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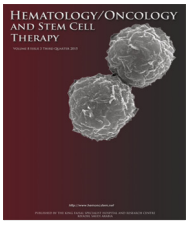

Thalassemia major: Transfusion and chelation or transplantation

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Abstract

Thalassemia is the most common monogenic hematologic disease that affects millions in the world and kills thousands of patients every year.

Without transfusion or transplantation, patients with thalassemia major are expected to die within months of diagnosis. However, long-term transfusion and chelation therapy is highly challenging for many developing countries where the disease is prevalent, representing a major and unsustainable health burden.

Stem cell transplantation is the only cure for thalassemia. It has witnessed major developments that have made it less toxic, more successful, and feasible for a larger number of patients with diverse comorbidities and from a wider range of donors. Advances in human leukocyte antigen typing have greatly refined alternate donor selection with results of matched unrelated donors similar to matched sibling donors.

Novel strategies such as haploidentical and cord blood transplantation have increased the possibility of patients with no healthy donor to get a better opportunity to survive and avoid chronic transfusion complications.

Cost-effectively, transplantation should be considered the primary treatment of choice in the presence of a suitable related or unrelated donor and at centers with a satisfactory experience in the field of transplantation and particularly, in managing those with thalassemia. Despite some complications such as graft-versus-host disease and late conditioning effects, the overall improvement in the quality of life of thalassemia is difficult to deny.

Unfortunately, the number of transplants for thalassemia represents only a minority of all transplants conducted globally. Moreover, the essential requirement for transplants for thalassemia in limited-resources countries should mandate the transplant societies, including

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Worldwide Network for Blood and Marrow Transplantation, to collaborate to help initiate and support specialized transfusion and transplant programs for managing thalassemia.

Introduction

Thalassemias are the most common monogenic hematologic disorders in the world. It is estimated that more than 60,000 babies are born annually with thalassemia major and more than 80 million peoples are carriers of B-thalassemia [1]. In 1920s, all thalassemia major patients used to die within 6 months of diagnosis [2].

Between 1949 and 1957, in Ferrara, only 9% of the patients reached the age of 6 years, and at the end of the 1970s, half of Italian thalassemic patients had died before reaching 12 years of age. In the 1980s onwards, with combined safe transfusions and regular chelation and/or transplantation, survival improved significantly but remained suboptimal and non-satisfactory at national levels (Fig. 1) [3].

In developing and limited-resources countries, survival rate for thalassemia major is expected to remain at the same low rate and may even have a detrimental impact on the survival of afflicted, but insufficiently-treated patients, similar to acute and chronic leukemias.

In 2009, in Italy, more than 60% of patients lived for more than 30 years. The majority of them were in good health, could have a family, and worked full time due to major advances in transfusion, chelation, and transplantation. Despite such advances and improvements, compliance and availability remain pivotal factors in determining the survival and quality of life (QOL). Relatively recent Thalassemia registry data from England showed a continuous decline in survival starting from adolescence with fewer than 50% of thalassemics surviving beyond 35 years due to poor compliance with comprehensive optimal chelation therapy [4]. There is a marked variation in the clinical practice of transfusions and iron chelation worldwide, from nothing to optimal, that is not only limited to thalassemia major but also affects all other transfusion-dependent anemias [5]. Unfortunately, with few exceptions, the distribution of thalassemia across the globe [6]

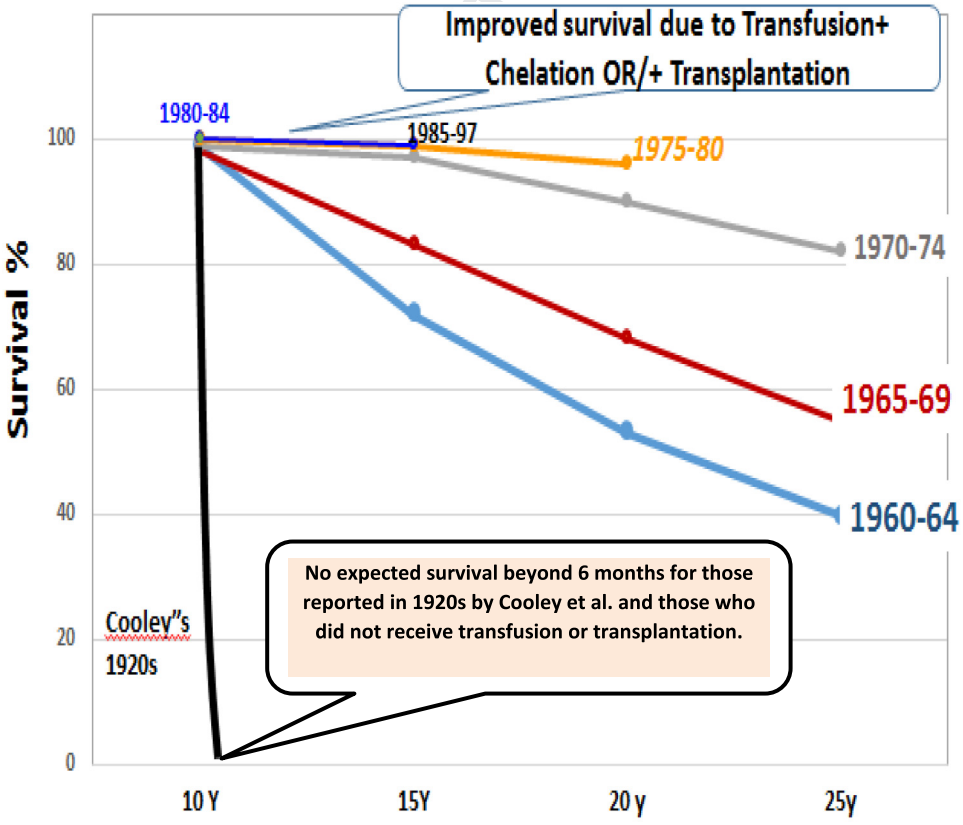

Fig. 1 Survival of thalassemia major by the date of birth. Modified from Borgna-Pignatti C et al., Ann. NY Acad Sci 2005.

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