



## **ArmaGen Presents Data from First Cohort of Phase 1/2a of AGT-182 for Treatment of Hunter Syndrome**

**Calabasas, Calif., July 14, 2016** – ArmaGen, Inc., a privately held biotechnology company focused on developing revolutionary therapies to treat severe neurological disorders, announced today the presentation of data from the first cohort of adult patients (n=4) enrolled in the Phase 1/2a Breaking Barriers clinical trial of AGT-182, an investigational enzyme replacement therapy (ERT) for the treatment of Hunter syndrome. Also known as MPS II, Hunter syndrome is a rare, severe, progressive, and life-limiting lysosomal storage disorder.

The Breaking Barriers trial is an open-label, sequential, multi-dose study designed to determine a safe and well-tolerated dose of AGT-182, a compound that utilizes the body's natural system for transporting products across the blood-brain barrier (BBB) by targeting the receptor that delivers insulin to all cells of the body, including the brain. Its ability to cross the BBB makes AGT-182 unique among potential treatments for Hunter syndrome.

The data, presented at MPS 2016, the 14<sup>th</sup> International Symposium on MPS and Related Diseases in Bonn, Germany, show that in a small (n=4) cohort, a weekly 1.0-mg/kg dose of AGT-182, administered as an intravenous (IV) infusion to attenuated Hunter patients, was generally well-tolerated. Based on a review of the available safety, clinical, and bioanalytical data, an independent Data Monitoring Committee (DMC) has recommended proceeding to the study's second cohort, in which adult patients are to receive a weekly IV infusion starting at a dose of 3 mg/kg. As with the first cohort, patients enrolled in the second cohort will be age 18 and over.

"We are making steady progress in developing a viable therapeutic for Hunter syndrome that crosses the BBB," said James Callaway, Ph.D., chief executive officer of ArmaGen. "Favorable data from the first cohort of patients suggest that AGT-182 can safely address somatic symptoms of the disease. We are grateful for the investigators and patients who have participated in the Breaking Barriers trial to date, and we look forward to beginning the second cohort as recommended by the data monitoring committee in an effort to confirm these preliminary data."

At MPS 2016, Patrice Rioux, M.D., Ph.D., presented data from four adult male patients with Hunter syndrome who received weekly IV infusions of AGT-182. Measurements taken at week eight showed clinically significant decreases in liver and spleen volumes in two of the four patients. Levels of urinary glycosaminoglycans (GAGs), a marker for the disease, also showed clinically significant decreases in four of the four patients.

The investigators observed transient decreases in glucose levels (blood glucose levels <70 mg/dL) in all patients but did not consider these episodes clinically significant. Other adverse events (AEs) possibly related to AGT-182, included anxiety, headache, throat itchiness, lightheadedness, nausea, jitters, clammy hands, and weakness. AEs unlikely related to AGT-182 included mild leg cramps, leg pain, sleep disturbances, hip pain, decreased endurance,

fatigue, abdominal rash, emesis prior to dose start, cough, headache, back pain, neck pain, and migraine.

The favorable data from cohort 1 informed the DMC's recommendation to proceed to cohort 2, in which up to four adult patients are to receive a weekly IV infusion starting at a dose of 3.0 mg/kg.

"We are very encouraged by the safety and tolerability data from cohort 1, as well as by the somatic effect of AGT-182, as these findings suggest that participating in a 6-week washout period followed by 8 weeks of AGT-182 does not have any negative impact on the symptoms of the disease, which allows us to advance to the next dose level (3 mg/kg)," said Dr. Rioux, who is senior vice president of Global Clinical Development at ArmaGen. "We look forward to initiating the second cohort of this study as we continue to advance the clinical development of this promising compound, which may address the developmental delays and other neurological symptoms associated with Hunter syndrome."

ArmaGen entered into a worldwide licensing and collaboration agreement with Shire in 2014 which could include potential payments of up to \$225 million to develop AGT-182 for the treatment of both the central nervous system (CNS) and somatic (body-related) manifestations of Hunter syndrome. Under the terms of the agreement, ArmaGen will receive R&D funding, development and sales milestones, and future royalties from Shire.

Further details on the Breaking Barriers trial can be found on <https://clinicaltrials.gov/ct2/home> using the identifier number NCT02262338, or at <http://breakingbarriershuntertrial.com/>.

### **About Hunter Syndrome**

Hunter syndrome, also known as mucopolysaccharidosis type II, or MPS II, is a lysosomal storage disorder caused by inadequate activity of the enzyme iduronate-2-sulfatase (IDS), which is needed to break down complex sugars produced by the body. The buildup of these complex sugars, known as mucopolysaccharides, interferes with functioning of certain cells and organs, leading to serious complications including developmental delays and mental impairment. Symptoms of Hunter syndrome include growth delay, joint stiffness and coarsening of facial features. In severe cases, patients experience respiratory and cardiac problems, enlargement of the liver and spleen, and neurological deficits that can lead to premature death. Hunter syndrome primarily affects males and is almost always severe, progressive and life-limiting. Available treatments for Hunter syndrome are not expected to cross the BBB in clinically relevant amounts and therefore do not address the progressive neurological complications of the disease.

### **About AGT-182**

AGT-182 is a novel, investigational ERT that has received orphan drug designation from both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Using ArmaGen's proprietary technology, AGT-182 is designed to take advantage of the body's natural system for transporting products across the BBB by binding to the same receptor that delivers insulin to the brain. AGT-182 is engineered by the fusion of the replacement IDS enzyme to an antibody that is attracted to a receptor on the BBB. The IDS enzyme is designed to travel through the BBB attached to that antibody.

## **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](http://www.armagen.com).

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