ArmaGen Announces FDA Acceptance of IND Application for AGT-182 for the Treatment of Hunter Syndrome

Calabasas, Calif., December 11, 2014 – ArmaGen, Inc., a privately held biotechnology company focused on developing novel therapies to treat severe neurological disorders, announced today that the Investigational New Drug (IND) application for the company's lead product candidate, AGT-182 for the treatment of Hunter syndrome, has been accepted by the U.S. Food and Drug Administration (FDA) and is now active. This will enable ArmaGen to initiate a Phase 1 clinical trial, which is expected to begin in the first quarter of 2015, to assess the safety and tolerability of AGT-182 in adult male patients with Hunter syndrome.

AGT-182 is an investigational enzyme replacement therapy (ERT) for the treatment of Hunter syndrome. Also known as mucopolysaccharidosis type II, or MPS II, Hunter syndrome is a rare, severe, progressive and life-limiting lysosomal storage disorder.

"Commercially available treatments for Hunter syndrome lack the ability to penetrate the blood-brain barrier in clinically relevant amounts, and therefore do not address the severe and progressive neurological complications of the disease," said James Callaway, Ph.D., Chief Executive Officer of ArmaGen. "Now that the IND is active, we hope to soon be able to demonstrate the safety of AGT-182 and eventually fill a significant unmet need for the Hunter syndrome community."

AGT-182 is designed to utilize 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.

"We encourage patients and caregivers living with Hunter syndrome to learn more about ongoing clinical trials of investigational therapies," said Barbara Wedehase, MSW, CGC, Executive Director of the National MPS Society. "Delivery of enzyme replacement therapeutics into the central nervous system has long been a priority for physicians, patients and caregivers seeking to address the developmental delays and other neurological symptoms experienced by many patients with MPS syndromes."

As previously announced, ArmaGen entered into a worldwide licensing and collaboration agreement with Shire plc valued at $225 million to develop AGT-182 for the treatment of both the central nervous system (CNS) and somatic (body-related) manifestations of Hunter syndrome. ArmaGen is responsible for conducting the Phase 1 study of AGT-182. Under the terms of the agreement, ArmaGen will receive R&D funding, development and sales milestones, and future royalties from Shire.

About Hunter Syndrome
Hunter syndrome 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 known 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 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 against the insulin receptor. 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 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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