October 8th, 2019

Dear Duchenne Community,

In response to your request, we would like to provide you with information for the Italian community about the next steps of Sarepta's micro-dystrophin gene therapy program.

Overall, we are very pleased to inform you that Sarepta's gene therapy clinical trial programs continue to progress well and that we are making progress in the build out of our commercial micro-dystrophin process and manufacturing facility. For specific details of the three major components to the Duchenne micro-dystrophin clinical trial program (now publicly available), please see this short summary.

Phase I, open-label study: in March 2019, we reported 9-month functional and creatine kinase (CK) data change from baseline for the 4 patients who received the micro-dystrophin gene therapy for Duchenne muscular dystrophy (DMD). In 2020, the goal is to publish one-year clinical data from this program.

Phase II, double-blind, placebo-controlled trial (also called Study 2): we have completed the dosing of 24 patients in the placebo-controlled micro-dystrophin trial. We are also delighted to inform you that Nationwide Children's Hospital has been able to provide us with additional study gene therapy material. On that basis, we have amended the study protocol to increase the number of participants from 24 to 40, increasing the size of the study by nearly 70%. We expect to complete dosing by end of this year.

Phase III (also called study 301): we are using the remainder of 2019 to continue optimizing process and analytical development for our commercial gene therapy supply. We plan to commence the Phase III, multi-center, double-blind, placebo-controlled global trial in the first half of 2020. Details will be found at www.clinicaltrials.gov.

Sarepta's goal is to develop transformative medicine for 100% of eligible individuals living with Duchenne. We plan to expand age range, range of mutations, and inclusion of non-ambulatory individuals in additional trials subject to the current clinical trial program results.

Finally, the local Italian team are working closely with the Italian experts to evaluate capacity and the support required to accommodate potential clinical trials for Duchenne patients. Details of selected sites will be included at www.clinical trials.gov. We remain committed to continuing to work closely with you and other patient organizations to best support the informational needs of the Duchenne community in a helpful and transparent manner. For further information, please refer to your treating physician and www.clinicaltrials.gov.

Best Wishes,
Team Sarepta