SAREPTA THERAPEUTICS

U.S. PATIENT AFFAIRS

NEWSLETTER FOR THE DUCHENNE COMMUNITY

SUMMER 2019

Dear Advocacy Leaders,
August 19, 2019 was the PDUFA date for golodirsen and Sarepta received a complete response letter. We are disappointed by this news. Sarepta remains deeply committed to the Duchenne community and committed to bringing meaningful therapeutic options worldwide. Please let us know if there are any questions about this recent news.

-Sarepta Patient Affairs

WHERE WE'VE BEEN

Spring continues to keep our team on the move! We've had the pleasure of attending and participating in CureDuchenne Cares, PPMD End Duchenne Tour and Ready, Set, Jett Family Workshops across the U.S. In April, members of our team attended the MDA Clinical and Scientific Conference in Florida. On May 19th, Sarepta employees joined the Muscular Dystrophy Association for the Greater Boston Muscle Walk to show support for the neuromuscular community. This June, we joined hundreds of others at the Parent Project Muscular Dystrophy 2019 Annual Conference June 27-30 in Orlando, Florida, marking the organization's 25th annual conference. We were honored to present on both our Gene Therapy and RNA platforms, gather family insights during a focus group, present posters about our research and learn more from the community by way of our advocacy booth suggestion box. Most importantly, we were so pleased to meet and talk to many community members–both those new to Duchenne and those who participate in the congress year after year. We look forward to implementing what we learned.

Our global patient affairs team has also been busy at work. Check out the pin drops on our map to see what events we've been joining around the globe!

MDA Greater Boston Muscle Walk, May 2019

PPMD Annual Conference, June 2019

GET TO KNOW OUR TEAM!

CAMBRIDGE, MA

Tamara

Tamara joined the team in March as Sr. Manager in Patient Affairs. She will focus on building relationships and collaborations with the Limb Girdle Muscular Dystrophy (LGMD) advocacy, patient and caregiver community.

Why did you join Sarepta?

The patient affairs team, the LGMD team, and the Sarepta organization at large is learning and figuring out our path forward day by day—and I joined Sarepta to stay for the long-haul to see a vision become a reality for patients.

Where could we find you on the weekends?

I enjoy working out in my free time; a goal of mine would be to run the Boston Marathon again, and run on behalf of an organization in the Limb Girdle or Duchenne muscular dystrophy communities. I am always trying a new restaurant in Boston or New York City (I love seafood and Middle Eastern food!), or walking my dog, Puffin (pictured here) at the park near my home in Boston, or in the woods up in Vermont.

Kate

Kate joined the team in May for her second summer as the Rare Disease Patient Affairs Intern. She will be with the team throughout the summer before returning to UMass Amherst to complete her senior year and receive her degree in Healthcare Policy.

This is your second year back for the Sarepta summer internship. What did you most enjoy about your work last summer that made you want to return?

It is truly inspiring to see what a difference a year can make! Last year, I absolutely fell in love with the patient advocacy team at Sarepta and was encouraged by the unceasing commitment to expanding treatments and cures for the rare disease population.

Where could we find you on the weekends?

Most weekends you will find me traveling - I love to explore new cities, both locally and throughout the US. Sometimes though, you will catch me having a relaxing day by the lake in Maine.

ROUTE 79 The DUCHENNE SCHOLARSHIP Program

We are pleased to announce that the 2019-2020 application period has closed and we have reviewed all submitted applications for Route 79, The Duchenne Scholarship Program. An independent selection committee has chosen recipients to award a $5000 scholarship for post-secondary education in the 2019-2020 academic year.

Stay tuned for our recipient announcement and to learn more about the Route 79 2019-2020 scholars!

LET'S STAY IN TOUCH!

Please feel free to reach out to us at Advocacy@Sarepta.com with any questions or concerns. To stay connected with us, please visit: www.duchenne.com/connect. You can also connect with Sarepta us on social media:

CLINICAL UPDATES

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

PPMO is an investigational exon skipping technology designed to efficiently get inside cells, with the goal of producing dystrophin in the muscle groups affected in Duchenne patients.

NEW! 5051-201 is a Phase 2, two-part, multiple-ascending dose study of SRP-5051 for Dose Determination, then Dose Expansion, in Patients with Duchenne Muscular Dystrophy Amenable to Exon 51-Skipping Treatment. 5051-201 is open for enrollment and there are currently active sites in the USA (Florida, Georgia). Visit ClinicalTrials.gov (NCT04004065) for more information.

5051-101 is a Single Ascending Dose (SAD) study for patients amenable to Exon 51-skipping. Enrollment is now complete for 5051-101. Visit ClinicalTrials.gov (NCT03375255) for more information.

Individuals who have completed Phase 1 (5051-101) study for males with Duchenne amenable to Exon 51 skipping have started enrolling in the open-label extension study (5051-102). Sarepta is pleased to announce that home and/or local lab draws may be set up for individuals who enroll in 5051-102. Please reach out to a site investigator for more information. Visit ClinicalTrials.gov (NCT03675126) for more details.

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 Duchenne 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.

SRP-9001 (Micro-dystrophin Gene Therapy)

In December 2018, we began dosing in a Phase 2 randomized, double-blinded, placebo-controlled trial. More information about this trial can be found here (NCT03769116).

We are currently in planning stages for future trials and will be sharing more information once available.

Please visit our Gene Therapy website to learn more about the science of gene therapy.

ADDITIONAL RESOURCES:

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

DUCHENNEGENETHERAPY.COM
Our online resource for the Duchenne community that provides information about Gene Therapy Science and the Sarepta programs implementing Gene Therapy interventions.

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.