



PROUD SPONSOR

For more information about this study, please contact:

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866-562-4620

Additional study information and participating sites:

[PTC-048-DMD Study](#)

Clinical trial participants will be reimbursed for costs associated with participating.

Contact study site for details.

PTC Therapeutics would like to express their gratitude to all those who have considered participation in this study.

PTC remains committed to the Duchenne Muscular Dystrophy community.

CLINICAL TRIAL ANNOUNCEMENT

An Open-Label Study Evaluating the Safety and Pharmacokinetics of Ataluren in Children From ≥ 6 Months to < 2 Years of Age with Nonsense Mutation Duchenne Muscular Dystrophy

We are pleased to share information with you today about a Nonsense Mutation Duchenne Muscular Dystrophy [nmDMD] clinical trial participation opportunity.

Individuals with nmDMD to participate in a phase 2 study. The primary goal of the study is to evaluate the safety and tolerability of Ataluren in boys ≥ 6 months to < 2 years old with nmDMD. The secondary goal is to evaluate pharmacokinetics.

This study is designed to evaluate the safety and pharmacokinetics of ataluren in participants with nmDMD. The study will include a 4-week screening visit, 24 weeks of treatment with ataluren and a 4-week post-treatment follow-up evaluation for a total of 6 study visits over 28 weeks. Subjects who complete the treatment period are offered participation in a follow-up extension period for at least 52 weeks from the date of first administration of ataluren in this parent study.

Eligibility criteria includes:

- Male participants ≥ 6 months to < 2 years of age.
- Any participant's sibling, if enrolled in another ataluren study, must be on open-label study drug at the time of participant's enrollment in this study.
- Body weight ≥ 7.5 kg.
- Diagnosis of DMD with documentation of the presence of a nonsense mutation of the dystrophin gene.
- No prior investigational drug treatment, including ataluren, within 3 months of screening visit.

Please see [PTC-048-DMD Study](#) for more details on specific inclusion and exclusion criteria. Please feel free to reach out to learn more about this study:
medinfo@ptcbio.com
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