NORDIC HAEMOGLOBINOPATHY FORUM YEARBOOK

THALIA

Co-funded by the European Union

THALassaemia In Action 2024 | Grant Agreement No 101176329
WP3/ T3.1 | Deliverable 3.1 (D8) | December 2024

YEARLY HANDBOOK
D3.1 / D8

Co-funded by the European Union

***WP3 / T3.1:* Stakeholder Network Development**

Disclaimer: This publication is funded by the European Union (Grant Agreement No 101176329). Views and opinions expressed are however those of the author(s) only and do not reflect those of the European Union or HaDEA. Neither the European Union nor the granting authority can be held responsible for them.

TABLE OF CONTENTS

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 243 members from 67 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 2024" (THALIA2024) focuses on Europe, targeting patients with thalassaemia and other haemoglobinopathies, healthcare professionals, policymakers and the general public to:

a) Reduce the burden of non-communicable diseases by reducing health inequalities and promoting access to healthcare
b) Strengthen health systems to improve their responsiveness in times of health emergencies
c) Improve the availability, accessibility and affordability of medicinal products and devices, including innovative therapies
d) Promote the implementation of best practices through the utilisation of digital transformation means.

EXECUTIVE SUMMARY

This Yearly Handbook details TIF's initiatives in awareness-raising and network-building, focusing on the development of the Nordic Haemoglobinopathy Forum (NHF), established under the THALIA 2022 work programme. This Forum, consisting of treating physicians from reference and expert centers across the Nordic countries, has identified several shared challenges, including knowledge gaps, a lack of standardized care, and limited networking opportunities. To address these issues, TIF organized one in-person meeting and three virtual meetings (T3.1), aiming to enhance healthcare services in the region and support the co-creation of policies and action plans. These interactions were highly productive, resulting in agreements for collaborative activities over the coming months and years, thus contributing to the long-term sustainability of these efforts.

The Yearly Handbook is available online at https://thalassaemia.org.cy/thalia2024/

MATCHING OBJECTIVES TO ACTIVITIES AND IMPACT

About Work Package 3 (Networking & Collaborations – Pillar II – Awareness Raising)

WP3 entails the further reinforcement of TIF's network of stakeholders (T3.1) and its participation in EU policy events (T3.2). The anticipated deliverable is a Yearbook outlining these efforts and their added-value for patients.

General objective

GO2: To raise awareness on thalassaemia and other haemoglobinopathies, with a specific focus on the importance of health and social care amongst the public at large, as well as amongst THALIA target audiences, at national and EU levels by expanding the Federation's Network and engaging in active dialogue with EU-based partners in the context of meetings, conferences and other events - Theme: Social Determinants of Health/Social Issues & Education

Specific objectives of T3.1

SO2.1. To activate TIF's EU network of stakeholders related to thalassaemia and other haemoglobinopathies and reinforce partnerships by organizing 4 meetings of the Nordic Haemoglobinopathy Forum and presenting the outcome in a dedicated Yearbook (T3.1)

***Activity**:* T3.1 Stakeholder Network Development
Results: TIF's Stakeholder Network Development concentrated on strengthening the Nordic Haemoglobinopathy Forum (NHF), a collaborative group of treating physicians from reference and expert centres located in 5 EU countries (v. Denmark, Sweden, Finland, Estonia and Latvia). Through three virtual meetings and one in-person meeting, the Forum has fostered new partnerships and reinforced existing ones.
Expected KPI: 170 participants in 1 in-person meeting (80 attendees) and 3 virtual meetings (30 attendees per meeting) from 5 EU countries.
Actual KPI: 177 participants in 1 in-person meeting (84 attendees) and 3 virtual meetings (93 attendees) from 5 EU countries.
Impact: KPI met
Impact Management / Mitigation Measures: N/A

ABOUT THIS YEARBOOK

A network is defined as “an arrangement of intersecting horizontal and vertical lines; a group or system of interconnected people or things”[1]. In this context, TIF applies thoughtful commitment and sustained effort to create, grow, support, and mobilize networks of medical professionals and patients to advance care, knowledge, and advocacy for haemoglobin disorders worldwide. By establishing regional haemoglobin disorder networks in areas where these disorders are either highly prevalent or considered rare (such as in the Nordic countries), TIF aims to create a robust platform connecting patient associations, healthcare professionals, researchers, and other key stakeholders. These networks are designed to enhance care and awareness, fostering collaboration and facilitating the exchange of expertise and resources to significantly improve the quality of care for individuals affected by these disorders.

Through its network expansion in Europe, the Thalassaemia International Federation (TIF) leverages a powerful tool to increase awareness of thalassaemia, advocate for optimal care, and enhance the visibility of its work as part of THALIA2024.

This Yearly Handbook analyses the development of TIF’s Stakeholder Network as part of WP3 – Networking and Collaborations, within the region-specific work programme of the Federation. It serves as both an internal reporting tool and a practical guide to assist patient organizations in expanding their own networks.

This deliverable follows the completion of Task 3.1 under WP3, which involved a series of meetings with healthcare professionals specializing in the prevention and clinical management of haemoglobinopathies in the Nordic region. Stakeholders were selected based on their expertise in treating patients with thalassaemia—a complex, multi-organ, rare, genetic, and hereditary disorder.

These meetings enabled TIF to reinforce its commitment to timely, high-quality patient care and to raise awareness of its work within the framework of THALIA2024.

[1] Oxford English Dictionary (2005 revised edition)

OVERVIEW OF ACTIVITY

TASK	T3.1: Stakeholder Network Development
RESPONSIBLE STAFF, SUPERVISING STAFF	***Responsible Staff:*** Senior Educational Programme Officer, Katia Pelides (Leader) Medical Advisor, Dr Michael Angastiniotis Deputy Director, Lily Cannon Senior Communications Officer, Aikaterini Skari National Liaison Officer, Rawad Merhi Communications Officer, Stella Eleftheriou ***Supervising Staff:*** Executive Director, Dr Androulla Eleftheriou
INDICATORS	▪ 80 participants at the annual NHF meeting (in-person) ▪ 30 participants at each virtual meeting of the NHF (3 virtual meetings)
CALENDAR OF ACTIVITIES	▪ 1st Meeting: 15 February 2024 (virtual) ▪ 2nd Meeting: 08 March 2024 (virtual) ▪ 3rd Meeting: 07 June 2024 (virtual) ▪ 4th Meeting: 13 September 2024 (in person)
DESCRIPTION OF THE ACTIVITY	

- **1st Meeting: 15 February 2024**

Meeting Format	Virtual
Summary	On February 15, 2024, Dr Androulla Eleftheriou, TIF's Executive Director, Dr Michael Angastiniotis, TIF's Medical Advisor and Ms. Katia Pelides, TIF's Senior Educational Programmes Officer, met with 32 medical professionals from 5 EU countries (Denmark, Sweden, Finland, Estonia and Latvia) and 1 non-EU country (Norway) to discuss the value of networking, exchanging best practices and avenues for collaboration. Attendees, members of the Nordic Haemoglobinopathy Forum (NHF), provided updates on national research initiatives, reference centers, and educational events in their country. Furthermore, during the meeting, Dr Farrukh Shah (Consultant Haematologist, Member of TIF's International Scientific Advisory Board, UK) delivered a presentation highlighting the value and contributions of the UK Forum on Haemoglobin Disorders as a best practice example.
Outcomes	The discussion revealed the similar challenges faced by healthcare professionals in the participating countries to provide optimum care for patients with thalassaemia and sickle cell disease due to the rarity of these disorders and the limited expertise available. Thus, the organization of a Nordic Red Blood Cell Meeting in Copenhagen, Denmark in September 2024, featuring international expert medical speakers to develop knowledge and best practices was agreed.

- **2nd Meeting: 8 March 2024**

Meeting Format	Virtual
Summary	On March 8, 2024, Ms. Katia Pelides, TIF's Senior Educational Programmes Officer, met with 30 medical professionals from 5 EU countries (Denmark, Sweden, Finland, Estonia and Latvia) and 1 non-EU country (Norway). During the meeting, representatives from each country provided updates on the epidemiology and ongoing research initiatives within their region. Additionally, progress on the preparations for the upcoming in-person Nordic Red Blood Cell Meeting in September 2024 in Copenhagen, were shared.
Outcomes	Collaboration on research initiatives among the physicians in all countries will be further discussed during the in-person meeting in September.

- **3rd Meeting: 7 June 2024**

Meeting Format	Virtual
Summary	On June 7, 2024, Dr Androulla Eleftheriou (TIF Executive Director), Dr Michael Angastiniotis (TIF Medical Advisor), and Ms. Katia Pelides (TIF Senior Educational Programmes Officer) met with 31 medical professionals from the Nordic Haemoglobinopathy Forum from 5 EU countries (Denmark, Sweden, Finland, Estonia and Latvia) and 1 non-EU country (Norway). The meeting focused on the clinical management of patients with severe iron overload with organ complications and the appropriate use of intensive chelation therapy, with Prof. John Porter (Consultant Haematologist and Member of TIF's International Scientific Advisory Board, UK) presenting best practices. Furthermore, updates were provided on clinical trials currently underway in Denmark, Sweden, Finland, and Norway. Additionally, progress on preparations for the Nordic Red Blood Cell Meeting, scheduled for September 2024 in Copenhagen, were shared.
Outcomes	Challenging cases of severe iron overload with organ involvement were shared with Prof. John Porter from UCH, an international medical expert on the topic, for his expert advice.

- **4th Meeting: 12-13 September 2024**

Meeting Format	In-person (Copenhagen, Denmark)
Summary	The 1st Nordic Red Blood Cell Meeting was held on September 12–13, 2024, in Copenhagen, Denmark, organised by the Nordic Haemoglobinopathy Forum with the support of the Danish Red Blood Cell Centre at Copenhagen University Hospital – Rigshospitalet and the endorsement of TIF. Representing TIF at this landmark event were Ms. Katia Pelides, Senior Educational Programmes Officer, and Ms. Catherine Skari, Senior Communications Officer. The Meeting brought together 84 leading experts in red blood cell

	disorders from across the Nordic countries and the broader Scandinavian region, including 5 EU countries (Denmark, Sweden, Finland, Estonia and Latvia). Additionally, prominent international speakers from the UK, Canada, and France enriched the discussions with their global expertise, offering their perspectives on the research and treatment of thalassaemia and other haemoglobinopathies. Over the course of the two-day event, participants heard about the latest advancements in haemoglobinopathy research and treatment protocols, as well as new advanced therapies, including gene editing technologies. The meeting served as a vital platform for exchanging knowledge, sharing best practices, and building collaborations to improve patient care across the region. Read the full report, including country reports from Sweden, Denmark and Finland here.
Outcomes	On September 13, medical doctors working with individuals living with thalassaemia or sickle cell disease from Norway, Denmark, Sweden, and Finland presented updates on the current state of haemoglobinopathy care in their respective countries. This was followed by a discussion on potential pathways for collaboration among the four nations, for the purpose of enhancing care and patient outcomes across the Nordic region.

RESULTS ACHIEVED	**Short-term** ▪ Increased visibility of THALIA2024 activities ▪ Continued engagement of network stakeholders ▪ Network expansion with new contacts **Long-term (anticipated)** ▪ Wider adoption of TIF Guidelines on the clinical management of transfusion-dependent thalassaemia ▪ Improved quality of care across EU Member States with special focus on Denmark, Sweden, and Finland.

IMPACT ASSESSMENT

Methodology (*The Impact Model)*

TIF's Impact Model consists of 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 focuses on the evaluation strand of the Logic Model.

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

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

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

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

Criteria ***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?*
T3.1: Stakeholder Network Development	Networking activities remain a key component to raise awareness on thalassaemia and other haemoglobinopathies and the development of synergies to address the different unmet needs of the clinical management of these disorders and improve patient outcomes.	The round of meetings held in 2024, particularly the in-person meeting, significantly strengthened collaborations among healthcare professionals from the Nordic countries and who are working in the field of haemoglobinopathies, with special focus on thalassaemia. This enhanced collaboration is especially critical considering the rising prevalence of haemoglobinopathies driven by increased migration from high-prevalence regions to these countries.	The "inputs" required for the implementation of this task were optimally used, namely: staff time, planning time, and knowledge base.	Through its interactions and joint events, TIF further reinforced its partnerships, expanded its network within these countries, and enhanced its visibility. These efforts also promoted greater adherence to TIF's Clinical Guidelines for the Management of Thalassaemia and amplified the impact of its activities both within the framework of the THALIA project and on a global scale.	Following its networking methodology, TIF shall maintain contacts to ensure that collaboration among healthcare professionals continues for the benefit of patients.