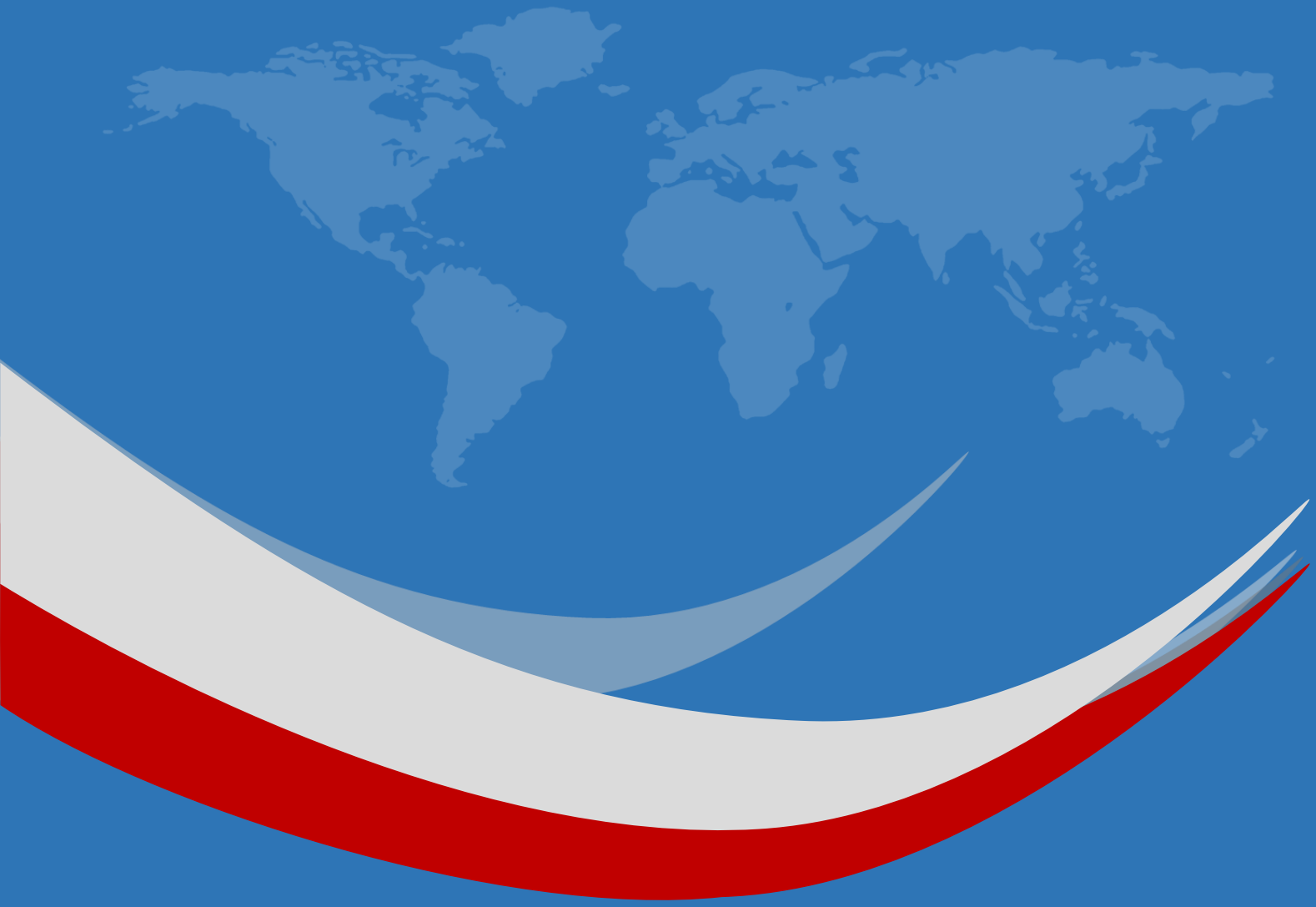
THALASSAEMIA INTERNATIONAL FEDERATION

Collaborating Reference Centres for Haemoglobinopathies Certification Programme



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PREFACE

Health care quality is the degree to which health care services for individuals and populations increase the likelihood of desired health outcomes. Quality in Healthcare is achieved through the use of standards, protocols, guidelines and other management tools. The principal purpose of such standards is to achieve improvement in the quality of care and ensure patient safety through the inspiration for excellence in healthcare. A set of standards comprises a list of standardized requirements in terms of quality-assuring infrastructure and processes that a healthcare organization has to fulfil. In other words, this set of standards determines how and against what the organisation's performance should be evaluated and monitored in the context of a certification or accreditation process.

The objectives are to protect the public from harm and to improve the quality of health services, by providing a quality assurance mechanism that tests whether relevant systems are in place to ensure that minimum standards of safety and quality are met, and a quality improvement mechanism is provided that allows health services to realise inspirational and developmental goals.

Quality is further usually evaluated using certain quality indicators, developed by many different organizations. An example is the indicators published by the Agency for Healthcare Research and Quality (AHRQ). Quality Indicators (QI) modules represent various aspects of quality such as Prevention Quality Indicators, Inpatient Quality Indicators, Patient Safety Indicators, and Paediatric Quality Indicators.

EXECUTIVE SUMMARY

Organizations and businesses around the world strive for continuous improvement using a well developed circular mode of activities and management processes to reduce human error, incorporate cost-effective procedures and eventually achieve the best quality for their products and services. Over the past few decades, quality management methodology through published standards used for accreditation and certification has evolved as a well established operation around the world applied in different fields. Healthcare represents an important sector where quality improvement is expected to lead to reduced human error thereby saving lives and reducing unnecessary injuries, disability and deaths. Although, several international and national organizations are involved in quality management in the healthcare industry, an important quality gap exists both nationally and internationally in the field of quality management for reference centres and professionals involved in health care services for patients with Thalassaemia and other Haemoglobinopathies.

The Thalassaemia International Federation (TIF), as a global stakeholder, a strategic partner of the WHO and the European Commission and an international organization establishing clinical practice guidelines and standards of care for haemoglobinopathies, in order to respond to this need and a growing number of requests to meet such an important challenge, has developed a program of evaluation and certification of haemoglobinopathy centres. This program aims to support the establishment of reference centres around the world with the ultimate goal to provide global quality-certified services for thalassaemia and other haemoglobinopathies. In this context, TIF has developed a set of **Standards for Quality Haemoglobinopathy Care** for the evaluation of centres providing health care services to these patients. The program evaluates candidate haemoglobinopathy centres against the TIF's set of standards and upon successful completion of the evaluation process, grants the **Certificate of TIF Collaborating Reference Centre for Haemoglobinopathies**.

In parallel, TIF is in the process of developing an International Commission for Quality in Thalassaemia and other Haemoglobinoptathies (ICQTH), an international independent accreditation body for haemoglobinopathies centres. Accreditation has been defined as "A self-assessment and external peer assessment process used by health care organisations to accurately assess their level of performance in relation to established standards and to implement ways to continuously improve". Accreditation is recognised as an important driver for safety and quality improvement and health accreditation processes are highly regarded internationally. The already established set of TIF Standards for Haemoglobinopathy Care and certification program of TIF Collaborating Reference haemoglobinopathy Centres will form the core of the proposed accreditation program under development. Under the umbrella of ICQTH, an International Board of Thalassaemia (IBTH) will also be established to provide specific educational courses and board certification to health professionals involved in the field of thalassaemia and other haemoglobinopathies. ICQTH will also support quality and safety and excellence of care through consultation services, scientific publications, regional and global conferences and targeted support of state-of-the-art research in this field.

INTRODUCTION

The Thalassaemia International Federation (TIF) was founded with the vision and mission to ensure the equal access to optimum care for all thalassaemia and haemoglobinopathy patients around the world. The quality of care is central to achieving the desired patient outcomes of reduced morbidity, good quality of life and long survival. It is in this context that TIF undertook a new initiative, which is the development of a system of certification for centres providing clinical services to haemoglobinopathy patients.

A) WHAT IS QUALITY CARE

Even though health care professionals and services in general are motivated to achieve the highest outcomes in the care of patients, such services, however, are not always supported to provide optimum care, which is patient centred and safe, resulting in the desired outcomes. This is particularly so when chronic and rare diseases are concerned. Chronic diseases are a significant burden on health services and usually require complex and multidisciplinary care models.

Health care quality is the degree to which health care services for individuals and populations increase the likelihood of desired health outcomes. Quality of care plays an important role in the triad describing the intricate relationships between quality, cost, and accessibility of health care within a community and a national healthcare system. Researchers measure health care quality to identify problems caused by overuse, underuse, or misuse of health resources. In 1999, the Institute of Medicine (IOM) released six domains to measure and describe quality of care in health. Based on IOM, quality in health care should be:

1. ***Safe*** - avoiding injuries to patients from care that is intended to help them.
2. ***Effective*** - avoiding overuse and misuse of care.
3. ***Patient-Centred*** - providing care that is unique to patients' needs.
4. ***Timely*** - reducing wait times and harmful delays for patients and providers.
5. ***Efficient*** - avoiding waste of equipment, supplies, ideas and energy.
6. ***Equitable*** - providing care that does not vary across intrinsic personal characteristics.

According to a WHO document, "there is evidence that internal mechanisms of organisational and personal development have repeatedly failed to ensure safety, efficiency, best practice and public accountability". It was therefore suggested that mechanisms of external regulation may contribute to quality improvement.

B) INTERNATIONAL STANDARDS AND EXTERNAL CERTIFICATION/ACCREDITING AGENCIES

During the last decade or so, the idea of providing comprehensive care and addressing all possible health care needs, through pooling of knowledge and expertise at the national and global level, can be achieved through the development of Centres of Excellence and Professional Networks. Focusing on patients with thalassaemia and other haemoglobinopathies has gained particular momentum in Europe and countries around the world. Therefore, it is of paramount important that such a global movement be accompanied by appropriate standards, protocols and guidelines to secure comprehensive care with the highest possible level of quality. TIF has long been involved in developing clinical practice guidelines and standards for quality care for patients with thalassaemia and other haemoglobinopathies that have been globally adopted and translated in many languages.

The significantly improved health and quality of life resulting from improved care and services provided to patients with thalassaemia and other haemoglobinopathies has been demonstrated in recent years through survival studies in Cyprus, Greece, Italy, UK and some other, mainly Western countries. Therefore, the need for the establishment of reference centres in different countries served by properly educated and trained health professionals is being internationally recognised as one of the best management approaches to move forward successfully. Along with the above, the need to develop a targeted international system of quality certification for these reference centres and their health professionals is highly recognized.

Assessment, certification and accreditation at a national level may be provided by the government, usually the Ministry of Health, based on nationally developed standards. More prestige, however is provided by international certificating or accreditating bodies.

C) THE HAEMOGLOBIN DISORDERS

Chronic diseases, including thalassaemia and other haemoglobinopathies, present a broad array of epidemiological, clinical, laboratory, social and other challenges, both for the management of patients and families alike and the provision of quality care. Current data demonstrate that 7% of the global population carries a pathological haemoglobin gene and more than 500,000 affected children are born annually. Patients with thalassaemia, sickle cell disease, and other rare anaemias, represent chronic conditions with challenging and difficult management requirements that demand a comprehensive multidisciplinary approach on different levels of care.

Specialized centres for thalassaemia and other haemoglobin disorders have been developed and exist since the early 1980s, Cyprus, Greece and Italy being amongst the first, followed by those gradually put together in the UK and France. The above centres act as reference centres with specific expertise in one or more components such as prevention, clinical care and/or research at the national level. Through their designation as WHO Collaborating Centres, they have, in addition, contributed to the promotion of training and awareness of Hb disorders at the regional and international levels, and among health professionals.

Several criteria for reference centres for haemoglobinopathies have been lately reviewed and consensus has been reached by European experts at the level of the EU, in the context of developing a European Reference Network of services. The above consensus process ensures that existing reference centres will upgrade their infrastructure and services, and new centres will follow the above suggested standards and guidelines to support network-based expert services on diagnosis, management, education and research at the regional and European level, while expanding gradually to other regions of the world.

D) THE INVOLVEMENT AND INTEREST OF THE THALASSAEMIA INTERNATIONAL FEDERATION

The objectives are:

- To promote centres of excellence as examples to other centres, within a country or internationally
- To create a competitive environment, between centres which may help the improvement of services

- To allow selected centres to be recognised by governments as reference centres, able to support secondary centres. This will allow governmental support for manpower and service planning.

There are in many countries in which centres are labelled “thalassaemia centres”. These vary considerably in their expertise, their services, the number of patients and their support from health authorities. Few have attained to an international or national accreditation. Patient outcomes are rarely recorded. Certification of these centres will promote harmonisation and raising of standards of care. It will also allow quality control and ultimately improve patient outcomes (Forni GL et al.)

TIF has long given emphasis on the required quality of services dealing with haemoglobin disorders and has researched and published over several years on the subject:

- TIF, in collaboration with other associations of chronic disease patients, conducts surveys on quality of services, addressing both professionals and patients.
- TIF develops and publishes the Guidelines for the Management of Thalassaemia that have gained global adoption and have been translated in several languages.
- TIF has published its views on the requirements for a reference or expert thalassaemia centre (Angastiniotis M, Eleftheriou A in Hemoglobin, vol 33(S1): 204-10)
- TIF contributed to the Enerca network and the writing of the White Book “recommendations for centres of expertise in rare anaemias” published in 2013
- TIF contributed a chapter in a book “Thalassaemia causes, treatment options and long term outcomes”, published in 2014. The chapter, “Assessing services for haemoglobin disorders: a toolkit for service planning”, was based on a survey conducted by TIF of 147 service centres across the world.

These studies and publications indicate the interest of TIF over the years, in setting criteria for centres serving thalassaemia patients. In the context of the present certification program, TIF has consolidated its previous studies and along with both medical specialists and patient involvement, has set criteria for quality services to be used for certification of centres.

In summary, a certification system ensures effective services and equity of care that patients can trust, through a system of quality control and quality improvement. To this system, TIF proposes networking and support of centres with few patients by the **TIF Collaborating Reference Centres for Haemoglobinopathies** to further promote equity of care.

The current document describes the haemoglobinopathy centre evaluation and certification program that TIF has developed, with the use of its rich international network, strategic partnerships with WHO and other international organizations, and collaborations with experts around the world, in order to serve the increasing European and global needs for quality certification in this special field of haemoglobinopathies.

This program strives to serve the needs of Centres of Excellence and their dedicated health professionals for continuous quality improvement and cost-effective care for patients with thalassaemia and other haemoglobinopathies.

NEEDS ASSESSMENT

Systems of quality certification have been developed over the past few decades at the national, regional and international level. Different national and international organizations have evolved through public and private efforts, based on inspirational individuals or organisations, and leading to appropriate legislation. The WHO, the US medical services, and the European Commission have recognized the importance of the introduction of quality standards in the healthcare sector and have followed appropriate legislative and statutory efforts to promote a quality system in health. In parallel to other initiatives and similar to other economic and industrial sectors, the ISO system has introduced different standards applicable to the health care industry.

Although quality certification has evolved around the world and many national and international organizations are involved in this operation, certification of the particular healthcare sector providing care to thalassaemia and other haemoglobinopathies is lacking. This is in contrast to other disease-oriented organisations, such as EUHANET for haemophilia, have already developed their certification/accreditation systems.

Due to the special needs of such patients and the particular operations and processes required to provide specialized care to thalassaemia patients, the development and application of specific quality standards is of paramount importance. Therefore, the need to establish an international program to promote the development and implementation of specific quality standards for reference centres providing healthcare services to patients with thalassaemia and other haemoglobinopathies, is viewed as an important step forward. This is in line with TIF's continuous efforts for improvement in the quality and safety of health services provided to haemoglobinopathy patients around the world.

Bearing in mind the above, TIF has developed a program of evaluation and certification of haemoglobinopathy centres against a set of standards of quality care, which reflect current evidence based standards and guidelines developed by TIF and experts in the field.

In parallel, TIF is in the process of creating of a daughter organisation, named 'the **International Commission for Quality in Thalassaemia and other Haemoglobinopathies** (ICQTH) that will provide independent accreditation to haemoglobinopathy centres. This organization will be developed with the guidance of the International Society of Quality in Health Care (ISQua). ISQua, in collaboration with WHO and other international organizations has been recognized as the single global stakeholder in providing accreditation to national and international certifying bodies, which operate in the health care industry. ISQua's International Accreditation Program (IAP) provides a mechanism for external evaluation and standards setting organizations to assure themselves that their standards, their surveyor training programs and they themselves, as an external evaluation organization, meet international best practice requirements and to demonstrate this to their client, funders and other stakeholders. The development of ICQTH is described in detail in a dedicated TIF document.

TIF Collaborating Reference Centres for Haemoglobinopathies: An international certification programme

VISION

The Vision of the international certification program of **TIF Collaborating Reference Centres for Haemoglobinopathies** is to encourage all countries with thalassaemia patients and patients with other haemoglobinopathies to establish centres of excellence and train their health professionals to provide quality care to these patients based on well defined and internationally accepted quality standards that ensure a comprehensive multi-disciplinary approach to patients' needs.

MISSION

The mission of the program is to help improve the quality and safety of the comprehensive and multi-disciplinary care provided to patients with thalassaemia and other haemoglobinopathies around the world by evaluating and certifying the provided services against a specific set of quality standards, the TIF Standards for Quality Haemoglobinopathy Care.

VALUES

The program is governed by a specific highly regarded set of values that are also enforced, embraced and followed by TIF. TIF continuously strives for Decency, Honesty, Solidarity, Equity, Transparency, and above all Excellence in quality and safety for patients and their families.

SERVICES

The program is a non-profit initiative dedicated to promote quality and safety for reference centres providing care to patients with thalassaemia and other haemoglobinopathies. The aim is to evaluate centres against TIF Standards for Quality Haemoglobinopathy Care and certify them as TIF Collaborating Reference Centres for Haemoglobinopathies. The centres elect voluntarily to apply for such a certification. This is expected to be with the approval of the health authority that is responsible for the centres.

QUALITY STANDARDS

The program focuses on the application of specific quality standards for reference centres involved with the care of patients will thalassaemia and other haemoglobinopathies. The already developed TIF Quality Standards of are based on the general principles already developed by the following organisations:

- The Joint Commission International (JCI): "Survey process Guide for Ambulatory Care (3rd Edition, 2015) **see annex 1**
- European Union Committee of Experts on Rare Diseases (EUCERD): Quality Criteria for Centres of Expertise for Rare Diseases in Member States (2011) and EUCERD recommendations on Rare Disease European Networks (2013) **see annex 2**
- Guidelines for Good Clinical Practice
- US Institute of Medicine: Quality Improvement
- US Department of Health and Human Services, Health Resources and Service Administration: Quality Improvement

- UK NHS, Peer review of health Services for People with Haemoglobin Disorders: (2015 Review)
- TIF "Guidelines for the management of transfusion dependent thalassaemia" 3rd edition 2014
- TIF "Guidelines for the management of non-transfusion dependent thalassaemia" 2013
- Specific standards, such as the "International Collaboration for Transfusion Medicine (ICTMG): "Red blood cell specifications for patients with haemoglobinopathies: a systematic review and guideline" 2017
- ENERCA White Book
- European Guidelines for the certification of Haemophilia Centres EUHANET 2013.
- Current literature reviews

The criterion for recognizing any centre a reference centre is the quality of services, and not just availability of various technical components necessary for thalassaemia care. Quality care involves patient-centred care. It includes following national or international evidence based guidelines, which allow for good patient outcomes. The services provided by the certified centres should be based on the European standards for reference centres, including:

1. The capacity to provide **expert diagnosis** of the disease as well as its long term complications
2. The capacity to provide **expert case management**, including a multidisciplinary approach and psychosocial support. These requirements imply **experienced healthcare personnel** in adequate numbers to ensure continuity of care
3. Health care workers should be in a structured environment with **clearly defined roles and hierarchy**.
4. Maintain a **patient registry** with ability to report patient outcomes and other epidemiological information. Electronic information systems must be regarded as essential tools to the provision of quality services.
5. Have **auditing and quality control** mechanisms
6. Serve **a sufficient number of patients** to maintain staff experience. What is a sufficient number of patients is not clear but a consensus should be reached – initial proposal is for 50 transfusion dependent patients, with a minimum of 25 if all other standards are satisfied.
7. Provide **patients with sufficient knowledge** and information to promote **partnership models** and self-management support
8. Have a contribution to **research** as evidenced by peer reviewed publications
9. **Networking** with secondary centres but also with other centres of expertise nationally and internationally.
10. Maintain **close links with patient organisations** and other community resources.

In addition, there must be evidence of **health system support** and **free access** of patients to treatment modalities. The centres' administrative structure, working hours and clinical space availability must also be taken into consideration, with the **patient experience** in mind. Any deficiencies and gaps must be identified and corrected.

There is also need to assess the **experience of professional staff**.

Patient perceptions of the quality of the services, should be monitored and taken into account in quality assessment. A separate questionnaire is designed, to assess patient views of the services and their relationship with the staff. Such a system will increase patient confidence in the services and may have a positive effect on patient compliance.

EVALUATION AND CERTIFICATION PROCESS

The objective of certifying centres by the Thalassaemia International Federation, is to ensure the provision of services aiming to achieve the best possible outcomes for patients with haemoglobin disorders. Issues of safety as well as good clinical practices according to evidence based guidelines are taken into consideration.

The evaluation and certification process to reach the Certificate of TIF Collaborating Reference Centre for Haemoglobinopathies follows a stepwise approach outlined below:

1. Submitting an application

The candidate centre applies for the certification program at TIF. TIF is engaged in the evaluation and certification of the centre only with the consent of or in collaboration with the country's health authorities, which will be copied in any future communication between the centre and TIF.

TIF asks for a description of centre's infrastructure, procedures and services to be provided before on-site evaluation.

2. First on-site evaluation visit

A TIF group of experts performs the first on-site evaluation visit of the candidate centre. This survey team visiting a centre will have a checklist of acceptable standards. Based on this checklist the team will assess compliance and existence of the listed services. The check list is based on long experience of visiting centers in many countries and on the advice of its expert advisory panel. The TIF survey teams base their final grading system according to the ISQua standards (ISQua Guidelines and Standards for external evaluation organisations. 4th edition, September 2015).

The thalassaemia checklist consists of 175 items. Each item will be considered according to the degree of compliance, which may be either:

- Yes service complies (score 1) or not (score 0)
- Service Complies (score 3), or complies partially (score 1 or 2) or does not comply (score 0)
- Further qualitative estimates will be assessed for certain items, which will be scored by the team according to its contribution to safety and effectiveness.

The score will not be given immediately to the centre, but certification will be finalised after due consultation of the team members, with a Board of Expert Advisors[1], within a period of one month.

3. Quality classification

The results of the first on-site evaluation visit will lead to a classification as follows:

1. A Centre of Excellence award is given to a centre which fulfils at least 70% of the items investigated or has successfully addressed all suggestions for improvement within 3 months of receiving the report
2. A Certification with follow-up survey is awarded if there are items which require improvement which can be made within 12 months of receiving the report.
3. Certification will be postponed if the centre must undertake a quality improvement programme, according to the recommendations of the team, which includes major safety and effectiveness issues. Re-assessment will be made when the centre re-applies.
4. Denial of certification is at the discretion of the team because of major deficiencies. The centre is expected to make major changes and upgrades of its services.

A team of experts[2] will be available to assist all centres in quality improvements.

The Certificate of TIF Collaborating Reference Centre for Haemoglobinopathies is valid for a period of 3 years from its date of issue, after which a re-evaluation is required, unless it is deemed necessary by the centre or TIF to perform the re-evaluation sooner.

The centre is required to submit to TIF an annual report of a predefined set of healthcare statistics before the end of each year, while a line of frequent communication is kept.

***Diagram 1**: 3-step evaluation process*

[1] The Board of Expert Advisors consist of TIF's expert medical advisor panel, which is also responsible to develop guidelines.
[2] The team of experts advising on quality improvements will vary according to the needs and improvements that will be required, and experts in particular areas (e.g experts in laboratory diagnosis, MRI standards etc.)

TECHNOLOGY CONSIDERATIONS

Nowadays, electronic and online services represent one of the most important sections of every centre. The TIF certification program in supported electronically and by a series of online resources, including the TIF Website that provides a wealth of information and resources for thalassaemia and haemoglobinopathy care and the TIF Educational e-Platform for Healthcare Professionals, an online education program based on TIF's guidelines; upon successful completion of the program targeted to healthcare professionals. Upon successful completion of the program, the candidate is certified by TIF.

SUGGESTED FURTHER READING

1. Joint Commission International Survey Process for Ambulatory Care. 3rd Edition 2015
2. Quality and accreditation in health care services, a global review. WHO Department of Health Service Provision. Geneva 2003
3. Clinicians, services and commissioning in chronic disease management in the NHS. The need for coordinated management programmes. Report of a joint working party of the Royal College of Physicians of London, the Royal College of General Practitioners and the NHS Alliance. 2004
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5. ENERCA White Book
6. Health services for people with haemoglobin disorders. University College London Hospitals, NHS Foundation Trust, Whittington Health NHS. UK forum on Haemoglobin Disorders 2016, (http://www.wmqrs.nhs.uk/review-programmes/view/haemoglobin-disorders-2014-16-reviews-adults-and-children.
7. Extracts from the ICH Harmonised Tripartate guideline for Good Clinical Practice E6(R1), www.ich.org
8. EUCERD Recommendations: Quality criteria for centres of expertise for rare diseases in member states. 2011
9. Heon-Klin V et al. European Reference networks for rare diseases: what is the conceptual framework? Orphanet J of Rare Diseases. 2017, 12: 137
10. US Department of Health and Human Services, Health Resources and Service Administration: Quality Improvement. 2011 HRSA
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13. Wagner EH, Austin BT, Davis C et al. Improving chronic illness care: translating evidence into action. Health Aff (Millwood). 2001, 20:64-78
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15. Angastiniotis M, Eleftheriou A. Assessing services for haemoglobin disorders: a toolkit for service planning. In "Thalassaemia: causes, treatment options and long term outcomes". Edited MakenzieGreene, Nova Science Publishers 2014. Series: Recent advances in hematology research

TIF STANDARDS FOR QUALITY HAEMOGLOBINOPATHY CARE CHECKLISTS

STANDARDS FOR CONSIDERATION IN ASSESSING CENTRES FOR HB DISORDERS (BASED ON JCI STANDARDS)

1. Governance

- The existence of a hierarchical structure, ordained by law and policy. This should include a chief executive/ managing director and a professional team which is coordinated and includes multidisciplinary services, recognising the complex pathology of haemoglobin disorders.
- A clear definition of the centre's mission and the existence of policies and programmes to fulfil the mission.
- Ensuring staff qualifications, experience and continual education
- Monitoring and evaluating the functions of the centre by the management, including staff performance and patient safety
- The existence of plans for quality improvement and advocacy to health authorities
- Connection with patient support associations, with patient representation on advisory bodies. Taking into account all stakeholders views regarding matters of priority and focus in any quality improvement activity.
- All decisions are based on data, obtained through patient records and outcomes, as well as any new developments that have been noted through publications and trials.
- A culture promoting ethical practices in all aspects of administration and clinical care. Considering internationally accepted patients' rights.

2. Safety concerns

- Staff education on safety is programmed
- Patient identification is clear in individual records (electronic or paper based), blood transfusions and lab results
- There is effective patient communication and explanation of all interventions.
- Haemovigilance and pharmacovigilance are practised, including drug safety alerts.
- There are evidence based hand hygiene guidelines
- There are measures to reduce accidents, such as falls in the centre. A secure environment is planned and regularly inspected. Hazardous material handling and disposal (such as needles), is part of the centre's daily procedures
- There are treatment rooms, and resuscitation equipment
- Fire safety and certification by the country's fire services is available. This includes regular testing of any devices required for fire control
- Cigarette and other smoking is forbidden on the premises
- Emergency procedures are in-place in the event of power and water cuts or contamination. Monitoring water quality is performed regularly.

3. Access to care

- The centre clearly serves benign haematology patients and does include malignancies as they constitute a dangerous and vulnerable cohabitator
- Patient flow: there must be adequate numbers of patients of each diagnostic group: at least 50 thalassaemia patients and/or 50 SCD patients for the centre to be regarded as experienced
- Continuity of care is safeguarded by low staff turnover and the presence of experienced and qualified caregivers.
- Clinical records with lifetime data are kept
- Multidiscplinary care is provided with a referral system where necessary, and collaboration with in-patient services
- Networking with secondary centres as well as with other centres of excellence, nationally or internationally is an added value. A twinning programme with an academic centre is also an additional advantage
- Any existing electronic health record must fulfil all the requirements of patient safety, including patient consent, confidentiality and anonymization in data storage and sharing of data for research
- Barriers to patient access, including distance, language, cultural or religious barriers are considered and dealt with
- Respect for patient rights and time is a must in all cases
- Informed consent for all procedures is obtained.

4. Partnership model

- Adequate information to patients/families about the disease and any treatment decisions, including possible side effects, is always provided
- Patients are given choices about their treatment
- Self management is encouraged
- Special attention to patient adherence is given and the patients supported appropriately
- Workshops for patients/families are held regularly, at least once a year

5. Guidelines and standards for clinical care

- Evidence based national guidelines, put together by experts in the field or international guideline (e.g. TIF's) are used in the centre and adhered to.
- Pain screening is performed and a pain management system is in place
- Assessing the quality of laboratory and other technologies used to monitor patients is the responsibility of the clinical team which must alert the providers of any divergent or inaccurate results
- Infection control procedures are part of the clinical standards of the centre
- Availability of food during day care is necessary and the quality and nutritional value must be monitored
- Blood transfusion procedures and standards according to international directives are kept
- Any medical treatment, such As IV fluids, exchange transfusions etc are provided according to standards that ensure patient safety.
- Continual medical and other professional education are part of the centres long term programme
- Staff qualifications, skills knowledge and experience are defined and described along with the job description of each
- Staff/patient ratio is defined approximately as 1 doctor per 100 patients and 1 nurse per 50 patients

6. Quality improvement

- Having surveyed all aspects of the service, and noted all strengths and weaknesses, the survey team will present a report and also suggestions for quality improvement where necessary
- Quality improvement is a systematic approach to changes aiming to upgrade services and correct any deficiencies in the governance, structure and functions of the service. “Quality improvement includes better patient experience and outcomes, by changing provider behaviour” (Dr John Ovretveit: “Does improving quality save money?”)
- The way in which change is introduced and implemented is a matter of concern and may require expert advice. In this process the following are considered:
- External influences, such as governmental policies or interest, budgetary support, professional requirements.
- Understanding the issues involved at all levels, including why a problem exists
- Setting goals and monitoring progress
- Choosing the tools to bring about change. These could be skills development, computerisation, updating guidelines etc
- Full staff engagement is necessary. There often needs to be a multidisciplinary approach to change making
- The patient’s voice must be involved in all stages of quality improvement. Patient/families can also effectively monitor the effects and benefits of change since they experience the whole “patient pathway”.
- Studying other centres experience in change making: have the changes been successful elsewhere?

7. Information Management

- Patient records (paper or electronic) are kept with due consideration to confidentiality, security and accuracy of data
- The retention time of records in a haemoglobinopathy setting, is lifelong, since the current clinical condition may be influenced by past events and disease control (such as iron levels)
- Standard diagnosis codes are kept (e.g. ICD10)
- E-health systems are assessed and tested prior to implementation, for quality and patient safety
- Protection against loss, unauthorised access or use is ensured
- Policies and procedures concerning record keeping are clearly directed to all the staff, through documents and training
- The patient should be clearly identified on each record
- Those authorised to have access to clinical records are clearly defined.

CHECKLIST FOR THE ASSESSMENT TEAM (repeated on an annual basis).

Centre identification:
Title……
Address……
City…….…… Country……..Zip code……
Tel……Fax……..…Email……..……

Staff

Administrative Director…….....…(Attach CV)
Clinical Director (if different)…….……(Attach CV)

- Is there an organisation chart of key personnel? Y/N
- **Other Medical**: (expand table according to numbers of doctors)

Specialty	Name/s	Age/s	Years of service in the centre
Haematologist			
Paediatrician			
Internist			

- **Supporting medical** (expand table according to numbers of specialists)

Specialty	Name/s	Age/s	Years of service to the centre
Endocrinologist			
Cardiologist			
Gynaecologist			
Hepatologist			
Psychologist			

NB: These specialists are not expected to be employed fulltime within the centre, rather employed in hospital departments, but committed to collaborate and serve thalassaemia patients having gained experience in the specific complications of the disease.

- **Nursing staff**

Head nurse: Name……..……
Years in centre……
2nd head nurse or assistant……..……
Years in centre……..……
Total number of nurses……..……

- **Social worker**: Is there a dedicate worker? Y/N
  Qualification……..……..

Doctor/ patient ratio …..
Nurse/ patient ratio……

- Support for **continuous medical education** (CME) for all levels of staff:

Activity	No provision	Provision occasionally	Provision for regular support
Support to attend international conferences			
Fellowships for staff			
Webinars facilitated			
Teaching facilities within centre			
Centre teaches staff from other centres, GP's, Nurses etc.			

Administrative structure:

- Direct administration by Ministry of Health Y/N

- Administered by NGO Y/N

- Is there a management committee for the centre? Y/N
- List members and the capacity of each…….
- Describe the role and terms of reference of the committee……

- Is there an scientific advisory committee? Y/N
- List members and the capacity of each……..…….
- Describe the role and terms of reference of the committee……
- Is their patient representation? Y/N

- Attached to hospital: Y/N
  a) Ministry Hospital ☐
  b) University ☐
  c) Private ☐

- Separate unit from Hospital Y/N

- The centres serve:
- Paediatric patients exclusively Y/N
- Adult patients (over the age of 16y) Y/N
- Mixed age unit Y/N

Budget

Budget from: (more than one answer possible)

a) Ministry ☐
b) Ministry but independent budget from the hospital ☐
c) Donations ☐

d) NGO supported entirely ☐
e) Private (out of pocket and/or insurance finance) ☐

- Is the funding sustainable? Comment……..….
- Is there political commitment to support the centre long term? Comment……..

Patient support:
a) Full financial coverage for all patients ☐
b) Full financial coverage according to age (e.g. children only) ☐
c) Coverage according to family income ☐
d) Partial coverage ☐

If partial coverage:
a) Out-of-pocket expenses for blood transfusion ☐
b) Out-of-pocket expenses for consumables ☐
c) Out-of-pocket expenses for iron chelation ☐
d) Out-of-pocket expenses for laboratory tests ☐
e) Out-of-pocket expenses for MRI ☐

Centre Accreditation:

Has the centre gained accreditation for any international body:
JACIE Y/N **JCI** Y/N **ISO** Y/N **AABB** Y/N

Functions:

- Is the centre a day care centre? Y/N
- What are the daily working hours of the centre? from…..am to …..pm
- Connectivity with inpatient services…….... (describe)
- Connectivity with emergency departments ……..…..…(describe)
- Connectivity to a patient support association……(describe)

Centre involved in **research**:
1. Peer reviewed publications Y/N (provide list)
2. Research grants Y/N (provide list)
3. Clinical trial involvement Y/N (provide list)
4. Basic research ☐

Centre involved in **teaching**:
1. Medical undergraduates ☐
2. Medical postgraduates ☐
3. Nurses ☐
4. Laboratory staff ☐

Epidemiological information

Does the centre maintain a **patient registry**: Y/N
If yes:
a) Is it paper based Y/N…. Electronic Y/N….
b) Is it confined to the patients followed in the centre Y/N

c) Is it part of a hospital general registry Y/N
d) Is it part of a regional registry Y/N
e) Is it part of the national haemoglobinopathy register Y/N
f) Is it part of a rare disease register Y/N
g) Are legal and ethical regulations followed in sharing patient data (e.g. informed patient consent)? Y/N

How many **patients** are currently being followed in the centre:
a) Transfusion dependent beta thalassaemia (TDT) ……
b) Non-transfusion dependent beta thalassaemia (NTDT)……
c) HbE/beta thalassaemia……..
d) HbH disease……
e) SCD patients (S/S,S/C or S/beta thalassaemia)…..
f) Other anaemias (please list)

Please provide an **age distribution** of the TDT patients (present a graph please)
a) Number of patients 1-10 years……
b) Number of patients 11-20 years…..
c) Number of patients 21- 30 years……
d) Number of patients 31-40 years……
e) Number of patients 41-50 years…….
f) Number of patients over 50 years……

Patient **deaths**/year:
- Current year….
- Previous year….
- Last 5 years….

Patient **education**: How many of the TDT patients are in the following educational groups:
a) Primary education (completed or not) no education…..
b) Vocational secondary education…..
c) Full secondary education…
d) Higher vocational, bachelors degree….
e) University mastersdegree and higher…..

What is the carrier rate, in your region/country?:
a) Beta thalassaemia genes ….. (from a population of……)
b) HbE genes …… (from a population of…..)
c) HbS genes …… (from a population of…..)

What is the expected birth incidence for thalassaemia in your region/country?…….

Building infrastructure

1. Number of transfusion beds for children under 10 ….
2. Number of transfusion beds for adolescents …..
3. Number of transfusion beds for adults …..

- Are patient rooms separated according to age group? Y/N
- Are there rest areas? Y/N
- Is there a play area? Y/N
- Is there an office for each doctor, ensuring privacy for patient examinations? Y/N
- Is there a secretariat with registration and patient record storage? YN
- Is there a procedures room?
- Caters for the needs of the disabled Y/N

Basic Equipment

1. Refrigerator for blood storage
2. Stadiometers (Harpender or wall),
3. Growth charts (in paediatric units)
4. Electronic systems for communication and exchange of information with other centres
5. Transcranial Doppler (if SCD children are being treated)

Availability of essential supplies

i. All three iron chelating agents available
ii. No interruptions in supplies of drugs
iii. No delays in blood transfusions due to inadequate supplies
iv. Blood filtration available for all transfusion units
v. Infusion pumps in adequate supply

Clinical Services

1. THE CAPACITY TO PROVIDE EXPERT DIAGNOSIS OF THE DISEASE AS WELL AS ITS LONG TERM COMPLICATIONS:

Diagnosis:

Expertise in phenotypic diagnosis of Hb disorders:

- Lab within centre Y/N
- Based on other reference labs Y/N
- Expertise in molecular diagnosis of molecular disorders
- Lab within centre Y/N
- Based on other reference labs Y/N
- Neonatal screening programme available Y/N

Is there a structured population screening programme run by the centre Y/N

Diagnosis of long term complications:

Which of the following, are practised in the centre?

1. Annual examination of patients by a cardiologist Y/N
   - Is the cardiologist dedicated and experienced in thalassaemia heart disease? Y/N
   - Are there regular discussion of the results with the clinic doctors? Y/N
   - Is cardiac MRI with T2* provided annually? Y/N
2. Annual examination by an endocrinologist Y/N
   - Is the endocrinologist dedicated and experienced in endocrine complications of the disease? Y/N

3. Are there regular discussion of the results with the clinic doctors? Y/N

4. Transfusion transmitted Infections
   - What is the proportion of HCV infected patients? ……
   - What is the proportion of HBV infected patients?.....
   - What is the proportion of HIV infected patients? .....

5. Do patients have to pay for MRI examinations?
   - Does the clinic know if the software used is validated? Y/N

6. Is liver iron measured annually? Y/N

- Is it measured T2* Y/N
- Ferriscan Y/N

7. Do patients benefit from annual examinations:
- Transcranial Doppler (if SCD children)? Y/N
- Abdominal U/S? Y/N
- DEXA measurements of BMD

8. Are patients seen by psychologist, at the centre? Y/N
- Are they referred to the psychologist according to personal needs? Y/N
- Does the specialist see all patients? Y/N
- Is the service free of charge? Y/N

2. THE CAPACITY TO PROVIDE EXPERT CASE MANAGEMENT

Evidence based guidelines

a) Not available ☐
b) Available but not integrated in the care delivery ☐
c) Available and used as standards of care in the daily management ☐

The guidelines used:
a) TIF guidelines Y/N
b) Nationally prepared standards Y/N
c) Local standards prepared at the centre Y/N
d) Decision support facilitated by the availability of references and updates, online or in a library setting: Y/N

Continual Medical Education CME

a) Provided sporadically in the centre
b) Staff sponsored to attend conferences, within country and international
c) Staff given fellowships to other centres of expertise
d) Follow on-line courses on thalassaemia and SCD management

Continuity of Care

a) Medical staff is all permanent ☐
b) Junior staff rotate while seniors are permanent ☐
c) There are no permanent doctors in the centre since all rotate ☐

3. AUDITING AND QUALITY CONTROL MECHANISMS

Adverse events:

a) Is the staff trained and do they comply with the haemovigilance system, reporting events in blood transfusion Y/N (review evidence of annual reports)
b) Is the medical staff compliant with the pharmacovigilance system? Y/N (review evidence reports in the past 3 years)
c) Is there an internal system of reporting and evaluating adverse events in centres? Y/N
d) Is there an internal system for investigating and responding to complaints? Y/N

Auditing:

a) Compliance with centre procedures and standards Y/N
b) Compliance with clinical guidelines: describe methods in use by the directors of the centre……..
c) Producing an annual report Y/N
d) Recording patient outcomes:
- **i.** Average and range of pre-transfusion Hb Y/N
- **ii.** Average and range of serum ferrtitn levels Y/N
- **iii.** Age distribution of patients Y/N
- **iv.** Patients infected with HCV, HIV Y/N
- **v.** Patients with cardiac iron overload, recorded by MRI or ECHO Y/N
- **vi.** Patients with liver iron above 7mg/kg dry wt Y/N
- **vii.** Patients achieving higher education Y/N
- **viii.** Proportion of patients in fulltime or part-time employment Y/N
- **ix.** Deaths and causes of death Y/N

(Each centre should choose the patient outcomes they intend to record, without neglecting the basic morbidity and mortality data)

Clinical monitoring:

Recommendations concerning patient care and clinical management

These recommendations are based on guidelines published by international experts and reviewed by peers in 2014.

Recommendations according to the latest international guidelines:

Guideline	Available in the Centre	Comments and Recommendations
Diagnosis:		
Haematological	**1.** CBC, indices ☐ **2.** HPLC ☐ **3.** Capillary electrophoresis ☐ **4.** Other electrophoresis ☐	
Molecular	**1.** For all cases ☐ **2.** Selected cases ☐ **3.** Not done ☐	Molecular confirmation of diagnosis will also indicate the existence of molecular modifiers which may affect treatment
HLA typing before the first transfusion	**1.** All new cases ☐ **2.** Not done ☐	This is recommended but is not a standard which all follow. Explore if SHE can take this over to facilit6ate future transplants
Blood transfusion:		
Voluntary blood donation	**1.** Below 50% ☐ **2.** 75% ☐ **3.** 100% ☐	
Before the first transfusion: extended red cell antigen typing, at least for C,E, Kell	**1.** Only ABO, Rh ☐ **2.** Extended antigen typing for all patients ☐	Guideline for future reaction management – strongly recommended

Before each transfusion: ABO, Rh(D) compatible blood	Done for all patients Y/N	However matching for C, E, Kell is strongly recommended. Collaboration of blood banks, essential
Cross match and screen for new antibodies before each transfusion	**1.** All cases ☐ **2.** Selected cases ☐	
Leukodepleted blood	**1.** Bedside ☐ **2.** Pre-storage ☐ **3.** No filtration ☐	Pre-storage filtration is strongly recommended
Washed red cells	**1.** All cases ☐ **2.** If indicated ☐	Recommended for patients with severe allergic reactions
Pre-transfusion Hb 9-10.5g/dl	**1.** All cases ☐ **2.** Some fall below ☐ **3.** Local guideline 8g/dl ☐	.
Pre-transfusion Hb 11-12g/dl for patients with heart complications	Y/N	Short transfusion intervals are necessary to avoid circulatory overload while good oxygenation of heart muscle is achieved
Keep post-transfusion Hb below 14-15g/dl	Y/N	
A record of reactions and annual transfusion requirements must be kept	**1.** National haemovigilance program ☐ **2.** Local requirement □ **3.** Not done ☐	
Iron overload monitoring:		
Serum ferritin	**1.** Monthly ☐ **2.** Every 3 months ☐ **3.** Annually ☐ **4.** Never ☐	
Keep ferritin levels below 1000microg/l in TDT Keep ferritin level below 800 microg/l in NTDT	**1.** Standard for all ☐ **2.** Few patients achieve this ☐ (what proportion?) **3.** No patients keep these levels ☐	
Liver iron concentration (LIC) Methods: Biopsy MRI	**1.** Available for all ☐ **2.** Available if payment ☐ **3.** Not available ☐	MRI is the non-invasive method of choice each patient needs a measure annually after the age of 8 years.
Cardiac iron measured by MRI T2*	**1.** Available for all ☐ **2.** If payment ☐ **3.** Not available ☐	This needs to be a validated method and regularly calibrated, annually
Liver fibrosis be estimated by: biopsy and an ultrasound instrument: the Fibroscan	**1.** Only biopsy ☐ **2.** Fibroscan ☐	Selected cases
Iron chelation:		
Chelation therapy cannot be effective unless taken regularly	**1.** Adherence support by staff ☐ **2.** Support by psychologist ☐ **3.** Patient left to self-regulate ☐	Optimising adherence requires making the drugs easily available and by proper family and professional support

All three chelating agents registered	**1.** All three provided according to the needs of each patient ☐ **2.** DFO not available ☐ **3.** DFX not available ☐ **4.** DFX for selected cases ☐ **5.** DFP not available ☐ **6.** DFP for selected cases ☐	Desferrioxamine (DFO)- SC or IV Deferiprone (DFP) - oral Deferasirox (DFX) – oral
Chelation therapy free of charge	**1.** Free from government ☐ **2.** Free from NGO ☐ **3.** Free from insurance ☐ **4.** Partial out of pocket ☐ **5.** Totally out of pocket ☐	
Combination of DFO + DFP or other may be used	**1.** Not used ☐ **2.** Selected cases ☐ **3.** Only for in-patients ☐	Beneficial in high overload and may be given to intensify treatment in heart failure and other emergencies
Monitoring side effects:		
▪ **DFO:** ▪ Audiometry annually ▪ Ophthalmology annually ▪ If fever stop therapy temporarily and establish organism (yersinia or klebsiella) ▪ Do not give prochorphenazine ▪ Stop if hypersensitivity ▪ Stop if pregnancy	 Y/N Y/N Y/N Y/N Y/N Y/N	
▪ **DFP:** ▪ Neutrophil count every 1-2 weeks ▪ Stop if ANC <500 ▪ Patient to report if symptoms of infection and stop if fever ▪ Stop if joint pains ▪ Watch liver function ▪ Stop if pregnancy	 Y/N Y/N Y/N Y/N Y/N Y/N	
▪ **DFX:** ▪ Avoid if renal disorder and creatinine clearance <60ml/min ▪ Avoid if liver impairment ▪ Monthly creatinine trends ▪ Monitor proteinuria ▪ Monitor liver function monthly ▪ Stop if pregnancy	 Y/N Y/N Y/N Y/N Y/N Y/N	
Cardiology management:		
▪ All echocardiography and T2* is reviewed by an cardiologist	**1.** Annual examinations ☐ **2.** Bi-annual examination ☐ **3.** Not available ☐ **4.** Not practiced for all patients ☐	

	5. Patients go privately ☐	
Liver Disease:		
Diagnosis of HCV and/or HBV	**1.** Serology only ☐ **2.** Molecular diagnosis ☐	HCV RNA is important if positive serology. HBV DNA is needed to differentiate active from inactive infection
Treatment of Chronic hepatitis	**1.** Specialist consultation ☐ **2.** No specialist available ☐ **3.** Antivirals too expensive ☐	
Splenectomy	**1.** All patients ☐ **2.** Selected because of hypersplenism or spleen size ☐	Indicated if excessive blood consumption
Immunoprophylaxis Before splenectomy	**1.** Always done ☐ **2.** Not available ☐	Immunoprophylaxis: vaccination for Hib. Pneumococcus and Neisseria meningitides at least 2 weeks before op and 3-5 years after op
Antibiotics post-splenectomy	**1.** Always ☐ **2.** Not prescribed ☐	Prophylactic penicillin depending on age of the patient
Infections:		Infection related mortality is the second leading cause of death after heart disease
If on DFO temporary discontinuation if fever and prompt use of antibiotics	Y/N	To avoid enhancing bacteria such as Yersinia or Klebsiella. put patient on oral chelator
If on DFP check ANC	Y/N	If agranulocytosis stop DFP and use granulocyte stimulating factors as well as antibiotics
Possible septicaemia	Referral to ITU	
Endocrine disorders:		
Growth	**1.** Charts not used ☐ **2.** Charts in all children's' folders ☐ **3.** In some children's' folders ☐	Keep growth chart throughout childhood
Deformities recorded	Y/N	Keep record of deformities and start transufions with Hb minimum at 9g/dl to stop further changes: important for quality of life and self image.
Tanner scale for sexual development kept	Y/N	Tanner scale for sexual development is kept at the clinic from age 8-9 years. If delayed puberty request endocrine consultation
Endocrine deficiencies:		
Bone disease (osteoporosis)		

Annual BMD	**1.** DEXA not available ☐ **2.** DEXA annual ☐ **3.** DEXA biannual ☐ **4.** Bisphosphonates for 1 year and not longer than 2 years (preferably IV pamidronate or zolendronic acid) ☐ **5.** HRT when indicated by an endocrinologist ☐	DEXA will determine if osteopenia or osteoporosis. 40-50% of patients affected
Nutritional status assessed	**1.** Vitamin D levels measured every 6 months ☐ **2.** Vitamin D never measured ☐ **3.** Zinc levels measured every 6 months ☐	
Examined by endocrinologist	**1.** Annually from childhood ☐ **2.** Annually from 8-10 years ☐ **3.** Never ☐	
Hormone measurements	**1.** By clinic ☐ **2.** By endocrinologist only ☐ **3.** Never ☐	
General examinations:		
Chemistry panel (including blood urea, creatinine and LFTs)	**1.** Every 3 months ☐ **2.** Every 6 months ☐ **3.** Annually ☐ **4.** Never ☐	
Dental care	**1.** Annual dental checks for all patients ☐ **2.** Left to patients ☐	Increased risk of caries, periodontal disease and malocclusion. At least annual check up
Psychological support:		
Psychological wellbeing impacts on adherence to long term treatment and so survival	**1.** Provided by professional ☐ **2.** Provided by medical and nursing staff ☐ **3.** Provided by social worker ☐ **4.** Not provided ☐	Patients and families are vulnerable and may need professional help
Cognitive defects	Patients referred for neuropsychological tests Y/N	Selected cases
Approaches for psychosocial support	**1.** Changes in institutional practices ☐ **2.** Group sessions ☐ **3.** Family therapy ☐ **4.** Patient camps ☐	▪ Expert psychological support has to be available, tailored to children's needs, adolescents and adults ▪ Behavioural and social science approaches ▪ Holistic approach to the patient
Additional lifestyle:		
Physical activity	**1.** Encouraged for all ages ☐ **2.** Adults after ergometry and cardiac assessment ☐ **3.** Discouraged in all patients ☐	Ergometry and cardiovascular assessment is recommended
.Calcium & Vit D supplements provided	Y/N	Provide 2000IU vit D daily. Check Vit D levels every 6 months. Calcium

		rich diet: milk, cheese, fish etc
Folic acid	Y/N	1 mg/day for all patients with low Hb
Zinc supplements	Y/N	Monitor levels. For cases with deficiency due to chelation, poor growth and reduced BMD, supplements may be given. Usual dose 125 mg of zinc sulphate 1-3 times daily
Vitamin E rich diet recommended	Y/N	Eggs, vegetable oils
Vitamin C supplements		Recommended only with DFO infusions at 2-3mg/kg/day or in proven deficiency
Dietary iron restriction		Recommended for patients on low transfusion regimens since they absorb increased amounts from the gut
Smoking, alcohol, drug abuse		Prohibited!

Other functions of the centre:

Medical records		
	1. Available as paper files ☐ **2.** Available as electronic files ☐ **3.** Excel files only ☐	
Security of records	**1.** Implemented according to national law ☐ **2.** Transferred using encryption ☐ **3.** Transferred only to centres which also comply with security standards ☐ **4.** Staff trained for data protection ☐	Especially needed when networking
Storage	**1.** Patient records kept for lifetime ☐ **2.** Records kept for 15 years ☐ **3.** Records kept for 5 years ☐ **4.** Records kept only for the current visit ☐	
Traceability of records and samples	Codification is done: **1.** For traceability ☐ **2.** Confidentiality ☐ **3.** Established procedure to transfer data or samples ☐	
Ownership	**1.** Patient ownership ☐ **2.** Patient consent to create a record ☐ **3.** Patient consent to share a record ☐ **4.** Patient consent to use data for research ☐	
Research	**1.** Full explanation given to patients concerning a research project in which patient data are used ☐	

	2. Full explanation concerning volunteering for clinical trials, including risks and benefits ☐ **3.** Patient written consent for involvement in any research ☐	
Networks		
	1. Centre networks with secondary centres and regularly provides clinical/diagnostic support ☐ **2.** Centre networks with other reference centres in the country ☐ **3.** Centre networks with other international centres for clinical/diagnostic support ☐ **4.** Centre networks with other centres for research ☐	
Laboratory attached to centre	**1.** For diagnosis only ☐ **2.** For prevention ☐	
	1. Screening with basic haematology (indices, microscopy, HPLC, CE) ☐ **2.** Molecular support ☐ **3.** Prenatal diagnosis ☐ **4.** PGD ☐	
Genetic counselling	**1.** By Qualified counsellors ☐ **2.** By clinic doctors ☐ **3.** By nurses ☐ **4.** By laboratory staff ☐	
HSCT		
	1. Available in the centre ☐ **2.** Available in the country ☐ **3.** Patients referred abroad ☐	
	1. Supported by government funds ☐ **2.** Out-of–pocket totally ☐ **3.** Out-of-pocket partially ☐ **4.** Other support ☐	
Patient reported outcomes and expectations		
Quality of Life measures	**1.** Assessed by validated questionnaire Every few years ☐ **2.** Assessed only as part of a research project ☐ **3.** Has never been done ☐	
Questionnaires for patients' opinion	**1.** Occasional ☐ **2.** Never ☐	
Patients involvement	**1.** In advisory committees ☐ **2.** In clinical decision making that concerns them ☐ **3.** Encouraged in self-management ☐ **4.** Poor level of education so not involved ☐	Partnership model
Information/education for patients	**1.** Leaflets ☐ **2.** Seminars/workshops ☐ **3.** Encouraged to use electronic media (apps, website) ☐ **4.** None of the above ☐	
Clinic times	**1.** Clinics work mornings only ☐ **2.** Transfusions after working/education hours ☐ **3.** Weekend transfusions allowed ☐	
Patients rights		
Patients are informed of their rights	**1.** On admission ☐ **2.** Through written material ☐ **3.** Not considered ☐	Parents in the case of children

ABOUT HAEMOGLOBIN DISORDERS

According to the World Health Organisation (WHO)[3], around 7% of the global population carries an abnormal haemoglobin gene, while 300,000-500,000 children are born annually with clinically significant haemoglobin disorders. About 80% of children with these disorders are born in developing countries, 30% of which are affected by Thalassaemia Syndromes. Each year 50,000-100,000 children with thalassaemia major die in low and middle income countries. This is a growing problem in more than 70 countries, where 89% of the annual affected births take place. It is a common belief that although these numbers reflect the huge burden imposed by haemoglobin disorders, the reality and true picture is even more alarming: these numbers are grossly underestimated due to the absence of national, regional and international registries.

Haemoglobin disorders are indigenous and most frequently currently found in malaria endemic or previously endemic regions including South East Asia, the Middle East, Mediterranean countries, and Northern and Central Africa. However, as a result of the mass migration of populations through the years from high prevalence areas, haemoglobin disorders are today occurring widely across the world. Despite the great need to proceed with updated and/or upgraded epidemiological work, published data, mainly from the WHO, anticipate that around 7% of the world's population is a carrier of an abnormal haemoglobin gene. Altogether, the resulting diseases contribute significantly to the global and national toll of birth defects and disease burden and are associated with high mortality, morbidity and disability rates, the accurate extent of which can only be assessed if national registries and control programmes are in place in all high-prevalence countries.

It is estimated that haemoglobin disorders are a significant health problem in the majority of WHO participating countries, where about 3.4% of all deaths in children under the 5 years of age[4] occur.

Haemoglobin disorders are present in many countries with huge public health, social and economic repercussions. The introduction and implementation of national programmes for the effective control and management of haemoglobin disorders[5] is thus essential for preventing the disease and for promoting quality health and other care for the patients. The work of patients'/ parents' non-governmental organisations (NGOs), such as the Thalassaemia International Federation (TIF), is of paramount importance in supporting and complementing the work of governments and other official national, regional and international health-related bodies and organisations to achieve this.

ABOUT TIF

Thalassaemia International Federation (TIF) is the global umbrella organisation of 204 thalassaemia patients' associations from 62 countries across the world. TIF is in official collaborations with the World Health Organization (WHO) since 1996 and the United Nations Economic and Social Council (ECOSOC) since 2017 and is dedicated to promoting equal access to appropriate care for all patients with thalassaemia. Its extensive educational programme includes a series of educational publications as well as events aiming to spread awareness and promote effective control programmes across the world.

[3] Management of birth defects and haemoglobin disorders : report of a joint WHO-March of Dimes meeting, Geneva, Switzerland, 17-19 May 2006. Available from: http://apps.who.int/iris/bitstream/10665/43587/1/9789241594929_eng.pdf

[4] Modell, B. and Darlison, M.: Global epidemiology of haemoglobin disorders and derived service indicators - Bull World Health Organ. 2008 Jun; 86(6): 480–487.

[5] Guidelines for the Management of Transfusion Dependent Thalassaemia (TDT), 3rd Edition (2014)

MISSION: To develop and establish National Control Programmes for the prevention and management of haemoglobinopathies in all affected countries.

VISION: To help ensure equal access to quality health care for every patient with thalassemia and other haemoglobin disorders around the world.

OVERALL PURPOSE: To act as the global united voice of patients with haemoglobinopathies and particularly for patients with thalassemia.

VALUES:
(i) *Transparency, ethos, accountability, independence and patient centeredness*. Objectives, decisions, activities, actions including policy development, communication, and financial issues are governed by the above values, with the patients' benefit being the driving force.
(ii) *Health and social equity*: Fight for securing patients' rights for equal access to quality health and social services regardless of age, gender, ethnicity, political belief or cultural and religious convictions. The disparities and challenges existing within and across countries in all regions of the world including the EU countries with regards to thalassaemia are many.
(iii) *Improving knowledge and competence*: Creation of strong, united, competent patients' associations' to support disease education and establish strong voice for achieving meaningful involvement at a national, European, and International level.

TIF PUBLICATIONS

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- *Compliance to Iron Chelation Therapy with Desferrioxamine*, Eleftheriou A.
- *All About Thalassaemia* (2010), Eleftheriou A. (Cartoon Booklet).
- *Prevention Of Thalassaemias And Other Haemoglobin Disorders* Vol.1(2013) Old J, Traeger-Synodinos J, Petrou M, Galanello R, Angastiniotis M, Eleftheriou A,Harteveld C.
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- *Guidelines for the Management of non-Transfusion Dependent Thalassaemia* (NTDT) (2013), A Taher, E Vichinsky, K Musallam, M.D Cappellini, V Viprakasit.
- *Haemoglobin Disorders* (Haemoglobinopathies), Eleftheriou A, Angastiniotis M.
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