ArmaGen Announces FDA Acceptance of IND Application for AGT-181 for the Treatment of Hurler Syndrome

Calabasas, Calif., April 8, 2015 – ArmaGen, Inc., a privately held biotechnology company focused on developing novel therapies to treat severe neurological disorders, announced today that the Investigational New Drug Application (IND) for the company's second product candidate, AGT-181 for the treatment of Hurler syndrome, has been accepted by the U.S. Food and Drug Administration (FDA) and is now active. ArmaGen expects to initiate a Phase 1 clinical trial in the second quarter of 2015 to assess the safety and tolerability of AGT-181 in adult patients with mucopolysaccharidosis type I (MPS I).

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.

"The FDA's acceptance of the IND allows us to begin activating clinical sites in the U.S. and enrolling patients into the study," said Steven L. Schoenfeld, M.D., Vice President of Clinical Affairs at ArmaGen. "We are looking forward to working hand-in-hand with physicians and the patient community to conduct the Phase 1 study of AGT-181, with the goal of eventually providing a treatment option for currently unaddressed neurological complications of severe Hurler syndrome."

The primary objective of the Phase 1 trial is to test the safety and determine a well-tolerated dose of AGT-181 in adult patients with Hurler-Scheie and Scheie, which are less severe forms of MPS I. ArmaGen plans to enroll nine patients 18 years and older into the study. These patients will be treated for a total of eight weeks, with all enrolled patients receiving AGT-181. Further details on the trial can be found on www.clinicaltrials.gov (https://clinicaltrials.gov/ct2/show/NCT02371226?term=AGT-181&rank=1) or using the identifier number NCT02371226. Pending the results of the Phase 1 trial, ArmaGen plans to conduct subsequent studies in a broader population of patients with MPS I, including those with Hurler syndrome.

"This is our second investigational product to enter clinical trials for the treatment of lysosomal storage disease, and we are proud to be independently advancing a potential therapy for patients with Hurler syndrome that may offer a potentially important alternative to experimental treatments such as stem cell transplantation," said James Callaway, Ph.D., Chief Executive Officer of ArmaGen. "This development marks a significant milestone for ArmaGen and our pipeline of therapies for severe neurological disorders."

ArmaGen announced in December 2014 that the company's IND for AGT-182 for the treatment of Hunter syndrome had been accepted by the FDA. ArmaGen is developing AGT-182 in partnership with Shire plc as part of a worldwide licensing and collaboration agreement.

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 in the U.S.[1] Hurler syndrome is also known as mucopolysaccharidosis I or MPS I. Less severe forms of MPS I include Hurler-Scheie and Scheie syndromes. Although less severe than Hurler syndrome, patients with Hurler-Scheie patients may suffer from mild cognitive impairment or problems with attention. Scheie patients 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 CNS 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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[1] Mucopolysaccharidoses Fact Sheet. National Institute of Neurological Disorders and Stroke. NINDS, April 16, 2014. http://www.ninds.nih.gov/disorders/mucopolysaccharidoses/detail_mucopolysaccharidoses.htm