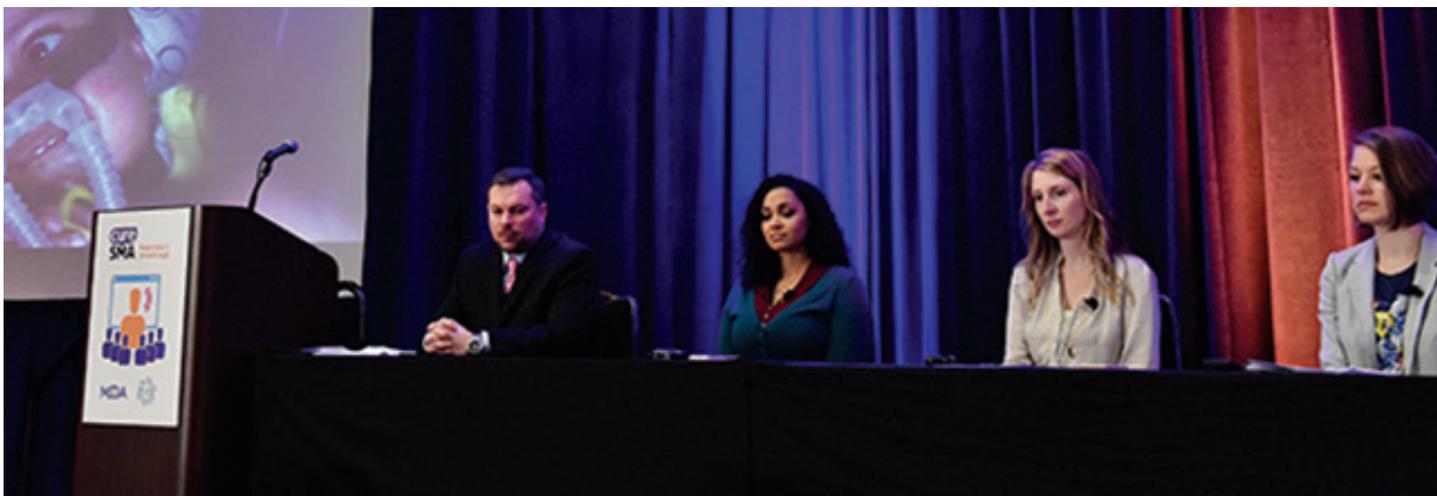
422 individuals registered for the meeting (204 in person, 218 via webcast). Sixteen members of the FDA attended the meeting (13 in person, 3 online) to hear directly from the patients, caretakers, and other patient representatives about patients’ experiences with SMA and the available treatments and management approaches. FDA attendees included Dr. Peter Marks, Director of the Center of Biologics Evaluation and Research (CBER) together with representatives from the Center of Drug Evaluation and Research (CDER), the Division of Neurology Products including Dr. William Dunn, Director of the Division of Neurology Products, and Dr. Jonathan Goldsmith, Associate Director for Rare Diseases, Office of Tissues and Advanced Therapies. The meeting also included presentations from clinical experts to provide a wider context on SMA.

After an initial introduction, twenty panelists representing all types, ages and stages of SMA brought their voices and stories to depict the impact of SMA upon their lives. The day was divided into a morning session on the burden of disease and unmet needs in people with SMA and their families, and an afternoon session focused on patient perspectives on treatment, including the benefit-risk balance of treatment options. Each round of panelists was followed by polling questions and a period of facilitated discussion with the participants at the meeting and from across the US and the world, via our live streaming webcast. In the morning, questions focused on disease symptoms and daily impact that matter the most to patients. In the afternoon, questions focused on patient and caregiver perspectives on current and future approaches to treatment and on clinical trial participation.

During the Patient-Focused Drug Development meeting, polling questions were posed to gather data on the burden of SMA and perspectives on treatment to both in-person participants and those watching online. The results were used as a discussion aid and to help gain a better understanding of the full impact of the disease.

Breakdown of PFDD Participants SMA Type





Panel 1 – Type I on disease impact

Meeting Findings:

- SMA has a far-reaching impact on the lives of individuals with SMA and that of their parents, caregivers and families.
- Individuals with SMA and their families experience deep emotional, psychological, relational and financial burdens due to SMA.
- Having and caring for a child with SMA type 1 or 2 is more than a full-time commitment, usually involving careful scheduling of events for the child, their caregivers and therapists, nursing help and night-time help.
- For those with SMA type 3, the commitment is not initially as constraining but as the child's abilities decrease, the demands on the parents become greater.
- As muscular function decreases, the list of things the child is no longer able to do mounts and eventually their level of independence is decreased.
- For caregivers as well patients, anything that increases or decreases the child's independence is acutely meaningful.

Impact of SMA Type II & III: Symptoms that Matter Most to Families

- Fatigue and muscle weakness affecting mobility and function
- Loss of upper body / core strength
- Fatigue limiting the amount activity per day
- Difficulty chewing or smiling
- Increasing caregiver dependence due to loss of ability to perform activities of daily living
- Joint contractures and severe scoliosis
- Respiratory complications
- Falls or fear of falling
- Loss of sleep or sleep problems
- Emotional impact, such as anxiety, depression, and isolation

Impact of SMA Type I: Symptoms that Matter Most to Families

- Respiratory difficulties:
 - Inability to cough and clear lung secretions
 - Respiratory failure requiring assistive devices
 - Respiratory infections
- Feeding and swallowing difficulties
- Progressive muscle weakness leading to complete loss of mobility
- Communication difficulties
- Complete dependency (the need for one-on-one care 24 hours a day)
- Emotional impact including; anxiety, depression, and social isolation

“We want to make sure we understand the impact of the disease and what patients prioritize in the treatment of their disease”

– Dr. Billy Dunn MD
Director, Division of Neurology Products, CDER, FDA

What Issues should a potential treatment address?

Type I

- Ability to speak, communicate how child feels
- Management of secretions
- Respiratory Complications – “being less dependent on machines”
- Upper body strength/diaphragmatic weakness
- Respiratory Complications (type II)
- Stopping disease progression, retaining mobility and function (stabilizing disease)

Type II/III

- Small changes have HUGE impact
- Fatigue
- Increased function – stronger arms, legs, spine
- Endurance

All Types

Conclusions

This meeting emphasized the urgent need for increased awareness, early diagnosis, and available treatments for SMA. Furthermore, the FDA was provided with a unique opportunity to hear directly from patients at this Patient-Focused

Drug Development meeting and to better appreciate their physical and emotional burdens related to living with spinal muscular atrophy. Presentations by renowned SMA researchers provided a unique insight into the complex issues faced by clinicians and scientists in developing better treatments for this disease. We are grateful to the patients and their representatives and to the physicians and scientific experts who participated.



Outstanding Group of Panelists Chosen to Represent the Voices of SMA

For more information on the current clinical trial's news visit www.cureSMA.org/currenttrials

Non-Profit Org.
U.S. Postage
PAID
Chicago, IL
Permit No.
1705

Cure SMA
925 Busse Road
Elk Grove Village, IL 60007
1.800.886.1762
Return Service Requested

