

Registry Study to Observe Long-term Safety of Vamorolone (AGAMREE®) in Patients With Duchenne Muscular Dystrophy-SUMMIT

Status: RECRUITING

Eligibility Criteria

Sex: MALE

Age: 2 years and over

Healthy Volunteers: This study is NOT accepting healthy volunteers

Inclusion Criteria:

1. Patient or parent/legal guardian is willing and able to provide written informed consent once the nature of the registry has been explained and prior to the start of any registry-related procedures.
2. Patient and/or parent/guardian are willing and able to complete QoL questionnaires.
3. Male patients at least 2 years old.
4. Confirmed diagnosis of DMD (via genetic testing or muscle biopsy with absent dystrophin staining to anti- dystrophin antibodies 3, 1, or 2, or dystrophin immunohistochemistry or western blot).
5. Patient has a current, active prescription for, or is on, AGAMREE®.

Exclusion Criteria:

1. Any contraindication to AGAMREE® or medical condition, which, in the opinion of the Investigator, would affect registry participation, performance, or interpretation of registry assessments.

Conditions & Interventions

Interventions:

DRUG: Vamorolone

Conditions:

Duchenne Muscular Dystrophy

Keywords:

Duchenne Muscular Dystrophy, Neuromuscular, AGAMREE®, Vamorolone, DMD, Muscular Dystrophy

More Information

Description:

The goal of this study is to collect additional information on the safety of long-term treatment with AGAMREE® and to explore long-term clinical impact of AGAMREE® on quality of life, as assessed by standardized patient-reported outcome measures (QoL questionnaires) in male patients aged 2 years and older with Duchenne muscular dystrophy (DMD).

Contact(s): Cuixia Tian, MD - Cuixia.Tian@cchmc.org

Principal Investigator:

Principal Investigator ID:

Phase:

IRB Number:

System ID: NCT06564974

Thank you for choosing StudyFinder. Please visit <http://studyfinder.cchmc.org> to find a Study which is right for you and contact clinicalstudies@cchmc.org if you have questions or need assistance.