



August 28, 2012

An open letter from Chris Garabedian, president and CEO of Sarepta Therapeutics

On behalf of everyone at Sarepta Therapeutics, I wanted to provide you with an update on our clinical program for eteplirsen.

As many of you are aware, on July 24th we released interim data from our ongoing Phase IIb clinical trial of eteplirsen, which provided the first indication that eteplirsen may be having a favorable clinical impact in slowing the progression of the disease for a group of DMD patients with a genetic mutation that is addressable by exon 51 skipping.

Since that time, we have received numerous calls and emails for more information on the program, and especially, about the potential availability of eteplirsen outside of the current clinical trial. We wish to be as transparent as possible about what we are doing to bring eteplirsen to patients who would benefit from the therapy. We know that drug development is a painfully drawn-out process for those who are waiting, and we take seriously our responsibility to act as a true partner to the DMD community as we move forward together.

Since the moment that we saw the interim results last month, we have been working internally and with outside experts to determine the fastest path forward to make this drug more widely available. To do so, our primary focus as a company must be on completing the ongoing clinical trial and demonstrating the safety and efficacy of eteplirsen in a rigorous and convincing way that allows us to initiate discussions with regulators regarding our path forward to secure approval.

In October, we will be able to review and assess the Phase IIb 48-week data. Depending on the results, we will request a meeting with the FDA to determine what next steps will be required. Those discussions will determine what additional clinical trial or trials are needed, what the design of future trials will be, how many patients will participate, and what the makeup of the trial population will be.

At the same time, we must begin to scale up manufacturing of eteplirsen to support future clinical development beyond the current ongoing 12-patient trial. We are currently producing eteplirsen at a scale that is sufficient to meet the needs of the 12 patients in this study. We cannot produce enough eteplirsen to meet needs outside of this trial until we have substantially scaled up our production from current levels.

It is important to stress that eteplirsen is a truly novel type of medicine; the complexity of ramping up production on a larger scale will require a substantial investment of time, money, and expertise in order to demonstrate to regulators that eteplirsen produced at a larger scale has the same characteristics and safety profile as what is currently being used in clinical trials. We have a plan in place to meet this challenge, but we must be thorough and methodical to ensure we are successful.



At the present time, we do not have excess drug supply to make eteplirsen available outside of a clinical trial setting on a compassionate use basis. We can only imagine how difficult this is to hear, but unfortunately we have no other options available to us right now. We must devote our resources to producing enough eteplirsen for our current and future clinical studies so that we can advance this therapy through the regulatory process and, ultimately, secure approval for all DMD patients who would benefit.

We fully understand that for the patients and families impacted by DMD, the pace of drug development can never be fast enough. We pledge to continue to advance eteplirsen through the clinical development process as rapidly and responsibly as possible, and to regularly update the DMD community on our progress to bring this promising therapy to the numerous children and young adults with DMD who so desperately need and deserve a safe and effective treatment option.

Thank you for your consideration.

Sincerely,

Chris Garabedian
President and CEO
Sarepta Therapeutics