

Summit- Ezutromid (PhaseOut DMD) [TERMINATED]



Please note the development of this drug was stopped after this trial failed to reach its objectives.

Hub Summary

This phase 2 clinical trial is designed to assess the activity and safety of Utrophin modulation in patients with DMD with SMT C1100 (Ezutromid). Ezutromid is an orally administered small molecule utrophin modulator.

Utrophin is a bodily protein which is structurally and functionally similar to dystrophin. Utrophin is naturally produced in the early stages of muscle development but is turned off as muscle fibres mature and dystrophin increases to perform the same role. Utrophin modulation aims to maintain the production of utrophin to compensate for the absence of dystrophin in patients with DMD.

This 1 year study has an optional extension phase.

Study Number: NCT02858362

Description by Summit Therapeutics

A Phase 2 Clinical Study to Assess the Activity and Safety of Utrophin Modulation with SMT C1100 (ezutromid) in Ambulatory Paediatric Male Subjects with Duchenne Muscular Dystrophy (DMD)

This is a Phase 2, open-label, study to assess the activity and safety of utrophin modulation with SMT C1100 (ezutromid) 2500 mg administered orally bid in ambulatory paediatric male subjects with DMD. Approximately 40 subjects with DMD will be enrolled in this study.

This study will be conducted in a multi-centre setting in both the United Kingdom and the United States of America and comprises of a Screening and Baseline Phase of up to 28 days, a 48-week open label Treatment Phase and a 30-day Safety Follow up Phase.

Primary Outcome Measures

- Change in MRI leg muscle parameters
- SMT C1100 (ezutromid) plasma concentrations

Secondary Outcome Measures

- Change in utrophin membrane staining via quantifiable imaging of immunostained biopsy sections.
- Change in muscle regenerating fibres by measuring via muscle biopsy a combination of fibre size and neonatal myosin positivity
- Treatment emergent adverse events (AEs) and safety laboratory abnormalities.

Can I take part?

Inclusion Criteria

- ✓ Male
- ✓ Age 5-10 years (from 5th birthday to 10th birthday)
- ✓ DMD diagnosis
- ✓ Willing and able to comply with study procedures, including 2 muscle biopsy procedures
- ✓ Able to undergo MRI
- ✓ Have used at least 6 months stable dose systemic corticosteroids
- ✓ Ability to walk 300 metres unassisted and below 80% predicted 6MWD

Exclusion Criteria

- ✗ Uncontrolled congestive heart failure (CHF) or recent change in CHF prophylaxis/treatment
- ✗ Use of beta blockers, herbal supplements, BCRP substrates, SNRIs, SSRIs, tricyclic antidepressants, or ADHD treatments such as methylphenidate or PEA.
- ✗ Use of over the counter, herbal or prescription CYP2B6, CYP1A1 or CYP1A2 inhibitors, inducers or substrates.

Trial Status

Trial terminated

UK Locations

Trial Sponsor
Summit Therapeutics

Phase
2

Length Of Participation
1 year

Recruitment Target
40

Ambulatory
Ambulant

Therapeutic Category
Utrophin Modulation

Age
5-10

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
Muscle Biopsy Required

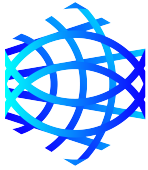
MRI
Yes

dmdhub.org



- ✗ Exposure to other investigational drug or DMD interventional agent within 3 months (except FOR-DMD Study participants are permitted)
- ✗ Require daytime ventilator assistance
- ✗ Be dairy or lactose intolerant
- ✗ Be a smoker, use other tobacco or nicotine products or be exposed to daily passive smoking
- ✗ Use of an approved DMD medication or anticipate use during the study (other than steroids)

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



**Duchenne
UK**

PDF created on 13/09/2025.