

BioMarin Update on Duchenne Muscular Dystrophy program.

March 2015

On behalf of families around the world BioMarin is pleased to update the Duchenne community on our plans for the development of our investigational products for Duchenne Muscular Dystrophy.

For the Duchenne Community, which is just starting to collaborate with BioMarin, we focus on developing first-in-class and best-in-class therapeutics that provide meaningful advances to patients who live with serious and life-threatening rare genetic diseases. BioMarin, founded in 1997, has brought five new treatments to rare disease patients, averaging just five years from IND to commercial approval.

We realize that time is critical to patients with rare diseases, and we strive to quickly develop important therapies for them. The efficiency and speed of our research, development, manufacturing, and commercial efforts is at the heart of our ability to urgently deliver therapies. Our track record of developing and commercializing new treatments has been significantly faster than the industry average and is engrained in our culture. BioMarin will apply its wealth of knowledge and experience gathered over nearly two decades in rare disease therapeutics and, more particularly, its extraordinary dedication and drive, to ensuring the timely and appropriate availability of a treatment for Duchenne .

BioMarin is thankful to the patients and their families, the clinical investigators, the patient associations, and the many thousands of individuals around the world who have contributed to the body of information that now exists around the Duchenne program.

We understand that after a period of uncertainty the community desires more detailed information about our investigational products for Duchenne. BioMarin plans to provide regular updates, as we have consistently done for our investigational products.

We look forward to understanding the experience and preferences of the Duchenne community, to earning the trust of Duchenne families and patients associations and to becoming a valued partner in the fight against this disease.

Frequently Asked Questions

What is the timeframe for re-dosing boys who have previously been in the Prosensa Clinical trial?

As of February 2015, re-dosing is actively underway in North America, Belgium and Sweden with sites open and active.

Maintenance of treatment for chronic disease has been a hallmark of BioMarin's programs to date, and we are committed to achieving the same goal with the Duchenne studies. The collection of safety and efficacy data under conditions of longer treatment serves as the

basis for generating comprehensive knowledge to support safe and appropriate use of medicines.

It is unfortunate that there has been an interruption in the ongoing evaluation of the continued safety and efficacy data of the Phase III trials due to decisions made prior to BioMarin's involvement.

BioMarin is committed to re-initiating treatment with investigational therapy in previously treated boys where possible. We are now working with medical teams at the treatment centres planning how to re-dose boys on a country-by-country, site-by-site basis. Once this groundwork is completed, we will have a better idea of the timelines for re-dosing at each centre.

What should families do to find out more about re-dosing?

Each country and site will have specific approvals and processes that we will need to complete before re-dosing can commence. Families are advised to keep in close contact with their treatment sites for updated information.

Will non ambulatory boys be eligible for re-dosing?

Ambulation will not be an inclusion/exclusion criteria for re-dosing.

What are BioMarin's plans for seeking regulatory approval?

We are on track to complete a New Drug Application (NDA) submission with the US Food and Drug Administration (FDA) by the end of April 2015. The submission to the European Medicines Agency (EMA) is expected to be completed in the summer of 2015

Will the work involved in the regulatory submissions delay the re-dosing activities?

There are two near-term objectives for the company. The application for regulatory authority review and approval, which is required to ensure that treatment becomes available for the greatest number of patients in need, and obtaining the approvals for the re-dosing of previously treated boys. We'll do what we can to achieve both as quickly as possible.

My son took part in the natural history study. Can he take part in future studies?

Enrollment to future BioMarin studies will be determined by whether the patient meets the inclusion criteria. Patients in the natural history study are not restricted from enrollment in any other studies for which they are eligible.

We enjoyed a close working relationship with Prosensa which meant they were able to share information and be transparent with us about this programme. Can we expect the same from BioMarin?

We understand the need for accurate and up to date information, and we will do everything we can, within the regulations, to share updates and information.

What is happening with the development of investigational products for other exons?

We are continuing the existing studies and evaluating future activities. We remain committed to developing additional therapies for Duchenne, and we will provide additional updates when those plans are finalized.

Will BioMarin consider Compassionate use, and if so, under what circumstances?

BioMarin has an existing policy on compassionate use, which describes under what circumstance an experimental therapy may be provided, this can be found on our website at www.bmrn.com/pipeline/compassionate-use-policy.php

Your own treating doctors and medical teams remain the best source of advice for the care of your sons. Families are encouraged to stay in close contact with their medical teams and also with the patient associations for regular updates about our Duchenne programs.

For patient and family group leaders, please contact Vivian Fernandez or Paul Humphrey from BioMarin Patient Advocacy.

vivfernandez@bmrn.com North America, Latin America, Asia-Pacific

paul.humphrey@bmrn.com Europe, Middle East and Africa

For individual patients, please contact BioMarin Medical Affairs.

Europe, Middle East Africa medinfoeu@bmrn.com

North America medinfo@bmrn.com

Latin America medinfo@bmrn.com

Asia Pacific medinfoasia@bmrn.com

