



PROGRESS OUTLINE 2019



PILLAR 4: RESEARCH

—
Activities
Impact



Co-funded by
the Health Programme
of the European Union



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.....	7

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 2019, TIF participated in the annual congress of the European Association for the Study of Liver (EASL), the International Society of Blood Transfusion (ISBT) and 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 by consolidating interest in this disease area through the establishment of working groups.



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 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	T5.2
Target Audience	HCPs / Research ers 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 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: 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 2 scientific congresses
CALENDAR OF ACTIVITIES	<ul style="list-style-type: none"> ▪ Registration to Annual Congress of the European Association for the Study of Liver (MS17) – March 2019 ▪ Participation in the Annual Congress of the European Association for the Study of Liver, 10 – 14 April 2019 ▪ Participation in the Annual Congress of the European Hematology Association, 13 – 16 June 2019 ▪ Participation in the Annual Congress of the International Society for Blood Transfusion, 22 – 26 June 2019
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 approximately 10,000 participants each.</p> <p>In 2019, TIF participated in the:</p> <ol style="list-style-type: none"> 1. Annual Congress of the International Liver Congress of the European Society for the Study of Liver (EASL) 2. Annual Congress of the International Society for Blood Transfusion 3. Annual Congress of the European Hematology Association 	

¹ <https://thalassaemia.org.cy/tif-collaborations/medical-health-organizations-ngos/>

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 2019, TIF Expert, Dr Anne Yardumian (member of TIF' International Scientific Advisory Board and Consultant Haematologist) participated in the Congress which took place in Vienna, Austria on 10 – 14 April. Dr Yardumian actively participated in a Multi-transfused Patient Forum Discussion, which focused on the development of clinical guidelines for the appropriate treatment of liver-related issues for patients with other underlying disorders such as thalassaemia. It is well – known that liver complications constitute one of the commonest organ complications of thalassaemia² and thus discussion on the development of disease-specific guidelines to manage liver complications are of particular interest to the patient community and TIF.

During the Forum, that was attended by approximately 300 delegates, Dr Yardumian 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, Dr Yardumian 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 Thalassaemia³ and its Position Paper regarding Viral Hepatitis C⁴.

Dr Yardumian, furthermore, had the opportunity to meet with 20 other specialists in the field and to discuss issues pertaining to the liver disease in patients with thalassaemia, particularly on identifying ways of supporting multi-transfused patients in Europe access innovative and appropriate drugs for the treatment of viral hepatitis. Moreover discussions also focused on the implications of the migration influx in Europe, including the possible increasing cases of Hepatitis B and / or C which have previously been eliminated in many EU countries.

The Annual Congress of the European Hematology Society (EHA) held on 13 – 16 June 2019 in Amsterdam, Netherlands and was participated by approximately 12,000 haematologists and researchers. International experts in the field of thalassaemia, members of TIF's International Scientific Advisory Board attended the Congress and delivered presentations on new advances in the care and cure of thalassaemia including gene therapy.

The Annual Congress of the International Society of Blood Transfusion (ISBT) held on 22 – 26 June 2019 in Basel, Switzerland was attended by TIF Expert, Prof. Constantina Politis (member of TIF's International Advisory Board and expert in Blood Transfusion) who delivered a presentation entitled 'Safeguarding Blood Safety for Patients with Thalassaemia' in the plenary session and TIF collaborator Dr Georgios Kaltsounis presented a poster abstract. The Congress gathered approximately 8,000 participants.

² Borgna-Pignatti C & Gamberini MR, 2011. Complications of thalassemia major and their treatment. *Expert Rev Hematol.* Jun;4(3):353-66. doi: 10.1586/ehm.11.29. Accessed: 19 Nov 2019.

³ <https://www.ncbi.nlm.nih.gov/books/NBK173969/>

⁴ <https://thalassaemia.org.cy/positions-policies/position-papers/>

RESULTS ACHIEVED

- Increased awareness about thalassaemia amongst members of the scientific community.
- Partnerships (cf. Working Group on Haemoglobinopathies at ISBT and Topics-in-Focus of the EHA) were strengthened through participation in the Annual Congresses.
- 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 <i>Are the "general objective" and "specific objectives" still meaningful?</i>	Effectiveness <i>Has the objective been achieved? How much contribution did the "outputs" make?</i>	Efficiency <i>To what extent have "inputs" been converted to "outputs"?</i>	Impact <i>What positive or negative, direct or indirect effects have happened?</i>	Sustainability <i>To what extent will TIF be able to maintain the positive results of its activities?</i>
Tasks					
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 3 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.

