

# TIF NEWSLETTER



↓ In this newsletter you will find:

TIF's latest news and activities globally, publications, projects and more!



# COUNTRIES CORNER

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

## INDIA



On December 1-2, 2023, Prof. Dimitrios Farmakis, Consultant Cardiologist at the National and Kapodistrian University of Athens Medical School and Medical Advisor for TIF, visited the Thalassaemia and Sickle Cell Society (TSCS) in Hyderabad, India. TSCS is a newly registered non-governmental organization affiliated with TIF, providing care to over 3,500 patients suffering from thalassaemia and sickle cell disease in the region.

During his visit as a TIF delegate, Prof. Farmakis had the opportunity to meet Dr. Chandrakant Agarwal, President of TSCS, along with other board members. He also toured the society's facilities in Hyderabad and engaged in discussions with healthcare professionals, patients, and their families.

Dr. Agarwal and the TSCS board members expressed a strong interest in collaborating with TIF across various domains and explored numerous potential activities to kickstart this partnership.



The Larissa General Hospital's Radiology Department in Greece recently hosted the 2nd Annual Scientific Meeting on "Imaging Assessment and Management of Haemoglobinopathies". Taking place from November 25-26, 2023, the event was organized under the auspices of the Thalassaemia International Federation (TIF).

A diverse array of professionals attended the meeting, including specialized radiologists, haematologists, hepatologists, cardiologists, and surgeons from all over Greece.

Representatives from major patient advocacy groups in the country were also present for this significant gathering. TIF's Board Member and President of the Greek Thalassaemia Association (ESTHA), Mr. Vasileios Dimos, represented the Federation at the event.

During the meeting, attendees discussed various advancements in haemoglobinopathies imaging and explored a wide spectrum of modern radiology applications in this essential medical field.

## GREECE





# TOPICS-IN-FOCUS

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

## TIF International Conference 2023

### A GLOBAL CATALYST FOR THE HAEMOGLOBINOPATHY COMMUNITY

The 16th International Conference on Thalassemia and Other Haemoglobinopathies, along with the 18th TIF Conference for Patients and Parents, took place from November 3-5, 2023, at the Grand Hyatt Hotel. This milestone event marked the **first in-person TIF international conference in six years**, drawing over **1,500 attendees from 62 countries**.

The conference was organized by TIF in partnership with the Ministry of Health of Malaysia, the Federation of Malaysian Thalassemia Societies, the Malaysian Society of Haematology and the Malaysian Society of Paediatric Haematology & Oncology.

Notable participants included Dr. Tedros Adhanom Ghebreyesus, WHO Director-General (virtually); Dr. Stella Kyriakides, EU Commissioner for Health and Food Safety (virtually); Dr. Rabindra Abeyasinghe, WHO Representative to Malaysia, Brunei Darussalam and Singapore; H.E. Dr. Zaliha binti Mustafa, Minister of Health, Malaysia; Prof. Vichai Tienthaworn, President of Praboromaarajchanok Institute (PBRI), Ministry of Public Health, Thailand; Prof. Zilfalil bin Alwi, UNESCO Chair in Human Genetics on Thalassemia; and Prof. Carmencita Padilla, Chancellor of the University of the Philippines.





# TOPICS-IN-FOCUS

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

## A GLOBAL CATALYST FOR THE HAEMOGLOBINOPATHY COMMUNITY



Continuing its legacy as a leader in the global thalassaemia community, the TIF International Conference 2023 sparked new conversations and provided a stage for groundbreaking scientific revelations. Three publications were unveiled at the event, each signifying a novel area in understanding and treating thalassaemia.

Moreover, TIF's Regional Networks were formally introduced to cultivate multi-stakeholder collaborations, share best practices among healthcare professionals and Reference Centres, and encourage patient education and empowerment for effective advocacy and policy development to enhance patient outcomes.

A policy session convened decision-makers, patients, and healthcare professionals to explore future directions in crafting disease-specific policies aimed at improving patient care and quality of life while tackling health disparities and unaddressed patient needs.



The General Assembly Meeting of TIF Members took place on November 4, 2023, during which elections for the TIF Board of Directors were held.

The newly elected TIF Board consists of 14 patients and caregivers from 13 countries, representing a wide geographical diversity. This board ensures the patient's voice remains at the forefront of international discussions.





# INTERNATIONAL RELATIONS

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



On December 20, 2023, the Thalassaemia International Federation - TIF held its first meeting with the **ASEAN Thalassaemia Federation**, an umbrella patient organisation with members hailing from the Southeast Asian region.

TIF is overjoyed to support this amazing collaboration that will empower, educate, and elevate patients, caregivers and associations that represent people with thalassaemia in this area.

On November 29, 2023, representatives from TIF held a productive meeting with the **International Federation of Gynecology and Obstetrics (FIGO)**.

TIF and FIGO, represented by Prof. Bo Jacobsson, Division Director of Maternal and Neonatal Health, and Prof. Chiara Benedetto, Head of the Gynaecology and Obstetrics Division at St. Anna Hospital, Torino, Italy, will collaborate in updating TIF's guidelines on pregnancy and fertility and will initiate campaign to raise awareness on these topics.



The quarterly meeting of the **European Hematology Association Patient Advocacy Committee (EHA PAC)** took place in The Hague, Netherlands on November 23, 2023.

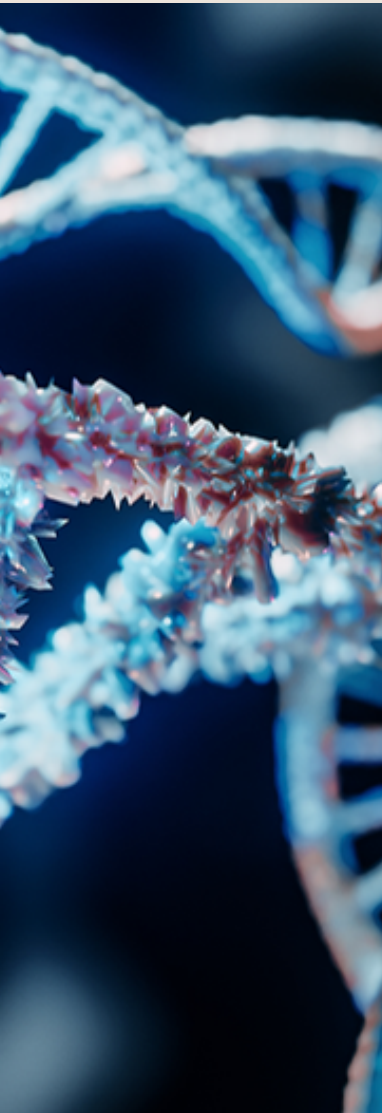
Mr. Loris Brunetta, TIF Board Member, represented the Federation and delivered a presentation on EU Health Policies on behalf of Dr. Androulla Eleftheriou, TIF's Executive Director.



# TOP STORIES

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

## BREAKTHROUGH GENE THERAPIES FOR HAEMOGLOBINOPATHIES RECEIVE APPROVAL



Recent breakthroughs in gene therapy have led to significant advancements in the treatment of haemoglobinopathies. The US Food and Drug Administration (FDA) has approved Casgevy (exa-cel), using the awarded CRISPR/Cas9 genome editing technology, and bluebird bio's Lyfgenia (lovo-cel) for the treatment of sickle cell disease (SCD). Both therapies are administered as a one-time, single-dose infusion and aim to provide long-lasting relief, although years of follow-up will be needed to confirm their efficacy.

Casgevy recently also secured approval from the UK's Medicines and Healthcare Products Regulatory Agency (MHRA) for treating SCD and  $\beta$ -thalassaemia in November. Concurrently, the European Medicines Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP) has recommended conditional approval for Casgevy, with a decision expected in the first quarter of 2024.

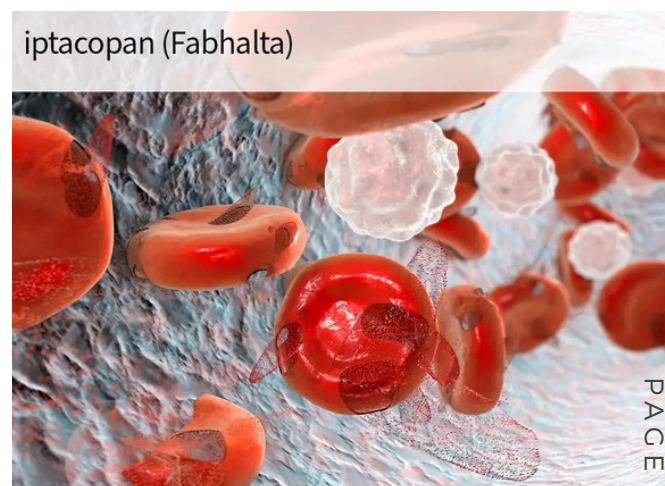
Dr. Androulla Eleftheriou, TIF's Executive Director, celebrated the historic milestones for patients with haemoglobin disorders and emphasized the importance of addressing challenges associated with these innovative treatments.



## FDA APPROVES IPTACOPAN FOR PAROXYSMAL NOCTURNAL HEMOGLOBINURIA

On December 6, 2023, Fabhalta® (iptacopan) received FDA approval as the first oral monotherapy for adults with the rare anaemia paroxysmal nocturnal hemoglobinuria (PNH).

This approval comes after the successful results of the APPLY-PNH study. 82.3% of patients experienced haemoglobin increases  $\geq 2$ g/dL, and 67.7% achieved a haemoglobin level  $\geq 12$  g/dL – all without needing red blood cell transfusions.





# EDUCATIONAL ACTIVITIES

*Offering lifelong education opportunities to patients, caregivers and healthcare professionals all across the world.*

## TIF PODCAST: NAVIGATING MENTAL HEALTH IN THE TDT COMMUNITY



TIF's 'Thalassaemia Champions: The Mental Health Sessions' Podcast is back with 3 brand new episodes.

Mr. George Constantinou, TIF Board Member & Expert Patient, Dr. Evgenia T. Georganda, Psy.D., ECP, Existential-Humanistic Therapist, and Dr. Kathleen Durst, Clinical Social Worker at Cooley's

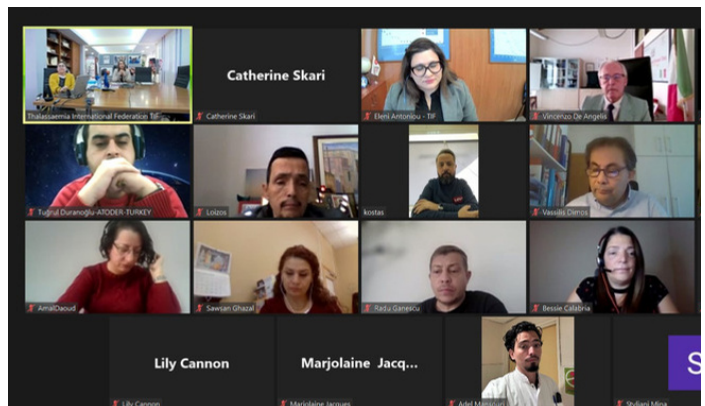
Anemia Foundation (CAF), dive into meaningful conversations on coping with illness, managing anxiety and depression, addressing body image concerns, and more. Three special guests from Sweden, Nepal, and Iran bring their unique perspectives to these important topics, enriching the discussion for everyone affected by transfusion-dependent thalassaemia (TDT).

'Thalassaemia Champions: The Mental Health Sessions' is a video and audio podcast series brought to you by the Thalassaemia International Federation that explores mental health issues for patients, caregivers, and the wider community of individuals with TDT. *cope of EU health policies.*

**WATCH NOW**

## WEBINAR: UNDERSTANDING EU HEALTH POLICIES

The "Empowering Patient Leaders: Understanding EU Health Policies" webinar took place on December 18, 2023, focusing on enhancing participants' knowledge of EU health policies and strengthening their patient advocacy skills within key EU institutions.



The discussion covered crucial regulations such as the EU SoHO Regulation, Health Technology Assessment (HTA) Regulation, Pharma Legislation, and Advanced Therapeutic Medicinal Products (ATMPs) Regulation.



# TIF PUBLICATIONS

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

NEW • NEW • NEW

## NEW FACTSHEET: UNDERSTANDING EU POLICIES ON BLOOD THROUGH THE JOURNEY OF RED BLOOD CELLS

TIF is excited to introduce its latest publication: 'Understanding EU Policies on Blood Through the Journey of Red Blood Cells!'

This comprehensive factsheet takes readers on a journey from start to finish, giving a behind-the-scenes look at the intricate process of blood donation. It contains substantial information about the European Union's policies and procedures for ensuring safe and sufficient blood supplies, as well as the latest advancements and innovations in blood donation practices.

To get your complimentary copy of this must-read factsheet, head over to: <https://thalassaemia.org.cy/publications/tif-publications/eu-policies-made-simple/>

**Understanding EU Policies on Blood Through the Journey of Red Blood Cells**  
FROM DONORS TO PATIENTS IN NEED OF TRANSFUSION

**1994** Prior to 1994 the European Union (EU) had little involvement in blood safety regulations and each country had its own legal framework and infrastructure of blood banks and transfusions to patients.

**2003** Directive 2002/98/EC, adopted in 2003 and known as the "European Blood Directive" was the first document transposed to national law in every EU country, supporting the effort for harmonisation of the standards and structure of blood banks and centres across the EU.

The aim of the Directive was to reassure the public that the framework governing the use of blood and its components in the EU was upgraded in a way to meet the same requirements in all EU countries.

The Blood Directive set standards of quality and safety for the collection, testing, processing, storage and distribution of human blood and blood components, addressing challenges and concerns of the time and indeed contributed significantly to improving the quality and safety of services across the European Union.

Improvements were achieved across the EU (to different levels and extents):

- Residual risk of clinically significant pathogens minimised;
- National Blood Establishments set standards and separated their work from the role of hospital transfusion centres;
- Processing, storage, data collection, non-remunerated blood donation (NRBD) practices improved;
- Research in blood and blood-related processes enhanced in various areas.

Across the European Union:  
**1400** blood establishments  
**20 million** blood donations every year  
**25 million** transfusions to patients  
Around **2/3** of red blood cell transfusions are used in medical cases of anaemia related to chronic disease.

Through the years, shortcomings and gaps in the legal framework were identified and an effort to revise it was initiated by the European Commission in 2019.

These were, amongst others, the following:

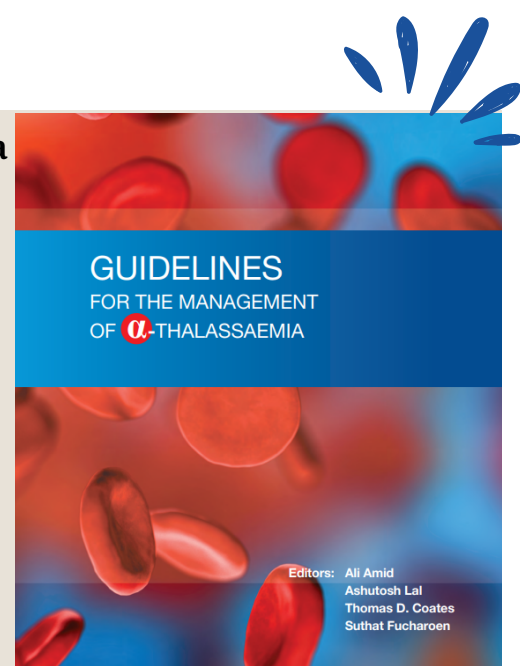
- Technical rules did not follow scientific progress;
- Protection of donors was inadequate;
- Oversight of blood establishments was not harmonised across the European Union;
- Legal clarity was needed to support innovation;
- Supply disruptions were not addressed.

Directive 2002/98/EC is available at <https://eur-lex.europa.eu/legal-content/EN/LJI/?uri=CELEX:32002L0098>

## GUIDELINES FOR THE MANAGEMENT OF $\alpha$ -THALASSAEMIA

TIF proudly presents the first-ever  $\alpha$ -Thalassaemia Management Guidelines, a crucial resource for healthcare professionals and researchers involved in the treatment of this complex genetic condition. Developed by leading medical specialists, these Guidelines provide evidence-based recommendations for diagnosis, treatment, and ongoing patient care.

Offering a comprehensive understanding of  $\alpha$ -thalassaemia's intricacies, the Guidelines aid healthcare professionals in diagnosis and risk assessment. They emphasize multidisciplinary care and patient education while investigating emerging therapies. This publication represents a significant milestone in TIF's mission to enhance the lives of those affected by thalassaemia syndromes.



PUBLISHERS: THALASSAEMIA INTERNATIONAL FEDERATION

**Read More** >



# Season's Greetings

*Be merry!  
Be bright!  
Be blessed!*

Sending joyful celebrations and abundant blessings for **health** and **happiness** to all our friends near and far during this Holiday Season!



**THALASSAEMIA  
INTERNATIONAL  
FEDERATION**