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HORIZON SCANNING OF LENTIGLOBINTM IN TRANSFUSION-DEPENDENT B-THALASSEMIA.

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OBJECTIVE: Beta thalassemia is a group of hereditary blood diseases. It is caused by a reduced or absent synthesis of the beta chains of haemoglobin, which may result in variable outcomes, ranging from severe anaemia to a clinically asymptomatic condition. It is caused by mutations in the HBB gene on chromosome 11, inherited in an autosomal recessive manner. Globally, 80-90 million people (1.5% of the population) are carriers of β-thalassemia. More than 40,000 babies with β-thalassemia are born each year. In Italy, more than 7,000 patients with β-thalassemia require transfusions. The transfusion-dependent β-thalassemia (TDT) is highly prevalent in Sicily, Sardinia, and Puglia; but also in other 10 Italian Regions. The aim of the study is to provide a professional support to build effective evidences according to the methodological guidelines shared within EuroScan. METHODS: Horizon scanning is a method for detecting early signs of potentially important developments through a systematic examination of potential threats and opportunities, with emphasis on new technology and its effects on the issue at hand. A systematic literature review was performed by querying main search engines. In order to get a deeper understanding of the use of this technology in Italy and its impact on clinical and economic outcomes a multidisciplinary advisory board with experts was established. RESULTS: Gene therapy for thalassemia is currently based on transplantation of autologous hematopoietic stem cells (HSC) genetically modified with a lentiviral vector (LV) expressing a globin gene under the control of globin transcriptional regulatory elements. Gene therapy using autologous, genetically modified HSCs is an alternative to allogenic HSC transplantation (HSCT) for treating β-thalassemia. It circumvents the need for a matched donor and thus avoids the risk of graft versus host disease and graft rejection after HSCT. Furthermore, no serious adverse effects have been attributed to ex vivo LV-based HSCT gene therapy for TDT. CONCLUSION: The infusion of the genetically modified cells and patient follow-up can potentially be performed in many pediatric and adult hematopoietic cell transplantation units, even those with limited expertise in allogenic HSCT. Thus, the gene therapy could represent a valid alternative for the treatment of these patients.