

Stem cell gene therapy safe for adrenoleukodystrophy

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(HealthDay)—Early results suggest that stem cell gene therapy is a safe

and effective treatment for boys with early-stage cerebral adrenoleukodystrophy, according to a study published online Oct. 4 in the *New England Journal of Medicine* to coincide with the 2017 Child Neurology Society Annual Meeting, being held Oct. 4 to 7 in Kansas City.

Florian Eichler, M.D., from Massachusetts General Hospital in Boston, and colleagues enrolled 17 boys with cerebral adrenoleukodystrophy in a single-group, open-label, phase 2-3 safety and efficacy study involving infusion of autologous CD34+ cells transduced with the elivaldogene tavalentivec (Lenti-D) lentiviral vector. Inclusion criteria included having early-stage [disease](#) and gadolinium enhancement on [magnetic resonance imaging](#) (MRI).

The interim analysis (median follow-up of 29.4 months) showed that all patients had gene-marked cells after engraftment, with no evidence of preferential integration near known oncogenes or clonal outgrowth. All patients also had measurable ALD protein. There were no reports of treatment-related death or graft-versus-host disease. Fifteen of the 17 [patients](#) were alive and free of major functional disability, with minimal clinical symptoms. One patient with rapid neurologic deterioration died of disease progression, while another patient with MRI evidence of [disease progression](#) withdrew from the study to undergo allogeneic stem cell transplantation but later died of transplantation-related complications.

"Additional follow-up is needed to fully assess the duration of response and long-term safety," conclude the authors.

Several authors disclosed potential conflicts of interest.

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