21 September 2018

Dear European Duchenne Community,

This morning Sarepta announced that following its request for re-examination, the Committee for Human Medicinal Products (CHMP) in Europe issued a negative opinion for eteplirsen. While we recognized the probability of success was low, we committed our full resources and energy to this re-examination because we believe that the European Duchenne community deserves access to eteplirsen.

We are grateful for your insights, contributions and thoughtful engagement during this process. We also appreciated the open additional dialogue and advice we received from the CHMP, who signaled an openness to considering a regulatory pathway that could balance the needs of patients and their families with the requirements for additional supportive data. For eteplirsen, our next step is to initiate a request for follow up scientific advice, which we plan to do in 2019.

Given our discussions and commitment to the Duchenne community, we will continue to engage the European Medicines Agency (EMA) and explore the most appropriate path forward for eteplirsen and our other investigational medicines. We will seek scientific advice for our clinical programs as we continue to advance our pipeline of Duchenne therapies, including next-generation PPMO RNA therapy and the micro-dystrophin gene therapy candidate.

Despite today's news, we continue to believe that there is a path forward in Europe to bring new therapies that can improve the lives of individuals living with Duchenne. Sarepta will continue to sponsor clinical trials in Europe. The ESSENCE trial is still enrolling in Europe as a method to understand the safety and efficacy of golodirsen and casimersen. There is also an eteplirsen study focused on indivduals with Duchenne who are 6 months-48 months old in process. We will soon be initiating our high dose eteplirsen study, as well as trials for our next generation PPMO candidates in Europe.

We look forward to continued engagement as we advance these programs together.

Sincerely,

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