ArmaGen Receives U.S. Orphan Drug Designation for AGT-184

Novel Enzyme Replacement Therapy in Development for the Treatment of Sanfilippo A Syndrome

Calabasas, Calif., August 2, 2018 – ArmaGen, Inc., a privately held biotechnology company focused on developing groundbreaking therapies to treat severe neurological disorders, today announced that the U.S. Food and Drug Administration's (FDA) Office of Orphan Products Development granted Orphan Drug Designation to GT-184 for the treatment of mucopolysaccharidosis type IIIA (also known as Sanfilippo Syndrome A or MPS IIIA). MPS IIIA is a lysosomal storage disease (LSD) that arises from a deficiency in the gene encoding for the enzyme N-sulfoglucosamine sulfohydrolase (SGSH), which results in a buildup of complex sugar polymers within the brain causing progressive intellectual disability and the loss of previously acquired skills (developmental regression).

AGT-184 is an IgG-SGSH fusion protein, where the IgG domain is a human anti-insulin receptor monoclonal antibody. The insulin receptor antibody domain triggers transport of the AGT-184 fusion protein across the blood brain barrier (BBB), via binding to endogenous insulin receptors present on the BBB. ArmaGen is currently conducting IND-enabling activities (manufacturing/toxicology) with the goal of filing an IND with the U.S. FDA in late 2019.

"We are very pleased to receive FDA orphan drug designation for AGT-184, as this designation is an important regulatory milestone for the Company as we work to develop a potential treatment option for patients suffering from this rare and life-threatening disease," said Mathias Schmidt, Ph.D., Chief Executive Officer of ArmaGen. "This moment represents a transformational period in the Company's evolution as we prepare to leverage our clinically validated BBB platform to advance additional pipeline assets."

The FDA grants Orphan Drug Designation to drugs intended to treat a rare disease or condition affecting fewer than 200,000 people in the U.S. This designation confers special incentives to the drug developer, including tax credits on the clinical development costs, prescription drug user fee waivers and may entitle a period of seven-year market exclusivity in the US upon FDA approval.

In a mouse model of MPSIIIA, a surrogate version of AGT-184 (AGT-m184) showed activity in both CNS and somatic organs, further substantiating the applicability of ArmaGen's Trojan Horse approach to MPS IIIA. Mice were treated three times per week for six weeks with AGT-m184. Compared to the control mice, there was a 70% reduction in brain heparan sulfate levels at the end of the six-week study, which provides direct evidence that the fusion protein crossed the BBB and delivered the SGSH enzyme payload into the brain. There was also an 85% reduction in liver heparan sulfate levels as compared to control, which is evidence of somatic or peripheral activity of the IgG-SGSH fusion protein.

About AGT-184

AGT‑184 is an investigational enzyme replacement therapy (ERT) for the treatment of the cognitive effects of Sanfilippo A syndrome, a lysosomal storage disease that arises from a deficiency in the gene encoding for the enzyme N-sulfoglucosamine sulfohydrolase (SGSH), which results in a buildup of complex sugar polymers in brain cells. Currently, there are no approved therapies for the treatment of Sanfilippo A syndrome. Unlike recombinant SGSH, which does not cross the BBB, AGT-184 is a re-engineered form of the SGSH enzyme that is able to penetrate the BBB, and which has the same enzyme activity of the native SGSH enzyme.

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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