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Efficacy and Safety of Cerliponase Alpha Therapy in Neuronal Ceroid Lipofuscinosis Type 2

Objective: Neuronal ceroid lipofuscinosis type 2 (CLN2) is a rare neurodegenerative disease characterized by epileptic seizures, loss of motor and language skills, vision loss and death between the ages of 7-10 years. The only approved treatment option is Cerliponase alpha. We report our treatment experience in two CLN2 patients receiving Intraventricular Cerliponase alpha therapy every two week for 18 months.

Material and Methods: We assessed the clinical, motor, cognitive function of our patients with CLN2 by Clinical Rating Scale (motor and language), Gross Motor Functional Scale (GMFS), Ataxia Evaluation and Grading Scale (SARA), Bayley III developmental screening scale before and every 3 months of the treatment. We also evaluated the retinal degeneration by optic coherence tomography before and every six months of the treatment.

Results: The CLN2 Clinical Rating Scale ,GMFS, SARA and Bayley III Developmental Screening Scale scores remained stable during the treatment period. We had to interrupt the treatment for 3 months in

one of our patients. Test scores worsened during that time period. She kept stable after the reinjection. No treatment related serious side effect was recorded. OCT didn't show any progression in retinal degeneration.

Conclusions: Regularly administered Cerliponase alpha delays the progression of motor, language, cognitive decline and inhibits retinal lipofuscin deposition in NCL2 patients

Keywords: NCL 2, Cerliponase Alfa

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