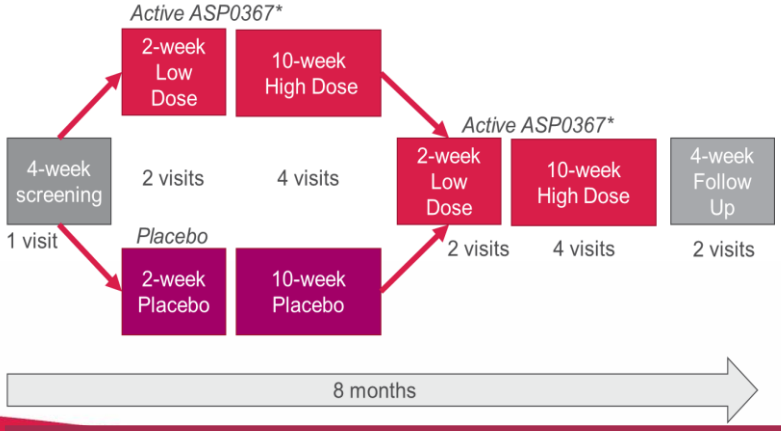
Astellas Phase 1b Study: A study to assess the safety, tolerability and preliminary efficacy of ASP0367*(MA-0211) in pediatric male participants with Duchenne muscular dystrophy (DMD)

Clinical Trial Now Open!

The study is now open at sites across the United States (US). Families can find all of them by visiting Astellas clinical trials for NCT04184882. We are adding more sites as they become ready to accept new participants.

The primary purpose of the study is to evaluate the safety and tolerability, and to evaluate how the body processes the drug and how it affects function (arm cycling test).



This study has four parts and takes up to 8 months to finish in total.

1) Screening portion (where your son would do tests to see if the study is a match for him)
2) Double - blind period (where you would not know whether he was taking ASP0367 tablets or placebo tablets [inactive tablets])
3) Open - label period (where everyone takes ASP0367).
4) Follow up period for 4 weeks (where your son would not take the study medicine).

Who can participate?

- Boys ages 8 to 16 years old
- Has a diagnosis of DMD
- Taking stable dose of corticosteroids and any cardiac therapy.
- Has at least some trouble walking quickly (10-meter walk test).
- Can move arms at least up to the shoulder height.

Who can 'NOT' participate?

- Has had an acute illness recently or has a serious infection, mental health condition, or behavioral problem, or is not healthy enough for the study.
- Has heart problems (ejection fraction <55%, long QT interval, high levels of cTnI on lab test).
- Has used certain types of drugs within 4 weeks of the study.
- Has kidney or liver problems.
- Is currently receiving an investigational drug. **

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Community Newsletter

Do you have questions?

Astellas Pharma Global Development can help.

Email: astellas.registration@astellas.com

Call: (800) 888-7704

The information described is from Astellas clinical trials for NCT04184882 and the latest protocol, and is current as of Sep13, 2021.

*The safety and efficacy of ASP0367 has not been established. There is no guarantee that ASP0367 will receive regulatory approval or become commercially available for uses being investigated. ASP0367 is not authorized for sale in any jurisdiction.

**Eligibility criterion to exclude a patient who receives exon-skipping therapy was modified on the study protocol amendment, which will become effective site by site. If the patient has been on stable dose regimen with a single commercially-available exon-skipping therapy for at least 6 months, the patient can participate in the study under the protocol amendment.

What should I know about the study visits?

We are working closely with trial sites to help families participate in the study safely. In the study, some visits are at your home and others are at the study site. Our sites closely follow safety protocols to protect patients and staff. Your study site can tell you more about what to expect at your visits. Travel support is available for clinical trial participants and their families.

Do I need a certain mutation to participate?

No. Anybody who has a diagnosis of DMD and meets other study criteria can participate.

What is ASP0367*?

- ASP0367 is an oral investigational drug targeting mitochondria and PPAR Delta energy related genes. It is being tested for the relief of fatigue on top of other therapies such as exon skipping treatments and corticosteroids.
- Inside your muscle cells are mitochondria. Mitochondria are the power generators in cells. Mitochondria turn the food (sugars and fatty acids) you eat into the energy cells need to survive and do work.
- Dystrophin is a protein inside your muscles that works with other proteins to keep your muscles strong and protect them from injury. When your muscle cells don't have dystrophin, they are damaged and this weakens the mitochondria.
- Damaged mitochondria can't produce energy properly. When this happens, you get tired easily.
- We are testing if ASP0367 can turn on the PPAR Delta pathways, which could activate the mitochondria with more fatty acid used more to make energy, which may decrease fatigue.

Vila et al., Mitochondria mediate cell membrane repair and contribute to Duchenne muscular dystrophy. Cell Death Differ. 2017
Wang et al., Regulation of muscle fiber type and running endurance by PPAR delta. PLoS Biol. 2004
Barish et al., PPARδ: a dagger in the heart of the metabolic syndrome. J Clin Invest. 2006

Thank You!

All of us at Astellas would like to thank all the families, researchers, trial sites, and advocacy groups for your support of this clinical trial. We are grateful for the opportunity to work with the community to learn about ASP0367.

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