



New therapeutical approaches in stem cell transplantation

Új terápiás lehetőségek az őssejt-transzplantáció során

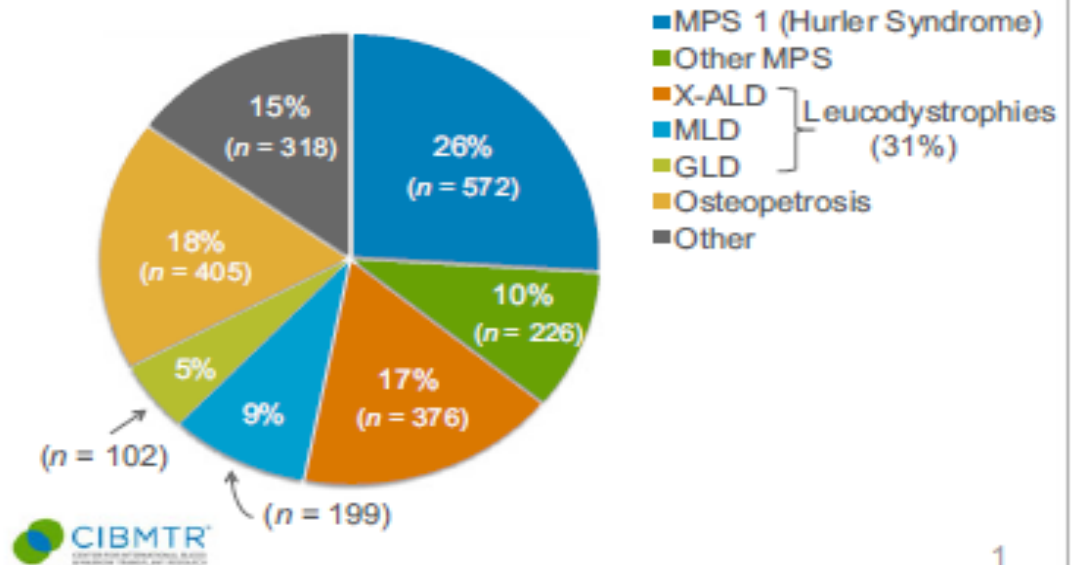
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Transplantation in inborn errors of metabolism

- Allogeneic hematopoietic stem cell transplantation (HSCT) was researched in numerous lysosomal diseases, including MPS I, MPS II, inherited lysosomal leukodystrophies and Krabbe disease.
- The first HSCT in Hurler-syndrome was performed in 1981.
- Since the 1980s, 2000 patients with an inborn error of metabolism have been transplanted.

Allogeneic Transplants for Inborn Errors of Metabolism Registered with CIBMTR, 1980-2013



Boelens et al. Transplantation in inborn errors of metabolism: current considerations and future perspectives. BJH 2014

How can stem cell transplantation in MPS help?

- Enzyme replacement therapy's efficacy has been demonstrated for visceral organ and soft tissue involvement, but poor or no efficacy was observed for brain involvement, because of poor penetration across the blood-brain barrier.
- HSCT allows donor-derived, enzyme-producing cells to migrate into the brain and other organs, providing a permanent form of enzyme replacement

Busulphan level monitoring

- Myeloablative conditioning therapy before stem cell transplantation
- Busulphan: high toxicity
- Test dose before the treatment



Our Department



Graft-versus-host disease

- Severe complication after stem cell transplantation
- Acute GVHD: skin, liver, gut involvement
- First line therapy: steroids (1-2 mg/kg MP)

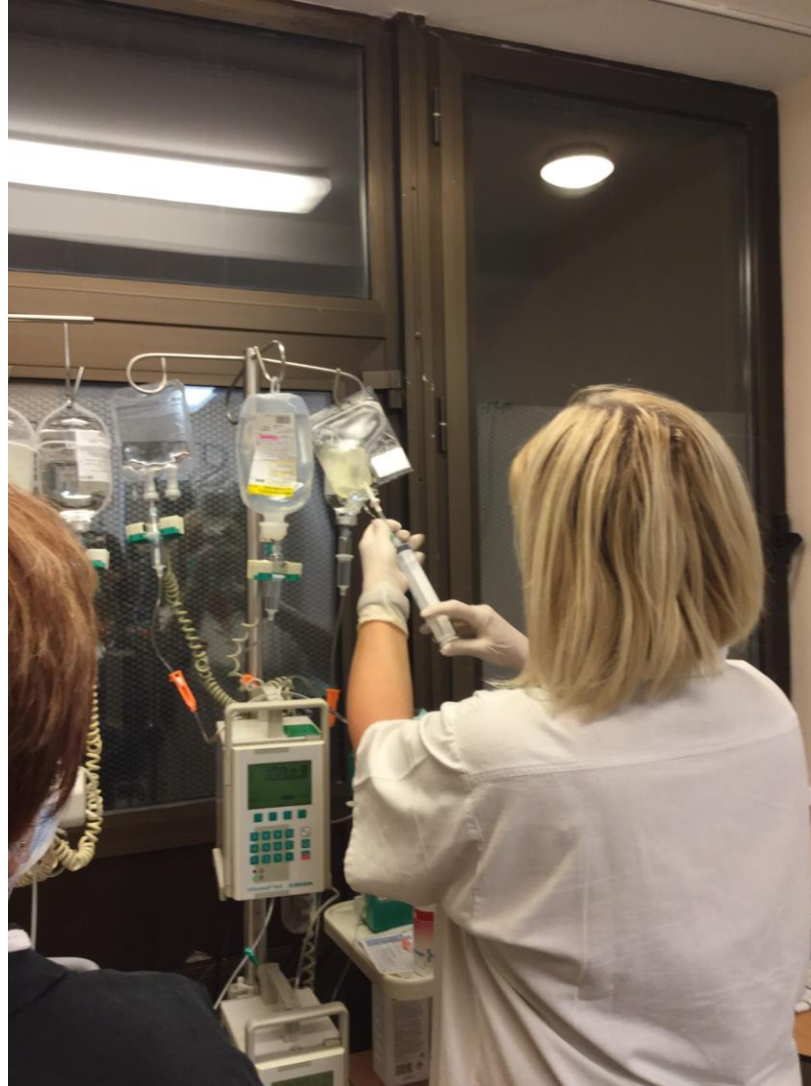


New treatment options in graft-versus-host disease

- Steroid resistant graft-versus-host disease
- **Ruxolitinib**: Janus kinase inhibitor, Tabl. Jakavi
- **Mesenchymal stem cell**: modulation of immune responses against allo- and autoantigens
- Lower MP doses
- Less toxicity, safe

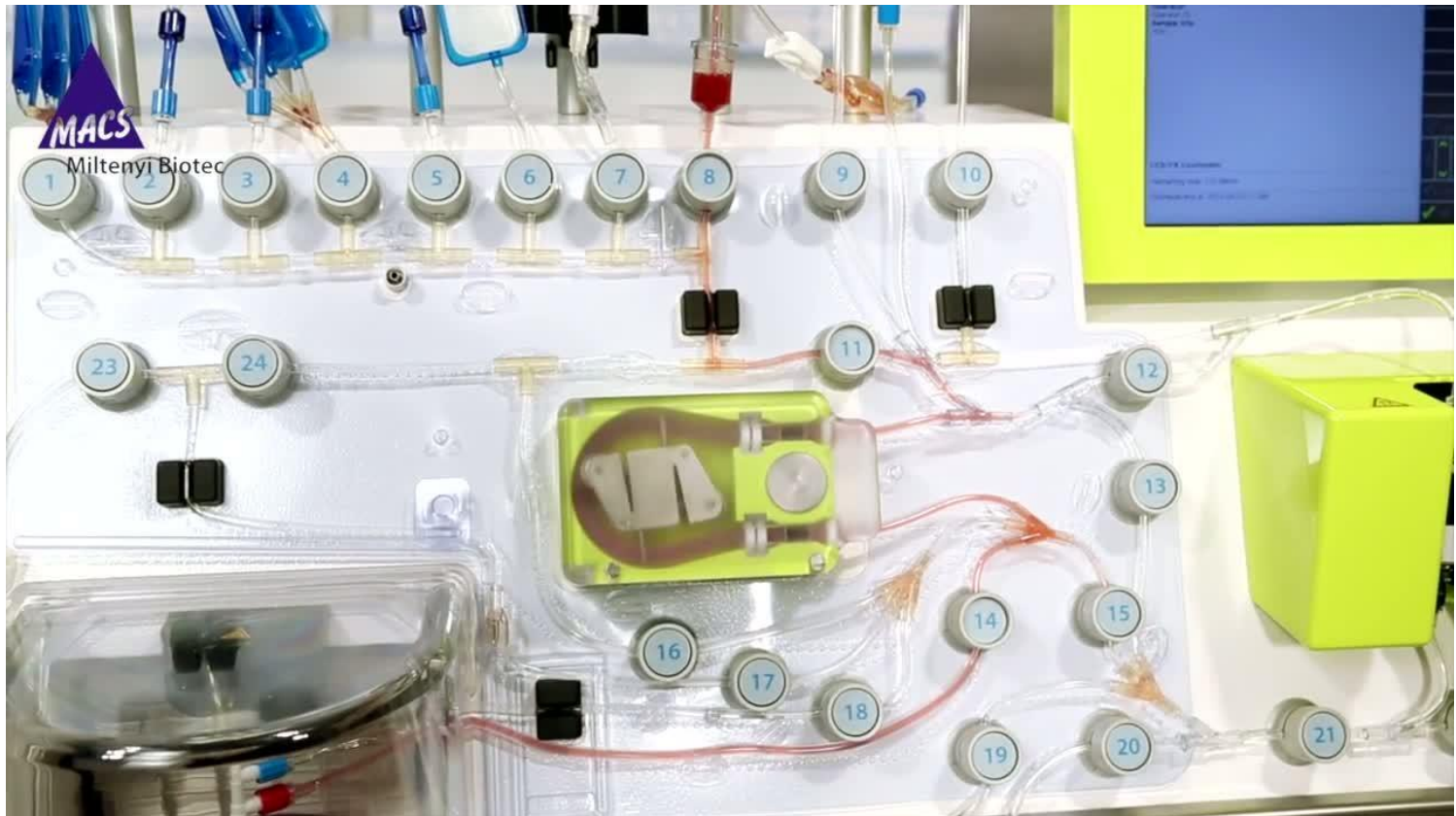


Mesenchymal stem cell transplantation



Antigen- specific T-cell therapy

Treatment option in severe EBV, CMV and adenovirus reactivation





Perspective

- Reduced intensity conditioning therapy in MPS
- Promising treatment options in complications
- More experiences in cell therapies
- In the future, >90% long-term overall survival in inborn error metabolism diseases

Thank you for your kind attention!

Köszönöm szépen megtisztelő figyelmüket!

