ArmaGen Reports Preliminary Evidence of Cognitive Improvement in Children with Hurler Syndrome (MPS I) Treated with AGT-181

Initial Results from Phase 2 Proof-of-Concept Trial Presented at 2017 WORLDSymposium

Findings Demonstrate Ability of ArmaGen's Proprietary Drug Delivery Technology to Transport Biopharmaceuticals Across the Blood-Brain Barrier

Calabasas, Calif., February 16, 2017 – ArmaGen, Inc., a privately held biotechnology company focused on developing groundbreaking therapies to treat severe neurological disorders, today reported preliminary evidence of cognitive improvement in children treated with AGT-181, the company's investigational therapy for the treatment of Hurler and Hurler-Scheie syndrome (also known as mucopolysaccharidosis type I, or MPS I). The initial results from an ongoing Phase 2 proof-of-concept (POC) study, presented today at the 13th annual WORLD*Symposium* in San Diego, California, suggest that AGT-181 may improve cognitive function in patients with MPS I, demonstrating the ability of ArmaGen's proprietary drug delivery technology to transport biopharmaceuticals across the blood-brain barrier.

In an oral presentation entitled, "Intravenous infusion of iduronidase-IgG and its impact on the central nervous system in children with Hurler syndrome," Roberto Giugliani, M.D., Ph.D., of Hospital de Clínicas in Porto Alegre, Brazil, reported improvements in neurological and cognitive function in four of five patients, and stabilization of neurological and cognitive function in the fifth patient. He also noted that AGT-181 was similarly efficacious somatically (related to the rest of the body other than the brain) to currently available enzyme replacement therapy (ERT) and displayed a favorable safety and tolerability profile.

"Existing enzyme replacement therapies improve many somatic manifestations of Hurler Syndrome, but they do not address the severe and progressive neurological symptoms, as they do not cross the blood-brain barrier," explained Dr. Giugliani. "While our findings are still preliminary, we are satisfied with the safety profile of AGT-181 and encouraged by the indications of potential improvement in cognitive markers from neuropsychological tests conducted with MPS I children treated with AGT-181. We hope to further confirm these results as the study continues."

At the WORLD*Symposium*, Dr. Giugliani presented up to 26 weeks of data from the first five children with MPS I (age 2 years or older) enrolled in the 6-month study. All five patients had previously been treated with standard ERT, and one had received a stem cell transplant which had failed engraftment. Once enrolled, the children received weekly intravenous infusions of AGT-181 at doses of 1.0 or 3.0 mg/kg. Developmental age-appropriate neurocognitive testing was conducted at 13 and 26 weeks of treatment, utilizing either the Bayley Scales of Infant Development III; or the Kaufman Assessment Battery for Children. Together, the two tests (commonly abbreviated as B-K) represent a summation of scores from cognitive, language and motor skills.

Four of the five patients demonstrated improvement in their B-K cognitive score domain, and the patient who had failed engraftment after stem cell transplantation showed stabilization in this domain. Somatic disease control under AGT-181 was similar to what is commonly observed under standard ERT, based on stabilization of urinary glycosaminoglycan (GAG) levels and either stabilization or reduction in liver and/or spleen volume. Additionally, a trend towards improvement in shoulder range of motion was observed.

"Successful delivery of biotherapeutics across the blood-brain barrier is a goal that has eluded the entire pharmaceutical industry for decades," said Mathias Schmidt, Ph.D., chief executive officer of ArmaGen. "We are therefore extremely gratified to see clinical data that indicate ArmaGen's proprietary 'Trojan horse' technology can deliver the missing enzyme into the CNS of patients and modulate cognitive function. Beyond MPS, the technology has immense potential utility in other CNS disorders, and we are excited about exploring these opportunities in further research. The ArmaGen staff wants to cordially thank the patients and their parents for engaging in this study."

Drug-related adverse events (AEs) in the Phase 2 POC trial included two infusion site reactions and two hypoglycemic events that were transient and well controlled by glucose administration. No serious AEs were observed that were likely to be drug-related.

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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