

Abstract presented at ESGCT about Aspa's Canavan Disease gene therapy, on October 22, 2019

Aspa Therapeutics is pleased to share the news that a poster was presented on October 22, 2019 at the European Society of Gene and Cell Therapy meeting, describing an important development in gene therapy for Canavan disease. The poster, written by David Scott and colleagues from the BridgeBio Gene Therapy laboratory, describes a study conducted in non-human primates (monkeys), comparing three different methods of delivering the gene therapy that Aspa Therapeutics is developing for patients with Canavan disease. The researchers compared IV, (intravenous delivery, in which the treatment is administered through a small needle placed into a vein), IT (intrathecal, an injection into the cerebrospinal fluid in the space surrounding the spinal cord), and ICV (intracerebroventricular injection, where the treatment is infused through the skull into one of the large collections of cerebrospinal fluid that sits within the middle of the brain). The researchers then measured how well the therapy was able to reach the parts of the brain, spinal cord, and other tissues most relevant to Canavan disease. While all 3 methods showed some ability to reach the targeted areas, IV delivery was optimal in reaching all brain regions, especially the deep brain structures that appear to be most relevant to Canavan disease.

The researchers therefore recommended that that IV infusion be utilized to administer the Aspa investigational gene therapy to patients with Canavan disease.

IV infusion is one of the most common ways to administer treatments, and is the same mode of delivery used in the clinical trial that Avexis / Novartis recently conducted, leading to approval of their gene therapy for children with Spinal Muscular Atrophy Type 1 (SMA-1).