Reply to: Reduced intensity conditioning haematopoietic stem cell transplantation with mesenchymal stromal cells infusion for the treatment of metachromatic leukodystrophy: a case report. Haematologica 2008; 93:e11-13

We have read with interest and concern the recent case report[1] of a patient with metachromatic leukodystrophy (MLD) who underwent hematopoietic stem cell transplantation augmented with an infusion of donor-derived mesenchymal stem cells.

First of all, it is not clear that the patient had MLD. The standard diagnostic methods for diagnosis of MLD,[2] such as levels of urinary sulfatides and leukocyte arylsulfatase A (only urinary arylsulfatase A was detected in the low range), or mutation analysis of the arylsulfatase A gene have not been reported in this case. The pathological description of the peripheral nerve disease is uncommon for MLD. The MRI lesions of both grey and white matter, as described in this patient, are very unusual for adult MLD. Second, the report suggests that infusion of mesenchymal stem cells was important for the engraftment and for improvement of the peripheral nerve dysfunction. This is clearly a possibility, as suggested previously by Koc *et al*.,[3] that warrants further investigations.

Since the diagnosis of MLD is questionable, however, these results are not interpretable.

The 40 months duration of survival after transplantation *with stabilization* alone is not evidence of therapeutic efficacy. It is necessary to say, that with the absence of the follow-up measures such as magnetic resonance imaging and neurophysiology evaluations it is impossible to state the potential efficacy of this approach. Treatment of rare metabolic diseases with stem cell transplantation requires a multidisciplinary approach for diagnosis, patient management, and outcome measures.

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