ArmaGen Announces Initiation of Phase 2 Proof-of-Concept Clinical Trial in Brazil to Study AGT-181 for the Treatment of Hurler Syndrome

CALABASAS, Calif., March 31, 2016 — ArmaGen, Inc., a privately held biotechnology company focused on developing revolutionary therapies to treat severe neurological disorders, announced today that the first patient has been dosed with AGT-181 in a Phase 2 proof-of-concept (POC) clinical trial treating pediatric patients with Hurler syndrome (also known as mucopolysaccharidosis type I, or MPS I) in Brazil. The initiation of this pediatric study follows the successful completion of a Phase 1 study in adult patients in Brazil.

AGT-181 is an investigational enzyme replacement therapy (ERT) for the treatment of Hurler syndrome. The most severe form of MPS I, Hurler syndrome is a rare, hereditary lysosomal storage disorder that affects the brain and spinal cord in children, resulting in a wide range of debilitating symptoms. Commercially available treatments for Hurler syndrome do not penetrate the blood-brain barrier (BBB), and therefore do not address the severe and progressive neurological complications of the disease. AGT-181 is designed to utilize the body's natural system for transporting products non-invasively across the BBB by targeting the receptor that delivers insulin to all cells of the body.

"We are extremely pleased to be working with Dr. Roberto Giugliani and his team at Hospital de Clínicas, as this site has a world-class reputation for clinical research and has contributed to numerous successful ERT clinical studies over the years," said Patrice Rioux, M.D., Ph.D., Senior Vice President of Global Clinical Development at ArmaGen. "This POC trial joins our ongoing U.S. Phase 1/2a trial in adults and will contribute important data on safety and tolerability for our platform technology. Additionally, this study will allow us to capture biomarker data to help assess the potential impact of treatment on both peripheral and central nervous system symptoms of Hurler syndrome."

The Phase 2 POC study, led by Dr. Giugliani, will be an open-label, multi-dose, dose-escalation study in children (age two or older) with Hurler or Hurler-Scheie syndrome and central nervous system involvement. The primary objective of this study is to determine the safety and tolerability of weekly infusions of 1.0 or 3.0 mg/kg of AGT-181 in children with Hurler or Hurler-Scheie syndrome.

Ten patients will be enrolled into the study and the period of observation will be six months. Patients for whom it can be demonstrated that a cognitive benefit was achieved will be offered an open label 12-month extension treatment protocol to collect additional long-term safety and efficacy data.

ArmaGen expects to complete the Phase 2 POC study by the end of 2016.

In advance of this Phase 2 study, a six-patient, Phase 1 safety study in adults (ages 18 or older) was conducted. The open-label, single-dose, dose-escalation study consisted of three cohorts (0.3, 1 and 3 mg/kg) of adult patients with Hurler-Scheie or Scheie syndrome, which are attenuated or less severe forms of MPS I. The primary objective was to determine the safety and tolerability of a single infusion of AGT-181. Secondary objectives included evaluation of the pharmacokinetics (PK) and pharmacodynamics (PD) of single doses of AGT-181, determination of well-tolerated dose(s) to take into the pediatric multi-dose stage of the trial, and evaluation of the occurrence of single-dose infusion reactions. AGT-181 was shown to be safe and well-tolerated and a review of the collected data supported the decision to move into the pediatric Phase 2 POC.

About Hurler Syndrome
Hurler syndrome is a rare, hereditary, lysosomal storage disease that arises from a deficiency or absence of the enzyme iduronidase (IDUA), which is needed to break down complex sugars produced by the body. Hurler syndrome affects the brain and spinal cord in children, resulting in debilitating signs and symptoms that include developmental delay, progressive mental decline, loss of physical function, impaired language development (due to hearing loss and an enlarged tongue), corneal and retinal damage, carpal tunnel syndrome, and restricted joint movement. Hurler syndrome affects approximately 3,000 patients worldwide, with approximately 6.7 percent of affected patients in the U.S.

Hurler syndrome is also known as mucopolysaccharidosis I or MPS I. Attenuated or less severe forms of MPS I include Hurler-Scheie and Scheie syndromes. Patients with Hurler-Scheie syndrome may suffer from mild cognitive impairment or problems with attention. Patients with Scheie syndrome generally have a later onset and milder symptoms with a slower disease progression, although they can develop significant systemic morbidity.

About AGT-181

AGT‑181 is a novel, investigational enzyme replacement therapy (ERT) for the treatment of neurological complications in patients with Hurler syndrome. Using ArmaGen's proprietary technology, AGT-181 takes advantage of the body's natural system for transporting products across the blood-brain barrier (BBB) by targeting the same receptor that delivers insulin to the brain. ArmaGen developed AGT-181 by re-engineering an enzyme called iduronidase (IDUA) as an immunoglobulin G (IgG) fusion protein. The fusion protein binds to insulin receptors located on the surface of the BBB, enabling its passage into the brain.

About ArmaGen

ArmaGen, Inc. is a privately held biotechnology company focused on developing revolutionary therapies for severe neurological disorders. The company is developing a robust pipeline of innovative therapies for the treatment of neurological complications of lysosomal storage disorders such as Hunter syndrome, Hurler syndrome, metachromatic leukodystrophy and Sanfilippo A syndrome, as well as central nervous system diseases such as Alzheimer's and Parkinson's. ArmaGen's pipeline is based on decades of scientific leadership in engineering therapies to cross the BBB and a dominant intellectual property portfolio. The company is advancing its pipeline through licensing and collaboration agreements, in-house development programs, and future partnering opportunities. For more information, visit www.armagen.com.

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