# ArmaGen's AGT-181 Granted Fast Track Designation for the Treatment of Hurler Syndrome (MPS I)

**Calabasas, Calif., November 30, 2017 –** ArmaGen, Inc., a privately held biotechnology company focused on developing groundbreaking therapies to treat severe neurological disorders, today reported that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to AGT-181, a novel, investigational enzyme replacement therapy for the treatment of both somatic and cognitive symptoms in patients with Hurler syndrome (also known as mucopolysaccharidosis type I, or MPS I).

The initial results from an ongoing Phase 2 proof-of-concept (POC) study were presented in February 2017 at the 13th Annual WORLDSymposium in San Diego, California. The data suggested that AGT-181 improves neurocognitive function in patients with MPS I, demonstrating the ability of ArmaGen's proprietary drug delivery technology to transport biopharmaceuticals across the blood-brain barrier. The study has since fully enrolled and final results will be presented at the 14th Annual WORLDSymposium in San Diego, California in February 2018.

"FDA's decision to grant Fast Track designation will allow us to interact with the Agency in the most efficient manner in order to advance AGT-181 into a pivotal trial and future registration," said Mathias Schmidt, Ph.D., chief executive officer of ArmaGen. "AGT-181 has the potential to provide patients with MPS I a treatment option that addresses the unmet cognitive disease burden facing these patients."

A Fast Track designation is aimed at accelerating the development and regulatory review of drugs meeting urgent needs. To receive the designation, a therapy candidate must demonstrate an advantage over currently available treatments such as superior efficacy, ability to meet an unmet medical need, or fewer side effects.

**About Mucopolysaccharidosis I (MPS I)**
MPS I 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. MPS I affects approximately 3,000-4,000 patients worldwide. The most severe form of MPS I, Hurler syndrome affects the brain and spinal cord in children, resulting in medical and cognitive challenges that can include developmental delay, progressive mental decline, loss of physical function, impaired language development, airway obstruction, corneal and retinal damage, carpal tunnel syndrome, and restricted joint movement. 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, milder symptoms, and a slower disease progression, although they can develop significant morbidity.

**About AGT-181**
AGT‑181 is a novel, investigational enzyme replacement therapy for the treatment of both somatic and cognitive symptoms in patients with MPS I. ArmaGen developed AGT-181 by re-engineering the enzyme iduronidase (IDUA) as fusion protein with an immunoglobulin G (IgG) antibody targeting the insulin receptor. Utilizing ArmaGen's proprietary "Trojan Horse" technology, AGT-181 takes advantage of the body's natural system for transporting proteins and other large molecules non-invasively across the blood-brain barrier (BBB), in this case by binding the same receptor that transports insulin across the BBB into the brain.

**About ArmaGen**
ArmaGen, Inc., is a privately held biotechnology company focused on developing groundbreaking therapies for severe neurological disorders. The company is developing a robust pipeline of innovative therapies for the treatment of lysosomal storage disorders including neurological symptoms such as

Hurler syndrome (MPS I), Hunter syndrome (MPS II), metachromatic leukodystrophy, Sanfilippo A and B syndromes, as well as other diseases with severe CNS manifestations. ArmaGen's pipeline is based on decades of scientific leadership in engineering therapies to cross the blood-brain barrier and a dominant intellectual property portfolio. The company is advancing its pipeline through licensing and collaboration agreements, in-house development programs, and other partnering opportunities. For more information, visit www.armagen.com.

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**Contacts:**

**ArmaGen, Inc.**
Derek Kelaita
Vice President, Business Development
818-252-8200
dkelaita@armagen.com

For media inquiries:
Alex Van Rees
SmithSolve LLC
973-442-1555 ext. 111
alex.vanrees@smithsolve.com