



## ArmaGen Receives U.S. Orphan Designation for Lead Product AGT-182

**Santa Monica, CA – July 18, 2013** – ArmaGen announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to its lead product AGT-182 for the treatment of mucopolysaccharidosis type II (also known as Hunter syndrome or MPS II.) Hunter syndrome is a rare, genetic lysosomal storage disease caused by a deficient or absent enzyme, iduronate-2-sulfatase. It is a life-threatening disease affecting children as young as 2 years of age.

AGT-182 is a human insulin receptor monoclonal antibody-fused iduronate 2-sulfate designed to cross the blood brain barrier (BBB) through the insulin receptors present on the BBB. ArmaGen is currently preparing an IND for AGT-182 in order to begin clinical investigation in the first half of 2014.

“We are very pleased to receive FDA orphan drug designation for AGT-182. This designation is an important strategic milestone in the development of our program,” said James Callaway, Ph.D., Chief Executive Officer of ARMAGEN. “It represents a transformational moment in the Company’s evolution as we prepare for the IND and transition from a research-stage to a clinical-stage organization.”

Orphan Drug Designation is granted by the FDA Office of Orphan Drug Products 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.

“Through the development of AGT-182, ArmaGen hopes to address the significant unmet medical need for patients who suffer CNS impairment as a result of Hunter syndrome,” said ArmaGen founder and Chief Scientific Officer, William Partridge, M.D. “In addition, the Company will continue applying our BBB-penetrating approach to other compounds internally as well as through external collaborations with pharmaceutical partners.”

### About Hunter Syndrome

Hunter syndrome, or [mucopolysaccharidosis](#) type II (MPS II), is a serious [genetic disorder](#) that primarily affects males ([X-linked recessive](#)). It interferes with the body’s ability to break down and recycle specific [mucopolysaccharides](#), also known as [glycosaminoglycans](#) (GAG). Hunter syndrome is one of several related [lysosomal storage diseases](#).

In Hunter syndrome, GAG builds up in cells throughout the body due to a [deficiency](#) or absence of the [enzyme iduronate-2-sulfatase](#). This buildup interferes with the way certain cells and organs in the body function and leads to a number of serious symptoms. As the buildup of GAG continues throughout the cells of the body, signs of Hunter syndrome become more visible. Physical manifestations for some people with Hunter syndrome include distinct facial features and large head. In the majority of cases of Hunter syndrome, [central nervous system](#) involvement leads to developmental delays and [nervous system](#) problems. Hunter syndrome is almost always severe, [progressive](#), and life-limiting.

### **About ArmaGen**

Founded in 2004 by William M. Pardridge, M.D., ArmaGen provides platform technology solutions to the blood-brain barrier problem and can non-invasively target recombinant proteins, therapeutic monoclonal antibodies, small molecules and siRNA to the brain. Further information about ArmaGen is available at <http://www.armagen.com>.