ArmaGen Clinical Trial Update

New Trial Design for MPS I Study, Cohort 2 Enrollment Ongoing for MPS II

- March 2016 -

AGT-181 Phase 1 Trial for MPS I: Washout Now Optional

We're pleased to announce that patients on current enzyme replacement therapy (ERT) can choose whether or not to discontinue ERT before start of the trial (washout). The study duration will vary for those who prefer not to discontinue. These changes are based in part on valuable feedback from the patient community.

The trial is to test the safety and determine a well-tolerated dose of AGT-181 in adult patients with Hurler-Scheie and Scheie, which are attenuated or less severe forms of MPS I. ArmaGen plans to enroll nine patients 18 years and older into the study, with all enrolled patients receiving AGT-181. Pending the results of the Phase 1 trial, we plan to conduct subsequent studies in a broader population of patients with MPS I, including those with Hurler syndrome.

There is also an extension study, meaning that people who complete the Phase 1 trial and meet other study criteria can continue to receive AGT-181 as part of a six-month safety study.

To learn more about participating in the trials visit **clinicaltrials.gov** and use the identifier number **NCT02371226**.

AGT-181 and AGT-182 are an investigational ERTs. They are designed to treat symptoms and complications of MPS I and MPS II, respectively, both in the body and the central nervous system (brain and spinal cord). The Food and Drug Administration (FDA) has granted both compounds Orphan Drug Status.

Meet Our New SVP of Global Clinical Development

We are excited to introduce Patrice Rioux, M.D., Ph.D., as Senior Vice President of Global Clinical Development at ArmaGen. As the latest addition to our clinical team, Patrice is leading the development of AGT-181 for Hurler syndrome and AGT-182 for Hunter syndrome. He brings nearly four decades of drug development experience with significant expertise in neurodegenerative and rare diseases, most recently at Raptor Pharmaceuticals. We're happy to have him on board as we continue to make strides in our clinical trial programs with the support of the patient community.

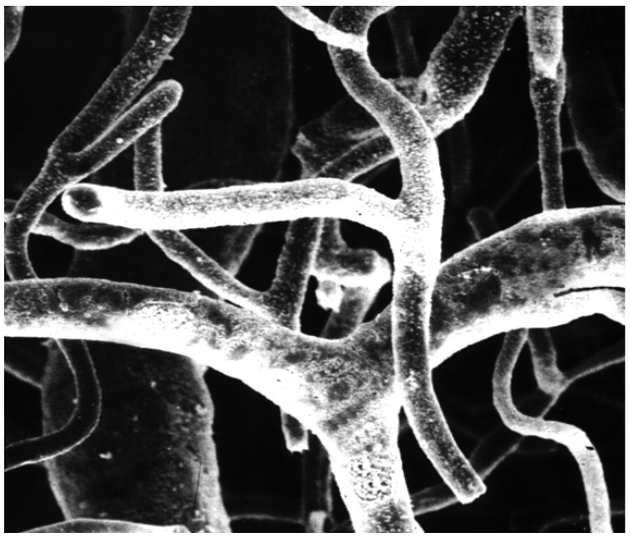
"I would like to thank everyone who is helping ArmaGen advance our compounds for devastating MPS diseases. Those who are selflessly participating in our trials truly are heroes and are potentially helping current and future generations of people with MPS I and MPS II."

AGT-182 Phase 1 Trial for MPS II:
Actively Enrolling Cohort/Group 2

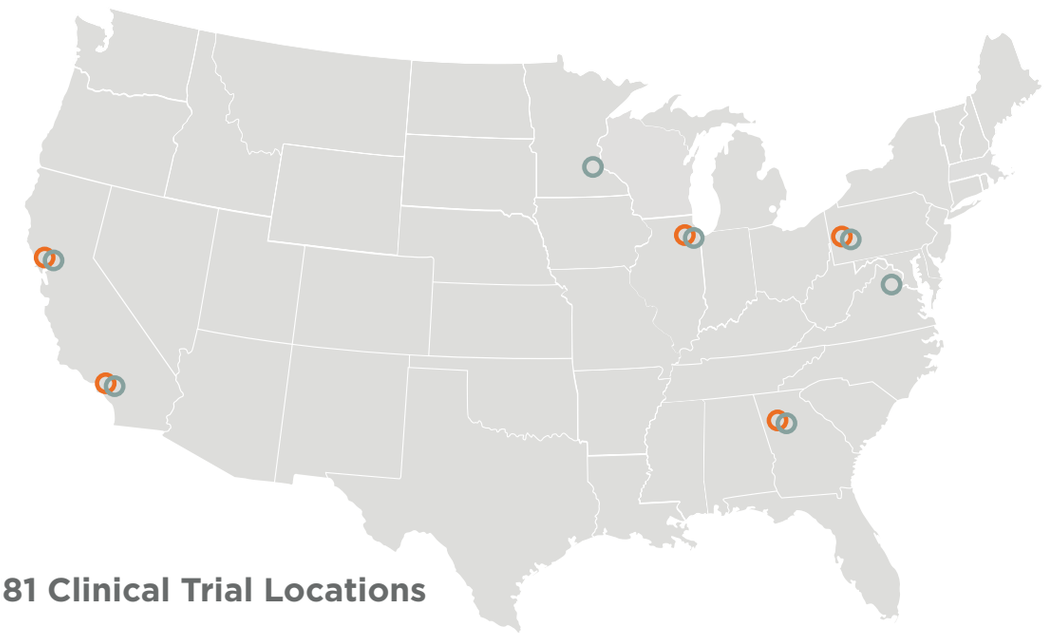
We're pleased to announce that we've completed enrollment of the first cohort/group of four patients. We are now enrolling in the second cohort.

ArmaGen plans to enroll 12 patients 18 years and older into the study. The purpose of this study is to test the safety and determine a well-tolerated dose of AGT-182 for male patients (age 18 years and older) with Hunter syndrome. These patients will be treated for a total of eight weeks, with all enrolled patients receiving AGT-182.

Please visit **breakingbarriershuntertrial.com** to learn more about participating in the trials



ArmaGen is developing therapies to non-invasively deliver drugs across the blood-brain barrier, a filter that protects the brain from toxins but allows vital nutrients like insulin to cross from the blood into the brain. Available medications for MPS I and II do not cross the blood-brain barrier in clinically relevant amounts and therefore do not address the progressive neurological complications of the diseases.



- **AGT-181 Clinical Trial Locations**
- **AGT-182 Clinical Trial Locations**

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MPS I & II TRIALS