

# ArmaGen Update on Hurler Syndrome Development Program

## AGT-181: Investigational Therapy for the Treatment of Patients with Hurler Syndrome

### About AGT-181

AGT-181 is an investigational enzyme replacement therapy designed to treat both the body-related and central nervous system-related symptoms and complications of MPS I.

Currently approved treatments for MPS I are unable to penetrate the blood-brain barrier (BBB), a filter that protects the brain from harmful substances like toxins and bacteria but allows vital substances like insulin to cross from the blood into the brain.

AGT-181 is designed to cross the BBB in the same way insulin does.

### 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 central nervous system 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](http://www.armagen.com).

### Phase 2

Proof-of-Concept Trial:

### What these results mean for the MPS community

Full 52-week data from the study were presented at the 14<sup>th</sup> Annual *WORLDSymposium* on February 8, 2018. These results confirm previous findings showing that ArmaGen's drug delivery technology can deliver therapies across the BBB, benefitting neurocognitive function in patients with severe MPS I. Now that ArmaGen has demonstrated proof of concept (POC), the company is considering next steps which may include a controlled Phase 3 clinical trial looking at long-term impact on cognition in patients with MPS I. The company will need to secure adequate funding or a collaboration partner in advance of initiating such a trial and these efforts are currently underway. ArmaGen is most grateful to the patients and families who participated in the Phase 2 POC study, and will provide additional details about the upcoming trial as they are available.



**Roberto Giugliani, M.D., Ph.D.,**  
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Porto Alegre, Brazil

*“While the existing enzyme replacement therapy improves many of the physical symptoms of MPS I, its inability to cross the BBB prevents it from addressing the severe and progressive neurological symptoms of the disorder that AGT-181 could potentially address.”*