Letter to the community

Hello Advocacy Leaders,

We hope this finds you well and enjoying your summer, despite world events. What a year it’s been so far! While it surely was not start to 2020 that we expected, we have seen incredible efforts by the advocacy community to manage this difficult time with compassion and focus. We’re excited to share our Sarepta updates in this newsletter and look forward to connecting with you soon. Thank you for your leadership during this trying time, and always!

-Sarepta Patient Affairs
Where We’ve Been

U.S. Events

While our in-person connections were cut short this year, we’re thrilled that we’ve gotten to connect with so many of you virtually and continue to listen and learn from Duchenne community voices. Check out some U.S. virtual events we’ve attended so far in 2020.

Event

- Embrace this Moment series and virtual webinars with Cure Duchenne
- CureRareDisease “Power in Community e-Conference”
- Jett Foundation Virtual Event Series
- PPMD End Duchenne Tour Virtual Stops in Milwaukee and Long Island
- PPMD Annual Conference

Sarepta News!

Roche partnership

In December 2019, we were excited to announce a new partnership with Roche for SRP-9001, our investigational micro-dystrophin gene therapy. This partnership means, if SRP-9001 is successful, Roche will have the rights to obtain approval and distribute SRP-9001 outside of the United States. Sarepta will continue to maintain all rights to SRP-9001 within the United States and will maintain responsibility for the global development plan and the manufacturing plan for SRP-9001.

Fast Track Designation

The FDA granted fast track designation to both SRP-9001 Micro-dystrophin Gene Therapy and SRP-5051 PPMO.

New research collaborations

We’re continuing to grow and develop new partnerships. Recently, we’ve developed partnerships and research agreements with Dyno Therapeutics, Selecta Biosciences, Codiak Biosciences and Hansa Biopharma. All of these partnerships are intended to enhance our Duchenne gene therapy program. We look forward to discussing these partnerships in the future.

Data Releases

- On June 15, JAMA published a paper with 1-year data from SRP-9001 Trial 1, Systemic Gene Delivery Clinical Trial for Duchenne Muscular Dystrophy. Mendell, et al authored the paper- the paper can be accessed via pubmed by searching for the PMID # (32539076).
- Limb-Girdle Muscular Dystrophy (LGMD) news: On June 8, we released data from the SRP-9003 Gene Therapy Trial to treat Limb Girdle Muscular Dystrophy Type 2E. Find more information about the data release here.
Where We’ve Been

Just because we’re not in person, doesn’t mean we’re not here to connect!

Some members of our Global Policy, Government and Patient Affairs team in our home offices
Rare Lessons

Lesson Plan Contest for Teachers K-12

Sarepta is hosting a rare disease lesson plan contest, Rare Lessons, to promote rare disease learning in the K-12 classroom. We believe that encouraging rare disease education will promote diversity and inclusion in the classroom and set an educational framework for future rare disease scholars.

We have announced open submissions for U.S. Based Teachers to submit Rare Disease lesson plans to our 2020 contest. Four winning lesson plans will be selected and posted on sharemylesson.com. The authors of the winning lessons plans will receive $2500 individually and $2500 will be awarded to their school/educational institution. You can find more information at sarepta.com/rarelessons.

Please share the news with your favorite teachers!
Route 79, The Duchenne Scholarship Program

We’re excited to announce our new class of Route 79 scholarship recipients—stay tuned for our announcement at the end of the summer!

New team members!

Kate Pecora
I hope to be a familiar face at Sarepta to many of you, but if not, we will become acquainted soon! My name is Kate Pecora, and I have recently joined the Cambridge Patient Affairs Team in the role of Specialist after serving as an intern for the past two years.

After graduating from the University of Massachusetts Amherst with degrees in Healthcare Policy and Political Science, I swiftly transitioned into this full-time role to contribute to advancing our mission and bringing life-changing therapies to patients.

My passion for advocacy is in my genes— as a young woman with Type III SMA, I have navigated similar challenges to the very patients we serve. Having this first-hand experience has been the cornerstone of my aspirations as a professional.

I look forward to continuing my time at Sarepta and getting to meet and know more of you in the coming months!

Kate Segal
I’m thrilled to join the team at Sarepta as the new Director for State Government Affairs. I’m excited to bring my experience in industry and elected office to the Sarepta mission. I most recently worked at Biogen where I pushed for newborn screening of SMA across the Midwest states and worked to ensure access to the first treatment for SMA to patients. Prior to my time at Biogen, I had the honor of serving as a State Representative in my home state of Michigan.

I look forward to meeting you as we work together to advocate for our patients and the rare disease community.
Clinical Updates

Please note: Clinical trials evolve and information is regularly updated on clinicaltrials.gov.

You can find more information about Sarepta Clinical Trials here.

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

MOMENTUM (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 U.S. (Florida, Georgia, Iowa) and Canada. Visit clinicaltrials.gov (NCT04004065) for more information.

**PMO (Phosphorodiamidate Morpholino Oligomer)**

**MIS51ON (eteplirsen high dose trial):** This is a Phase 3, two-part trial. Part 1 will evaluate the safety and tolerability of two high doses. Part two will be conducted for the selection of a high dose (dose finding), and the comparison of the selected high dose to the FDA approved dose. This study is currently recruiting — please visit clinicaltrials.gov (NCT03992430) to learn more.

**ESSENCE:** The global Phase 3 clinical trial is ongoing, examining golodirsen, for patients amenable to Exon 53 skipping and casimersen, for patients amenable to Exon 45 skipping. Enrollment at U.S. ESSENCE sites is closed but sites continue to enroll outside of the U.S. Visit clinicaltrials.gov (NCT02500381) to learn more.

**SRP-9001 (Micro-dystrophin Gene Therapy)**

We completed enrollment for Phase 2 randomized, double-blinded, placebo-controlled trial in 2019. Please visit clinicaltrials.gov (NCT03769116) to learn more. We plan to initiate the next trial in the 2nd half of 2020.

Please visit our website to learn more about the science of gene therapy.
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: duchenne.com/connect.

You can also connect with Sarepta us on social media:

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.