



EBMT Pediatric Diseases Working Party

Cellular Therapies Subcommittee

Goals and action plan 2018 - 2022

Goals

The mission of the Pediatric Diseases Working Party (PDWP) of the European Group for Blood and Marrow Transplantation Cellular Therapies Subcommittee (EBMT PDWP-CT) is to support the development of collaborative research and implementation of cell and gene engineering approaches to improve outcomes after stem cell transplantation in children. EBMT-PDWP-CT will:

1. Promote collaborative basic and translational research on improving immune reconstitution, biomarkers, GVHD and anti-leukaemic responses after paediatric SCT using cellular therapies.
2. Implement retrospective and prospective clinical trials on immune effector cell therapy in combination with SCT or as an alternative to SCT in children.
3. Update paediatric transplant physicians on novel developments in Cellular Therapies
4. In collaboration with the EBMT Cellular Therapy and Immunobiology Working Party, develop established standards for the safe and effective delivery of cellular therapies in paediatric patients.
5. Interact with other PDWP subcommittees and EBMT Working Parties as appropriate

Main Projects

1. Phase III trial of adoptive transfer multi-virus specific T cells after paediatric SCT
2. Randomised Phase III trial of CD19CART vs SCT in high risk relapsed paediatric ALL
3. Registry of “real world” outcomes for Tisagenlecleucel for paediatric ALL
4. Retrospective study on experience with 3rd party virus-specific T cells
5. Others as suggested by members

Action plan

1. Set up of Phase III study of virus-specific T cells for refractory viral infections post-SCT
2. Generate a study proposal for CART vs SCT and liaise with commercial partners
3. Engage with Novartis on registry of Tisagenlecleucel outcomes + set up working group to develop questionnaire
4. Collaborate with Cell Manipulation Working Party on paediatric aspects of EBMT “Management of Adults and Children undergoing autologous CAR T cell therapy: Recommendations of the European Society for Blood and Marrow Transplantation (EBMT)” guideline
5. Combined scientific/educational meeting with CTIWP in 2020.