THALASSAEMIA INTERNATIONAL FEDERATION
In official relations with the World Health Organization

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TIF's Statement on Accessibility of Gene Therapy for Thalassaemia

'a miracle we have been waiting for many many years, but it must not become a privilege for some instead of a right for all.' Patient, United Kingdom

The Thalassaemia International Federation (TIF)[1] expresses its disappointment about the outcome of the reimbursement negotiation process concerning gene therapy (Zynteglo™) for transfusion-dependent thalassaemia patients, announced last week.

The Federations' distress is further heighted not only by the potential of a national healthcare system like that of Germany – universally acknowledged as one of the most advanced and robust in the world – but also by the substantial involvement of the German medical community in the long-lasting clinical trials that resulted in the conditional marketing authorisation of this gene therapy product by the European Medicines Agency (EMA). Indeed, the commitment of the medical community in the country has been exemplary towards the needs of patients for a final and holistic cure of this chronic and rare disorder.

A similar anguish has been voiced by the Federation as a result of the appraisal committee decision of NICE in the United Kingdom[2] seeking that gene therapy is available as an option to patients.

Nonetheless, the lifelong burden of disease that affects every aspect of the patients' life has not been sufficiently recognized, nor has the potential lifelong benefit that gene therapy will offer – first and above all reducing the need for blood transfusion, currently needed every 2 – 3 weeks – and all related consequences and associated co-morbidities. This is an element that is well reflected in the age distribution of patients even in countries with strong public health infrastructure and economies.

'We do not live to be treated. We are treated to live. So that we can fulfil our goals in life, integrate into society as individuals and professionals, and enjoy the simple, everyday gifts of life. A once-off therapy can hopefully help us achieve these' Patient, United Kingdom

Gene therapy has been a long-awaited scientific development for thalassaemia patients and has given hope, optimism and opened new horizons; a study of the patients' perspective on gene therapy spearheaded by TIF in 2020 has shown that more than 80% of respondents (patients and parents) would undergo gene therapy should they (or their children) meet the eligibility criteria[3] and given the choice and opportunity.

As the voice of thalassaemia patients and their families in 62 countries across the world, TIF will continue to strive, with the same commitment, passion and sensitivity and to collaborate closely and productively with decision-makers, the industry, academia, healthcare professionals, patients/parents and every single

[1] The Thalassaemia International Federation is patient-oriented, non-profit, non-governmental umbrella federation, established in 1986 with Headquarters in Nicosia, Cyprus.Our mission is to promote access to optimal quality care for all patients with thalassaemia worldwide. To-date membership boasts 232 members from 60 countries across the globe. TIF works in official relations with the World Health Organization (WHO) since 1996 and enjoys active consultative status with the United Nations Economic and Social Council (ECOSOC) since 2017. Most remarkably, TIF has been awarded, in the context of the 68th World Health Assembly in May 2015, the 'Dr Lee Jong-wook Memorial Prize' for the Federation's outstanding contribution to public health. More information about the Federation is available at www.thalassaemia.org.cy.
[2] Read TIF's Statement at https://thalassaemia.org.cy/wp-content/uploads/2021/03/TIFs-submission-on-the-UK-NICEs-consultation-document-on-Zynteglo.pdf
[3] Read the Report at https://thalassaemia.org.cy/news/now-published-the-tif-gene-therapy-thalassaemia-survey-report/

involved stakeholder to ensure that such innovative therapies are not only made **available** but also **accessible** to patients in need, and those who wish to go through it, across the world.

'The promise of gene therapy as a cure for thalassaemia has been a hope for generations of patients but also of parents like myself. Unfortunately, I lost my son before this dream could be realized – but now it is here and within our reach and I truly hope with all my heart that no one will ever again feel the pain of losing a child to this serious disease.' Parent, Cyprus

It is imperative for ground-breaking scientific advances and innovative therapies that will ultimately improve the quality of life of patients with severe genetic diseases, such as thalassaemia, be sufficiently recognized by national health authorities in conjunction with not only an understanding of the impact of the disease on the daily lives of patients (and to a great extent their families) but also in regards to the actual cost-of-illness for healthcare providers. TIF in its efforts to fulfil its mission – the implementation of national disease-specific control programmes (prevention and clinical management) in every *affected* country in the context of healthcare systems based on universal coverage – has developed a thalassaemia-specific model to be used by national health authorities to assess the disease-related resources required in their settings, and to acknowledge the added-value of implementing polices and promoting the availability of innovative therapies, such as gene therapy. The model, described within the Global Thalassaemia Review 2021, will be officially announced during a dedicated event held on 8th May 2021 – International Thalassaemia Day[4].

[4] More information on International Thalassaemia Day 2021 at https://thalassaemia.org.cy/itd2021/