Iduronidase Replacement Therapy of the Brain in Hurler's Syndrome

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Challenge/Problem:

Lysosomal storage disorders are genetic diseases, and most of these syndromes affect the brain. Enzyme replacement therapy, or ERT, is not effective in the brain, because the recombinant enzyme does not cross the blood-brain barrier (BBB).

Current/Near Term Products:

ArmaGen has developed a new IgG-enzyme fusion protein for the treatment of the brain of Hurler's Syndrome, also called MPS-I. The missing enzyme, iduronidase (IDUA), has been re-engineered as an IgG-IDUA fusion protein.

Approach:

A new approach to the BBB drug delivery problem is the use of molecular Trojan horses to ferry the biopharmaceutical across the human BBB. The recombinant enzyme is re-engineered as a fusion protein with the Trojan horse, which is a monoclonal antibody against a BBB-specific receptor.

Future Plans:

The new IgG-IDUA fusion protein has been shown to rapidly cross the primate BBB in vivo and enter the brain. The fusion protein is now being tested in Phase 0 GLP toxicology and safety pharmacology in Rhesus monkeys. Orphan Drug Designation has been granted by the FDA.

Business Name and Point of Contact:

Ruben J. Boado, Ph.D.
Vice-President
ArmaGen Technologies, Inc.
914 Colorado Ave., Santa Monica, CA 90401
Email: rboado@armagen.com
URL: www.armagen.com

Collaboration/Partnering Opportunities:

The Company is seeking partners to finance and/or execute a open label phase I-II clinical trial in Hurler's syndrome patients, treated with weekly intravenous injections of the IgG-IDUA fusion protein. This technology can be applied to other recombinant protein biopharmaceuticals.

Keywords: blood-brain barrier, monoclonal antibody, fusion protein, biopharmaceuticals, enzyme