Sustaining Patient Registries in Ireland

Future of Registries Taskforce Recommendations

PREPARED BY

FoRT
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Contents

Common abbreviations

ABBREVIATION	EXPLANATION
CDM	Common Data Model
CFRI	Cystic Fibrosis Registry of Ireland
DASSL	Data Access, Storage, Sharing and Linkage
DoH	Department of Health
DPO	Data Protection Officer
EEHRxF	European Electronic Health Record Exchange format
EHDS	European Health Data Space
EHR	Electronic health record
ERNs	European Reference Networks
EUCERD	EU Expert Committee on Rare Diseases
FAIR	Findable, Accessible, Interoperable, Reusable
FoRT	Future of Registries Taskforce
GDPR	General Data Protection Regulations
HDAB	Health Data Access Body
HIQA	Health Information Quality Authority
HRB	Health Research Board
HSE	Health Service Executive
IHI	Individual Health Identifier
ILD	Interstitial Lung Disease
ILFA	Irish Lung Fibrosis Association
MDI	Muscular Dystrophy Ireland
NCRI	National Cancer Registry of Ireland
NISR	National & International Skin Registry Solutions
NREC	National Research Ethics Committee
OMOP	Observational Medical Outcomes Partnership
OpenEHR	OpenEHR is an open standard specification in health informatics that describes the management and storage, retrieval and exchange of health data in electronic health records
PESTL	Political, economic, sociological, technological, legal
PPI	Public/patient involvement
REC	Research Ethics Committee

Summary

Patient registries - a critical lifeline for Ireland's Healthcare Future

Patient registries are more than just electronic databases; they are tools to transform real-world clinical, social, and patient-reported data into actionable insights. A registry is an organised system that collects standardised data over time using observational methods to collect data for a defined population (e.g., by disease, condition, or treatment). Registries can support research, public health, health care planning and delivery, policy decision-making, and inform clinical practice, differing from electronic health records (EHRs) by focusing on longitudinal data for specific scientific or policy purposes rather than individual patient care.

Through statistical analysis, registries identify patterns in clinical, epidemiological, and social data over time, guiding healthcare providers and policymakers in making informed decisions. As centralised repositories of information on specific diseases, registries enable better understanding and management of health conditions at a population level. Registries are crucial in demonstrating the real-world effectiveness of new treatments or public health initiatives, facilitating healthcare planning and delivery, and enabling clinical trial access. Without them, patient care and outcomes, research and development and our healthcare system suffer.

Ireland's registries play a significant role in collecting and leveraging patient data for research, policy, and practice. Many existing Irish registries have over a decade (some 20 years+) experience collecting, digitising, and using data to inform research policy and practice as well as contributing data to international registries, enhancing patient access to therapies, clinical trials and global evidence bases.

The Future of Registries Taskforce (FoRT) is a multi-stakeholder group, led by members of the Cystic Fibrosis Registry of Ireland and National and International Skin Registry Solutions, with over 50 members dedicated to securing the future of patient registries in Ireland. In 2024, the group met four times on a pro-bono basis collating evidence from expert opinion, a registry survey, and registry case studies to identify the challenges facing Irish registries and develop recommendations for their sustainability.

Challenges and opportunities for registries in Ireland

Despite their critical importance, Irish registries face several challenges:

- Many registries operate on self-sourced funding, investing millions with limited, or no, exchequer support.
- While excelling in research and patient engagement, more can be done with registry data and Ireland lags behind in data linkage and integration compared to other countries.
- Key registry barriers include limited core funding, fragmented ethics and data protection systems, challenges in accessing and maintaining expertise, and difficulties in procuring and maintaining a cost-effective registry database technology.

These challenges hinder long-term planning and the sustainability of registries.

Continuing with the current fragmented approach will limit the long-term impact of Irish registries on research, policy, and healthcare practice. Registries are a source of secondary health data and the European Commission is mandating the use of this data to slingshot pan-European research and meet the requirements of the European Health Data Space.

The standardisation and integration of registries across Ireland will exponentially increase the value of registries. The implementation of this proposal will provide the opportunity to enhance patient care, streamline healthcare processes, support healthcare research and positively impact the economy.

Proposed Solution – creating a sustainable and integrated registry infrastructure

To safeguard and enhance Irish registry data and comply with the European Health Data Space (EHDS) regulations, a national, sustainable approach to patient registries is needed. FoRT recommends that the Department of Health collaborate with registry stakeholders to do the following:

1. Develop and implement a Proof-of-Concept Model for the sustainability of registries

A pilot study should assess the feasibility of a sustainable registry model and ensure compliance with EHDS regulatory requirements. The proposed pilot would involve two phases:

Phase 1 (6-9 months): Collaborate with the Department of Health and stakeholders, to create a detailed plan and budget. This collaboration will be important to ensure project financing is secured but also to ensure the proposed project can, as far as possible, be designed to meet the needs and expectations of key stakeholders.

Phase 2 (Minimum 3 years): Pilot study implementation across selected registries (minimum 5), testing a new model of sustainability through a public-private partnership (PPP), where costs are shared between registries, the Department of Health, and industry partners.

The pilot study would focus on:

- **Implementing a common data model** to standardise data capture and enable integration across registries and health systems.
- **Developing a scalable, cloud-based registry database** to enhance data management, interoperability with electronic health records (EHRs), and reduce existing and future operational costs.
- **Establishing standardized governance structures and procedure**s to ensure compliance with ethical and regulatory guidelines and build trust with patients.
- **Pooling human resources and expertise** (e.g., statistical, data protection, ethics expertise) to sustain registry operations efficiently.

2. Engage with and leverage FoRT as a Community of Practice

Communication and expertise are vital in health informatics. The Department of Health should engage with and utilise FoRT's expertise to sustain communities of practice that will:

- Support the adoption of **internationally accepted standards** such as OpenEHR, Observational Medical Outcomes Partnership (OMOP), and the new European Electronic Health Record Exchange format to bridge the gap between EHRs and registries.
- Ensure registry representation in **stakeholder groups** implementing the EHDS regulation and the National Electronic Health Record system.
- Advocate for a **centralised ethical approval process** for registries and improved guidance for local ethics committees and data protection officers.

Expected Impact

By adopting a harmonized, sustainable approach, Ireland can:

- Strengthen its clinical trial and real-world evidence research landscape by providing standardised and accessible registry datasets.
- Ensure registry readiness for EHDS compliance and the secondary use of data in Ireland and internationally
- Exploit the experience of Irish registries in digitising and analysing patient data in Ireland for over 20 years (before the introduction of EHRs)
- Leverage the implementation of a National Electronic Health Record by making registry data more accessible and useful for research, policy and practice.
- Advancing the use of data linkage and combined analytics

Call to Action

If the future of registries is not secured, patient care and the Irish healthcare system will suffer. Registries are more than data repositories and EHRs—they are lifelines that drive innovation and improve healthcare outcomes. Without sustainable registries, Ireland risks inefficient and unproductive use of public funds in healthcare and diminished patient access to essential treatments and care. Immediate action is needed to secure the future of registries, ensuring they continue to provide critical insights that save lives and improve healthcare in Ireland and to comply with the European Commission's regulation on the secondary use of health data.

The FoRT group call on the Department of Health to action the above proposed solutions by working together with the view to developing an innovative and cost-effective way of handling a vital component of Ireland's secondary health data that will support the Government with compliance with the European Commission's EHDS and ultimately that will have a transformative impact for Irish healthcare and for Ireland.

Introduction

Patient registries are an essential component of improving patient outcomes and advancing care through the secondary use of data. Patient registries collect accurate, valid, reliable and timely information about a particular patient population or condition over time. Such information can inform research, policy, clinical audit, health service planning, and can also be used to monitor and improve the treatment and care of individuals[1].

This document presents a set of key recommendations to the Department of Health to secure the future sustainability of patient registries in Ireland. The document has been developed by the Future of Registries Taskforce (FoRT), which is a multistakeholder taskforce with membership reflective of the registry, health system, health information, industry and wider health research landscape, led by members of the Cystic Fibrosis Registry of Ireland (CFRI) and National and International Skin Registry Solutions (NISR). This document aims to give a sense of the national landscape which registries currently operate in, their value, and a vision for their future.

The document reports on the work of the taskforce and outlines the registry landscape in Ireland. The findings from a survey of registries and registry case studies highlight the distinct value, impact and success registries have on improving patient outcomes and care but, conversely, also their challenges including sustaining operations. It outlines key asks to establish a future sustainable model for patient registries in Ireland, providing a strong case with recommendations for future support. Registries help to ensure patients receive the best care possible and improve health outcomes in the long-term. Registries are a source of secondary health data and the European Commission is mandating the use of this data to improve pan-European research. We have the opportunity to exponentially increase the value of Irish registries in the process of meeting the requirements of the European Health Data Space.

1. https://hrci.ie/unlocking-the-potential-of-patient-registries-a-guide-for-success/

01 Defining a patient registry and its role in the healthcare system

A patient registry is defined as:

"…an organised system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves one or more predetermined scientific, clinical, or policy purposes"[2].

There are several different types of registries outlined in Table 1. For the remainder of this document, 'patient registries' is used as a catch-all term to refer to registries within the scope of FoRT work as detailed in Table 1.

Table 1: Types of patient registries

Type of Registry	Definition	Within FoRT scope
Disease/condition registries (including rare diseases)	Registries collecting uniform data relating to a population with a particular diagnosis of a disease/condition	✔
Patient population registries	Registries collecting uniform data relating to a population predefined by some particular health characteristic	✔
Treatment/procedure registries	Registries collecting uniform data relating to a population who have undergone a particular procedure or treatment	✔
Health services/clinical audit registries	Registries collecting uniform data relating to a population having received a pre-defined service	✔
Medical device registries	Registries collecting uniform data relating to a population who are utilising a particular medical device and companion diagnostics or technology	✖[3]

Registries are designed to gather data over time to support various healthcare objectives such as:

i. monitoring disease progression,
ii. evaluating treatment outcomes or public health initiatives,
iii. improving patient care,
iv. service planning, management and evaluation,
v. quality assurance,
vi. facilitating clinical research and audit,
vii. and informing public health policies.

2. Registries for Evaluating Patient Outcomes: A User's Guide: A User's Guide, 4th Ed. 2020, Agency for Healthcare Research and Quality, https://effectivehealthcare.ahrq.gov/products/registries-guide-4th-edition/users-guide

3. Medical device registries are outside of the scope of FoRT work because they are considered to have a wider scope of purposes than patient registries and deal with the safety of the device not necessarily linked with health outcomes medical devices are regulated in the European Union under Regulation (EU) 2017/745 and In Vitro Diagnostic Devices under Regulation (EU) 217/746.

Patient registries can also be used to track clinical practices, understand the natural history of diseases, and identify patient populations for clinical trials or targeted interventions. Data collected in a patient registry typically includes demographic information, medical history, diagnostic test results, treatment details, clinical outcomes, and often patient-reported outcomes. Moreover, whilst biobanks and registries are ordinarily independent from each other, there should be consideration on how registries and biobanks can interface to improve knowledge. In theory, all registries would have a common set of operational requirements and methodologies to support consistency in, and meet guidelines under the European Health Data Space (EHDS) regulation for the secondary use of data. Registries will fall under this EHDS defined secondary use of data and as such will require member-state community compliance.

Patient registries are often confused with electronic health records (EHRs). While both involve the collection of health-related patient data, they differ in terms of purpose, structure, scope and use. Patient registries are often focused on collecting longitudinal patient data for the purposes of specific research, public health and/or policy objectives whereas EHRs are focussed primarily on facilitating the delivery and improvement of care to individual patients. In the ideal world, these two sources of data are complimentary to and interoperable with one-another, i.e., when electronic health records are freely available, registry pertinent data could be extracted and imported into a registry platform.

More widely, patient registries are distinct from other health datasets in terms of their purpose and focus, data content and structure, data collection methods, and intended use.

a. **Purpose and focus** – patient registries are usually developed with a specific purpose and focus in mind. That might be to collect longitudinal data on a particular patient group/disease to inform research, healthcare planning, delivery and quality improvement, or to inform policy.

b. **Data content and structure** – the content and structure of patient registries are usually standardised according to the purpose and focus of the registry as well as to any clinically or internationally defined sets of variables.

c. **Data collection methods** – data collection methods in a patient registry are usually predefined protocols and can involve collection of data from patient charts, EHRs, patient self-reported measures and healthcare providers. Methodologically patient registries are observational studies, as opposed to clinical trial methodologies which often involve intervention and prescriptive trial conditions. Data collection is ideally facilitated via the use of an online registry technology platform/database.

d. **Intended use** – the intended use of registry data is usually defined by its purpose but can involve the use of registry data to support research, clinical practice, quality improvement and to inform/develop policy.

Types of registries in Ireland

There are a large number of existing patient registries in Ireland operating in different capacities. For example, the National Cancer Registry of Ireland (NCRI) was established as a public body, primarily funded by the Department of Health. Other registries, e.g., CFRI/Alpha1, operate in the voluntary sector, funded by a mixture of grant funding, public funding, fundraising, and industry support. Naturally, some registries are based within the Health Service Executive, sometimes operating directly out of an individual hospital/group of hospitals.

Irish and European Rare Disease registries

Rare diseases are defined as having a prevalence of fewer than five cases for every 10,000 persons[4]. Though individually rare, collectively they affect 30 million people in Europe and an estimated 300,000 people or around 6% of the population in Ireland. There are over 8,000 known rare diseases. Small and geographically dispersed patient populations coupled with scarce clinical knowledge and experience mean that conducting rare disease research, running rare disease registries, and conducting clinical trials is particularly challenging.

4. Low prevalence means rare diseases affect a small portion of the population; less than 5 per 10 000 people in the Community' (Decision No 1295/1999/EC of the European Parliament and of the Council of 29 April 1999)

Orphanet[5] reports 845 rare disease registries, databases and cohorts listed in Europe with 16 listed in Ireland[6]. As recommended by the EU Expert Committee on Rare Diseases (EUCERD) development of rare disease registries over the last decade has been underpinned by the principles of interoperability between registries, patient involvement, adaptability and sustainability as well as commitment to the FAIR data principles[7]. Interoperability has been further enabled by the collection of a standard minimum set of 16 data elements including personal details, diagnosis, disease history and care pathway, as recommended by the European Platform on Rare Disease Registration (EU RD platform)[8].

The European Reference Networks (ERNs) are cross-border networks that bring together European hospital centres of expertise and reference to tackle rare, low prevalence and complex diseases and conditions requiring highly specialised healthcare. ERNs have set up transnational registries to collect pseudo-anonymised data on patients with rare diseases. They are an important component of digitalised European healthcare establishing a rare disease registry ecosystem based on the principles of interoperability and FAIR data. They harmonise data on rare disease patients across the EU, and make data collected available to researchers, public authorities, industry and other stakeholders on different conditions to improve the medical care of patients. Examples of ERNs are given in Appendix 6.

5. https://www.orpha.net/

6. Rare Disease Registries, cohorts and databases – Orphanet report series April 2024

7. EUCERD Recommendations on European Reference Networks for Rare Diseases, 2013; https://eu-rd-platform.jrc.ec.europa.eu/set-of-common-data-elements_en; The Role of the European Reference Network for Rare Bone Diseases (ERN BOND) and European Registries for Rare Bone and Mineral Conditions (EuRR-Bone) in the Governance of the Management of Rare Bone and Mineral Diseases, Zurita et al, 2024

8. https://eu-rd-platform.jrc.ec.europa.eu/set-of-common-data-elements_en; Data collection on rare bone and mineral conditions in Europe: The landscape of registries and databases, Zurita et al, 2022

02 Future of Registries Taskforce (FoRT)

In February 2023, the CFRI co-hosted an event with Health Research Charities Ireland (HRCI) entitled: The Future of Patient Registries in Ireland. This event was attended by over 100 people and had speakers including patient representatives, the Department of Health (DoH), Health Information and Quality Authority (HIQA), registries and other registry stakeholders. The meeting highlighted that there was considerable interest in taking forward work to ensure the sustainability of registries. Following the meeting, interested individuals were added to a mailing list which subsequently became the Future of Registries Taskforce (FoRT), led by members of CFRI and NISR. FoRT membership is intended to be as inclusive as possible to reflect the wider registry, health information and health research landscapes. Membership is made up of the following stakeholders: registry representatives, patient representatives, clinicians, researchers, industry, funders, Data Protection Officers (DPOs), and the private and public sector.

The main aim of FoRT is stated in the FoRT Terms of Reference:

The purpose of this working group is to achieve multi-stakeholder consensus and influence decision-making on a future model of operating patient registries in Ireland.

To meet this aim, FoRT had several core objectives to guide taskforce activity:

i. Develop a consensus statement for a future sustainable model for patient registries in Ireland

ii. Develop policy recommendations/asks on the 'future of patient registries' agreed by all stakeholders (including consideration of patient registry readiness for the EHDS)

iii. To engage with relevant groups/committees related to policy decision-making on health information/EHR/eHealth/EHDS

The first FoRT meeting was held in January 2024. The group met another 3 times in 2024 (March, May and September). Meetings were structured around expert presentations and group discussion. Expert presentations were given on data protection, EHDS, Health Information Bill, Individual Health Identifiers (IHI), the HIQA health data catalogue and experiences of rare disease registries. Discussions focussed on both the challenges facing patient registries and opportunities to sustain registries into the future, resulting in this document.

In order to meet its overall objectives, the FoRT group conducted a number of activities which are detailed in subsequent sections of this report and form the basis of the group's recommendations.

i. Define key pillars to underpin a future model of patient registries in Ireland

ii. Describe the patient registry landscape in Ireland & experiences in setting up and operating registries. In order to do this, the group

   a. Conducted a survey of patient registries in Ireland, and

   b. Developed comprehensive case studies to document and analyse the experiences and operational practices of patient registries.

iii. Develop policy recommendations on the future of patient registries agreed by all stakeholders

Approach

Defining pillars to underpin a future model of patient registries in Ireland

During the first FoRT meeting, the group brainstormed ideas around supporting pillars to underpin a future model for registries in Ireland. This initial discussion was captured in meeting minutes and on a virtual whiteboard. Between the first and second FoRT meeting, these ideas were summarised, reviewed and finalised by

the group. The discussion summary and detailed pillars can be found in Appendix 1 and 2.

Survey of patient registries in Ireland

To characterise patient registries in Ireland, a survey of existing patient registries/patient registry planning groups was conducted. The first section for the survey asked respondents key demographic information about their registry (size, organisation type, years in operation etc.) The survey questions (Appendix 3) are structured around a set of criteria developed by the FoRT group (detailed in Section 4) as part of an exercise to define a future vision for registries in Ireland. These criteria fall under the 5 pillars:

(A) Public & patient involvement & benefit

(B) Good governance & sustainable financing

(C) High-quality & efficient operations

(D) Effective partnerships

(E) Ensuring & demonstrating impact

The criteria under each of these pillars represent an 'ideal scenario' for registries and act as a useful guide to identify where particular challenges are experienced across the registry landscape. Respondents are asked to rate the extent to which they agree they can meet each measure.

An initial survey distribution list was developed based on the HIQA Catalogue of National Data Collections[9]. Those data collections which met the FoRT scope definition of a registry were included where a contact e-mail was available. This list was reviewed by FoRT members and additional contacts were added that had not been included in the 2022 iteration of the HIQA catalogue or that were in the more developmental stages of registry development. The resulting distribution list represented around 30 registries/diseases/conditions and consisted of 55 contact e-mail addresses. The survey was completed by 25 individuals representing 18 registries/disease/condition areas. Most survey respondents were operating in a senior/management/clinical lead position within the registry/disease area. Survey responses are summarised in text and in Appendix 4.

Registry case studies

Six registry case studies were developed using a qualitative semi-structured interview approach. Interviews were carried out with senior members of the registry. Interviews focussed on the registry's journey so far and then discussed their experiences of the registry across the 5 pillars. A case study document (see Appendix 5) for each registry was then drafted and reviewed iteratively by the responding registry. The case studies are representative of those at different stages of development and with various organisational/governance structures. The case study registries included are:

i. Alpha-1 Antitrypsin Deficiency Registry

ii. Irish Lung Fibrosis Association – establishing an Interstitial Lung Disease Registry

iii. Muscular Dystrophy Ireland – establishing a neuromuscular registry

iv. National Cancer Registry of Ireland

v. National & International Skin Registry Solutions CLG

vi. Cystic Fibrosis Registry of Ireland

9. https://www.hiqa.ie/reports-and-publications/health-information/catalogue-national-health-and-social-care-data

03 Pillars underpinning a future model of patient registries in Ireland

In order to establish a shared vision for the future of registries in Ireland, the FoRT group worked collaboratively to develop a set of pillars to underpin a future model of registries. It is intended that the pillars should be the foundation of a future model, ensuring that registries can be governed and operate effectively, ethically and sustainably while maintaining a focus on improving patient care and outcomes.

The pillars are summarised in Figure 1; the central pillar agreed by all is to ensure public and patient involvement and benefit. The other required complementary pillars are: ensuring good governance and sustainable financing, ensuring high quality & efficient operations (across data, technology and human resources), facilitating and benefiting from effective partnerships, and ensuring and demonstrating registry impact. Within each of these pillars, the group developed a series of criteria which should be met in any future model for registries. More detailed description of each of the pillars and criteria are in Appendix 2. Likewise, the pillars and criteria serve as a useful basis to consider the current strengths and challenges experienced by registries in Ireland.

Figure 1: FoRT pillars summary

04 Describing the current patient registry landscape in Ireland

Patient registries work in a complex landscape, operating at the interface between healthcare providers, research, industry and policy. The current internal and external landscape facing patient registries in Ireland is detailed below to demonstrate the factors impacting the current sustainability of patient registries.

Characterising patient registries in Ireland

Of the 18 registries that completed the survey[10], most registries considered their operations to be mature (n=11; 61%). Despite this, 50% of registries (n=9) had been operating for 5 years or less. Two registries (11%) labelled their operations as 'early operations', and 3 (17%) were in the planning stages and not yet operational. A further 2 (11%) registries were listed as 'other' and both had previously been operational and were looking to re-establish operations. Seven registries (39%) have been operating for over 15 years, reflecting a strong registry culture and expertise in Ireland, as well as acknowledgement of the importance of registries across disease areas.

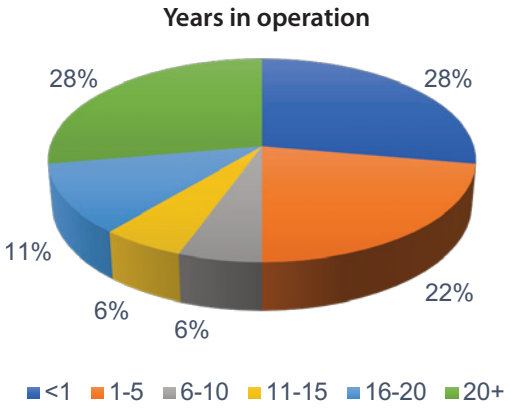


Figure 2: Years in operation (based on survey responses)

In terms of staffing requirements, the picture varies. Overall results show that most registries rely on a very small number of people to operate. Whilst some registries report having no full-time staff, the median number of full-time staff is 3, which is also the most commonly reported number of staff. The maximum number of full-time staff reported was 56. The median number of part-time staff is 1, with 0 part-time staff being most commonly reported. Registries also make use of contracted staff, but to a slightly lesser degree. The median number of contracted staff is 0.5 and the most commonly reported is 0.

Ten (56%) responding registries[11] collect data across multiple hospital/health care settings, with many having a national remit. A further 2 (11%) operate in a single centre but have the intention to operate across multiple centres. Three (17%) registries operate out of a single centre. A further 3 (17%) are classed as 'other' as they are not yet fully operational. The high number of registries operating in, or having the intention to operate in multiple centres signals the need for resources to support this level of operation. In terms of support for data entry operations, over three-quarters of registries (n=14) reported having a data dictionary. Of those that responded, 12 (over 70%) reported sharing data (either directly or indirectly) with international registries.

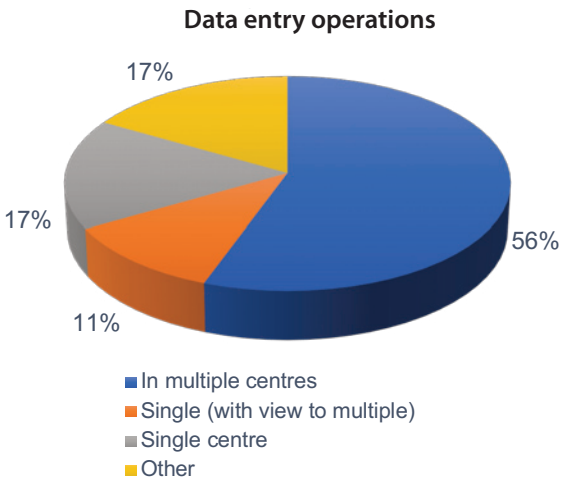


Figure 3: Data collection spread (based on survey responses)

In terms of how registries in Ireland are structured, it is a mixed picture. Nearly 40% of those surveyed classify themselves as an independent organisation i.e., independent from provider/state/industry, although they may receive some funding from these bodies. Over a quarter

10. Chart totals rounded

11. Chart totals may not be exactly 100% due to rounding

(28%) of responding registries classify themselves as provider-based, i.e., based within a healthcare provider such as a hospital/HSE. Just over one fifth (22%) of registries classified themselves as a national body i.e., as primarily state-funded/mandated.

What is clear is that while registries have a large remit, either nationally or across multiple centres, they rely on small amounts of human resources. The variety of registry structures in Ireland illustrates a lack of consistency in how registries are set-up, governed and funded. However, significant knowledge and experience has been accumulated in Ireland, with 7 of the 18 surveyed being in operation over 15 years.

Understanding the influence of external factors on patient registries

The internal registry landscape presents a varied picture of the type, size and scope of registries operating in Ireland. It also reflects the central role registries play across a number of disease/condition areas. Despite this, there are a number of important external factors which not only impact on the sustainability of currently operating registries, but also on the potential for new registries to be set-up.

The following PESTL analysis outlines a number of these pertinent external factors impacting the registry landscape across the political, economic, sociological, technological, and legal spaces:

Political landscape

Political support for health research in Ireland is clear in terms of its inclusion across a number of strategic policy documents including, Ireland's Research and Innovation Strategy 'Impact 2030'[12], the HSE National Policy for Consent in Health and Social Care Research, the Health Information Bill, and the Health Research Board (HRB) published proof-of-concept report on Data Access, Storage, Sharing and Linkage (DASSL). Health research strategy points to its importance in the delivery of healthcare services in Ireland, its role in global research and development initiatives and the critical need for research to improve the health and wellbeing of those living in Ireland. More specifically, the secondary use of data (which includes patient registries) has been a key consideration in a number of policy and legislative areas including the development of the EHDS, the Health Information Bill, as well as part of national standard setting for Health Information[13].

However, in the case of patient registries, these political ambitions are, in many ways, yet to be realised on the ground with limited core funding available to registries, and key infrastructure projects providing limited support for patient registries at this stage. Progress on the General Scheme of the National Research Ethics Committees Bill has stalled, but certain areas of the Bill have been enacted, e.g., the establishment of some National Research Ethics Committees (NRECs). These NRECs operate on a statutory basis to meet EU regulatory requirements for clinical trials and medical devices for example. However, there is currently no NREC for processing ethics applications for patient registries, meaning patient registries operating in several hospitals/healthcare settings must seek ethical approval multiple times and compete with differing interpretations of health research and data protection regulations, and a mixed understanding of the role of a patient registry.

Likewise, whilst plans are afoot, the lack of delivery of an Electronic Health Record to date presents enormous data entry and data integration burdens for patient registries. The significance of a lack of digitisation in the Irish Health system is that health data does not exist in sufficient quantities or in a format amenable to the use of such data for research and innovation anticipated by EHDS, putting Ireland at a distinct disadvantage in terms of the adoption of next generation technologies[14]. Despite being behind in digitisation overall, registries in Ireland demonstrate a significant amount of expertise in digitising registry-specific clinical variables from paper records e.g., both the CF and National Cancer Registries have been doing so for over 20 years.

Despite these political challenges, a new government presents an opportunity to change the tide on infrastructure to facilitate patient registries, secondary use of data and health research more broadly. Likewise, greater calls for investment in patient registries will only serve to strengthen this. For examples, see the Health

12. https://www.gov.ie/en/publication/27c78-impact-2030-irelands-new-research-and-innovation-strategy/

13. https://www.hiqa.ie/reports-and-publications/standard/national-standards-information-management-health-and-social-care

14. https://eithealth.eu/wp-content/uploads/2023/11/Implementing-the-European-Health-Data-Space-in-Ireland-EIT-Health-report-1.pdf

15. https://hrci.ie/hrci-manifesto-general-election-2024/#:~:text=HRCI%20members%20demand%20a%20strong,reducing%20the%20burden%20of%20illness.

Research Charities Ireland 2024 election manifesto[15] and similarly the National Rare Disease Steering group will deliver on the Programme for Government commitment to develop a new National Rare Disease Strategy for Ireland, a process with which a number of registry stakeholders have already engaged.

Economic landscape

Despite movements towards greater support and governance of health research in Ireland in recent years, Ireland still falls short on investment in health research and development. The Health Research Charities Ireland 2024 election manifesto[16] details that Ireland is fifth from bottom across 31 OECD countries with regards to government investment in research and development. More specifically, they report that only 13.9% of the budget is directed towards research aiming to improve health. This continued lack of funding means the research community, including patient registries, are often limited by lack of supports and infrastructures as well as sustainability concerns. As a result, HRCI member charities recommend an increase on current investment of 0.29% GDP per year to the EU average of 0.71% GDP per year over the life of the next government.

Sociological landscape

There are a number of sociological patterns and trends in society which impact the future of patient registries.

i. **Engagement, inclusion and empowerment in health care and health research** – patient centred care represents a movement towards engaging with patients as active, rather than passive, recipients in their care. Patient centred approaches to care involve ensuring people are treated with dignity, respect, and involving them in decision-making about their health. These same principles are reflected in the health research space, where research participants should be engaged in all stages of research. Public/Patient Involvement (PPI) is the terminology used to describe approaches to involving and engaging with patients in research. There is an expectation that PPI is incorporated at all stages of research from design to dissemination; the same ideas should therefore be applied to registries, supporting the secondary use of data. The public/patients should feel empowered and engaged in decisions about how their data is being collected and used and feel like their lived experience input is valued. This can be reflected in patient representation on registry boards/advisory committees, patient engagement activities, accessible consent processes, and in communicating the work of the registry.

ii. **Changing attitudes towards and use of technology** – with greater access to smartphones and internet connection comes greater ability and acceptability for patients to use and potentially benefit from the use of remote technologies (e.g., telehealth, virtual clinics and home monitoring). This presents challenges and opportunities for healthcare providers and ultimately registries. Registries should be able to adapt to collect data from such telehealth and digital health interventions as they become more widely used and more a part of core service delivery. Likewise, registries have an opportunity to capitalise on the use of technology to support the collection of additional patient-reported measures e.g., symptom scores, well-being and quality of life measures. Whilst these opportunities come with potential efficiencies for registries, they require resources to develop and implement such solutions.

Technological landscape

In the context of EU policy and pathways to digitalisation, healthcare is recognised as a complex and continuously evolving knowledge domain presenting several challenges to the informatics community due to the complexity of a highly networked structure that must be aligned with the HSE Digital Health Roadmap[17].

At the heart of eHealth national programs, is a need to engage with people, processes, and technology to reorient models of care and to provide supporting infrastructure to facilitate interoperability. Such an approach can deliver improved data analytics, meet defined targets for 2030 and provide enhanced and safer patient centred outcomes. These identified needs are core to the requirements of functionality. A first milestone in this journey was met with the launch of the HSE Health App[18], a secure way for patients to access HSE information, find health services, and keep personal health information. The use of this app to facilitate the secondary use of

16. https://hrci.ie/hrci-manifesto-general-election-2024/#:~:text=HRCI%20members%20demand%20a%20strong,reducing%20the%20burden%20of%20illness.

17. https://about.hse.ie/publications/digital-for-care/

18. https://www2.hse.ie/health-app/

data, including interoperability with patient registries, is unclear.

From the Irish health and social care perspective, the recently published Digital Health Framework 2024-2030[19] sets in place a foundation for digital transformation, including the establishment of a Health Data Access Body (HDAB), and a public services digital roadmap (HSE/eHealth Ireland) with an agreed operational plan. Currently in Ireland digital data projects are hospital based and are limited in scope for sharing data across and between third party providers. Core features within the roadmap include (but are not restricted to), involving patients and healthcare professionals more effectively, ensuring transformation teams implement recommended national data standards, supporting a safe and effective clinical workflow, and delivering digital health records and services which are accessible in accordance with emerging legislation and recent legislation. In order for the digital data to be a valuable source of secondary data it will need to be harmonised from the outset and coded for interoperability with other data sources (e.g., through the use of OpenEHR and the OMOP Common Data Model (CDM)[20]). The EHDS regulations will outline the specifications for the European Electronic Health Record Exchange format (EEHRxF) which will be the common language that EHR systems must be able to speak to one another.

Legal, regulatory & governance landscape

i. **Data protection & GDPR**

Compliance with national interpretation of GDPR and data protection legislation has added considerable but necessary workload to registries. As a result, registries are experiencing unnecessary extended delays in the submission and approval of interrelated ethics and data protection impact assessments that in the experience of the registries interviewed, reflect a hypersensitivity to the interpretation of data protection legislation at national level.

Data concerning health is the fuel on which most registry projects run; however, the processing of data for health research is governed by additional national legislation. Section 3(1)(a) of the Irish Health Research Regulations states that "a controller who is processing (primary) or further (secondary) personal data for the purposes of health research shall ensure that … suitable and specific measures are taken to safeguard … the rights and freedoms of the data subject …. in such a way that damage or distress is, or is likely to be, caused to the data subject…" The majority of Irish patient registries must comply with the GDPR condition for the processing of special category data (Article 9 GDPR), the only exception being the National Cancer Registry of Ireland which was established by Statutory Instrument.

ii. **EHDS & Health Information Bill**

The European Health Data Space (EHDS) has the overall aim of promoting health data exchange across the EU. This is being led by two programmes of work. MyHealth@EU is a programme of work to implement summary care records at the national level which can be accessed across the EU, and HealthData@EU is a programme of work facilitating access to health data sets across the EU for public interest uses e.g., better policy-making, research and innovation. Access to data sets will be facilitated by national health data access bodies (HDABs); data holders (e.g., registries) will have to make data available to HDABs for the purposes of secondary use. While HDABs will facilitate additional data applications to registries, this will not preclude registries from receiving direct requests for data which would be processed at the level of the registry.

HealthData@EU has a number of implications for patient registries e.g., ensuring registries meet minimum data set requirements and are harmonised to facilitate comparability and interoperability. Moreover, registries will require support to provide high quality data sets to HDABs when required. At the national level, the Health Information Bill is Ireland's legislative framework for the development of digital health records, enhanced patient access to their data as well as the implementation of EHDS requirements, as described above.

With the development of a national HDAB, due to be in place by 2027, and associated secure processing environment, there are a number of considerations for registries. Registries will have additional compliance demands to ensure they meet the required data structure when exporting data to the HDAB's secure processing environment. Following the expected implementation of a national HDAB by 2027, registries will fall under categories of data expected to be available under EHDS by 2029. Achieving the level of

19. https://www.gov.ie/en/publication/0d21e-digital-for-care-a-digital-health-framework-for-ireland-2024-2030/

20. https://www.ohdsi.org/data-standardization/

core/structured datasets will take time and be facilitated via multi-stakeholder communication and consultation.

Likewise, the implementation of a national HDAB will need to interface with ethics processes; currently for patient registries, ethics has to be approved at a local level – the fragmented nature of this process for registries may impact on harmonisation, consistency in registry operations, data availability and ultimately data quality, e.g., different ethics committees influence what data can be collected in a specific centre.

iii. **Facilitating the use of real-world data**

Registries collect longitudinal observational data to inform better future healthcare decisions, aligning with the vision of a learning healthcare system that integrates evidence generation, quality, and innovation[21]. Real-world evidence derived from patient data in clinical care can enhance clinical practice, but in order to do this effectively data needs to be harmonised across registries and healthcare systems[22].

A key example of progress in this area is the Observational Medical Outcomes Partnership (OMOP) Common Data Model, which standardises observational data structure and semantics, enabling reusable statistical analyses across different sites[23]. Observational data, including registry, EHR, and claims data, serve research, healthcare planning and delivery, and payment management purposes but often have disparate formats, requiring harmonisation for effective analysis and mitigating capture bias.

To maximise insights and statistical power, multiple data sources must be integrated while ensuring robust patient data protection. A common data standard eliminates the need for traditional data extraction by allowing analyses to run directly in the native data environment. Implementing a common data model is essential for advancing Ireland's healthcare system.

iv. **Ethics and National Research Ethics Committees (NRECs)**

Ireland operates a mixed model ethics system. NRECs are mandated under legislation, or by ministerial instruction, to deliver a 'single national ethics opinion' that is respected nationally. The NRECs are a key component of the Irish national health research infrastructure, working in parallel with local research ethics committees (RECs). NRECs support specific areas of research.

- NREC-MD: Clinical investigations of medical devices and performance studies for in vitro diagnostic devices.
- NREC-CT: Clinical trials on medicinal products for human use.
- NICB-REC: National Irish COVID-19 Biobank
- COVID-19 research studies: Assessment of COVID-19 research studies formerly the NREC-COVID-19

Patient registries in Ireland tend to be national registries with clinical data collected from healthcare settings across the country. Whilst registries tend to have a national focus, they do not fall under the remit of any of the NREC, and fall under individual RECs. This can cause considerable delays in deploying national registries and creates a major burden in the management of compliance when applying across different individual local RECs and their requirements, different site GDPR interpretations and a requirement to have DPO review before an ethics review. There are considerable differences in interpretations regarding the role of a registry in the research environment and a lack of understanding of the importance of registries as a source of quality secondary data.

v. **Rare disease regulation**

European Reference Networks, co-funded by the European Commission, were set up to increase access to high quality care for patients with rare diseases through collaborative knowledge sharing and coordination of care. It is a legal requirement for EU countries to participate in ERNs. The legal basis for ERNs is set out in the Directive 2011/24/EU on the application of patients' rights in cross-border healthcare [European

21. https://ohdsi.github.io/TheBookOfOhdsi/OhdsiCommunity.html#

22. https://ohdsi.github.io/TheBookOfOhdsi/OhdsiCommunity.html#

23. Overhage, J. M., P. B. Ryan, C. G. Reich, A. G. Hartzema, and P. E. Stang. 2012. "Validation of a common data model for active safety surveillance research." J Am Med Inform Assoc 19 (1): 54–60

Parliament and the Council of the European Union 2011]. Each ERN has an associated registry or registries, funded and hosted by the EU to ensure sustainability[24]. This provides a model that combines better care for patients with a deeper understanding of rare conditions and facilitates enhanced interaction with stakeholders including regulators, patients and governments. ERN Registry governance, interoperability and ERN interaction is facilitated by a number of projects including the European Rare Disease Research Coordination and Support Action consortium (ERICA), European Rare Disease Research Infrastructure (ERDRI), the European Joint Programme on Rare Diseases (EJ PRD), the work of which continues through the European Rare Diseases Research Alliance (ERDERA), and the Joint Action on Integration of ERNs into National Healthcare Systems project (JARDIN)[25].

24. Tumiene, B., Graessner, H., Mathijssen, I.M. et al. European Reference Networks: challenges and opportunities. J Community Genet 12, 217–229 (2021). https://doi.org/10.1007/s12687-021-00521-8

25. The Role of the European Reference Network for Rare Bone Diseases (ERN BOND) and European Registries for Rare Bone and Mineral Conditions (EuRR-Bone) in the Governance of the Management of Rare Bone and Mineral Diseases, Zurita et al, 2024

05 The value of patient registries in Ireland – registry use cases

Patient registries play a distinct role in the health care system and health information system in Ireland. The role played by registries materialises in value and impact across a number of areas which ultimately work to improve patient outcomes and care; these areas include strengthening the planning, delivery and evaluation of care, increasing access to therapies and other healthcare initiatives, informing policy decision-making, and leveraging the use of registry data. We outline below a number of registry use cases, where Irish registries have had particular impact. This list of examples is by no means exhaustive but rather, based on a small number of experiences, demonstrates the enormous positive impact the registries and the use of registry data can have.

Strengthening the delivery of care

Cystic Fibrosis Ireland commissioned the Pollock report, published in 2005. The ultimate aim of the report was to recommend measures to structure CF services in Ireland and improve patient outcomes. Registry data was central to evidencing and supporting the recommendations of the report; this included data on patient numbers, patient distribution in CF centres in Ireland, and data to support healthcare utilisation. Latterly, the registry is mentioned as a core component of CF care in the National Clinical Programme for CF (NCPCF). The registry currently sits on the working group of the NCPCF.

While not yet operational, a number of registries acknowledge the need to address fundamental gaps in evidence of patient numbers, geographical spread and access to services as critical pre-cursors to strengthening access to services, the delivery of care and resource allocation in Ireland. The Interstitial Lung Disease Registry and the Neuromuscular Registry teams are examples of work in this area.

Increasing access to therapies & clinical trials

One of the core benefits of the Alpha1 registry experienced directly by patients is the rapid identification of patients for participation in clinical trials, which was much more difficult prior to the existence of the registry. Further to this, involvement of the Irish Alpha-1 registry and use of registry data in international projects and papers has further benefitted Irish patients through clinical trial access and encouraging the use of Irish registry data in pharmaceutical safety and efficacy studies.

The CFRI has been able to contribute data to post-authorisation efficacy and safety studies of CFTR Modulator therapies, including data on real-world effectiveness of therapies. These studies have contributed to continued access for many CF patients in Ireland & across Europe which has been furthered strengthened with the qualification of the European Cystic Fibrosis Registry by the European Medicines Agency[26].

Improving knowledge of disease landscapes at an international level

Irish registries play an important role at an international level, with many registries contributing Irish data into European and global disease registries aimed at improving understanding of disease landscapes across the globe. Sharing data at this level is particularly important in rare disease spaces, where patient numbers in Ireland are often insufficient to make statistical claims. The below list is non-exhaustive but provides several examples of international registries and groups receiving Irish data and input (based on survey responses):

- European Cystic Fibrosis Patient Registry (ECFSPR)
- Movember Global Registries

26. https://www.ema.europa.eu/en/events/joint-heads-medicines-agencies-hma-european-medicines-agency-ema-multistakeholder-workshop-patient-registries

- European Registry of Cardiac Arrest (EuReCa)
- European Congenital Anomaly Registries (EuroCAT)
- GRASS International
- ASTAR - Dream2Treat Federated Registry (an EU consortium of 6 Atopic Dermatitis Registries)
- ERN Lung
- International Agency for Research in Cancer
- European Cancer Information System
- European Cancer Inequalities Register

Informing policy decision-making

Data from the National Cancer Registry of Ireland (NCRI) has underpinned every cancer strategy since 1997. Data provided by the NCRI for the 2006 National Cancer Strategy showed inequitable outcomes across Ireland and relatively poor outcomes compared to our European peers. Irish evidence provided by the NCRI supported policy makers in their decision making to establish the National Cancer Control Programme.

Alpha-1 registry data has been pivotal in making several key policy recommendations. For example, registry data and associated research publications have highlighted risk factors and the high smoking cessation rates in different Alpha-1 genotype populations. This knowledge led to a renewed focus on smoking cessation as the number one way to protect the lung health of people with Alpha-1. During the COVID-19 pandemic, the registry was vital to the rapid identification of those who needed COVID-19 vaccination most urgently.

Leveraging the use of registries

Within the GRASS registry (part of National and International Skin Registry Solutions CLG), publications have resulted from both international collaborations in which Irish data was shared. The international collaboration on covid-related registries resulted in a publication which highlighted the value of the rapid deployment of COVID-related registries in response to the pandemic. This highlighted what can be achieved in a relatively short-space of time with collaborative working collaboratively at the international level.

Many registries are seen as exemplars by their peers and as such are a source of knowledge and experience on how to set-up and run registries. The CFRI, NISR and NCRI registries all have experience acting in an advisory capacity for those setting up registries.

06 The experiences of patient registries in Ireland

In the sections that follow, we discuss reflections based on findings from both the registry survey and registry case studies to exemplify some of the challenges and also strengths of registries across each of the pillar areas.

Experiences in ensuring public/patient involvement

Overall registries feel that they perform well in terms of ensuring patient/public involvement at all stages of registry development, including ensuring experiences and outcomes important to the public/patient are accounted for, and in strengthening patient trust (Q1, 2, 4). In particular, registries are very confident in their ability to apply best practice in terms of patient consent (Q3).

SUB-THEME 1: Meaningful engagement and inclusive activities for patients and the public

According to the case studies, positive experiences derive from including patient/public representatives in advisory committees, scientific committees, or as board members. Overall, case study registries feel that the input of public/patient representatives has distinct value for work from more developmental elements of registry start-up to informing ongoing strategic direction and advising on research projects. In turn, this level of involvement ensures patient needs are better served by the registry, registry operations are strengthened and there is patient buy-in to the registry. Examples from registry case studies include:

- Strategy development at the National Cancer Registry of Ireland (NCRI): there are 2 patient representatives on the NCRI advisory committee who directly advise on strategic planning & governance matters. Alongside this, the NCRI directly engaged with patients in developing their latest strategy (2024-2026); there was a patient representative on the strategy steering group and patient focus groups were supported through IPPOSI.
- Understanding acceptability of an Interstitial Lung Disease (ILD) registry from the patient perspective
- Multiple examples of patient/public representation on advisory committees & boards e.g., NISR, MDI, Alpha1, CFRI
- Multiple examples of patient registries being set-up under the guidance and governance of patient associations e.g., CFRI, Alpha1, MDI, ILD

Facilitating engagement with patients is supported by a number of factors. Several case study registries are either organisationally attached to or very closely linked with their respective patient association/charity. This, alongside resources from bodies like IPPOSI and PPI Ignite Networks, is reported by those interviewed to facilitate patient engagement and to identify patients for involvement. Educating patient/public on their involvement/on registries is an important factor in facilitating engagement, but also a resource intensive task.

There are some reported challenges of engaging patients/the public in registry governance structures including where governance structures were difficult to amend; in managing expectations of the remit of the registry vs individual patient experiences; in the time and resources required to educate/train/maintain engagement; time constraints for patients working in a voluntary capacity; and managing the accessibility of governance structures/meetings.

SUB-THEME 2: Conflicting experiences of consenting

While the survey responses show that registries overall are very confident in their ability to apply best practice in terms of consent, this conflicted with some more practical challenges in implementation. The case study registries largely feel that their consent processes and documentation are strong; some registries reflect on good engagement with consent and buy-in from clinical teams as well as helpful patient information materials.

However, many also reflect on how slow/difficult consenting can be, particularly for larger patient populations, those with less severe conditions, or those not accessing specialist services leading to underrepresentation in the data. Underpinning this is an acknowledgement of the resource required to undertake effective consenting processes.

SUB-THEME 3: Challenges in leveraging public awareness of the registry

The survey highlighted that overall, registries are less confident in their ability to leverage the use of data. Case studies demonstrate the distinct value of the use of registry data e.g., in identifying patients for clinical trials or in informing policy. Nevertheless, several registries emphasise that not only do they think they could do more with their data with sufficient resource, that they could also improve public awareness of the registry and its remit so as to further increase buy-in from patients/public and to manage expectations of what can be achieved with registry data.

Experiences in establishing good governance & sustainable financing

Overall, survey data points to positive experiences in establishing and maintaining good governance structures and processes for registries. However, this is contrasted by greater challenges in building and maintaining a sustainable financing model. These findings were further reflected on in the case studies.

SUB-THEME 1: Strength in building on existing governance structures and processes

Overall, surveyed registries reflect positively on establishing and maintaining good governance structures and processes and in turn in their ability to meet regulatory requirements and sufficiently manage risk. Case study registries who are governed from within another organisation e.g., a patient association, reflect positively on the well-established governance structures and processes that this affords them, with most sitting within already registered charities/Companies Limited by Guarantee. In these cases, registries operate under the main governance structure of an overall board with executive committees or scientific committees overseeing the operations of the registry. For those registries operating independently, well-established and robust governance structures are noted as key to ensuring trust and efficient registry operations. Likely, this is a result of being registered charities and/or registered companies and as such, they must meet the requirements of both the Charity Regulator and the Companies Registration Office. For state-mandated registries, governance is clearly outlined in the Code of Practice for the Governance of State Bodies. Either way, links with patient associations and clinical teams are also noted as important to establishing governance structures that were representative of all stakeholders.

Relatedly, a number of registries comment on challenges with the fragmented ethics approval processes for patient registries that involved submitting applications to each hospital/setting in which data is to be collected. The lack of a National Research Ethics Committee/central approval process with the remit of registries is a real limitation.

SUB-THEME 2: Insufficient core funding and limited capacity to secure external funding

A strong theme across the survey and case study reports is the challenge in building and maintaining a sustainable funding model for the registry. This was particularly concerning for those running registries with limited ability to plan beyond short time-frames, which is against the nature of longitudinal patient registries.

Firstly, core funding is reported to be limited, and where it exists, it is either insufficient to cover operating costs or not secured for long enough periods of time. In cases where no core funding exists, registries cannot resource themselves and rely on external resources such as students to complete data entry. Publicly funded organisations do not necessarily have these concerns and enjoy security of funding, but common to all such organisations, operating on annualised budgets can also make it difficult to scale operations, to plan longer-term, and to anticipate changes in years ahead.

Secondly, registries largely reflect that it is important, but difficult, to leverage multiple funding streams to sustain the registry over the longer-term. Over 40% of survey respondents state that they somewhat or strongly disagreed with their ability to benefit financially from wider partnerships and funding sources. Registries report devoting resources for example to building relationships with pharmaceutical companies from the earliest stage of registry development to ensure strong links can act as the foundation for future funding and support.

Experiences in maintaining high-quality and efficient operations

The survey data highlights that even though registries feel they are well-able to support data entry, data quality, data audit, data harmonisation, and accessibility, the practical challenges they face on the ground are limiting factors. These factors include the efficiencies in the cost of technology and limited ability to resource the registry long-term. Only 36% of responding registries felt their technology platform is cost efficient and only 24% felt that their financing model could facilitate long-term resourcing. Case study registries reflect similar challenges and allude to others.

SUB-THEME 1: Robust data management processes contrasted with difficulties in data access and entry

Case study registries are confident that they had robust processes and procedures in place to support data entry, data quality and audit, and data standardisation/harmonisation. Over three-quarters (78%) of registries responding to the survey state having a data dictionary/variable list or equivalent. Even those registries who were pre-operational point to the importance of devoting time and resource in the early phases of registry development to support defining harmonised variable lists and data management procedures. The importance of engaging with all stakeholders in establishing these processes is key to ensuring the registry is able to meet multiple needs and requirements as well as securing buy-in.

Despite having well-developed and robust processes and protocols in place, registries reflect on some of the practical challenges which limit the implementation of these policies in practice. Most challenges faced are in terms of accessing and entering data onto registry databases. The continued use of paper charts, and the fragmentation of hospital systems/other sources of data impacts the availability, interoperability and accessibility of data for the registry. Access to hospitals is also challenging, particularly when new data collectors are on-boarded; they may need access to physical buildings/rooms, PCs, and lab-based systems, all of which can impede the timeliness of data entry. One registry reported that it can take 3-6 months to get sufficient access in some centres. Access to multiple settings is also a challenge for those registries where the patient population does not necessarily access specialist hospital services. In such cases, to ensure the data is not skewed towards those accessing only specialist services, access to primary care and community settings is an additional complexity that registries must navigate.

SUB-THEME 2: Advanced technology platform functionality constrained by restrictive pricing models

Most case study registries, who are operational, are operating on an externally procured technology platform which they felt performed well in terms of functionality e.g., adaptability, scalability, security and data querying. A number reflect that they would like the platform to be used more in terms of clinical dashboard/instant reporting features to be used in a clinical setting. Likewise, registries who are yet to procure a platform were cognisant of these requirements. That being said, most feel that the costing model related to changing/updating their platform was prohibitive and unsustainable in the face of not having secure long-term resources.

In one case, a registry developed their registry database technology in-house with significant investment in IT infrastructure. Despite the significant financial investment required, the team feel, in this case, that there were distinct advantages of this approach including control over platform structure and functionality, enhanced data security, and the ability to adapt the platform on demand. However, the high amount of resource required to maintain the platform is an ongoing financial commitment and key person dependency is a concern should staff turnover become an issue.

SUB-THEME 3: Long-term resourcing limited by current registry financing

Overall, case study registries reflect positively on having dedicated teams, albeit small teams, with a variety of skill-sets and expertise to support registry operations. However, the challenge in operating with such small teams comes with concerns about key person dependency and the need to rely on external support to undertake core registry tasks e.g., data entry. Some reflected on the opportunity to share resources across disease areas and across registries; a lack of access to skills/expertise was noted in statistics, legal, data protection and co-ordinator input. There is acknowledgement that these are specialised skill-sets and there is some concern around the challenges in recruitment, particularly in a cost-of-living crisis.

Experiences in establishing and facilitating effective partnerships

Survey responses reflect a mixed picture of registry experiences in establishing and facilitating effective partnerships. Over 70% of responding registries feel confident in their ability to facilitate academic partnerships through data requests and research partnerships. In contrast some responding registries feel unable to facilitate industry partnerships. The case studies provided more detail on experiences of facilitating more 'structured' partnerships, i.e., partnerships with agreements, funding, and Memorandums of Understanding, as well as the significant role of more informal partnerships and relationships, to driving the impact of the registry.

Overall case study registry experiences reflect the importance of partnerships and relationships with a variety of stakeholders as key to strengthening the impact of registry data and ensuring that data is informed by and can influence change. Nevertheless, there is acknowledgement that collaboration and tangible benefit can take time and resource as well as presenting challenges in managing expectations, juggling competing schedules and securing feedback.

SUB-THEME 1: Importance of structured partnerships to long-term sustainability

Registry experiences of more structured partnerships include partnerships where funding was received in return for registry data/research as well as involvement in national and international research projects. Likewise, partnerships are considered structured in cases where there was a Service Level Agreement or Memorandum of Understanding or associated core funding. The financial benefit of such partnerships has been realised by a number of registries and also acknowledged by those at earlier stages as central to ensuring long-term sustainability.

Moreover, in terms of strengthening registry impact, experiences of sitting on steering groups/strategy development groups/having standing agendas with state bodies was considered important to ensuring registry data can inform policy decision-making and clinical practice.

SUB-THEME 2: The crucial role of informal partnerships in fostering participation, securing buy-in, enhancing access, and driving impact

While not always financially beneficial, registries acknowledge the critical importance of more informal partnerships and relationships with wider groups of stakeholders to registry operations. Close links and relationships with patient associations, condition specific charities, and clinical teams are seen as key to ensuring that patient and clinical perspectives are central to registry work, to ensure access to data and secure buy-in to the registry, and to strengthen impact potential. Likewise access to wider supports such as IPPOSI/Rare Ireland/HRCI are seen as beneficial in providing access to information from the sector and in supporting advocacy efforts.

Developing relationships with international registries and other registries in the same disease/condition area is key for registries in supporting harmonisation efforts and in learning from the expertise and experience of registries who have been operating for longer-periods of time.

Experiences in generating, evaluating and communicating registry impact

Survey responses on generating, evaluating and communicating registry impact reflect a mixture of experiences. A notable finding is that registries generally feel less confident about impact communication supporting public awareness of the registry, a finding which also emerged from the registry case studies.

SUB-THEME 1: Clear understanding of the impact and potential impact of registry data

Case study registries represent different stages of registry development. As such, some registries have been on a journey to realising impact, whereas others are only at the start of that journey and acknowledge that it will take time for meaningful change to occur. That being said, for those registries, the anticipated impact in terms of the gaps in data/evidence, are clear. Examples of impact/anticipated impact are:

- Scientific impact (via journal publication & use of registry data in international projects) to strengthen buy-in and registry participation

- Informing policy, health service planning, practice and investment in new therapies
- Identification and inclusion of patients in clinical trials
- Informing national clinical programmes/models of care

SUB-THEME 2: Evaluating impact involves measuring the incremental steps along the journey toward achieving meaningful change

While it was not clear that registries have rigorous evaluation frameworks in place, it is clear that registries are aware of the incremental steps along the journey to achieving meaningful change and use these steps as measures of impact over time. Some of the measures of the journey to impact which registries tracked included:

- Scientific output & use of registry data in research (number of data requests)
- International partnerships and the use of registry data in international projects
- Use of registry data to inform condition/disease specific strategies & models of care
- Communications engagement and website traffic
- Presentations to policy-makers and service providers as a precursor to registry data being used

SUB-THEME 3: Raising public awareness, building trust, and securing resources are essential for effectively generating and communicating impact

One of the challenges noted across registry case studies is in strengthening public awareness of the registry and its remit. This is seen as an important precursor to generating impact. It is acknowledged that in order to generate impact, public trust was key. However, resource is noted as a limiting factor to strengthening external communications and showcasing the impact or potential impact of registry data. Likewise, to reinforce public trust, the importance of maintaining high quality data and continually communicating data insights is highlighted. Similarly, ensuring researchers are aware of the registry as a resource is important to ensuring the data is used.

07 FoRT Summary and Recommendations

Key messages on the experiences of registries in Ireland

1. Registries show significant strengths in integrating patient/public voices into governance structures and operations, ensuring patient priorities are central. Examples include patient/public representation on advisory boards and in consultation activities. While this is a strength of registries, it requires significant time and resources. Challenges include managing expectation, accessibility and delivering appropriate education/training activities.
2. Registries in general experience good engagement from their respective populations in consenting to participate in the registry; however practical challenges such as underrepresentation of certain populations, and resource demands to undertake the consent process present challenges.
3. Similarly, limited public awareness of registries can impact patient buy-in, participation and ultimately, the use of registry data to achieve impactful outcomes.
4. Registries feel as though they benefit from robust governance structures often from official links to patient associations or being registered charities/companies, which further supports operational efficiency and trust. Ethical oversight remains fragmented and requires significant resource within registries to navigate creating barriers to registry operations. Such barriers include differing interpretations of a registry's role and differences in site-specific documentation and approval processes.
5. Insufficient core funding and limited capacity to secure multiple funding streams threaten the long-term sustainability of registries and limit the ability to plan beyond short time-frames.
6. Registries maintain robust data management policies and procedures but face practical challenges in accessing and entering data on-site in healthcare settings across the country which often operate across many different hospital level systems. Electronic Health Record systems need to have the capability to export data to registries. Further operational challenges are presented by the high costs associated with implementing and maintaining a fit-for-purpose technology platform to house registry data.
7. Both structured (e.g., research partnerships with industry/government/ academia) and informal partnerships (e.g., with clinical teams, patient associations etc.) are key to sustainability, impact and driving buy-in, access and operational efficiencies.
8. Whilst many registries are aware of the actual and potential impact of the use of their data, limited resources, in terms of key personnel with specific training in communications and public relations, can impact their ability to demonstrate that impact. In addition to being able to effectively communicate impact, public trust and high-quality data are vital to building impact.
9. Resource constraints and small teams requiring specific sets of expertise face challenges in recruitment, retention, and key person dependency.

Key asks to ensure the future sustainability of registries in Ireland

In order to safeguard and strengthen the use of Irish data and meet the requirements of the EHDS legislation, in terms of the secondary use of data, a harmonised, consistent and sustainable national approach to operating patient registries is required.

1. Develop and implement a Proof-of-Concept Model for the sustainability of registries

The Department of Health should work together with registry stakeholders (i.e., FoRT) on the development of a proof-of-concept model delivered via a pilot study. The pilot study should outline the feasibility and viability of

the pilot as an ongoing approach to sustain patient registries in Ireland and to ensure the readiness of registries to comply with EHDS regulatory requirements in terms of the secondary use of data.

This will include developing an economic model to demonstrate the cost efficiencies and economies of scale of the model as compared with business-as-usual. Should the proof-of-concept be implemented longer term, the desired outcome would be a funding commitment from public, private, and registry organisations (in the form of a public-private partnership). The pilot study would be undertaken in two phases:

a. Phase 1 (6-9 months) – a co-funded project planning & budgeting exercise with the Department of Health and other stakeholders to prepare a detailed plan and budget for a registry pilot that focuses on efficiency and sustainability alongside compliance to new and existing regulatory requirements.

b. Phase 2 (minimum 3 years) – pilot study delivery: once the first phase is reviewed and approved a minimum 3-year deployment of the pilot study would be initiated across a pre-defined number of registries. The ambition is that the pilot would be funded through a Public Private Partnership (PPP) where the exchequer is not carrying the total cost. For example, a PPP partnership would involve dedicated funding from registries themselves and the Department of Health, with industry (e.g., pharmaceutical and medical device industry providing additional funding to support Health Technology Assessment and Post-Authorisation Safety Studies across strategic areas of interest).

The pilot study will be a 3-year initiative and involve bringing together a defined number of disease registry groups either operating registries, or in the early phases of establishing registries, in their respective disease areas. The aim of the pilot will be to establish a proof-of-concept model for a more efficient and sustainable operating and financing model for patient registries which is based on a core registry 'body'/unit which facilitates the work of individual registries. This would involve:

i. **Implementing a common data model** across registries to harmonise data capture across registries in compliance with EHDS requirements for secondary use of data and to facilitate the integration of registry data across disease areas and across health information systems.

ii. Setting-up an adaptable and scalable registry database (based on a secure cloud concept) across registries to facilitate high-quality registry data management, database interoperability (including with EHRs), and to ensure more sustainable costs in developing and maintaining a **fit-for-purpose platform** across disease areas. A common technology platform would benefit registries in terms of cost-economies, use of common technology, consistent processes and procedures for data management, and facilitating integration with wider national IT systems. A similar approach is being adopted by the National Heart Programme (with EuroHeart partners).

iii. Building and utilising a set of inclusive **common governance structures** and standard operating procedures to support registry operations which facilitate compliance with regulatory, ethical, and risk management guidelines in a proportionate manner.

iv. Share a **set of core human resources** to support registry operations, creating a more sustainable and effective way to maintain registry operations and maximise the use of registry data in a timely manner. Core resources which are frequently unavailable to registries due to funding are statistical, data protection and ethics expertise as well as registry co-ordination roles.

2. Engage with and leverage FoRT as a 'community of practice'

The most rare, valuable and difficult commodity within health informatics is the missing "communication". Effective communication relies on a motivated, educated, and engaged community capable of articulating their needs. These needs can be translated into a unified model that, when digitised, seamlessly integrates with larger systems. This integration generates meaningful data, empowering the community to enhance practices, research, and care delivery. That's what FoRT as a group has that no others have.

The Department of Health should capitalise on and engage with the significant expertise in FoRT to sustain communities of practice that will:

a. Build on the concepts of interoperability and data harmonisation of health data to explore how standards like OpenEHR, OMOP and the European Health Exchange Format can be used to help Ireland bridge the gap between EHRs, registries and the data messaging between them.

b. With the recent publication of the European Health Data Space regulation, ensure that registries are represented in stakeholder groups consulted on the implementation of the EHDS regulation, the establishment of the Health Data Access Body, and the National Electronic Health Record implementation programme

c. Support the development of a central ethical approval process for all registries (perhaps via mechanisms similar to the establishment of the NICB-REC)

Also, in the absence of a central ethics approval process for registries, educate and improve the advice and guidance provided to local/regional ethics committees and data protection officers, ensuring appropriate and proportional procedures are in place for patient registries.

Anticipated impact

The FoRT group is uniquely positioned to collaborate with stakeholders to support the development and delivery of a pilot program that ensures registries remain central to Ireland's healthcare future. National collaboration is required to shape an integrated, innovative, and sustainable digital health ecosystem which ultimately places improved outcomes for patients at its heart. Rather than investing in individual registry projects, having an integrated, consistent and sustainable registry infrastructure in Ireland would bring huge benefits for patients by:

- Supporting healthcare planning, management and delivery
- Strengthening Ireland's clinical trials landscape, real-world evidence study landscape and industry partnerships through access to standardised and harmonised registry datasets
- Ensuring registry readiness for the EHDS and facilitating the secondary use of data in Ireland and internationally
- Exploit the experience of Irish registries in digitising and analysing patient data in Ireland for over 20 years (before the introduction of EHRs)
- Leveraging the impact of the implementation of a national Electronic Health Record through making data readily exportable to registries and available in an appropriate format for analysis and research.

Call-to-action

If the future of registries is not secured, patient care will suffer. By uniting stories and statistics, registries become more than a repository—they are a lifeline, fostering hope and driving innovation through structured data that saves lives, improves care, and ensures that no patient's journey is overlooked or forgotten. Without registries, the Department of Health risk the inefficient use of public funds to support healthcare delivery and ultimately leaving patient lives with the hope and lifeline they deserve in accessing the right support and treatment at the right time. Registries and EHR's must not be confused, they are equally critical to the provision of a safe and progressive health service, both are critical requirements of the European Health Data Space Regulations (EHDS).

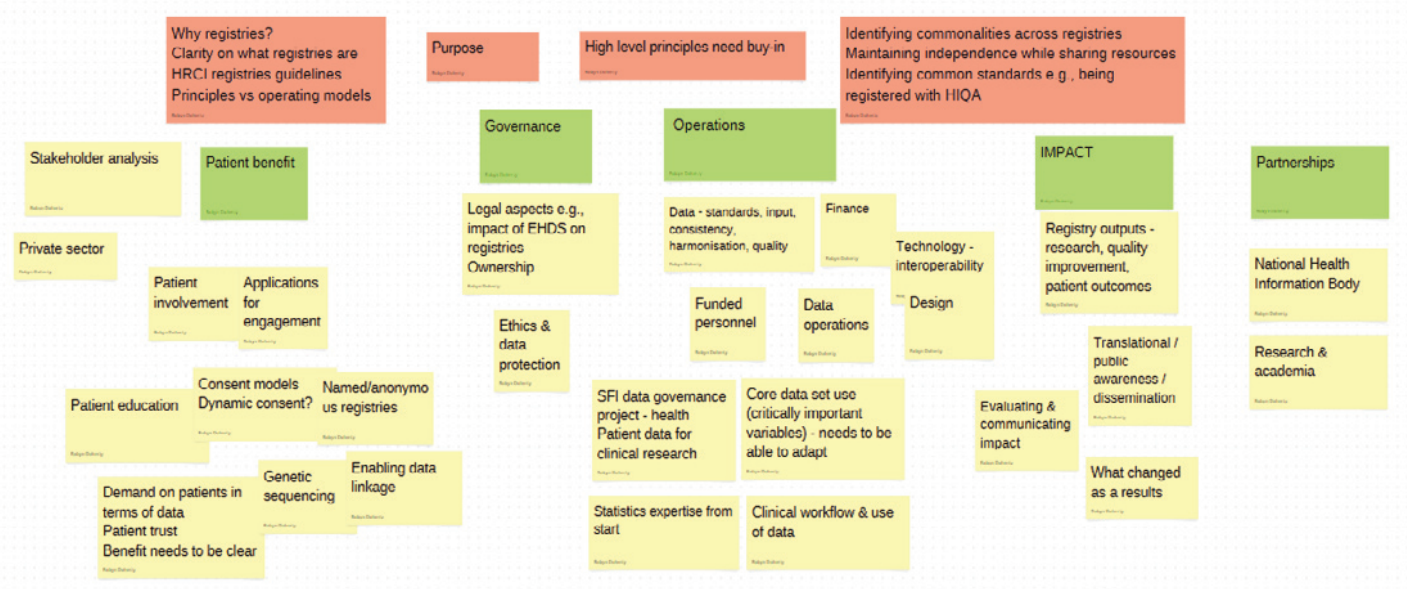
The FoRT group call on the Department of Health to action the above proposed solutions that will provide a cost efficient and sustainable system that will improve patient outcomes, enrich the quality of data that drives research and assist Ireland's compliance to the EHDS.

Appendices

Future of Registries Taskforce

Appendix 1

Summary of initial discussion defining pillars to underpin a future model of patient registries



Appendix 2

FoRT Pillars to underpin a future model of patient registries in Ireland

PILLAR 1: Public & patient benefit

A future model for patient registries needs to ensure public/patient involvement & benefit at all stages[27]

Criteria for future model to meet

Facilitate the involvement of and engagement with the public & patient representatives at all levels & stages of registry development (including in governance & through the use of innovative applications of co-design and co-creation principles and a human-rights based approach)

Ensure that experiences and outcomes important to patients are captured

Apply best current practice in patient consent processes, in line with legislation.

Strengthen patient trust through patient education and engagement

Minimise demands on patients and Health Care Providers (e.g., data collection burden)

Maximise the use of the data in a timely & efficient manner, minimising the need to re-collect data in multiple locations (via data standardisation).

Effectively communicate the outputs of patient registries to public & patient community

PILLAR 2: Good governance

A future model for patient registries needs to operate under good governance[28]

Criteria for future model to meet

Legal structure & governance allows individual registries to maintain level of independence (a central-common governance model with autonomy)

Meet National and European legal and regulatory requirements laid down in various agencies (such as Health Information Bill, EHDS, GDPR, and EMA, etc.)

The registry can efficiently apply for and meet local ethics requirements (e.g., centre level ethics applications/NREC)

Governance structures are inclusive of all stakeholders

Governance facilitates risk management & financial management of sustainable funding streams

Individual registries should have access to the support, expertise and guidance from a core set of professionals to ensure high levels of governance in all registries.

27. https://hrci.ie/unlocking-the-potential-of-patient-registries-a-guide-for-success/ & https://cfri.ie/wp-content/uploads/2023/09/6589-HRCICFRI-Patient-Reg-Guide_v4.pdf

28. https://hrci.ie/unlocking-the-potential-of-patient-registries-a-guide-for-success/ & https://cfri.ie/wp-content/uploads/2023/09/6589-HRCICFRI-Patient-Reg-Guide_v4.pdf

A clear pathway to establishing disease registries for individual diseases or areas should be in place so that individual registries can be supported to becoming established and ensuring a sustainable pathway towards future governance and financial security.

2.1: Sustainable financing

A future model for patient registries needs to govern a sustainable financing model to ensure the longevity of registries e.g., multi-year funding models

Criteria for future model to meet

Funding for the core to support expertise in the legal governance and regulatory aspects of registries and support the establishment of individual disease registries should be provided by the state and be secure.

External funding from industry, charities and other partners should be maximised to most efficiently leverage state investment.

Partnerships between state funded entities and universities, hospitals and industry should be encouraged to deliver registry services in as cost-efficient a model as possible.

PILLAR 3: High-quality & efficient operations

3.1: High-quality operations - data

A future model for patient registries needs to ensure high-quality data[29,30,31]

Criteria for future model to meet

A future model facilitates data harmonisation, standardisation & interoperability through relevant frameworks (e.g., Common Data Models (CDM) /core data sets/Individual Health Identifiers/Findable, Accessible, Interoperable, and Reusable (FAIR)) which will minimise duplicate data collection.

Data quality & audit is central to registry operations supported by relevant frameworks (e.g., Findable, Accessible, Interoperable, and Reusable (FAIR)/European Medicines Agency (EMA)/HIQA guidelines). Data is readily accessible and available for research purposes nationally and internationally in line with European Health Data Space and Health Data Access Body.

Facilitate the use of Irish registry data in regulatory and HTA submissions (e.g., by meeting data standardisation & quality requirements) – link to Pillar 1 (e.g., through access)

3.2: High quality operations - technology

A future model for patient registries needs to be supported by adequate technological infrastructure

Criteria for future model to meet

Technological infrastructure is adaptable, scalable and facilitates interoperability with other datasets (e.g., facilitates FAIR data)

Future proofed technology with full knowledge transfer from vendor to registry management, with functionality to support system upgrades/retirements built in from the start

29. https://www.nature.com/articles/sdata201618

30. https://www.ohdsi.org/data-standardization/

31. HIQA Data Quality Framework 2018 & https://www.hiqa.ie/reports-and-publications/standard/national-standards-information-management-health-and-social-care

	Technological infrastructure costing models are efficient to requirements of scalability & adaptability.
3.3: High quality operations - resourcing	**A future model for patient registries needs to be adequately resourced with specific skill-sets, expertise and sustainable finance**
Criteria for future model to meet	Sharing of specific skill-sets is facilitated through centralised resources (e.g., ethics/data protection/statistics/governance/regulation/legal)
	Training & continued upskilled of human resources to keep pace with changing research & regulatory landscape in EU.
	Financing model facilitates long-term centralised resources
Pillar 4: Effective partnerships	**A future model for patient registries needs to facilitate & benefit from effective partnerships for different purposes**
Criteria for future model to meet	Research and academic partnerships facilitated through data access requests and collaborative research partnerships (including through appropriate data sharing through EHDS)
	Industry partnerships are facilitated in a transparent and uniform manner
	Partnerships are facilitated through PPI initiatives and patient engagement at all levels
	Facilitate the use of Irish registry data in regulatory and HTA submissions.
	Facilitate partnerships within registry/disease-specific networks, with state bodies (HDAB), EHR owners, health system partners, hospitals, and general practice etc.
Pillar 5: Ensuring & demonstrating impact	**A future model for patient registries needs to generate, evaluate and communicate impact to a wide range of audiences**[32]
Criteria for future model to meet	Registry impact evaluation is supported by a rigorous evaluation framework designed to facilitate monitoring of registry outcomes and impact across patient outcomes, research impact, quality improvement, and policy/regulatory decision-making
	Registry impact evaluation should support public awareness, registry quality improvement and assessments of 'fit-for-purpose' registries.
	Impact is communicated via tailored communications to multiple stakeholder audiences (e.g., public, academic/research/industry/clinical)
	Registry output is disseminated in multiple formats to facilitate public awareness of the role of registries

32. https://cfri.ie/wp-content/uploads/2023/09/6589-HRCICFRI-Patient-Reg-Guide_v4.pdf

Appendix 3

Registry survey questions

General registry questions

1. Which registry/disease area do you represent?
2. What is your role with respect to the registry?
3. At what stage are your registry operations?
    a. Planning stage
    b. Early operations
    c. Mature operations
    d. Post-registry / registry ended
    e. Other
4. How many years has/was your registry been in operation?
    a. <1
    b. 1-5
    c. 6-10
    d. 11-15
    e. 16-20
    f. 20+
5. How many full-time personnel work for the registry?
6. How many part-time personnel work for the registry?
7. How many contracted staff work for the registry?
8. Does your registry operate in a single centre or in multiple centres?
    a. Single-centre
    b. Single-centre (with a view to operating in multiple centres in the future)
    c. In multiple centres
    d. Other
9. How do you classify your registry set-up?
    a. Provider-based (i.e., healthcare provider based)
    b. Independent (i.e., independent from provider/state/industry although may receive some funding from these sources)
    c. National body (state funded/mandated)
    d. Industry-based
    e. Other
10. Is your registry governed as any of the following?
    a. Registered charity

b. Registered company (e.g., Company Limited by Guarantee)

c. A registry with a Service Level Agreement with the HSE

d. None of the above

e. Other

11. Does your registry have a data dictionary/variable list or equivalent? [Y/N]
12. Does your registry link in (directly or indirectly) via an international group [Y/N] e.g., European Reference Network or international registry?

    Link in directly = has an agreement to provide an agreed dataset on a regular basis (e.g., annually)

    Link in indirectly = provide data/engage with international registries on a project-by-project basis/ad-hoc basis
13. If yes, please briefly describe this group

PILLAR 1 – public & patient involvement & benefit

Definition: the criteria in this section capture the fact that registries should be supported to ensure public/patient involvement & benefit at all stages and levels of registry operations. Please rate the extent to which you agree that your registry, as it currently operates, is able to meet the following criteria.

All questions answered on Likert scale from (1) strongly disagree to (5) strongly agree

1. The registry can facilitate the involvement of the public & patient representatives at all stages in the design/development/governance of the registry (through the use of innovative applications & co-design/co-creation principles)

   Co-design/co-creation principles refer to approaches to involving patients/public in the design/development/governance of registries.
2. The registry can ensure that experiences and outcomes important to patients are captured
3. The registry can apply current best practice in patient consent processes, in line with legislation.
4. The registry can strengthen patient trust through patient/public education and partnership
5. The registry minimises demands on patients and Health Care Providers (e.g., data collection burden)
6. The registry maximises the use of data in a timely manner
7. The registry effectively communicates the outputs of the registry to the public & patient community
8. Are there any particular barriers which prevent the registry from meeting any of the above criteria relating to public & patient benefit? If so, please briefly note these below [Free text].

PILLAR 2 – Good governance & sustainable financing

Definition: the criteria in this section capture the fact that registries need to operate under good governance and with sustainable funding models. Please rate the extent to which you agree that your registry, as it currently operates, is able to meet the following criteria:

All questions answered on Likert scale from (1) strongly disagree to (5) strongly agree

1. The current legal structure & governance allows the registry to maintain level of independence
2. The registry can meet national and European legal/regulatory/standards requirements (such as European Health Data Space (EHDS), Health Information Bill, General Data Protection Regulations (GDPR), European Medicines Authority (EMA), HIQA etc.)
3. The registry can efficiently apply for and meet local ethics requirements (e.g., centre level ethics applications/NREC)

4. All relevant & appropriate stakeholders are represented in registry governance structures e.g., clinicians/patients/public
5. Registry governance facilitates risk management & financial management of sustainable funding streams
6. The registry has access to support, expertise and guidance to ensure high levels of registry governance e.g., data protection, ethics, governance, legal, financial etc.
7. It was/is clear how to establish the registry when it was being set-up/is being set-up
8. The registry has sustainable core funding to support expertise in the legal, governance, regulatory, and operational aspects of setting up & running the registry
9. The registry maximises external funding from industry, charities and other partners to most efficiently leverage state/core funding/investment
10. The registry benefits financially from partnerships between state funded entities and universities, hospitals and industry to deliver registry services in as cost-efficient a model as possible
11. Are there any particular barriers which prevent the registry from meeting any of the above criteria relating to good governance and sustainable financing? If so, please briefly note these below [Free text]

PILLAR 3 – establishing & maintaining high-quality & efficient operations

Definition: the criteria in this section reflect the fact that registries need to gather, process and output data of high-quality in an efficient manner through operations (including technology and human resources).

Please rate the extent to which you agree that your registry, as it currently operates, is able to meet the following criteria:

All questions answered on Likert scale from (1) strongly disagree to (5) strongly agree

Data

1. Registry data collection can support data harmonisation & interoperability through the use of relevant frameworks (e.g., Observational Medical Outcomes Partnership (OMOP) Common Data Model (CDM) /core data sets/Individual Health Identifiers/Findable, Accessible, Interoperable, and Reusable (FAIR))
2. Data quality & audit is central to registry operations supported by relevant frameworks (e.g., Findable, Accessible, Interoperable, and Reusable (FAIR)/European Medicines Agency (EMA)/HIQA guidelines). As a result, registry data is readily accessible and available for research purposes nationally and internationally in line with legislative/regulatory requirements.
3. The registry facilitates the use of registry data in regulatory and HTA submissions (e.g., by meeting data standardisation & quality requirements)

Technology

4. The technology platform used by the registry is adaptable, scalable and facilitates interoperability with other datasets (e.g., facilitates FAIR data)
5. The technology platform used by the registry is cost efficient in terms of scalability and adaptability requirements

Resourcing

6. The registry team has relevant & specific skill-sets (e.g., ethics/data protection/statistics/governance/regulation/legal)
7. The registry can support training & continued upskilling of human resources to keep pace with the changing research & regulation landscape in the EU.
8. The registry's financing model facilitates long-term resourcing

Long-term resourcing refers to resourcing beyond 5 years

9. Are there any particular barriers which prevents the registry of meeting any of the above criteria relating to operations (data, technology & resourcing)? If so, please briefly note these below [Free text]

PILLAR 4 – establishing and maintaining effective partnerships

Definition: the criteria in this section reflect the need for registries to facilitate and benefit from effective partnerships with a variety of stakeholders for different purposes.

Please rate the extent to which you agree that your registry, as it currently operates, is able to meet the following criteria:

All questions answered on Likert scale from (1) strongly disagree to (5) strongly agree

1. The registry has research and academic partnerships facilitated through data access requests and collaborative research partnerships.
2. The registry facilitates industry partnerships in a transparent and uniform manner.
3. The registry facilitates partnerships through PPI initiatives and patient engagement at all levels.
4. The registry has wider partnerships with disease-specific networks, state bodies, EHR owners, healthcare providers, etc.
5. Are there any particular barriers which prevent the registry from meeting any of the above criteria relating to effective partnerships? If so, please briefly note these. [Free text]

PILLAR 5 – Ensuring & demonstrating impact

Definition: the criteria in this section reflect the need for registries to be able to generate, evaluate and communicate their impact to a wide range of audiences.

Please rate the extent to which you agree that your registry, as it currently operates, is able to meet the following criteria:

All questions answered on Likert scale from (1) strongly disagree to (5) strongly agree

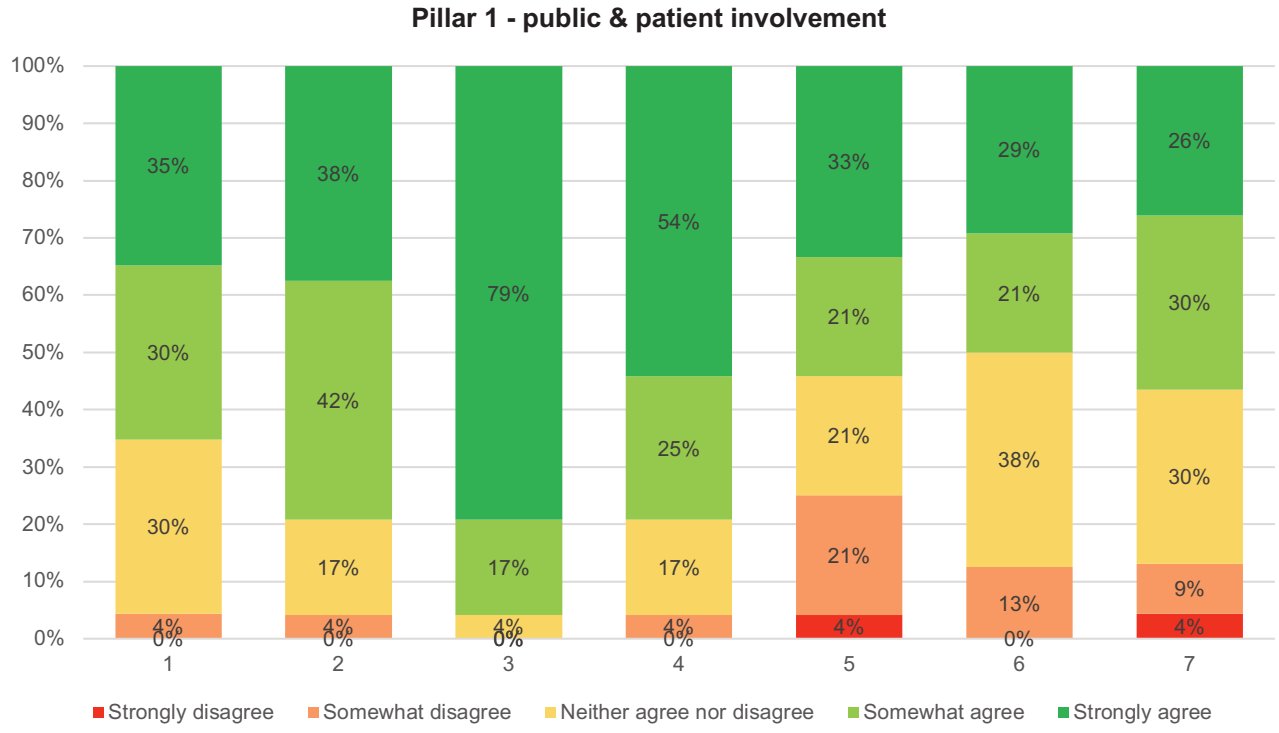
1. The registry's evaluation of impact is supported by a rigorous evaluation framework designed to monitor outcomes and impact across various areas.
2. The registry's impact evaluation supports and informs public awareness, registry quality improvement, and assessments.
3. The registry communicates its impact via tailored communications to multiple stakeholder audiences.
4. The registry disseminates output in multiple formats to facilitate public awareness of the role of registries.
5. Are there any particular barriers which prevent the registry from meeting any of the above criteria relating to ensuring and demonstrating impact? If so, please briefly note these.

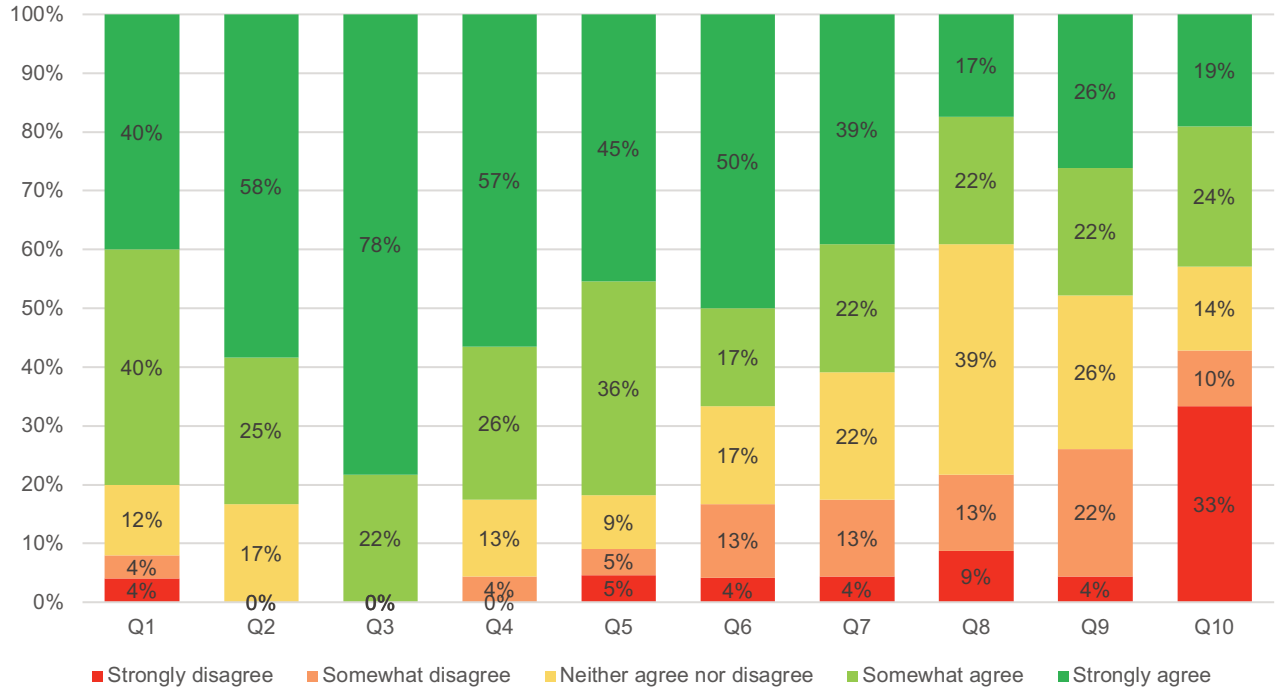
Final comments

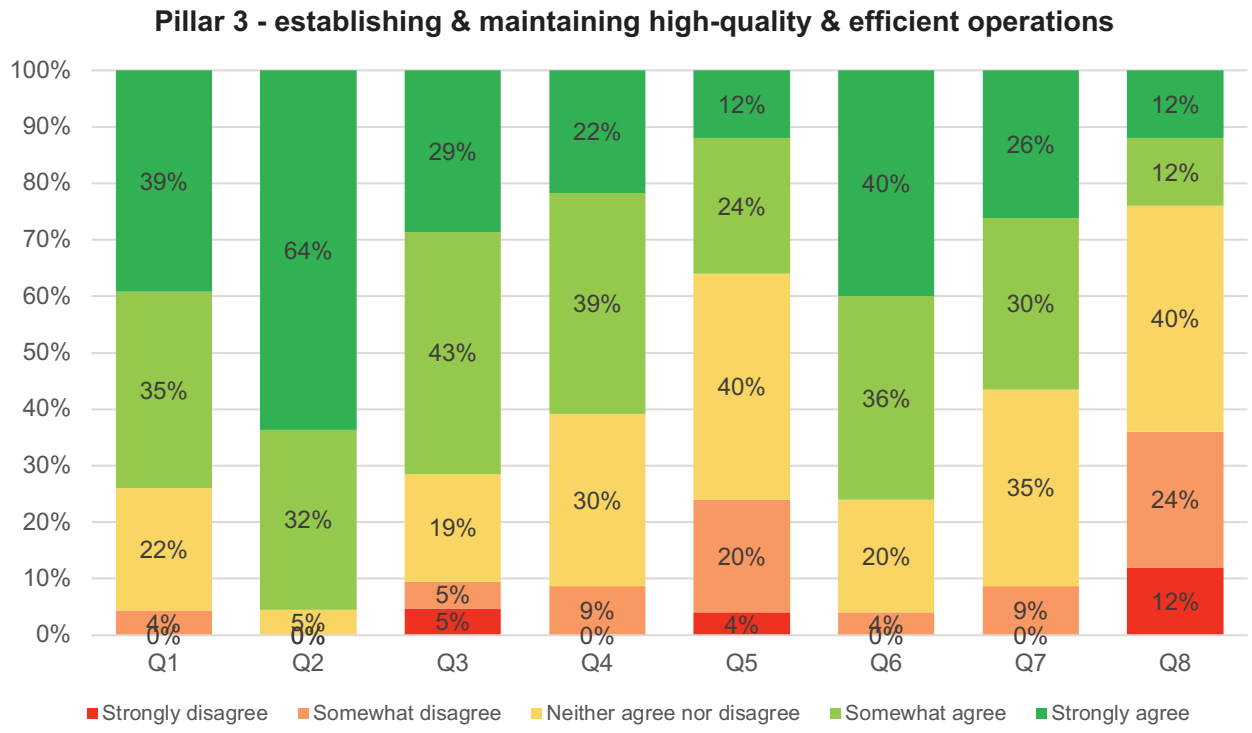
1. Do you have any other comments on the challenges facing your registry?
2. Would you be interested in providing more information on your registry experiences in the form of a case study?

Appendix 4

Summarised registry survey responses






Pillar 3 - establishing & maintaining high-quality & efficient operations
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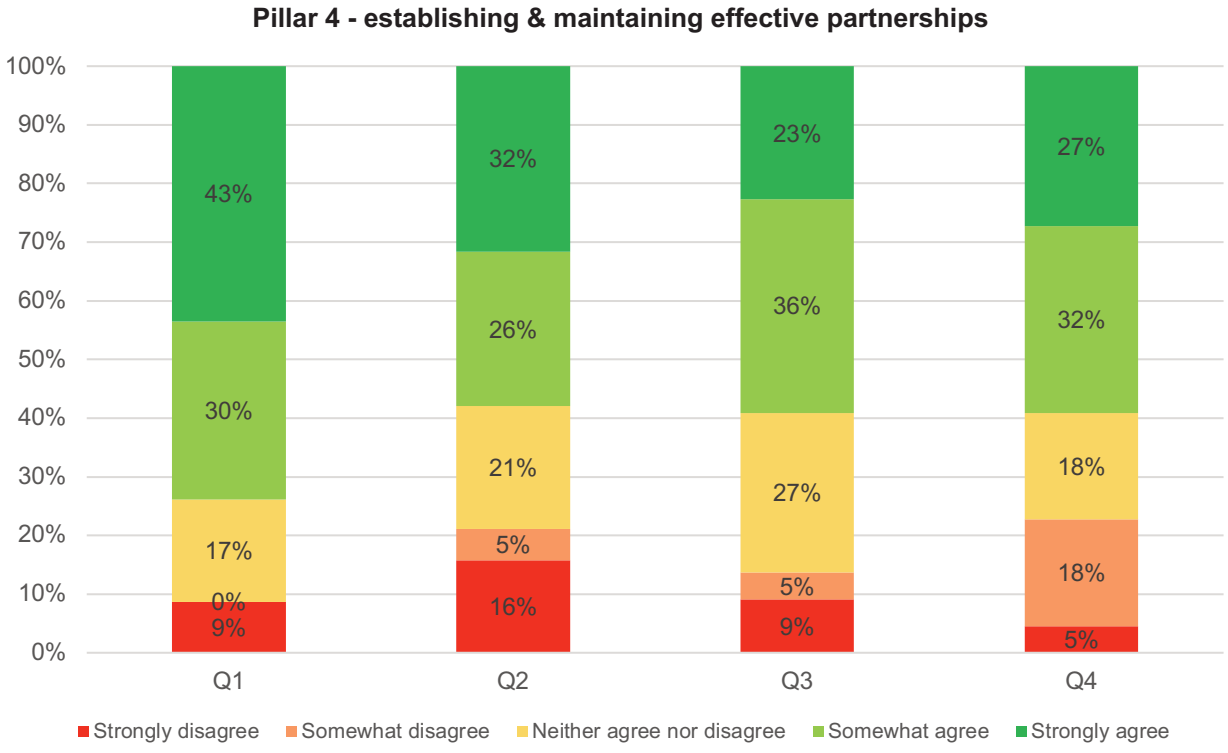
Pillar 4 - establishing & maintaining effective partnerships
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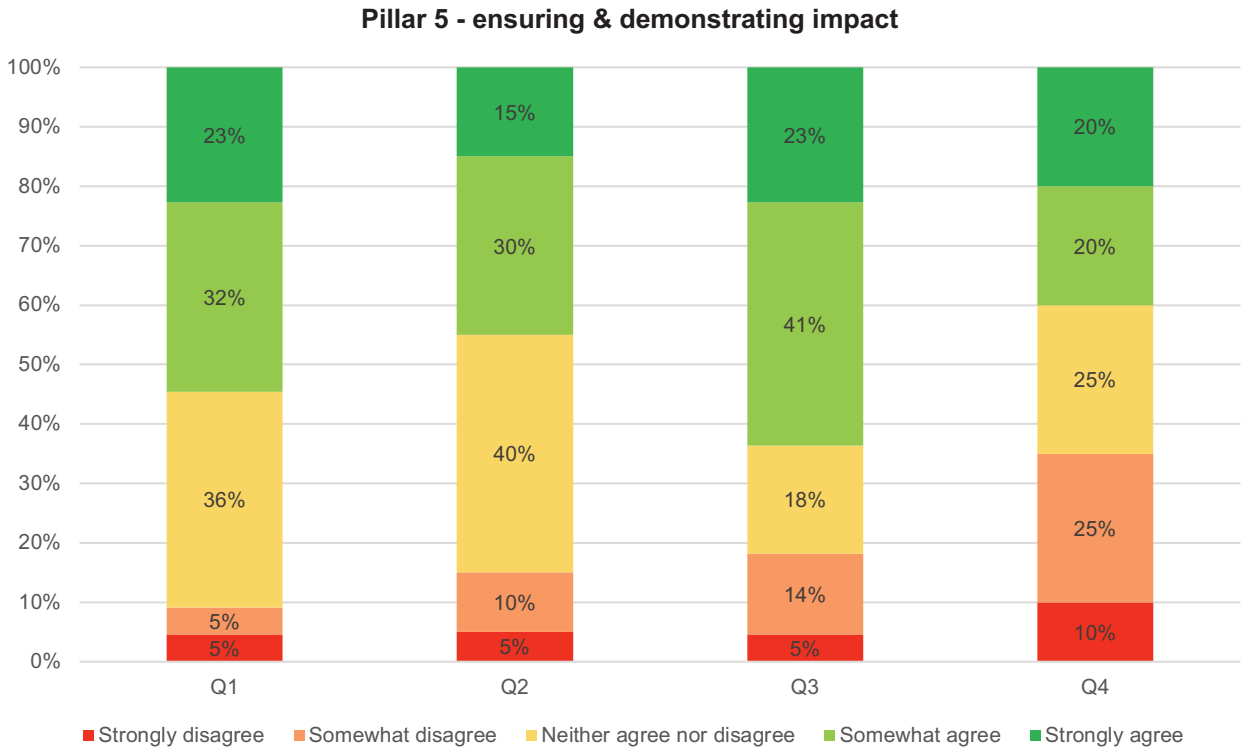
Pillar 5 - ensuring & demonstrating impact
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Q1
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Q4
Strongly disagree
Somewhat disagree
Neither agree nor disagree
Somewhat agree
Strongly agree

Appendix 5

Registry case studies

Alpha-1: The National Alpha-1 Antitrypsin Deficiency Registry case study

1. REGISTRY PROFILE

The Alpha-1 registry is the National Alpha-1 Antitrypsin Deficiency Registry. Alpha-1 is a genetic condition which can cause lung, liver and skin disease. There are an estimated 3,000 people with severe Alpha-1 (ZZ) and 250,000 with moderate Alpha-1 (MZ) on the island of Ireland. The key objectives of the Alpha-1 Registry are to:

1. Increase our understanding of AATD (knowledge)
2. Inform and improve clinical care (care)
3. Provide early access to new treatments via clinical trials (treatment)

The registry was founded in 2005 as a direct result of a new HSE-funded national targeted detection programme which was helping to identify Alpha-1 patients. The registry updated their platform in 2018 with a major IT overhaul. The registry is governed and coordinated from within Alpha-1 Foundation Ireland, meaning overall governance is overseen by the Alpha-1 Foundation Ireland board. A new scientific committee is also supporting registry governance and operations. Alpha-1 Foundation Ireland is a registered charity and company. Registry activities sit alongside the wider work of Alpha-1 Foundation Ireland (as shown in Figure 1).

Core activities of Alpha-1 Foundation Ireland

The registry differs from a number of other Alpha-1 registries as it includes a wider spectrum of Alpha-1, not only those with severe Alpha-1, so it includes individuals with moderate Alpha-1. Data collection is coordinated at a single specialist centre operating at Beaumont Hospital, Dublin, the site of the HSE-designated National Centre of Expertise for Alpha-1. Data is captured on an informed consent basis from both medical charts and directly from patients on an encounter basis, meaning every time a patient visits the Alpha-1 clinic. Currently there are around 700 patients participating in the registry, around 300 of which suffer from severe Alpha-1. Data is collected longitudinally on diagnosis, prognostic information, clinical outcomes, and therapies.

2. EXPERIENCES ENGAGING WITH PUBLIC & PATIENTS

Due to its proximity to both Alpha-1 Foundation Ireland and clinical care, patients and public are naturally at the centre of the Alpha-1 registry. The team acknowledge that without clear patient benefit on the ground, patient buy-in will be limited. Having patient representation on the Alpha-1 Foundation Ireland board is key to ensuring the patient point of view remains central to the work of the foundation, including the registry.

One of the core benefits of the registry experienced directly by patients is the rapid identification of patients for participation in clinical trials, which was much more difficult prior to the existence of the registry. Further to this, involvement of the Irish Alpha-1 registry and use of registry data in international projects and papers has further benefitted Irish patients through clinical trial access and encouraging the use of Irish registry data in pharmaceutical safety and efficacy studies.

In terms of participation in the registry, registry staff oversee consenting patients to the registry when they come

to clinic. While consenting doesn't always occur on the patient's first appointment at the clinic, to avoid overwhelming them with information, patients are usually very open to participation. Currently an Alpha-1 Foundation staff member meets with patients individually at clinics to provide information on services/supports offered by Alpha-1 Foundation, including the registry. Rather than a traditional clinical approach, the more familiar approach taken by the foundation staff is about getting to know people, hearing their concerns and addressing real-world questions they might have. The benefit of this approach is that trust is built with the Foundation staff and the clinical team, and this encourages participation in the registry. While engagement is generally high, registry staff acknowledge that it is easier to recruit patients with more severe Alpha-1 as they likely attend clinic more often.

EXPERIENCES IN ESTABLISHING & MAINTAINING GOOD GOVERNANCE & SUSTAINABLE FINANCING

Alpha-1 Foundation Ireland is a registered charity and company. The registry is governed and coordinated from within Alpha-1 Foundation Ireland, meaning overall governance is overseen by the board. The Alpha-1 Foundation Ireland board is a representative group of stakeholders, including clinicians and a patient representative (soon to be two patients). A scientific committee further support registry governance and operations ensuring high quality data collection and appropriate registry data use through developing, implementing and monitoring registry specific policies and procedures.

In the past, the question has been raised about whether the Alpha-1 registry should be an organisation independent of Alpha-1 Foundation Ireland but its embeddedness within the organisational structure and with clinical teams is currently seen as a great advantage for the registry.

In terms of sustainable funding, the registry does not have its own funding stream, rather it is funded under Alpha-1 Foundation Ireland, which relies heavily on fundraising as well as core HSE funding. While there is a distinct funding stream for the National Targeted Detection Programme from the HSE, this does not extend to the registry. The registry is coordinated by Alpha-1 Foundation staff and relies on external support to operate e.g., medical students helping with data entry as part of short-term research projects. To ensure sustainable funding for the registry, Alpha-1 Foundation Ireland staff members believe the registry needs to be recognised as a part of and for its contribution to patient care and improving patient outcomes:

> *"Without a registry, you can't treat a condition"*

3. EXPERIENCES IN MAINTAINING HIGH-QUALITY & EFFICIENT OPERATIONS

The Alpha-1 registry have well-established protocols to ensure high-quality data collection. They are however faced with a number of operational challenges in terms of accessing and entering data into the registry. The continued use of paper charts can impact data quality and presents challenges in terms of integrating different sources of data. Timeliness of data entry is also impacted by resourcing issues – with no dedicated data collection team, the Alpha-1 registry relies on post-graduate students or on medical students to enter the weekly clinic data in blocks of 6-weeks as part of student projects. Likewise, limited resources to support consenting can impact participation rates meaning some patient groups are at risk of being underrepresented in the registry.

Likewise limited resources impact on the ability of the Alpha-1 registry to analyse and disseminate its own data. For example, access to specialist skills such as biostatistics and machine learning would amplify what the registry can do with the valuable data it collects. Currently data is often analysed as part of international projects with project teams based elsewhere. However, the team feel it would be hugely beneficial to have access to such skills locally to maximise data use. Likewise, having a dedicated individual to manage the registry overall would further leverage the impact the registry currently has for patients. The registry is also conscious of their dependency on the knowledge, experience and relationships of a small group of people and the impact any turnover might have; however, current governance arrangements try to mitigate against this as much as possible.

Operations at the registry are supported by the current registry database technology, which works well and is well-protected in terms of data security. It is hosted by Health Atlas Ireland, part of the HSE IT ecosystem. Current technology supports updates and changes to the database and data entry requirements; however, the registry team feel the cost associated with making changes can be prohibitive. In future, the registry hope that they can build on their current technology to facilitate more use of the registry data as a real-time clinical dashboard. Likewise, in the Alpha-1 space, there is increasing interest in the collection of more nuanced data on clinical

outcomes and quality of life. The team are confident the technology will be able to adapt to meet these needs but are less confident in the ability to sustain both the financial and human resource costs to allow for this.

External regulatory requirements have presented challenges to the registry as well. This included the implementation of the Health Research Regulations which the registry felt were too risk averse in Ireland, negatively impacting on the work of the registry. Huge amounts of resource had to be diverted from day-to-day registry operations to ensure the registry was meeting these requirements at the time. A consent exemption application to the HRCDC was finally approved after a 12 month wait. Currently, regulations present difficulties to the sharing of pseudonymised data outside the EU, often with major Alpha-1 international partners. Likewise, the impacts of new regulatory requirements on ethical amendments and approvals presented further challenges – the Alpha-1 registry team experienced issues communicating with hospital Data Protection Officers, where they felt legal & ethical matters were becoming confused.

4. EXPERIENCES IN FACILITATING & BENEFITING FROM PARTNERSHIPS

The Alpha-1 Foundation & registry benefit in a number of ways from different partnerships. The fact that the registry is embedded with Alpha-1 Foundation Ireland ensures that the registry is meeting the needs of patients. Likewise, the close links with clinical teams and Beaumont Hospital ensures that registry data collection is facilitated and patients are recruited to the registry in as timely a manner as possible. Practically, the long-standing and successful partnership with RCSI has enabled the Foundation to have access to office space located conveniently in both a research and clinical setting.

As already noted, international research partnerships have ensured the use of Irish registry data in clinical trials and further supported the access of Irish patients to clinical trials. Likewise, links with national bodies and groups focused on genetic and rare lung conditions, such as IPPOSI, Rare Disease Ireland, and HRCI, has improved access to information & care for Alpha-1 patients, particularly in supporting advocacy work around genetic discrimination.

5. EXPERIENCES IN GENERATING, EVALUATING AND COMMUNICATING IMPACT

Alongside providing access to clinical trials for Irish patients, Alpha-1 registry data has been pivotal in making several key policy recommendations. For example, registry data and associated research publications have highlighted risk factors and the high smoking cessation rates in different Alpha-1 genotype populations[33]. This knowledge led to a renewed focus on smoking cessation as the number one way to protect the lung health of people with Alpha-1. During the COVID-19 pandemic, the registry was vital to the rapid identification of those who needed COVID-19 vaccination most urgently. The rapid roll-out of the vaccine in severe AATD also allowed us to gather important information about attitudes to the vaccine among people with AATD[2]. Registry data and evidence is also continually being collated to make the case for access to disease-modifying treatments, such as intravenous (IV) augmentation therapy in Ireland[3]. However, despite the fact Irish registry data has been used in several international studies[3,4], resulting in the reimbursement of augmentation therapy in other countries, there are challenges in making the case for its reimbursement in Ireland. If accurate healthcare utilisation data, such as hospital admission data, was available to the registry, data could show how augmentation therapy is keeping people with Alpha-1 out of hospital, thus improving the chances of reimbursement.

At the most basic level, the first scientific paper resulting from the registry was key in securing clinical buy-in to the registry. Clinical buy-in is ultimately key to ensuring participation in the registry as well as its value and longevity. Likewise, the registry provides an ongoing evidence base to support conversations with the HSE and Department of Health on healthcare resource planning & care provision.

Alpha-1 Foundation Ireland also publish an annual report each year which has a dedicated section for the registry. Nevertheless, with additional resource, the foundation would like to strengthen their external communications to further showcase the value and impact of the registry as well as improve awareness.

33. Franciosi, A. N. et al. (2020) Alpha-1 Antitrypsin Deficiency and Tobacco Smoking: Exploring Risk Factors and Smoking Cessation in a Registry Population. COPD: Journal of Chronic Obstructive Pulmonary Disease. 18(1), pp. 76–82. doi: 10.1080/15412555.2020.1864725.

2. McElvaney O.J. et al. (2022) Attitudes Towards Vaccination for Coronavirus Disease 2019 in Patients with Severe Alpha-1 Antitrypsin Deficiency. Chronic Obstr Pulm Dis. Apr 29;9(2):266-273.

Irish Lung Fibrosis Association (ILFA)/Interstitial Lung Disease (ILD) Registry Case Study

1. REGISTRY PROFILE

The Irish Lung Fibrosis Association (ILFA) is in the early phases of establishing an Interstitial Lung Disease (ILD) Registry. ILD is a group of progressive fatal lung diseases characterised by fibrosis or scarring of the lung. While an exact number is not available, it is estimated that there are around 5000 people living with ILD in Ireland, with around 1000 patients newly diagnosed each year[34]. Unfortunately, median survival time from diagnosis is around 3 years. Currently, there is no ILD National Clinical Programme.

The Irish Thoracic Society (ITS) has played a core role in emphasising the need for registries in the ILD space including the publication of a 2018 report[35] on the burden of respiratory diseases in Ireland, reinforcing the need. In addition to this, a pilot project, governed by the Irish Thoracic Society (ITS) Multidisciplinary Steering Committee and funded by an industry grant, developed and ran an ILD registry from around 2016-2018. There were some challenges in getting ethical approval from multiple specialist centres with different ethics approval processes which impacted operations and ultimately, the funding for the project was unsustainable over the longer term. This, paired with the introduction of new regulations around data protection, health research and the impacts of the Covid-19 pandemic put a pause on the work of the registry. Now several years later, the team at ILFA, alongside the ITS ILD Steering Group, are looking to re-establish an ILD registry to serve the initial aims of the pilot project registry as well as supporting further research areas. The aims going forward include:

a. Understand the prevalence and distribution of ILD cases to support planning of healthcare resources

b. To support studies of safety, efficacy and cost-effectiveness of new and established treatments

c. To establish a sense of the financial burden of ILD

d. To contribute to wider studies on ILD

e. To provide data to highlight challenges with provision of healthcare for ILD

f. Link with stakeholders with an interest in ILD e.g., researchers, patients, clinicians, and industry

g. Highlight areas for further research

The below sections outline ILFA's experiences in attempting to re-establish the ILD registry, rather than prior experiences in running the pilot project.

2. EXPERIENCES ENGAGING WITH PUBLIC & PATIENTS

ILD patient engagement in the pilot project was strong, collecting data on 154 Idiopathic Pulmonary Fibrosis (IPF) patients. Subsequent work has been undertaken by ILFA to understand attitudes and acceptability of an electronic registry from the patient perspective. This work highlighted that while patients had some concerns about data security and privacy, they clearly saw the value and utility of an ILD registry. Patients also emphasised the importance of registry sustainability, recognising the distinct value of registry data over the long-term. Moreover, focus group research with ILFA stakeholders highlighted aspects of the registry that were important from a patient perspective including:

- The need to have a registry covering all patients with ILD (including in private hospitals)
- Mapping the patient journey
- Evidencing type of patient and geographic spread of patients
- Incorporating patient feedback for the registry

In the next phase of re-establishing the registry, ILFA isn't underestimating the importance of engaging with patients at this early stage. The team is aware of the need to engage with patients as well as clinical teams to

34. https://irishthoracicsociety.com/wp-content/uploads/2019/04/Chapter-8-Chronic-Interstitial-Lung-Disease-and-Sarcoidosis-1.pdf

35. https://irishthoracicsociety.com/wp-content/uploads/2018/11/ITS-ILD-Registry-Annual-Report-2018.pdf

ensure buy-in to the registry, to support future registry operations, and ultimately to ensure the registry will meet the needs of patient and other stakeholders from the earliest stage.

3. EXPERIENCES IN ESTABLISHING & MAINTAINING GOOD GOVERNANCE & SUSTAINABLE FINANCING

The process of re-establishing the ILD registry is being led by ILFA alongside ITS, both are registered charities. However, questions remain over where the registry should ultimately 'sit', who should govern it and who should be custodian(s) of the patients' data. Initial discussions indicated a preference for the registry, once established through a project in conjunction with the National Office for Clinical Audit, to sit within an HSE governed structure. This was primarily due to early ideas that the registry may be funded and resourced by the HSE. However, concerns arose about whether this option was viable. Moreover, the ILFA team recognise the need to maintain links to the patient association (ILFA), and they also have concerns about the sustainability of HSE funding over the long-term.

While the ILFA team is not at the stage of establishing a registry funding model, they have requested an initial EUR 150,000 from the Department of Health (DoH) to fund a National Office for Clinical Audit (NOCA) audit of ILD in Ireland. This would serve as the basis on which to develop the registry, but would be insufficient to maintain registry operations. The team acknowledges the need to be able to benefit from multiple funding streams and to establish sources of long-term funding. There is concern in this respect that a forthcoming election will delay any financial decisions regarding DoH funding, at least in the short-term.

4. EXPERIENCES IN MAINTAINING HIGH-QUALITY & EFFICIENT OPERATIONS

Despite not being operational at this stage, the ILFA team are confident that they know what they want to achieve with the registry, based on learnings from the pilot project and subsequent consultation with patients and other stakeholders. However, in advance of designing the registry more formally, ILFA are aware of the need to ensure harmonisation with international standards and to integrate standards across disease areas, particularly in the respiratory space. Working closely with the ITS will support harmonisation and integration in the registry due to its in-house knowledge and expertise across all respiratory diseases.

One of the main operational challenges presented to the registry during its pilot phase was in gathering data beyond specialist ILD centres. While data collection was facilitated at these centres, registry data was potentially skewed towards patients with more well-managed disease. They acknowledge potential challenges in gathering data from primary care settings e.g., GPs.

While the pilot project adopted a more manual approach to data collection, the ILFA team is keen to ensure that the registry going forward is supported by the appropriate technology platform with the ability to adapt and scale as required. The potential cost and complexity of doing so is challenging in the face of no sustainable funding model.

Considering resourcing the registry into the future, ILFA acknowledges the opportunities to share resources with other respiratory conditions given similarities across healthcare provision and clinical outcomes. Based on their experiences so far, key roles to support the development of the registry include a registry coordinator role, clinical data expertise, support for data protection requirements, and links with Electronic Healthcare Records initiatives.

5. EXPERIENCES IN FACILITATING & BENEFITING FROM PARTNERSHIPS

Partnerships benefited the establishment of the pilot ILD registry including via industry funding and governance from ITS. Going forward, ILFA sees partnerships as key to the establishment and sustainability of an ILD registry including the continued support from ITS. The central role of ILFA in re-establishing the registry will be to maintain a strong partnership with the organisation and ultimately ensure that patient engagement and patient benefit are central to the work of the registry. Likewise, in maintaining strong partnerships with specialist ILD centres, the registry can capitalise on clinical knowledge, ensure data entry is supported at each centre, and enable the practical benefit of registry data to be realised on the ground.

6. EXPERIENCES IN GENERATING, EVALUATING AND COMMUNICATING IMPACT

While the registry is not yet operational, the ILFA team is clear on the potential impact the registry will have for the ILD population in Ireland and where registry data can address unanswered questions. Pilot project data was published in the 2018 ITS annual report[36], which demonstrates the value such data can have. However, *"…if you don't have data, you can't do anything".*

At the most basic level, there is no national clinical programme for ILD in Ireland meaning a lack of consistency in treatment and outcomes for ILD patients. At this level, an ILD registry can help provide evidence of patient numbers, geographical spread and access to specialist services.

At the treatment level, the ILFA team is aware of a number of disease-modifying drugs in pipeline development with unanswered questions on their effectiveness, safety, and cost-effectiveness in real-world settings – without a registry these questions become difficult to address. Similarly, descriptive information about the ILD population will support assessments of demand for pharmaceutical and other treatments (e.g., oxygen). The team acknowledges the need to proactively establish the registry, so data is available when real-world studies and data requests are required.

Ultimately, the establishment of a registry will support budgeting and healthcare resource planning as well as making arguments for upstream intervention. Accurate knowledge of the number of ILD patients and the health impacts of their condition can help develop economic arguments for early delivery of effective treatments (e.g., pharmaceutical, pulmonary rehabilitation) to reduce hospitalisations and the financial cost of care. Likewise, being able to access data from patients not accessing specialist services creates the opportunity to highlight some of the challenges in variability of care delivered in primary care settings.

Without a registry, ILFA is less able to support future research in the area, research which will be critical to improving the quality of life of those living with ILD and ultimately to formalise the status of ILD as a disease, driving investment into the care and treatment of those living with ILD. Without accurate knowledge of the incidence and impact of ILD, and ultimately a clinical programme for ILD, patients are missing out of specialist care such as community pulmonary rehabilitation which could significantly improve their quality of life.

36. https://irishthoracicsociety.com/wp-content/uploads/2018/11/ITS-ILD-Registry-Annual-Report-2018.pdf

Muscular Dystrophy Ireland - Establishing a Registry Case Study

1. REGISTRY PROFILE

Muscular Dystrophy Ireland (MDI), a Company Limited by Guarantee (CLG), is a voluntary member organisation established in 1972 providing support across Ireland for those living with neuromuscular conditions and their families. Their mission is to provide information and support to people with neuromuscular conditions and their families through a range of support services. MDI also aims to support and fund research into neuromuscular conditions. As part of this aim, they are currently in the process of establishing a neuromuscular disease registry, which consists of up to 60 different conditions across all age groups.

The idea to establish a registry was discussed in 2018 however, due to COVID-19 and changes in staff and management, the proposal did not progress until 2022. The following sections detail MDI's progress and experiences to date in establishing their registry.

2. EXPERIENCES ENGAGING WITH PUBLIC & PATIENTS

MDI places significant value on the contributions of individuals with lived experience in shaping and informing their work. People with lived experience sit on all MDI boards and sub committees including the registry advisory group. Members of MDI were invited to express interest in being part of the registry advisory group. Interested candidates were offered a one-to-one session to learn about the purpose of a registry and the responsibilities involved in working with the advisory committee. Lived experience representatives represent a gender balance and a variety of neuromuscular conditions.

The advisory group consists of a variety of stakeholders including clinicians, people with lived experience, members of MDI board of directors, health care professionals and MDI staff. The first meeting included all stakeholder groups but to ensure the meetings were accessible to all and relevant, smaller meetings were subsequently held for the specific stakeholder groups while working through elements of the dataset. This ensured the content of meetings was relevant for the group, medical jargon was explained, and everyone had time to contribute into the discussions. The insights of people with lived experience on the advisory group have been invaluable in guiding decisions about which variables to include or exclude, selecting a Patient-Reported Outcome Measure (PROM), and determining data collection methods. Their involvement has been key to ensuring the registry meets the needs of multiple stakeholders and gains broad support.

Future engagement will focus on strategies to inform and promote the registry to the wider neuromuscular community. The focus will initial be on individuals attending specialist neuromuscular clinics. A national registry is preferred; however, achieving this goal is difficult without a centralised national ethics process for registries to gain ethic approval. Many people with neuromuscular conditions receive care at regional hospitals rather than specialist centres, making it impossible to apply for ethics approval at all these health care settings. As a result, the registry will be gradually rolled out across neuromuscular clinic sites and other recognised relevant locations.

3. EXPERIENCES IN ESTABLISHING & MAINTAINING GOOD GOVERNANCE & SUSTAINABLE FINANCING

Although the registry is still in the planning stages, it is expected to be integrated within the MDI organisational structure. MDI is a CLG & registered charity and has a well-established governance model. Currently, an advisory group operating under specific terms of reference, overseas the registry development. This group of stakeholders will likely go on to form part of a scientific committee which will oversee the registry once operational. Maintaining this group will provide continuity as the registry progresses. In terms of operational governance, the registry is not yet at the stage of submitting ethics applications; however, with 7 specialist neuromuscular centres across the country, the team are mindful of the need to coordinate ethics approval processes at each centre. This challenge is amplified by the need to gather data beyond hospital sites, in primary and community care settings.

In terms of financing, MDI has secured funding for the next three years; however, this does not guarantee long-term sustainability. MDI's Research Committee acknowledges the need to be able to benefit from multiple funding streams and to establish sources of long-term funding.

4. EXPERIENCES IN MAINTAINING HIGH-QUALITY & EFFICIENT OPERATIONS

Planning for high-quality and efficient operations has been key for the MDI team throughout their journey so far. In terms of ensuring the neuromuscular registry collects a relevant and feasible dataset, the advisory committee have worked over a period of 9-12 months to iteratively and collaboratively refine the existing draft variable list. The importance to doing this in a step-wise approach was key to finalising a variable list all stakeholders are happy to sign-off on. There were some challenges in defining the variable list and thinking through the logistics of data collection including:

- Defining a variable list to work across 60 different neuromuscular conditions but yet to be feasible in terms of data collection
- Incorporating the right PROMs and the need to be able to capture data directly from patients, via PROMs, as well as from clinical notes
- Considerations about being able to access patients outside of specialist neuromuscular clinics i.e., in primary care and in the community
- The need to be interoperable and harmonised with datasets internationally – having existing international registry examples has been useful but there have been difficulties in maximising the ability of a variable list to be harmonised across all.

While it has taken some time to iteratively refine the initial variable list to ensure it is fit-for-purpose and has stakeholder buy-in, a technology platform has already been procured. In hindsight, the team recognise that it would have been beneficial to finalise the variable list before committing to a platform, but various factors influenced this decision at the time. Nevertheless, the team remains confident that the technology solution will meet their needs.

At this early stage, the registry team is small, consisting of one full-time MDI staff member who is leading the registry's development in collaboration with the advisory committee. Their role in the development of the registry represents only a portion of their overall responsibilities. In the near future, the team anticipates needing additional resources for data entry, as well as the involvement of MDI's communications team to support the registry's launch, build awareness and encourage participation. Likewise, in the short-term, support may be required to compile the documentation for the ethics submission.

5. EXPERIENCES IN FACILITATING & BENEFITING FROM PARTNERSHIPS

At such an early stage, the registry team at MDI have not had the opportunity to form formal partnerships, although this is something the team would like to achieve in the coming years, particularly in terms of building a sustainable registry financing model. However, currently the team benefit from other types of relationships and partnerships with stakeholders that support registry planning.

Establishing relationships with international registries in the neuromuscular space has greatly supported MDI in defining their variable list in a harmonised manner and to avoid duplication in registry efforts e.g., been compatible with the European Reference Network Neuromuscular Disease Registry. Likewise, establishing links with the TREAT-NMD Global Registry Network, which brings together independent neuromuscular diseases (NMD) patient registries from across the world, has been helpful.

MDI have also been building relationships with pharmaceutical industry companies. – These relationships and communications are important to build in advance of the registry being operational.

More locally, building relationships with other national registries in Ireland has been instrumental in learning from their experiences in building and managing registries.

Finally, building relationships with clinicians and health care professionals who specialise in neuromuscular conditions (NMC), along with individuals with lived experience has been important for building a strong advisory group. The experience and expertise of the group has been beneficial in ensuring the questions and endpoints in the dataset are accurate, informative and relevant.

6. EXPERIENCES IN GENERATING, EVALUATING AND COMMUNICATING IMPACT

While the registry is not yet operational and as such does not yet have data to use, the team can anticipate the impact of the data they will collect in terms of the unanswered questions for the population of people living with neuromuscular conditions in Ireland. A key aim is to understand the prevalence and epidemiological data for people in Ireland living with a neuromuscular condition e.g., how many people are living with the particular conditions, geographical spread and access to particular services. Such core data will support healthcare resource planning and improve access to services. As the registry grows and develops in the future it is hoped it can support, effectiveness and cost-effectiveness studies of different treatment options, thereby potentially improving access and justifying reimbursement decisions. Relatedly, for very new treatment options, the registry presents an important opportunity to systematically identify patients for recruitment onto clinical trials.

It is clear that there are significant data gaps concerning people living with neuromuscular conditions in Ireland, some of which can be addressed through the implementation of the registry. This presents a significant opportunity for registry data to guide healthcare, influence practice-based recommendations, inform policy decisions, support research and ultimately improve outcomes and quality of life for those living with neuromuscular conditions in Ireland.

National Cancer Registry of Ireland (NCRI) case study

1. REGISTRY PROFILE

The National Cancer Registry of Ireland (NCRI) was established in 1991 by the then Minister for Health by Statutory Instrument, meaning the registry operates under a legal mandate to collect data on cancer and related tumours from all healthcare institutions in Ireland i.e., no patient consent is required. As a result, the NCRI collects data on all tumours that meet a particular set of criteria[37]. Data collection for the registry began in 1994 and the data collected by NCRI has the following remit:

- To identify, collect, classify, record, store and analyse information relating to the incidence and prevalence of cancer and related tumours in Ireland;
- To collect, classify, record and store information in relation to each newly diagnosed individual cancer patient and in relation to each tumour which occurs;
- To promote and facilitate the use of the data thus collected in approved research and in the planning and management of services;
- To publish an annual report based on the activities of the Registry;
- To furnish advice, information and assistance in relation to any aspect of such service to the Minister.

The overall aims of NCRI are to collect, manage and analyse data to gather comprehensive information on the patterns and trends of cancer incidence, risk, treatment and outcomes over time, to support planning and evaluation of cancer services and cancer policy development. The NCRI also conducts research on the causes and prevention of cancer, health service research as well as the assessment of cancer control in Ireland.

NCRI operate under the leadership of a director and report to the NCRI board who operate under the Department of Health. On the ground, NCRI operate with a team of around 50 operating across 5 departments including corporate services, research & analysis, data integration, registration and information technology.

Data is notified to the NCRI primarily by electronic feeds from health information systems such as pathology laboratories, hospital patient information system (HIPE), radiation oncology systems and the Central Statistics Office. These notifications are further expanded and validated using hospital based electronic systems and hospital medical records by a team of Electronic Cancer Data Registrars based at hospital sites across the country and covers around 100 variables (defined by a data dictionary). About 42,000 tumours are registered every year and of those, over 24,000 will be invasive cancers[38]. Alongside diagnosis, NCRI primarily collects data on primary course of treatment up to 12 months after the diagnosis date, which is the treatment plan which is decided on soon after diagnosis. Subsets of NCRI data can be accessed via the NCRI website and more bespoke requests require further documentation.

2. EXPERIENCES ENGAGING WITH PUBLIC & PATIENTS

The NCRI engages with patients via a number of routes. NCRI has an advisory council, made up of a number of stakeholders including 2 patient representatives. The advisory council offers input and expert advice to the NCRI on its Strategy and other issues. Patient representatives are an important component of the advisory council and their input includes useful insights and input on how NCRI communicate and enhance awareness of what they

37. The date of incidence is after the 01/01/1994

Resident in the Republic of Ireland - The residence is defined as the place the person has lived for the previous twelve months. The purpose of recording residence is that the rate of tumour incidence can be related to a specific population.

The list of registerable tumours are as follows:

All tumours described as "malignant"(/3), "in situ"(/2), "of uncertain behaviour"(/1) or "borderline malignancy"(/1) listed in the World Health Organisation (WHO) ICD-O Manual.

All intracranial (inside the dome of the skull) and spinal cord tumours. This includes benign tumours of the Central Nervous System, meninges, cranial nerves (e.g. acoustic neuroma), pituitary gland and pineal gland.

In some cases, subsequent tumours may be diagnosed in someone who is already known to have cancer and these tumours are registered if they meet the NCRI's registration criteria.

38. https://www.ncri.ie/sites/ncri/files/pubs/NCRI_AnnualStatisticalReport_2023.pdf

do. Likewise, patient engagement was central in developing the latest NCRI strategy (2024-2026). A patient representative was a member of the steering group and focus groups were held with patient representative groups through IPPOSI (Irish Platform for Patients' Organisations, Science & Industry). Patients were very willing to engage in this process and to give their opinions. As a result, the 2024-2026 NCRI strategy has ensured the patient is central to future work of the registry.

Despite high levels of patient engagement, NCRI have challenges in engaging patients at the highest level and in governance structures as their Board complement is currently restricted to 7 members.

Moreover, a further challenge in engaging patients with a population-based registry is being able to manage expectations of the remit of the registry to present population-level data and the limited ability to present the individual stories of smaller groups of patients. There is often a disconnect between the sometimes-difficult statistics presented and the experiences of the individuals behind the data. It comes back to the point about awareness and communications regarding the registry and its remit. Overall, without the need for consent, patients are not always aware of the registry, what they do and their important remit within the cancer care and research landscape. The NCRI works hard on communication, particularly on social media sites, where reports are made more digestible for patients and the general public.

3. EXPERIENCES IN ESTABLISHING & MAINTAINING GOOD GOVERNANCE & SUSTAINABLE FINANCING

The National Cancer Registry Board comprises seven members and meets at least four times a year. The board reports into the Department of Health. Prof Deirdre Murray is the Director of the National Cancer Registry, responsible for the implementation of the Board's policies. The National Cancer Registry is divided into five functional areas, each of which reports to the Director. Despite a lot of in-house expertise internally & on the board, there are areas which are more challenging to acquire expertise e.g., legal. Moreover, with the current size of the board it can be challenging to maintain representation from all key stakeholders e.g., clinical, business, legal and patient.

In terms of maintaining a sustainable financing, NCRI undoubtedly benefit from continued funding from the Department of Health. The relationship with the Department is strong and is maintained via quarterly meetings. However, in line with other publicly funded bodies, challenges presented by this model include difficulty in scaling operations due to annualised budget. Multi-year financing in this case might facilitate more strategic long-term recruitment, work and projects for the registry.

4. EXPERIENCES IN MAINTAINING HIGH-QUALITY & EFFICIENT OPERATIONS

In order to populate the registry, NCRI rely on a number of sources of data. Hospitals/health centres feed data into the registry on diagnosed patients; this information allows NCRI to identify whether this is a new patient to the registry or one that was previously identified. The process of identifying patients and adding data can be challenging due to a lack of single identifier; however, with the continued implementation and roll-out of the Individual Health Identifier (IHI), NCRI hope to facilitate more accurate matching of records to patients across hospital sites and clinical settings in the future.

A team of around 20 full-time Electronic Cancer Data Registrars (eCDRs) review and validate initial information received and enter subsequent data primarily based on paper medical records. Data is also fed into the NCRI from the Central Statistics Office on recorded deaths. Likewise, where possible, NCRI receive data from other settings, including hospices, although this information is not fully accessible across all settings in Ireland. Primary data points relate to diagnosis, survival and treatment pathways for patients, although data on treatment is not always readily available. The National Cancer Information System collects data on medical oncology treatments; it is hoped that this data source will further improve treatment data that is captured by the NCRI in future.

As well as being able to find the relevant data, NCRI are reliant on their team of eCDRs working across data entry sites. In some cases, this may be 1 eCDR per site creating key person dependency; this presents challenges to new eCDRs at a site who will need to gain access to site systems/data. Across the board, there is no single process to get access and being 'external' to hospitals often means it can take 3-6 months for an eCDR to get the access they require. A further challenge is the fragmentation in hospital systems/procedures in Ireland, including differing pathology reports and medical records. Despite some of the challenges in actually accessing data, with over 30 years' experience, the NCRI are confident in the processes and procedures that they have in-place regarding data

quality, audit and processing. Moreover, feeding data into European and global level databases (e.g., European Cancer Information System ECIS, European Cancer Inequalities Register, International Agency for Research on Cancer, EUROCARE, International Cancer Benchmarking Partnership, CONCORD, Cancer in 5 Continents) also provides check-points for data quality.

NCRI enter and process data via an in-house technology platform which has been developed with significant investment in the IT infrastructure over the last 10 years. Despite the size of the IT infrastructure implementation project, NCRI heavily invested in taking on the project in-house. Prior to this, NCRI were operating on a distributed model for data collection and processing which was fit-for-purpose at the time; however, that model presented difficulties in the face of increasing amounts of data and security concerns over time leading to the adoption of a centralised database structure.

A secure, centralised, in-house database offers NCRI distinct advantages including control over system structure and functionality, economies of scale, high levels of database security, and the ability to adapt and grow the database on-demand over time in response to changing disease landscapes. The latter also may present challenges in terms of having too much scope to change and asking the question of how much change should be made to the database? A further challenge with an in-house system is IT human resource demands and the dependence on the individuals who have developed and implemented the database over the last decade; a concern for NCRI is the potential loss of experience and skills that comes with hosting the database internally. To mitigate against this risk, there are a third-party company who have some familiarity with the technology should any input/resource ever be required.

In terms of supporting the work of the registry, NCRI operate with a team of around 50 staff across their 4 functions. This reflects a scaling-up over the last couple of years, but NCRI have had challenges with recruitment. A perfect storm of staff moving on & the need to fill multiple vacancies has at times left the team light on the ground. A current priority is building up the research team – it is recognised that this team is vital to ensuring the registry data is used in appropriate and impactful ways including in policy work and communicating the value of the registry. More generally there is a feeling that recruitment in the sector is faced with challenges around finding, attracting and maintaining the right skill-sets, changing viewpoints of working life and the cost-of-living crisis.

5. EXPERIENCES IN FACILITATING & BENEFITING FROM PARTNERSHIPS

Relationships are pivotal to the work of NCRI. The team maintain strong links with the Department of Health to ensure the value of NCRI is communicated and prioritised. Likewise, good contacts and networks with the HSE have bolstered the work of the registry and ensured access to hospital sites be facilitated.

NCRI have relationships and partnerships with multiple agencies, working groups and stakeholders aiming to strengthen the impact of registry work but also to ensure that registry data is informed by and can influence change at different levels of policy and practice. For example, NCRI have a memorandum of understanding with the National Screening Service, HSE and have a strategic and working groups that conduct bodies of work that are meaningful to both organisations. We have a standing forum with the National Cancer Control Programmes where mutual priorities are discussed.

NCRI staff are on multiple HSE groups related to cancer, representing the Registry. NCRI also have a formal arrangement with University College Cork with NCRI staff providing lectures and we host public health students in the NCRI and the team have research partnerships with other academic institutions, in particular with the RCSI.

Understanding the needs of service providers help the NCRI to target their outputs to meet their needs. A multidisciplinary overview of the data that we collect is also helpful and data insights are gleaned from working with people from different backgrounds. A good understanding of the changes in service provision is also essential in the interpretation of NCRI data.

Internationally, more formal partnerships are being established at the European level and the registry hope to be in a position to capitalise on these partnerships in the next year.

6. EXPERIENCES IN GENERATING, EVALUATING AND COMMUNICATING IMPACT

The NCRI holds a very valuable position in Ireland in that we are seen as a trusted source of data and cancer planning is subsequently seen to be data driven. The NCRI does not take this trust lightly but works hard every

day to obtain the highest quality data and to feedback appropriately to policy makers, service providers and the public.

Data from the NCRI has underpinned every cancer strategy starting with the first one in 1997. Data provided by the NCRI for the 2006 National Cancer Strategy showed inequitable outcomes across Ireland and relatively poor outcomes compared to our European peers. Irish evidence provided by the NCRI supported policy makers in their decision making to establish the National Cancer Control Programme.

The NCRI is seen as a high-quality registry in Europe (where many cancer registries are not population based) and the team receive a lot of queries from people seeking to establish registries for cancer and other diseases

The NCRI vision is excellent data insights for better cancer outcomes and the NCRI seeks to remain the final word when it comes to evaluation of cancer control at a population level. They monitor communications and website traffic as intermediate estimates of impact.

NCRI are lucky to be given a lot of opportunities to present findings directly to policy makers and service providers which helps to ensure that reports are seen and data insights communicated. They also work to publish elements of reports in peer reviewed journals to ensure their longevity in the scientific community.

National & International Skin Registry Solutions (NISR CLG) CLG case study

1. REGISTRY PROFILE

The National and International Skin Registry (NISR CLG) Solutions CLG are a registered charity and registered company (Company Limited by Guarantee) who are the umbrella organisation overseeing the coordination and operations of several dermatology registries in Ireland. These registries include:

Registry name	Disease area	Developmental stage	Years in operation
The UK-Irish Atopic Eczema Systemic Therapy Register or A-STAR	Atopic Eczema	Early operations	2
GRASS Ireland (The Global Registry of Alopecia areata disease Severity and treatment Safety)	Alopecia Areata	Early operations	2
EB Registry Ireland	Epidermolysis Bullosa	Mature operations	4
NF-1 Registry	Neurofibromatosis type 1	Planning/design phase	<1
Covid-related	Atopic Eczema and Alopecia Areata	Post-operational	3

NISR CLG has been in operation since 2015 with the individual registries having been set-up and running for between 1-4 years during that time. NISR CLG operates under the governance of the NISR CLG board with each individual registry having oversight from their own Executive Committee. While the NISR CLG board oversee overall NISR CLG governance matters, the individual registry Executive Committees oversee decisions regarding the collection and use of their respective registry data.

The day-to-day operations of NISR CLG are coordinated by a core team of 4 staff including a CEO, Registry Coordinator, Clinical Research Associate and a Clinician/Informatician. Each registry also has between 1 and 3 contracted staff to support data entry at different sites. Different contracts are required according to the data entry hospital site and data entry requirements of each registry.

Currently, for all registries, data is collected at multiple centres on an encounter basis, meaning following each time the patient visits their hospital clinic, data is recorded. The dermatological conditions represented by each registry are characterised by symptoms and clinical outcomes that can change quickly over time and so an encounter-based system is seen as appropriate for these registries, but places a large data entry burden on those collecting data and requires a lot of coordination and oversight to ensure high data quality is collected.

2. EXPERIENCES ENGAGING WITH PUBLIC & PATIENTS

Public/patients are central to NISR CLG governance. On each registry Executive Committee there is at least one patient or parent representative, with a view to increasing this number over time. Recruitment onto the respective registry Executive Committees is facilitated via patient organisations and the PPI Ignite networks. NISR CLG experienced some challenges in recruiting patients to participate; there was some reluctance potentially due to time commitments required to participate in Executive Committee meetings. Recruitment was also challenging due to large populations in some of their patient populations and thus difficulty communicating directly with these patients, particularly in earlier stages of registry development. The PPI Ignite opportunities noticeboards proved useful in these instances to communicate with and target different groups more widely.

Once representatives were recruited, NISR CLG have had largely positive experiences with patient participation. Time is devoted to train & educate representatives on what their involvement will entail as well as ensuring that meetings are accessible, particularly in terms of language used and the pace of meetings. Patient representatives

are involved in Executive Committee meetings and are invited to weigh in on any decisions regarding registry data e.g., requests for registry data for research.

Public & patient involvement on the Executive Committees has been hugely beneficial for NISR CLG in bringing a patient perspective to group decision-making e.g., patients offer experience of what it is like to receive care & help identify pertinent questions to ask. What becomes clear in meetings is that clinicians and patients can have different needs and expectations from the registries and a challenge is trying to align those views. Whilst the benefit of patient engagement in governance structures is invaluable, the resource required to recruit, train & maintain engagement should not be underestimated.

In terms of patient participation in the registries, NISR CLG has observed that the recruitment of consenting patients to participate in registries has been slow but steady, and they believe that the reasons for this vary. For example, for more severe conditions, patients can often be more motivated to participate but for larger patient populations or for less severe conditions, identifying willing patients can be more difficult. It is also challenging to find clinics that have the available resources to support the registry work in addition to their already very busy day-to-day workload. Overall, participation is good and recruitment is supported by detailed patient information documentation and buy-in from centres to ensure patients can ask questions of what participation in the registry means. Also, the population of Ireland is small which reduces the enrolment potential across most conditions. In future, once outputs are available, NISR CLG hope to be able to communicate outputs with patients including through annual reports & lay-person summaries/communications; the first NISR annual report is forthcoming.

3. EXPERIENCES IN ESTABLISHING & MAINTAINING GOOD GOVERNANCE & SUSTAINABLE FINANCING

NISR CLG is a registered charity and Company Limited by Guarantee operating under a constitution. NISR CLG operates under the governance of a NISR CLG board with each individual registry having oversight from their own Executive Committee. One of the main challenges of operating as the umbrella organisation for several registries is sustainable core funding; naturally funding sources want to fund a particular disease area/condition. Managing different funding streams for each individual registry presents sustainability concerns at the NISR CLG level.

Good governance at NISR CLG is supported by a robust and clearly established governance structure and constitution. Moreover, good communication between the board and staff teams ensure key information, concerns and other matters are shared easily and acted upon.

4. EXPERIENCES IN MAINTAINING HIGH-QUALITY & EFFICIENT OPERATIONS

Across current operating registries, NISR CLG have well-established data entry protocols; an important aspect of establishing the NISR CLG registries and data entry protocols was clearly defining datasets which can be harmonised internationally. Establishing clearly defined datasets takes time, resource and multi-stakeholder input, even before data entry can begin.

Despite the well-established protocols in place, NISR CLG have been faced with challenges in accessing data, particularly historical data. Moreover, data entry is restricted by the amount of resource required to enter data based on accessing paper charts. Not only does working through paper-based clinical charts take time, a significant amount of clinical expertise and logic is required to interpret chart notes and enter the correct information onto the registry.

The above operations are also strongly dependent on the technology platform used by NISR CLG. A strength of their current technology is its ability to facilitate adapted data entry and changes to variables, the ability to investigate the data entered, and query data points directly with data collectors. In future, NISR CLG hope to see their platform support more instantaneous reporting and dashboards for use in the clinical practice setting and within the registry to support data quality.

In developing new registries, NISR CLG have also experienced significant road-blocks in navigating hospital level ethics committees and Data Protection Officers. Having to submit individual ethics applications and Data Protection Impact Assessments (DPIA) to each centre paired with differing interpretations of what a patient registry is and data protection regulations has stalled potentially valuable work being done by dermatology groups trying to establish a registry and required significant resource from the NISR CLG team.

NISR CLG operations are supported by current staff and their wide-ranging skills & expertise. Staff with clinical backgrounds, expertise in data protection and health informatics, and skills in project management, administration and bureaucratic skills are core to the day-to-day functioning of NISR CLG. Concerns for NISR CLG remain over how easy it is for a small organisation to attract such skills & expertise in the face of a cost-of-living crisis. Moreover, depending on a small number of individuals for such skill-sets poses risks for NISR CLG in terms of siloed skill-sets and key person dependency. While NISR CLG are able to operate in a high-quality & efficient way on a day-to-day basis, the team lack some core skills and expertise which limit what they can do. These core skills and expertise relate to access to statisticians and legal advice (particularly for contracted staff). Currently NISR CLG can lean on outside resources and links with other registries on an ad hoc basis but recognise this isn't sustainable in the long-run.

5. EXPERIENCES IN FACILITATING & BENEFITING FROM PARTNERSHIPS

NISR CLG have benefited from a number of partnerships. Several of the individual NISR CLG registries engage with their respective patient organisations, which particularly supports patient & public engagement. NISR CLG also partner informally with other groups e.g., with registries inside & outside of their own disease areas; such partnerships allow NISR CLG to draw on knowledge and experiences of more established registries that have been active in the Irish clinical landscape for an extended period.

The partnerships with registries focused on the same disease/condition areas initially facilitated the harmonization of data to meet international standards. Once the registry is established, these partnerships also support the composition of oversight committees for the members involved. On the other hand, further collaborative projects can move slowly with great resource required to maintain them before benefits can be realised over the longer-term.

6. EXPERIENCES IN GENERATING, EVALUATING AND COMMUNICATING IMPACT

While NISR CLG, and its associated registries, is still in its infancy, the team are always considering the potential impact of their work. Currently however, the main challenge is making researchers aware of the existence of the registries so that they are aware it is a resource that can be used to generate impact. Likewise, wider partnerships, as described above, have enabled NISR CLG to engage on an international stage, resulting in impact. Within the GRASS registry, publications by Dr Wall have been the result of both international collaborations and the use of registry data (see publications list below). An example of this is a paper which came about as a result of international collaboration on covid-related registries – Dr Wall & colleagues demonstrate the value of the rapid deployment of covid-related registries in response to the pandemic as a case example of what can be achieved. Likewise, NISR CLG are also using emerging registry data to support arguments for access to new therapies to treat Alopecia Areata using registry data.

There is also acknowledgement that impact generation can be slow when collecting the data takes time, primarily due to lack of funding for man-power to perform data entry and a lack of electronic health care record to pull data from.

Examples of NISR CLG work & impact (any papers/involvement in international groups)

1. Global reporting of cases of COVID-19 in psoriasis and atopic dermatitis: an opportunity to inform care during a pandemic. Mahil SK, Yiu ZZN, Mason KJ, et al. Br J Dermatol. 2020 Aug;183(2):404-406. doi: 10.1111/bjd.19161. Epub 2020 Jun 10.
2. International collaboration and rapid harmonization across dermatologic COVID-19 registries. Freeman EE, McMahon DE, Hruza GJ, et al. J Am Acad Dermatol. 2020 Sep;83(3):e261-e266. doi: 10.1016/j.jaad.2020.06.050. Epub 2020 Jun 17.
3. The Alopecia Areata Consensus of Experts (ACE) study: Results of an international expert opinion on treatments for alopecia areata. Meah N, Wall D, York K, et al. J Am Acad Dermatol. 2020 Jul;83(1):123-130. doi: 10.1016/j.jaad.2020.03.004.
4. Shedding light on therapeutics in alopecia and their relevance to COVID-19. Fagan N, Meah N, York K, et al. Clin Dermatol. 2021 Jan-Feb;39(1):76-83. doi: 10.1016/j.clindermatol.2020.12.015. Epub 2020 Dec 16.

5. Learning from disease registries during a pandemic: Moving toward an international federation of patient registries. Wall D, Alhusayen R, Arents B, et al. Clin Dermatol. 2021 May-Jun;39(3):467-478. doi: 10.1016/j.clindermatol.2021.01.018. Epub 2021 Apr 6.

6. A Global eDelphi Exercise to Identify Core Domains and Domain Items for the Development of a Global Registry of Alopecia Areata Disease Severity and Treatment Safety (GRASS) Dermatology COVID-19 Registries: Updates and Future Directions. Freeman EE, Chamberlin GC, McMahon DE, et al. Dermatol Clin. 2021 Oct;39(4):575-585. doi: 10.1016/j.det.2021.05.013. Epub 2021 May 31.

7. The Alopecia Areata Consensus of Experts (ACE) study part II: Results of an international expert opinion on diagnosis and laboratory evaluation for alopecia areata. Meah N, Wall D, York K, et al. J Am Acad Dermatol. 2021 Jun;84(6):1594-1601. doi: 10.1016/j.jaad.2020.09.028.

8. Advances in hair growth. Wall D, Meah N, Fagan N, et al. Fac Rev. 2022 Jan 12;11:1. doi: 10.12703/r/11-1. eCollection 2022.

9. Signposts to the Promised Land in Alopecia Areata. Wall D, Rees H, Bokhari L, et al. J Invest Dermatol. 2023 Jan;143(1):9-10. doi: 10.1016/j.jid.2022.08.031. Epub 2022 Sep 17.

10. The effects of systemic immunomodulatory treatments on COVID-19 outcomes in patients with atopic dermatitis: Results from the global SECURE-AD registry. Musters AH, Broderick C, Prieto-Merino D, et al. J Eur Acad Dermatol Venereol. 2023 Feb;37(2):365-381. doi: 10.1111/jdv.18613. Epub 2022 Oct 12.

11. The Alopecia Areata Severity and Morbidity Index (ASAMI) Study: Results From a Global Expert Consensus Exercise on Determinants of Alopecia Areata Severity. ASAMI Consensus Survey Study Group, JAMA Dermatol. 2024 Mar 1;160(3):341-350. doi: 10.1001/jamadermatol.2023.5869.

NISR CLG Solutions CLG Presentations:

- NISR CLG Solutions CLG Registries And How They Were Built 2nd of February 2023

Contributed to the 2023 HRCI Guidance document

- Unlocking the Potential of Patient Registries: A Guide for Success

Cystic Fibrosis Registry of Ireland case study

1. REGISTRY PROFILE

The Cystic Fibrosis Registry of Ireland (CFRI) is the patient registry for people living with Cystic Fibrosis (CF) in Ireland. Since the early 2000s, the registry has been collecting data on consenting participants. The registry was originally housed within the CF patient association (CF Ireland), but latterly became an independent organisation; CFRI is a registered charity and Company Limited by Guarantee. The registry is overseen by a board.

Currently the registry collects data on around 1400 patients representing just over 92% of the CF population in Ireland from all CF centres across Ireland. The registry collects data on an encounter basis, meaning each time the patient visits their CF centre, the registry captures the data based on information in the patient's medical record. Registry data is used by a number of stakeholders for a variety of purposes including: healthcare planning, post-authorisation safety & efficacy studies, and research.

2. EXPERIENCES ENGAGING WITH PUBLIC & PATIENTS

Over the years, CFRI have engaged and involved patients/public in a number of ways. The registry was originally founded by the CF Patient association (CF Ireland), with the registry later becoming an independent organisation. Nevertheless, this foundation in the patient association and strong relationships built as a result have facilitated much of the patient-facing engagement work of the registry. Moreover, there is representation of CF Ireland on the board of CFRI, ensuring the patient voice stays central to registry operations.

People with CF (PwCF) have also had input into a number of research projects led by CFRI. For example, a recent research proposal on pregnancy in CF was guided by the input of a focus group containing women with CF who have experienced pregnancy. The input was greatly valued and strengthened many aspects of the project proposal.

In terms of communicating registry outputs, CFRI ensure that data outputs are as accessible as possible, making use of infographics and social media to communicate about CFRI work. CFRI also contribute quarterly to the patient association publication aimed at PwCF and their families. CFRI recently ran a 'you ask, we answer' feature in this publication to get feedback from PwCF about what they would like to know about the registry.

Improving public awareness of the registry is important to the team; however, challenges arise as CFRI do not have direct contact with PwCF and so are reliant on other channels to communicate. Despite this, participation in the registry is excellent; the registry operates on an informed consent model where consent is undertaken through CF centres. The registry has consistently maintained over 90% coverage of the CF population in Ireland. Nevertheless, there are challenges in ensuring all parents of babies diagnosed via newborn screening are given the opportunity to consent and in implementing a programme of re-consent as a requirement of GDPR.

3. EXPERIENCES IN ESTABLISHING & MAINTAINING GOOD GOVERNANCE & SUSTAINABLE FINANCING

The registry was initially established as a pilot study that was jointly funded by the Department of Health and CF Ireland and was organisationally based within the association. However, the decision was made later on by the patient association that the registry should be an independent organisation to solidify its position as trusted source of data. This was important during years where advocacy work was critical to securing services for PwCF. CFRI is a registered charity, a registered company limited by guarantee and maintains strong governance structures in compliance with the Charities Regulator and the Companies Registration Office. CFRI operates under a constitution and is overseen by a board and a variety of subcommittees relating to governance and risk, finance, and research. Maintaining good governance at the registry is resource-intensive but essential for ensuring ethical operations and high-quality performance.

Despite being in existence for over 2 decades, the registry has continually had to fight for sustainable financing options. In 2011, the registry signed a Service Level Agreement contract with the HSE which secured a small amount of core funding to the registry – while this does not cover the full operating costs of the registry, it has provided a level of security in times where registry funding was limited. Outside of this core funding, the registry has relied on grant income from industry, involvement in large-scale research projects and one-off grants. Current registry financial planning is secure for the next 3-4 years; however, beyond this timeline, funds are less

secure. In more recent years, larger funding streams from industry have allowed the registry to increase resources in terms of staff and to upgrade its registry platform. The ongoing costs associated with both these measures are considerations for future financial planning efforts.

4. EXPERIENCES IN MAINTAINING HIGH-QUALITY & EFFICIENT OPERATIONS

CFRI maintains high-quality data via a number of mechanisms. The team have a strong set of policies, procedures and training modules in place regarding all phases of data management. The policies and procedures are signed off by senior management and are reviewed annually. To support data quality, CFRI implement an annual programme of data validation visits, whereby a subset of CFRI data for a sample population at a CF centre is validated against source data in medical charts.

CFRI feed annualised deidentified data into the European Cystic Fibrosis Patient Registry (ECFSPR), which captures data from over 54,000 PwCF. Not only does this annual submission support data quality, e.g., by flagging missing data, it also supports overall data harmonisation and standardisation in the registry ensuring CFRI data is collected in the same way as international counterparts, which in turn supports global research and analyses. CFRI, as part of ECFSPR, has been qualified by the European Medicines Agency as an appropriate platform for the collection of CF data for Post-authorisation Safety and Efficacy Studies (PASS), supporting regulatory decision making for medicines used in the treatment of CF. This reflects the high quality, coverage and utility of the data set to important observational, real-world evidence studies.

That being said, despite best efforts, there are some practical challenges which impact upon data quality in the registry which can be difficult to overcome. This includes a lack of electronic health record system and at times, limited access to paper charts and differences in chart management practices from centre to centre. Likewise, it can be difficult to get access to centres themselves, particularly where space is limited, as well as any online systems (e.g., for lab results) – each time a new data collector needs to be set-up at a centre, it can take some time to ensure all adequate access to such systems is facilitated.

Data entry at the registry is supported by a database technology platform, which the registry has been lucky to update over the years. Most recently the registry migrated to a new version of their database which entailed a meticulous process of data mapping, review of the CFRI variable list and restructuring of some elements of the database. The registry platform has significant adaptability and scalability to allow changes and additions as well as the facility to conduct registry-based trials as 'add-on' modules to the platform. Despite this, the registry is mindful of the significant costs associated with not only maintaining the platform but also with making changes. This limits the extent to which changes can be made.

The registry currently operates with a very dedicated and experienced team. Benefiting from more significant funding has enabled CFRI to maintain and build skills in statistics, data analysis, and clinical research amongst others. However, in an ever-growing data environment, there is a huge demand for additional resources and skills. The lack of electronic records and unique health identifiers puts a huge burden on data collection. More resources and expertise are required for data protection and in terms of meeting the requirements of the forthcoming introduction of the European Health Data Space and the use of secondary health data. As CFRI move forward they recognise the requirement to maintain the skills-sets they have and build on this as handling and processing of health data becomes more complex. The registry is also mindful of being a small team which can lead to key person dependencies; while documenting policies and procedures goes some way to mitigating against this risk, staff turnover is a concern for the registry.

5. EXPERIENCES IN FACILITATING & BENEFITING FROM PARTNERSHIPS

CFRI have benefited from partnerships with multiple stakeholders. Partnerships with more formal agreements and contracts have both provided financial benefit to the registry and ensured the use of registry data in large-scale research and post-authorisation studies. Such partnerships include an SLA with the HSE, multi-year research grants with industry, and involvement in multi-year large-scale research projects at both the national & international level e.g., RECOVER.

Likewise, more formal arrangements of sitting on steering groups and working groups helps support public awareness of the registry, particularly in clinical, research, and wider registry circles. The registry sits on both the Steering Group and Executive Committee of the European Cystic Fibrosis Patient Registry and the Global Registries Group. The registry has a nominated representative on the National Clinical Programme for Cystic

Fibrosis. The registry also leads on the Future of Registries Taskforce. CFRI offices are located within University College Dublin and a member of the team holds an adjunct Professorship within the School of Public Health, further solidifying the links with public health research and facilitating collaborative projects.

In addition to more formal partnerships, the registry acknowledges the benefits from strong relationships with a variety of groups of stakeholders which have been built throughout the years including clinicians, Multidisciplinary Team Members, and researchers throughout Ireland. The team acknowledge the value such relationships have in terms of public awareness of the registry as well as buy-in and trust in registry data. However, it is not underestimated that these relationships require maintenance through regular meetings and communication.

6. EXPERIENCES IN GENERATING, EVALUATING AND COMMUNICATING IMPACT

There are a number of examples of the tangible impact of the CF registry across policy, research and practice.

Cystic Fibrosis Ireland commissioned the Pollock report, published in 2005. The ultimate aim of the report was to recommend measures to structure CF services in Ireland and improve patient outcomes. Registry data was central to evidencing and supporting the recommendations of the report; this included data on patient numbers, patient distribution in CF centres in Ireland, and data to support healthcare utilisation. Latterly, the registry is mentioned as a core component of CF care in the National Clinical Programme for CF (NCPCF). The registry currently sits on the working group of the NCPCF.

The registry has also been able to establish a strong and impactful research programme. The registry team has undertaken a wide variety of research projects in-house and also been involved in numerous external research projects including large-scale real-world evidence studies, e.g., RECOVER, Post-authorisation Safety and Efficacy Studies, and European and global collaborations.

The registry communicates this impact through its annual reports and in particular through its 20-year celebration report which describes the journey of CFRI alongside presenting key longitudinal data trends.

There is a challenge in capturing the full impact of the registry. The registry has an important role in providing specific unbiased data to support policy makers, the patient association and service providers. While much of this impact can be captured through the above examples, the registry also provides such support on an informal day-to-day basis through conversations with these groups. Moreover, due to its established role in the registry landscape in Ireland, the CFRI continues to play an advisory role for many registries looking to establish operations and alongside this, CFRI lead the Future of Registries Taskforce aiming to build a consensus document on recommendations for the future of patient registries in Ireland.

Appendix 6

Examples of ERN rare disease registries

ERN Registry Examples

Although ERN Registries are established around broadly common objectives - models and structures may vary - this is demonstrated in the summary descriptions of selected ERN Registries below.

i. REDgistry

The Rare Eye Diseases European Registry currently in development is being led at European level from the Mater Misericordiae University Hospital. This registry will be structured around three tiers: REDgistry - collated All Rare Eye disease data; RETgistry – disease grouping eg retina, paediatric, cornea neuro-orph; and detailed disease specific data eg LHON, XLRP, Leighs.

Further information is available at https://redgistry.eu/

ii. EURR Bone Registry

EuRR-Bone is the first pan-European registry for Rare Bone and Mineral Disorders (RBMD) and is linked to the ERN for rare bone diseases ERN BOND. It is also closely linked to the European Registries for Rare Endocrine Conditions (EuRRECa), the registry that support the European Reference Network on Rare Endocrine Conditions (Endo-ERN). Collectively these registries are known as EuRREB and both are managed by a team at the Leiden University Medical Centre in the Netherlands. EuRREB consists of two platforms e-REC and the Core Registry.

E-REC is an electronic reporting system of new cases of any condition reported by the network(s). No personal identifiers are collected and no informed consent from the patient is required. An example would be a report of 10 new patients with osteogenesis imperfecta having been seen at the Irish centres in 2023. From 2025 the EU has made it a mandatory requirement for participating ERN centres to enter their data in E-REC.

The Core Registry collects common and condition specific datasets for a wide range of endocrine and bone conditions. These include the European standard common data element set for rare disease registration as recommended by the European Platform on Rare Disease Registration (EU RD platform) as well as condition-specific and patient-reported outcomes. Clinicians can enter data, complete outcomes, and view their own patients and those entered by contributors from the same centre. Patients can view the data entered by their clinician and complete condition-specific and generic outcomes assigned to their role.

Both platforms can be uses by members of both ERNs but also by non-ERN HCPs who have a special interest in RBMDs.[7]

Further information, including a list of publications arising, is available at https://eurreb.eu/about/eurreb/.

iii. ERKReg

ERKReg is a registry for all patients with rare kidney diseases linked to the European Rare Kidney Disease Reference Network (ERKNet). It collects core demographic and diagnostic information as well as selected disease- and treatment-specific prospective follow-up data. It is designed to generate epidemiological information, identify current patient cohort for clinical research, explore diagnostic and therapeutic management practices, and monitor treatment performance and patient's outcomes. It has a modular design that allows integration of sub-registries (registry studies) to the core database that will collect more detailed information on individual rare kidney diseases or disease groups.

The registry is also open to non-ERKNet sites, and a growing number of sites in non-EU countries.[9]

Further information is available at https://www.erknet.org/

iv. ERN-LUNG Registry

ERN-LUNG has two registries - ERN-LUNG PRIME (Patient Registry for Improving Medical Excellence) and BREATHeREGISTRY, a patient driven registry.

ERN-LUNG PRIME is designed to improve the diagnosis and standards of care for people with rare respiratory diseases. It gathers data on disease symptoms, course and treatment and on the quality of care and utilization of services within ERN-LUNG. It enables harmonisation of data on respiratory patients across the EU to address key epidemiological, cohort composition, care quality and care resource questions. It makes data available to researchers, public authorities, industry, and other stakeholders.[1]

The BREATHeREGISTRY is a population registry that collects self-recorded data from patients directly, irrespective of whether they are seen by an ERN-LUNG member or in another specialized institution. Experts can track patterns in disease progression, treatment outcomes and patient demographics. This enables researchers to identify knowledge gaps and research needs. By gathering information from patients' descriptions, specialists can examine trends that can direct the development of new drugs, therapies, and diagnostic techniques.[1]

urther information is available at https://ern-lung.eu/patient-registry/ern-lung-registry/

The Future of Registries Taskforce (FoRT) is a multistakeholder coalition of registry operators, clinicians, researchers, and industry representatives, and patient representatives from across the public, private and voluntary sectors committed to advancing patient care through high-quality patient registry data. The taskforce is chaired by Godfrey Fletcher, CEO of the Cystic Fibrosis Registry of Ireland (CFRI) and CEO of National & International Skin Registry Solutions (NISR). The publication of this report has been co-funded by CFRI & NISR.

FoRT

Future of Registries Taskforce

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