Opinion

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# Juggling Between the Cost and Value of New Therapies: Does Science Still Serve Patient Needs

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**ABSTRACT**

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## Opinion

Decades of hope for a cure vanished into thin air when cost outweighed the value of the first gene therapy for thalassaemia, obliging the manufacturing company to withdraw it from Europe. This may create a precedent for other curative therapies that are currently in the pipeline after many years of research, raising questions over their future acceptance by payers and the fulfilment of their purpose: to cure as many patients as possible.

Frequent transfusions, chronic pain, absence from school and work, discrimination, mental health issues are just a few of the daily challenges that patients with thalassaemia face. Standard care with lifelong regular blood transfusions, iron chelation therapy and multidisciplinary care has achieved an increase in life expectancy [1,2]. However, a curative therapy would further allow patients to lead a new life with equal opportunities and challenges, as every other person not suffering from a severe chronic disease. The Thalassaemia International Federation (TIF), representing the united voice of people with thalassaemia and their families globally, has been striving for more than three decades to empower research on a curative approach for thalassaemia.

Haematopoietic stem cell transplantation (HSCT) offers the possibility of cure, but bears specific limitations, i.e. HLA-identical sibling matching and young age [3]. Gene therapy may overpass these challenges, covering more patients and a larger age span. Research on genome-based therapies persisted for decades and this journey has been difficult and immensely challenging until a few years ago [4,5]. The small US-based biotech company bluebird bio that undertook the improvement of a vector produced by Leboulch in 1994, finally succeeded in what predecessors failed [6], partly because it paid attention to the patients' perspective and their everyday journey with this debilitating disease. Stakeholders always knew that an innovative and complex therapy for thalassaemia would be expensive but always supported its development. Governments and academia provided grants, the industry invested in product's improvement, healthcare professionals and patients monitored the pipeline and hoped for access. But when the European Medicines Agency granted the gene therapy product of bluebird bio, called Zynteglo™, a conditional market authorisation in May 2019, everyone focused on numbers and cost-effectiveness studies [7] However, no health economist

would ever capture accurately the real cost of thalassaemia in terms of pain, uncertainty and fear.

The withdrawal of an authorised gene therapy from Europe will most probably slow down or even halt the access of people with thalassaemia to curative approaches, rendering the future of thalassaemia treatment gloomy at the very least. And if the developed countries of Europe cannot afford an innovative therapy, what will happen to low- and middle-income countries, where the 80% of the global thalassaemia population lives? Depriving patients from a chance to be cured is at the minimum unethical and constitutes a violation of human and patients' rights endorsed for decades now by relevant European Union and international bodies. It is also a discriminatory behaviour against people with thalassaemia, given that patients in other disease areas do receive innovative therapies bearing a hefty price tag. It is of utmost important for all stakeholders engaged in the development of medicines, and especially the industry and academia, to seek early and transparent dialogue during the long course of drug development to identify timely safety and cost hurdles.

The developers of medicines, after having invested considerable funds in developing and commercialising a product, should not be left exposed to failure but be given the necessary space, time and motivation to mitigate problems in market access. Additionally, a central, special fund on innovative therapies should be created and managed by the European Commission to compliment national funds of Member States for novel therapies. Governments need to develop synergies and discuss pricing early on, considering the lessons of the past and the challenges to come. As countries do not have unlimited financial resources, governments may opt for joint procurements in the context of regional alliances, such as the Valletta Declaration or the BeneluxA initiative, that increase their negotiating power for the purchase of expensive therapies. Finally, treating physicians and patients should become actively and meaningfully involved in the development process from the very early stages to provide concrete information on medical and other priorities, the potential number of patients that could benefit from innovative therapies, and short and long-term plans for the access of eligible patients to such therapies. Everyone has a role to play and we all share the same responsibility for the sad decision of bluebird bio to withdraw its services (even temporarily) from Europe. Life cannot be measured using mathematical models. Science needs to be available, accessible and most importantly, at the service of patient needs.

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