March 25, 2026

Dear Members of the Myotonic Dystrophy Community,

We are reaching out today for the first time to briefly introduce Sarepta and share an important update.

Sarepta has been dedicated to developing meaningful therapies for rare diseases, having developed four approved therapies to treat Duchenne. It is our work in neuromuscular disease that inspired us to expand our scientific pursuits with a belief that we could make a meaningful difference advancing science in other neuromuscular diseases.

Sarepta is advancing investigational SRP-1003 (formerly known as ARO-DM1), is an siRNA study drug being researched in DM1 to target and suppress DMPK (dystrophia myotonica protein kinase) in skeletal muscle. The *DMPK* gene, located on chromosome 19, provides instructions for making a protein expressed primarily in skeletal, cardiac, and smooth muscles.

Earlier today, we shared clinical data from this research program. This is the first time this investigational medicine has been used in people.

While these results are early, we want to communicate them transparently and clearly:

- The data showed an acceptable tolerability profile;
- siRNA delivery into the muscle was observed in the participant dosed at the lowest dose in the study ; and
- The safety and tolerability data support continued clinical development

These findings provide an initial basis for further research, and much more work remains ahead to understand what this therapy may ultimately mean for people living with DM1.

You may hear terms like “single ascending dose (SAD)” in which the investigational therapy is administered once and “multiple ascending dose (MAD)” studies in which the investigational therapy is given more often to study different doses of the study medicine. These are early-stage clinical trials designed primarily to evaluate safety, understand how the therapy behaves in the body, and help determine appropriate dosing for future studies. This is an essential step in the development process.

We are encouraged by these initial results and are planning the next phase of clinical research. The continuation of the current trial will explore higher dose levels of investigational SRP-1003 to better understand its potential effects.

We have been working with the DM1 advocacy community from the beginning and will continue to do so. We expect to share additional updates later this year.

If you are living with DM1, or supporting someone who is, please know that we will listen to you as our work continues. We recognize the strength, resilience, and advocacy within this community, as too many rare diseases do not have treatments. We are sincerely grateful for all of the families who participate in clinical trials and the physicians and their teams who support clinical trials.

This is the beginning of our partnership. We are committed to listening, learning, and engaging with you as our work progresses. Your perspectives are essential in shaping how we move forward. We look forward to staying connected and continuing this conversation in the months ahead.

We look forward to introducing ourselves to many of you during the upcoming Myotonic Dystrophy Foundation: Meet the DM Drug Develop session on April 3 at 12:00 PDT

Registration link

If you have any questions or would like to connect with us, please reach out to advocacy@sarepta.com.

Sincerely,

Wendy Erler

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Senior Vice President, Patient Affairs


SAREPTA
THERAPEUTICS