BioMarin - BMN 351

A Phase 1/2, Open-Label, Dose Escalation Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Multiple Intravenous Doses of BMN 351 in Participants with Duchenne Muscular Dystrophy

Hub Summary

The purpose of this study is to test the safety and tolerability of BMN 351 in participants aged 4-10 with Duchenne Muscular Dystrophy (DMD) with a genetic mutation amenable to exon 51 skipping. BioMarin Pharmaceutical Inc (BioMarin), the sponsor of this study, wants to find out what effects, good and/or bad, BMN 351 has on your child and their DMD. BMN 351 is an experimental study medication that is given intravenously (through a needle or tube inserted into a vein); each infusion lasts about an hour.

Study Number: NCT06280209

Description by BioMarin Pharmaceutical Inc

This is Phase 1/2, open-label, multi-center study consisting of 2 parts to evaluate the safety and tolerability of BMN 351 at escalating doses in participants with Duchenne Muscular Dystrophy (DMD) with genetic mutations amenable to exon 51 skipping.

Participants will be assigned to one of three groups called cohorts (Cohort 1, 2 or 3). Cohort 1 participants are further divided into Cohort 1A and Cohort 1B. In Cohort 1A, 3 participants will receive increasing doses once every 2 weeks with a visit to assess safety measures collected the week after dosing prior to escalating doses of BMN 351. In part 2, the participants in cohort 1A will transition to once weekly dosing. The participants in Cohort 1B, 2, and 3 will initiate low, medium, and high doses of BMN 351 and continue once weekly dosing at that same dose. The study will enroll approximately 18 participants.

Primary Outcome Measures

To evaluate and safety and tolerability of single and multiple doses of BMN 351 (incidence, severity, and dose-relationship of adverse effects and changes in laboratory parameters).

Secondary Outcome Measures

Pharmacokinetics (PK) concentration of BMN 351 in plasma, urine and muscle approximately every 8 weeks for up to 48 weeks.

Other Outcome Measures

To evaluate the immune response to BMN 351.

To evaluate the effect of BMN 351 on physical function.

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Change from baseline in dystrophin expression measured by Liquid chromatography-mass spectrometry (LC-MS).

Can I take part?

Inclusion Criteria

- ✔ Age 4 to 10
- ✔ Diagnosis of Duchenne muscular dystrophy with a specific genetic change amenable to exon 51 skipping
- ✔ Able to walk
- ✔ Not requiring assistance from a ventilator to breathe
- ✔ Currently on consistent doses of steroid treatment for the last 12 weeks

Exclusion Criteria

- ✖ The participant will have some initial clinical labs and studies to assess baseline level of heart and lung function.
- ✖ Treatment with an exon skipping therapy within 12 weeks prior to the first visit.
- ✖ Any history of treatment with gene therapy

Trial Status
Fully recruited

UK Locations
London - GOSH, Fully recruited

Trial Sponsor
BioMarin Pharmaceutical Inc

Phase
Phase 1/2

Length Of Participation
7 to 17 months

Recruitment Target
18

Ambulatory
Ambulant, Ambulatory at Screening, defined as able to walk independently without assistive devices and able to complete the timed 10 meter walk/run test in 8 seconds or less

Therapeutic Category
Exon Skipping

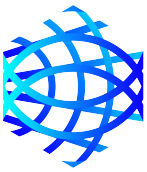
Age
4-10

Mutation Specific
Mutation specific therapies, Exon 51 Skipping

Muscle Biopsy
Muscle Biopsy Required

dmdhub.org

For contact details and to find out more, please refer to dmdhub.org.



Duchenne UK

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