

Understanding the Holistic Rare Disease Patient Experience Through a Community Advisor Program



Jeffrey Smith¹, Gretchen Hover², Kelly Damm¹, Janet Chien²

¹Wave Life Sciences Ltd., Lexington, Massachusetts, USA; ²Imbue Partners LLC, Middleton, Massachusetts, USA

Conclusions

- A community-focused approach was developed to offer families of patients with Duchenne muscular dystrophy (DMD) the opportunity to highlight their unique experiences and establish meaningful connections with other members of the DMD community
- Through this approach, an open, caring environment was created, which enabled:
 - Members of Wave Life Sciences to gain deeper insight into the challenges faced by patients with DMD and their families, and to create opportunities to inform the development of new medicines and services for families
 - Community advisors to voice their experiences and to contribute in a way that has a clear impact on other families with DMD and on programs at Wave Life Sciences
 - Community advisors to better understand the drug development process and ways they can contribute to developing new medicines and services for families
- Insights gained through this community-focused approach may be valuable in developing therapies and enhancing the patient experience for DMD, and potentially for other illnesses

Introduction

- Understanding the holistic experience of individuals and families living with a rare disease, such as DMD, is vital to the development of new therapies, programs, and services that meet the needs of families
- DMD is an inherited disorder of progressive muscle weakness that takes a significant toll on physical, psychological, and social well-being, as well as on the overall quality of life of patients and their families¹⁻³
- Patients with DMD and their families have the potential to play an important role in informing the development of new therapies and programs that improve quality of life and facilitate the delivery of optimal care
 - Patient advocacy plays an important role in DMD, and one advocacy group, the Parent Project Muscular Dystrophy, developed and submitted a draft guidance document for industry on DMD to the US Food and Drug Administration (FDA)⁴
 - However, engagement with patients and families with DMD for clinical development is lacking, with only one study reporting on caregivers' knowledge, attitudes, and perceptions of engagement in clinical research in DMD⁵
- Presented here are learnings from a community advisor program, a valuable initiative intended to engage families of patients with DMD in a manner that enables caregivers to provide meaningful contributions to the drug development process

Objective

- To describe learnings from a community advisor program, which was intended to engage families with DMD, understand patient/family needs and perspectives, and integrate learnings into the drug development process at a biopharmaceutical company, Wave Life Sciences

Methods

Participants

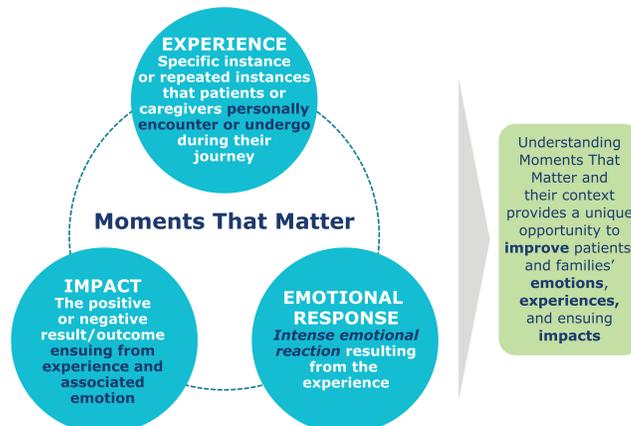
- Families (eg, parents or caregivers) of patients with DMD representing diverse backgrounds and experiences were identified in partnership with patient advocates and patient advocacy organizations and invited to serve as community advisors
- Members from various functions of Wave Life Sciences also participated in these meetings

Community Advisor Meeting Design

- The advisor meetings were structured to build trust and facilitate open communication between families affected by DMD and members of Wave Life Sciences

- Pre-meeting interviews were conducted to obtain background information, set expectations, and initiate the trust-building process
- Pre- and post-meeting surveys were conducted, and a follow-up engagement plan was prepared
- The advisor meeting agenda included workshops focused on:
 - Learning about living with DMD by defining "Moments That Matter"
 - Moments That Matter is composed of: 1) an intense experience or defining moment that causes 2) a visceral emotional response resulting in 3) a long-term impact for a patient, caregiver, or their family (Figure 1)
 - Moments That Matter were defined using storyboarding, a participatory, experiential method that allows advisors to rephrase their significant/critical moments to:
 - Share their story while tapping into a creative space
 - Find similarities and common threads with fellow advisors while holding on to their uniqueness
 - Identify unmet needs
 - Advisors were grouped into teams and asked to co-create "Living With Duchenne" storyboards, in which imagery, phrases, and symbols were chosen to capture their experiences, feelings, and challenges
 - As a large group, the advisors and members of Wave Life Sciences worked together to co-create opportunities to address key Moments That Matter
 - Current and future DMD care
 - Advisors were asked to share their perspectives on the current standard of care for DMD and their expectations for the future
 - Identifying communication preferences
 - Advisors were asked to share their preferred sources of information and/or channels for sharing information with the community

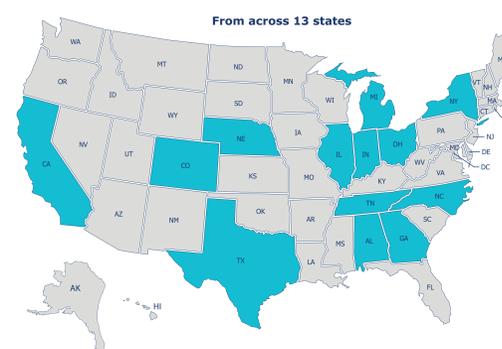
Figure 1. Pillars of Moments That Matter



Results

- Three 1-day community advisor meetings were held in Cambridge, Massachusetts; Detroit, Michigan; and Dallas, Texas from August to October 2019
- Thirty parents participated, representing 36 children with DMD (35 boys, 1 girl), with diverse backgrounds and experiences (Figure 2)

Figure 2. Advisor Characteristics



DMD, Duchenne muscular dystrophy.

Key Insights

- Insights from storyboards co-created by advisors related to the often-difficult path to diagnosis, sacrifices during treatment and their child's loss of ambulation, family strain, coverage for services and treatments, school support, and efforts to enjoy and live in the moment (Figure 3)
- Advisors shared their perspectives and feedback on clinical trials, including topics surrounding trial design, communication of results, informed consent, and education on the basics of a clinical trial

Figure 3. Key Themes That Resonated Across the 3 Community Advisor Meetings



DMD, Duchenne muscular dystrophy; FDA, Food and Drug Administration; HCP, health care provider; OT/PT, occupational therapy/physical therapy; PAO, patient advocacy organization.

References

- Uttley L, et al. *Health Qual Life Outcomes*. 2018;16:237.
- Landfeldt E, et al. *Develop Med Child Neurol*. 2018;60(10):987-996.
- Uzark K, et al. *Pediatrics*. 2012;130(16):e1559.
- Furlong P, et al. *Orphanet J Rare Dis*. 2015;10:82.
- Bendixen RM, et al. *Clin Ther*. 2016;38(6):1474-1484.

Acknowledgments

This work was funded by Wave Life Sciences Ltd. We thank all parents who attended the advisor meetings and shared their valuable insights, and their families, for supporting this work. We also thank Disha Patel, PhD (Chameleon Communications International with funding from Wave Life Sciences Ltd.) for editorial assistance in the preparation of this report.

Disclosures

Jeffrey Smith: Employee of Wave Life Sciences USA, Inc. **Gretchen Hover:** Employee of Imbue Partners LLC. **Kelly Damm:** Employee of Wave Life Sciences USA, Inc. **Janet Chien:** Employee of Imbue Partners LLC.

- Following the community advisor meetings, Wave Life Sciences identified new approaches for addressing the unmet needs identified by the DMD community advisors. Key recommendations focused on community education (eg, drug development process, clinical trials); data sharing with the community (eg, white papers, guidelines); using multilingual communication and technology to improve outreach; and forming a coalition to facilitate research collaborations and community education/engagement

Advisor Feedback on the Community Advisor Meetings

- Advisors viewed the meetings as a unique opportunity to share their voice with a biopharmaceutical company and to have a meaningful impact on the development of new therapies
- The meetings were also considered to be an important opportunity to meet other families living with similar challenges
- Of the 30 participants, 21 (70%) responded to a post-meeting web-based survey (Figure 4)
 - The survey revealed that even in a rare disease with highly active and sophisticated advocacy organizations, families have very little interaction with drug companies and would like greater cooperation and participation

Figure 4. Post-meeting Survey Feedback

