Vamorolone Phase II Extension

Long-term extension Study to Assess Vamorolone in Boys with Duchenne Muscular Dystrophy (DMD)

Hub Summary

This extension study is designed to assess the safety of using vamorolone long term in children with DMD. The study will also compare muscle function to boys with DMD in other studies who did not take steroids as well as comparing weight gain with boys taking vamorolone and boys taking traditional steroids (prednisone).

Vamorolone is a steroid alternative which is designed to be an alternative to corticosteroids with reduced side effects.

Study Number: NCT03038399

Description by ReveraGen BioPharma, Inc.

This long-term extension study is an open-label, multiple-dose study to evaluate the long-term safety, tolerability, efficacy and PD of vamorolone administered once daily by liquid oral suspension over a Treatment Period of 24 months to young boys with DMD who participated in the VBP15?002 Phase IIa and VBP15-003 Phase IIa extension core studies.

This study will evaluate if it is safe to use a new medication called vamorolone for more than two weeks in children with DMD, if boys with DMD who take the study medication have improved muscle function compared to boys with DMD in other studies who did not take any type of steroid, and to see if boys with DMD who take the study medication gain less weight compared to boys with DMD in a prior study who took another type of steroid called prednisone. Enrolled participants will take the study medication for 24 months.

Primary Outcome Measures

- Number of participants with treatment-related adverse events as assessed by CTCAE Version 4.03
- Muscle function measured by Time to Stand Test (TTSTAND)
- Body size as measured by body mass index (BMI) z-score

Secondary Outcome Measures

- Serum pharmacodynamics biomarkers measured by levels of cortisol
- Serum pharmacodynamics biomarkers measured by levels of ACTH
- Serum pharmacodynamics biomarkers measured by levels of PINP
- Serum pharmacodynamics biomarkers measured by levels of osteocalcin
- Serum pharmacodynamics biomarkers measured by levels of CTX
- Serum pharmacodynamics biomarkers measured by levels of 17-hydroxyprogesterone
- Serum pharmacodynamics biomarkers measured by levels of testosterone
- Serum pharmacodynamics biomarkers measured by levels of corticosterone
- Serum pharmacodynamics biomarkers measured by levels of 11-deoxycortisol
- Serum pharmacodynamics biomarkers measured by levels of glucose
- Serum pharmacodynamics biomarkers measured by levels of insulin
- Muscle strength measured by Quantitative Muscle Testing (QMT)
- Muscle function measured by Time to Climb Test (TTCLIMB)
- Muscle function measured by Time to Run/Walk 10 Meters Test (TTRW)
- Muscle function measured by North Star Ambulatory Assessment (NSAA)
- Muscle function measured by Six-minute Walk Test (6MWT)

Trial Status
Trial complete

UK Locations
London - GOSH, Trial complete/terminated, Newcastle, Trial complete/terminated

Trial Sponsor
ReveraGen BioPharma, Inc.

Age
4-7

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

Phase
2

Length Of Participation
2 years

Recruitment Target
48

Ambulatory
Ambulant

Therapeutic Category
Steroid alternative

dmdhub.org

Can I take part?

Inclusion Criteria

- Subject's parent or legal guardian has provided written informed consent and HIPAA authorization (if applicable) prior to any VBP15-LTE long-term extension study-specific procedures;
- Subject has previously completed study VBP15-003 up to and including the Week 24 Final assessments, prior to enrolling in the VBP15-LTE study at the conclusion of the VBP15-003 Week 24 Visit [Note: if entering the dose-tapering period, subject is enrolling within 8 weeks after the VBP15?003 final visit following dose?tapering]; and
- Subject and parent/guardian are willing and able to comply with scheduled visits, study drug administration plan, and study procedures.

Exclusion Criteria

- Subject had a serious or severe adverse event in study VBP15-003 that, in the opinion of the Investigator, was probably or definitely related to vamorolone use and precludes safe use of vamorolone for the subject in this long-term extension study;
- Subject has current or history of major renal or hepatic impairment, diabetes mellitus or immunosuppression;
- Subject has current or history of chronic systemic fungal or viral infections;
- Subject has used mineralocorticoid receptor agents, such as spironolactone, eplerenone, canrenone (canrenoate potassium), prorenone (prorenoate potassium), mexrenone (mexrenoate potassium) within 4 weeks prior to the first dose of study medication;
- Subject has evidence of symptomatic cardiomyopathy. [Note: Asymptomatic cardiac abnormality on investigation would not be exclusionary];
- Subject is currently being treated or has received previous treatment with oral glucocorticoids or other immunosuppressive agents [Notes: Past transient use of oral glucocorticoids or other oral immunosuppressive agents for no longer than 3 months cumulative, with last use at least 3 months prior to first dose of study medication, will be considered for eligibility on a case-by-case basis. Inhaled and/or topical glucocorticoids prescribed for an indication other than DMD are permitted but must be administered at stable dose for at least 3 months prior to study drug administration];
- Subject has used idebenone within 4 weeks prior to the first dose of study medication;
- Subject has an allergy or hypersensitivity to the study medication or to any of its constituents;
- Subject has severe behavioral or cognitive problems that preclude participation in the study, in the opinion of the Investigator;
- Subject has previous or ongoing medical condition, medical history, physical findings or laboratory abnormalities that could affect safety, make it unlikely that treatment and follow-up will be correctly completed or impair the assessment of study results, in the opinion of the Investigator
- Subject is currently taking any investigational drug, or has taken any investigational drug other than vamorolone within 3 months prior to the start of study treatment.

Note: Subjects may be re-evaluated if ineligible due to a transient condition which would prevent the subject from participating.

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

Duchenne UK