# Duchenne Muscular Dystrophy Clinical Development Program

## An Update for the Community

BioMarin is a global pharmaceutical company with 7 approved therapies and more than 20 years of experience in developing innovative medicines for rare genetic conditions. Each investigative medicine we pursue is guided by a fundamental understanding of the genetics and underlying biology of the condition it will address.

## Continuing our Commitment to Duchenne Muscular Dystrophy

In 2014, BioMarin acquired drisapersen, an investigational therapy for Duchenne; however, in 2016, clinical development was discontinued after discussions with regulatory authorities. Since that time, BioMarin has continued pre-clinical studies on various compounds and therapeutic approaches and is excited to announce a new pre-clinical candidate, BMN 351, which is currently being investigated in non-human studies.

## About BMN 351 for Duchenne Muscular Dystrophy

BMN 351 is a third-generation antisense oligonucleotide therapy under investigation for exon 51 skipping. BMN 351 is designed to target a distinct site that regulates exon 51 splicing and is delivered intravenously (IV), or directly into a vein.

As an investigation compound, BMN 351 has not been determined to be safe or effective or approved for use. Prior to entering the clinical trial process, pre-clinical studies in non-human models must be conducted, and if authorized to proceed to clinical trials, it will go through careful testing in clinical studies to see if it is safe and effective. BMN 351 has not yet begun clinical studies in humans and has not been proven safe or effective. Pre-clinical studies are intended to support an investigational new drug (IND) application with regulatory authorities. If results from the ongoing pre-clinical studies are supportive, BioMarin anticipates filing an IND with the FDA for BMN 351 in the first half of 2022, which would allow clinical studies to begin in humans.



## What are Antisense Oligonucleotide Therapies?

In those with Duchenne, there is a mutation (or change) in the DMD gene that encodes for dystrophin. Dystrophin is a protein needed by muscles in the body. The DMD gene is made up of 79 exons, regions that ultimately lead to the production of the functional protein. Many patients with Duchenne have deletions of specific exons that disrupt the production of functional dystrophin. Antisense oligonucleotide therapies (ASOs) are used to treat many different types of conditions. For Duchenne, ASO therapy is intended to increase the production of dystrophin in muscle cells by causing the cells to skip over an exon (in addition to those that are genetically deleted) with the goal of restoring expression of a shorter, functional dystrophin.

For additional information:

- Contact BioMarin Medical Information at 1-800-983-4587 or medinfo@bmrn.com
- For inquiries or to provide feedback from advocacy organizations, please contact patientadvocacy@bmrn.com