Advocacy Letter

Hello Duchenne Advocacy Community,

This quarter, Sarepta had the great opportunity to meet and learn from Alan Chaulet, Vice President of All Wheels Up, an organization working to promote wheelchair accessible airplane travel. Their work has resonance. Challenging boundaries, thinking expansively, and creating meaningful change is something that we strive to do in our work for the Duchenne Community every day.

To that end, we have been moving our drug development programs forward as quickly and safely as possible. Our first PPMO drug candidate for individuals amenable to exon 51 skipping is enrolling (NCT03375255), preparation of the golodirsen new drug application is underway with the aim of completing submission in late 2018, and our DMD gene therapy partnerships are advancing with multiple individuals dosed. Unfortunately, our plan to bring eteplirsen to patients in Europe has met a hurdle in the regulatory process. As stated in our last newsletter, drug development is not linear or highly predictable as evidenced by the negative CHMP opinion. Our thoughts are with the families impacted by this decision, and we are working with the regulatory bodies in Europe to determine a path forward.

In other news, we recently announced a new gene therapy collaboration focused on Limb Girdle Muscular Dystrophy (LGMD), marking our first venture outside Duchenne. The company is excited to learn more about LGMD and to support the progress made by Myonexus, our partner. Importantly, this expansion does not change our commitment to Duchenne. As Doug, our CEO, often says – we are a Duchenne-focused company, and this will not change.

For the first time, Sarepta will be hosting an R+D day for investor audiences on June 19, 2018. This is another platform to bring awareness to DMD and to highlight Sarepta's approach for creating meaningful change in the lives of those impacted. The R+D day will be live-streamed, and we will share the link with you in advance.

Additionally, we would like to invite Advocacy leaders to join us for a town hall teleconference following R+D day. Your thoughts and questions are important to us as we shape the future as a company and further partner with the Duchenne community.

Thank you for your continued partnership!

A Look Inside

Sarepta is headquartered in Cambridge, MA, with a manufacturing facility in nearby Andover, and European headquarters in Switzerland. We are growing rapidly. Also joining us this summer are an eagerly awaited group of undergraduates who will be interning in various departments, including Advocacy!

Where We've Been & Where We're Going

Meeting and connecting with the Duchenne community is at the heart of everything we do. This spring, we have been privileged to interact with you at events sponsored by: CDMD at UCLA, CureDuchenne, Duchenne Parent Project Spain, EURODIS, JB's Keys, Jett Foundation, Life Science Cares, MDA, NORD, and Parent Project Muscular Dystrophy (PPMD).

We look forward to meeting with you in person this summer. We will continue to attend Advocacy-led family workshops run by CureDuchenne, Jett Foundation, and PPMD across the US. We will be participating in PPMD's annual conference, and are looking forward to additional events throughout the world. We also will be attending MDA muscle walks and galas periodically across the country.

As always, please let us know if you are in the Boston area and wish to meet with us and visit Sarepta's offices!

Alan Chaulet, Vice President of All Wheels Up, joined Sarepta to speak about the major issue of accessible airplane travel for individuals in wheelchairs, and gave a wonderful overview of the innovative solutions his team is employing through a combination of engineering, advocacy, and policy. Thank you, Alan, and all of All Wheels Up for your work and for sharing your news with us!

Advocacy Leaders Are Invited To Join Us!

June 19th – Livestream Sarepta's 1st annual R+D Day (Sarepta Advocacy will email you the link).

June 22nd – Town Hall teleconference with Duchenne Advocacy Leaders, Sarepta Advocacy, and Doug Ingram, CEO and president, to discuss your thoughts and questions following R+D Day.

Sarepta Expands its Washington D.C. Office

We are thrilled to announce that our department of Government Affairs, Policy, and Patient Advocacy has added 2 talented team members.

- **Carolyn Hickey**, Sr. Director of Government Affairs and Reimbursement Policy, joins us most recently from Alexion, bringing expertise in rare disease access and reimbursement policy.
- **Stacey Frisk,** Manager of Government Affairs and Policy, has deep policy experience, much of which was gained from her recent work at the Cystic Fibrosis Foundation.

Carolyn and Stacey will join Diane in the Washington D.C. office while Siobhan and Allison will remain based in Cambridge. Welcome, Carolyn and Stacey!

Our department was excited to support NORD in their 35th year celebration. Congratulations to NORD and to all those honored with Rare Impact Awards. From left: Siobhan Fitzgerald, Diane Berry, Stacey Frisk, Carolyn Hickey, and Allison Craney.

US Clinical Trial Updates

PPMO (Peptide Conjugated Phosphorodiamidate Morpholino Oligomer) (SRP-5051)

- **PPMO** is an exon skipping technology designed to efficiently get inside cells, with the goal of producing more dystrophin in the muscle groups affected in DMD patients.
- Doug, our CEO, presented at a PPMD webinar on May 9th to provide additional education on the PPMO technology. You can find a link to the presentation on PPMD's website, or via this link: https://bit.ly/2J8uJdz.
- Update: The details of the open-label extension study (5051-102) will be posted on clinicaltrials.gov soon, and will be open to all individuals who complete our currently enrolling Phase 1 (5051-101) study for males with DMD amenable to Exon 51 skipping. Please reach out to a site investigator for more information. Visit clinicaltrials.gov (NCT03375255) for more.

PMO (Phosphorodiamidate Morpholino Oligomer)

- **ESSENCE**: The global Phase 3 clinical trial is ongoing, examining two distinct drug candidates: **golodirsen**, for the potential treatment of individuals with DMD who are amenable to Exon 53 skipping; and **casimersen**, to potentially treat those amenable to Exon 45 skipping. Enrollment at US ESSENCE sites is closed. Visit clinicaltrials.gov (NCT02500381) to learn more.
- Preparation of the golodirsen New Drug Application (NDA) to the FDA is underway, with the aim of completing submission in late 2018.

The Sarepta Boston Walking Team at the Greater Boston MDA Muscle Walk on May 19, 2018.

Sarepta Walks for Muscular Dystrophy

Along with attending Advocacy-led family workshops across the country, Sarepta frequently attends MDA Muscle Walks to participate in raising awareness and bolstering education of Muscular Dystrophy diseases.

At the Greater Boston MDA Walk, we were fortunate to join together with families, advocacy partners, and other companies for an inspirational day. Also on May 19, the Washington D.C. office was thrilled to participate in their local MDA walk.

Let's Stay in Touch!

To stay connected with us, please visit: www.duchenne.com/connect. You can also connect with us on social media:

Additional Resources:

Duchenne.com: Our online resource to help those with DMD and caregivers better understand DMD, clinical trials, and the importance of genetic testing.

Decode Duchenne: In partnership with PPMD, Decode Duchenne provides free genetic testing and counseling to eligible people with Duchenne or Becker muscular dystrophy who otherwise could not afford genetic testing.