SAREPTA THERAPEUTICS

3 October 2018

Dear Duchenne Community,

At the World Muscle Society in Argentina today, Dr. Jerry Mendell of Nationwide Children's Hospital shared additional data relating to our micro-dystrophin gene therapy program. In particular, as a follow up to his presentation of the first three patients at our R&D Day in June of this year, Dr. Mendell today shared micro-dystrophin results for the fourth patient and provided positive functional signals for all patients.

It is important to remember that while we are very encouraged by all of the results we have to date, these are early days relating to our first four patients. It is important that we quickly commence a controlled trial to confirm these results. Fueled by our preliminary data, we are moving with a sense of urgency to move to a study that, if successful, could bring this therapy to those patients who can benefit from it.

As mentioned in June, the next micro-dystrophin trial will take place in the United States, will be carried out by Nationwide Children's Hospital, and will be limited in size. Sarepta is mapping ways to expand the gene therapy clinical program to a broader population with considerations of study inclusion and geography. We will update the community about the plans as we finalize them.

Given the volume of inquiries received about this investigational effort, we know that there is a high level of anticipation within the worldwide Duchenne community surrounding gene therapy. Physicians and advocacy groups alike have expressed hope that families will stay focused on current treatment plans and investigational options selected in consultation with their healthcare team.

Let us once again be reminded that as encouraged as we are, these are preliminary results and we must continue to follow our initial patients and commence a controlled trial. But also know this, we are investing our energy, resources and creativity to moving the development forward as fast as is possible, planning meetings with the FDA and other agencies around the world to take their input, building a compelling access and reimbursement package, and establishing sufficient manufacturing capacity to fully serve the community if our program is successful.

I will provide additional updates as the program moves forward.

Sincerely,

Douglas S. Ingram
President and Chief Executive Officer
Sarepta Therapeutics, Inc