FibroGen - Pamrevlumab (Non-ambulatory)

A Phase 3, Randomized, Double-Blind, Trial of Pamrevlumab (FG-3019) or Placebo in Combination With Systemic Corticosteroids in Subjects With Non-ambulatory Duchenne Muscular Dystrophy (DMD)

Summary

This is a phase 3 trial to test the safety and efficacy of pamrevlumab (FG-3019) versus placebo in combination with systemic corticosteroids in subjects with non-ambulatory Duchenne muscular dystrophy (aged 12 and above). Pamrevlumab (35 mg/kg) or a matching placebo will be given intravenously every 2 weeks for 52 weeks.

Subjects that complete the study may be eligible for rollover into an open-label extension treatment (OLE) with pamrevlumab + systemic corticosteroids.

Study Number: NCT04371666

Description by FibroGen, Inc.

This is a global, randomized, double-blind trial of pamrevlumab or placebo in combination with systemic corticosteroids in subjects with non-ambulatory Duchenne muscular dystrophy, aged 12 years and older. Approximately 90 male subjects will be randomized at a 1:1 ratio to Arm A (pamrevlumab + systemic corticosteroid) or Arm B (placebo+ systemic corticosteroid), respectively.

Subjects must be fully informed of the potential benefits of approved products and make an informed decision that they prefer to participate in a clinical trial in which they could be randomized to placebo.

Subjects will be randomized in a 1:1 ratio to one of the two study treatment arms; pamrevlumab or placebo in combination with systemic steroids.

This trial has three study periods:

- Screening period: Up to 4 weeks

AUSNMD

Trial Status
Fully recruited

Locations
Melbourne - Melbourne Children's Campus, Not yet recruiting

Trial Sponsor
FibroGen, Inc.

Age
12 and above

Mutation Specific
No

Muscle Biopsy
No

MRI
Yes

Phase
3

Length Of Participation
56 weeks

Recruitment Target
90

Ambulatory
No

Therapeutic Category
Anti-fibrotic

- Treatment period: 52 weeks
- Safety Follow-up period/final assessment: 4 weeks (Week 56 (+/-3 days))

In the screening period, subjects will be evaluated per the protocol inclusion/exclusion criteria to determine eligibility for participation in this trial.

During the treatment period, each subject will receive pamrevlumab or placebo at 35 mg/kg every 2 weeks for up to 52 weeks.

Subjects who complete the 52-week study (either arm) may be eligible for rollover into an open-label extension treatment (OLE) with pamrevlumab + systemic corticosteroids.

Subjects who discontinue study treatment for any reason should be encouraged to return to the investigative site to complete final safety and efficacy assessments.

Primary Outcome Measures

- Functional assessment: Change in the total score of Performance of Upper Limb (PUL) 2.0 version [Time Frame: Baseline to Week 52]

Secondary Outcome Measures

- Pulmonary assessment: Change in percent predicted forced vital capacity (ppFVC) assessed by spirometry. [Time Frame: baseline to Week 52]
- Pulmonary assessment: Change in percent predicted peak expiratory flow (ppPEF) assessed by spirometry [Time Frame: baseline to Week 52]
- Performance assessment: Change in the Grip strength of the hands assessed by Hand Held Myometry (HHM). [Time Frame: baseline to Week 52]
- Cardiac assessment: Change in Left Ventricular Ejection Fraction percentage (LVEF %) assessed by MRI. [Time Frame: baseline to Week 52]

Can I take part?

Inclusion Criteria

- Males at least 12 years of age, non-ambulatory at screening initiation
- Have a medical diagnosis of DMD and confirmed Duchenne mutation using a validated genetic test
- Brooke Score for Arms and Shoulders 5
- Able to undergo MRI test for the upper arm extremities (Biceps Brachii muscle) and cardiac muscle
- Able to perform spirometry
- Average (of Screening and Day 0) percent predicted FVC between 45 and 85, inclusive
- Left ventricular ejection fraction 50% as determined by cardiac MRI at screening or within 3 months prior to randomization (Day 0)
- If there is a prior diagnosis of cardiomyopathy, subjects must be on a stable regimen dose for cardiomyopathy/ heart failure medications (e.g., angiotensin converting enzyme inhibitors, aldosterone receptors blockers, angiotensin-receptor blockers, and betablockers) for at least 1 month prior to screening
- On a stable dose of systemic corticosteroids for a minimum of 6 months, with no substantial change in dosage for a minimum of 3 months (except for adjustments for changes in body weight) prior to screening. Corticosteroid dosage should be in compliance with the DMD Care Considerations Working Group recommendations (e.g.prednisone or prednisolone 0.75 mg/kg per day or deflazacort 0.9 mg /kg per day) or stable dose. A reasonable expectation is that dosage and dosing regimen would not change significantly for the duration of the study.

- Received pneumococcal vaccine (PPSV23) (or any other pneumococcal polysaccharide vaccine as per national recommendations) and is receiving annual influenza vaccinations
- Adequate renal function: cystatin C 1.4 mg/L
- Adequate hematology and electrolytes parameters:
  - Platelets >100,000/mcL
  - Hemoglobin >12 g/dL
  - Absolute neutrophil count >1500 /L
  - Serum calcium (Ca), potassium (K), sodium (Na), magnesium (Mg) and phosphorus (P) levels are within a clinically accepted range
- Adequate hepatic function:
  - No history or evidence of liver disease
  - Gamma glutamyl transferase (GGT) 3x upper limit of normal (ULN)
  - Total bilirubin 1.5xULN

Exclusion Criteria

- Previous exposure to pamrevlumab
- BMI 40 kg/m2 or weight >117 kg
- History of allergic or anaphylactic reaction to human, humanized, chimeric or murine monoclonal antibodies
- Exposure to any investigational drug (for DMD or not), in the 30 days prior to screening initiation or use of approved DMD therapies (e.g., eteplirsen, ataluren, golodirsen) within 5 half-lives of screening, whichever is longer, with the exception of the systemic corticosteroids, including deflazacort

- Severe uncontrolled heart failure (NYHA Classes III-IV), including any of the following:
  - Need for intravenous diuretics or inotropic support within 8 weeks prior to screening
  - Hospitalization for a heart failure exacerbation or arrhythmia within 8 weeks prior to screening
- Arrhythmia requiring anti-arrhythmic therapy
- Requires 16 hours continuous ventilation
- Hospitalization due to respiratory failure within the 8 weeks prior to screening
- Poorly controlled asthma or underlying lung disease such as bronchitis, bronchiectasis, emphysema, recurrent pneumonia that in the opinion of the investigator might impact respiratory function

Other inclusion/exclusion criteria apply.

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

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