



*EBMT Pediatric Diseases Working Party*  
**Hemoglobinopathies Subcommittee**  
Goals and action plan 2018 - 2022

### **Introduction**

Hemoglobinopathies is one of the most prevalent haematological disorders worldwide. Advances in the care of patients have led to a significant reduction of mortality in childhood, but cumulative end-organ damage with reduction in the overall quality of life and early adult mortality/morbidity remain a significant problem. In emerging economies, infant mortality and morbidity remains a significant problem. Blood and Marrow Transplantation is at present the only proven curative treatment modality but there are limitations due to toxicity and donor availability, which are only now being understood. The advent of gene therapy and disease modifying agents will lead to a more sophisticated and complex approach to the management of these patients in the future, requiring a better understanding of HSCT in addition to the history of the disease to enable the development of patient pathways.

### **Goals**

In keeping with the mission of the Pediatric Diseases Working Party (PDWP) of the European Group for Blood and Marrow Transplantation (EBMT), the activities of the Haemoglobinopathy Subcommittee (PDWP-H) aim to study and develop best practice in the field of haemoglobinopathies. PDWP-H will focus on producing evidence and openly sharing expertise with the primary goal of enabling safe transplants with optimal outcomes irrespective of donor for patients with haemoglobinopathies. PDWP-H will collaborate and share information with other subcommittees as appropriate to enable this, for example working with the Outreach subcommittee to reach the largest number of patients and the Late Effects subcommittee to understand the long-term disease modifying effect.

### **Main Projects**

1. Impact of different regimens on the main complications of transplantation.
2. Impact of disease status to identify appropriate patient selection and preparation of patients pre-transplantation.
3. Study the factors leading to endothelial disease and best approach to management.
4. Study the best alternative donor approach for haemoglobinopathies.
5. Study the long-term outcomes and late effects in both sickle cell disease and thalassaemia.
6. Create the platform to enable the study of gene therapy outcomes and support its development.
7. Others as proposed by other members and subcommittees.

### **Action plan**

1. Analyze impact of conditioning regimens on the different outcomes and complications.
2. Analyze the impact of disease status at transplantation on transplant complications and disease outcomes.
3. Analyze the factors leading to endothelial disease and develop a study to modify these.
4. Develop a prospective EBMT study comparing and studying variables of haploidentical transplantation and sibling transplantation in sickle cell disease.
5. Analyze the long-term end-organ damage modifying effect of transplantation in haemoglobinopathies and the late-effects.
6. Develop a strategy to ensure the different variables of gene therapy approaches are collected.