AAV gene therapy shows positive results in Batten disease trial

Batten disease is a neurologic disease that affects a patient's ability to walk, speak, think and see. Credit: Reimund Bertrams from Pixabay.

Amicus Therapeutics has announced interim data from a Phase I/II clinical trial of an AAV gene therapy for the treatment of CLN6 Batten disease in children.

Batten disease is a neurologic disease that affects a patient's ability to walk, speak, think and see. It currently lacks approved therapies.

The interim data is from an initial eight participants who were monitored for up to 24 months after a single intrathecal administration of the AAV-CLN6 gene therapy.

A positive impact on motor and language function was observed in seven patients treated with the gene therapy when compared to a natural history dataset and during comparisons in sibling pairs.

The impact was measured using the Hamburg Motor and Language Score, which assesses ambulation and speech. Seven patients maintained their Hamburg score or had an initial change and later stabilised.

Three children experienced stabilisation compared to their siblings in the natural history data set who were not treated and either had substantial declines in motor and language ability or died over the same period.

In the case of two in-study sibling pairs, the younger siblings had an increase or stabilisation relative to their older siblings, who had stabilisation after an initial change.

According to the results, the AAV-CLN6 gene therapy was generally well tolerated. Among 12 participants, most adverse events (AEs) were mild and not related to the therapy.

Amicus Therapeutics chairman and CEO John Crowley said: “These interim clinical data suggest that our gene therapy in CLN6 Batten disease has the potential to halt the progression of this devastating fatal disease that, untreated, destroys brain function and kills children.

“It is remarkable that most children in this study appear to show stabilisation, particularly the younger children who were able to maintain high baseline motor and language scores for up to two years.”

The company licensed the AAV-CLN6 gene therapy from the Abigail Wexner Research Institute (AWRI) at Nationwide Children’s Hospital.