Dyne Therapeutics - DYNE-251

Safety, Tolerability, Pharmacodynamics, Efficacy, and Pharmacokinetics Study of DYNE-251 in Participants with Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

Hub Summary

A Randomized, Double-Blind, Placebo-Controlled, Multiple Ascending Dose Study Assessing Safety, Tolerability, Pharmacodynamics, Efficacy, and Pharmacokinetics of DYNE-251 Administered to Participants with Duchenne Muscular Dystrophy, aged 4 to 16 years, Amenable to Exon 51 Skipping.

Study Number: NCT05524883

Description by Dyne Therapeutics, Inc

DYNE-251 is Dyne's product candidate being developed for people living with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping. DYNE-251 consists of a phosphorodiamidate morpholino oligomer (PMO) conjugated to a fragment antibody (Fab) that binds to the transferrin receptor 1 (TfR1) which is highly expressed on muscle. It is designed to enable targeted muscle tissue delivery and promote exon skipping in the nucleus, allowing muscle cells to create a truncated, functional dystrophin protein, with the goal of stopping or reversing disease progression. In preclinical studies with Dyne's FORCE™ platform, robust and durable exon skipping and dystrophin expression were observed in the mdx mouse model in skeletal and cardiac muscle as well as reduced muscle damage and increased muscle function. In non-human primates, DYNE-251 demonstrated a favorable safety profile and achieved impressive exon skipping, especially in the heart and diaphragm, muscles in people living with DMD that weaken over time leading to mortality.

The DELIVER study is testing an investigational drug called DYNE-251 in males with Duchenne muscular dystrophy (DMD) with mutations amenable to exon 51 skipping. The DELIVER study is designed to understand if DYNE-251 is safe, what the best dose of DYNE-251 is, and if it can increase muscle dystrophin and improve muscle function. Participants will be administered DYNE-251 intravenously once every 4 weeks for up to 3 years, including multiple scheduled visits to the study site. Approximately 46 participants will be entered into the study globally. There are inclusion and exclusion criteria, and therefore not every patient is eligible.

In the DELIVER study, a computer will assign a participant to receive either DYNE-251 or placebo, at random, for the first 24 weeks. Neither the participant nor parent or caregiver, or study staff will know which study treatment will be received for the first 24 weeks. In this study, comparing DYNE-251 to placebo will help doctors determine if any effects seen in your body are due to DYNE-251 or not. All participants on placebo will receive DYNE-251 after 24 weeks.

The study consists of 3 periods: a multiple-ascending dose (MAD) / placebo-controlled period (24 weeks), an open-label period (24 weeks) and a long-term extension period (96 weeks)

Primary Outcome Measures

1. To evaluate the safety and tolerability of multiple intravenous doses of DYNE-251 administered to participants with Duchenne muscular dystrophy (DMD).
2. To evaluate the change from baseline in dystrophin levels as measured by Western blot in muscle tissue following multiple intravenous doses of DYNE-251 administered to participants with DMD

Secondary Outcome Measures

1. To evaluate effects on muscle tissue exon skipping and percent dystrophin positive fibers

Trial Status
Fully recruited

UK Locations
London - GOSH, Fully recruited, Alder Hey, Fully recruited, Bristol, Fully recruited, Leeds, Fully recruited, Newcastle, Fully recruited

Trial Sponsor
Dyne Therapeutics, Inc

Phase
I/II

Length Of Participation
3 Years

Recruitment Target
~46

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Exon Skipping

Age
4 to 16 years

Mutation Specific
Mutation specific therapies, 51 Skip-amenable only

Muscle Biopsy
Muscle Biopsy Required

MRI
No

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(PDPF), and plasma CK, following multiple intravenous doses of DYNE-251 administered to participants with DMD.
2. To evaluate muscle function following multiple intravenous doses of DYNE-251 administered to participants with DMD

Can I take part?

Inclusion Criteria

- ✔ Age 4 to 16 years inclusive, at the time of informed consent.
- ✔ Male with a confirmed DMD mutation in the dystrophin gene characterized by exon deletion amenable to exon 51 skipping.
- ✔ Upper extremity muscle group that is amenable to muscle biopsy.
- ✔ Brooke upper extremity score of 1 or 2.
- ✔ Ambulatory or non-ambulatory. A non-ambulatory participant must have been non-ambulatory for <2 years before enrolment.
- ✔ Receiving a stable dosage of glucocorticoids (eg, prednisone, prednisolone, deflazacort) for at least 12 weeks prior to the start of study drug administration.
- ✔ Left ventricular ejection fraction of 50% by echocardiogram or 55% by cardiac magnetic resonance imaging (MRI).
- ✔ Additional eligibility criteria may apply

Exclusion Criteria

- ✖ Uncontrolled clinical symptoms and signs of congestive heart failure (CHF).
- ✖ History of major surgical procedure within 12 weeks prior to the start of study drug administration or an expectation of a major surgical procedure (eg, scoliosis surgery) during the duration of the study.
- ✖ Requirement of daytime ventilator assistance.
- ✖ Percent predicted FVC <40 % (applies only for participants who are age 7 years).
- ✖ Receipt of eteplirsen, or alternative exon-skipping/dystrophin-modifying therapy, within 12 weeks of randomization.
- ✖ Receipt of non-exon skipping investigational drug within 4 months before the start of study drug administration.
- ✖ Receipt of gene therapy at any time.
- ✖ Additional eligibility criteria may apply

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

Duchenne UK

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