

# BONE MARROW TRANSPLANT CENTER

## Clinical activities

Hematopoietic Stem Cell Transplantation (HSCT) represents the only chance of cure for a variety of pediatric diseases, including high-risk leukemias, lymphomas, selected solid tumors as well as for non-neoplastic diseases, either congenital and acquired, such as haemoglobinopathies, bone marrow failures, immunodeficiencies, inborn error of metabolism, etc.

HSCT is an extremely complicated and expensive procedure, requiring multidisciplinary specialist skills that are organized in a "Transplant Program", able to assist the patient throughout the whole treatment process, from donor search in the family context and in worldwide registries of voluntary donors/cord blood banks, to long-term follow-up.

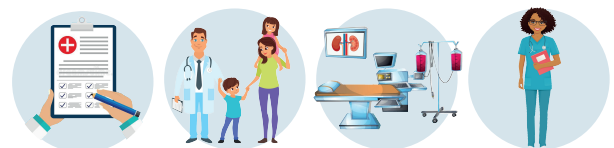
The transplant activity takes place in a dedicated inpatient ward with 6 single-bed HEPA filtered rooms, with 24h parent's assistance; all pediatric specialists and services are available on site. In the pre-post HSCT phase patients are nursed in the Day Hospital of the Dept. of Pediatric Hematology-oncology and, whenever possible, at home in collaboration with the Home Care Unit. The long-term follow-up is ensured in the framework of the "program for Recovered patients" of the Department of Pediatric Sciences and Hematology-oncology.

The Bone Marrow Transplantation Centre began its activity in 1984, and since then more than 1600 transplants have been performed, with an average of 45 procedures/year. In 80% of the cases, the patients come from outside the Ligurian Region and, in the last decade, 30% of them came from abroad Italy, in the context of institutional cooperative programs.

The Bone Marrow Transplantation Program is accredited by the national organisms GITMO/CNT to provide any type of transplantation procedure (autologous, allogeneic from HLA-identical or haploidentical related, allogeneic from unrelated adult volunteers or umbilical cord units).

## Areas of excellence/research

- ■ ■ Allogeneic transplantation from haploidentical related donors, with the option of 2 different platforms in patients suffering from neoplastic and non-neoplastic diseases (post-transplant cyclophosphamide, negative selection of TCR $\alpha\beta$ /CD19 positive cells from the apheretic product) in children lacking an HLA-identical relative or a 10/10 compatible unrelated donor.
- ■ ■ Use of repeated high-dose chemotherapy cycles followed by autologous HSCT in neoplasms of the central nervous system and other poor-prognosis solid tumors.
- ■ ■ Use of pathogen-specific CTLs (CMV- EBV- Adenovirus) in patients who developed viral diseases resistant or refractory to conventional therapeutic approaches
- ■ ■ Prospective study to evaluate the efficacy of haploidentical HSCT in patients with refractory or relapsed neuroblastoma



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- ■ ■ Study of fertility and reproductive function in pre- and post-pubic patients undergoing different transplant preparation regimes.
- ■ ■ Prospective phase I-II-III studies on the treatment of acute and Chronic graft-versus-host disease (GVHD) refractory to first-line treatment (monoclonal antibodies; extracorporeal lymphofotapheresis; TKI; JAK inhibitors)
- ■ ■ Prospective study on oral and fecal microbioma in HSCT recipients and its impact on acute and chronic complications

### Directors

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