Saving lives with patient data registries

amrc
1. Summary

2. Introduction

3. Why are registries important for patients?
   3.1. Registries support research
   3.2. Registries support care and service planning
   3.3. Registries accelerate clinical trials
   3.4. Registries support more rapid access to safe medicines

4. Where do charities add value?

5. How the UK can build on the potential of charity registries

6. Background
1. SUMMARY

• Health data has the power to revolutionise care and research, bringing transformative outcomes for patients and boosting the UK’s economy. It is vital that the potential of charity-supported registries in the UK’s life sciences sector is harnessed to ensure maximum benefit to UK health and wealth.

• Patients are at the heart of charity registries, some of which are being used in pioneering ways to lower barriers to research, drive efficiency and reduce costs. Many members of the Association of Medical Research Charities (AMRC) fund registries and innovative data-collections. From well-established registries such as the Cystic Fibrosis Trust’s Cystic Fibrosis Registry and Crohn’s and Colitis UK’s Inflammatory Bowel Disease Registry, to smaller ones such as Reverse Rett’s registry for patients with the rare condition Rett Syndrome, charity-supported registries play a crucial role.

• Patient registries are standardised collections of information about patients that are usually disease specific. The information collected into registries may include a patient’s medical history, disease diagnosis, NHS number, and Patient-Reported Outcome Measures (PROMs) data. Collected data can help produce a real-world picture of disease, current treatment practices, and outcomes.

• Registries can make a significant impact across the research process, ultimately resulting in accelerated patient access to new medicines and treatments. From supporting basic research by revealing more about the natural course of a disease, to helping the translation of research by aiding evaluation of clinical trial feasibility, as well as supporting innovative clinical trials, registries play crucial roles. Registries also contribute to bringing treatments to patients once a drug has been licenced for use, through drug safety monitoring, and the drugs commissioning process that deems new treatments suitable for NHS prescribing.

• The involvement of medical research charities adds significant value to registries. Not only do charities fill gaps where other organisations are not delivering data collections, charity-supported registries have a unique focus on research, and benefit from a range of expertise, facilitated by charities’ wide networks and collaborations. Charity involvement in a registry also brings a high level of trust, as charities strive to exceed the high level of accountability and transparency expected of them.

• The contributions that charity registries make to the diversity and strength of the UK’s data sources should be built on as government seeks to deliver the vision outlined in their Industrial Strategy. The potential of charity-supported registries should be harnessed for the benefit of patients through a supportive environment that is driven by government and relevant healthcare policymakers, including NHS England, NHS Digital and Public Health England.

• The Life Sciences Industrial Strategy recommended the establishment of new registries across the NHS in England, with involvement of the relevant charity. To take this forward, as part of the life sciences ‘sector deals’ process, government should work with the relevant healthcare bodies and charities to help create the optimum environment for data registries. Registries can also help to deliver the Strategy’s vision for clinical trials.

• In an environment where the NHS is looking to cut costs and optimise processes, registry data can support improvements in care and support planning, as well as accelerating the development of medicines. This would ultimately deliver operational savings to the healthcare system, aligning with the aims of NHS England’s Long-Term Plan.

• Data linkage must be a key consideration for the future of data registries. As the NHS modernises and electronic care records become mainstream across the system, it will be important to ensure that registries and other data sources are compatible with new systems. It is important that charity registries and data collections are supported in aligning their processes with new developments across the NHS and healthcare system, including the development of new Digital Innovation Hubs led by Health Data Research UK.

• As new data-driven technologies are developed, charities can play an important role, bringing registry data, the patient voice and charity expertise to the collection and use of patient data. Charities should be considered as important partners in the development of ground-breaking new data initiatives, bringing expertise and knowledge of their patient community.

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2. INTRODUCTION

Medical research charities fund a range of registries that collect information from people affected by medical conditions. These registries are of considerable value to the UK’s research landscape as they:

• facilitate and accelerate research;
• help to improve patient access to new treatments; and
• provide the evidence base to deliver better care and NHS services to patients.

The Association of Medical Research Charities (AMRC) is the membership organisation of the leading medical and health charities funding research in the UK. We represent 140 medical research charities of a wide range of size including Wellcome, Cancer Research UK, the British Heart Foundation and Versus Arthritis as well as smaller organisations. In 2017, AMRC’s members collectively invested over £1.6 billion in UK medical research; almost half of the total public investment and more than the Medical Research Council (MRC) or National Institute for Health Research (NIHR).

While the NHS is an unparalleled data source encompassing the diversity of the UK’s population, not all data are collected and accessible through the NHS. In many cases, data is currently not collected nor easily accessible through national bodies such as NHS Digital and Public Health England, the two main national data repositories.

Registries can play a significant role in accelerating patient access to medicines by supporting various stages of the research process. This includes enabling basic research by revealing more about the natural course of a disease, research translation by aiding evaluation of clinical trial feasibility, innovative clinical trials and drug safety monitoring after medicines are licensed for use.

Registries can be essential tools in patient-driven innovation. With the cost of developing a new medicine rising from around $1 billion in 2010, to just under $2 billion in 2018, the UK must harness their power to help drive research translation

Patients are at the heart of charity registries. Many AMRC members fund registries and innovative data-collections, of which several are being used in pioneering ways to lower barriers to research, drive efficiency and reduce costs. This report highlights the variety of ways in which charity-funded registries are important for patients.


3. WHY ARE REGISTRIES IMPORTANT FOR PATIENTS?

Registries can be extremely important tools that aid researchers, clinicians, and industry; by lowering barriers to conducting research and speeding up the development of new treatments. Centralised registries containing patient information help drive efficiencies at different stages of the medicines development process, thereby decreasing the cost of translating research into clinical practice.

As outlined in the following sections, registries benefit patients by facilitating research, accelerating clinical trials and supporting faster access to safe medicines.
The contribution of registries across the pathway to new patient treatments

Stage along the pathway

Medical and care research – Sections 3.1 + 3.2

Clinical trials

Supporting decision-making when approving new treatments in the NHS

Drug safety monitoring

Identifying unmet medical need

Feasibility studies: Identifying eligible participants for clinical trials

Providing the evidence base for NICE and NHS commissioners to approve the use of new treatments

Monitoring the safety of newly licensed medicines

Understanding the progression and prevalence of disease

Novel clinical trial design

Charity involvement

Improving care and service planning across the NHS

Feasibility studies: Identifying eligible participants for clinical trials

Drug safety monitoring

Monitoring the safety of newly licensed medicines

Where registries add value

Charity involvement
3.1. REGISTRIES SUPPORT RESEARCH

Learning more about the progression and prevalence of the disease

For many rare conditions, the number of patients present in the UK is based on estimates. In many cases, charities are the only organisation centrally collecting data for their disease area. Therefore, registries can be valuable tools to gain a more accurate understanding of the prevalence and geographical spread of disease. This can help inform the way that NHS services are planned and allocated. Furthermore, this can identify areas of unmet need, and provide essential insights when charities are planning research and care.

CASE STUDY: MND Association – MND Register

Motor neurone disease (MND) is a rare, rapidly progressive neurodegenerative disease that is thought to affect about 5,000 people in the UK at any one time. As there is no single source of information about who is affected, there is a vital lack of information about MND, including the true number of people with the condition. The MND Register, part funded by the MND Association, is working to enable the collection and storage of information in a central location about every person living with MND in England, Wales and Northern Ireland. This will allow the determination of the true incidence and prevalence of the disease.

The MND Register will also help researchers understand characteristics that make a person more likely to develop MND, learn more about disease progression and provide information for better care planning for people living with MND.

The MND Association also has a biobank of whole genome DNA samples and cell lines which are helping researchers grow stem cells that can be programmed into motor neurones. This biobank, called MND Collections, allows researchers to understand how genetic mutations are affecting motor neurones and causing them to die, as well as the possibility of testing potential drugs targets directly on motor neurones from people living with MND.

For more information:
https://mndregister.ac.uk/
https://www.mndassociation.org/research/for-researchers/ukmndcollections/

While not strictly fitting the definition of a traditional disease registry, several charities have funded biobanks, which are collections of biological material (i.e. human tissue) used in research projects. Like more traditional patient registries, biobanks are often associated with the collection of clinical and health data.

Furthermore, charities are responsible for funding some world-leading biobanks, which are helping researchers learn more about the causes and progression of various diseases. Again, this highlights charity commitment to funding innovative data collections for patient benefit.

CASE STUDY: Bloodwise – Haematological Malignancy Research Network

Haematological Malignancy Research Network (HMRN), a specialist blood cancer registry funded by the blood cancer research charity Bloodwise, it was possible to calculate the duration of time between onset of symptoms and diagnosis. Time-to-diagnosis could be unacceptably long. Patients experienced many different symptoms, depending on the type of blood cancer they had and the part of the body it was affecting. Many symptoms were not included in UK Referral Guidelines. Some patients did not have any symptoms at all before diagnosis, but were diagnosed incidentally from a routine blood test, often for another health issue. Evidence from the HMRN registry can be used to guide future Referral Guidelines, with findings about variations between disease subtypes being particularly useful in identifying different strategies to promote early diagnosis.

For more information see:
https://www.hmrn.org/home

Better diagnosis

Studies using registry data can reveal information about how patients are diagnosed with their condition. This can lead to better awareness and more tailored diagnoses. This can especially be the case where the disease is rare or less common. The small numbers of patients cared for by any one centre provides challenges to effective diagnosis. Analysis of data from larger patient populations helps doctors diagnose patients earlier by monitoring trends and identifying diagnostic markers. The ability to make an earlier diagnosis can be key to improving outcomes, lowering the burden on the NHS and ultimately improving patient’s lives. As an example, survival rates for many cancers are significantly higher when diagnosis is made earlier.

CASE STUDY: Alzheimer’s Research UK and Alzheimer’s Society – Brains for Dementia Research

With the support of Alzheimer’s Society and Alzheimer’s Research UK, Brains for Dementia Research was set up in 2007 to establish a network of brain bank facilities across England and Wales, in partnership with the Medical Research Council. So far, over 3,200 people have signed up to donate their brain through Brains for Dementia Research which provides valuable tissue for researchers to study.

This initiative is unique amongst brain banks, as the memory, thinking and behaviour of each prospective donor are monitored throughout their later life through regular assessments. This provides researchers with a complete medical history to accompany the donated brain tissue, allowing them to see how brain changes correlate with symptoms.

Using this resource, a collaborative team working across the UK has rewritten the text book on the features of vascular dementia in the brain. This marks an important milestone in helping pathologists reach consensus on diagnosis, and on enhancing future studies into the condition. The tissue bank has also been used improve understanding of the genetic risk factors for Alzheimer’s disease.

For more information:
http://www.brainsfordementiaresearch.org.uk/

**Better care planning**

Inform the way that NHS services are planned and allocated. This includes providing insights into the geographical spread of disease and can help inform the way that services are planned and allocated. This can help to identify areas of unmet need and provide essential insights when charities are planning research and care.

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For more information:
http://www.brainsfordementiaresearch.org.uk/
3.2. REGISTRIES SUPPORT CARE AND SERVICE PLANNING

Service improvement and planning

Researchers, charities, NHS commissioners and others use registries to improve care for patients and support decision-making in service planning and delivery. Drawing on information from registries ensures that the resources allocated to different NHS Trusts are sufficient, as well as helping to identify inequitable provision of care.

For example, NHS England and NHS Scotland have contracts with the Cystic Fibrosis (CF) Trust for the provision of information from the UK Cystic Fibrosis Registry to help inform the planning and commissioning of their services. The Registry therefore helps the NHS make better informed funding decisions and aims to improve the quality and efficiency of care available to patients with cystic fibrosis. Additionally, registries can provide the infrastructure for collaborative working between research partners to inform and improve patient care – the Inflammatory Bowel Disease Registry (IBD Registry) being an example.

TREAT-NMD is a neuromuscular network that provides international infrastructure to ensure that the most promising new therapies reach patients as quickly as possible. It includes an international registry for patients with Duchenne Muscular Dystrophy (DMD). Data from the UK DMD registry is sent into the international DMD registry and therefore contributes to this global network. TREAT-NMD also provides support for the UK Myotonic Dystrophy registry which is part-funded by Muscular Dystrophy UK.

Other key areas where registries can support improvements in care include:

- monitoring the effectiveness of a service or medical intervention;
- identifying problems and monitoring trends to inform local or national policy; and
- identifying at-risk groups and enabling product recalls.

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CROHN’S & COLITIS UK

CASE STUDY: Crohn’s and Colitis UK – Inflammatory Bowel Disease Registry

The Inflammatory Bowel Disease (IBD) Registry is the first UK-wide collection of anonymised adult and paediatric IBD data for prospective audit and research. The IBD Registry is run by the British Society of Gastroenterology, the Royal College of Physicians and AMRC member, Crohn’s and Colitis UK.

Its aim is to find out more about the long-term impact of the condition and collect better information on treatments and outcomes. By gathering data electronically, the registry provides information that IBD teams can use locally to support patient care, and feed into a national database for benchmarking, quality improvement and research.

With a major focus on biological medicines (biologics), the Registry aims to develop a near-complete UK register of IBD patients on such treatments. This is an increasingly important area with the growing number of biologics and biosimilars becoming available. Furthermore, the IBD Registry provides a comprehensive infrastructure for research partners through its web tool, which is currently used in over 60 sites, thereby facilitating collaborative research projects.

One example is VEST, an investigator-initiated study funded by Takeda, looking at real-world experience of vedolizumab. The Anaemia Service Evaluation, a joint working initiative with Pharmacosmos, evaluated data on IBD patients in five centres. Iron levels were monitored over 12 months to determine how well iron deficiency anaemia is managed. Both projects have shown that the Registry web tool is an efficient and cost-effective solution for studies.

For more information: https://ibdregistry.org.uk/

Better advice and care for patients

Once information is analysed from a registry, the data can be used to give more accurate advice to patients, and improve care pathways. When patients and their families are equipped with more information about their condition, they can make more accurate decisions about their care and treatment.

For instance, The Brain Tumour Charity’s registry/database, BRIAN, will feature an online platform that will be accessible to consenting patients. This online data platform will provide brain tumour patients with content relevant to them, including new information and research news. It will also allow patients to compare themselves to aggregated data to see how other people with brain tumours are doing.

Additional, procedure-based registries, such as the National Joint Registry, can help support tracking of patient outcomes after surgery, which supports surgeons and hospital Trusts to compare themselves to others, thereby identifying areas of improvement.

The Francis report, published in 2013, made recommendations around the use of data registries for evaluating care treatments. The report explained that without registries there may be no way for anyone to know how a service compares to others around the country, with implications for patient safety. Registries are the essential tools that help monitor how effective treatments are, allowing outcomes to be better understood, and, where necessary, to help implement corrective measures.


3.3. REGISTRIES ACCELERATE CLINICAL TRIALS

Clinical trials are an essential feature in the translation of basic research findings into clinical practice. With the involvement of volunteer participants, they help confirm whether medicines and products are safe and effective for their intended purpose. Registries are increasingly able to support and drive innovation at different phases of a clinical trial by accelerating progress and potentially lowering costs.

Conducting clinical trial feasibility studies

In advance of clinical trials, registries are useful tools for helping to identify and randomise patients (i.e. the process of separating patients into comparison groups). Indeed, registries can be used to conduct feasibility studies; helping identify whether there is appropriate infrastructure and suitable recruitment potential to advance to a full randomised controlled trial (RCT).

In the case of charity-funded registries, several charities work with industry partners to help advise on the feasibility of clinical trials. In setting up a clinical trial, there are strict inclusion and exclusion criteria for patients. These ensure that patients are exposed to minimal risk and that the study’s findings will be sufficiently robust to demonstrate the effectiveness of the intervention.

CASE STUDY: Versus Arthritis – Norfolk Arthritis register

People with rheumatoid arthritis have a 47% increased risk of premature death compared to the general population. Therefore, a better understanding of the condition is crucial in identifying the causes of the condition, predicting those most at risk and developing more effective and targeted treatments.

The Versus Arthritis funded Norfolk Arthritis Register (NOAR) involves over 4,500 patients with recent onset inflammatory arthritis and is the largest study of its kind in the world.

The principal objective of the NOAR study is to provide a national resource for detailed studies into rheumatoid arthritis to address the cause of the disease, its onset and progression, changes in disease course, and health service use.

Over 90,000 samples, including DNA and serum, are stored and curated as part of the study. These have been crucial in supporting other research studies, both domestically at the Versus Arthritis Centre for Genetics and Genomics, and in international collaborations.

The study has been key in understanding the incidence of rheumatoid arthritis in the UK and in the identification of risk factors such as smoking, obesity and diet. It has also highlighted the links to other conditions such as cardiovascular disease and the importance of early treatment.

For more information: http://www.nnuh.nhs.uk/departments/rheumatology/norfolk-arthritis-register/
More efficient clinical trials: conducting trials within the registry

Embedding a clinical trial in a registry means that researchers can access routinely collected data held in the registry – like medical history, diagnosis and Patient-Reported Outcome Measures – as part of their trial data. This means the trial can be completed more efficiently, saving money and time.

A registry can be used either as an observational data source which will itself generate clinical evidence to evaluate a new medicine, or as an important reusable part of the clinical trial infrastructure which will support the conduct of RCTs.

There are potentially significant advantages to collecting data through existing infrastructure. This is an area where the charity sector is an exemplar. For example, The CF Registry is currently running an initiative whereby a clinical trial is embedded into the registry. Without the infrastructure of the CF Registry, this trial would not have been able to progress to a full-scale study. This would have deprived patients and researchers of invaluable information and led to potential delays for patients in benefiting from the results of this research.

3.4. REGISTRIES SUPPORT MORE RAPID ACCESS TO SAFE MEDICINES

Pharmacovigilance and accelerating the approval of new treatments

After a new medicine is approved for use by the European Medicines Agency (EMA), the pharmaceutical company who holds the licence will be required to conduct drug safety monitoring, usually for a period of five years after approval, to look at the long-term impact and safety of the new medicine.

As part of the approval process, some companies are expressly required by the EMA to conduct registry-based studies to evaluate the impact of their medicines. This is predominantly the case where there is a high level of innovation and for orphan drugs (drugs intended for rare disease conditions), for which there is high medical need. Often these drugs are licensed for use on the back of more limited clinical trials due to lower numbers of trial participants. The need to establish a drug safety registry to evaluate the impact of a new medicine can act as a deterrent for pharmaceutical companies when they are deciding whether to pursue a license for a new drug. The use of existing disease registries can therefore be a mechanism to overcome this and streamline data collection, ensuring that barriers are minimised for patients in gaining access to new treatments.

In addition, the EMA is currently exploring ways of encouraging collaboration between existing patient registries to support the use of infrastructure already in place. This work aims to evaluate the barriers to collaborative working between existing registries and organisations pursuing a drug license, as well as collating a list of suitable registries that can support the collection of appropriate evidence.10

CASE STUDY: Cystic Fibrosis Trust – embedding clinical trials into registries

Since 2016, the UK Cystic Fibrosis Registry has been used to support the first randomised registry trial in the UK involving children. The Registry, sponsored and managed by the Cystic Fibrosis Trust, holds health data for 99% of consenting people living with cystic fibrosis in the UK.

The CF START clinical trial is assessing whether prescribing antibiotics for infants with cystic fibrosis as a preventative measure reduces infection, or risks exposing children to earlier infection from harmful bacteria. Over a four-year period, half of the participants (who must be under 70 days old at the time of enrolment) will follow the established UK practice of prescribing a long-term antibiotic, usually Flucloxacillin, as a ‘prevent and treat’ approach to combatting bacterial infection. The other half will be put on a ‘detect and treat’ programme, where antibiotics are only given if harmful bacteria are found.

CF START’s innovative approach to clinical trials will benefit patients, researchers, and the wider NHS, potentially resulting in a change to clinical practice on a global level. Results on the Registry will record how these infants are progressing, which means families in CF START will not have any additional burden aside from their usual clinic visits.

For more information: https://www.cysticfibrosis.org.uk/the-work-we-do/uk-cf-registry

CASE STUDY: Cystic Fibrosis Trust – Cystic Fibrosis Registry

Around 10,500 people live with cystic fibrosis in the UK. It is a life-limiting, inherited disease. Disease-modifying cystic fibrosis therapies are being developed for greater numbers of people with the condition. Vertex Pharmaceuticals Inc. have licensed two such medicines for use in Europe; ivacaftor mono-therapy (Kalydeco®) and lumacaftor/ivacaftor combination therapy (Orkambi®).

Kalydeco® is prescribed through the NHS in the UK for around 410 eligible patients with indicated cystic fibrosis-causing genetic mutations. Orkambi®’s license indicates that over 3000 people with cystic fibrosis in the UK could receive the drug.

The UK Cystic Fibrosis Registry currently monitors the safety and efficacy of ivacaftor, compiling reports for the European Medicines Agency (EMA), as part of a scalable post-marketing surveillance programme. This enables a comparison of people on the drug with patients’ own legacy data, in addition to a comparator cohort matched from the entire CF population.

For more information: https://www.cysticfibrosis.org.uk/the-work-we-do/uk-cf-registry


Supporting the Health Technology Assessment (HTA) process

As well as assisting the pharmaceutical sector in conducting pharmacovigilance, patient registries can support national regulators, such as the National Institute for Health and Care Excellence (NICE) in conducting Health Technology Appraisals (HTAs) for current and upcoming treatments that may be recommended for use in the NHS. NICE base their recommendations on a review of clinical and economic evidence. As an example, genotype data from the CF Trust have been used to determine the number of patients that are likely to benefit from therapies such as ivacaftor and lumacaftor-ivacaftor. This data can contribute to the evidence base for determining how well the medicine or treatment works in relation to how much it costs the NHS. Registries can support the NHS by providing a clearer picture of the actual uptake of a particular treatment amongst the patient population.

4. WHERE DO CHARITIES ADD VALUE?

There are a number of areas where charities add specific value to data registries. Charities often fill gaps where other organisations are currently not funding data collections, meaning that, without charity involvement, there would be a lack of data for these conditions. This could create significant barriers for healthcare professionals, researchers and industry in conducting research in these areas, resulting in research progress being stifled and patients being deprived of essential treatments.

Patient-centredness

Charity-funded registries prioritise the patient community that they represent. Patients are key partners in the translational research process and charities recognise the importance of the active partnerships between patients, clinicians and researchers in driving improvements in healthcare and research. This can manifest in many ways, from patients being part of the steering group that oversees the direction of the registry, to using the registry to assess whether potential research outcomes are relevant to the target population.

Surveys have shown that patients are supportive of sharing their data for research. In 2017 as they were preparing to launch their BRIAN databank, The Brain Tumour Charity conducted a survey which revealed that 97% of patients are supportive of sharing their anonymised data to improve brain tumour care and treatment11. Furthermore, other studies, including one from Asthma UK, reveals that 88% of people with asthma would be willing for their confidential health data to be used for service improvement12.

Charities in the UK are uniquely close to their patient communities, and the voice of the patient is central in setting research agendas. Registries can further enable patient involvement and a recent report from the US-based organisation FasterCures highlights the value that a patient-centred approach can bring to encouraging patient involvement13.

Engaging with a wide range of expertise

When charities are setting the direction of their registry, and more generally their research, they generally engage with a broad range of stakeholders. This includes the public, patients, carers, researchers, specialist clinicians, clinicians outside their area of speciality, as well as policymakers and other professions. Therefore, charity registries are guided by the broadest range of expertise, views and input. Fundamentally, charities ensure that the registry is guided by patients.

In setting priorities for research, charities often undertake a priority setting partnership (PSP). This is a process that involves bringing together a range of people including clinicians, academicians and patients. For example, with the support of the James Lind Alliance, Diabetes UK embarked on a PSP in 2017 to set the top 10 research priorities for people with type II diabetes. This process brought together patients and healthcare professionals, with more than 8000 patients responding to the initial survey14. In this way, research priorities are then established as a partnership between patients and specialists.

12 https://www.asthma.org.uk/datareport
13 https://www.fastercures.org/assets/Uploads/PDF/Patient-Registries.pdf

15 Saving lives with patient data registries

16 Saving lives with patient data registries
**CASE STUDY: MS Society – UK MS Register**

The UK MS Register is the world’s first registry to combine information from people living with a condition with their clinical and NHS data. Launched in partnership between the MS Society and the University of Swansea in 2011, the Register is building an accurate picture of life with multiple sclerosis in the UK that will revolutionise understanding of the condition.

It is now recognised that Patient-Reported Outcome Measures (PROMs) are key to delivering excellent patient-centred care, because they assess the quality of care delivered to people from their own perspective. The Register offers healthcare providers a unique opportunity to understand the impact of social policies and medical interventions on the lives of people living with MS.

As of 2018, over 18,000 people with MS had joined the Register, including over 3,000 with linked clinical records provided by over 45 clinical partners across the UK. Participants have contributed nearly 400,000 surveys, allowing the Register to provide comprehensive anonymised data sets to clinical and social science researchers, and build our understanding of MS. These include enabling new studies into the relationship between factors including quality of life, symptoms, cost effectiveness, depression and employment. They are now investigating the potential of using the UK MS Register as a platform to collect and link data from various sources and to support the delivery of clinical trials in the UK. Through the UK MS Register, the MS Society is able to build the evidence base for fair and relevant social policies and improved healthcare, as well as assess the true impact of medical interventions.

For more information: [ukmsregister.org](http://ukmsregister.org/)

**Focus on research**

Medical research charities are committed to investing in research to improve patient lives. Since the sector started collecting data in 2008, medical research charities have invested nearly £13 billion in research in the UK. Charities that support data collections are committed to maximising the potential of their registry for patients. While various national bodies, such as NHS Digital, may collect many useful clinical audits, some of these are focused on operational service improvement and their specific aim is not on progressing research to develop new treatments. Charity registries are often set up to prioritise research. For example, the Brain Tumour Charity’s BRIAN project aims to decrease the barriers to research, by lowering the time and costs for researchers to access data.

**CASE STUDY: The Brian Tumour Charity**

The Brain Tumour Charity are developing BRIAN ([brain tumour](http://brain) Information Analysis Network), a databank for those affected by a brain tumour to learn from each other’s experiences and about different parts of the journey.

Up until now, there has never been an easy way of learning on a large scale how and what people living with a brain tumour have been through. BRIAN will securely and anonymously store data about treatments, tumour types, experiences, side-effects, decisions, and more, giving researchers important insights into the disease, to help reach a cure more quickly.

By bringing together national clinical datasets with Patient-Reported Outcome Measures (PROMs), BRIAN will provide a uniquely rich dataset for use by brain tumour researchers to accelerate the discovery of new treatments and better understand existing treatments. This will ensure patients are treated more precisely.

Researchers will be able to make a single application to access a source of brain tumour data, meaning research projects will cost less time and money. Additionally, easier access should increase the number of projects and, in turn, the number of researchers working in the field of brain tumours.

For more information: [https://www.thebraintumourcharity.org/understanding-brain-tumours/getting-a-diagnosis/brian/](https://www.thebraintumourcharity.org/understanding-brain-tumours/getting-a-diagnosis/brian/)

**Providing an evidence base for policy decisions**

Registries support charities in building the evidence base for fair health policies and improved healthcare. This allows charities to campaign more effectively for their communities, for instance.

**CASE STUDY: Parkinson’s UK – UK Parkinson’s audit of care services**

Every two years, Parkinson’s UK works with more than 450 care services to conduct an audit of the quality of Parkinson’s care across the UK. This involves care professionals looking at the medical records of people with Parkinson’s to document what type of care they provide to each patient.

They are then able to identify whether people with Parkinson’s are receiving care to the standard laid out in the NICE and Scottish Intercollegiate Guidelines Network (SIGN) guidelines and spot any strengths or weaknesses of services. For example, they can calculate what percentage of people with Parkinson’s in the last year were properly informed about the side effects of their medication or had a discussion about advanced care planning.

This audit is the largest collection of data about the quality of care for people with Parkinson’s in the UK. Using the audit results Parkinson’s UK professionals and service providers work together to develop a service improvement plan to improve care for people with Parkinson’s. They then re-audit every two years to demonstrate improvements in services.

For more information: [https://www.parkinsons.org.uk/professionals/uk-parkinsons-audit-transforming-care](https://www.parkinsons.org.uk/professionals/uk-parkinsons-audit-transforming-care)
Trust and transparency
Charities represent patients and are trusted organisations that work with, and for, their patient communities. Charities strive to meet and exceed expectations of accountability and transparency set for the charity sector. Additionally, being trusted partners, charities can significantly contribute to the dialogue with patients and the public about the ways in which data are used for research. One example of a charity-supported registry that has developed a robust and transparent framework for industry collaboration is the IBD Registry.

As well as involving patients in the co-design of research, a recent public survey undertaken by the Health Research Authority (HRA) suggests that research funded by medical research charities enjoys similar public trust to studies undertaken by the NHS. The proportion of individuals who are very comfortable with charity-funded research is significantly higher compared to industry-funded research15.
Charities are also committed to making research results open and accessible. For instance, AMRC has recently established an open access research platform with F1000. Currently, 24 AMRC members are using this platform to accelerate the publication of research – all articles on the platform benefit from immediate publication, transparent refereeing and the inclusion of all source data16.

CASE STUDY: IBD Registry – Industry Working Group

The Inflammatory Bowel Disease (IBD) Registry was set up to find out more about the long-term impact of the condition and collect better information on treatments and outcomes. By gathering data electronically, the Registry provides information that IBD teams can use locally to support patient care, and feed into a national database for benchmarking, quality improvement and research.

The Registry’s Industry Working Group was set up in 2016, bringing together a broad set of skills to find innovative solutions to some of the Registry’s challenges. The small working group provides an opportunity for industry expertise to complement the clinical skills of the Registry team, and acts as an efficient communication channel to link with and represent all the IBD companies.

The group has developed a robust and transparent framework for industry collaboration, funding and support for the Registry. Moreover, the Industry Working Group has shown that industry has much more to offer than sponsorship and has harnessed their strategic skills to help solve challenging Registry problems together. The industry partners are getting as much out of the partnership as the IBD Registry team, learning from each other and gaining a better appreciation of the pressures and challenges within the NHS.

Data linkage to support research into multiple long-term conditions

Multimorbidity is usually defined as the presence of two or more long-term conditions in the same individual. There is an increasing acknowledgement of the need to consider the effect that multiple conditions have on patients, and there is an evidence gap in the data that exists for patients with multimorbidities. This data void can be addressed, in part, by linking a patient’s data from registries to the data held about the patient in national datasets and those held in other patient registers. This will help to build a clearer picture of this patient and allow greater insight into their multimorbidity.

As well as linking data to other disease registries, there is high value in linking data to other data sources such as socio-economic, environmental, and social care data. The registry is an essential data source but through data linkage, the value of the registry can be amplified.

Charities are enterprising, and they are already considering the interoperability of registry data with other data collection mechanisms such as electronic health records (EHRs) and remote, connected devices. For instance, Asthma UK are looking at the development of Smart inhalers and innovative data systems that could help to ensure that those most at risk of an asthma attack are identified. This could include linking various tools used in managing asthma to ensure that GPs are informed when people have an emergency admission17.

In addition, studies using registry data have revealed risk factors for certain conditions. For example, Versus Arthritis produce an annual report, State of Musculoskeletal Health18, using data sources including the UK’s National Joint Registry. These studies demonstrate that obesity is a risk factor for the development of osteoarthritis and increases the need for joint replacement. This helps inform how preventative measures can be implemented as well as helping to identify patients at higher risk.
Charity registries add significant value to both patients and the UK’s life sciences sector by contributing to the diversity and strength of the UK’s data sources. There are significant opportunities to build upon this platform for the benefit of patients as government seeks to deliver the vision outlined in their Industrial Strategy.

To build on the success of registries, there must be a supportive environment that is driven by government and relevant healthcare policymakers; including NHS England, NHS Digital and Public Health England. The richer and more detailed the data collected into registries, the greater the resources available to clinicians, researchers, industry, service providers (such as the NHS and charities) and policymakers to develop new treatments and care pathways that benefit patients.

5. HOW THE UK CAN BUILD ON THE POTENTIAL OF CHARITY-SUPPORTED REGISTRIES

Charity registries add significant value to both patients and the UK’s life sciences sector by contributing to the diversity and strength of the UK’s data sources. There are significant opportunities to build upon this platform for the benefit of patients as government seeks to deliver the vision outlined in their Industrial Strategy.

Industrial Strategy Grand Challenges

Data and Artificial Intelligence (AI) are one of the government’s four Industrial Strategy Grand Challenges, with a specific focus on using emerging technologies to prevent, diagnose and treat long-term conditions. New and existing registries, as well as medical research charity expertise in the collection and use of patient data, will be essential to achieve this ambition.

Charities are entrepreneurial and pioneering in embracing new technologies, and many already have partnerships with many of the academic organisations leading these technologies. For instance, the Alan Turing Institute has recently used an extract of CF Registry data to train machine learning tools which may be able to learn and make personalised forecasts of the course of the disease for each individual patient. The Versus Arthritis funded Centre for Epidemiology based at the University of Manchester has a major focus on technology-based research. This includes developing a remote monitoring smartphone app that collects research quality data from rheumatoid arthritis patients in-between clinical appointments and integrates the data into the electronic health record, thus supporting self-management, clinical care and research.

Driving innovation to ensure the financial sustainability of the NHS

Improving uptake of innovation in the NHS will be essential to ensure its future sustainability. The NHS long-term plan and Accelerated Access Collaborative will address this issue, and it is essential that data registries are considered as a mechanism to support improving the uptake of innovation.

Registries can also assist in leveraging industry involvement in developing and testing new therapies, potentially leading to faster uptake of innovation across the NHS. This should be reflected in the life sciences ‘sector deals’ process.

As the cost of bringing new drugs to market increases, we must harness all the tools that can drive efficiencies, including data registries. This will ensure that the UK provides the best environment for researchers and industry to develop new treatments and cures for patients. In an environment where the NHS is looking to cut costs and optimise processes, registry data can support improvements in care and support planning, and accelerate the development of medicines, ultimately delivering operational savings to the healthcare system.

Future-proofing the UK’s patient data landscape

Digital Innovation Hubs will be established across parts of the NHS by Health Data Research UK (HDR UK) in support of the Life Sciences Industrial Strategy, with hubs intended to provide researchers with access to healthcare data in real time. As the NHS modernises and electronic care records become mainstream across the system, it will be important to ensure that registries and other data sources are compatible with new systems.

A number of AMRC member charities are starting to engage with HDR UK about future partnership projects, including British Heart Foundation, who are looking at the possibility of developing a new UK cardiovascular data centre. Charities should be considered as important partners in the development of ground-breaking new data initiatives, bringing expertise and knowledge of their patient community.

As the healthcare system faces a rise in the number of patients with multiple long-term conditions, the ability for researchers to link datasets will be fundamental in building the fullest possible picture of patients. Building such a picture will be vital for the future of healthcare and clinical research. Data linkage therefore must be seen as integral to the future of data registries.

As well as supporting current charity registries, we need to ensure there is a fertile environment for the development of new data collections and for the linkage of data between registries and national datasets held by NHS Digital, Public Health England, CPRD and others. Part of HDR UK’s programme of work involves improving the interoperability of different datasets; it is important that charity registries and data collections are supported in aligning their processes with new developments across the NHS and healthcare system.

The amount of health data available continues to grow exponentially, posing a challenge for organisations in how to manage, analyse and make use of this ever-increasing resource. As data takes on an increasingly valuable role in healthcare and research, there is a requirement to determine effective and fair models of data sharing – ensuring that there is equitable sharing of the benefits. As guardians of data and patient representatives, charities are well positioned to engage with the public and patients to ensure that there is a real and genuine partnership when data is used by collaborators. To answer some of the ethical questions posed by digital and data partnerships, the AMRC is working with our members to develop an ethical framework that addresses these issues.

The future of healthcare will be powered by data. Therefore, it is vital that the potential of charity-supported registries in the UK’s life sciences sector is harnessed to ensure maximum benefit for UK health and wealth.

20 RIF in comment if required
21 https://www.turing.ac.uk/research/research-projects/improving-cystic-fibrosis-healthcare
5. BACKGROUND

What is a registry?

In simple terms, patient registries are standardised collections of information about patients, which are usually disease specific. The information collected into registries may include a patient’s medical history, disease diagnosis, NHS number, and Patient-Reported Outcome Measures (PROMs). Collecting this data can help to produce a real-world picture of disease, current treatment practices, and outcomes.

As well as disease registries, charities also use data from procedure-based registries. For instance, the British Orthopaedic Association are in the process of establishing several emerging procedure-based registries. Alongside patient registries, these are useful datasets for conducting medical research.

How are data collected about patients?

The data collected into registries often flows from two primary sources.

1) Clinical data is often reported at the point of care delivery by members of the patient’s healthcare team (i.e. doctors, nurses) while they receive a service through the NHS. This may include name and date of birth, as well as the patient’s personal health records including their diagnosis and treatment plan.

2) Data may also be collected directly from the patient. PROMs data cover information about the patient’s care and treatment via self-reporting.

Charity-funded registries increasingly prioritise patient self-reporting. Discrepancies exist between patient and clinician estimates of both the prevalence and severity of patients’ symptoms as well as functional impairments, highlighting the importance of PROMs data in proving a greater insight into the individual’s condition. For instance, Multiple Sclerosis (MS) Register, funded by MS Society, have recruited over 18,000 consenting patients to share their data as part of the MS Registry. This includes asking patients to self-report on their treatment at six-month intervals.

About the AMRC

In 2017, AMRC’s members:

• invested over £1.6 billion of research funding in the UK – almost half of the total public investment and more than either the Medical Research Council or National Institute for Health Research;
• funded the salaries of over 17,000 researchers in the UK; and
• recruited 200,000 patients into clinical trials funded by charities.

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