

T I F

# NEWSLETTER

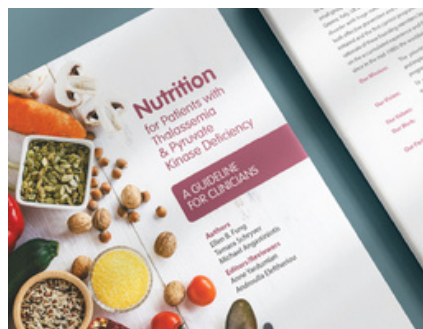
AUGUST 2023 / Vol.18



## IN - FOCUS

The Thalassaemia International Federation (TIF), in collaboration with the Open University of Cyprus, recently initiated a survey project, entitled '*Thalassaemia Care Delivery from a Patient Perspective*' to gain a deeper understanding of health and social care services for chronic patients, specifically those affected by thalassaemia.

More inside



### NUTRITION GUIDE FOR THALASSAEMIA & PKD

TIF proudly presents its most recent publication, the 'Nutrition Guide for Patients with Thalassaemia and PK Deficiency,' which has been prepared expressly to meet the dietary needs of people living with these conditions.



### TIF INTERNATIONAL CONFERENCE 2023

One of the most anticipated annual events in Thalassaemia and Haemoglobinopathies is fast approaching! Mark your calendars for November 3-5, 2023, and join us at the 16th TIF International Conference.



# COUNTRIES CORNER

Providing expertise and support to patient associations all over the world.

## AMERICA

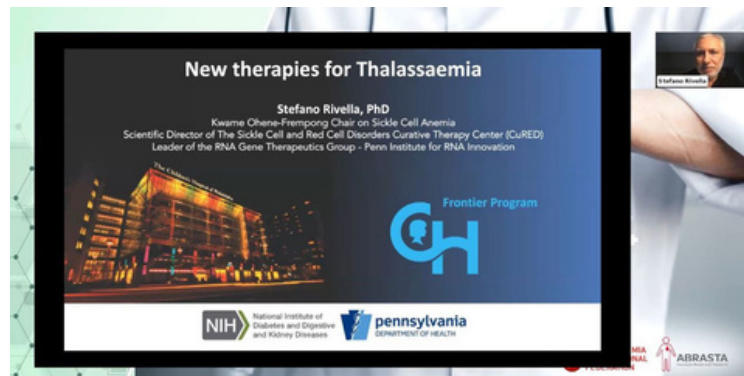


***On July 28, 2023, the Thalassaemia International Federation (TIF) joined forces with the Brazilian Thalassaemia Society to host a highly informative webinar, entitled 'Management and Control of Thalassaemia'.***

This webinar was specifically tailored towards healthcare professionals based in the Americas, aiming to enhance their knowledge and understanding of this genetic blood disorder.

Professors Maria D. Cappellini, John Porter, and Stefano Rivella, alongside Drs Alessia Pepe and Michael Angastiniotis, presented a comprehensive overview of various topics related to thalassaemia.

Their discussions encompassed new treatments and advances in managing thalassaemia, as well as the importance of multidisciplinary care and evolving epidemiological trends of the disorder.



## EGYPT

***TIF has established a significant collaboration with the Egyptian Ministry of Health to enhance the standard of care, address current challenges, and meet the pressing health and societal needs of thalassaemia patients in Egypt.***

This milestone, marked by the initiation of formal bilateral relations between the two organizations, was celebrated in an online meeting on July 17, 2023.

Representatives from TIF and the Egyptian Ministry of Health convened to discuss a range of topics, including the recently launched Presidential initiative for premarital screening in Egypt, recommendations for improving and expanding access to thalassaemia services, and public-private collaborations aimed at medical capacity building and education.





# INTERNATIONAL RELATIONS

*Upholding the thalassaemia patient community's rights and facilitating cross-border sharing of expertise.*

## TIF'S MEETINGS WITH MEDICAL PROFESSIONAL ORGANIZATIONS

TIF is currently holding meetings with medical professional organizations and societies to enhance patient care within its community. These discussions aim to emphasize the critical role of medical specialists from various fields in reducing morbidity and premature death rates among patients with thalassaemia, SCD, and other anaemias.

### **International Society of Radiology (ISR)**

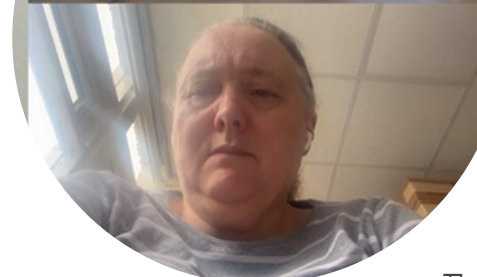
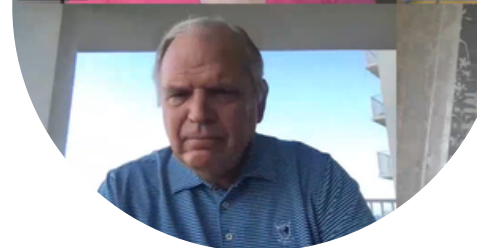
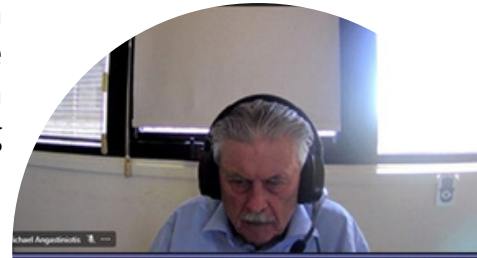
On August 7, 2023, a meeting took place between TIF and Dr. Bibb Allen, President of the International Society of Radiology (ISR), and a delegate from the European Society of Radiology (ESR). The central topic of discussion revolved around the imperative integration of precise quantitative imaging in point-of-care MRI devices, particularly within low- and middle-income nations, a technology that empowers medical professionals to effectively circumvent iron overload and its associated complications.

### **European Society for Gene and Cell Therapy (ESGCT)**

On July 19, 2023, TIF representatives convened with Professor Juan Bueren, President of the European Society for Gene and Cell Therapy (ESGCT). United by a shared enthusiasm, both entities discussed the possibility of forging a meaningful partnership to generate sustainable solutions to enable access to gene therapy for those afflicted with rare diseases. TIF's unwavering commitment lies in championing the rights of all patients suffering from thalassaemia, sickle cell disease, and other rare anamias, who necessitate - or opt for - cell and gene therapies in their pursuit of a cure.

### **Haematology Nurses and Healthcare Professionals Group (HNNHCP)**

On August 9, 2023, an introductory meeting took place between TIF and the Vice-President, Ms. Mairéad Ní Chonghaile, and Secretary, Ms. Sarah Liptrott, of the Haematology Nurses and Healthcare Professionals Group (HNNHCP) Board. The purpose of this meeting was to foster a closer collaboration with HNNHCP and discuss potential joint educational initiatives. Additionally, the partnership aims to update TIF's publication, 'A Guide for the Haemoglobinopathy Nurse,' which was initially released in 2013.





# INTERNATIONAL RELATIONS


## THE EU AS A HOTSPOT FOR R&D IN CELL & GENE THERAPIES: INSIGHTS FROM THE TRANSFORM ALLIANCE EVENT


On July 6, 2023, the **TRANSFORM Alliance** hosted a **national roundtable** in Paris, to discuss the ATMP landscape in France and share TRANSFORM's positions at the EU level **on ensuring safe and timely access to advanced therapies for all EU patients**. TIF, a member of the Alliance, joined the discussion virtually with Mrs Eleni Antoniou, Senior Policy Officer, to highlight the added value of patient participation throughout the value chain.


Panellists agreed that the EU has a window of opportunity to act and deliver safe and timely access to advanced therapies. Policymakers should strive to achieve a high degree of harmonisation across the Union and engage with all stakeholders involved, leaving no one behind.



Key points and recommendations raised during the roundtable included:

 **Access to Advanced Therapies:** There was consensus among panellists that the EU should assume leadership on medicines evaluation, due to severe delays for patient access to advanced therapies. The 2021 EU Health Technology Assessment Regulation is paving the way for better harmonisation.

 The EU is lagging behind the US and China on R&D, therapeutic innovation, and clinical trials. To compensate, public and private actors must collaborate and invest not only in infrastructure, but also in dialogue with patients.

 The EU should have a clear added value once it manages to collect patient data in a harmonised manner. Such data must be further taken into consideration in scientific evaluations, to understand the effects of innovative therapies on the daily lives of patients, as well as to reflect on how future treatments could better accommodate their needs.



# TOPICS-IN-FOCUS

*Targeted information on TIF's key activities and projects that stand out.*

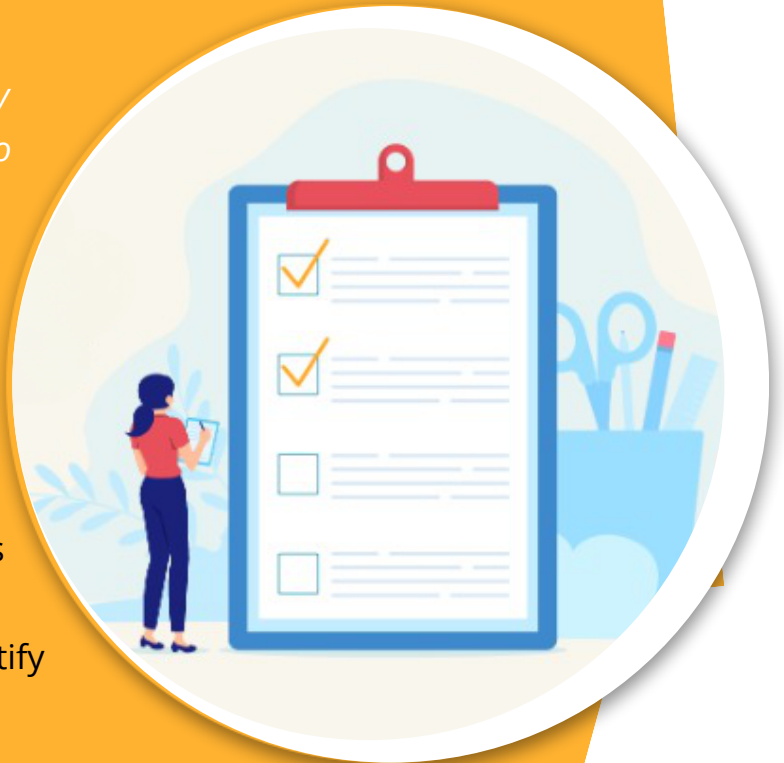
## TIF SURVEY: THALASSAEMIA CARE DELIVERY FROM A PATIENT PERSPECTIVE

*The Thalassaemia International Federation (TIF), in collaboration with the Open University of Cyprus, recently initiated a survey project to gain a deeper understanding of **health and social care services for chronic patients**, specifically those affected by thalassaemia.*

This initiative seeks to collect **valuable insights into the experiences and challenges faced by individuals living with thalassaemia** in various countries, as well as their access to healthcare services. Through this survey, TIF also hopes to identify care gaps, showcase best practices, and advocate for enhanced support for thalassaemia patients.

The survey encompasses numerous aspects of thalassaemia care delivery, such as **medical treatment options, healthcare service accessibility, the availability of specialized professionals, treatment affordability, and patients' overall quality of life.** The data gathered will contribute to formulating evidence-based recommendations for policymakers and healthcare providers.

By directly involving patients in this project, TIF strives to ensure that **their voices are heard and their perspectives drive future improvements in thalassaemia care.**



**Take the Survey** >





# TOPICS-IN-FOCUS

## 16TH TIF INTERNATIONAL CONFERENCE: Advancing Knowledge in Thalassaemia and Haemoglobinopathies

03

04

05

November  
2023

TIF International Conference on

**Thalassaemia and Other  
Haemoglobinopathies for  
Healthcare Professionals  
and Patients/Parents**

Grand Hyatt Hotel, Kuala Lumpur, Malaysia

*Mark your calendars for November 3-5, 2023, as Kuala Lumpur transforms into a hub for discussions on thalassaemia and haemoglobinopathies at the 16th TIF International Conference.*

The **16th TIF International Conference (TIF2023)**, a highly anticipated event in thalassaemia and haemoglobinopathies, offers a forum for patients, parents, researchers, experts, healthcare professionals, and industry representatives to enhance global understanding of these conditions. By **fostering collaboration and idea exchange, as well as presenting cutting-edge research**, the conference advances knowledge in this critical field.

Attendees will have the opportunity to participate in informative sessions, workshops, and presentations covering various aspects of thalassaemia, sickle cell disease, and rare anemias. **Key topics** will encompass advancements in treatment options, health services for hemoglobin disorders, screening programs, patient advocacy efforts, innovative research findings, and much more.

By uniting experts from around the world, the TIF International Conference plays a pivotal role in enhancing care standards while fostering a collaborative environment that can lead to breakthroughs in research and improvements in patient care.

Key attending officials include **Dr Tedros Adhanom Ghebreyesus**, WHO Director General, **Mrs Stella Kyriakides**, EU Commissioner for Health and Food Safety, and **Prof. Zilfalil bin Alwi**, UNESCO Chair in Human Genetics on Thalassaemia, Universiti Sains Malaysia, as the Conference Keynote Speaker.



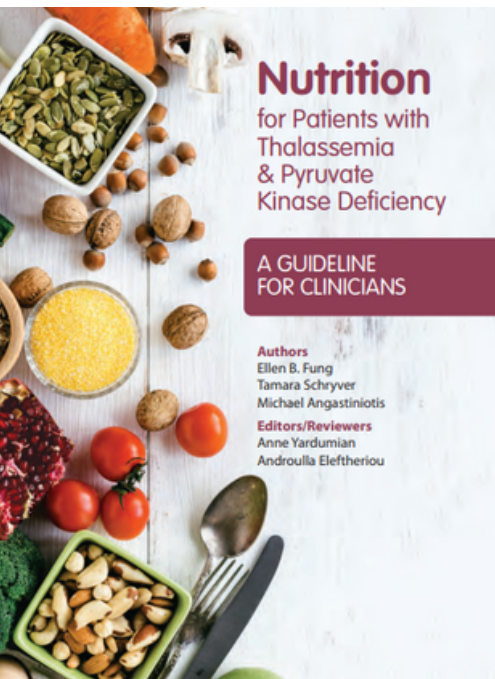
**REGISTER NOW**



# TIF PUBLICATIONS

*A wealth of useful TIF-generated publications and resources for patients and healthcare professionals.*

## NUTRITION GUIDE FOR PATIENTS WITH THALASSAEMIA & PK DEFICIENCY



Nutrition is critical in the lives of patients affected by thalassaemia and other hereditary anaemias.

TIF is proud to present its most recent publication, the '**Nutrition Guide for Patients with Thalassaemia and PK Deficiency**,' which has been prepared expressly to meet the dietary needs of people living with these conditions.

This amazing 184-page book brings together the experience of prominent global specialists in the industry. Everyone may benefit from the critical examination of current information available within these pages, from haematologists to dieticians, in helping patients create better eating habits and achieve a healthier lifestyle.

[Read More >](#)

## TRAVEL GUIDANCE FOR PATIENTS WITH THALASSAEMIA, SCD & RARE ANAEMIAS

Are you enthusiastic about discovering new destinations, yet apprehensive that your medical condition may hinder your aspirations?

TIF's '**Travel Guidance for Patients and Families**', designed for individuals dealing with thalassaemia, SCD, and rare anaemias, provides invaluable advice for those intending to embark on educational excursions, relocation endeavors, or leisurely vacations in foreign lands.

This exceptional manual prioritizes your well-being throughout your travels by offering astute recommendations on medication management and insightful information on accessing medical assistance in international locations.

[Read More >](#)





# TOP STORIES

*Drugs, therapies and other haemoglobinopathies-related clinical and policy news that made the headlines.*

## WHO UPDATES ESSENTIAL MEDICINES LIST, ADDS NEW MEDICINES FOR THALASSAEMIA



The **World Health Organisation (WHO)** published the **updated Model Lists of Essential Medicines (EML)** and Essential Medicines for Children (EMLc) on July 26, introducing all three iron chelating drugs for thalassaemia treatment; deferoxamine, deferiprone, and deferasirox.

Previously, only deferoxamine was recommended for iron chelation. TIF has been lobbying for inclusion of deferiprone and deferasirox in the EML since 2019, striving to ensure global access to all viable options. The Federation is grateful for the decision and believes it will positively impact patient care.

For over 40 years, countries all over the world have relied on the WHO EML and EMLc for the development and updating of national essential medicines lists.

[Read More >](#)

## EU REVOKES APPROVAL OF SICKLE CELL DISEASE DRUG ADAKVEO

**Novartis announced the withdrawal of Adakveo (crizanlizumab), a SCD medication, from the European market** following the formal revocation of its authorization by EU officials on August 4.

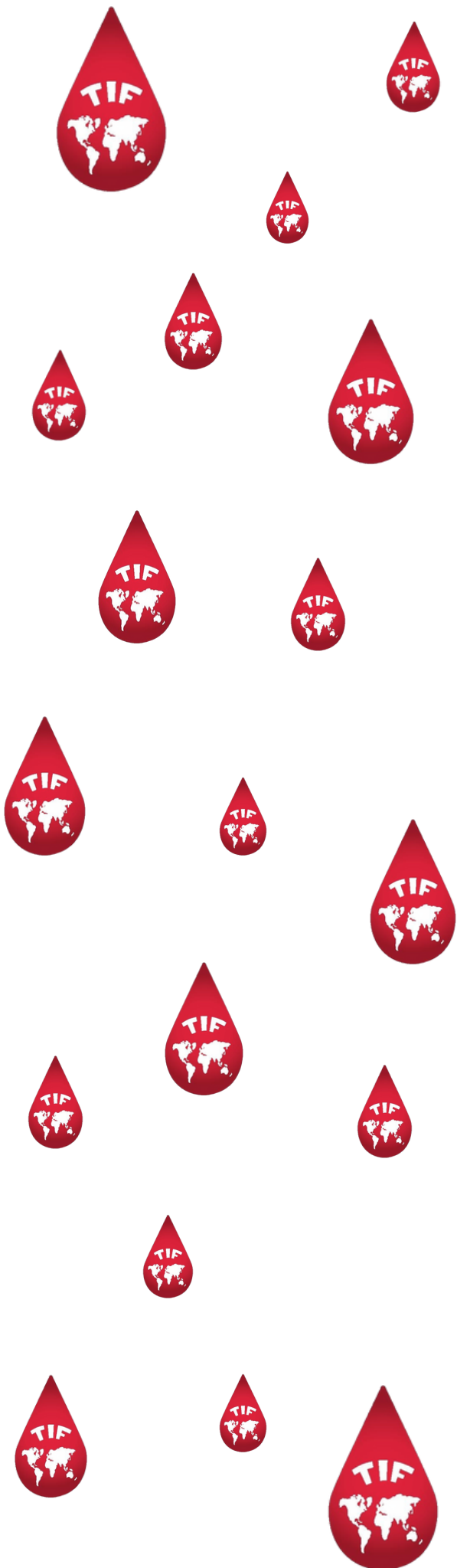
The European Commission made this decision after the Committee for Medicinal Products for Human Use (CHMP) recommended in May that the European Medicines Agency (EMA) revoke Adakveo's conditional clearance.

This regulatory change resulted from a late-stage clinical trial that compared the medication to a placebo and found no significant difference in reducing rates of pain crises (VOCs) experienced by many SCD patients. However, the treatment remains available for SCD patients in the US.



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