T-cell-depleted peripheral blood stem cell transplantation for alpha-mannosidosis.

Abstract:

Alpha-mannosidosis (alpha-mannosidosis) is a lysosomal storage disease characterized by accumulation of oligosaccharides in various tissues leading to symptoms such as coarse facial features, dysostosis multiplex, hearing disabilities, mental developmental delay and skeletal involvement (dysostosis multiplex). Without treatment, the severe infantile onset form of this autosomal recessive disease leads to progressive neurodegeneration and sometimes to early death. Stem cell transplantation has been shown to be an effective treatment. In the five patients published so far, correction of skeletal abnormalities and improvement of neuropsychological capabilities have been observed. We report the first patient who received a T-cell-depleted peripheral blood stem cell transplantation (PBSCT) for alpha-mannosidosis. The diagnosis of alpha-mannosidosis was made at the age of 14 months. At the age of 24 months, he underwent PBSCT with T-cell depletion by CD34-positive selection from his HLA phenotypically identical mother. Conditioning was carried out with busulfan (20 mg/kg), cyclophosphamide (200 mg/kg), OKT3 and methylprednisolone. The patient is alive and well 27 months after PBSCT and has made significant developmental progress. The pattern of urinary oligosaccharides has returned to almost normal.
CD34-positive-selected PBSCT is a feasible option to reduce risk for GVHD for these patients.

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