ArmaGen Receives Rare Pediatric Disease Designation from FDA for AGT-181 for the Potential Treatment of Hurler Syndrome (Mucopolysaccharidosis Type I)

CALABASAS, Calif., November 5, 2015 -- ArmaGen Inc. today announced that the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation to AGT-181, a potential treatment for patients with Hurler syndrome, a disease that is also known as mucopolysaccharidosis type I (MPS I). The FDA previously granted AGT-181 Orphan Drug status, based on the prevalence of Hurler syndrome, which affects approximately 3,000 patients worldwide. The designation of rare pediatric disease status will make ArmaGen eligible to request a Rare Pediatric Disease Priority Review Voucher ("Rare Pediatric DPRV") upon approval of AGT-181 by the FDA.

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 and into the central nervous system by targeting the receptor that delivers insulin to all cells of the body.

"Receipt of rare pediatric disease designation strengthens ArmaGen's commitment to providing children, families and physicians with treatment options for the currently unaddressed complications of Hurler syndrome," said James Callaway, Ph.D., Chief Executive Officer of ArmaGen. "As we advance this therapeutic option towards approval, ArmaGen will be positioned to utilize the Rare Pediatric DPRV to expedite review of future investigational products, or leverage its sale to reinvest in other programs in our pipeline."

About Rare Pediatric Disease Designation

The FDA defines a "rare pediatric disease" as a disease that affects fewer than 200,000 individuals in the U.S. primarily aged from birth to 18 years. Under the FDA's Rare Pediatric DPRV program, a sponsor who receives an approval of a new drug application (NDA) or biologics license application (BLA) for a rare pediatric disease may be eligible for a voucher, which can be redeemed to obtain expedited FDA review for any subsequent marketing application. The DPRV may be sold or transferred by the recipient.

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 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 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 orphan disease pipeline of innovative therapies for the treatment of neurological complications of lysosomal storage disorders such as Hunter syndrome, Hurler syndrome, metachromatic leukodystrophy and Sanfilippo 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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