AAN: Cerliponase alfa beneficial for form of Batten's disease

April 26 2018

(HealthDay)—For children with neuronal ceroid lipofuscinosis type 2

(CLN2) disease, intraventricular infusion of cerliponase alfa is associated with reduced decline in motor and language function, according to a study published online April 24 in the *New England Journal of Medicine*. The research was published to coincide with the annual meeting of the American Academy of Neurology, held from April 21 to 27 in Los Angeles.

Angela Schulz, M.D., from the University Medical Center Hamburg-Eppendorf in Germany, and colleagues conducted a multicenter study to examine the effect of intraventricular infusion of cerliponase alfa every two weeks in children with CLN2 disease aged 3 to 16 years. Treatment was initiated at a dose of 30, 100, or 300 mg; all patients received the 300-mg dose for 96 weeks or more. Twenty-four patients were enrolled in the study; the efficacy population consisted of 23 patients.

The researchers found that the median time until a 2-point decline in the motor-language score was not reached for treated patients and was 345 days for 42 historical controls. The mean unadjusted rate of decline in the motor-language score per 48-week period was 0.27 ± 0.35 and 2.12 ± 0.98 points in treated patients and in the historical controls (mean difference, 1.85; P

"Intraventricular infusion of cerliponase alfa in patients with CLN2 disease resulted in less decline in motor and language function than that in historical controls," the authors write. "Serious adverse events included failure of the intraventricular device and device-related infections."

The study was funded by BioMarin Pharmaceutical.

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