ArmaGen and Rett Syndrome Research Trust Collaborate to Develop Novel Therapeutic for the Treatment of Rett Syndrome

CALABASAS, CA, and TRUMBULL, CT, July 7, 2016 — ArmaGen, Inc., a privately held biotechnology company focused on developing revolutionary therapies to treat severe neurological disorders, announced today a collaboration with the Rett Syndrome Research Trust (RSRT) to develop a novel therapeutic for the treatment of Rett Syndrome, a rare genetic postnatal neurological disorder that affects girls almost exclusively.

Rett Syndrome is caused by mutations in a protein called MeCP2 (methyl CpG binding protein 2) that is critical for maintaining the health of brain cells. Under the terms of the research collaboration, ArmaGen will utilize its expertise and technology to develop a form of the MeCP2 protein that can cross the blood-brain barrier (BBB) in an effort to compensate for the mutated protein. RSRT, a non-profit organization dedicated to advancing a cure for Rett Syndrome, will fund the development of this novel fusion construct as well as lend technical support to the experiments. ArmaGen will be responsible for any future development activities.

“RSRT is especially interested in strategies that target the root of the problem in Rett Syndrome, insufficient levels of MeCP2 in the brain, as this will likely provide a profound improvement in symptoms. Protein replacement therapy is one such strategy so we are delighted to launch this effort collaboratively with ArmaGen,” said Randall Carpenter, MD, Chief Scientific Officer at RSRT.

The ArmaGen technology of delivering proteins into the brain takes advantage of naturally occurring receptors on the surface of the BBB that shuttle complex biologics into the brain. By fusing the MeCP2 protein together with an antibody to the receptor, the “Rett fusion protein” essentially piggybacks its way into the brain. In order for this program to be successful, the MeCP2 protein will need to retain the ability to function despite being fused, and importantly, will need to enter the brain in sufficient quantities to be efficacious.

“Rett Syndrome is a unique disorder in that restoring normal levels of MeCP2 in preclinical models dramatically reverses symptoms, even in late stage disease. This offers the distinct possibility that protein replacement therapy for Rett could be disease modifying. This partnership will allow us to continue to expand our pipeline beyond lysosomal storage diseases and into other orphan neurological diseases, which may benefit from delivery of the target molecule across the BBB,” said James Callaway, Ph.D., President & CEO of ArmaGen.

About Rett Syndrome

Rett Syndrome is a genetic childhood neurological disorder that almost exclusively affects girls. It strikes randomly, typically at the age of 12 to 18 months, and is caused by random mutations of the *MECP2* gene on the X chromosome. Rett Syndrome is devastating as it deprives young girls of speech, hand use, normal movement often including the ability to walk. As the girls enter childhood the disorder brings anxiety, seizures, tremors, breathing difficulties, severe gastrointestinal and orthopedic issues. While their bodies suffer, it is believed that their cognitive abilities remain largely intact. Although most children survive to adulthood, they require total round-the-clock care.

About the Rett Syndrome Research Trust

The Rett Syndrome Research Trust (RSRT) is a non-profit organization with a highly personal and urgent mission: a cure for Rett Syndrome and related MECP2 disorders. In search of a cure and effective treatment options, RSRT operates at the nexus of global scientific activity. We enable advances in knowledge and drive innovative research through constant engagement with academic scientists, clinicians, industry, investors and affected families. These relationships catalyze the development and execution of a research agenda that neither academia nor industry could achieve alone. RSRT refutes the conventional practice of labs working in isolation, instead seeking out, promoting and funding collaborations and consortia in which scientists work across multiple disciplines. Since 2008, RSRT has awarded $36 million to research projects, more than any other Rett organization in the USA and abroad. To learn more about RSRT, please visit www.ReverseRett.org.

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.

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