Catabasis - Galaxy DMD [TERMINATED]

An Open-Label Extension Study of Edasalonexent in Boys With Duchenne Muscular Dystrophy

Hub Summary

This is an open label extension trial for patients who completed the POLARIS-DMD trial, and their siblings who meet the inclusion criteria between the ages of 4-12yrs (up to their 13th birthday).

This trial is looking at the safety, tolerability and durability of taking edasalonexent over a long period of time. Edasalonexent has been shown to delay the progression of DMD and could provide an alternative to steroids. It is in tablet form and taken orally (by mouth) three times a day.

Study Number: NCT03917719

Description by Catabasis Pharmaceuticals

The GalaxyDMD study is a global Phase 3, open-label, treatment extension study to evaluate the safety, tolerability, and durability of effect in long-term dosing of edasalonexent in pediatric patients with a genetically confirmed diagnosis of DMD. Patients who completed CAT-1004-201 or CAT-1004-301 or siblings of these boys from 4-12 years of age (up to 13th birthday) will be enrolled.

Edasalonexent is an orally administered small molecule that inhibits NF-kB, which is a key link between loss of dystrophin and disease pathology and plays a fundamental role in the initiation and progression of skeletal and cardiac muscle disease in DMD.

The study includes a 104-week open-label treatment period with edasalonexent. Patients who completed CAT-1004-201 or CAT-1004-301 and eligible siblings of these boys will be enrolled in this trial.

Primary Outcome Measures

- Safety and tolerability of long-term treatment with edasalonexent measured by number of treatment-emergent adverse events (TEAEs) and serious adverse events (SAEs) [Time Frame: 104 Weeks]

Secondary Outcome Measures

- Durability of effects of edasalonexent on physical function as measured by the North Star Ambulatory Assessment (NSAA) [Time Frame: 104 Weeks]
- Durability of effects of edasalonexent on physical function as measured by the 10-meter walk /run test [Time Frame: 104 Weeks]
- Durability of effects of edasalonexent on physical function as measured by the time to stand from supine [Time Frame: 104 Weeks]
- Durability of effects of edasalonexent on physical function as measured by the 4-stair climb [Time Frame: 104 Weeks]

Can I take part?

Inclusion Criteria

For Patients who Completed CAT-1004-201 or CAT-1004-301:

- ✔ Written consent/assent by patient and/or legal guardian as per regional and/or Institutional Review Board (IRB)/Independent Ethics Committee (IEC) requirements
- ✔ Completion of either CAT-1004-201 or CAT-1004-301

For Siblings of Patients who Completed CAT-1004-201 or CAT-1004-301:

- ✔ Written consent/assent by patient and/or legal guardian as per regional and/or Institutional Review Board (IRB)/Independent Ethics Committee (IEC) requirements
- ✔ A sibling of a patient who completed either CAT-1004-201 or CAT-1004-301
- ✔ Diagnosis of DMD based on a clinical phenotype with increased serum creatine kinase (CK) and documentation of mutation(s) in the dystrophin gene known to be associated with a DMD phenotype
- ✔ Followed by a doctor or medical professional who coordinates Duchenne care on a regular basis and willingness to disclose patient's study participation with medical professionals

Trial Status
Trial terminated

UK Locations
London - Evelina, Trial complete/terminated, London - GOSH, Trial complete/terminated, Bristol, Trial complete /terminated, Manchester, Trial complete/terminated, Temple Street, Trial complete/terminated

Trial Sponsor
Catabasis Pharmaceuticals

Phase
3

Length Of Participation
104 weeks

Recruitment Target
140

Ambulatory
Ambulant

Therapeutic Category
Steroid Alternative

Age
4-12

Mutation Specific
All treatment types

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Exclusion Criteria

For Patients who Completed CAT-1004-201 or CAT-1004-301:

- In the Investigator's opinion, unwilling or unable for any reason to complete all study assessments and laboratory tests and comply with scheduled visits, administration of drug, and all other study procedures

For Siblings of Patients who Completed CAT-1004-201 or CAT-1004-301:

- Use of oral corticosteroids at screening; use of inhaled, intranasal, and topical corticosteroids is permitted
- Use of another investigational drug, idebenone, or dystrophin-focused therapy within 4 weeks. Exception: Patients who are currently on or plan to initiate treatment with approved oligonucleotide exon-skipping therapies, and expected to continue treatment throughout the study, will be eligible
- Use of the following within 4 weeks prior to Day 1: immunosuppressive therapy, anticoagulants, cyclosporine, dihydroergotamine, ergotamine, fentanyl, alfentanil, pimozide, quinidine, sirolimus or tacrolimus
- Use of human growth hormone within 3 months prior to Day 1
- Other prior or ongoing significant medical conditions

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

Duchenne UK

PDF created on 28/11/2026.