

# 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


## Trial Status


Recruiting


 **UK Locations**  
London - GOSH,  
Recruiting


 **Trial Sponsor**  
BioMarin  
Pharmaceutical Inc

 **Age**  
4-10


 **Mutation Specific**  
Mutation specific  
therapies, Exon 51  
Skipping

 **Muscle Biopsy**  
Muscle Biopsy  
Required

 **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

[dmdhub.org](https://dmdhub.org)



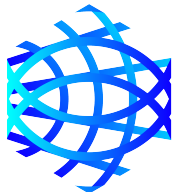
DMD HUB

- 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

For contact details and to find out more, please refer to [dmdhub.org](https://dmdhub.org).



**Duchenne  
UK**

PDF created on 16/05/2024.