



PROGRESS OUTLINE 2018



Co-funded by
the Health Programme
of the European Union

PILLAR 4: RESEARCH



Activities

Impact

Lessons Learned

TABLE OF CONTENTS

EXECUTIVE SUMMARY	2
INTRODUCTION.....	3
MATCHING OBJECTIVES TO ACTIVITIES AND IMPACT	4
OVERVIEW OF ACTIVITIES (PILLAR 4 – WP 5).....	5
IMPACT ASSESSMENT.....	8
LESSONS LEARNED.....	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 2018, TIF published one article on the role of the European Reference Networks and how these can promote research in the area of the clinical management of haemoglobinopathies. Moreover, two TIF experts participated in the annual congress of the International Society of Blood Transfusion (ISBT) and two in the annual conference of the European Haematology Association (EHA) to get informed about the latest advances and development in the treatment of haemoglobinopathies and the progress of clinical trials and at the same time promote research in the area of haemoglobinopathies.



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 204 members from 62 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 Europe. 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.

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	T5.1	T5.2	T5.3
Target Audience	HCPs / Researche rs Patients	HCPs / Researche rs Patients	HCPs / Researche rs Patients
KPI Met	✓	✓	✓
Justification	✓	✓	✓

Specific Objectives

IV.1. Raise awareness among researchers on Thalassaemia.

Activity: T5.1: TIF Guidelines in peer-reviewed academic journals; **T5.2:** Participation of TIF experts in scientific conferences

Results: One article was published on the added value of European Reference Networks, while TIF experts participated in the annual scientific conferences of the European Haematology Association and the International Society of Blood Transfusion. 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: TIF will continue to actively promote research in the area to achieve its mission and vision.

OVERVIEW OF ACTIVITIES (PILLAR 4 – WP 5)

TASK	T5.1: TIF Guidelines in peer-reviewed academic journals
RESPONSIBLE STAFF, SUPERVISING STAFF	<p>Responsible Staff: Medical Advisor, Dr Michael Angastiniotis (Leader) Operations Manager, Lily Cannon</p> <p>Supervising Staff: Executive Director, Dr Androulla Eleftheriou</p>
INDICATORS	<ul style="list-style-type: none"> ▪ 1 article published in a peer-reviewed academic journal. ▪ Number of downloads: 300
DESCRIPTION OF THE ACTIVITY	<p>Seeking to ensure the wider access of healthcare professionals to TIF's International Guidelines for the Management of Transfusion Dependent Thalassaemia a review article entitled "<i>European Reference Networks (ERNs): Could they become game-changers for patients with rare and complex diseases?</i>" was published in Thalassaemia Reports – a reputable international medical peer-reviewed, open-access journal. The article has been viewed 362 times can be accessed at https://www.pagepressjournals.org/index.php/thal/announcement/view/308</p> <p>The article builds on TIF's International Guidelines for the Management of Transfusion Dependent Thalassaemia for the provision of quality care, and stresses the importance of enhancing collaborations between regional and national Centres of Expertise with the European Reference Networks, as a viable solution to many of the problems and challenges rare disease patients are facing daily, including patients with thalassaemia in Europe.</p>
RESULTS ACHIEVED	<ul style="list-style-type: none"> ▪ Publication of 1 article in peer-reviewed academic journal and increased awareness of healthcare professionals about the possibilities of ERNs.

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> <p>Supervising Staff: Executive Director, Dr Androulla Eleftheriou</p>

INDICATORS	<ul style="list-style-type: none"> ▪ Participation of 2 TIF Experts in the Annual Congress of the International Society of Blood Transfusion (ISBT) ▪ Participation of 2 TIF Experts in the Annual Congress of the European Haematology Association (EHA)
CALENDAR OF ACTIVITIES	<ul style="list-style-type: none"> ▪ Registration to Annual Congress of the International Society of Blood Transfusion (ISBT) and Annual Congress of the European Haematology Association (EHA) (MS13) – March 2018 ▪ Participation in the Annual Congress of the International Society of Blood Transfusion (ISBT), 02 – 06 June 2018 ▪ Participation in the Annual Congress of the European Haematology Association (EHA), 14 – 17 June 2018
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 health-related professional associations, namely the International Society for Blood Transfusion (ISBT) and European Hematology Association (EHA) since 2011, 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 approximately 12,000 participants each.</p> <p>In 2018, TIF experts participated in the following Congresses:</p> <ol style="list-style-type: none"> 1. Annual Congress of the International Society of Blood Transfusion (ISBT), and 2. Annual Congress of the European Haematology Association (EHA) <p>The Annual Congress of the International Society of Blood Transfusion (ISBT) was held in Toronto, Canada between 02 – 06 June 2018. TIF Expert, Prof. Constantina Politis (member of TIF’s International Advisory Board and expert in Blood Transfusion) moderated a dedicated session on Hemoglobinopathies on 05 June 2018, which focused on the burden of transfusion in thalassaemia alloimmunization prevention and management. During this session, that was attended by approximately 300 delegates, Prof. Politis also was able to present the work of the Federation emphasizing the importance of developing partnerships with members of the scientific community and showcasing the new educational tools for healthcare professionals (e.g. e-Thal-Ed platform). In addition, Prof. Politis made reference to TIF’s involvement in the field of blood transfusion for many years now, most notably with its response to the Public Consultation for the Evaluation of the Blood, Tissues and Cells Directives of 2002 and 2004¹.</p>	

1

https://ec.europa.eu/health/sites/health/files/blood_tissues_organ/docs/2018_consultation_evaluationbtc_report_en.pdf

Prof. Politis, furthermore, had the opportunity to meet with 20 other specialists in the field and to discuss issues pertaining to the safety and adequacy of blood for patients with thalassaemia. Discussions also focused on the implications of the migration influx in Europe, including the possible increasing demands on blood supply by haemoglobinopathy patients as well as cross-matching challenges which may occur a result of the limited pool of fully-matched/ suitable donors. Cross-matching of donor and recipient is essential in order to avoid transfusion related reactions (with a wide range of severity). Moreover, there may be delays in transfusion, which in itself may cause additional complications to the patients' well-being (including bone marrow expansion and severe anaemia), due to a limited supply of fully matched/ suitable donors in the newly formed migrant communities in Europe. This limited supply may be the result of blood donation practices within the community. It is noted that compatible donors are more likely to be found within donors of the same geographic origin as the recipient as the development of antibodies will be matched. As a result of this active participation, the ISBT has proposed the establishment of a specific Working Group on Haemoglobinopathies to discuss these issues further and promote actions to ensure blood safety and availability.

The Annual Congress of the European Haematology Association (EHA) took place in Stockholm, Sweden between 14 – 17 June 2018. TIF Expert Delegates, Dr Androulla Eleftheriou (TIF Executive Director), Mr Loris Angelo Brunetta (TIF Board Member) and Lily Cannon (TIF Operations Manager) actively participated in the Patient Advocacy Sessions which this year focused on Quality of Life and Patient – Reported Outcomes (PRO's). In addition, they participated in the Capacity Building Session for EHA Patient Fellows organised by the EHA-PAG (Patient Advocacy Group) in collaboration with the European Reference Network for Haematology, EuroBloodNet and its constituent patient representatives (e-PAGs), of which Mr Brunetta is a member. The Capacity Building Session focused on providing an overview of the EuroBloodNet's work and progress as well as introducing PRO's and QoL measurement instruments. The role of the Federation as an integral member and leader of the EHA Patient Advocacy Group (EHA-PAG) has been well established through TIF's involvement in the development of the Patient Advocacy Track of the Congress, as well as participation in the EHA Patient Fellowship Programme since 2011.

The EHA, in the context of the Congress, has announced its focus on Haemoglobinopathies, with TIF at the centre of these activities along with international experts in the field. Amongst the first activities undertaken by the core group is updating the Haematology Research Road-Map (due to be completed by the end of 2018).

In the context of this participation, the TIF Expert Delegates conducted an additional 24 face-to-face meetings with a total of 30 haematologists and researchers who are active in clinical trials in the field of thalassaemia, as well as 12 other healthcare professionals who were interested to learn about the Federation and its role in working with the healthcare professional community.

RESULTS ACHIEVED

- Increased awareness about thalassaemia amongst members of the scientific community (cf. 300 participants at dedicated session of ISBT Congress; meetings with 42 scientists at EHA Congress).
- Visibility of thalassaemia augmented (cf. dedicated session at ISBT Congress).
- Strengthening of strategic partnerships (cf. creation of Working Group on Haemoglobinopathies with ISBT; EHA focus on Haemoglobinopathies)
- Publication of TIF's scientific update and improved knowledge of patients on ongoing 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>
T5.1: TIF Guidelines in peer-reviewed academic journals	Publications are an efficient way to reach healthcare professionals and inform them about thalassaemia-related issues.	The publication was downloaded at least 300 times.	The "inputs" required for the implementation of this task were optimally used, namely: staff time, planning time, volunteer hours, knowledge base, technology, partners, contacts.	TIF contributed in the support of EC's flagship initiative to develop the European Reference Networks, highlighting their added value for the optimal treatment of thalassaemia and haemoglobinopathies.	TIF shall continue working on a number of publications to maintain contact with the research community.
T5.2: Participation of TIF experts in scientific conferences	Through its participation in 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 2 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.

LESSONS LEARNED

TIF's research activities in 2018:

STRENGTHS	WEAKNESSES
<ul style="list-style-type: none"> ▪ were well-defined and targeted, involving both the research and patients' communities; ▪ focused on popular means (journal/conferences) to multiply impact; ▪ confirmed that the treatment of thalassaemia remains a popular field among researchers; ▪ produced a unique publication that is a point of reference for thalassaemia research. 	<ul style="list-style-type: none"> ▪ N/A
OPPORTUNITIES	THREATS
<ul style="list-style-type: none"> ▪ Through its scientific network, TIF has useful insights on current research trends, monitors ongoing trials and makes sure that the patients' views are heard throughout all processes. 	<ul style="list-style-type: none"> ▪ N/A