2024 at LifeArc

Making life science life changing

LifeArc

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Forewords

Note from Chair of the Board

2024 was a pivotal year for LifeArc, marked by significant strides towards tackling underserved conditions. We enhanced our role in bridging the gap between scientific innovation and patients, advancing discoveries that have the potential to transform lives.

We have always focused on tackling complex and overlooked health challenges, bringing together expertise and resources to move science forward.

We also invest in the infrastructure and networks that underpin progress. In rare diseases, we committed £40 million to establish four Translational Centres for Rare Disease. Similarly, we co-founded the UK's first network of Cystic Fibrosis Lung Health Innovation Hubs. And through our experienced Ventures team, we continued to invest in life changing science.

The gene therapy innovation centres we are sponsoring in London, Bristol and Sheffield provide vital facilities for researchers working on gene therapies. We are increasingly focusing on this critical area, opening new avenues for discovery and development to ensure that patients with rare diseases are not left behind.

Partnerships remain central to our work. We're participating in groundbreaking programmes such as Our Future Health and the Fleming Initiative, as well as collaborating with the UK Dementia Research Institute to advance new innovations for dementia. These partnerships highlight how we seek to address health challenges, in the UK and around the world, in collaboration with diverse stakeholders including research institutes, policymakers and industry.

Our people have been at the heart of this progress. Dr Sam Barrell joined us as CEO in the autumn, bringing energy, vision and strong leadership which put us in a powerful position going forward. We have also been joined by our first Chief Medical Officer, Dr Jonathan Morgan, who will play a crucial role in shaping our clinical research agenda. I'm delighted that we have appointed two additional scientific chairs who bring invaluable expertise and have expanded our global networks, accelerating progress for patients and ensuring LifeArc remains at the forefront of translational science.

I am incredibly proud of what we have achieved. Looking ahead, we're excited to build on this momentum and pursue bold, long-term initiatives.

With a sharper focus, strong partnerships and an exceptional team, we're positioned to make significant progress in 2025. Together, we're harnessing innovation to make a lasting difference to the lives of people with underserved conditions.

Ian Gilham, Chair of the LifeArc Board of Trustees

Note from Chief Executive Officer

I had the privilege of joining LifeArc in October 2024. The year was drawing to a close, but it was immediately clear to me that 2024 was a year brimming with exciting new beginnings for LifeArc.

We hosted our first ever Translational Science Summit, co-started an international drug discovery consortium, and funded multiple new clinical trials.

We launched a series of innovative translational centres and hubs, conducted our first Motor Neuron Disease (MND) Insights Group workshops and community surveys, and refreshed our antibody discovery capabilities.

We started building our data science function, joined Our Future Health – a world-leading health dataset driven by volunteers – and the Fleming Initiative – a global, multidisciplinary collaboration to tackle antimicrobial resistance. We also made great strides in our plans for a flagship building in London's King's Cross Knowledge Quarter, where we will consolidate our Stevenage laboratories and our London offices.

Underneath this breadth of activities, there lie strong themes. Chief among them is the theme of rare and underserved conditions. We focus on areas where limited commercial incentives have hindered progress. LifeArc's not-for-profit status leaves us perfectly positioned to step in and accelerate advancements. People suffering from these conditions shouldn't miss out on innovation because of complexity, cost or risk.

I'm delighted to have joined such an active and growing organisation and have great hope for what LifeArc can achieve in the future. Our foremost goal is to create positive impact for people with rare and underserved conditions and to tackle global health challenges such as resistant infections. I am eagerly anticipating 2025 and beyond, where I believe we can truly make life science life changing.

Sam Barrell,
CEO of LifeArc

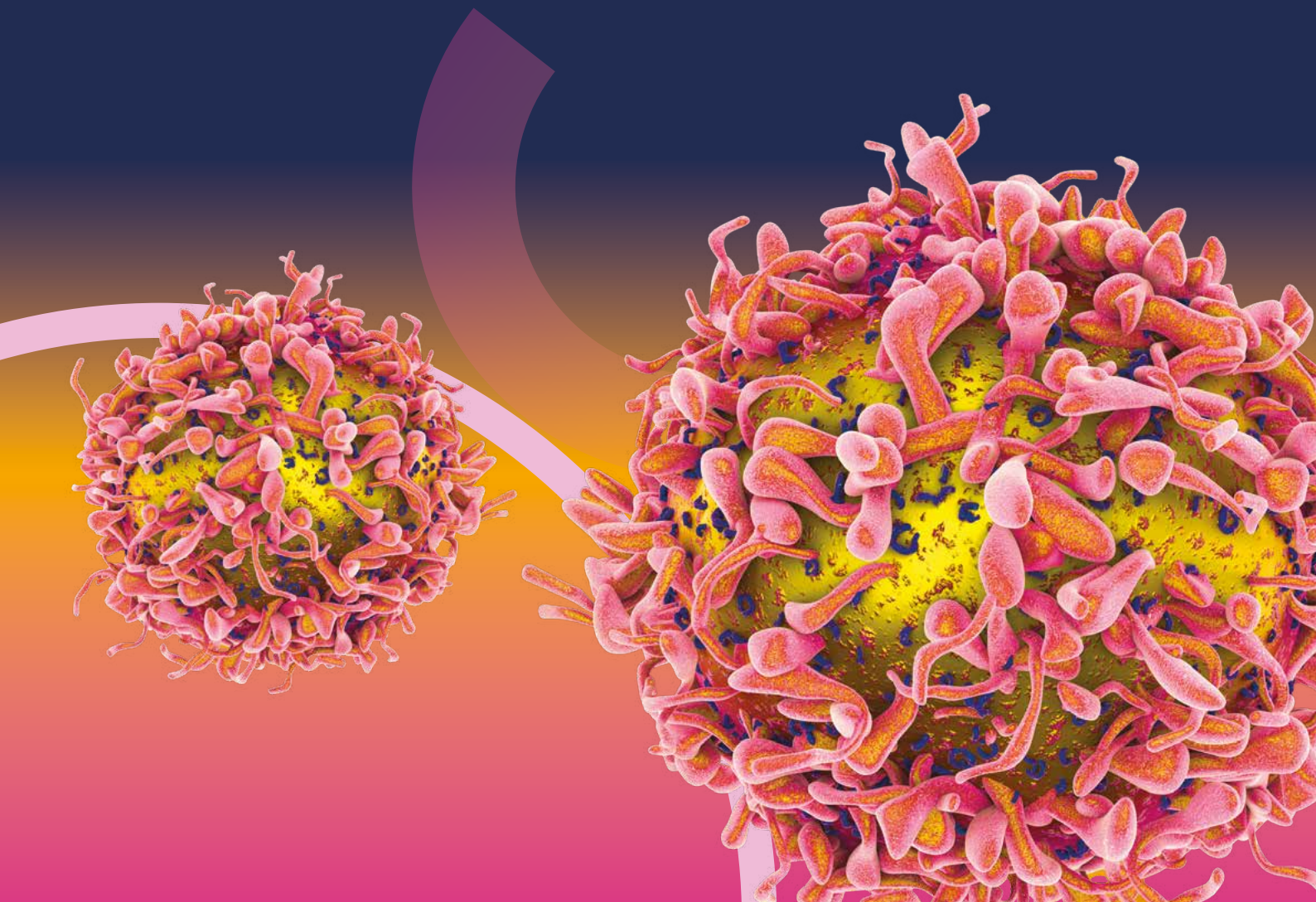
Who we are

We are experts in translating promising science. We look for ways to strengthen the pipeline stretching from early discoveries to clinical trials, to get promising science to people with unmet health needs, faster.

Sometimes that's lending scientific support or commercialisation advice. Sometimes it's providing funding or investment. Sometimes it's gathering experts and patients in one room to tackle a challenge. Frequently, it's a combination of those.

Collaboration is critical to making real changes. This year, we worked with hundreds of people and organisations, including 27 new partners, to address the barriers that are keeping innovations from leaving the lab and getting to the people who need them.

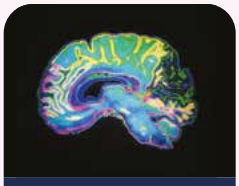
Only together can we find new interventions that can aid in earlier detection, getting the right diagnosis and better treatments.



We work with patients to understand what they most need, whether that's a transformative new drug, diagnostic, device or digital solution.

We do this for some of the most underserved challenges facing human health through our ambitious Translational Challenge research programmes and science projects. See the diagram below for where we put our focus.

Rare Conditions



Rare Neuro-degeneration

Rare Respiratory

Childhood Cancer

Other Rare Disease

Global Health

Global Health Infection

with a focus on...

MND and rare dementias

Chronic Respiratory Infection

We supported over

160 projects

this year across these focus areas

The highlights of our year

We enhanced and added several **new scientific capabilities,** including targeted protein degradation, data science and induced pluripotent stem cells.

Read more starting on page 38

The **DefINe Trial** we supported with the University of Cambridge started trialling a repurposed treatment for an ultra-rare disease called neuroferritinopathy. Media coverage from BBC News helped increase the known size of the global population living with this disease by 10%.

Dr Sam Barrell joined us in October as our new Chief Executive Officer.

We launched our clinically validated **antibody discovery platform, B-SMArT.**

Read more on page 40

4 LifeArc Translational Centres for Rare Disease were established across the country to unlock new tests, treatments and potential cures for people with rare conditions.

Read more on page 30

The Sheffield **Innovation Hub for Gene Therapies** opened its doors, with Princess Anne in attendance.

With Cancer Research Horizons, we set up the international **C-Further Consortium** to create more effective, targeted medicines for children and young people with cancer.

Read more on page 25

We joined the largest health research programme in the UK, **Our Future Health.**

Read more on page 47

Our **patient advisory group for MND** began sharing insights that will be used to inform our future work in MND.

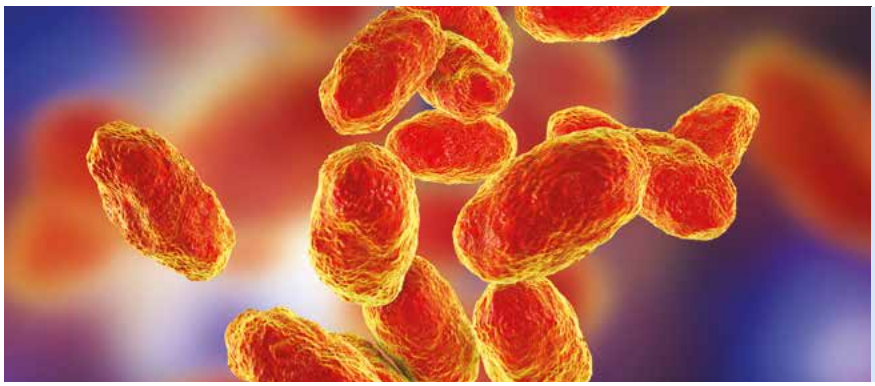
Read more on page 12

We supported Great Ormond Street Hospital (GOSH) and GOSH Children's Charity to **explore taking on the license to manufacture and administer a gene therapy** that can treat an ultra-rare condition that leaves babies without a functioning immune system.

Read more on page 32

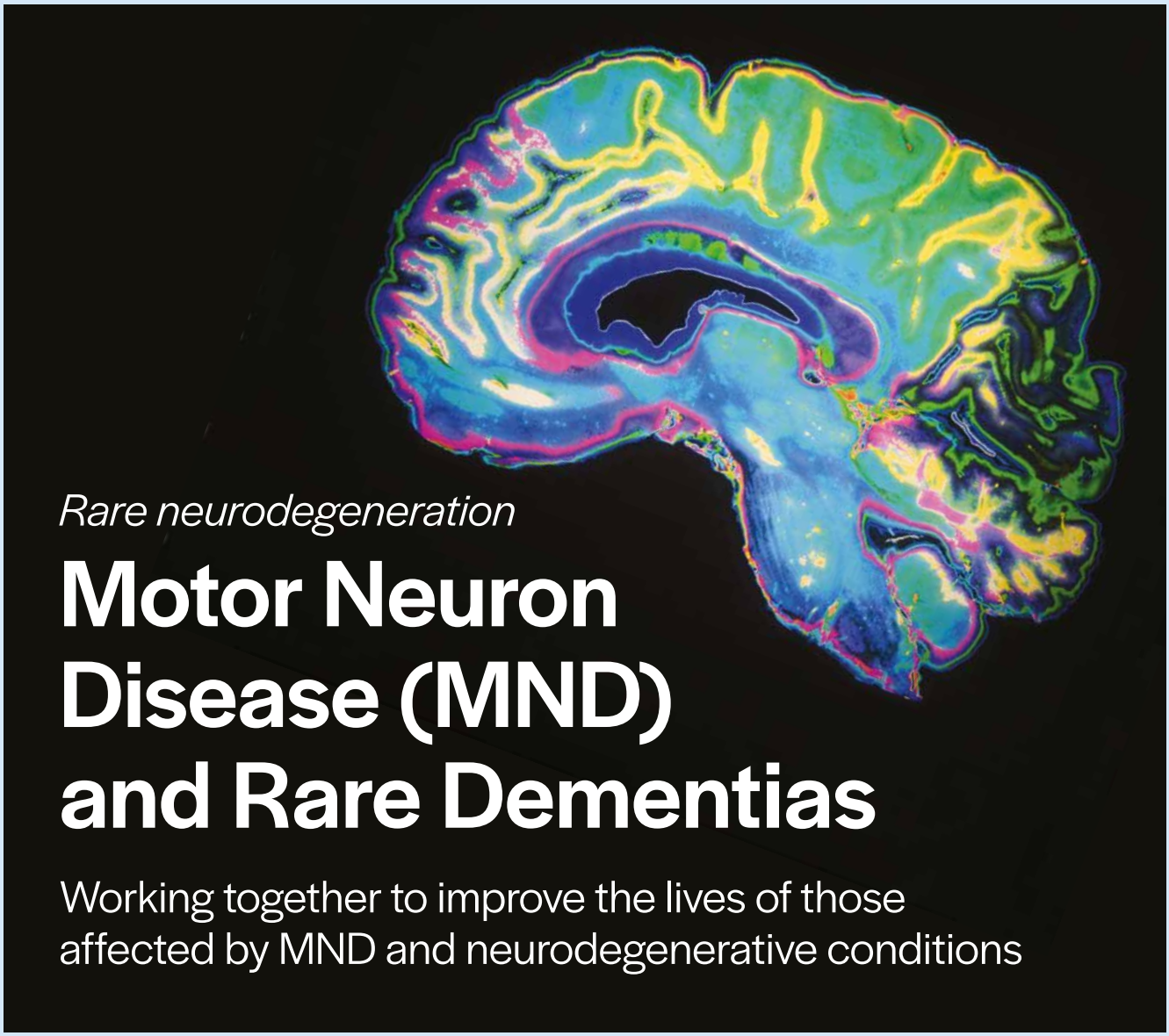
With Cystic Fibrosis Trust, we launched **4 Hubs for Lung Health and Infection in Cystic Fibrosis** to transform how lung infections are diagnosed, treated and managed.

Read more on page 18



We became a **founding partner of the Fleming Initiative** to help tackle the global threat of antimicrobial resistance.

Read more on page 37



Rare neurodegeneration

Motor Neuron Disease (MND) and Rare Dementias

Working together to improve the lives of those affected by MND and neurodegenerative conditions

MND is a progressive condition that affects the motor neuron cells in the brain and spinal cord that help tell muscles what to do. This causes them to gradually weaken, affecting a person's ability to walk, talk, eat, drink and breathe.

Despite advances, we still know far too little about how to predict the onset or progression of MND, and effective treatments remain limited. This means the quality of life for those affected and their families is severely impacted and the prognosis for patients is poor.

Our vision is a world where MND is both preventable and treatable. By partnering with experts, applying our expertise in translational science, and working with people living with MND, we hope to find life changing treatments and ultimately cures.

Neurodegeneration

Beyond our work in MND, we also focus on rare dementia and other neurodegenerative diseases, which are becoming more common as the population ages. Despite the growing need, effective treatment options remain limited.

To tackle this unmet need, we have a 5-year, £30 million partnership with the UK Dementia Research Institute to advance research into new diagnostic tests, treatments and devices for people with dementia.

Read more on page 49

What do we aim to achieve?

- Improve patient quality of life
- Contribute to making medicines and diagnostics
- Improve prediction and earlier diagnosis of MND
- Connect experts across neurodegeneration research
- Unblock barriers that keep scientific discoveries from reaching patients

MND affects up to

5,000

people at any one time in the UK

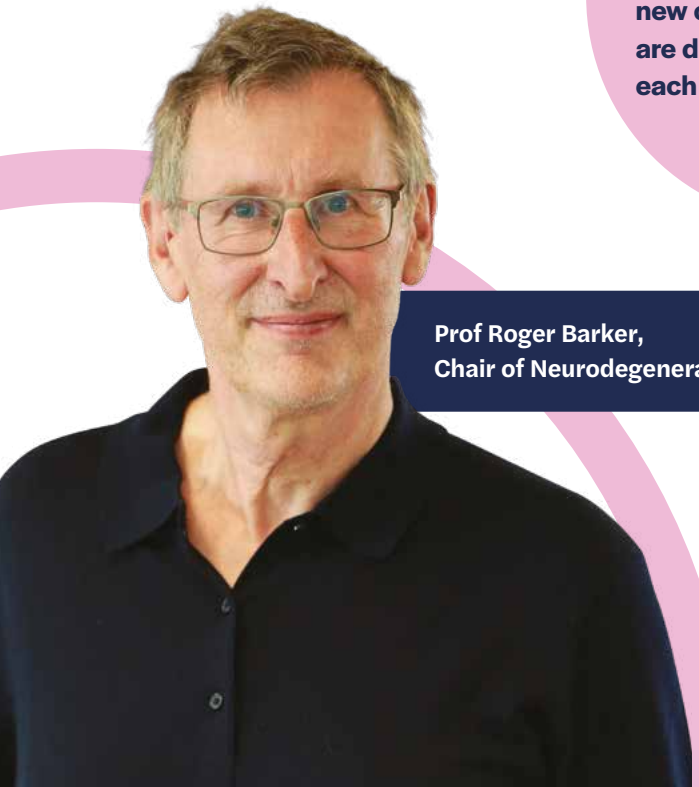
Over

1 million

people in the UK live with neurodegenerative conditions

140,000

new cases of MND are diagnosed worldwide each year



Prof Roger Barker, Chair of Neurodegeneration

Paul Wright, Head of Motor Neuron Disease & Rare Dementia

At our MND biomarker roundtable, experts explored the unmet needs and opportunities in the MND biomarker field to guide future MND research

Connecting the MND ecosystem

MND and neurodegenerative diseases are highly complex and not yet fully understood. We are working hard to build a network in the UK and beyond to collaborate across academia, industry, patient groups, and research organisations. By pooling expertise, funding, and resources, we can overcome challenges and address the most urgent needs.

In 2022, we partnered with the UK Dementia Research Institute (UK DRI) in a five-year, £30 million collaboration which combines our expertise in translational science with the UK DRI's discovery research to bring innovative solutions to patients faster. We have 7 major programmes already underway.

In 2024, we funded 5 early-stage projects with up to £300,000 each and supported 8 early-career researchers to generate critical preliminary data and advance their careers. Read more on page 49.

Last year, we also joined forces with the ALS Therapy Development Institute (ALS TDI) to identify, validate, and develop **new biomarkers that are crucial for ALS/MND prognosis and drug development.** By working together, we aim to better understand the disease, improve how it's monitored, and ultimately deliver better care for people living with ALS/MND.

Working with people with lived experience of conditions like MND is vital to ensure research focuses on what really matters. That's why we set up the **MND Insights Group** – to listen to their experiences and use their insights to guide research that drives real progress in prevention and treatment. Read more on page 12.

We hosted a MND biomarker roundtable which brought together experts to discuss the **unmet needs and opportunities in the MND biomarker field.** Biomarkers have revolutionised personalised medicine for many conditions and hold significant potential for MND. We published a report summarising key insights from the discussion, providing guidance and support for future MND biomarker research **(read the report here)**.

Bridging the gap between lab and clinic

A lack of funding and translational science expertise often means that many promising discoveries never progress beyond the lab. We bridge this critical gap to help turn research into real-world treatments, diagnostics or devices that benefit patients.

Over the last year, we have supported several promising projects. With King's College London, we launched the MND STaR (Solution for Motor Neuron Disease Target Validation and Research) programme which aims to de-risk and **validate a portfolio of human genetic-driven MND therapeutic targets.** Through this collaboration, we hope to pave the way for new, disease-modifying MND treatments.

We also partnered with Eisai, Health Data Research UK (HDR UK), and the University of Edinburgh to launch NEURii, a two-year, **£4.8 million research collaboration to fast-track scalable digital solutions for dementia care.**

The pilot phase includes 3 projects leveraging Scottish NHS data to develop non-invasive digital biomarkers. The first 2 projects are now underway and include SCAN-DAN, a project using AI and brain imaging to detect dementia early by analysing CT and MRI scans, and NeurEYE, a project developing an AI-powered eye scan tool to identify early signs of dementia, offering a non-invasive and scalable diagnostic approach.

Recognising the potential for drug repurposing, we launched a **£5 million MND drug repurposing call** for innovative projects focused on preclinical translation of repurposed drugs.

By supporting researchers in repurposing already approved drugs, we aim to bring new treatments to patients more quickly and cost-effectively.

As a founding partner of Our Future Health, we now have access to the **world's largest healthcare dataset.** We are using our data science expertise to explore ways to improve diagnosis of Alzheimer's disease and other dementias. Read more on page 47.

Our science projects in MND and neurodegeneration

BioHermes-002

Identifying blood and digital tests that could help early diagnosis of Alzheimer's disease.

Read more on page 49

Using iPSCs

Improving motor neuron disease modelling with induced pluripotent stem cells.

Read more on page 48

In the spotlight

MND Insights Group: Listening to those living with MND

We want to uncover the most important research questions and urgently address the needs of people living with MND and their families. But we can't do this alone, we need to hear from those who live with the condition.

We know that for medical research to be most effective, it is crucial that the people we are trying to benefit are involved throughout. That's why we created our MND Insights Group – a group of people with lived experience of MND – to better understand the most pressing challenges faced by those directly affected by the condition. We're also conducting community surveys to capture perspectives from the wider MND community, ensuring that a broad range of voices shape our approach.

In the first half of 2024, we held 2 workshops with the MND Insights Group and conducted our first survey to explore treatment burden, preferred drug administration routes, perspectives on side effects, and the most meaningful treatment outcomes for people with MND and caregivers.

The workshops and survey highlighted that there was a strong preference for non-invasive, home-based treatments that enhance quality of life with manageable side effects. Although oral medications are often assumed to be the most convenient route of administration, they can be problematic for people with MND – swallowing difficulties often require crushing tablets, a process that is both time-consuming and challenging for carers.

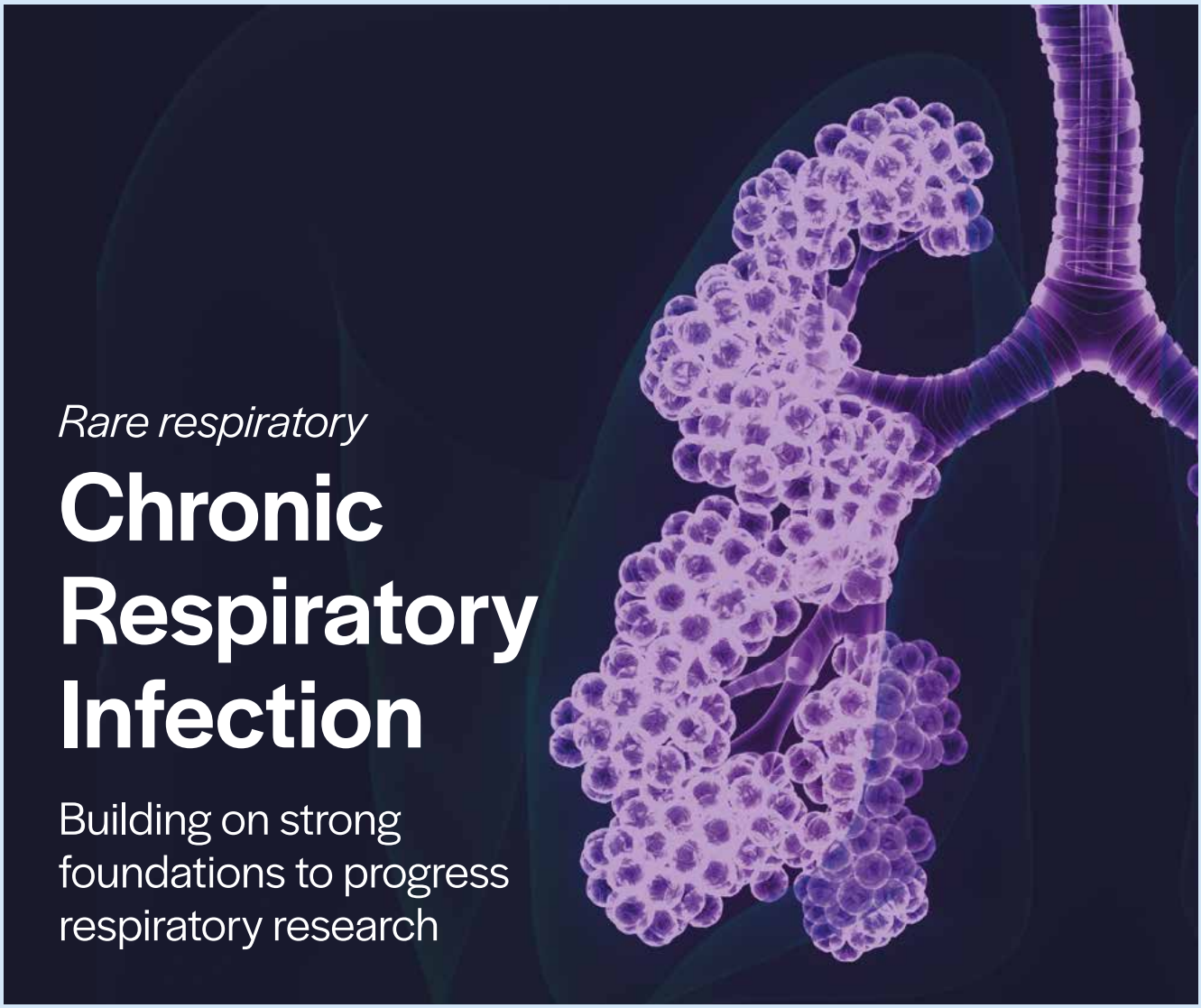
Insights revealed that receiving medication as a monthly or bi-weekly injection at a hospital may be preferred to administering a large number of oral medications at home. While caregivers prioritised dignity and well-being, those with MND or at high genetic risk were more willing to endure short-term burdens to slow disease progression. Our findings have been shared in the MND Insights Report.

Later in the year, we held a third workshop and second community survey to improve our understanding about the daily challenges of living with MND. Insights from these will inform investment into our future research aimed at creating technologies to improve the lives of those affected by MND.

In November 2024, we held a workshop with the MND Insights Group to review applications for our MND drug repurposing call. Their insights were shared with the scientific review panel to inform decision-making and ensure that we support projects with the highest potential for impact.

Moving forward, we will continue to engage with the MND community. The important insights from people with lived experience will be instrumental in guiding the work of our Translational Challenge, shaping our priorities and ensuring we are addressing the most pressing MND needs. Only through this sustained partnership can we hope to create a world where MND is truly preventable and treatable.

Read the MND Insights report >



Rare respiratory

Chronic Respiratory Infection

Building on strong foundations to progress respiratory research

People living with bronchiectasis and cystic fibrosis are caught in a vicious cycle of infection, inflammation and permanent lung damage – and running low on treatment options due to antibiotic resistance or a lack of effective therapies.

Since 2021, our Chronic Respiratory Infection Translational Challenge has been laying the groundwork to progress discoveries that can intervene earlier, reduce treatment burden and improve the quality of life for those living with these conditions. This year, we've focused on delivering work based on those foundations.

Whether through impactful collaborations or supporting promising science, we're committed to creating a legacy that can evolve and hopefully improve lives for decades to come.

Katy Kettleborough,
Head of Chronic Respiratory Infection

Fostering collaborations and partnerships

Collaboration is critical in respiratory health, where knowledge, data and resources can be fragmented and hard to access.

Since we began our work in this area, we've focused on bringing together the voices and expertise of this ecosystem, which includes those with lived experience, charities, researchers, clinicians and industry. Together, we hope to overcome those barriers and move promising innovations along the pipeline toward patients.

Our largest contribution to connecting the respiratory infection ecosystem this year was establishing an innovation **network of 4 hubs dedicated to lung health** across the UK with Cystic Fibrosis Trust. Read more in the spotlight on page 18.

Our other major collaboration with the Cystic Fibrosis Trust and Medicines Discovery Catapult – the Cystic Fibrosis Syndicate in Antimicrobial Resistance **(CF AMR Syndicate)** – continued supporting the development and progression of new and effective antimicrobials for cystic fibrosis (CF), winning a 'Most Impactful Industry Collaboration' Oxford Biotech Network award for its efforts.

25 projects
this year

2 funding calls
completed

10 projects
funded

Bronchiectasis impacts more than 200,000 people in the UK. For some, the cause may be a rare genetic disease. We are looking at ways to identify these patients, to enable them to seek more appropriate treatment options.

In 2024, the CF AMR Syndicate began funding 3 exciting projects to deliver antimicrobial drugs for people with CF and agreed on plans for 3 more.

Prior to the European Cystic Fibrosis Conference, we held an industry symposium with Cystic Fibrosis Trust which showcased the support available to researchers or companies developing new CF interventions.

We spent a significant proportion of the year **increasing awareness of bronchiectasis** and its research potential to future industry partners, including hosting a sponsored breakfast for 60+ delegates at the World Bronchiectasis Conference.

Our active membership in EMBARC, a European research network for bronchiectasis, saw the launch of a clinical trial platform to investigate repurposed drugs for bronchiectasis, a collaboration involving LifeArc scientists to develop a molecular diagnostic test, and the start of data science collaborations to improve diagnosis and management of bronchiectasis.

People living with these lung conditions play a vital role in shaping our work, helping to prioritise the most needed and most useful tools, treatments and tests.

With support from Asthma + Lung UK, we convened **a focus group of over 160 people living with bronchiectasis.** Their lived experience will provide insights to shape our future activities and funding decisions, and we will facilitate access to this group for others.

We established a network of

4 hubs

dedicated to lung health across the UK with Cystic Fibrosis Trust

Additionally, in partnership with Asthma + Lung UK, we hosted a **diagnostics workshop in 2023** to better understand the immediate needs of patients. Building on the insights from this workshop, we have launched a project to develop a series of **target product profiles** (TPPs) to guide future innovations in diagnostics. Read more about our work with TPPs on page 20.

As we look ahead, we will continue building these strong relationships to pave the way for a better future for those living with chronic and rare respiratory diseases.

Insights from our one-day diagnostics workshop in November 2023 with Asthma + Lung UK brought together over 100 researchers, clinicians, SMEs and industry partners and set the scene for several of our exciting 2024 projects.

Supporting great science

We support science that finds new ways to detect and diagnose diseases earlier, ensures people receive the right diagnosis and improves treatment options.

Early detection of infection is an important step in breaking the cycle of inflammation and lung damage.

The **BronchX clinical trial** began testing a digital tool and app to detect and manage exacerbations in people with bronchiectasis. The trial is part of our longer-term support of Royal Papworth Hospital's Project Breathe, in parallel with the ACE-CF trial that is testing the same tool in people with cystic fibrosis.

Our diagnostics team is also collaborating with the University of Dundee and EMBARC to **develop a bacterial molecular load test** for one of the most persistent infections in people with bronchiectasis, *Pseudomonas aeruginosa*. Getting the right diagnosis, and earlier, remains an important factor in reducing long-term side effects and improving patient care.

This year, we published the **Transforming Diagnostics report,** based on our joint workshop with Asthma + Lung UK. It outlines 4 priorities to better understand unmet needs and barriers to developing improved respiratory health diagnostics.

The second round of our

£10 million

funding call opened for drug repurposing projects for bronchiectasis or cystic fibrosis

We also developed and published **target product profiles** for devices that could aid in early detection and diagnosis. Read more in the spotlight on page 20.

Alongside detection and diagnosis, we continue to support the development of new treatments, including repurposed drugs, that tackle infection, inflammation and could reduce side effects and the overall burden of treatments required for those with respiratory conditions.

We supported the launch of a clinical trial platform, EMBARC-**AIRNET**, to investigate repurposed drugs for bronchiectasis.

The **second round of our £10 million funding call** opened, in search of further exciting drug repurposing projects that could help people with bronchiectasis or cystic fibrosis. Meanwhile, **6 innovative early drug discovery projects** were announced in 2024, funded via a joint £3 million call via the CF AMR Syndicate.

A therapeutic for CF

A new drug to overcome antibiotic resistance in cystic fibrosis

Read more on page 50

Diagnostics tests for BE and CF

New diagnostic tests to detect lung infections in bronchiectasis and cystic fibrosis

Read more on page 51

Data science for BE and CF

Analysing healthcare data using machine learning to improve and understand the impact on patient lives

Read more on pages 45 and 46

In the spotlight

Translational Innovation Hub Network

for lung health & infection

Funded by

LifeArc

Cystic Fibrosis Trust

Convening the respiratory health ecosystem

In partnership with Cystic Fibrosis Trust, we have launched a translational innovation hub network across the UK. The network of 4 Hubs will combine knowledge, expertise and resources to accelerate the development of new diagnostics and treatments for lung infections.

Over

200,000

people worldwide are affected by cystic fibrosis

Over

11,000

people in the UK are affected by cystic fibrosis

Cystic fibrosis (CF) is a genetic condition that results in the build-up of thick, sticky mucus in the lungs and other organs. This can lead to persistent lung infections, inflammation, and exacerbations – sudden flare-ups that worsen symptoms. These episodes can require hospitalisation and may contribute to long-term lung damage.

Despite recent advances, there is still no cure for CF. Many people with CF must manage their health through a daily routine of physiotherapy and oral, nebulised, and sometimes intravenous antibiotics. However, the bacteria responsible for CF lung infections can develop resistance to these antimicrobial treatments, limiting treatment options.

We urgently need to develop new ways to detect, diagnose, treat and manage lung infections and lung health to improve the lives of people with CF. But to accelerate research in this space, we must find ways to collaborate better and combine knowledge and resources.

This is why, together with Cystic Fibrosis Trust, we've invested £15 million into the Translational Innovation Hub Network for Lung Health and Infection, which launched in 2024. The network consists of 4 Hubs in Cambridge, Liverpool, Manchester and London, bringing together teams of globally recognised scientists and clinicians to combine their research and expertise and dramatically speed up progress.

The first dedicated network of its kind, the Hubs foster strong partnerships across the ecosystem, integrating lived experience to translate research into practical solutions, advance potential new treatments and tests, and expand scientific knowledge. They also serve as an access point for industry collaboration and provide a specialised training platform for emerging respiratory health professionals.

Now that these Hubs are up and running, we hope to start delivering outputs that can transform CF lung health and help people live happier, longer lives.

Trailfinder CF
Innovation Hub

University of Liverpool
Lead PI: Jo Fothergill

Pulse CF
Innovation Hub

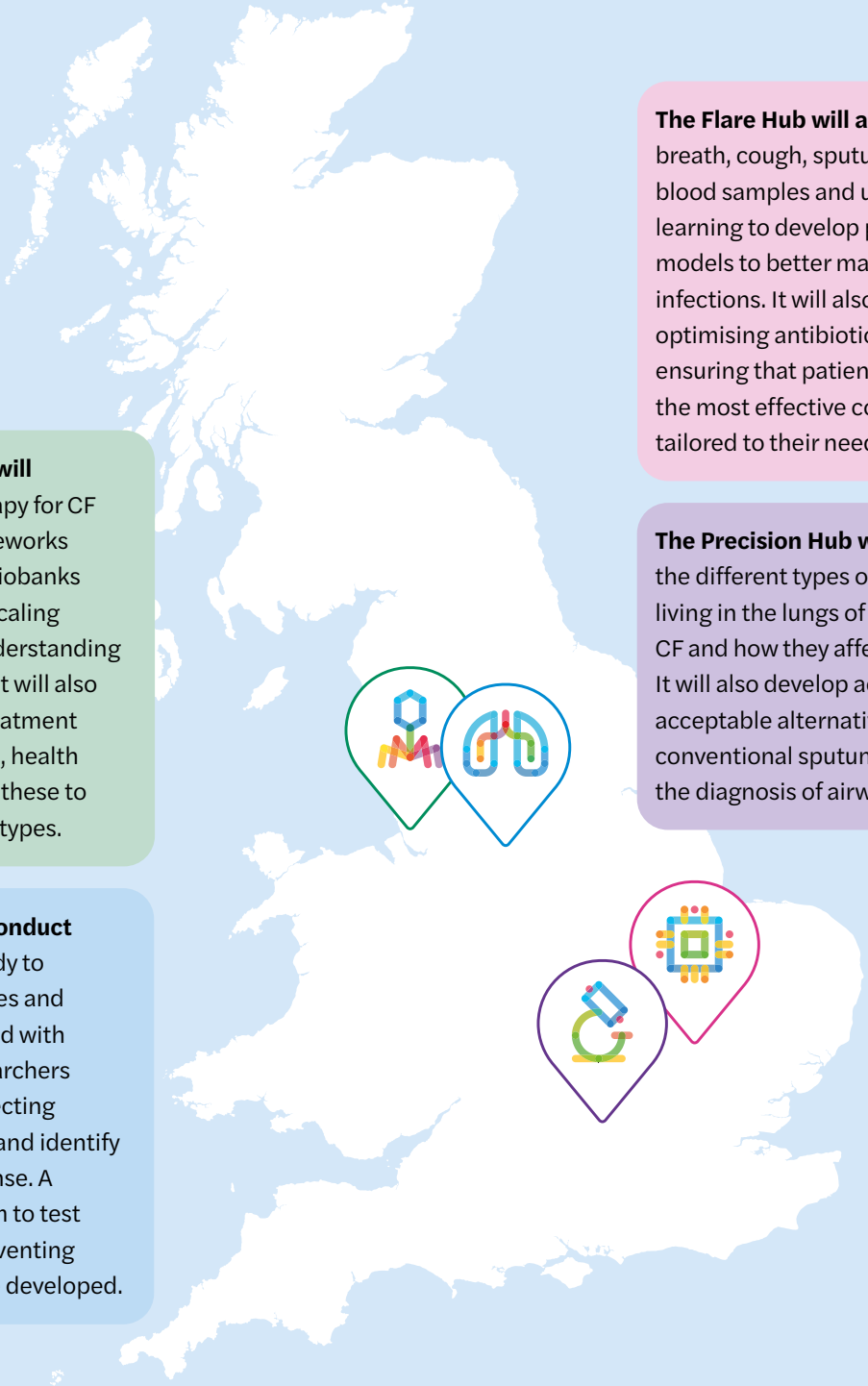
University of Manchester
Lead PI: Alex Horsley

Flare CF
Innovation Hub

University of Cambridge
Lead PI: Andres Floto

Precision CF
Innovation Hub

Imperial College London
Lead PI: Jane Davies



The Trailfinder Hub will advance phage therapy for CF by establishing frameworks for use, developing biobanks and methods for upscaling manufacture and understanding dosing and delivery. It will also improve infection treatment by analysing bacteria, health records, and relating these to different disease subtypes.

The Pulse Hub will conduct an observational study to understand the causes and risk factors associated with exacerbations. Researchers will study factors affecting response to therapy and identify biomarkers of response. A clinical trials platform to test interventions for preventing exacerbations will be developed.

The Flare Hub will analyse breath, cough, sputum and blood samples and use machine learning to develop predictive models to better manage lung infections. It will also work on optimising antibiotic treatments, ensuring that patients receive the most effective combinations tailored to their needs.

The Precision Hub will focus on the different types of microbes living in the lungs of people with CF and how they affect health. It will also develop accurate, acceptable alternatives to conventional sputum culture for the diagnosis of airway infections.

In the spotlight

Target product profiles (TPPs): A blueprint for developing new tools to help cystic fibrosis patients

To support the development of new cystic fibrosis (CF) tests and treatments and ensure that they meet the real-world needs of people with CF, the Cystic Fibrosis Antimicrobial Resistance (CF AMR) Syndicate – a partnership between us, Cystic Fibrosis Trust, and Medicines Discovery Catapult – has created targeted guidance documents for researchers.

The development of effective treatments and tests for CF poses significant challenges for the research community.

To manage flare-ups and control infections, treatments must tackle a wide range of bacterial, viral, and fungal infections, but no single treatment works for all. While research is advancing, there are still no clear guidelines for testing new treatments in the lab or designing clinical trials. Traditional measures like bacterial load and lung function may not fully capture patient experiences, and there is limited data on alternative markers like symptom reporting and quality of life.

Diagnostics also face challenges. Standard culture techniques are slow, taking days or even weeks, and there is no clear definition of pulmonary exacerbations, making it difficult to determine when to treat. Additionally, the availability of sputum, essential for many tests, is decreasing due to the widespread use of highly effective modulator therapy (HEMT).

Find the target product profile for cystic fibrosis diagnostics >

To address these challenges, the CF AMR Syndicate has developed patient-focused Target Product Profiles (TPPs) to guide the creation of effective new treatments and tests for CF. These TPPs serve as strategic frameworks outlining the desired characteristics of new solutions, ensuring they align with the specific needs and priorities of people with CF.

In early 2022, the CF AMR Syndicate published the first therapeutic TPP guidance document for treating lung infections in CF. The development of this began with a focus group of people with CF and their relatives to identify unmet needs, followed by targeted engagement with clinical and industry experts. An international survey then established consensus on key recommendations and areas needing further discussion.

In 2024, in collaboration with the NIHR Newcastle HealthTech Research Centre (HRC), we began developing diagnostic TPPs for CF lung infections. We mapped current diagnostic practices, assessed emerging tools, and engaged with over 150 stakeholders, followed by a consensus survey to pinpoint key diagnostic challenges.

Both TPPs hosted virtual symposiums, bringing together people with CF and industry experts to discuss critical needs in CF therapeutics and diagnostics. Insights from these discussions, alongside survey findings, helped refine our TPPs which are now both publicly available and we hope their guidance will help bring more effective treatments and tests closer to the patient.

TPPs serve as a clear 'wish list' for developers, prioritising features such as:

non-invasive
easy-to-collect sample types e.g. breath or saliva

fast and accurate
results

usability
at home or in clinical settings

See the latest on the development of a simple lung test target product profile >

Childhood Cancer

Launching bold plans to transform the future for children with cancer

Cancer remains a leading cause of death by disease in children around the world. To save lives, we need better tests to monitor more accurately and less invasively, and rapidly match each child to the best available treatment for their cancer. And these treatments need to be bespoke – matched to the unique biology of childhood cancers.

Currently, children are often treated with drugs developed for adult cancers which can result in long-term chronic health issues, including secondary cancers, heart defects, deafness and other morbidities that can increase the risk of dying far too young.

Above all, we must accelerate the pace at which these breakthroughs reach the clinic.

95%

of childhood cancer survivors experience serious health issues by age 45*

9.1x

increased chance of premature death in childhood cancer survivors**

400K

cases of childhood cancer diagnosed globally each year***

What are the greatest unmet needs in childhood cancer?

Read the findings from our consultation with the childhood cancer community:

References: *Bhatia et al. JAMA (2023) 330:12 **Fidler et al. BMJ (2016) 354:i4351 ***World Health Organization (2019)

Doing better for children with cancer

We're working to create a better future for children with cancer.

This year, we launched our bold new strategy, shaped by working closely with the childhood cancer community, including clinicians, researchers, charities, and children and families with lived experience. Their insights helped us identify barriers to progress and prioritise the areas where they saw that LifeArc could make the most significant impact.

Collaboration and partnerships are at the heart of our approach. Together, we hope to speed up the development of new innovations and catalyse sustainable change for children with cancer.

Our strategy has 3 themes:

1. Better treatments

Developing therapies that specifically target the unique biology of childhood cancers.

2. Better trials

Improving the way clinical trials are run to help make sure the most promising new treatments get to the children who need them more quickly.

3. Better decisions

Developing less invasive biomarkers to inform clinicians' decisions throughout treatment.

Read our full strategy >

Driving innovations in childhood cancer therapeutics

Childhood cancers are rare, which can make it more difficult to translate new discoveries into the clinic. Traditional commercial models struggle to support progress because small patient populations often result in low financial returns, leading to limited investment and slower progress in research and development.

Addressing the challenges of childhood cancer can't be done alone. We are collaborating with others in this field to deliver our strategy, supporting innovative projects with promising science and convening the childhood cancer ecosystem to combine resources, expertise and foster collaborations.

With Cancer Research Horizons, we have created C-Further, an international consortium of experts seeking to create more effective, targeted medicines for children and young people with cancer, bridging the gap between the lab and patients. Read more in the spotlight on the next page.

We are committing

£4.9 million

of in-kind contributions to PROTECT

We are also committing up to £4.9 million of in-kind contributions as part of a collaborative international effort to develop new cancer therapies specifically for children. The £20 million project, named PROTECT, is part of Cancer Grand Challenges, supported by Cancer Research UK and the National Cancer Institute (USA) alongside 9 of the world's leading cancer research centres.

In a partnership with CureSearch, we are proud to be supporting exciting research combining a genetically modified cold sore virus with a cancer vaccine for children with aggressive brain tumours, who urgently need targeted, less-toxic therapies. This approach offers new hope for effective treatment.

Our science projects in childhood cancer

Targeted protein degradation

Advancing novel childhood cancer treatments using our targeted protein degradation expertise.

Read more on page 53

In the spotlight

C-Further: Creating a new model to accelerate the development of innovative treatments for childhood cancers

Addressing cancers in children demands a fundamental change in how drugs are discovered, developed and brought to market. Through C-Further, we are combining expertise from around the world to challenge conventional approaches to developing therapies.

Treatment options for children with cancer are extremely limited and are often based on adult therapies. But the physiology, immune systems, and biological drivers of childhood cancers differ significantly from those in adults, making these treatments less targeted and more harmful. Concerningly, **95% of childhood cancer survivors** experience serious health issues by age 45, including chronic conditions, neurocognitive deficits, and secondary cancers.

The need for innovative, child-specific therapies without long-term health effects is urgent.

However, the pipeline for childhood cancer drugs remains sparse. From 2007 to 2022, only **2 drugs** were approved exclusively for children by the EMA, and **5 by the FDA,** compared to the **15 cancer drugs** approved for adults by the FDA in 2024 alone.

This is partly because the rarity of childhood cancers creates barriers. Small patient populations slow progress in understanding and developing effective therapies and limit the potential revenue for new drugs, making investment for development very challenging. Additionally, high costs associated with manufacturing, regulatory compliance, and maintaining drugs on the market further impede progress.

Other challenges include limited access to specialised expertise, difficulties translating new discoveries into the clinic, and the complexity of targeting key drivers of childhood cancers.

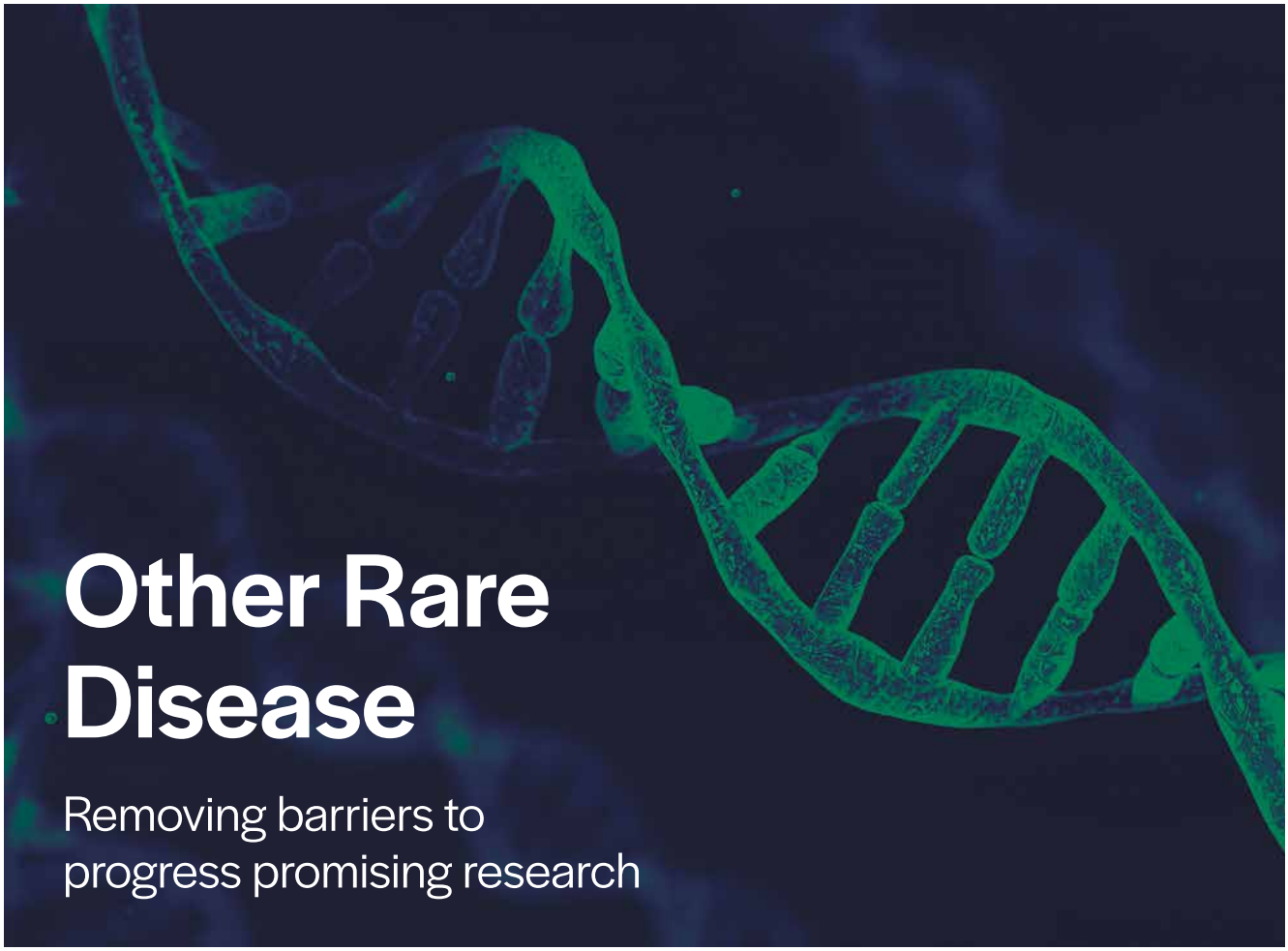
C–FURTHER
Children's Cancer Therapeutics Consortium

Advancing research efforts together

Improving outcomes for children with cancer requires global collaboration and investment to accelerate the development of new treatments and overcome the barriers to progress. We have joined forces with Cancer Research Horizons — the innovation engine of Cancer Research UK — to launch C-Further. This international consortium of researchers, clinicians, scientists, partners and investors hold a shared commitment to creating new cancer therapeutics for children and young people.

C-Further provides drug discovery lab support and investment to de-risk early-stage therapeutic projects and increase their chances of being picked up and moved closer to the clinic by industry and impact investors. Starting with an initial investment of £27 million, it is driving change by helping to remove barriers and accelerate promising ideas towards the children who need them. This collaborative approach also reduces the financial burden of developing treatments for small patient populations and minimises duplicative research efforts.

C-Further will continue to grow and evolve. We are calling on researchers, philanthropists, industry and other partners to join us with expertise, capabilities, ideas and funds. Our initial call for collaboration received expressions of interest from 12 countries, across 9 different drug modalities for 10 different childhood cancer indications. We hope that in time, together we can achieve our vision of a world where childhood cancers are treated effectively, with tailored, and well-tolerated therapies.



Other Rare Disease

Removing barriers to progress promising research

Rare diseases may be individually uncommon, but their collective impact is considerable. There is an urgent need for tests and treatments to improve and save the lives of the millions of people living with these conditions.

Progress in rare disease research is often slowed by fragmented expertise, small and dispersed patient populations, and limited funding. These barriers can also present commercial challenges that prevent new treatments from getting to people living with rare diseases.

We aim to bridge the gap between scientific discoveries and real-world benefits for people with rare diseases. By using our expertise and collaborating with others in the field, we work to overcome barriers to ensure tests and treatments get to people more quickly.

There are over

7,000

different types of rare diseases

Around

1/3

of patients take more than 5 years to be diagnosed

More than

95%

of rare diseases lack approved treatments

Projects supported in 2024

3

co-funded with DEBRA Austria

Supporting projects focused on identifying and developing existing drugs that can be repurposed to treat epidermolysis bullosa, a rare and debilitating genetic skin disorder.

5

co-funded with Action Medical Research

Working together to advance research to improve the lives of children with rare diseases.

12

Philanthropic Fund

Providing funding to academic researchers working to advance new treatments and diagnostics for rare diseases.

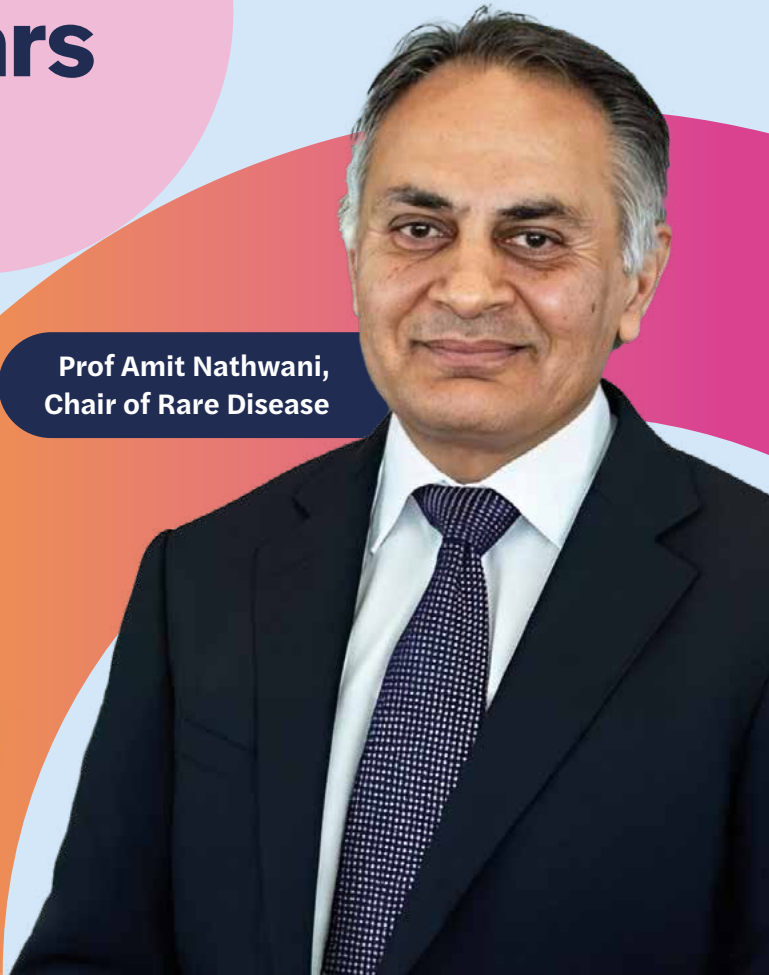
We've been funding rare disease research for more than

5 years

Committing more than

£30m

through funding schemes



Prof Amit Nathwani, Chair of Rare Disease

Connecting the ecosystem to bridge gaps

There is already great research happening in rare diseases. But the rare disease ecosystem in the UK is fragmented, with patients, researchers, clinicians, and industry often working separately instead of together. This makes it harder to progress research and overcome challenges.

We are working with partners to connect the rare disease community to improve knowledge sharing, avoid duplicate research efforts, speed up progress and ultimately bring hope for those living with rare conditions.

As part of this effort, we established a £40 million network of **4 Translational Centres for Rare Disease,** which launched in 2024. These centres bring together academia, industry, other charities and patient groups to tackle key challenges and accelerate the development of new tests, treatments, and potential cures for rare diseases. Read more on page 30.

In 2021, we also partnered with the Medical Research Council (MRC), to jointly offer £18 million (with support from the Biotechnology and Biological Sciences Research Council (BBSRC)) to create **3 Innovation Hubs for Gene Therapy** in Sheffield, London and Bristol.

4

Translational Centres for Rare Disease launched in 2024 (see page 30)

These hubs are advancing the development of new genetic treatments, with potential to transform care for millions of patients. In 2024, The University of Sheffield Gene Therapy Innovation Manufacturing Centre (GTIMC) officially opened. It aims to produce clinical-grade viral vectors for trials and offer training to upskill researchers.

Many proven gene therapies with the potential to cure a rare condition remain out of reach due to commercial barriers that prevent them from getting to and staying on the market. We supported Great Ormond Street Hospital (GOSH) and GOSH Children's Charity to secure the licence to manufacture and administer a gene therapy for an ultra-rare condition that leaves babies without a functioning immune system. Read more on page 32.

Supporting promising science

There are often promising new treatments for rare diseases in development, but they struggle to move beyond the early stages due to funding and commercialisation challenges. As a result, even therapies with strong potential often fail to reach the patients who need them most.

We aim to bridge this gap and support the translation of potentially life-changing treatments through our funding, scientific expertise, and commercialisation advice.

Since 2018, we have been supporting promising research through the LifeArc Philanthropic Fund, which provides grants to academics working on innovative treatments, devices, or diagnostics aimed at improving the lives of people with rare diseases. Our funding allows research projects to move further along a development pathway – by which point they may be more attractive to follow-on funders.

The fund has awarded £21.9 million to 43 projects since 2019, including 12 in 2024 alone. Among these, 2 have progressed to clinical trials, 4 have secured commercial partnerships, and 2 have resulted in spinout companies.

In 2024, our **Gene Therapy Innovation Fund** progressed exciting gene therapy research through the Innovation Hubs for Gene Therapies. This dedicated scheme was created to address the current UK gap in funding for academic-led gene therapy research. It allows early research to remain in academia and flourish to the point where it's more likely to attract follow-on investment.

Drug repurposing–finding new uses for existing medicines–can sometimes provide a faster, more cost-effective pathway to developing rare disease treatments by using already approved therapies. With DEBRA Austria, we provided **£2.5 million for repurposing projects** developing therapeutics for epidermolysis bullosa, a group of rare inherited disorders affecting the skin. We are also funding several drug repurposing projects through the Philanthropic Fund.

In the spotlight

LifeArc Translational Centres for Rare Disease

LifeArc Translational Centres for Rare Disease: A collaborative approach to rare disease research

These centres strengthen the rare disease ecosystem to fast-track the development of new treatments and tests to improve the lives of people with rare conditions.

Globally, over 300 million people live with rare diseases, yet more than 95% of these conditions have no approved treatments and getting a diagnosis can take years. This is because rare disease research is hindered by the very nature of the diseases themselves – they are rare, which means working with small patient populations.

As such, research and expertise are fragmented. Researchers can lack access to specialist facilities, as well as advice on regulation, trial designs, preclinical regulatory requirements, and translational project management.

Limited funding and difficulties in attracting downstream investment pose significant barriers, as small patient populations are associated with limited financial returns and early-stage research is inherently risky.

Plus, dispersed patient populations pose challenges such as difficulties in recruiting enough participants for robust clinical trials, delayed or inaccurate diagnoses due to limited knowledge and expertise, and the absence of comprehensive patient registries needed for effective research and treatment progress.

While the rare disease field in the UK has a solid foundation built on decades of scientific progress and engaged communities, a more integrated, and coordinated approach is crucial to overcome these persistent barriers. Research can happen more quickly when working with the right people at the right time, and with the right infrastructure in place.

This is why we have established a £40 million network of 4 Translational Centres for Rare Disease, which officially opened in 2024. These centres will serve as focal points for rare disease research translation, knowledge-sharing and engagement with the patient community in the UK. They will provide researchers with access to important resources and facilities and provide open access to guidance and training, which is particularly important for academics who don't have the necessary experience to take an innovation through to commercialisation.

Importantly, our centres focus on areas where there are significant unmet needs. They tackle barriers that ordinarily prevent new tests and treatments from reaching patients with rare diseases and speed up the delivery of rare disease clinical trials.

By bringing together leading scientists and rare disease clinical specialists from across the UK, they encourage new collaborations across different research disciplines so that we can work together more effectively to transform the lives of people living with rare diseases.

In association with

GENETIC ALLIANCE UK

beacon for rare diseases
no rare journey alone

LifeArc Centre

Rare Respiratory Diseases

University of Edinburgh
Lead PI: Prof Kev Dhaliwal

LifeArc Centre

Rare Mitochondrial Diseases

University of Cambridge
Lead PI: Prof Patrick Chinnery

LifeArc Centre

Rare Kidney Diseases

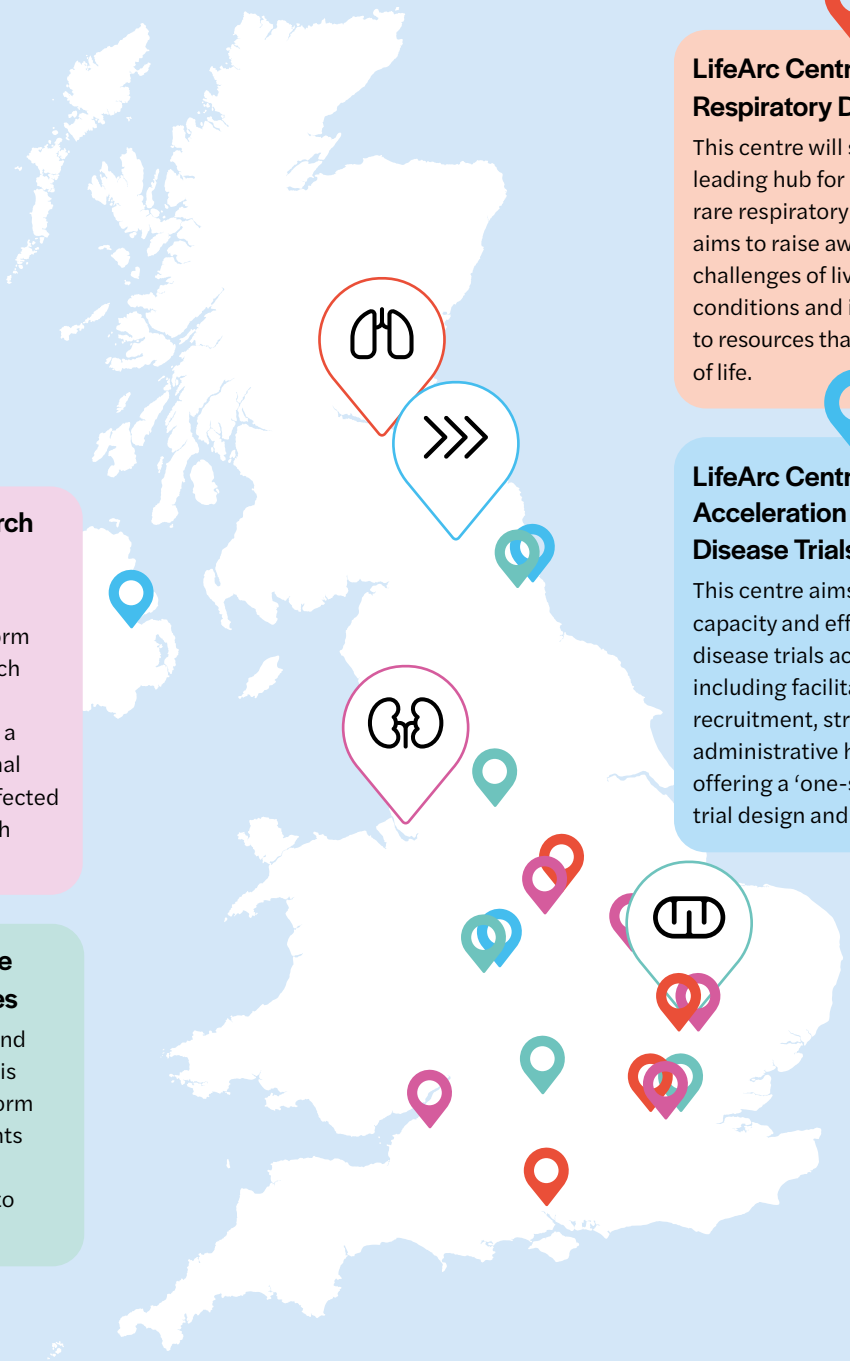
In partnership with Kidney Research UK

University of Liverpool
Lead PI: Dr Louise Oni

LifeArc Centre

Acceleration of Rare Disease Trials

Newcastle University
Lead PI: Prof David Jones



LifeArc Centre for Rare Respiratory Diseases

This centre will serve as the leading hub for researching rare respiratory diseases. It also aims to raise awareness of the challenges of living with these conditions and improve access to resources that enhance quality of life.

LifeArc Centre for Acceleration of Rare Disease Trials

This centre aims to boost the capacity and efficiency of rare disease trials across the UK – including facilitating equitable recruitment, streamlining administrative hurdles and offering a 'one-stop shop' for trial design and support.

LifeArc-Kidney Research UK Centre for Rare Kidney Diseases

This centre aims to transform rare kidney disease research by launching a clinical trial to reduce kidney failure by a third, establishing a national biobank, and uniting all affected UK children in one research network.

LifeArc Centre for Rare Mitochondrial Diseases

With The Lily Foundation and Muscular Dystrophy UK, this centre will develop a platform to fast-track new treatments and biomarkers for rare mitochondrial diseases into clinical trials.

Seeing our impact for patients

Over the past year, we have also seen several projects we have supported since 2019 show promise in delivering potentially life-changing impact for people living with rare diseases.

The DefiNe clinical trial, which we supported, began work to **investigate the potential of repurposing deferiprone** – a UK-licensed treatment for certain blood conditions – for patients with neuroferritinopathy. This ultra-rare genetic disease causes severe disability and currently has no cure. If successful, this drug could stop the progression of the disease and offer hope for affected families.

A study we funded, together with partners Action Medical Research, got underway developing a new blood test to **identify children at risk of hypertrophic cardiomyopathy** – a rare but potentially fatal heart condition with no cure. Researchers at University College London (UCL) and Great Ormond Street Hospital (GOSH) hope that by enabling earlier diagnosis, this test could pave the way for better treatments and ultimately help save lives.

Finally, in collaboration with the Aplastic Anaemia Trust, a phase 1 trial – the TIARA trial – led by King's College Hospital and King's College London began, testing a **cell therapy as a potential new treatment for aplastic anaemia,** a rare life-threatening blood disorder. If successful, it could introduce a personalised, donor-free cell therapy that restores healthy blood production and significantly improves patients' quality of life.

In the spotlight

Exploring a new gene therapy to treat an ultra-rare genetic condition in children

Many proven gene therapies for rare conditions remain out of reach due to commercial barriers that prevent them from reaching or staying on the market.

We've partnered with Great Ormond Street Hospital (GOSH) and GOSH Charity to support the exploration of a pioneering approach for ADA-SCID (adenosine deaminase severe combined immunodeficiency) — an ultra-rare genetic condition that leaves babies without a functioning immune system and affects up to 3 children in the UK each year.

A new lentiviral gene therapy, co-developed by GOSH and the UCL Great Ormond Street Institute of Child Health in the UK, and UCLA in the USA, works by replacing the faulty ADA gene with a working copy, restoring the production of an essential enzyme that allows immune cells to grow and divide.

Clinical trials at GOSH and UCLA Mattel Children's Hospital have shown promising results, with children successfully treated. However, commercial challenges have made continued access to the therapy difficult.

With our funding and support, GOSH will now explore taking on the licence to manufacture and deliver this therapy directly – marking the first time an NHS Trust has done so. The aim is to make this life-saving treatment available not only to children at GOSH, but also to others across the NHS through collaborative agreements.

Supporting rare disease clinical trials

We are supporting early-stage rare disease clinical trials testing new therapeutics, diagnostics and devices to increase the likelihood that promising innovations will reach patients. In 2024, we continued to fund a growing number of trials across the UK.

Trial name	Description	Phase	Area
MPS II Hunter disease gene therapy trial	Testing a lentiviral gene therapy in young children with MPS II Hunter syndrome — the first trial of this kind approved in the UK, led from Manchester.	I–II	Therapeutic
TIARA trial	Evaluating expanded autologous regulatory T cells in patients with refractory aplastic anaemia to assess safety and early immune responses. Early results are promising.	I	Therapeutic
STOPFOP2A trial	Evaluating the kinase inhibitor saracatinib in adults with fibrodysplasia ossificans progressiva (FOP) as part of an extended Phase IIA study.	II	Therapeutic
FARGO trial	Investigating faecal microbiota transplantation as a treatment for primary sclerosing cholangitis.	II	Therapeutic
DefiNe trial	Evaluating deferiprone to slow disease progression by reducing brain iron accumulation in patients with neuroferritinopathy, a genetic neurodegenerative disorder.	II	Therapeutic
CADET trial	Testing deep brain stimulation as a medical device intervention in children with Lennox-Gastaut syndrome, a severe childhood epilepsy. Early results show reduced seizure frequency.	II	Device
CoA-Z trial	Investigating a novel vitamin metabolite (CoA-Z) for the treatment of Pantothenate Kinase Associated Neurodegeneration (PKAN), a rare, genetic neurodegenerative disorder characterized by brain iron accumulation.	II	Therapeutic
KARSARC trial	Exploring the use of KARSARC, a gene expression-based risk classifier, in patients with soft tissue sarcomas treated with pazopanib (GENPAZ).		Diagnostic

Global Health Infection

Addressing the world's most urgent medical challenges

Infectious diseases are among the greatest threats to health across the world. They can spread rapidly, disregard borders and are responsible for millions of deaths each year.

The impact of infectious diseases is projected to escalate due to factors such as climate change, migration and intensive farming and the rise of antimicrobial resistance (AMR) adds to the challenge, making some infections increasingly difficult to treat.

In 2024, under the global health umbrella, we prioritised infectious diseases with a special focus on AMR as well as areas of neglected tropical diseases and emerging infections where we can make an impact.

We are working in collaboration with a range of stakeholders to accelerate the development of affordable and accessible solutions to better understand, prevent, treat and control infectious diseases around the world, saving and changing the lives of millions of people.

In 2019, over

13 million

people died from infections, with respiratory and bloodstream infections being the deadliest

AMR is one of the top 10 global health threats, predicted to cause more than

39 million

deaths between 2025 and 2050

Tackling global health challenges

Global health challenges can be complex, and understanding the ever-changing landscape is not always easy. It is therefore critical for us to reflect on our Global Health strategy, to refine its focus and activities to address the most pressing unmet need. As part of this new direction, we now have a new Head of Global Health, Dr Ghada Zoubiane, and Chair of Global Health, Professor Kelly Chibale.

LifeArc supporting the team in Zambia and Kenya to deliver on the ASPIRE programme to mitigate AMR.

Growing partnerships in Africa

AMR and infectious diseases are global challenges, with the highest burden experienced by those living in low- and middle-income countries. In many regions, insufficient infection prevention and control measures combined with weaker health systems, limited access to antibiotics, and their inappropriate use, can hinder progress.

Recognising this urgent need, we are working closely with global experts and a range of partners and stakeholders across Africa to strengthen research capacity and capabilities and advance the development of solutions that will improve diagnosis and treatment of infectious diseases.

We partnered with the University of Cape Town to launch a new **Centre for Translational AMR Research (CTAR)** to accelerate the development of new treatments for drug-resistant infections.

CTAR provides a platform and a go-to place combining our unique capabilities in R&D with the University of Cape Town to strengthen local research capacity in Africa, and to develop new treatments for multidrug-resistant infections. Alongside our funding, we will provide multidisciplinary support, training, and scientific placements to support CTAR.

In addition, in partnership with ReAct Africa, we launched the Antibiotic Stewardship Programme through Innovation, Research and Education (ASPIRE) **to combat AMR in Zambia and Kenya.** The project aims to enhance antibiotic stewardship through digital tools, education, and policy improvements, creating a model for wider adoption in low- and middle-income countries.

In 2024, the **Crick Africa Network (CAN)**, a partnership between LifeArc, the Francis Crick Institute and 5 African institutions, awarded 8 new African Career Acceleration Fellowships to support researchers working on HIV, tuberculosis, sleeping sickness, and other health challenges. This type of support will empower fellows to address unmet medical needs in their communities and develop their independent work into fully-fledged translational research programmes.

Lastly, in partnership with the Gates Foundation, we have co-funded, as part of the Grand Challenges African Drug Discovery Accelerator programme (GC ADDA), **5 African-led drug discovery projects** to strengthen the continent's drug discovery capabilities and create a project-driven virtual African drug discovery network. Funding from GC ADDA has already supported two cohorts of 8 projects focusing on tuberculosis and malaria.


Prof Kelly Chibale, Chair of Global Health


Ghada Zoubiane, Head of Global Health

In the spotlight

PACE | Pathways to Antimicrobial Clinical Efficacy

PACE: Advancing new treatments and tests to mitigate antimicrobial resistance

Collaboration is key to tackling AMR. That's why we partnered with Innovate UK and Medicines Discovery Catapult to launch PACE (Pathways to Antimicrobial Clinical Efficacy) – a £30 million initiative to accelerate innovation and build a world-leading preclinical AMR pipeline that can save lives.

Several financial, commercial and technical barriers stall the speed and success of developing new antimicrobial treatments and tests. Securing investment is challenging because new antimicrobials have limited opportunities for use and provide low return on investment.

Developing new antibiotics also requires significant scientific expertise for them to be specific and selective for particular pathogens. While a targeted approach can help overcome resistance, it again further limits their use to smaller patient groups and minimises financial returns. What's more, precise and rapid diagnostic tests are needed to ensure these types of selective antibiotics are used appropriately.

Due to these challenges, many pharmaceutical companies have had to scale down or abandon their efforts in this area. Subsequently, the antibiotic discovery and development burden has shifted to small and medium enterprises and academic groups, which don't necessarily have the full range of capabilities and financial backing to take a new drug or test all the way to market.

PACE is a

£30 million

initiative to accelerate innovation and build a world-leading preclinical AMR pipeline that can save lives

An initiative to accelerate progress

PACE provides wrap-around support including advice, funding and expertise to help innovators move their projects forward with greater speed and confidence, giving the very best AMR innovations the greatest chance of succeeding.

PACE announced its first funding call in 2023, with up to £10 million available to support early-stage, novel antibacterial therapeutics aimed at treating bacterial infections with high unmet needs. Selected projects received up to £1 million in grant funding for a duration of up to 2 years.

In 2024, PACE launched its second funding round, offering up to £5 million to support the development of in vitro diagnostics that can improve the speed and precision of infection diagnosis. Up to 8 projects will receive awards ranging from £300,000 to £1 million.

Moving forward, PACE aims to de-risk and progress these projects, build a collaborative network of researchers to sustain and propagate knowledge in the field, and look to provide further funding and support to address gaps in the AMR R&D landscape.

Read more about the unmet needs in AMR diagnostics >

Supporting promising science

We back great science focused on tackling infectious diseases through innovative treatments, tests and devices.

One of the projects we are supporting is a partnership between the UK and India aimed at developing a simple and affordable test to detect urinary tract infections in India. The **Diagnostics for One Health and User Driven Solutions for Antimicrobial Resistance 2** (DOSA2) project, will enable early detection and guide appropriate treatment decisions, improve access to diagnostics, curb antibiotic misuse, reduce stigma around visiting the doctor, and serve as a model for other hard to reach patients in other countries.

The **Dengue Innovation Awards** have funded 3 projects in India and Australia to advance dengue research. These aim to develop new treatments, improve targeted drug delivery, and identify biomarkers to predict disease severity.

Last year, with iiCON and the Liverpool School of Tropical Medicine, we launched a new £2.7 million Translational Development Fund and initiated 4 projects across Africa that will **develop diagnostics and treatments to tackle emerging viral threats and neglected tropical diseases.**

In collaboration with Drugs & Diagnostics for Tropical Diseases (DDTD) and Novarum Dx, we are supporting scientists **testing a new smartphone app to improve the detection of NTDs** and improve clinical practice.

In partnership with The Gates Foundation, we launched a funding call for **innovative solutions for exceptionally low-cost monoclonal antibody (mAb) manufacturing.** mAbs are highly effective treatments but their high production costs limit access in low- and middle-income settings.

Through this call, we aim to support manufacturing platforms that reduce costs and enhance the production efficiency of mAbs to expand access and affordability to these life-saving treatments.

Tuberculosis molecular bacterial load assay (TB-MBLA)

A new assay to transform tuberculosis monitoring.

Read more on page 52

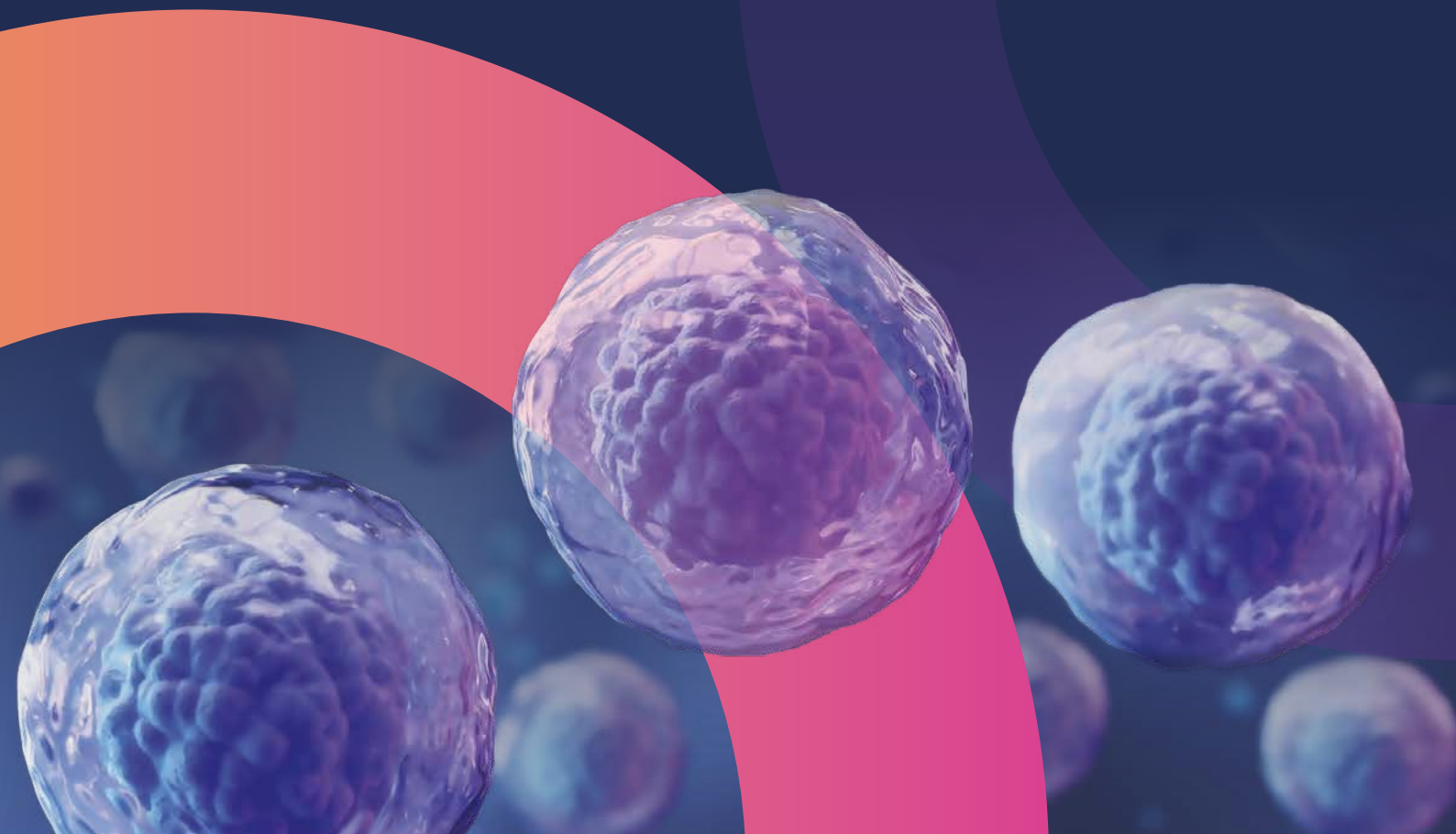
LifeArc has joined the Fleming Initiative as a founding partner, contributing to the initiative reaching a £100m milestone.

The Initiative is a new approach focused on addressing unmet and emerging needs that individual organisations and teams would be unable to tackle alone. The combined funding will be used to kickstart global programmes and outline strategic research themes to rapidly advance solutions to these urgent challenges. We are contributing £25 million, and will harness our expertise in drug discovery, intellectual property management and technology transfer to mitigate AMR globally.

Science at LifeArc

While we support many promising science projects through partnerships and investment, we also conduct our own research.

We offer personalised strategic advice on potential target product profiles and development routes to market, and can facilitate connections with commercial partners, patient groups and other not-for-profit organisations.



We advance early-stage antibodies, drugs and diagnostics to a point where they can become the next generation of tests, treatments and cures.

We closed our molecular diagnostics laboratory in 2024 so that we may align our resources to a broader range of diagnostic solutions through partnership.

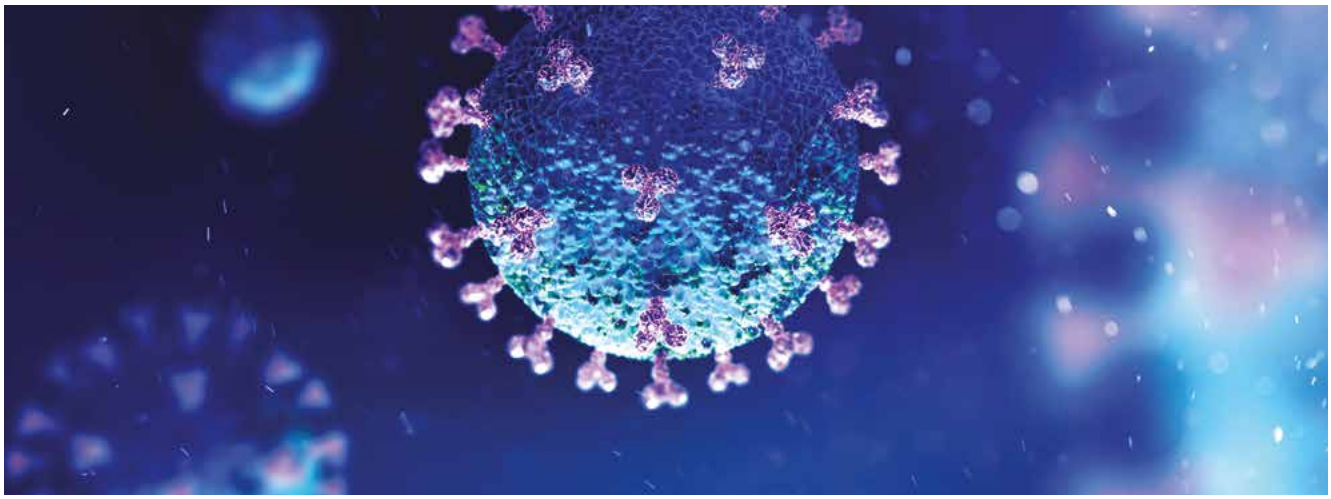
Our capabilities

Therapeutics

- Antibody discovery
- Antibody humanization
- Targeted protein degradation

Supporting technology

- Induced pluripotent stem cell (iPSC) technology
- Microbiology
- Data science



Antibody discovery

A clinically validated fully human transgenic mouse platform to enhance our antibody capabilities.

For over 30 years, we've used our antibody expertise and worked with innovators across the world to translate their scientific discoveries into treatments for improving patient health by humanizing promising antibodies – bringing 5 drugs to market, including Keytruda®.

This year, we have further built on our experience and expertise with the launch of our B-SMArT™ (B-cell screening method for antibody therapeutics) platform at PEGS (Protein and Antibody Engineering Summit) Europe to external partners within the life science ecosystem.

Our B-SMArT™ platform uses genetically engineered mice with human V-regions to generate high-quality antibodies tailored to even the most challenging targets.

Through innovative technologies like single B-cell sorting and the Beacon® Optofluidic System, combined with next-generation sequencing and bioinformatics, we rapidly identify and select the most promising antibodies. This precision reduces weeks of screening time and ensures we focus on candidates with optimal drug-like properties.

This allows partners to work with us in one of two ways: with a promising molecule from a parent species (humanization), or from any innovative target – bypassing the need for humanization (discovery).

Offering both discovery and humanization allows a wider range of partners to work with us, on projects that have the most promising potential for patient impact. Our goal is to enhance the success rate of projects advancing drug candidates toward downstream development while accelerating their path to the clinic.

Collaboration is at the heart of what we do

Working closely with partners throughout the project, we provide access to our world-class expertise and cutting-edge technology, as well as post-delivery translational guidance, including publication assistance and patent support.

Our partners retain full intellectual property rights and commercialisation control after the project ends.

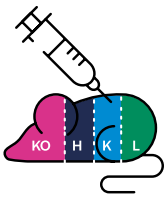
We offer comprehensive end-to-end discovery capabilities with flexible partnering models where we fund projects with strong scientific rationale and clear commercial routes to maximise patient impact.

We understand the risks in the discovery and development of novel therapeutics, and we minimise our partners' risk by funding the projects and in return seeking single digit royalties upon market approval.

With the addition of this antibody discovery capability, we continue to translate innovative science into therapies that have the potential to change the lives of people living with some of the most critical unmet needs.

Fully human antibodies for almost any viable target

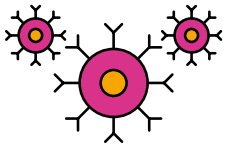
Immunisation



Multiple choices

(protein, peptides, DNA, cells, mRNA LNP).

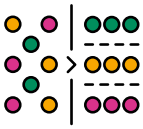
Single B cell screening (FACS or Beacon)



10^6 B cells

Antigen-specific B cells or plasma cells secreting antibodies are identified.

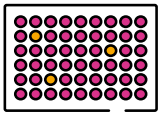
Single B cell sequencing and analysis



1000+ Ag+ B cells

Antibodies with desired properties are prioritised.

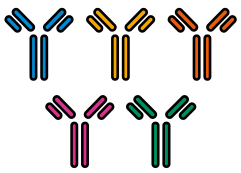
High throughput production and screening



100+ candidates

Antibodies are screened for key features.

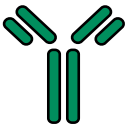
Lead selection



5 to 10 lead antibodies

Promising hits are assessed to identify lead candidate.

Delivery of fully human antibodies



1 to 3 candidates

Lead antibodies, ready for pre-clinical testing and further development.

Antibody humanization

We have been providing expert antibody humanization services for more than 30 years and have helped to humanize nearly 100 therapeutic monoclonal antibodies with a 98% success rate.

We collaborate with academics, healthcare professionals and industry and have worked with 51 partners worldwide, from 13 countries.

We have experience of humanizing antibodies from a diverse range of species and target classes and produce as many candidates as we need to match the affinity of the parent molecule – we don't limit ourselves to a fixed number of variants and produce therapeutic candidate molecules from various modalities.

We've helped to bring

5 drugs to market

for patients

- **Keytruda** ® / pembrolizumab
- **Tysabri** ® / natalizumab
- **Entyvio** ® / vedolizumab
- **Actemra** ® / tocilizumab
- **Leqembi** ® / lecanemab

In the spotlight

Humanizing novel antibodies against an important cancer target

Working with KisoJi Biotechnology in Canada, we've humanized a single domain antibody targetting TROP-2, a highly attractive cancer cell target.

TROP-2 is highly overexpressed on most solid tumours but is present at very low levels on normal tissues, making it an ideal therapeutic target. However, drugging this target has been challenging. Most anti-TROP-2 agents in development, including an FDA-approved drug for breast cancer, are antibody-drug conjugates (ADCs).

Although effective, ADCs rely on a toxic payload that can also harm healthy cells, leading to significant side effects and limiting their therapeutic potential.

Seeking a safer and more effective approach, KisoJi Biotechnology developed a range of single-domain antibodies against TROP-2 using their KisoMouse platform. These antibodies demonstrated the ability to inhibit tumour growth in preclinical models without requiring a cytotoxic payload.

Recognising our expertise and reputation in antibody humanization, KisoJi partnered with us in May 2022 to humanize their antibodies. Together, within a two-year period we've identified humanized candidates with strong therapeutic potential and the lead antibody, KJ-103, has now entered a clinical development partnership with Cancer Research UK.

This innovative antibody will undergo phase 1/2a trials in TROP-2-expressing tumours, including hard-to-treat cancers like lung, ovarian, and pancreatic cancer. If successful, it could provide an effective and less toxic treatment option for patients.

This is the first unconjugated anti-TROP-2 antibody to show strong efficacy in in vivo models, offering exciting new possibilities for cancer treatment.

> "Even though we're in Canada and LifeArc is in the UK, working together was like working as one team.
>
> We were very impressed with LifeArc's thoroughness, efficiency and comprehensive approach to antibody humanization."

Gordon Ngan,
VP Business Development
KisoJi Biotechnology Inc.

Data science: delivering patient impact through a new capability

Data scientists analyse and interpret complex datasets by applying techniques from statistics, computer science, and machine learning to extract meaningful insights.

This can uncover patterns and predict outcomes to improve disease diagnosis, drug target identification, molecule design, and treatment strategies, among many other potential applications. Most importantly, it has the potential to deliver tangible patient impact.

Recognising the importance of data science, in 2022 we established a dedicated team to consolidate our expertise and expand our capabilities in this area. What started as a team of 6 people has now grown to 19, with specialised skills in bioinformatics, computational chemistry and cheminformatics, clinical and healthcare data applications, AI/machine learning and data engineering.

Our data scientists are exploring new ways to deliver patient impact through digital approaches.

We are currently working on a range of projects focussing on clinical and healthcare data analysis, drug target identification, and designing antibodies and small molecules.

This year, we also reached an exciting milestone by becoming a founding partner in the Our Future Health project. Read more on page 47. This gives us access to data from the world's largest health research programme. We plan to use this data in multiple projects across our Translational Challenges, some of which are already underway.

As we continue to expand our expertise in data science, we aim to drive forward new healthcare solutions faster and more effectively.

In 2022 we established a dedicated team to consolidate our expertise and expand our capabilities in this area

Project

Using data to guide treatment centre planning for a rare pain condition

Trigeminal neuralgia (TN) is a rare condition that causes intense nerve pain – similar to an electric shock – on one side of the face. The condition is treatable once it's been correctly diagnosed. But currently, that process takes 4 to 5 years, leaving patients in immense pain as they search for answers.

In 2020, we partnered with Professor Joanna Zakrzewska at University College London to develop an evidence package on TN's prevalence and incidence in different UK regions. Using de-identified electronic health records accessed through the Clinical Practice Research Datalink (CPRD) and our data science expertise, we mapped patient journeys to identify diagnostic delays and critical touchpoints for early intervention.

This package will be used to advocate for centres of excellence dedicated to TN diagnosis and treatment. It will inform decision-making, ensure that resources are allocated where they are most needed and ultimately improve patient outcomes by addressing barriers to timely care.

Accessing sensitive GP health records for this project involved gaining approvals through a strict governance process, which we achieved at the beginning of 2024. The team then analysed the data to pinpoint the clinical events that could flag TN earlier in a patient's journey.

With analyses complete, we're now supporting Professor Zakrzewska in using this evidence to influence TN care.

Beyond TN, this project has already led to follow-up projects in other disease areas, such as bronchiectasis and cystic fibrosis. These projects apply similar methodologies to identify signs and symptoms for earlier diagnosis and treatment.

Project

Accelerating diagnosis for bronchiectasis with machine learning

Bronchiectasis is a chronic lung condition that can cause long-term damage to the lungs if left untreated. Early diagnosis is crucial for effective treatment, but current diagnostics and knowledge about the disease are lacking.

This means the path to diagnosis can be extremely long, resulting in further complications that could otherwise be avoided, and missed opportunities for early intervention.

Working with Clinical Professor James Chalmers at Dundee University, we are using our expertise in data science and machine learning to analyse healthcare data from the EMBARC registry and NHS East of Scotland to identify early warning signs that suggest a patient should see a bronchiectasis specialist. We're also planning how to turn our results into real changes in the healthcare pathway.

By mapping patient journeys and analysing prescription patterns and other data, we aim to uncover insights that could help speed up diagnosis and enable patients to get the treatment they need more quickly.

This project started in January 2025 and marks the start of building our clinical data science and machine learning capabilities to address gaps in early disease detection for underserved conditions like bronchiectasis.

Project

Uncovering the impact of lung infections on cystic fibrosis outcomes

People living with cystic fibrosis (CF) often face recurrent lung infections that can significantly impact quality of life. Despite advances in care, a significant gap remains in tailoring treatments to the unique needs of each patient. To be able to do this, we need to gain a deeper understanding of infection dynamics.

This year, we launched an initiative to use machine learning to investigate how lung infections influence clinical outcomes for CF patients.

We will study microbiological and clinical data from the Royal Brompton Hospital and Professor Jane Davies at Imperial College London to understand how different types of lung infections affect breathing and overall lung health. By identifying patterns, we hope to see if the presence, combination, or severity of infections can help predict changes in lung function and lead to better treatments.

Through this project, we hope to support vital research that addresses unanswered questions about CF and provide insights that could guide future treatment strategies.

Project

Amplifying impact of a longitudinal study to accelerate epidermolysis bullosa research

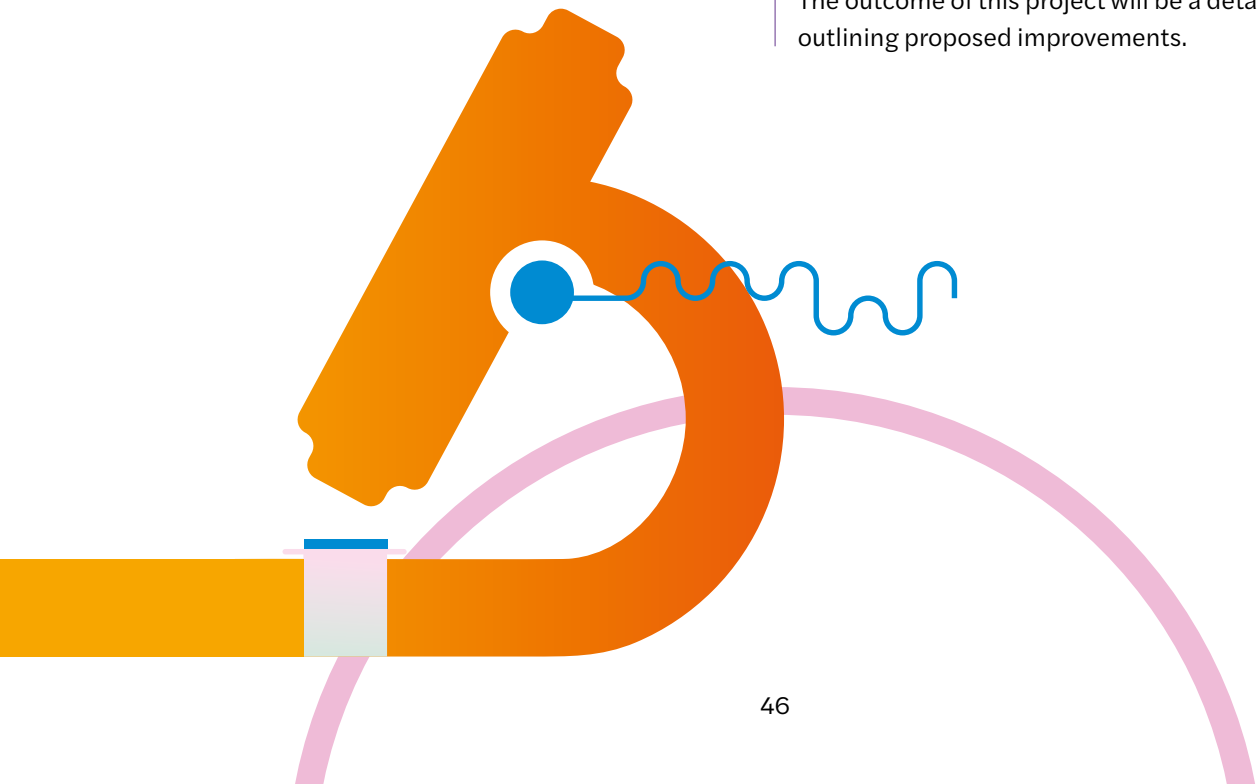
Epidermolysis bullosa (EB) is a rare genetic skin condition characterised by extreme fragility of the skin, causing painful blisters and wounds from minor friction. There is currently no cure, and effective treatments are limited.

To help accelerate research in this field, our data science experts are auditing data from a longitudinal study, PEBLES, to identify gaps and provide actionable recommendations to enhance its potential for use in devising clinical trials, developing new treatments and supporting regulatory submissions.

This project, funded by us in collaboration with our partner DEBRA Austria, aims to enhance the data set's value for translational research and therapeutic development.

The registry currently includes data from approximately 100 individuals with this rare condition. The audit focuses on giving independent advice on ensuring the registry includes essential information, such as treatment histories, which are critical for drug repurposing and designing clinical studies.

The outcome of this project will be a detailed report outlining proposed improvements.



In the spotlight

Our Future Health: using healthcare data to transform the way we fight disease

In 2024, we became a funding charity partner of Our Future Health (OFH), the UK's largest health research programme.

OFH aims to build one of the most detailed pictures of the nation's health by gathering data from up to 5 million volunteers and using it to transform the prevention, detection, and treatment of diseases.

This ambitious initiative brings together the public sector, life sciences organisations, and leading UK health charities, combining their expertise and resources to improve health for the entire UK population.

OFH is designed to truly represent the diversity of the UK population. By including health data from individuals across all backgrounds, including historically underrepresented minority groups, it aims to ensure equitable health insights.

This year, OFH reached a significant milestone, recruiting over one million participants.

Clinical and health data programmes like this have immense potential to deliver significant patient impact.

By analysing large-scale longitudinal population data through advanced digital methodologies, it will be possible to improve diagnosis and earlier detection of disease risk, in turn enabling earlier intervention or even disease prevention. This will also accelerate trial recruitment and advance opportunities for precision medicine.

During the event to celebrate one million OFH participants, our CEO Sam Barrell participated in a panel discussion, highlighting the immense potential of this data to transform people's health and its vital role in driving scientific advancements.

Science supporting our Translational Challenges

Much of the science we do directly supports our Translational Challenges. Read on to hear about some of the exciting projects underway in our labs.

Project

Improving motor neuron disease modelling with induced pluripotent stem cells

Since 2022, we have been developing our in-house induced pluripotent stem cell (iPSC) capability.

iPSCs are generated from biological samples donated by living patients, for example a blood sample or skin biopsy, and are capable of becoming any cell type in the body. Unlike the cells frequently used in drug discovery, which are most commonly derived from tumour tissues, these types of cells are invaluable because they provide a more accurate representation of human disease, improving the chances of finding effective treatments.

This capability supports our research in motor neuron disease (MND), where modelling neuronal function is challenging with traditional methods. iPSCs allow us to generate specific neuronal cell types affected by MND, creating precise disease models for drug discovery.

In 2024, we increased our capabilities in high-quality motor neuron production, generating up to 20 million neurons per cell line every 2 weeks. This ensures that neuron availability is no longer a limiting factor, enabling us to support multiple projects simultaneously.

We are currently collaborating on several MND projects, aiming to progress projects with novel targets to impact patients.

Looking forward, we plan to expand the platform's capabilities to include other brain cell types, such as astrocytes. This will allow the platform to support a wider range of neuroscience research projects including those related to both MND and rare dementias.

Project

A finger-prick blood test for early Alzheimer's diagnosis

Early detection of Alzheimer's disease is crucial for timely intervention, allowing patients to access emerging treatments that can slow disease progression and improve quality of life.

In 2024, we partnered with the Global Alzheimer's Platform Foundation (GAP), leaders of BioHermes-002, an international study aiming to identify blood and digital tests that could help early diagnosis of Alzheimer's disease. Within BioHermes-002, we are supporting the validation of a finger-prick blood test for early detection of Alzheimer's disease. The test could also help identify those at risk of developing the disease before symptoms appear. Current diagnostic methods, like brain scans and spinal taps, are expensive, invasive, and inaccessible to many.

The LifeArc-supported study aims to evaluate a simple alternative test that measures three blood biomarkers known to be associated with Alzheimer's. The biomarker analysis will be carried out by our partner, the UK Dementia Research Institute (UK DRI) through its UK DRI Biomarker Factory based at University College London. Results will be compared to the current gold standard diagnostic testing using imaging of Alzheimer's hallmarks in the brain.

BioHermes-002 is the largest, most diverse, international study of its kind, involving 1,000 participants from diverse backgrounds across the UK, US, and Canada, with at least 25% from under-represented ethnic communities. This diversity is key to understanding the test's effectiveness across populations.

If successful, it could lead to a new scalable, accessible and much cheaper test that could revolutionise early diagnosis of Alzheimer's disease.

1 million

people living with neurodegenerative conditions in the UK

Partnership

Establishing a strong pipeline of translational neurodegenerative research with the UK Dementia Research Institute

There are more than 1 million people in the UK living with neurodegenerative conditions including Alzheimer's disease, motor neuron disease, frontotemporal dementia and Parkinson's disease. Despite significant advancements in understanding these diseases, effective treatment options are lacking.

To address this significant unmet patient need, we partnered with the UK Dementia Research Institute (UK DRI) in 2022 to accelerate the development of diagnostic tests, treatments, and devices for people living with dementia and other neurodegenerative diseases. This five-year, £30 million collaboration combines the UK DRI's discovery science expertise with our translational capabilities to transform promising research into tangible solutions that improve lives.

Through this collaboration, £14.5 million was allocated to 7 pioneering projects in 2023. These include developing antibody therapies, gene therapies, and scalable diagnostics, as well as digitally enhanced care solutions.

In 2024, 5 additional early-stage, proof-of-concept projects received up to £300,000, helping researchers generate critical preliminary data to position themselves for larger funding opportunities.

We also provided further early career support through the Translation Pilot Award scheme, with 8 researchers receiving £50,000 each. This initiative fills a critical funding gap, providing young scientists with the resources and mentorship needed to advance their careers while driving innovation in neurodegenerative disease research.

Looking ahead, the partnership will sustain a pipeline of translational research. Future plans include a new large-scale funding call, aligning investments to support the most critical areas of unmet need, and continuing collaborations such as the BioHermes-002 study on dry spot blood tests for early Alzheimer's detection.

Project

A new drug to overcome antibiotic resistance in cystic fibrosis

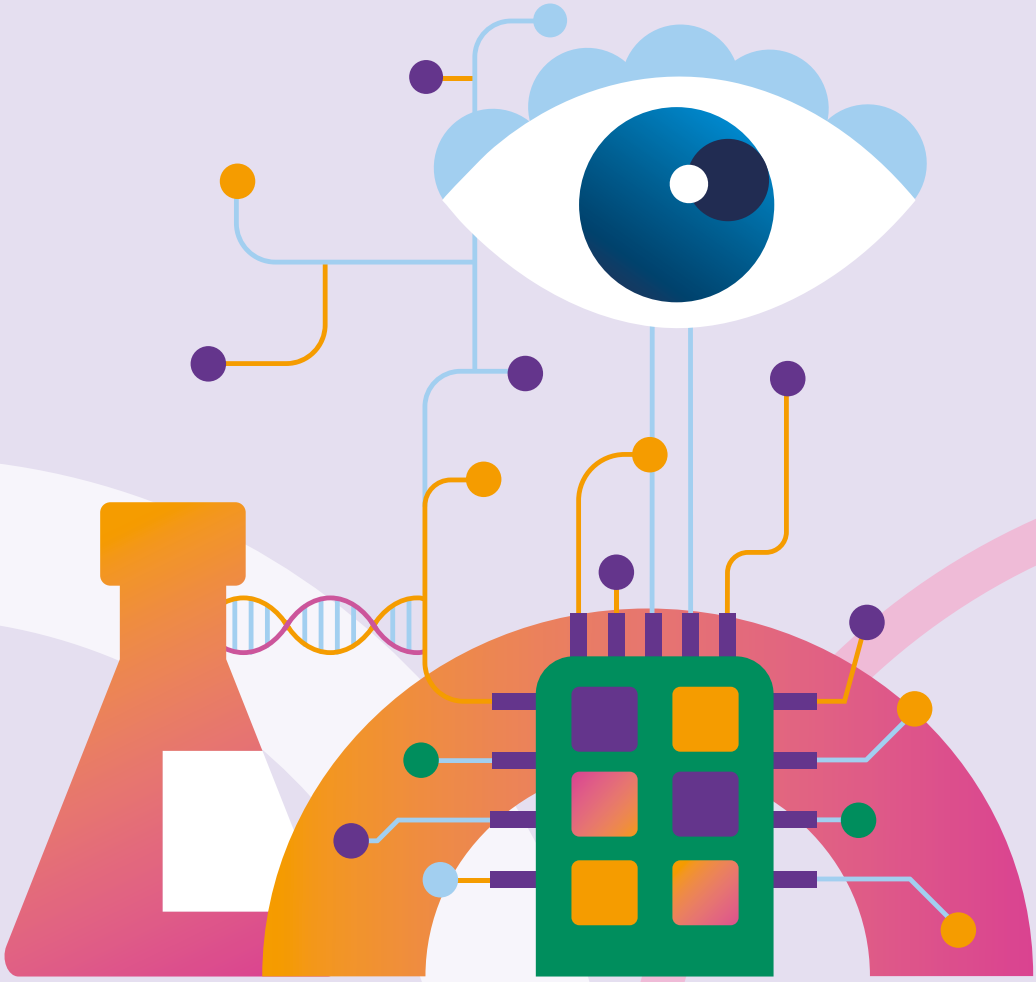
Since 2022, our in-house scientists have been leading an ambitious project to develop a new class of antibiotics for cystic fibrosis (CF).

This chronic lung condition causes sticky mucus to build up in the lungs, promoting bacterial growth and persistent infections that can lead to lasting lung damage if untreated. However, bacteria have evolved mechanisms of resistance that prevent antibiotics used to treat CF infections from working.

We've developed compounds that target multiple bacterial mechanisms simultaneously, making it significantly harder for resistance to develop. By combining this innovative approach, we should increase the range of bacterial species our antibiotics can be used to treat and reduce the chance of resistance developing.

Over the past year, our compounds have shown outstanding results in vitro, performing as well as some of the antibiotics currently used to treat CF infections. These proof-of-concept studies provide evidence that our compounds are viable drug candidates.

We are now preparing for the next stage – testing their effectiveness in real-world settings. If successful, our compounds could offer a much-needed option for people who suffer from chronic lung infections associated with cystic fibrosis. This dual-action approach could also pave the way for broader applications in developing drugs to tackle antimicrobial resistance.



Project

New tests to detect lung infections in bronchiectasis and cystic fibrosis

People living with chronic lung conditions like bronchiectasis (BE) and cystic fibrosis (CF) suffer from recurring lung infections caused by bacteria such as non-tuberculous mycobacteria (NTMs) or pseudomonas.

Monitoring these bacteria is essential for assessing how well treatments are working, guiding clinical decisions, and preventing disease progression. However, the current standard of care methods (sputum culture) are slow, costly and often unreliable, making effective monitoring a significant challenge. Building on what we learned from the TB-MBLA project (read more on page 52), we are developing rapid and accurate diagnostic tests that quantify bacterial load in respiratory infections.

These tests feature in 2 projects

Project 1: non-tuberculous mycobacteria

Project 2: *Pseudomonas aeruginosa* bacteria

The test targeting NTMs will provide a quantitative alternative to traditional cultures, enabling clinicians to identify these resistant and hard-to-detect pathogens. Meanwhile the test for pseudomonas will be used in clinical trials to improve infection measurements during drug development, increasing the likelihood of success so that drugs can reach patients more quickly.

In the past year, we proved that these diagnostic tests work reliably on clinical samples (sputum), providing valuable information which could help doctors diagnose and treat patients effectively.

Project

A new assay to transform tuberculosis monitoring

Tuberculosis (TB) is one of the world's deadliest infectious diseases, with 10.8 million cases and 1.25 million deaths in 2023.

Current detection methods using sputum cultures are slow, unreliable, and prone to contamination, meaning improvements in TB diagnosis and monitoring are crucial in the fight against the disease.

In 2018, we began a collaboration with the University of St Andrews to develop the TB-MBLA (tuberculosis molecular bacterial load assay), a RT-qPCR test that measures bacterial load by amplifying 16S ribosomal RNA from mycobacterium tuberculosis. This assay is one of the first quantitative tools for monitoring live bacterial growth and provides a fast, reliable method for diagnosing TB and tracking the progression of infection over time.

It has the potential to inform adjustments in treatment regimens based on how patients respond and assist with compliance monitoring to ensure patients complete treatment to avoid incomplete infection clearance.

To evaluate its usefulness in clinical settings, we launched our first sponsored clinical study, TIME (TB Diagnosis and Monitoring Evaluation), in 2023. Conducted over 20 months at 4 clinical sites in Tanzania and Uganda with 535 participants, the study concluded in October 2024, generating valuable data to support the assay's potential.

After six years of development and rigorous testing, we are now seeking a commercial partner to bring this transformative TB disease monitoring tool to the clinic. We are also applying the technologies and learnings from this project to develop tests for other types of bacteria present in chronic respiratory infections.

Milestones of the TIME study

Conducted over 20 months, the study generated valuable data to support the TB-MBLA assay's potential

400 TB-MBLA kits shipped to Sub-Saharan Africa

535
participants enrolled

4
study sites in Tanzania and Uganda

11K+
standard of care tests performed

99%
of data points independently verified

32K+
completed forms in the electronic database

43
ethics and regulatory authority submissions and renewals

Project

Advancing childhood cancer treatments with targeted protein degradation

Targeted protein degradation (TPD) is a revolutionary approach in drug discovery that is generating worldwide excitement for its potential to transform small molecule science.

Unlike traditional small molecules that inhibit protein function, TPD uses small molecules called proteolysis-targeting chimeras (PROTACs) to hijack cellular protein disposal systems, enabling the selective elimination of disease-causing proteins altogether.

Recognising the potential of TPD, we have been developing it as a core scientific capability over the past 5 years, building on our long-standing expertise in classical medicinal chemistry. Our team of chemists and biologists now also specialise in TPD and have made some exciting achievements in 2024, particularly in beginning to utilise our TPD expertise in childhood cancer.

One of our first initiatives is the Cancer Grand Challenges PROTECT project, a global collaboration addressing the urgent need for improved treatments for childhood cancers. The aim is to identify novel paediatric E3 ligases—cellular machinery enzymes that tag proteins for degradation—and harness them to create PROTACs designed to target and eliminate cancer-causing proteins. This differentiated approach unlocks new opportunities to tackle historically undruggable proteins.

With TPD in our arsenal, we are leading the way in developing new approaches to tackle some of the toughest diseases. Beyond childhood cancer, this technique has potential for application in our other Translational Challenges, such as advancing treatments for motor neuron disease.

Read our summary of the challenges and opportunities of TPD as an innovative approach to treating neurodegenerative diseases in The Frontiers in Molecular Neuroscience journal >

LifeArc Ventures

We invest in innovative early-stage life sciences companies that can benefit from our investment, advice and scientific insight.

We're committed to investing in companies with promising therapeutics and medical devices that have the potential to positively impact the lives of patients. We offer a strong combination of scientific, commercial and financial support.

Our access to leading scientists with expertise in translational science enables us to make informed decisions for our investments, and work with our portfolio companies to achieve meaningful progress.

Financial returns from our portfolio are returned to LifeArc to help sustain LifeArc in the longer term.

In 2024, we made

3 new investments

17 companies

in our portfolio

Multiple follow-on

investments

Clare Terlouw,
Head of LifeArc Ventures

Portfolio company highlights

LifeArc Ventures made substantial progress in 2024, with 3 new investments, several follow-on investments, and scientific advances by a number of our portfolio companies.

Fluid Biomed Inc. is a clinical-stage medical device company based in Canada advancing the first hybrid polymer-metal stent to treat brain aneurysms.

LifeArc Ventures invested in Fluid Biomed's US$27 million Series A equity financing which closed in December alongside Amplitude Ventures, IAG Capital Partners, ShangBay Capital, METIS Innovative and an undisclosed industry leader.

Kavigale (sipavibart; AZD3152), a monoclonal antibody discovered by RQ Biotechnology and licensed to AstraZeneca in 2022, received a positive CHMP opinion for prevention of COVID 19 in immunocompromised individuals.

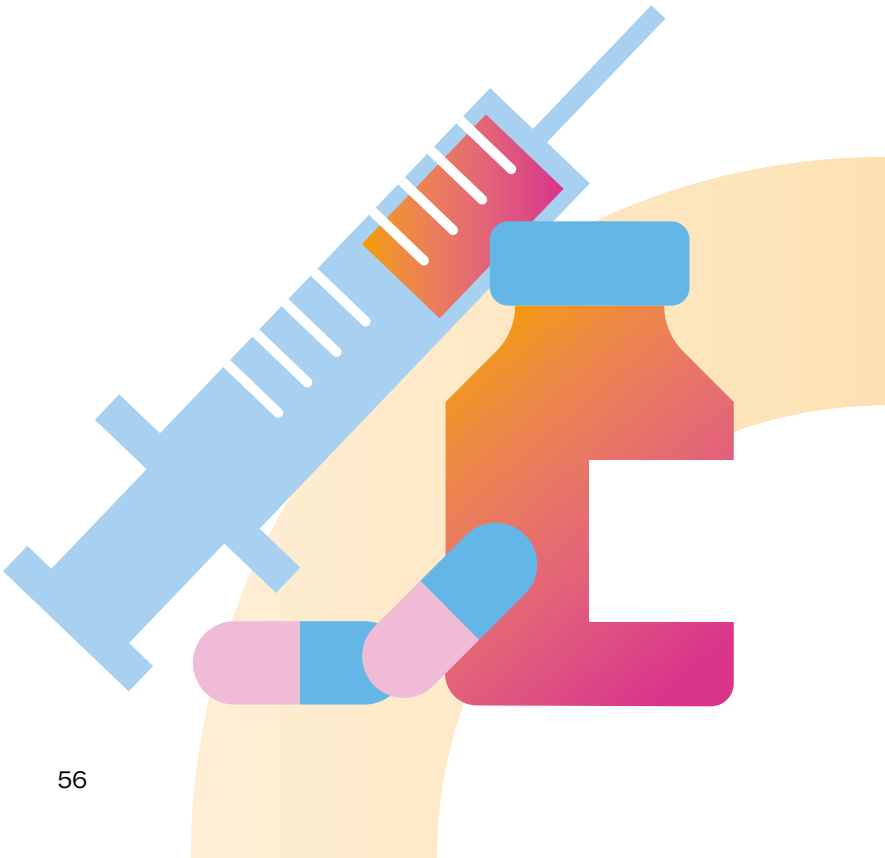
The advance from discovery to a positive CHMP opinion in three years illustrates the speed with which long-acting monoclonal antibodies can be developed as a drug class to protect vulnerable populations against serious viral diseases. The decision further validates RQ Bio's antiviral drug discovery expertise and model for early partnership with the pharmaceutical industry.

AviadoBio announced an exclusive option and license agreement with Astellas for its gene therapy AVB-101 targeting frontotemporal dementia and other indications.

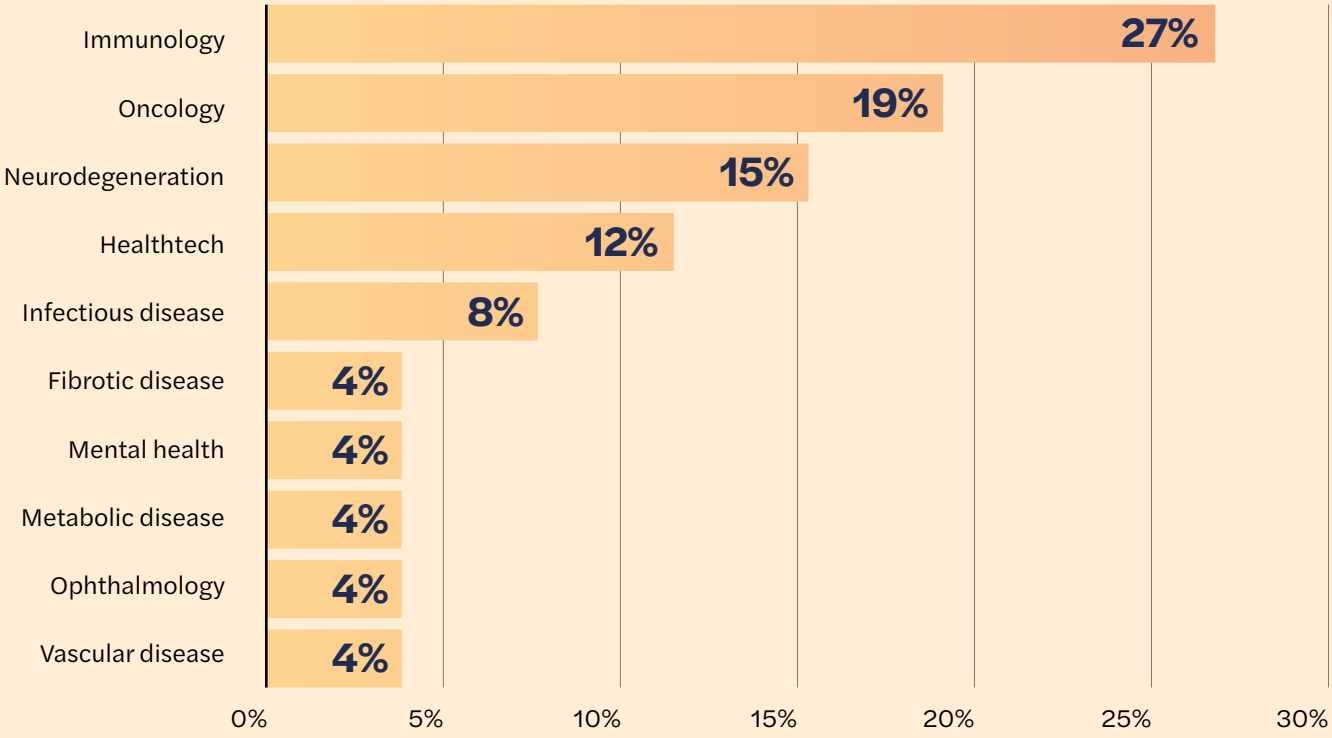
AviadoBio is eligible to receive up to $2.18 billion in license fees and milestone payments, plus royalties, if Astellas exercises its option. A Phase 1/2 clinical trial of AVB-101 is underway in the U.S. and Europe.

LifeArc Ventures further supported Ikarovec's successful seed funding round.

Ikarovec is advancing the novel gene therapy programme IKC159V towards clinical trials in geographic atrophy (GA), a cause of sight loss in millions of people which is often a precursor to wet age-related macular degeneration.



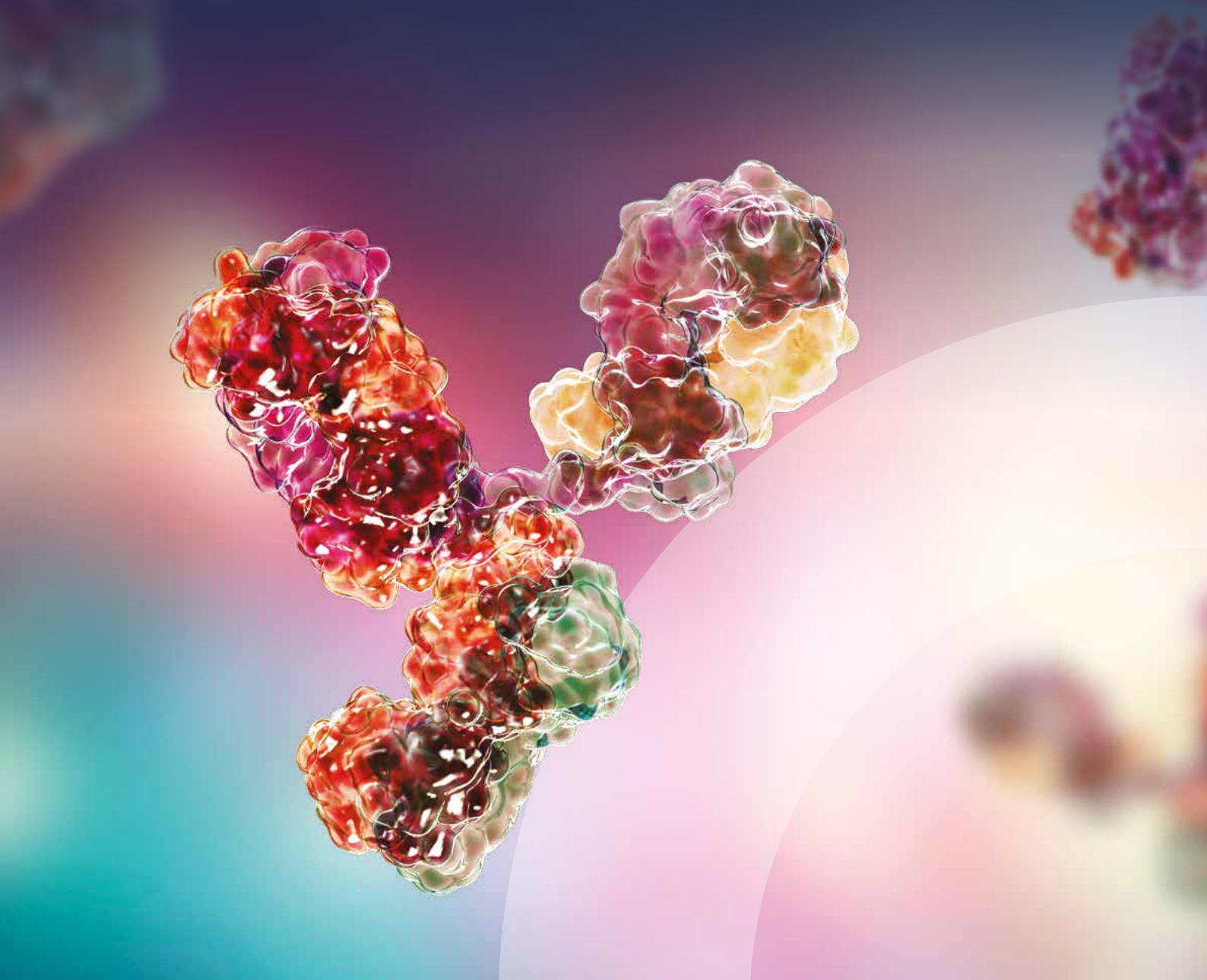
Ventures portfolio composition by area



Commercialisation advice

LifeArc started as the technology transfer arm of the Medical Research Council, and this legacy lives on in the advice we provide partners.

For over 30 years, we've given commercialisation, intellectual property management and technology transfer guidance.



The impact of our work with the MRC in 2024

18
patents filed on behalf of the MRC

12
new licence agreements concluded for MRC reagents

26
new technology disclosures received

£27.4m
total commercialisation income

546
IP assets under management

309
assets managed in the MRC reagents catalogue

80
non-patented assets managed

155
patent families managed

220
agreements concluded

£1.2m
total income received from licensing of MRC reagents catalogue

2
spinout companies from the MRC LMB and a third spinout planned

We help charities...

unlock the potential of existing research portfolios

identify how best to allocate their research investments

with translational support and training

commercialise research through our extensive network of biotech and pharma partners

We advised over 50 charity partners in 2024

In the spotlight

Applying our expertise to support the spin out of Constructive Bio from the Medical Research Council Laboratory of Molecular Biology

We protected, managed and licensed the intellectual property portfolio and provided strategic guidance on commercialisation and technology transfer to drive the successful spin out of Constructive Bio.

Constructive Bio is a synthetic biology company that we helped spin out in 2022, to turn technologies developed in Professor Jason Chin's lab into real-world applications. They are now pioneers of whole genome writing and engineered translation, creating novel next-generation biomolecules for use across therapeutics, chemicals, agriculture, biomaterials and beyond.

Constructive Bio leverages their next generation genome synthesis platform technology 'CONEXER' to enable large scale assembly of synthetic DNA, for the creation of novel organisms with advantageous industrial properties.

Syn61, their flagship recoded *E.coli* strain, is the first of a new class of synthetic organisms with a precise and programmable protein translation machinery which can be used to introduce new chemical functionalities and build novel biomolecules.

Our support of the technology began right from the start, providing early support to the academic founding team before the technology had a clear commercial application. This included helping to secure funding for technologies developed in Prof Chin's lab and evaluating the translational potential of the research.

As the company spun out, we played a pivotal role in its formation, from reviewing the corporate documents to negotiating the IP licence with investors, which helped Constructive Bio secure $15 million in seed funding. The substantial IP portfolio licensed into the company included the core platform technologies, Syn61 and CONEXER, and supplementary patents and materials as part of the wider portfolio.

Since the spin-out of the company, we have remained actively involved, supporting Constructive Bio with IP matters and annual reporting as well as engaging in further licensing discussions.

Constructive Bio recently closed a $58 million Series A funding round, showing strong interest in their technology. While the full impact is still emerging, the pioneering work Constructive Bio is doing could change how we produce medicines, materials, and bio-based products in the future.

We are proud to have supported a company with technology that has the potential to revolutionise multiple industries.

Policy and public affairs

We talk to decision-makers, across the political spectrum, who influence UK science, innovation, technology and health. By feeding in our expertise, we aim to make the UK an ideal place to translate science into impact for people with rare and underserved conditions.

Activity in 2024

We began the year by helping to establish a new **government taskforce on childhood cancer** – an initiative now relaunched under the new government, ensuring continued focus on improving outcomes for young patients.

In March we contributed to crucial discussions on **antimicrobial resistance** at a parliamentary roundtable alongside Dame Sally Davies, reinforcing the need for urgent action in this space.

April saw us convene a private, high-level policy roundtable on the **future of life sciences,** coinciding with the LifeArc Translational Science Summit.

Across the spring we also supported a major policy report from the Campaign for Science and Engineering (CaSE), advocating for a **stronger UK environment for business R&D investment.**

Our engagement with the **Fleming Initiative** from April to July helped shape a new LifeArc partnership, including on policy, which was officially announced in September.

In Autumn we hosted another high-level roundtable, this time focusing on the critical **link between R&D and economic growth,** in collaboration with Wellcome and UK Day One.

And in October our efforts were further amplified by Dr Sam Barrell's article in the New Statesman, highlighting the **importance of rare disease research** in driving UK innovation and growth.

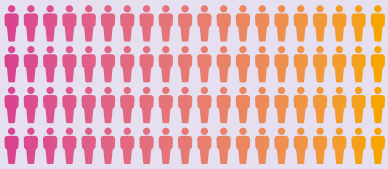
We are proud of our work with decision makers in 2024, and its contribution to making life science life-changing for those with rare and underserved conditions. Looking ahead, we remain committed to shaping policies, fostering collaboration, and driving innovation to make an even greater difference in 2025.

Life at LifeArc

Our people are central to our aims to turn promising scientific breakthroughs into impact for patients.

We thrive on innovation, collaboration and excellence and want all employees to flourish, be challenged and build a rewarding career with us – in an environment that supports them and prioritises their health and wellbeing.

More than
80
people joined LifeArc



21
fellows participated in our 2 fellowship programmes

8
women recruited into senior roles

9
industrial placement students

Highlights from this year

- Received the **TIDE bronze award** from the Employers Network for Equality and Inclusion (following their benchmarking assessment of our progress), recognising our commitment to the Inclusive Culture pillar of (ED&I) strategy
- We now have **4 thriving colleague support networks:** LGBTQ+, Neurodiversity, Parents and Carers, and Menopause Networks
- A new **carers policy** was introduced to support employees with caring responsibilities
- In April 2024, employees received bonus payments under LifeArc's new **annual incentive plan** rewarding performance at both individual and organisational levels
- Significant **growth in the data sciences team,** addressing a key talent gap in a highly competitive field
- Introduced a pilot mentoring scheme.
- A **data-driven performance management platform** was introduced to promote consistency, equity and reduce bias in pe approach to performance management
- The LifeArc Manager Accelerator (LMA) programme trained over 90 leaders & managers in core capabilities, leading to a **49% increase in self-assessed leadership skills**
- Successfully transitioned to an **in-house recruitment model,** reducing hiring costs and supporting sustainable growth
- Of our 72 LifeArc Knowledge Transfer Innovation fellows to date, **88% are female, 29% from under-represented ethnic groups**, and they reside in 14 countries across Europe

Interested in shaping the future of translational science?

Scan to see our latest vacancies and learn about working here

A look to the future

We are excited for a future focused on turning promising scientific research into impact for people living with rare diseases and resistant infections.

Translating scientific discoveries into the next drug, diagnostic or device is a long, expensive, and unpredictable process.

It requires the right expertise and resources coming together at the right time to progress promising innovations. As we look ahead, we remain dedicated to forming partnerships and providing the science, investment and expertise that will help remove barriers to this translation.

We're grateful to the incredible partners who have joined forces with LifeArc to overcome these challenges.

Together, we'll continue to turn great science into new innovations that transform the lives of people living with rare or underserved conditions.

Making life science life changing

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