

# Medical Record

## A General Questions

Proposed Insured's Name:   
(Please use capital letters)

Birth Date:

Gender:

Address:

Email Address:

Phone Number:

Social Security Number:

ID Number:

Status:  Single  Married  Divorced  Others

Are you a retiree?  Yes  No

Occupation:

## 13. THALASSAEMIA REGISTRIES: A CALL FOR ACTION - A REPORT OF THE THALASSAEMIA INTERNATIONAL FEDERATION

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### EXECUTIVE SUMMARY

Registries are organised systems that use observational study methods to collect uniform data on individuals of a specific population defined by a disease, condition, or exposure, in order to serve a predefined clinical, scientific, administrative, or other purpose. They can be extremely powerful evidence-generating tools while providing a central meeting point for all implicated stakeholders, facilitating their networking and interaction.

Registries can play a major role in addressing the challenges that the care of thalassaemia patients is currently facing. By collecting updated and representative data on disease burden, features, management, and outcomes at local, national, regional, and global levels, thalassaemia registries may allow the evaluation and benchmarking of provided healthcare services, the detection of unmet clinical needs, and the identification of inequalities in healthcare delivery.

A total of 16 national thalassaemia registries have been in place since 1984, being characterised by heterogeneity and incomplete geographic coverage. Representativeness, interoperability, harmonisation, quality assurance, and sustainability are important features that thalassaemia registries should pursue.

The Thalassaemia International Federation aims at playing a key role in promoting the coordination and collaboration among existing thalassaemia registries and the establishment of new ones, with particular focus on areas of emerging economies. In this regard, TIF has undertaken the design, development, and implementation of a web-based platform to host a global thalassaemia registry.

### INTRODUCTION

In 2014, the Agency for Healthcare Research and Quality (AHRQ) defined registries as organised systems that use observational study methods to collect uniform data on individuals of a specific population defined by a disease, condition, or exposure, in order to serve a predefined clinical, scientific, administrative or other purpose [1].

Registries can be classified by various criteria such as their primary objective (public health, clinical, administrative, or product registries); geographical coverage (population or non-population-based and local, national, regional or international); drivers (physician- or patient-driven); data collection methods (paper or electronic-based); and administration schemes [2].

Registries can be extremely powerful evidence generating tools. They collect real-world data on the epidemiology, clinical characteristics, physical history, management; and outcomes of specific diseases and conditions. These pieces of evidence are valuable for the evaluation and benchmarking of provided healthcare services, the identification of unmet clinical needs and the detection of inequalities in healthcare. These information sources may in turn inform all implicated stakeholders to better plan their goals and activities. Stakeholders include governments and healthcare authorities, regulatory bodies, physicians, basic and clinical researchers, industry stakeholders, and patient organisations and advocacy groups. Registries can further

provide a central meeting point for all the aforementioned stakeholders, facilitating their networking and interaction [3].

In this context, the European Medicines Agency (EMA) has stressed the important role that registries may play in pharmacovigilance, regulatory decision-making, and post-authorisation monitoring. For this reason, the EMA launched in 2015 an initiative to make better use of existing registries and facilitate the development of quality new ones, promoting more systematic and standardised approaches that enhance the contribution of registries to the safety of medicines [4].

To serve the aforementioned purposes, registries should be populated by accurate, representative, prospective and usable data. The introduction and widespread distribution of electronic databases and digital healthcare records has considerably facilitated the development and maintenance of quality clinical registries through the use of integrated software solutions [5].

Registries are particularly important for rare diseases (RD). RD are defined as medical conditions with a prevalence of 1 in 2,000 or less; almost 80% of these disorders have a genetical origin [6]. Although RD collectively are not rare, as they affect 1 in every 17 people [6], the low prevalence of each individual disorder poses an important barrier for the collection of a critical bulk of evidence, which may in turn adversely affect the quality of healthcare provided to these people [3]. Registries are the ideal tool to overcome this barrier by ensuring pooling of data from different populations to create the necessary volume of evidence. The European Organisation for Rare Diseases, the National Organization for Rare Disorders, and the Canadian Organization for Rare Disorders (EURORDIS, NORD, CORD, respectively) defined in 2012 a list of 10 principles for the development of RD registries [7]. These principles are summarised in *Table 1*.

*Table 1. European Organisation for Rare Diseases, the National Organization for Rare Disorders, and the Canadian Organization for Rare Disorders (EURORDIS-NORD-CORD) principles for rare disease (RD) registries [7].*

1	RD registries should be recognised as a global priority.
2	RD registries should encompass the widest geographic scope possible.
3	RD registries should be centred on a disease or group of diseases rather than a therapeutic intervention.
4	Interoperability and harmonisation between RD registries should be consistently pursued.
5	A minimum set of Common Data Elements should be consistently used in all RD registries.
6	RD registries data should be linked with corresponding biobank data.
7	RD registries should include data directly reported by patients along with data reported by healthcare professionals.
8	Public-Private Partnerships should be encouraged to ensure sustainability of RD registries.
9	Patients should be equally involved with other stakeholders in the governance of RD registries.
10	RD registries should serve as key instruments for building and empowering patient communities.

## THE NEED FOR REGISTRIES IN THALASSAEMIA

### Disease Burden

Haemoglobinopathies, including thalassaemias and sickle cell disease, are inherited haemoglobin disorders, caused by genetically determined, quantitative, or qualitative impairment of the synthesis of globin chains, resulting in chronic haemolytic anaemia and other complications [8, 9]. Haemoglobinopathies constitute the most common monogenic disorders in humans. It is estimated that 5.2% of the world population carries a clinically significant haemoglobinopathy gene variant, while 1.1% of couples worldwide are at risk of having

children with a haemoglobinopathy [10]. Although highly prevalent and once confined to certain geographical areas, including sub-Saharan Africa for sickle cell disease and the Mediterranean Basin, Middle East, South and Southeast Asia for thalassaemia, migration of populations has distributed them globally (for more information see Chapter 15 Epidemiology).

Thalassaemias are caused by the reduced or depleted synthesis of the alpha ( $\alpha$ ) or beta ( $\beta$ ) globin chain that results in chronic anaemia in many cases. In thalassaemia major, anaemia is severe and requires repetitive blood transfusions for survival. Over time, complications arise in vital organs, which require regular monitoring and multidisciplinary care. Thalassaemia was once a fatal disease of childhood or adolescence but has now become a chronic manageable condition due to the therapeutic advances of the past decades and the systematisation of patient care. As a result, patients' survival, with access to modern therapy and monitoring, tends to reach that of the normal population [11]. In parallel, prevention programmes have limited the number of affected births [12]. Still,  $\beta$ -thalassaemia remains a very demanding medical condition, requiring intensive, multidisciplinary and lifelong care [8]. For more information see Chapter 2 Prevention, Chapter 5 Iron Overload, Chapter 6 MRI, and Chapter 7 Multidisciplinary Care.

Thalassaemia patients and their families, along with the many different healthcare professionals involved in their management, currently face a number of important challenges. These challenges are described briefly below.

## Current Challenges

The complexity of thalassaemia clinical outcomes and care makes for complex clinical records, for which an electronic recording system is ideal. Patient ageing also requires data collection over long periods of time, which again requires an electronic system for storing and processing.

Equal access to modern systematic therapy and monitoring for all thalassaemia patients across the world remains the primary unmet need. Despite the global distribution of the disorder, the majority of thalassaemia patients are born and live in areas of emerging or developing economies [10], where disease management programmes are often absent or suboptimal. As a result, patients living in these areas are deprived of at least some, if not all, of the important pillars of current thalassaemia management including regular blood transfusions, chelation therapy, iron overload monitoring, multidisciplinary care, and others, which adversely impacts their prognosis and survival. For more information, see Chapter 14 Unmet Needs.

The global epidemiology of thalassaemias is rapidly changing due to the current refugee crisis. Many patients originating from high-prevalence areas of the Middle East, Southeast Asia, and Africa have migrated or are in a process of migration to European countries. As a result, the number of patients with haemoglobin disorders located in Europe is significantly increasing. However, haemoglobinopathies in Europe fall within the official EU definition for RD and thus the prioritisation of thalassaemias in national health agendas remains low. In this context, TIF has launched the THALassaemia In Action (THALIA) project, funded by the EU Commission, to promote prioritisation of thalassaemia in national healthcare plans and policies in Europe [13].

The absence of adequate disease management programmes in several high-prevalence regions is coupled with the lack of systematic patient recording. This leads to insufficient epidemiological data on disease prevalence, clinical features, incidence of complications, management, and outcomes. The existing evidence on the current global and regional burden of the disease remains suboptimal. This in turn precludes the accurate description of unmet needs, which further prevents proper adaptation of healthcare policies, research, and advocacy to address these needs.

As previously stated, advances in therapeutic and monitoring modalities and the systematisation of care accomplished over the past decades have considerably improved the prognosis and survival of thalassaemia

patients. As a result, patients having access to organised disease management programmes have entered a new era of prolonged survival. This accomplishment, however, comes at the cost of a changing clinical spectrum with increasing incidence of age-related disorders that were once unknown to thalassaemia populations [11]. The evolving clinical spectrum of the disease requires proper adaptations to management programmes that need to take into account the risk of conditions related to ageing.

Motivated by the therapeutic advances of the past decades that have dramatically changed patients' prognosis, the thalassaemias remain a field of rigorous research. Novel drugs that improve patient outcomes have lately been approved or are under investigation. Luspatercept, a recently FDA/EMA authorised drug, has been shown to improve ineffective erythropoiesis, the hallmark of thalassaemia's pathophysiology, thus leading to improved haemoglobin levels and significantly reduced transfusion needs in a phase III clinical trial [14], and based on this the drug has gained approval and is in clinical use.

Other new therapies are in an earlier stage of development [15]. Besides new drugs for disease management, a fascinating new era of cure may emerge by virtue of the recent advances in gene therapy [16]. However, even if they are cost-effective in the long term, new therapies increase significantly the immediate costs of disease-related healthcare. Given the high cost of these new additions, coverage by healthcare systems is not secured, even in high-income countries. Registries can help in patient selection and best utilisation of resources.

The provision of regular care that is vital for patients' survival and wellbeing, such as blood transfusions, should not be interrupted or jeopardised as a result of emergencies such as the COVID-19 pandemic, with reallocation of healthcare resources. Therefore, the proper adaptation of care during the current and potential future public health emergencies is crucial [17].

## Expected Benefits from Registries

Registries can play a major role in addressing the aforementioned challenges. They constitute the ideal tool for generating accurate and updated data on disease burden, features, management, and outcomes at local, national, regional, and global levels. Collected data may concern the prevalence of the disease, the demographic and clinical characteristics of patients, the natural history of the disease (including the incidence of complications and the prevalence of comorbidities), the applied therapeutic and monitoring modalities, the organisation of care, and patient outcomes (including both hard clinical endpoints but also patient-reported outcomes). These pieces of evidence allow the evaluation and benchmarking of quality and appropriateness of provided healthcare services; the detection of insufficiently covered clinical needs, including unaddressed comorbidities or complications, but also the degree of adherence to recommendations; and the identification of inequalities in healthcare delivery.

Registry derived evidence would provide valuable feedback to all implicated stakeholders. More specifically, governments, healthcare authorities, and policymakers will be able to understand the need to prioritise thalassaemia care, to gauge the performance of thalassaemia care, to plan improvements to provided services, and to balance the allocation of resources. Physician associations and scientific bodies would be facilitated in developing clinical practice guidelines and plan training programmes for physicians, but also in issuing proper recommendations addressed to healthcare authorities, researchers, and the pharma industry [3].

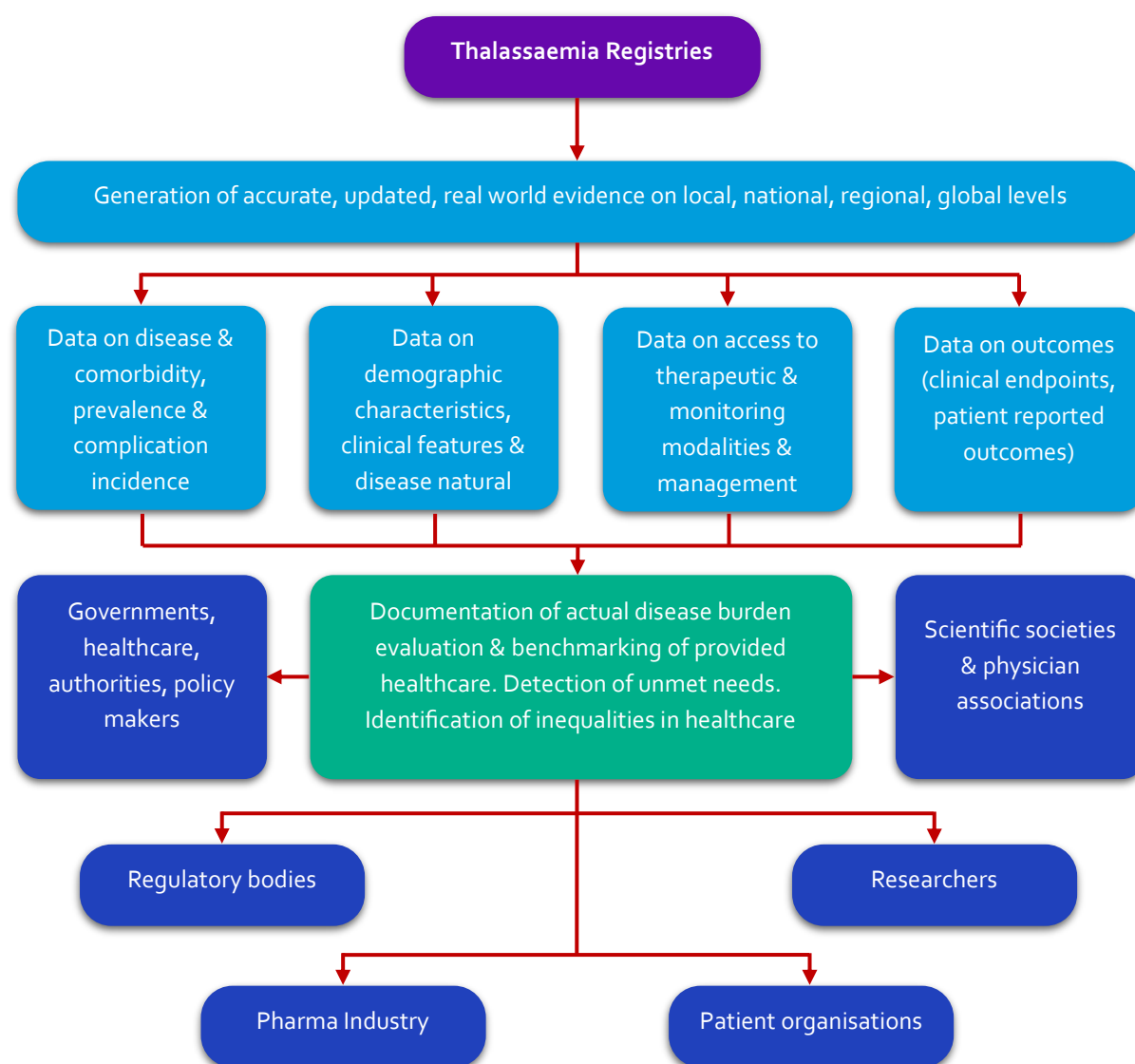
Regulatory authorities would be able to prioritise drug approval in the field of thalassaemia and organise more efficient post-approval pharmacovigilance programmes. The pharma industry would be able to prioritise investments in the demanding field of thalassaemia. Researchers would be provided with updated epidemiological evidence and large sample sizes to analyse, while they would be further facilitated in the generation of clinical hypotheses for new therapeutic targets, drugs, and interventions; and be supported in

the conduct of clinical trials by the low cost, rapid enrolment, efficient follow-up and enhanced generalisability of results that registry-based trials provide [2, 3, 18].

Last but not least, patient organisations and advocacy groups would have the necessary evidence at hand to better advocate patient needs, but also to promote the education of patients and families. Well-informed patients and families are empowered to better handle the disease, help other patients, and claim the improvement of their care. The endpoint is the improvement of patient outcomes through the enhancement of healthcare quality. Table 2 summarises the expected benefits from thalassaemia registries and Figure 1 depicts the central role of registries in the interactions among all stakeholders implicated in the healthcare of thalassaemia patients.

*Table 2. Main areas and issues related to thalassaemia healthcare where registries are expected to contribute*

<b>Epidemiology</b>	<ul style="list-style-type: none"> <li>• Current disease burden at local, national, regional, and global levels</li> <li>• Changing epidemiology due to current refugee crisis and migration flows</li> </ul>
<b>Organisation and provision of care</b>	<ul style="list-style-type: none"> <li>• Evaluation and benchmarking of provided healthcare services</li> <li>• Update of healthcare services, reprogramming of resource allocation</li> <li>• Changing clinical spectrum due to ageing</li> <li>• Unmet needs in developing/low-income countries (e.g., prevention programmes, access to regular transfusions and iron chelation, established multidisciplinary care, and social support)</li> <li>• Unmet needs in medium/high-income countries (e.g., access to latest developments, access of refugees and migrants to care)</li> </ul>
<b>Research</b>	<ul style="list-style-type: none"> <li>• Research collaboration, large sample sizes</li> <li>• Generation of research hypotheses, identification of treatment targets</li> <li>• Randomised trials with low cost, rapid enrolment, efficient follow-up &amp; increased generalisability</li> </ul>
<b>Advocacy</b>	<ul style="list-style-type: none"> <li>• Unmet needs in developing/low-income countries</li> <li>• Unmet needs in high-income countries</li> </ul>
<b>Public health emergencies &amp; crises</b>	<ul style="list-style-type: none"> <li>• Implications for thalassaemia patients</li> <li>• Proper &amp; timely response of healthcare providers and authorities</li> </ul>



*Figure 1. Central role of thalassaemia registries in the interactions among all stakeholders implicated in the healthcare of thalassaemia patients*

## Existing Thalassaemia Registries

In a study of thalassaemia registries across the world, published in 2019, Noori and colleagues were able to identify 16 different thalassaemia registries, developed between 1984 and 2016 [19]. The majority of these registries are national, while only one is multinational and two are regional. Most of the identified registries rely on government funding, while a small number receive funding from industry. Six of 16 registries are focused on thalassaemia, while the rest also include patients with sickle cell disease and other haemoglobinopathies. In contrast to the geographical distribution of thalassaemia, the majority of these registries are based in European countries, while the authors were not able to identify any thalassaemia registry in Africa. Recently, the Sickle Pan Africa Research Consortium (SPARCO) reported results from the first African registry on sickle cell disease in three countries, Nigeria, Ghana and Tanzania [20]; however, this registry does not include thalassaemia patients, in accordance with the epidemiology of haemoglobinopathies in Sub-Saharan Africa. In addition to the above registries, we were able to identify another one in Malaysia [21].

The authors of the aforementioned report identified a considerable heterogeneity among the existing thalassaemia registries [19]. They serve diverse objectives and goals; they use various data sources and types; they rely on different information, standardisations, and management systems; they are governed by diverse models; and they have been developed in different languages. Table 3 summarises the features of the existing thalassaemia registries.

*Table 3. Existing thalassaemia registries (Modified from NooriT et al. [19])*

Registry	Country or Region	Geographical Coverage	Disease Coverage	Launch Year
<b>Europe</b>				
Sicilian Registry Thalassaemia & Haemoglobinopathies (ReSTE)	Sicily, Italy	Local	Thalassaemia, SCD, Other	1984
European Haemoglobinopathy Registry (HER)	UK/Europe	Regional	Thalassaemia, Other	2004
National Registry for Thalassaemia	France	National	Thalassaemia	2005
Italian Multiregional Thalassaemia Registry (HTA-THAL Registry)	Italy	National	Thalassaemia	2008
National Haemoglobinopathy Registry (NHR)	UK	National	Thalassaemia, SCD	2009
National Registry of Patients with Thalassaemia in Greece (NRHG)	Greece	National	Thalassaemia, SCD, Other	2009
National Registry of Patients with Thalassaemia in Bulgaria	Bulgaria	National	Thalassaemia	2009
Turkish Society of Pediatric Hematology National Hemoglobinopathy Registry	Turkey	National	Thalassaemia, SCD	2012
Spanish Registry of Haemoglobinopathies (REHem)	Spain	National	Thalassaemia, SCD	2014
<b>Asia</b>				
National Thalassaemia Registry (NTR)	Singapore	National	Thalassaemia	1992
National registry of Symptomatic Haemoglobinopathies	Oman	National	Thalassaemia, SCD	2000
Malaysian Thalassaemia Registry	Malaysia	National	Thalassaemia	2007
Pediatric Non-Malignant Blood Disorders Registry	Saudi Arabia	National	Thalassaemia, SCD	2008
Electronic Thalassaemia Registry Mazandaran (ETR Mazandaran)	Mazandaran Province, Northern Iran	Local	Thalassaemia	2016
Punjab Thalassaemia Prevention Programme (PTPP), Ministry of Health Punjab	Punjab, Pakistan	National	Thalassaemia	2022
<b>North America</b>				
Registry and Surveillance System for Hemoglobinopathies (RuSH)	USA	National	Thalassaemia, SCD	2010
Data Information System for Hemoglobinopathies (DISH)	Canada	National	Thalassaemia, SCD	2014
<b>Oceania</b>				
Haemoglobinopathy Registry (HeR)	Australia	National	Thalassaemia, SCD, Other	2014
*SCD: Sickle Cell Disease				

## Comments

The paediatric non-malignant blood disorders registry in Saudi Arabia is not, at this time, functioning as a national registry according to a virtual meeting with haematology experts from Saudi Arabia in November 2022.

Thalassaemia support associations in some countries have developed their own registries. In Indonesia the Yayasan Foundation has attempted to cover the whole country. In Bangladesh the Thalassaemia Foundation Hospital has an internal registry, which in 2021 included 4,373 patients (3,042 have Hb E beta thalassaemia and 890 have beta thalassaemia). These may evolve to national registries with governmental support.

One approach suggested by a team from the National Center on Birth Defects and Developmental Disabilities, CDC, USA, is to extract useful information from administrative healthcare data sets as a unique means to study healthcare use among people with SCD or thalassaemia because of the ability to examine large sample sizes at fairly low cost, resulting in greater generalisability than is the case with clinic-based data. Such datasets include:

- Hospital data sets, which include data on hospitalisations, emergency department and ambulatory clinic data, as well as inpatient records.
- Health insurance claims databases, since both public and private health insurance plans maintain detailed records on healthcare use and expenditures.

Administrative data have both strengths and limitations for health services research on hemoglobinopathies and other blood disorders. The main advantages are the large numbers of observations and the low cost of data acquisition. However, researchers are unable to validate information in diagnoses by referring to medical records. The diagnostic codes used for administrative purposes might not meet the diagnostic criteria used for surveillance case definitions, codes for underlying conditions might not be reported, or codes might be applied incorrectly by those recording data. Nevertheless, these datasets provide a source for better understanding of the magnitude of the public health and economic effects of these conditions [22].

## THALASSAEMIA REGISTRIES: A CALL FOR ACTION

### Challenges of Thalassaemia Registries

The amount and complexity of data that are collected for each thalassaemia patient and the length of time that records are kept render the definition of the exact use of each registry and the type and amount of data to be included quite challenging. The existing thalassaemia registries are characterised by considerable heterogeneity. These inconsistencies may pose significant barriers to the interoperability of registries, the sharing and pooling of data, and the overall coordination of similar concurrent initiatives, as well as the future re-use of data for different purposes, including administration, planning, research, and advocacy. In addition, the provided geographic coverage is incomplete, as most registries are based in European countries.

It is important that registries provide longitudinal data in a continuous manner, which allows the provision of updated evidence and the identification of trends in disease epidemiology, management, and outcomes. An additional challenge that thalassaemia registries may face in this regard is their long-term sustainability, which is directly related to the sources of funding. Hitherto, thalassaemia registries have been funded mostly by governments, with considerable workloads being covered on a volunteer basis, often by clinic staff [19]. Therefore, continuation of public funding and voluntary work is crucial, while complementary sources of financial support should be attracted in parallel by contacting other public or private bodies, humanitarian organisations, or the pharmaceutical industry. The particularly active thalassaemia patient organisations and advocacy groups have to play an important role in this regard.

Technical challenges are also important. Pooling data from different registries is necessary to reach large numbers of patients and allow comparisons among different populations. This is particularly relevant as several registries are already in place in different parts of the world. At the same time, linking registries with existing electronic medical records and administrative healthcare databases would ensure exploitation of large amounts of representative data at a low cost and workload while avoiding inaccuracies in data entry. However, linking data from different databases with diverse language, structure, and software protocols requires proper technical support and collaboration [23]. As previously stressed, the existing thalassaemia registries are quite heterogeneous.

Ethical issues are also involved and should be taken under consideration when designing and implementing a registry. All patients enrolled in a registry should give their consent for their data to be collected, recorded, stored and processed, and potentially shared, after being fully informed about the aims of the registry. At the same time, confidentiality and safety of data should be ensured by applying the proper protocols in compliance with national and international regulations for the protection of personal data. For example, the recent European Union Data Protection Regulation has set clear principles that apply to all use of patients' data and to all data controllers (Article 5 of the regulation) [24].

## Harmonisation and Quality Assurance

In an effort to address the heterogeneities of existing registries and to ensure the overall quality of registries, the Italian National Center for Rare Diseases has recently issued a list of recommendations [2]. These recommendations constitute a quality assurance framework and concern nine sequential areas of activities including (i) governance, (ii) data sources, (iii) data elements, (iv) information technology (IT) infrastructure, (v) data quality, (vi) information, (vii) documentation, (viii) staff training, and (ix) audit [2]. These recommendations are particularly relevant for thalassaemia registries and provide a solid ground for their harmonisation and quality assurance. Table 4 provides an overview of these recommendations.

*Table 4. Recommendations of the Italian National Centre of Rare Diseases for quality assurance and management of registries [2]*

Main areas	Activities
<b>1</b> Governance	<ul style="list-style-type: none"> <li>• Define registry objectives</li> <li>• Determine database structure</li> <li>• Involve all relevant stakeholders</li> <li>• Build a registry team</li> <li>• Ensure compliance with relevant ethical &amp; legal rules</li> <li>• Ensure start-up budget and complementary sources</li> </ul>
<b>2</b> Data source	<ul style="list-style-type: none"> <li>• Select primary &amp; secondary data sources</li> <li>• Define inclusion/exclusion criteria</li> <li>• Ensure representativeness</li> </ul>
<b>3</b> Data elements	<ul style="list-style-type: none"> <li>• Define data types according to objectives</li> <li>• Determine what data from each source</li> <li>• Build proper case report forms</li> <li>• Ensure use of standardised systems (e.g. disease classification, phenotype descriptions)</li> </ul>
<b>4</b> IT infrastructure	<ul style="list-style-type: none"> <li>• Ensure compliance with FAIR principles:                             <ul style="list-style-type: none"> <li>○ Findable</li> <li>○ Accessible</li> <li>○ Interoperable</li> </ul> </li> </ul>

Main areas	Activities
	○ Reusable
5 Data quality	<ul style="list-style-type: none"> <li>• Establish regular central and local monitoring</li> <li>• Programme regular quality reporting</li> </ul>
6 Data information	<ul style="list-style-type: none"> <li>• Define statistical analysis plan</li> <li>• Ensure dissemination to all stakeholders</li> </ul>
7 Documentation	<ul style="list-style-type: none"> <li>• Determine documentation to ensure transparency</li> </ul>
8 Training	<ul style="list-style-type: none"> <li>• Ensure systematic training of: <ul style="list-style-type: none"> <li>○ Registry staff</li> <li>○ Data providers</li> </ul> </li> </ul>
9 Audit	<ul style="list-style-type: none"> <li>• Develop an audit system</li> <li>• Define red flags and audit triggers</li> </ul>

## New Registries

**Pakistan (Punjab)** the first Electronic Medical Registry of thalassaemia of patients living in the Punjab is now established. This was created in collaboration with the Specialized HealthCare & Medical Education Department of the Ministry of Health of Punjab and with the help of the Punjab Information Technology Board (PITB). It includes data from 64 treatment centres (government and NGO run) in nine regions of the Punjab. The registry so far includes 12,536 patients out of an estimated total of 21,431 (58.5%). The package includes a Registration Module (demographics), a Clinical Module, and a treatment module.

## CONCLUSION

### TIF's e-Registry Initiative

Umbrella organisations like TIF have a crucial role to play in the coordination of different registry initiatives. Such an organisation can bring together existing thalassaemia registries across the globe; establish registry standards to support cohesion and interoperability across registries; develop strategies to attract sustainable funding from government and other public or private sources to support the existing and promote the development of new registries, with particular focus on areas and countries of emerging economies; and to promote the use of registry data for translational research to address current gaps in knowledge and improve patients' care [3]. In addition, patient-driven organisations such as TIF can ensure that patients will play an important role in the development and exploitation of disease registries by incorporating data related to patients' perspectives, such as patient-related outcomes, as well as the use of registry findings to inform and support patients' advocacy.

TIF has long recognised the need for a well-coordinated initiative towards the development of an international, truly representative and quality thalassaemia registry, as well as the role that an umbrella organisation has to play in this regard. In this context, TIF has previously contributed to the development of an electronic registry system for the purposes of the European Network on Rare Anaemias (ENERCA) that aims serve as an epidemiological tool to improve the management of patient services and ultimately improve patient care [25]. Later on, TIF conceived and launched a project for the design, development, and implementation of an electronic, web-based platform to host a global thalassaemia registry. The specific aims of this project are defined as follows:

- To generate reliable national and international epidemiological data.
- To identify and document the unmet clinical needs regarding the care of patients with haemoglobinopathies in order to plan and improve the provided services.
- To provide a structured approach to taking a patient's history, conducting a physical examination, and evaluating a haemoglobinopathic patient in order to enhance the clinical management of the disease.
- To allow the efficient sharing of records within a thalassaemia unit or between disparate units, thus enhancing physicians' interaction to improve healthcare provision.
- To promote and coordinate multidisciplinary care.
- To provide a platform for national and international networking for clinical and research purposes.
- To enhance physicians' supervision and auditing.
- To address medico-legal and ethical issues.
- To promote awareness, education, and training on haemoglobinopathies.

TIF's e-Registry aims at recruiting consecutive patients treated in thalassaemia centres across the world. To perform this task, TIF designed and developed a dedicated proprietary web-based electronic platform that combines the features of a structured electronic medical record and the features of a medical database for data processing and research use. This application complies with international security and data safety protocols regarding web-based electronic medical records and web-based medical databases, also safeguarding subject anonymisation. To ensure widespread access, this application can be accessed with common web browsers on PCs, while an additional application for portable devices will be developed at a later stage. A pilot-phase project for the testing of the platform has already been launched. Feedback from this pilot project will be used to evaluate and update the platform structure and functions.

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