AGT-182-101 Phase I Clinical Trial in Patients with Hunter Syndrome

Key Facts

March 2016

What is the purpose of the trial?

To test the safety and determine a well-tolerated dose of an investigational treatment AGT-182 in people with Hunter syndrome.

Why should I participate in this trial?

People choose to enroll in clinical trials for various reasons. By participating in this study, you could contribute to medical research and potentially help future generations of people with Hunter syndrome. Please speak with your physician for guidance on enrolling.

What is AGT-182?

- AGT-182 is an investigational enzyme replacement therapy designed to treat both body-related and central nervous system-related symptoms and complications of Hunter syndrome.
- Currently approved treatments for Hunter syndrome are unable to penetrate the blood-brain barrier (BBB), a filter that protects the brain from harmful substances like toxins and bacteria but allows vital substances like insulin to cross from the blood into the brain.
- AGT-182 is designed to cross the BBB in the same way insulin does.

What is the study design?

- The study is a Phase 1 trial in adults with Hunter syndrome. A Phase 1 trial tests a new drug in a small group of patients to evaluate the drug's safety, identify potential side effects, and determine a dose of the medication for further testing.
- Patients in the trial will receive weekly infusions of AGT-182 at assigned doses that range from 1 mg/kg for the first dose group of patients enrolled and increase to 3.0 mg/kg. Additional higher dose levels may be added.
- AGT-182 will be administered intravenously over a 3-hour period for eight weeks.
- Before and after treatment, study investigators intend to collect a sample of cerebrospinal fluid, a fluid that surrounds and protects the brain and spinal cord, through a minimally-invasive diagnostic test called a spinal tap. This fluid will be tested to help confirm that AGT-182 is crossing the BBB.

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Who is eligible to participate in the clinical trial?

Key criteria for participation are included below:

- Male patients age 18 years or older diagnosed with Hunter syndrome.
- Must provide voluntary written consent.
- Patients on current enzyme replacement therapy (ERT) must discontinue ERT for at least 6-weeks before and during the duration of the trial.

Who is sponsoring the trial?

The trial is sponsored by ArmaGen, a privately held biotechnology company focused on developing revolutionary therapies for severe neurological disorders.

Learn more about the study and eligibility criteria by visiting breakingbarriershuntertrial.com or www.clinicaltrials.gov using the identifier number NCT02262338. You may also contact investigators at the study centers below who will evaluate your eligibility and provide detailed information about reimbursement of expenses.

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