BioMarin Clinical Trial Program Update for **Duchenne Muscular Dystrophy**

BioMarin is pleased to update the Duchenne Muscular Dystrophy (DMD) community about our ongoing clinical development program.

What has BioMarin been working on for DMD?

2014

BioMarin acquires and continues development of drisapersen, an investigational medicine for DMD.

2016

BioMarin stops clinical development for drisapersen. This decision was made after discussions with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

2016-2021

BioMarin continues **pre-clinical research** on other compounds. Pre-clinical research occurs in the lab before a treatment is tested in people.

2021

BioMarin selects and announces a new investigational medicine also known as **BMN 351** based on years of pre-clinical research.

2024

BioMarin announces the **initiation of a clinical trial** called BMN 351-201**.**

What is BMN 351?

BMN 351 is a next generation of antisense oligonucleotide (ASO) therapy for boys with exon 51 amenable DMD. **ASOs** are drugs that affect how cells make certain proteins. They are being used to treat many different types of genetic health conditions.

BMN 351 is an **investigational medicine**, which means it has not been found to be safe and effective in people and has not been approved for use outside of clinical trials.

How could BMN 351 work to treat DMD?

BMN 351 will be researched to understand its safety and if it can improve levels of the protein **dystrophin** in muscle cells. Here is how this may work:

- BMN 351 is designed to cause cells to "skip" over exon 51, an area of the DMD gene that causes disease in some patients.
- Skipping over exon 51 may allow cells to make a functional (or working) form of dystrophin.

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Who could join the clinical trial?

- Assigned male at birth
- Aged 4 -10 years old
- Diagnosed with Duchenne Muscular Dystrophy who have a mutation amenable to exon 51 skipping
- Able to walk and stand without help
- On a stable dose of steroids for at least 12 weeks (3 months) before joining the trial
- Has not received gene therapy

How long will the study last?

The **BMN 351-201 study** is designed to measure safety in a small number of children living with DMD. If safety is acceptable, participants will continue to receive BMN 351 in an extension study. The initial trial will last up to 17 months, depending on when a participant joins.

What will be required of study participants?

- BMN 351 will be given through a weekly 1-hour intravenous infusion
- Safety is the primary endpoint and will be evaluated with several study health tests. These include blood tests, urine collection, heart function tests, physical examination, muscle function tests, and muscle biopsies.

How do I get more information?

- Contact BioMarin Medical Information at medinfo@bmrn.com
- Visit ClinicalTrials.gov and search for Identifier: NCT06280209 or BMN 351

About BioMarin

BioMarin is a global biotechnology company that treats life-threatening genetic conditions. BioMarin applies its scientific expertise in understanding the root cause of genetic conditions to create transformative medicines. The company's distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and preclinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options. The company was founded in 1997.

