



PROGRESS OUTLINE 2021



PILLAR 4: RESEARCH

—
Activities
Impact



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TABLE OF CONTENTS

EXECUTIVE SUMMARY	2
INTRODUCTION	3
MATCHING OBJECTIVES TO ACTIVITIES AND IMPACT	3
OVERVIEW OF ACTIVITIES (PILLAR 4 – WP 5).....	5
IMPACT ASSESSMENT.....	10

EXECUTIVE SUMMARY

The work of the Thalassaemia International Federation (TIF) can be categorised in four distinct pillars: a) Education; b) Awareness Raising; c) Policy Advocacy and d) Research. In the area of research, TIF aims to raise awareness among researchers on thalassaemia and also inform patients on their rights regarding clinical trial research and patient recruitment.

In 2021, TIF participated in the annual congress of the European Association for the Study of Liver (EASL), the European Haematology Association (EHA), EASL Patient Forum and the Greek Thalassaemia Association, in addition to two policy events of the Blood & Beyond consortium (T5.2). Through this participation, the latest advances and developments in the care and cure of haemoglobinopathies and the progress of clinical trials were made available to TIF, whilst at the same time awareness about haemoglobinopathies was enhanced by members of the research, academic and healthcare professional communities. Moreover, the distribution of such news obtained at the aforementioned congresses and policy events through a Scientific Update (T5.3) every two months further facilitated the engagement of patients in the drug development process.



INTRODUCTION

About Us

The Thalassaemia International Federation (TIF) is a 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.

About Haemoglobinopathies

Haemoglobin Disorders, mainly thalassaemia and sickle cell anaemia, are a group of hereditary (genetic) blood disorders. Approximately 7% of the global population is a carrier of an abnormal haemoglobin gene and more than 500,000 children are born each year with these disorders globally, due to the lack of implementation of effective national programmes for their prevention. In Europe, haemoglobin disorders fall within the official EU definition for rare diseases and it is estimated that approximately 44,000 patients with a haemoglobin disorder live in the region. However, the number of patients located in Europe is increasing due to migration flows from high prevalence countries of the Middle East, South East Asia and Africa.

About THALIA

Thalassaemia, a previously fatal childhood genetic disease, can today be effectively prevented and adequately treated, as a result of the medical and scientific advances that took place in the last three decades. However, thalassaemia's prioritisation on national health agendas and the development of national plans for its effective control in the EU has been hampered by the many challenges involved, mainly related to its rarity and migration.

"THALassaemia In Action" (THALIA) focuses on Europe, targeting patients with thalassaemia and other haemoglobinopathies, healthcare professionals and policymakers. THALIA has a special focus on:

- 1) countries that receive most refugees and migrants from countries with high prevalence in thalassaemia; namely France, Germany, Sweden;
- 2) major transit countries for migrants; namely Serbia and Austria.

About the impact of COVID-19

The continuing evolution of COVID-19 throughout 2021, resulted in the implementation of the COVID-19 Contingency and Mitigation Plan as described in the THALIA2021 SGA, to ensure the fulfilment of activity objectives whilst simultaneously ensuring the safety of participants (patients, healthcare professionals, policy-makers, TIF Staff and affiliates) at all times.

MATCHING OBJECTIVES TO ACTIVITIES AND IMPACT

General objective

IV.0. Support research programmes and studies focused on treatment improvements and the final cure.

Task No	T5.2	T5.3
Target Audience	HCPs / Researche rs Patients	Patients
KPI Met	✓	✓
Justification	✓	✓

Specific Objectives

IV.1. Raise awareness among researchers on Thalassaemia.

T5.2: Participation of TIF experts in scientific conferences

Results: TIF experts participated in the annual scientific conferences of the European Association for the Study of Liver, the European Haematology Association and the Greek Thalassaemia Association, in addition to multi-stakeholder virtual events concerning blood policies in Europe and health literacy. Following these conferences, the TIF scientific update was produced to inform patients on the latest developments in clinical trial research and updates on patient recruitment.

Impact: These awareness raising activities increased visibility on thalassaemia-related issues and provided TIF with useful insights on the latest advancement in the area of the treatment of haemoglobinopathies.

Impact Management / Mitigation Measures: N/A

IV.2 Inform patients on their right concerning clinical trial research and contributing in patient recruitment.

T5.3: Distribution of quarterly update on the current ongoing clinical trials

Results: TIF produced 4 Scientific Updates to inform patients on the latest developments in clinical trial research and updates on patient recruitment. The combined readership from the distribution list of the Scientific Updates and the page views on TIF's website amounts to 4,103 patient and patient associations across Europe. Furthermore, the preparation and distribution of the Sickle Cell Disease Update and the Scientific Bulletin further bolstered this effort.

Impact: These awareness raising activities increased visibility of thalassaemia focused scientific advances generating interest in patients for participating in clinical trials.

Impact Management / Mitigation Measures: N/A

OVERVIEW OF ACTIVITIES (PILLAR 4 – WP 5)

TASK	T5.2: Participation of TIF experts in scientific conferences
RESPONSIBLE STAFF, SUPERVISING STAFF	<p>Responsible Staff: Medical Advisor, Dr Michael Angastiniotis (Leader) Operations Manager, Lily Cannon Communications Officer, Aikaterini Skari</p> <p>Supervising Staff: Executive Director, Dr Androulla Eleftheriou</p>
INDICATORS	<ul style="list-style-type: none"> ▪ Participation in 5 scientific congresses
CALENDAR OF ACTIVITIES	<ul style="list-style-type: none"> ▪ Registration to Annual Congress of the European Hematology Association (MS15): June 2021 ▪ Participation in the Annual Congress of the European Hematology Association: 9 – 17 June 2021 ▪ Participation in the World Blood Donor Day Digital Workshop: 14 June 2021 ▪ Participation in the Annual Congress of the International Liver Congress of the European Society for the Study of Liver (EASL): 23 – 26 June 2021 ▪ Participation in the EASL Patient Forum 2021: 22 Nov. 2021 ▪ Participation in the Blood & Beyond Policy Event: 11 Oct 2021 ▪ Participation in the Annual Congress of the Greek Thalassaemia Association: 28 Nov. 2021
DESCRIPTION OF THE ACTIVITY	
<p>Increasing awareness about thalassaemia and haemoglobinopathies amongst the medical community, including healthcare professionals, researchers, clinicians, academics and scientists, constitutes a priority for TIF.</p> <p>To achieve this objective, TIF has developed strategic partnerships with medical health-related professional associations¹, participating each year in their respective Annual Congresses in Europe and abroad. Through this participation, with a dedicated exhibition booth where TIF publications are distributed and/or presentations / abstracts in the Congress Programmes, TIF contributes to raising the visibility of thalassaemia and haemoglobinopathies to a wide audience of scientists since these Congresses gather over 10,000 participants each.</p>	

¹ <https://thalassaemia.org.cy/who-we-are/collaborations/>

In 2021, TIF participated in the:

1. Annual Congress of the European Hematology Association
2. World Blood Donor Day Digital Workshop
3. Annual Congress of the International Liver Congress of the European Society for the Study of Liver (EASL)
4. EASL Patient Forum 2021
5. Blood & Beyond Policy Event
6. Annual Congress of the Greek Thalassaemia Association

The **Annual Congress of the European Hematology Association** (EHA) held on 09 -17 June 2021, held this year virtually, was participated by over 15,000 haematologists and researchers.

Dr Androulla Eleftheriou (TIF Executive Director), Dr Michael Angastiniotis (TIF Medical Advisor), Ms Lily Cannon (TIF Operations Manager) and Ms Eleni Antoniou (TIF Senior European & International Affairs Officer) participated (MS15) in a number of sessions on behalf of the Thalassaemia International Federation. Moreover, Mr George Constantinou (TIF Ass. Secretary, Lead T-PAGs) chaired a patient advocacy session focused on *Lessons from COVID-19*. Most notably, TIF Delegates attended three satellite symposia focusing on the new scientific advances in thalassaemia in addition to numerous oral presentations and e-poster sessions that provided ample information on the progress of newly approved therapies and those still in clinical trials. A dedicated article about the information shared during the Congress on pyruvate kinase deficiency has also been disseminated through TIF's website [here](#). An extended report about TIF's participation can be found on TIF's website [here](#).

Information obtained at the Congress about thalassaemia and sickle cell disease has been included the scientific update (v. T5.3) distributed to patients and has been further disseminated through social media posts in addition to being included on the dedicated webpage of TIF's website on [Clinical Trials](#).

Dr Androulla Eleftheriou (TIF Executive Director) had the opportunity to form part of the **World Blood Donor Day Digital Workshop** held on 14 June 2021. The Workshop, held as part of the satellite activities of the official WHO World Blood Donor Day activities of the host country (Italy), focused on 'Revisiting & Rethinking the Use of Blood in Chronic Diseases across Europe'. Thus Dr Eleftheriou had the opportunity to highlight the needs of transfusion-dependent thalassaemia patients as an example of a chronic disease with increasing needs in blood with an array of challenges.

The **International Liver Congress of EASL** takes place on an annual basis gathering around 10,000 delegates and 250 media representatives from all over the world. In 2021, Dr Androulla Eleftheriou (TIF Executive Director) and Mr George Constantinou (TIF Ass. Secretary) participated in the Congress which took place virtually, on 23 – 26 June 2021. Most notably, TIF had the opportunity to interact with the Congress participants through a dedicated digital booth where information on thalassaemia, migration, liver complications, guidelines for clinical management and other TIF publications were exhibited.

The TIF Delegates attended a number of sessions including the Joint EASL – CDC, ECDC, WHO Symposium entitled *Viral Hepatitis Elimination - Assessing the progress in 2021*. The Symposium eminent faculty discussed the new WHO guidance on viral hepatitis elimination as well as presented data on mortality rates in Europe.

Furthermore, in an effort to strengthen collaboration and partnership with EASL, TIF has continues to form an integral part of [EASL Patient Synergies](#), thus taking an active role in the annual **EASL Patient Forum 2021** entitled ‘Health literacy to empower and address inequities. The Forum took place virtually on 22 November 2021. Ms Eleni Antoniou (TIF Senior European & International Affairs Officer) represented the Federation and delivered an insightful presentation on thalassaemia and health literacy focusing on health education as the driver for better quality of care, and thus a better quality of life. Ms Antoniou showcased TIF’s continuously evolving educational programme which includes organising events and publications and promoting academic courses, that aims to contribute to creating educated and knowledgeable patient advocates that can be partners to healthcare professionals and decision-making bodies at national, regional, and international levels. Accordingly, TIF has invested in making its resources accessible, both in terms of their form (online, offline) and their language.

Over 75 participants including national health authorities, EU institutions (European Parliament, European Commission), industry, patient organisations, healthcare professionals and others came together in a **multi-stakeholder Policy Event** held on 11 October 2021 to discuss policies and practices that would support blood sustainability in Europe, against a current backdrop that is seeing the revision of the Blood, Tissues and Cells (BTC) Directive.

Chronic, multi-transfused thalassaemia patients were represented by Dr Androulla Eleftheriou (TIF Executive Director) who highlighted the need for ‘*more robust oversight and stringent coordination at the national but most importantly the EU level to ensure the safety, quality and adequacy of blood*’. The revision of the Directive should, Dr Eleftheriou continued ‘*provide an opportunity to see more harmonization of quality provided by blood transfusion services across each EU Member State*’.

A summary report of the Policy Event was shared with European Commission culminating in a meeting held on 16 December 2021. Dr Androulla Eleftheriou (TIF Executive Director) participated in the meeting on behalf of the Blood and Beyond initiative, and more specifically representing the voice of multi-transfused patients. Dr Eleftheriou highlighted the advocacy efforts of the Federation since its establishment in key priority areas that would ensure the safety and adequacy of blood for thalassaemia patients

The highlights of the TIF International Conference, especially in terms of the new scientific advancements as well as the impact of living with thalassaemia, was the focus of Dr Androulla Eleftheriou (TIF Executive Director) and Dr Michael Angastiniotis (TIF Medical Advisor) presentation during the **annual congress of the Greek Thalassaemia Association**. The Congress, held in a hybrid format on 28 November 2021, provided the opportunity to TIF Delegates to further raise awareness about thalassaemia, advancements

in the field and patient challenges in Greek, thus overcoming the linguistic challenges of those healthcare professionals who could not attend the event (v. T2.2.2) whilst strengthening partnership with the organising Association.

Scientific Publications:

Additionally, TIF authored / co-authored the following scientific articles on thalassaemia in 2021:

1. The prevention of thalassaemia revisited: A historical and ethical perspective by the Thalassaemia International Federation.
Published by Hemoglobin. Read it [here](#)
2. Haemoglobin Disorders in Europe: A Systematic Effort of Identifying and Addressing Unmet Needs and Challenges by the Thalassaemia International Federation.
Published in Thalassaemia Reports. Read it [here](#).
3. Policy briefing on Blood Use in Europe: Learning from the impact of COVID-19
Read it [here](#)
4. Juggling between the Cost and Value of New Therapies.
Read it [here](#).
5. Existing challenges to thalassaemia care exacerbated by pandemic
Published in Hematology Advisor. Read it [here](#).
6. COVID-19 pandemic and the great impulse to telemedicine: the basis of the WONCA Europe Statement on Telemedicine at the WHO Europe 70th Regional Meeting September 2020
Published in Primary Healthcare Research and Development. Read it [here](#).

RESULTS ACHIEVED

- Increased awareness about thalassaemia amongst members of the scientific community.
- Partnerships were strengthened through participation in the Annual Congresses.
- Patient community was informed through dedicated Scientific Reports about the progress of clinical trials.

TASK	T5.3: Patient Information
RESPONSIBLE STAFF, SUPERVISING STAFF	<p>Responsible Staff: Medical Advisor, Dr Michael Angastiniotis (Leader) Operations Manager, Lily Cannon Communications Officer, Aikaterini Skari</p> <p>Supervising Staff: Executive Director, Dr Androulla Eleftheriou</p>
INDICATORS	<ul style="list-style-type: none"> ▪ Distribution of 4 reports to a readership of 4,000
CALENDAR OF ACTIVITIES	<ul style="list-style-type: none"> ▪ Scientific Update: 08 January 2021 ▪ Sickle Cell Disease Scientific Update: 08 January 2021 ▪ Scientific Update: 30 June 2021 ▪ Sickle Cell Disease Scientific Update: 30 June 2021 ▪ Scientific Bulletin: September 2021 ▪ Scientific Bulletin: October 2021 ▪ Scientific Update: 27 October 2021 ▪ Sickle Cell Disease Scientific Update: 27 October 2021 ▪ Scientific Update: 31 December 2021 ▪ Sickle Cell Disease Scientific Update: 31 December 2021
DESCRIPTION OF THE ACTIVITY	
<p>Providing information to patients about the latest scientific developments in thalassaemia and haemoglobinopathies, including recently authorised therapies and medicinal products, as well as those in the pipelines, constitutes a priority for TIF.</p> <p>To achieve this objective, TIF distributes a quarterly Scientific Update to its membership base. The information contained in the Update is subsequently uploaded on a dedicated webpage (Clinical Trials Update) on TIF’s website.</p> <p>In 2021, TIF has distributed at total of 4 Scientific Updates to a total of 1,996 recipients.</p> <p>The webpage Clinical Trials Update has received a total of 9,011 page views in 2021 of which 23,4% (i.e. 2,107 page views) are from Europe.</p> <p>Additionally, seeking to provide easy to understand information on scientific issues, TIF has proceeded in 2021 to develop and distribute two new bulletins:</p> <ol style="list-style-type: none"> 1. Sickle Cell Disease Scientific Update. A quarterly update including developments of clinical trials for drugs and therapies in the field of sickle cell disease distributed to members of the TIF-International Sickle Cell Disease Group, to build knowledge and capacity of the group, ensuring that they are productive partners of regulatory bodies and national health authorities. A total of 4 updates were distributed in 2021, to 181 recipients. The information contained is subsequently uploaded on the Clinical Trials Update webpage on TIF’s website. 	

2. Scientific Bulletin. A monthly digest of selected scientific articles presented in a simplified form for patients and families. A total of 2 bulletins were distributed in 2021. A total of 4 updates were distributed in 2021, to 2,177 recipients. The Bulletin is also available on TIF’s website [here](#).

**RESULTS
ACHIEVED**

- Patient community was informed through dedicated Scientific Reports about the progress of clinical trials.

IMPACT ASSESSMENT

Methodology (*The Impact Model*)

TIF’s Impact Model serves to compile a complete set of questions which, when answered, allows a conclusive and comprehensive impact assessment of the annual activities. Did the activities fulfil the expectation of the THALIA stakeholders? What were the main outputs of THALIA? What effects did THALIA produce, directly and on the long term? These are examples of questions the impact assessment intends to answer. The Impact Model is focusing on the evaluation strand of the Logic Model.

In order to take the whole process of THALIA into account, TIF’s Impact Model is structured according to four main stages: (1) inputs, (2) outputs, (3) outcomes and (4) impacts. The first stage (1) consider aspects that can be evaluated before or at the beginning of THALIA, whereas the three others stages (2-4) consider aspects that can be measured conclusively only after the programme has been finished. All have been analysed in the respective THALIA proposal and have been linked to specific indicators. More specifically:

1. Inputs: Inputs include not only financial means but also human resources, equipment, knowledge and ideas.
2. Outputs: Outputs represent the direct results of the activities realised.
3. Outcomes: Outcomes are defined as the effects of the outputs on the target audiences. Outputs may benefit in terms of increased knowledge, improved networking and cooperation skills or access to new markets.
4. Impacts: Impacts are the wider effects of TIF’s THALIA activities. These are defined as the benefits for the immediate target audience and users of the outputs. The benefits for the society at large are called relative impacts.

To assess impact, it is important to have a set of basic criteria and compare them against the indicators set in the THALIA annual plan:

	CRITERIA	KEY QUESTIONS
1	RELEVANCE	Are the “general objective” and “specific objectives” still meaningful?
2	EFFECTIVENESS	Has the objective been achieved? How much contribution did the “outputs” make?
3	EFFICIENCY	To what extent have “inputs” been converted to “outputs”?
4	IMPACT	What positive or negative, direct or indirect effects have happened?
5	SUSTAINABILITY	To what extent will TIF be able to maintain the positive results of its activities?

Criteria	Relevance	Effectiveness	Efficiency	Impact	Sustainability
Tasks	<i>Are the "general objective" and "specific objectives" still meaningful?</i>	<i>Has the objective been achieved? How much contribution did the "outputs" make?</i>	<i>To what extent have "inputs" been converted to "outputs"?</i>	<i>What positive or negative, direct or indirect effects have happened?</i>	<i>To what extent will TIF be able to maintain the positive results of its activities?</i>
T5.2: Participation of TIF experts in scientific conferences	Through its participation in online scientific conferences, TIF was able to raise awareness among healthcare professionals about thalassaemia-related issues and share information on clinical trial updates with the patients' community.	TIF experts participated in 6 online conferences and drafted TIF's Scientific Update, made available to patients through the website.	The "inputs" required for the implementation of this task were optimally used, namely: staff time, planning time, knowledge base, partners, contacts.	The participation of TIF experts in conferences had a dual impact: on researchers/clinicians and on patients. The former were informed about thalassaemia and the latter on the latest research advancements.	TIF shall continue participating in conferences to increase visibility on thalassaemia and keep gathering information about the latest developments in the field.
T5.3: Patient Information	The distribution of updates on clinical trials to patients enabled TIF to keep the patient community informed of ongoing scientific developments involving thalassaemia.	TIF produced and distributed 10 Updates (on Scientific Update, Sickle Cell Disease Update & Scientific Bulletin) to inform patients on the latest developments in clinical trial research and updates on patient recruitment.	The "inputs" required for the implementation of this task were optimally used, namely: staff time, planning time, knowledge base, partners, contacts.	Keeping abreast of the progress of clinical trials facilitated the knowledge acquisition of patients as well as enabled TIF to remain engaged with companies in order to ensure trials were patient-focused meeting expectations of the community.	TIF's Scientific Update has been well received by the patient community and TIF shall continue to gather and disseminate information about the latest developments in the field.

