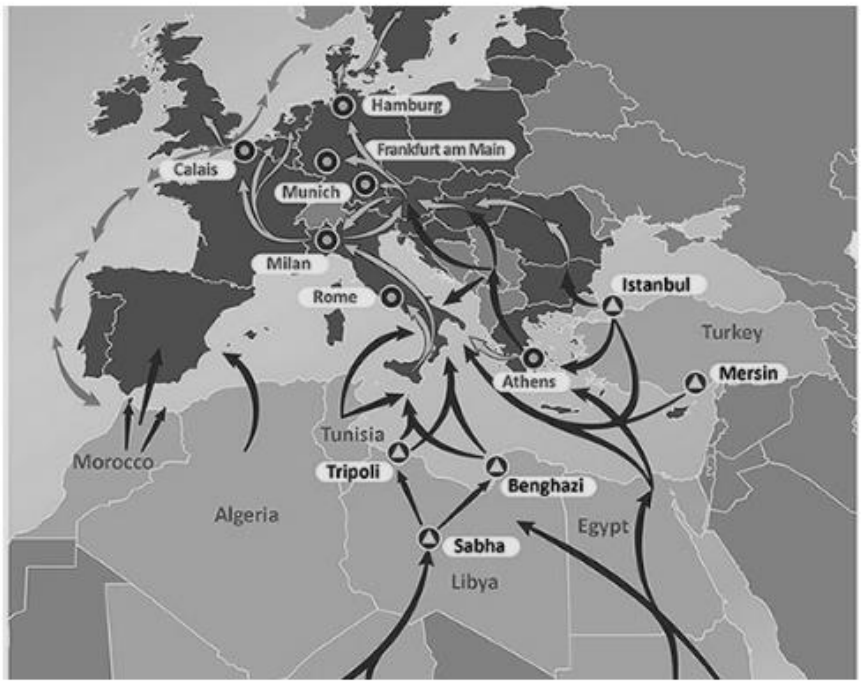

PROGRESS OUTLINE 2019

PILLAR 3: POLICY ADVOCACY

Activities

Impact

THALIA

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TABLE OF CONTENTS

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. For TIF, policy advocacy is all about the rights, choices and capacities of patients and involves delivering messages that are intended to influence thoughts, perspectives and actions of leaders, politicians, policy makers, planners and others in authority. Aiming to prioritise haemoglobin disorders and their control (prevention and management) at European and international levels, TIF acts directly to increase people's access to information about thalassaemia and other haemoglobinopathies and strives to enhance patients' capabilities to influence policy processes that affect their lives.

In 2019, the intensive efforts put forward in 2018 that yielded a thorough needs analysis of THALIA priority countries, namely France, Germany, Austria, Sweden and Serbia was further strengthened through delegation visits to each of the aforementioned countries (except Serbia where an opportune time for a visit was not reached) (T4.1.). The Delegation visits consolidated partnerships and collaborations in addition to enabled a deeper penetration in making new and more stakeholders aware of thalassaemia across all THALIA target groups: patients (especially migrants and refugees with thalassaemia), healthcare professionals treating thalassaemia and sickle cell disease patients and decision-making bodies. The empowerment of patients from Germany that have limited expertise in policy advocacy by patients from Greece who have a long history of participating in the making of ground-breaking policies on thalassaemia prevention and management was achieved through the THALIA Twinning Programme (T4.2). Moreover, to support evidence-based policy-making, TIF continued the development of an EU Electronic Health Record for thalassaemia and haemoglobinopathies completing the pilot, making necessary adjustments and initiating discussions with 2 EU-based centres for installing the software (T4.3).

Increased awareness about the impact of migration and thalassaemia on public healthcare infrastructures in Europe was achieved through the development of correspondence with Ministries of Health and Diplomatic Missions across Europe for the purposes of the High Level Round Table Discussion on Thalassaemia in the EU (T4.6) despite the fact that procedural delays led to its postponement for 2020.

TIF's activities in Work Package 4 have capitalised on previous efforts, and have ensured a continued interest of an increased number of EU-based stakeholders in thalassaemia and the need for developing specific policies for this increasing public health issue.

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

III.0. Prioritise haemoglobin disorders and their control (prevention and management) at international/United Nations (UN) and European Union (EU) levels

Task No	T4.1	T4.2	T4.3	T4.6
Target Audience	Patients Policy/ decision makers HCPs	Patients	Policy/ decision makers HCPs	Policy/ decision makers
KPI Met	✓	✓	×	×
Justification	✓	✓	✓	✓

Specific objectives

III.1. Facilitate the exchange of knowledge and good practices between national Thalassaemia associations.

Activity: T4.2: TIF EU Thalassaemia Associations' Twinning Programme

Results: Patients from Germany and Greece met for a face-to-face capacity-building / sharing-of-best-practices meeting

Impact: A core group of thalassaemia patients was formed, willing to empower peers.

Impact Management / Mitigation Measures: N/A

III.2. Support European countries with no tradition or experience in managing Thalassaemia and haemoglobinopathies and receive a high number of migrants and refugees.

Activity: T4.1: Establishment of national associations in France, Germany, Austria, Sweden and Serbia

Results: TIF visited all countries of priority (France, Germany, Austria, Sweden) except Serbia and consolidated relationships and partnerships created in 2018.

Impact: Steps were made for the establishment of thalassaemia patient groups all countries, as grass roots work increased the interest of all target audiences. .

Impact Management / Mitigation Measures: N/A

III.3. Monitor the spread of Thalassaemia and haemoglobinopathies in Europe.

Activity: T.4.3: Thalassaemia EU Electronic Health Record

Results: The infrastructure of this decision-making support tool was piloted to 6 thalassaemia centres and feedback was adopted and implemented prior to deployment.

Impact: Clinics in Europe expressed interest in participating in the 2020 installation, given the unavailability of such a tool in their respective countries. This validates the need to complete the dissemination of the tool and to further promote it.

Impact Management / Mitigation Measures: The interest of clinics will be utilised in 2020 to further promote the tool. The higher the number of participating clinics, the more effective the tool will be in the hands of policymakers.

III.4. Improve EU policies on Thalassaemia

Activity: T.4.6: High-level round table discussion on Thalassaemia in the EU

Results: The high-level event was postponed due to the limited availability of Ministers of Health and high-level officials.

Impact: The preparatory activities have helped to raise awareness on the migration of haemoglobinopathies among policy makers at EU level.

Impact Management / Mitigation Measures: The event will take place in 2020 to allow for better response rate and attendance.

OVERVIEW OF ACTIVITIES (PILLAR 3 – WP 4)

TASK	T4.1: Establishment of national associations in France, Germany, Austria, Sweden and Serbia
RESPONSIBLE STAFF, SUPERVISING STAFF	***Responsible Staff:*** Medical Advisor, Dr Michael Angastiniotis (Leader) Policy Officer, Eleni Antoniou Communications Officer, Aikaterini Skari ***Supervising Staff:*** Executive Director, Dr Androulla Eleftheriou
INDICATORS	▪ Number of delegation visits: 5 ▪ Memorandum of Understanding between patient groups in Germany: 1
CALENDAR OF ACTIVITIES	▪ Delegation visit to France: February & May 2019 ▪ Delegation visit to Sweden: March 2019 ▪ Delegation visit to Austria: April 2019 ▪ Delegation visit to Germany: July 2019 ▪ Establishment of thalassaemia collaboration network in Germany (MS13): October 2019

DESCRIPTION OF THE ACTIVITY

A main constituent of TIF's work globally and at the European level is provide support to local patient and healthcare communities and governments to create robust policy-making for the improvement of patient care. In this context a delegation team from TIF, comprising of medical specialists and patient advocates, travels to various countries every year to offer technical knowledge and experience on key issues touching on thalassaemia prevention and care.

These delegation visits serve to establish partnerships with the local communities on the ground, especially in countries where thalassaemia is not found within the indigenous population but rather the result of migration flows from thalassaemia-prevalent areas of the world such as the THALIA priority countries (i.e. France, Germany, Sweden, Austria and Serbia).

The TIF delegation team, in collaboration and with the consent of the local medical community and patients association (where one exists), observe a situation analysis through the documentation of (1) epidemiological data (taking into consideration the influence of migration and population movements), (2) the provided medical and other services and (3) unmet patient needs. Together with all stakeholders immediate and long-term actions are considered for implementation to advocate for improvement of these. Where an organised patient association does not exist TIF makes every effort to identify

patients and parents who can provide feedback on their unmet needs, and proposes the establishment of a patient association as a priority activity, and provides expert assistance on how to proceed (TIF Publication - 'A Guide to Establishing a non-profit patient support organisation'[1]). Where an association exists then TIF consider ways to strengthen it, providing patient education, and organising capacity building activities.

The foundations for collaboration across the THALIA priority countries was laid in 2018 where two delegation visits (one investigational and one follow up) took place, with a TIF delegation team comprised of an expert TIF Officer or Medical Adviser or Member of TIF's International Scientific Advisory Board and at least one patient from TIF's Expert Group. During the 1st visit, TIF delegation meets with medical specialists, national health authorities and national rare disease association (if exists) to map the situation and tailor the support according and secure the supporting of medical professionals in identifying patients for TIF. During the 2nd visit, TIF delegation meets with patients and parents (where necessary) to ensure support for the establishment of patients' associations with specific goals and timeframes.

Thus, in 2019, aiming primarily to strengthen the relationships and partnerships created through the previous delegation visits, as well as to document progress on mutually agreed upon immediate and long-term actions, TIF revisited each priority country (with the exception of Serbia).

Objectives of each Visit

The objectives of these delegation visits, in accordance to the three main stakeholder groups involved in the control of thalassaemia, are:

Stakeholder Group 1: Healthcare professionals

- To inform healthcare professionals working in the field of haematology and paediatric haematology about the migration of thalassaemia (how the recent migration flows have led thalassaemia patients and carriers to Europe);
- To ascertain the epidemiology and prevalence of thalassaemia
- To discuss response mechanisms / programmes, including multidisciplinary care and prevention programmes;
- To seek guidance and support on how disseminate knowledge about thalassaemia to the other stakeholder groups (see below) as well as the general public (for community awareness and prevention purposes);
- To involve healthcare professionals in TIF's International Scientific Advisory Committee and;
- To provide information about TIF's tools for this stakeholder group, and how they can become involved (e.g. Healthcare Professionals Educational Platform, Publications – especially Guidelines for the Clinical Management of TDT, Global e-Registry & electronic health record, Preceptorships, TIF Digital Library, Renzo Galanello Fellowship etc).

Stakeholder Group 2: Patients/Parents

- To empower and create core groups of patients, promoting the creation of a National Patients' Associations, for providing support (in kind and knowledge-based) to the patients and their families;
- To provide tools and education for the development of the necessary skills and capacities for advocacy and productive participation in decision making at the national level;

- To educate patients/parents on disease-specific knowledge, as well as the latest policies, projects and legislative developments in the fields of Haemoglobin disorders, rare diseases, medicines, patients' rights and mobility, reference networks, etc;
- To invite patients/parents to participate in specific TIF organized activities e.g. Train-the-Trainers Capacity Building Workshop, Twinning Programmes etc;
- To provide information about TIF's tools for this stakeholder group, and how they can become involved (e.g. Thal e-Course, TIF Mobile app, Publications, Thalassaemia Patients Connect etc).

Stakeholder Group 3: Health Authorities

- To discuss the policies relevant to the prevention and clinical management of haemoglobin disorders;
- To demonstrate the relation between migration and thalassaemia and possible implications for the healthcare system;
- To deliberate regarding possible avenues of collaboration with TIF to increase and ensure access to optimum care[2] for all patients (e.g. Reference Centres, life-long learning opportunities for healthcare professionals, Global e-Registry & electronic health record etc.)

The visits in 2019 have yielded the following notable information (per country):

Delegation visit to France

February 2019

Introduction

A TIF Delegation visit to Paris, France took place on 01 February 2019. The Delegation Team consisted of Lily Cannon (TIF Operations Manager) and Eleni Antoniou (Policy Officer).

The Delegation Team had the opportunity to participate in the 7th National Meeting of the MCGRE Network, and to meet with physicians working in the field of the thalassaemia in France.

During the course of the Meeting, TIF had the opportunity to present an overview of its work to the gathered audience (doctors, healthcare professionals and patients). The presentation, jointly delivered by the TIF Delegates was translated into French to facilitate the understanding of the audience. In addition, the Delegation held a follow-up meeting with the thalassaemia patients who participated in TIF's Twinning Programme (Dec. 2018; France/Cyprus) as well as with the leadership of SOS Globi (Federation of Haemoglobin Disorders Patient Associations), to discuss the further strengthening and empowerment of the thalassaemia patients' community in France. In addition, the Delegation had the opportunity to meet and discuss issues of concern with clinicians and other healthcare professionals regarding specific matters pertaining to the management of thalassaemia and policy/service planning tools.

[1] https://thalassaemia.org.cy/publications/tif-publications/a-guide-to-establishing-a-non-profit-patient-support-organisation-2007-eleftheriou-a/

1. [2] As per TIF's Guidelines (3rd Edition), 2014 http://thalassaemia.org.cy/publications/tif-publications/guidelines-for-the-management-of-transfusion-dependent-thalassaemia-3rd-edition-2014/

Main Findings:

- Thalassaemia in France is a rare disease, with patients scattered across the 63 different cities. The French Thalassaemia Registry is a vital tool for reaching these patients.
- The rarity of the disorder leads many physicians to having limited experience in the management of thalassaemia, and others with accumulated knowledge.
- The establishment of a Thalassaemia Working Group within SOS Globi which would serve to 'organize' the thalassaemia community – a first activity will be the organisation of a patients' conference on the occasion of International Thalassaemia Day 2019

May 2019

Introduction

A TIF Delegation visit to Marseille, France took place on 03 – 05 May 2019. The Delegation Team consisted of Mr. George Constantinou (Assistant Secretary of the TIF Board of Directors and Expert Patient), and Dr Maria Sitarou (member of TIF' International Scientific Advisory Board).

The Delegation Team participated in the 1st National Thalassaemia Conference. The Conference, the first of its kind was attended by over 60 thalassaemia patients and parents.

The TIF Delegation had the opportunity to meet with thalassaemia patients to discuss the further strengthening and empowerment of the thalassaemia patients' community in France. In addition, the Delegation had the opportunity to meet and discuss issues of concern with clinicians and other healthcare professionals regarding specific matters pertaining to the management of thalassaemia and policy/service planning tools, showcasing the Cyprus National Thalassaemia Control Programme (prevention and management). To overcome linguistic barriers, a leaflet of the educational opportunities and services which TIF provides in French was distributed.

Main Findings:

i. The lack of peer-to-peer support is a major issue for patients and their families leading to social isolation. Meeting other patients in the context of the Conference has made them feel part of a community.
ii. Linguistic barriers were highlighted throughout, with immense gratitude to TIF representatives for acknowledging the confined knowledge of the English language and making efforts to communicate in French.
iii. Educational material should be developed in French to overcome any linguistic barriers and provide wider access to information amongst French-speaking physicians and patients/parents.
iv. Strengthening of SOS Globi infrastructure, especially the Thalassaemia Working Group is necessary in order to promote more tangible and impactful activities for thalassaemia.

Delegation visit to Sweden

March 2019

Introduction

A TIF Delegation visit to Sweden took place on 23 – 25 March 2019. The Delegation Team consisted of Dr Androulla Eleftheriou (TIF Executive Director), and Dr Michael Angastiniotis (TIF Medical Advisor).

The Delegation Team had the opportunity to meet with representatives of the following:

- Dept. of Hematology Uppsala University Hospital
- Capio Saint Göran's Hospital
- Swedish Blood and Cancer Association NGO
- Patients & Parents

The meetings further explored the quality of life of patients with haemoglobinopathies in Sweden, and identified avenues of attaining the support of and closer partnership with TIF. The consensus of all stakeholders focused on the lack of prioritisation by the National Health Authorities to haemoglobin disorders (thalassaemia and sickle cell disease) both in terms of their multidisciplinary management but also concerning the societal integration of patients. Subsequent actions have been taken to sensitize the Swedish Ministry of Health.

Main Findings:

i. The unavailability of a complete national registry for thalassaemia hinders the ability of health authorities to make appropriate and targeted service planning.
ii. There is adequacy of blood and safety regulations are adhered to. There are mobile thalassaemia units which contribute to sufficiency and safety.
iii. Blood transfusion times often interfere with professional and educational commitments as clinics do not work in the afternoons or evenings.
iv. Iron load monitoring requires standardisation for appropriate interpretation of results.
v. Transition from paediatric to adult care is poorly defined.
vi. Further strengthening of efforts for the creation of a thalassaemia patient community / organization is required to combat isolation and marginalisation.
Few patients / parents are becoming empowered to take the next steps towards this direction.

Delegation visit to Austria

April 2019

Introduction

A TIF Delegation visit to Vienna, Austria took place on 11 – 13 April 2019. The Delegation Team consisted of Mr. George Constantinou (Assistant Secretary of the TIF Board of

Directors and Expert Patient), and Dr. Anne Yardumian (member of TIF' International Scientific Advisory Board). The Delegation was accompanied by Dr Eva-Maria Knoll (a passionate volunteer and Medical Anthropologist working with TIF).

The Delegation Team had the opportunity to meet with representatives of the following:

- Hematology & Oncology Department of Saint Anna's Children's Hospital
- Hematology & Hemostaseology Department of Vienna General Hospital
- Department of Haematology and Oncology, Hanusch Hospital
- Labors Medical Lab & Diagnostic Services

Main Findings:

i. Treatment is sufficient however, patients with thalassaemia presented in some cases limited adherence to blood transfusions and iron chelation treatment.
ii. There is a necessity for strengthening of genetic counselling and psychosocial support provided to patients is necessary.
iii. The promotion of a national registry with epidemiological and clinical data on the thalassaemia population is required for appropriate service planning.
iv. language barriers were prominent as well, making communication between doctors and patients rather intricate.
v. Patients stated to often feel isolated and marginalized, due to the lack of patient support groups. Hence, the successful establishment of the Thalassaemia & Sickle Cell Forum (THALSIFO) Austria, as a result of the delegation visits in 2018 and 2019 is a huge milestone.

The first meeting of THALSIFO took place on 17 May 2019, with 16 attendees (patients, parents and family members of thalassaemia and sickle cell disease patients). It is noted that this is the first meeting of this kind to take place in Austria of the haemoglobinopathy community. Not a single of the participating patients had contacts with other patients before this meeting!

The organisation of this meeting and Forum has gone a long way to combat this marginalisation and has contributed to the exchange of experiences with peers. The meeting also has demonstrated the heterogeneity of Austrian patients, both in terms of ethnicity and cultural background, but also linguistically (the majority of attendees spoke only German, 5 spoke only English, a few spoke English and German).

Delegation visit to Germany

July 2019

Introduction

A TIF Delegation visit to Ulm & Düsseldorf took place on 04 – 05 July 2019. The Delegation Team consisted of Mr. George Constantinou (Assistant Secretary of the TIF Board of Directors and Expert Patient), and Dr Eva-Maria Knoll (a passionate volunteer and Medical

Anthropologist working with TIF). The Team was accompanied by Mr. Zabihullah Safai (Board Member of SAM Deutschland e.V. AM - TIF Member Association).

The Delegation visited Dusseldorf and Ulm for an exploratory visit, meeting with patients and healthcare professionals. In 2018, TIF had visited Hamburg where a large number of patients live, and has focused in 2019 on strengthening its partnership with SAM Deutschland e.V. AM, The visit to Dusseldorf and Ulm aimed to strengthen TIF's presence throughout Germany, building relationships, identifying challenges and opportunities.

The Delegation Team had the opportunity to meet with representatives of the following:

- Paediatric Clinic for Oncology, Haematology and Clinical Immunology, University Hospital of the Heinrich Heine University Duesseldorf
- IST (Interessensgemeinschaft Sichelzellkrankheit und Thalassämie e.V) – Patient Group
- Clinic of Paediatric and Adolescent Medicine Universitätsklinikum Ulm

In addition, meetings were held with individual patients who run WhatsApp Group's either nationally or locally (Düsseldorf).

During the meetings, the TIF Delegation Team had the opportunity (1) to present the work of the Federation, sharing best practice examples from across the world, and possible applications for each stakeholder, (2) to discuss the epidemiology of thalassaemia in the country, healthcare service structure and provisions to patients as well as the socioeconomic challenges witnessed, (3) to talk about the challenges that patients face in their everyday lives (including social, occupational and education development) and their quality of life, and (4) to consider the impact of the recent migration influx and the consequent additional pressure on the national healthcare systems and its provision of quality healthcare services. Furthermore, possible avenues of collaboration were discussed with each stakeholder party.

<u>Main Findings:</u>

- Thalassaemia in Germany is a rare disease, with patients scattered across the country, leading to social isolation and marginalisation.
- Awareness and knowledge about thalassaemia amongst healthcare professionals, patients, carriers, the migrant communities and the society at large is limited. Healthcare professionals working in this field are often transferred to other centres, departments, thus making continuity of care and interest difficult.
- The unavailability of a complete national registry for thalassaemia hinders the ability of health authorities to make appropriate and targeted service planning. The establishment of a German network of associations would be a powerful vehicle for exchange of knowledge, experiences, promoting advocacy and alleviating isolation and marginalisation.
- Empowerment of patient associations through knowledge (e.g. Thal e-course, workshops, publications in Germany language) and advocacy tools (e.g. registry) is essential.

▪ There is interest for building of a thalassaemia community, but due to lack of knowledge and advocacy capacities, the ground for moving forward with a national network is still not ideal.	
RESULTS ACHIEVED	▪ Identification of healthcare professionals and patients with thalassaemia in each of the THALIA priority countries (FR, DE, SWE, & AUS). ▪ Strengthening of established and building new collaborations with key stakeholders in the medical and patient (where they exist) communities, in addition to other health bodies in each of the THALIA priority countries (FR, DE, SWE, & AUS). ▪ Initiating the development of an updated epidemiological mapping of the situation regarding thalassaemia in Europe. ▪ Empowerment of patients and associations (where they exist) with provision of patient educational material. ▪ Recognition of the social challenges that patients with thalassaemia in Europe face (isolation and marginalisation) in excess of other discriminations. ▪ Acknowledgement of 'invisible' patients who are not part of the healthcare systems and the necessity to work with agencies dealing with migrants and refugees in order to find them. ▪ Dissemination of TIF's educational materials for patients and healthcare professionals as well as other initiatives (e.g. electronic healthcare record, registry, network of collaborating centres etc) to each of the THALIA priority countries (FR, DE, SWE, & AUS).

TASK	**T4.2: TIF EU Thalassaemia Associations' Twinning Programme**
RESPONSIBLE STAFF, SUPERVISING STAFF	***Responsible Staff:*** Policy Officer, Eleni Antoniou (Leader) Medical Advisor, Dr Michael Angastiniotis Communications Officer, Aikaterini Skari ***Supervising Staff:*** Executive Director, Dr Androulla Eleftheriou
INDICATORS	▪ Number of participants: 5 ▪ Follow up actions: 3 actions within 6 months ▪ Outreach of actions: 600 patients & 1,000 members of the wider community
CALENDAR OF ACTIVITIES	▪ Identification of participants (MS 14): March 2019 ▪ Twinning Programme (Germany – Greece): 11 – 14 October 2019

DESCRIPTION OF THE ACTIVITY

To facilitate the exchange of knowledge and good practices between patients and associations throughout Europe, TIF has established a Twinning Programme between countries with newly established / inexperienced patient associations (where they exist) or active patients and expert patient associations.

TIF's EU Twinning Programme in 2018 served the needs of the patient community in France (THALIA2018 priority country, see FPA No. 785243), and as a result a Thalassaemia Working Party was set up in France (See T4.1).

In 2019, the Twinning Programme focused on the building of a relationship between the patient communities of Germany (THALIA2019 priority country, see FPA No. 785243) and Greece.

To this end, and taking into consideration the specific situation of the German thalassaemia patient community (several associations scattered across the country, individual patients with social media groups, leading to disjointed and localised actions rather than national coordinated efforts), the Twinning Programme took place on 11 – 14 October 2019 in Hamburg, Germany. The date and location coincided with the Capacity Building Workshop (see WP2) in order to maximise the use of resources and to take advantage of the fact that patients from all over Germany were gathered in one location.

The Twinning Programme was participated by 20 thalassaemia patients from Germany (some belonged to one of the four patient association in existence whereas others did not) and 4 thalassaemia patients representing the Greek Thalassaemia Association and Greek Thalassaemia Federation. In addition, 1 thalassaemia patient representing the Cyprus Thalassaemia Association also participated, sharing knowledge and experience from last years' programme.

The Greek Thalassaemia Federation was selected as an appropriate 'role model' for Germany as it hosts 25 local associations, many of which have been amongst the first thalassaemia patient associations created in 1970s and 1980s. The Federation, formally established in 1991, hash a long and vast array of actions and achievements at the national and international levels including (1) the establishment and monitoring of 36 designated thalassaemia centres across Greece, (2) strong advocacy presence leading to retaining of the provision of free-of-charge treatment and laboratory tests even in times of economic crises, (3) achieving an array of state benefits to facilitate social, educational and professional integration, (4) active educational role with events for patients/parents and healthcare professionals on an annual basis as well as raising awareness about thalassaemia in the community thus contributing to the national prevention programme, (5) founding member of the Thalassaemia International Federation with continuous involvement in global activities.

The Twinning Programme focused on the following topics: ▪ Social integration and community building ▪ The role of a patient association ▪ Effective advocacy methodology ▪ How to gain respectability and reliability ▪ Communication Methods ▪ Relationship building with healthcare professionals ▪ The importance of obtaining disease-specific knowledge ▪ Development of patient education materials	
RESULTS ACHIEVED	▪ Establishment of a partnership between patients in Germany and Greece, with the latter providing assistance and guidance to the former in any area required. ▪ Increase of knowledge amongst German patients about patient associations and social implications and importance. ▪ Dissemination of information and tacit knowledge to their German counterparts by the Greek patient community.

TASK	**T4.3: Thalassaemia EU Electronic Health Record**
RESPONSIBLE STAFF, SUPERVISING STAFF	***Responsible Staff:*** Policy Officer, Eleni Antoniou (Leader) Medical Advisor, Dr Michael Angastiniotis Communications Officer, Aikaterini Skari ***Supervising Staff:*** Executive Director, Dr Androulla Eleftheriou
INDICATORS	▪ Data collection from 3 centres (MS15)
CALENDAR OF ACTIVITIES	▪ Completion of architectural development: January 2019 ▪ Receipt of feedback: February – August 2019 ▪ Interest from centres (MS15): April (Greece) & August (Cyprus)
DESCRIPTION OF THE ACTIVITY	
The development of an EU-wide comprehensive disease-specific database in the form of a thalassaemia-specific electronic health record, which provides data for an EU Thalassaemia Registry, is a pivotal tool for monitoring the spread of thalassaemia and haemoglobinopathies in Europe. This is especially important in the current climate, considering the influx of migrants from thalassaemia prevalent areas of the world to European countries where thalassaemia is not found in the indigenous population and hence there is limited expertise on the management of the disease or understanding of its medical, social and economic repercussions.	

The need for complex healthcare services for lifelong disorders like thalassaemia, make it essential to understand the real burden of disease; this is an important issue for budgetary and public health planning. Registries, health records and other databases are essential tools for gathering information, which help to define the epidemiology, clinical outcomes and the natural history of these rare conditions. Such information will help to improve quality of care and to plan services, as well as to assist in research initiatives including clinical trials and the recruitment of volunteer patients. Policies concerning reference centres, networking and cross-border health, make the development of registries at healthcare facility, national and international level, necessary tools to facilitate the creation and implementation of these policies.

The current lack of reliable and comprehensive national registries for thalassaemia and haemoglobinopathies suggests a gross underestimation of the prevalence of these disorders in Europe. In addition, knowledge and understanding of the distribution of patients and prevalence of the disease in each country is a huge contributing factor to public health planning. Thus, in the effort to assist the development of targeted public health policies for the provision of quality healthcare for thalassaemia patients, TIF has in 2018 developed an electronic health record for thalassaemia, with due consideration of EU GDPR[3] legislation in regards to ethical and privacy issues.

In 2019, treating physicians from 6 thalassaemia centres in Greece and Cyprus were given the opportunity to pilot the Thalassaemia Electronic Health Record and review the fields for the extrapolated Registry and Patient Summary features (M2 – M8).

Feedback received from these centres, either in face-to-face meetings or via correspondence, were used to evaluate and update the architecture of the software to be ready for data collection (M8 – M11).

Moreover, 2 centres have officially declared their interest (MS15) in adopting the Thalassaemia Electronic Health Record, and procedures are on-going to reach agreements with hospital administrations regarding the installation of the software to ensure safety of data and server security. Moreover, official interest is expected before the end of the year by additional 3 centres.

Data collection is expected to begin in early 2020.

RESULTS ACHIEVED	▪ Piloting of the Thalassaemia Electronic Health Record has been completed. ▪ Official interest from 2 centres in Greece and Cyprus received.

[3] General Data Protection Regulation (https://eur-lex.europa.eu/legal-content/EN/TXT/HTML/?uri=CELEX:32016R0679&from=EN)

TASK	T4.6: High-level round table discussion on Thalassaemia in the EU
RESPONSIBLE STAFF, SUPERVISING STAFF	***Responsible Staff:*** Policy Officer, Eleni Antoniou (Leader) Medical Advisor, Dr Michael Angastiniotis Communications Officer, Aikaterini Skari ***Supervising Staff:*** Executive Director, Dr Androulla Eleftheriou
INDICATORS	▪ Organisation of 1 event in March 2019 ▪ Number of participants: 30 ▪ Number of Members States, Organisations etc represented: 12
CALENDAR OF ACTIVITIES	▪ Invitations sent to panellists (MS16): July 2019 ▪ Invitations sent to participants: August 2019 ▪ Venue & Equipment secured: July 2019 ▪ Development of material - THALIA poster: August 2019

DESCRIPTION OF THE ACTIVITY

The THALIA2019 Forum is the first informal forum of dialogue aimed at providing EU Member States with the opportunity to informally exchange views and experiences on addressing the multifaceted problem of the migration of thalassaemia. Traditionally an endemic disease of the Mediterranean region, thalassaemia was first addressed in Cyprus, Greece, Italy and the United Kingdom through the establishment of national control programmes. The THALIA2019 Forum would thus provide participants with the opportunity to learn from each other and touch upon a much neglected topic that consists an emerging public health: the rise of thalassaemia in all EU countries.

Given that not only affected patients are increasing but also healthy carriers and that these disorders follow a Medelian inheritance pattern (i.e. from parents to children), the phenomenon is expected to greatly affect the future birth incidence of these conditions in Europe. This calls for long term policy considerations and has urged TIF to create a platform of dialogue for EU Member States, as there are no formal prevention policies in place in most countries, while specialised clinical care is only provided in selected centres. Also, there is a general absence of national registries that would allow for better monitoring, planning and resource management.

To ensure the participation of representatives of Ministries of Health and other high-level decision makers, the TIF Board of Directors decided in early 2019 that the most appropriate venue for the organisation of a high-level round table discussion on Thalassaemia in the EU would be Brussels, in parallel to the EU Health Programme High Level Conference (30 September 2019). This would allow high-level officials of European
Ministries of Health to attend both events. Between February and May 2019, the support of the Republic of Cyprus was sought and eventually secured to ease communication with the Ministries of Health of EU Member States. A number of face-to-face meetings was

held with the ambassadors of Cyprus in Brussels and Geneva and finally with the Minister of Foreign Affairs, H.E. Mr. Nikos Christodoulides on 31 May 2019. In August 2019, the THALIA poster was designed and printed to be used in both the EU Health Programme High Level Conference and the THALIA2019 Forum, as a key means to raise the visibility of THALIA and awareness on the migration of thalassaemia.

Subsequently, the venue and equipment were selected in June 2019 and secured in July 2019, the preliminary agenda was prepared and invitations to speakers and participants were sent in July and early August 2019, respectively. Despite all follow-up efforts, the availability of Ministers or their representatives was disappointingly low, as only Croatia, Cyprus, Greece and Spain confirmed attendance. Other countries were either not available (Germany, Ireland, Belgium, Hungary, Estonia, Slovenia) or never responded.

This led the Board of Directors to decide on the postponement of the event to allow for better planning and coordination on behalf of both the organisers and invitees. Moreover, following the RSVP efforts, a letter was sent to the President of the Republic of Cyprus with the request to sign a Memorandum of Understanding with the Federation to make collaboration official and strategic. No reply has been received to date.

RESULTS ACHIEVED	Despite the event's postponement, TIF has managed to: 1) Raise awareness on the migration of haemoglobinopathies and attract the interest of certain Ministries of Health; 2) Come in touch with diplomatic missions in Cyprus and Brussels and inform them about the phenomenon and the community's concerns; 2) Pave the ground for the organisation of a successful event in 2020.

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 Tasks	**Relevance** *Are the "general objective" and "specific objectives" still meaningful?*	**Effectiveness** *Has the objective been achieved? How much contribution did the "outputs" make?*	**Efficiency** To what extent have "inputs" been converted to "outputs"?	**Impact** *What positive or negative, direct or indirect effects have happened?*	**Sustainability** *To what extent will TIF be able to maintain the positive results of its activities?*
T.4.1: Establishment of national associations in France, Germany, Austria, Sweden and Serbia	Establishing national associations or networks of thalassaemia patients is key to support EU countries with no tradition in the prevention and management of thalassaemia.	Follow up delegation visits to the priority countries (FR, DE, AUS & SWE) allowed TIF to build on partnerships with local thalassaemia-related stakeholders created in 2018, and to further enrich TIF's knowledge on the wide and deep implications of thalassaemia in the migrant communities of these countries. This enabled TIF to further encourage patients to join forces and take action to safeguard of their right to health and quality of life in addition to creating avenues for their psychological support and breaking social isolation and marginalisation. Such penetration was not possible in Serbia, where patients seem to be few and unknown. The interest of the Serbian Ministry of Health in	The "inputs" required for the implementation of this task were optimally used, namely: staff time, planning time, volunteer hours, knowledge base, technology, partners, contacts.	THALIA2019 allowed patients to come together, develop synergies with doctors and participate in disease-specific policy dialogue. The very presence of TIF in all countries reactivated local communities and showed the way forward to more inclusive societies.	To maintain results and the momentum created, follow-up visits in each country were added in the 2020 work programme and regular communication with all stakeholders and local contacts was established. More targeted efforts in collaboration with local health authorities are needed to achieve greater results in Serbia.

		haemoglobinopathies needs to be utilised.			
T4.2: TIF EU Thalassaemia Associations' Twinning Programme	This task is directly related to the general objective and serves to facilitate the exchange of best practices between national thalassaemia associations. The Twinning Programme is a promising tool to bring patients of Europe together and bridge the knowledge gaps between associations.	The specific objective was achieved as German patients were inspired to reactivate their associations and to work more harmoniously by the best practices of their peers from Greece.	The "inputs" required for the implementation of this task were optimally used, namely: staff time, planning time, financial means, knowledge base, technology, partners.	Through the Twinning Programme, TIF was able to focus on the specific needs of patients living in Germany and suggest tailored solutions to address them. The patients who participated in the programme showed interest in furthering their efforts for raising awareness about thalassaemia in their local communities, as a first step.	Patients were invited to participate in all 2020 workshops organised by TIF. Associations were encouraged to maintain communication and also join TIF's Thalassaemia Patient Connect platform in addition to completing the Thal e-course, thus ensuring their access to quality information.
T4.3: Thalassaemia EU Electronic Health Record	This tool is essential for evidence-based policy making and is considered to be the best way to monitor the spread of thalassaemia and haemoglobinopathies in Europe.	The Electronic Health Record underwent rigorous piloting with plenty of feedback from users. Moreover, official interest in adopting the Electronic Health Record has been received.	The "inputs" required for the implementation of this task were used as best as possible, namely: staff time, planning time, volunteer hours, knowledge base, technology, partners. The feedback received from the pilot phase pushed timelines back due to the time required to receive feedback and time for its implementation.	A number of clinics from Greece and Cyprus have expressed their official interest to installing the tool once development is finalized. Moreover, clinics in Sweden, Austria and Germany have also expressed interest in utilising the tool.	TIF will be sharing news on the tool's development progress to increase visibility and invite more clinics to join in.

T4.6: High-level round table discussion on Thalassaemia in the EU	This task serves the need to further inform policymakers about migrant populations with thalassaemia, especially those from Syria.	This activity was partially implemented due to the limited availability of invitees and the low response rate. The corresponding awareness raising efforts (e.g. participation in the EU Health Programme Conference) were highly successful.	The "inputs" required for the implementation of this task were optimally used, namely: staff time, planning time, volunteer hours, knowledge base, technology, partners.	Increased interest of the online community and of certain Ministries of Health on public health issues pertaining to the migration of thalassaemia. The publication of a joint statement is expected to have more substantial impact on the work of the Member States' health authorities.	More intensive and systematic efforts will need to be made in early 2020 to ensure that all Member States are aware of the epidemiological shift that threatens the national health systems.