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Long-term effect of bone-marrow transplantation for childhood-onset cerebral X-linked adrenoleukodystrophy

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Summary

Background

The childhood-onset cerebral form of X-linked adrenoleukodystrophy, a demyelinating disorder of the central nervous system, leads to a vegetative state and death within 3–5 years once clinical symptoms are detectable. The hypothesis to be tested was whether bone-marrow transplantation can over an extended period of time halt the inexorable progressive demyelination and neurological deterioration.

Methods

12 patients with childhood onset of cerebral X-linked adrenoleukodystrophy have been followed for 5–10 years after bone-marrow transplantation. Magnetic resonance

imaging (MRI), neurological, neuropsychological, electrophysiological, and plasma very-long-chain fatty acid (VLCFA) measurements were used to evaluate the effect of this treatment.

Findings

MRI showed complete reversal of abnormalities in two patients and improvement in one. One patient showed no change from baseline to last follow-up. All eight patients who showed an initial period of continued demyelination stabilised and remained unchanged thereafter. Motor function remained normal or improved after bone-marrow transplantation in ten patients. Verbal intelligence remained within the normal range for 11 patients. Performance (non-verbal) abilities were improved or were stable in seven patients. Decline in performance abilities followed by stability occurred in five patients. Plasma VLCFA concentrations decreased by 55% and remained slightly above the upper limits of normal.

Interpretation

“10-year follow-up of 12 patients with childhood-onset cerebral X-linked adrenoleukodystrophy shows the long-term beneficial effect of bone marrow transplantation when the procedure is done at an early stage of the disease.



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