Health Data Strategy

LifeArc

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Message from our CEO and CMO

“The LifeArc Health Data Strategy will deliver our ambition to realise the translational potential of health data for people with rare disease. Secure access to high-quality data at scale will allow us to drive early diagnosis and new treatment options, which are desperately needed.

This strategy will build on our excellent in-house data science expertise and strong partnerships to support data-driven innovation that transforms the lives of people living with rare disease.”

“Trusted data makes successful trials possible. This strategy shows how LifeArc will work as a dependable partner across the ecosystem to enable access to secure, high-quality data to identify patients, refine endpoints and ensure execution of fast, feasible studies in rare disease.

Together we will make sure evidence for adoption of new, effective therapies reaches clinicians and patients sooner.”

Sam Barrell,
Chief Executive Officer

Jonathan Morgan,
Chief Medical Officer

Executive summary

Advances in health data science present a unique opportunity to deliver real change for those living with rare disease – an estimated 300 million people worldwide[1] – most of whom currently have little hope for effective treatment.

There is momentum to harness the transformative potential of data for patients, including unprecedented commitment from the government to the UK's health data research infrastructure. But with this comes the risk that the unique needs of people living with rare disease are overlooked.

We are pleased to share the LifeArc health data strategy: a targeted vision that couples our unique translational expertise with the rapidly evolving health data landscape to deliver measurable impact and transform lives.

It builds on our foundational investment in Our Future Health, which we are both a founding charity member and strategic user of. The strategy is also a direct response to the LifeArc and Genetic Alliance UK *Accelerating R&D for rare disease in the UK* report, which outlines recommendations to harmonise and streamline rare disease research.

Our strategy sets out 4 goals that will unlock access to high-quality, multimodal data that is currently siloed, to drive impact for adults and children with rare disease:

1

faster, accurate diagnosis – by identifying early signals of rare disease in health data

2

better treatment and care – by working with national initiatives to accelerate patients' access to clinical trials

3

improved access – by enhancing the connection of health data across specialist paediatric services

4

influence the ecosystem – by ensuring research we support achieves maximum sustainable impact

Through our health data work, we will ensure people with rare disease reap the benefits of health data innovation.

[1] The landscape for rare diseases in 2024, The Lancet Global Health, Volume 12, Issue 3, e341

Introduction

At LifeArc, our mission is to make life science life changing for people with rare disease and drug-resistant infections.

Rare diseases are collectively common, with over 7,000 identified affecting around 300 million people worldwide[2].

Despite their diversity, rare diseases share common problems

- **Delayed diagnosis:** it typically takes 5 to 7 years to be diagnosed with a rare disease[4]. Without a formal diagnosis, patients may miss the opportunity to access appropriate care and support or to participate in clinical trials to develop therapies
- **Limited treatment options:** around 95% of rare diseases have no licensed therapy[5]
- **Access and adoption barriers:** in many EU countries, less than half of orphan drugs are reimbursed[5]. The UK Health Technology Appraisal (HTA) can significantly undervalue treatments[3] that may have long-term benefits for rare diseases

These problems are exacerbated by 3 connected factors

- **Rarity:** small patient populations make trials slow, expensive and failure prone[7]. Due to misaligned evidence requirements, therapies that progress to regulatory approval over-rely on qualitative evidence, which often does not demonstrate HTA cost-effectiveness[3]
- **Returns:** there are weak or non-existent commercial incentives for industry[3]
- **Remit:** systemic health data needs for shared standards, access and operation are often beyond the reach of individual patient organisations and charities

[2] The landscape for rare diseases in 2024, The Lancet Global Health, Volume 12, Issue 3, e341

[3] LifeArc & Genetic Alliance UK. Accelerating R&D for Rare Disease in the UK: An Opportunity to Change Millions of Lives. (2025)

[4] Mendelian. A Preliminary Assessment of the Potential Impact of Rare Diseases on the NHS. (Imperial College Health Partners, 2018).

[5] Center for Drug Evaluation and Research and Rare Disease Cures Accelerator. FDA (2024)

[6] Zamora B et al. Comparing access to orphan medicinal products in Europe. Orphanet J of Rare Dis. 14, 95 (2019)

[7] The UK Rare Diseases Framework. (DHSC, 2021)

In 2025 LifeArc and Genetic Alliance UK published a report, *Accelerating R&D for rare disease in the UK*[3]. This report is a call to action, outlining how we can smooth the rare disease research and development pathway in the UK, ultimately to speed up diagnoses and get treatments to patients faster. Diagnostics and data are highlighted as both a critical market failure and huge opportunity, with the potential to revolutionise rare disease treatments.

Channelling momentum for rare disease

The Health Data Strategy is an important way that LifeArc is responding to the data recommendations in that report.

There is growing potential for the use of high quality, longitudinal, multimodal health data in care and research. In rare disease especially, there is strength in numbers: each record within an intrinsically small population carries significant value.

And there is unprecedented government investment and political will to ensure NHS data saves lives, including a £600m planned investment into a health data research service[8].

However, a focus on the greatest population level impact means infrastructure, analytical tools and data strategies are often designed to serve common conditions, where the greatest returns are expected. Less attention is given to rare diseases. As a result, national infrastructures that support the identification and management of rare diseases are underdeveloped and underutilised, further reinforcing the perception of limited impact in this field. This strategy will elevate rare diseases from an afterthought to an exemplar for use of health data that drives innovation at scale, now and in the future.

The LifeArc health data strategy: delivering patient impact for people with rare disease

At LifeArc, we have a clear and important role in shifting the trajectory for people with rare diseases.

Our patient-first charitable mandate and financial independence mean we can prioritise health areas with unmet need that might otherwise be overlooked. Meanwhile, our in-house capabilities in data science and translation and our convening power enable us to address complex, system-wide challenges. We have already made a major investment in Our Future Health, as both a founding member and strategic user, and continue to evolve our data science expertise.

Our health data strategy builds on these strong foundations.

In it, we propose 4 interconnected goals that act upon recommendations from the *Accelerating R&D for rare disease in the UK* report. Together, they will reduce friction across the rare disease translation pathway – ensuring the UK's health data environment becomes more inclusive, more usable and, ultimately, more capable of supporting rare disease innovation.

Goal	How we'll do it
Faster diagnosis Turning multimodal data into actionable insights	Identify early signals of rare disease in health data
Better treatment and care Delivering therapies faster with smarter trials	Support trial feasibility and recruitment with fast, scaled data-driven capabilities
Improved access Supercharging the power of children and young people's data	Deliver insights from health data across specialist paediatric services
Influence the ecosystem Maximising the benefits of existing strategic LifeArc investments	Enhance LifeArc-supported research with data science expertise

[8] GOV.UK. www.gov.uk/government/news/prime-minister-turbocharges-medical-research (2025)

Faster diagnosis: turning multimodal data into actionable insights

People with rare diseases are diagnosed late and inconsistently. A major challenge is that early signs of the condition are hard to spot and often sit across imaging, genomics and routine health record data.

Rare dementias – including frontotemporal dementia, progressive supranuclear palsy and corticobasal degeneration – collectively account for up to 1 in 5 cases of dementia under age 65[9]. Yet they are frequently mistaken for more common conditions, such as Alzheimer's disease or Parkinson's disease. Patients face delays in treatment, inappropriate care and missed opportunities to take part in clinical trials[10].

This is compounded by weak research infrastructure: recruitment to dementia clinical trials in the UK significantly lags behind other major health conditions[11]. Without early identification and stratification, patients miss out on emerging therapies, and researchers lack the evidence needed to evaluate efficacy and safety.

[9] Dementia UK. www.dementiauk.org/information-and-support/young-onset-dementia/young-onset-dementia-facts-and-figures/ (Accessed 2025)

[10] Rare Dementia Support. www.raredementiasupport.org/what-is-rare-dementia/ (Accessed 2025)

[11] Alzheimer's Research UK. www.alzheimersresearchuk.org/news/recruitment-to-dementia-trials-lags-behind-other-major-health-conditions/ (Accessed 2022)

We will build upon our existing partnership with Our Future Health and harness broader government-backed infrastructure to harness artificial intelligence (AI) to identify rare dementias

Comprehensive UK data assets and developments in AI have huge potential to find, validate and translate early signals of disease in real care settings. We will pilot and test AI to find these signals, which are currently hidden in siloed data – working across previously unconnected data assets to make faster and more accurate diagnoses of disease. Our focus will initially be on rare dementias before expanding to additional priority areas across LifeArc's Translational Challenges.

How we'll do it

- Influence the development of the government-backed Health Data Research Service to cater for rare disease by collaborating as a 'super user' with the NHS health data ecosystem – including Regional Secure Data Environments (SDEs), Genomics England and UK Biobank
- Deliver translational research projects that aim to increase diagnosis and stratification – initially for people with rare dementia before expanding to other priority disease areas
- If successful, pilot embedding validated signals for rare dementia into real-world use, such as electronic health record prompts and referral rules
- Assemble regulatory-grade evidence packages aligned to AI as a medical device (AIaMD) standards
- Demonstrate that rapid, federated access to multimodal data can be achieved and meet the needs of rare disease research

"We very much welcome the LifeArc Health Data Strategy, which will build upon their successful collaboration with Our Future Health. For people with underserved conditions like neurodegenerative disorders, LifeArc's translational science expertise is an invaluable asset to deliver data-driven innovations in diagnosis and prevention."

Dr Raghib Ali,
CEO,
Our Future Health

Better treatments and care: delivering therapies with smarter trials

Recruitment to rare disease clinical trials is held back by fragmented data, siloed trial sites, and underused national datasets[12].

NHS DigiTrials is a service that helps researchers run and recruit for clinical trials more efficiently by using routinely collected NHS data. For example, it is currently helping Our Future Health recruit 5 million volunteers from diverse backgrounds to create the UK's largest health research programme.

Despite the critical importance of this globally unique service, it has limited capacity to deliver multiple studies and currently lacks access to specialist datasets needed to support rare disease research.

[12] Zhang, J et al. Mapping and evaluating national data flows: transparency, privacy, and guiding infrastructural transformation. Lancet Digit. Health 5, e737–e748 (2023).

We will unlock faster, more reliable and equitable recruitment into rare disease trials.

Through collaborative working with NHS national data assets, we will shape service capabilities to support rare disease research that we fund, and across the wider life sciences landscape.

How we'll do it

- Develop partnership working with national NHS data assets and services to support UK-based clinical trials within our rare disease pipeline
- Collaborate with Our Future Health to help shape and maximise its emerging capability for clinical trials and interventional studies
- Starting with 2 to 3 rare disease use cases, test how national infrastructure reduces set-up time and enables more reliable, equitable recruitment
- Support the integration of the National Disease Registration Service data into the NHS England Secure Data Environment and NHS DigiTrials, enabling precise and seamless rare disease patient finding and enrolment
- Facilitate interoperability and collaboration between NHS DigiTrials and the LifeArc Centre for Accelerating Rare Disease Trials (ARDT) to streamline rare disease recruitment

> "The LifeArc Health Data Strategy brings a welcome focus to the opportunities for using data held by the NHS to accelerate rare disease research. We look forward to working closely with the team to turn insights into impact for patients, powered by NHS data.
>
> **Dr Michael Chapman,**
> **Director of Research and Clinical Trials,**
> **NHS England**

Improved access: supercharging the power of children and young people's data

Speciality children's data remains fragmented and absent from national-level programmes, often leaving paediatric care without the evidence needed to access innovations.

Children account for 75% of all rare disease patients, with one third of rare disease deaths occurring before the age of 5[13]. This is why intervening as early in childhood as we can offers the most promising window to transform lives.

However, there are critical gaps in national datasets, research pipelines and translational opportunities for childhood rare diseases. Structural barriers include consent and ethics complexities, which are compounded by limited commercial incentives for small cohorts. Further, there is currently no national paediatric Secure Data Environment (SDE). This leaves existing efforts confined to individual NHS hospitals, which limits the ability to generate scalable insights, recruit effectively and validate innovations.

How we'll do it

- Leverage strong clinical and research networks that already exist for paediatric disease, as well as our own established partnerships and existing data science capabilities
- As part of a collaborative, generate insights across current organisational boundaries to seamlessly deliver robust evidence based on high quality data

We aim to unlock the as yet unrealised value of specialist paediatric data – so that it can be used to rapidly evaluate the impact of changes in care, including supporting validation and deployment of AI as a medical device (AIaMD) and other technologies.

> "Harnessing paediatric specialist data is pivotal for rare disease research. We welcome LifeArc's interest in building on existing momentum and partnerships with their wealth of expertise and experience. Collaboration is key to transforming fragmented insights into actionable knowledge that accelerates innovation and improves outcomes for children with rare disease."
>
> **Professor Neil Sebire,**
> **Chief Research Information Officer,**
> **Great Ormond Street Hospital**

[13] National Institute for Health and care research. www.nihr.ac.uk/news/uk-rare-disease-research-landscape-mapped-first-time (2025)

Influence the ecosystem: maximising the benefits of existing LifeArc investments

Our translational network investments deliver the greatest patient benefit when they operate as a cohesive system

At LifeArc, we use our translational expertise to help people living with rare conditions and address some of the major infection-related threats facing human health. To support this aim, we have made bold investments in translational centres and networks, including those with a focus on rare disease[14].

We will employ our in-house data science expertise to help harness the full potential of projects we support.

Our internal data science team will drive and enhance collaboration across our portfolio to align standards, close gaps in evidence and reduce the time it takes for discoveries to deliver patient benefit.

How we'll do it

- Act as an advisor, analytics partner and system integrator to support our networks' work in data science
- Apply our in-house data science expertise to find opportunities to securely link datasets, and leverage existing infrastructure and standards

> "The LifeArc Centre for Accelerating Rare Disease Trials (ARDT) aims to integrate health data into a unified, trusted ecosystem. Data driven approaches are likely to be one of the major advances in the next few years, speeding up trials and insight generation. We really welcome this Health Data Strategy, which will put LifeArc and the ARDT at the forefront of this revolution."
>
> **Professor David Jones,**
> **Lead PI, LifeArc Centre for Accelerating Rare Disease Trials**

[14] www.lifearc.org/project/lifearc-translational-centres-for-rare-disease/

A targeted vision to deliver impact

In 2024, we decided to strategically invest in health data as an exciting new opportunity to deliver patient impact, including joining Our Future Health as a funding charity partner and growing our in-house data science capabilities.

Our health data strategy builds on this foundational investment. Through it, we will deliver targeted activities that couple our translational expertise with the evolving health data research landscape. We will build and nurture strong partnerships that unite fragmented systems, break down barriers and advance scientific discoveries.

Through delivery of this strategy, we will realise the translational potential of health data to transform the lives of people living with rare disease.


LifeArc