



PROGRESS OUTLINE 2020



PILLAR 4: RESEARCH

—
Activities
Impact



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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 2020, TIF participated in the annual congress of the European Association for the Study of Liver (EASL), the European Haematology Association (EHA) and the PanHellenic Congress on Economics and Health Policy, in addition to a webinar of the European Hematology Association focused on thalassaemia in the time of COVID-19 (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 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 nature of many of THALIA2020 activities include travel and physical interaction to better assess the community needs and deliver efficient educational and policy-making tools. Therefore, the surgent COVID-19 pandemic required a stringent vigilance throughout 2020 by the Internal Evaluation Committee (IEC) and TIF Board of Directors so as on the one hand to ensure the materialisation of activities, and on the other hand to ensure the safety of involved parties. Close monitoring by the IEC of the evolving epidemiological situation and the measures being taken across EU countries to further inform and revise the mitigation measures for each activity continued through the year, so as to identify new ways to fulfil the objective of the activity (e.g. virtual attendance) or to proceed as planned changing some features (e.g. selecting an alternative venue with the same profile characteristics), aiming to ensure 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 / Researcher 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 PanHellenic Congress on Economics and Health Policy, in addition to a webinar of the European Hematology Association focused on thalassaemia in the time of COVID-19. 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 6 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 4,037 patient and patient associations across Europe.

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 P.A. to the Executive Director, Maria Peletie Communications Officer, Aikaterini Skari Office Administration, Stella Eleftheriou</p> <p>Supervising Staff: Executive Director, Dr Androulla Eleftheriou</p> |
| INDICATORS | <ul style="list-style-type: none"> ▪ Participation in 2 scientific congresses |
| CALENDAR OF ACTIVITIES | <ul style="list-style-type: none"> ▪ Participation in the European Hematology Association webinar: 16 April 2020 ▪ Registration to Annual Congress of the European Hematology Association (MS15): June 2020 ▪ Participation in the Annual Congress of the European Hematology Association: 11 – 21 June 2020 ▪ Participation in the Annual Congress of the International Liver Congress of the European Society for the Study of Liver (EASL): 27 – 29 August 2020 ▪ Participation in the Annual PanHellenic Congress on Economics and Health Policy: 07 – 10 December 2020 |
| DESCRIPTION OF THE ACTIVITY | |
| <p>Increasing awareness about thalassaemia and haemoglobinopathies amongst the medical community, including healthcare professionals, researches, 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/tif-collaborations/medical-health-organizations-ngos/>

In 2020, TIF participated in the:

1. Annual Congress of the European Hematology Association
2. Annual Congress of the International Liver Congress of the European Society for the Study of Liver (EASL)
3. European Hematology Association webinar
4. Annual PanHellenic Congress on Economics and Health Policy

The Annual Congress of the European Hematology Society (EHA) held on 11 – 21 June 2020 virtually as a result of the COVID-19 pandemic and was participated by approximately 27,000 haematologists and researchers.

Dr Androulla Eleftheriou (TIF Executive Director) and Ms Lily Cannon (TIF Operations Manager) attended (MS15) a number of sessions on behalf of the Thalassaemia International Federation. Most notably, Dr Eleftheriou and Ms Cannon joined 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. An extended report of the satellite symposia can be found on TIF's website [here](#).

Information obtained at the Congress 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](#).

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 2020, Dr Androulla Eleftheriou (TIF Executive Director) and Dr Michael Angastiniotis (TIF Medical Advisor) participated in the Congress which took place virtually, as a result of the COVID-19 pandemic, on 27 – 29 August. Most notably, TIF had the opportunity to interact with the Congress participants through a dedicated booth where information on thalassaemia, migration, liver complications, guidelines for clinical management and other TIF publications were exhibited.

Furthermore, in an effort to strengthen collaboration and partnership with EASL, TIF has joined the EASL Patient Organisations Community. This will provide the opportunity for TIF to bring to the table the experience of multi-transfused patients from around the world, thus assisting hepatologists gain a better knowledge and more insight in the needs of our patients. More information available [here](#). Dr Androulla Eleftheriou (TIF Executive Director) and Mr George Constantinou (TIF Board Member) have participated in the first meeting of the Community, held virtually on 04 November 2020. Dr Eleftheriou, presented the work of the Federation emphasizing the importance of developing partnerships and made reference to TIF's involvement in the field of hepatology for many years now, most notably with the dedicated chapter in TIF's Guidelines for the Clinical Management of Transfusion Dependent Thalassaemia² and its Position Paper regarding Viral Hepatitis C³.

The fast escalation of the COVID-19 pandemic in Europe in the spring of 2020 prompted the European Hematology Association to organize a dedicated webinar, entitled

² <https://www.eha.com.ni/cewa/2020/11/30/399>

³ <https://thalassaemia.org.cy/positions-policies/position-papers/>

“SCD/Thalassaemia and COVID-19: Possible Risks and a Proposal for a Patient Pathway During the Pandemic”. The webinar held on 16 April 2020 and was attended by over 700

haematologists. The speaker panel included ⁶ had the opportunity to present the data on thalassaemia patients infected with COVID-19 (Prof. MD Cappellini), the impact of COVID-19 on SCD patients (Prof. R. Colombatti) in addition to TIF's global data of patient infections and a proposed pathway for safer access to transfusion areas and day clinics

**RESULTS
ACHIEVED**

- Increased awareness about thalassaemia amongst members of the scientific community.
- Partnerships (cf. Topics-in-Focus of the EHA, & EASL Patient Organisations Community) 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: 31 January 2020 ▪ Scientific Update: 31 March 2020 ▪ Scientific Update: 31 May 2020 ▪ Scientific Update: 25 August 2020 ▪ Scientific Update: 05 November 2020 ▪ Scientific Update: 31 December 2020 |
| 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 Scientific Update every two months 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 2020, TIF has distributed at total of 6 Scientific Updates to a total of 1,996 recipients.</p> <p>The webpage Clinical Trials Update has received a total of 7,809 page views in 2020 of which 26.1% (i.e. 2,041 page views) are from Europe.</p> | |
| RESULTS ACHIEVED | <ul style="list-style-type: none"> ▪ 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 THALIA2018 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 4 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 6 Scientific Updates 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. |

