# MND Association research and innovation strategy

MND Association

# Introduction

**Motor neurone disease is a devastating progressive disease, which robs people of the ability to move, speak and eventually breathe. Research is improving understanding of MND and helping us look after people with the disease more effectively. But we must find new, more effective treatments.**

**A third of people die within a year of being diagnosed with MND, half within two years. That has to change.**

For more than 40 years, the Motor Neurone Disease Association has been leading research efforts that have helped revolutionise our understanding of the biology of MND and taken us to the point where we are starting to see effective treatments coming through for a small proportion of people with the disease. Our new research and innovation strategy will give us the focus we need to progress as fast as possible towards developing new treatments so everyone with MND can live better, longer lives. We know too that we need to diagnose MND earlier and more precisely, so we can intervene faster and personalise treatment.

MND is a hugely complex disease that affects people in various different ways, and we still have a lot to learn about how and why it develops. Only the best and most innovative thinking will give us those answers. At least one in 10 cases of MND are caused by a person inheriting one of several faulty genes – and researchers are making progress developing treatments for these genetic forms. But for the majority of people with MND who have non-inherited or 'sporadic' disease, there have been far fewer advances. Sadly, most clinical trials of new treatments have failed to show any benefit.

This research and innovation strategy has been developed together with people affected by MND and our research partners. We ran surveys, focus groups and a special ranking panel to understand which areas of research the MND community wanted us to prioritise. People with MND told us they need to know what's caused their disease and how quickly their symptoms will progress, as well as to see faster progress towards new treatments. Researchers helped us to understand how we could address these priorities, and deliver real impact for everyone with MND.

With that invaluable insight, we've built a strategy which focuses on the needs of people affected by MND. By working together, we will make tomorrow's treatment a reality.

**Today,**

**six people will be told they have MND and six people will die from the disease.**

**People with MND lose their voice, movement and ultimately their ability to breathe.**

**A third of people will die within a year of diagnosis. More than half will die within two years.**

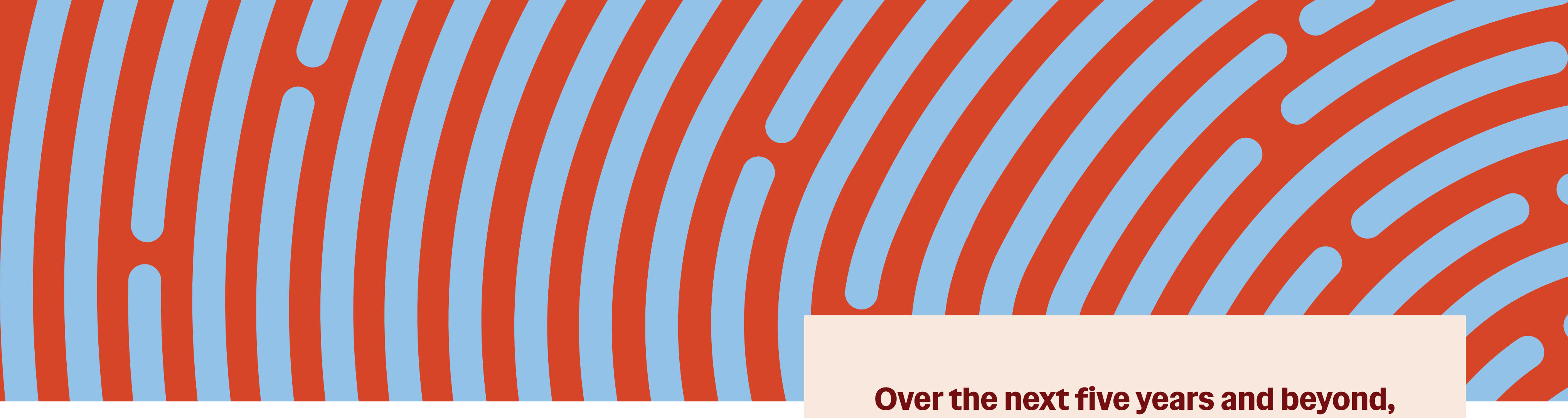
**"We're proud of the progress we've made through research, but there is still so much to do. The next five years offer real potential for change. We will invest more in research than ever before – and do all we can to turn that funding into new treatments, taking bold steps towards our vision of a world free from MND."**

**Tanya Curry, Chief Executive**

**"This is an exciting time for MND research, but we recognise we need to do more to make a real difference for the majority of people affected by the disease. This strategy is unapologetically ambitious, as we seek to make further inroads into our understanding of MND, innovate in the development of new treatments, and ensure more people have the opportunity to take part in clinical trials."**

**Mike Rogers, Director of Research and Innovation**

# Our track record of discovery and impact



**We've supported some of the most exciting initiatives in MND research, and funded discoveries which have grown our understanding of the disease, improved quality of life and opened new avenues to treatment.**

**Over the next five years and beyond, we will harness this progress to deliver tangible improvements in MND diagnosis, treatment and care.**

## Major impacts

**2006**

A world-first trial of non-invasive ventilation, which we co-funded, shows it helps people with MND to breathe, improves quality of life and allows them to live longer.

**2008-2011**

Our research supports the discovery of TDP-43, FUS and c9orf72 gene variants, the cause of three of the four key inherited forms of MND.

**2012**

With our support, researchers use stem cells to model MND in the lab. We also join Project MinE, an innovative genetic testing study now stretching across 23 countries.

**2015**

MND Association-funded researchers are part of collaborative efforts to develop neurofilament as a potential biomarker of MND progression.

**2021**

Our funding supports the development of the innovative HeadUp collar, which helps people with MND overcome head drop.

**2024**

An MND Association-supported trial finds acceptance and commitment therapy can maintain and improve quality of life in people with MND.

**2025**

Innovative genetic therapy tofersen is approved by the MHRA in the UK for people with SOD-1 MND, after trials supported by MND Association-funded care centres.

— **2000** — **2025** —

## Landmark initiatives

**2003**

The UK MND DNA Bank (renamed MND Collections in 2017) is established, collecting over 3,000 samples from people with MND and family members.

**2008**

We establish clinical fellowships with the Medical Research Council and Lady Edith Wolfson Programme, nurturing a new generation of leaders in MND research.

**2009**

We fund the LiCALS trial of lithium carbonate with the UK's first academic-led MND drug trial.

**2017**

With our support, Project AMBRoSIA launches in the UK, collecting samples and data from people with MND and those without to identify new biomarkers of disease.

**2020**

Innovative new clinical trial platform MND-SMART launches, allowing multiple new drugs to be rapidly assessed against a single control group.

**2022**

We're a major partner in the launch of the UK MND Research Institute. In 2023, we are a major funder of the Institute's flagship project, EXPERTS-ALS, a 'pre-trial' platform to screen candidate drugs in people living with ALS to look for early signals of slowing of disease.

**2025**

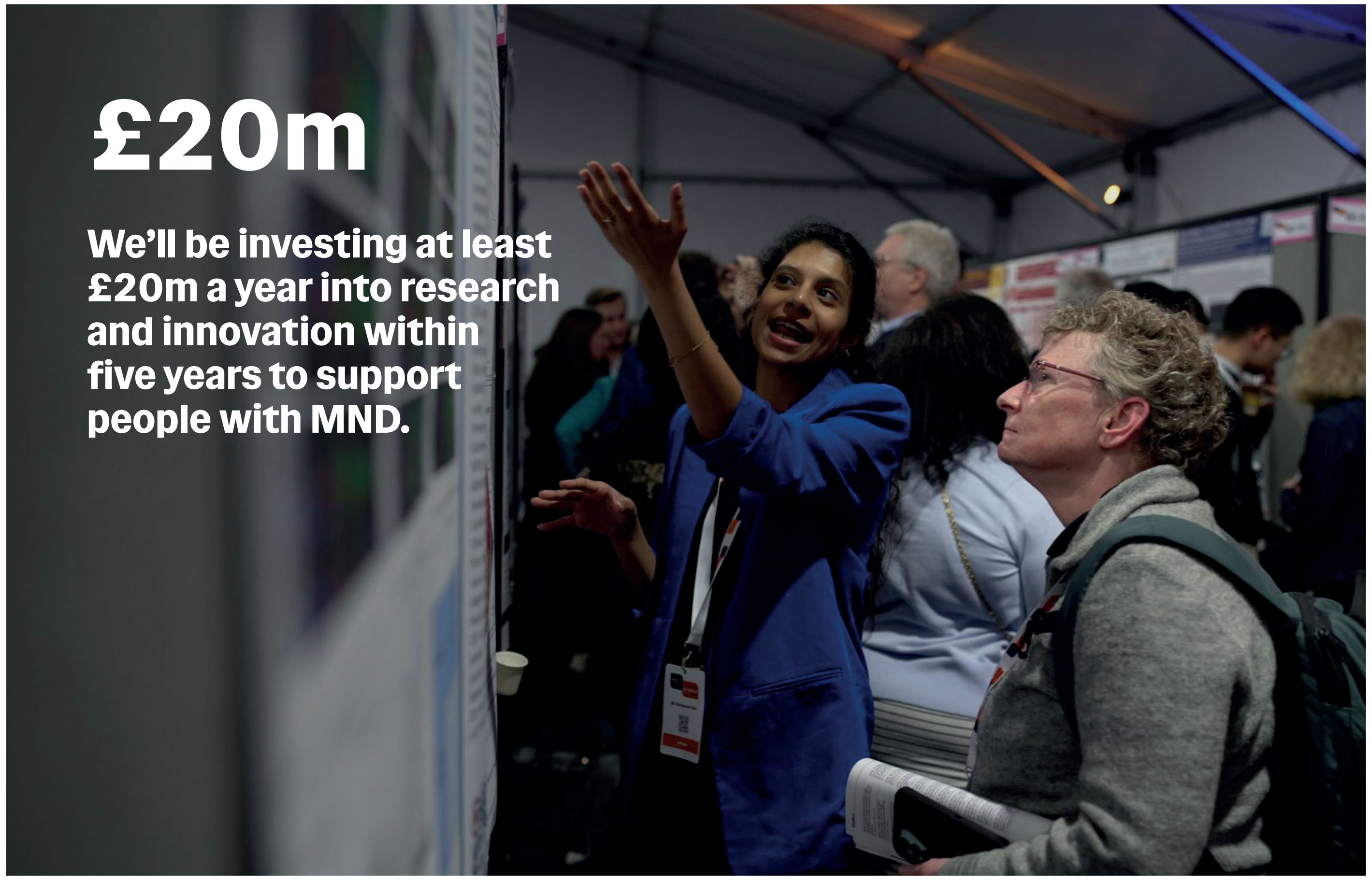
We are the major funder of the Longitude Prize for ALS, an initiative to draw innovative AI technology into the search for potential new treatments.

# Our vision

**This strategy will transform the lives of people affected by MND. We will improve diagnosis, treatment and care for people with MND by funding pioneering science, broaching the big questions and taking a bigger, bolder approach to supporting research.**

We are especially determined to drive advances in sporadic MND, which has seen far less progress towards treatments than forms with known genetic causes. We also want to be more responsive to the needs and priorities of people with MND – answering the questions they've told us are important to them, such as why people develop the disease.

If we're to have a real impact in improving the lives of people with MND, we'll need to harness the most innovative thinking and the latest cutting-edge technologies. We will fund research at a greater scale than ever before, and drive pioneering collaborations between researchers. To make this possible, we'll be investing at least £20m a year into research and innovation within five years.



**£20m**

**We'll be investing at least £20m a year into research and innovation within five years to support people with MND.**

# Our research and innovation aims

# Our strategic aims

**We have four strategic aims for research and innovation over the next five years:**

## Understand

### Revealing causes and targets

We will answer fundamental questions about how and why people develop MND, and identify which causes and triggers are the most promising targets for new treatments.

## Detect

### Better and earlier diagnosis

We will enable neurologists to diagnose MND earlier and more precisely, by creating tests to detect MND at its earliest stages, reveal the specific type of disease and predict how it will progress in each person.

## Discover

### New treatments for everyone

We will lead the search for tomorrow's treatments for people with all forms of MND – especially those with sporadic disease – by supporting major drug discovery collaborations and widening access to clinical trials.

## Innovate

### Innovations in healthcare

We will fund research that addresses the needs of people with MND today by improving their healthcare and quality of life, and assess technologies to support them day to day.

# Understand

## Revealing causes and targets

**Far too much about MND remains poorly understood. We still don't know what causes most cases of MND – a question people really want the answer to – and there are significant gaps in our knowledge about the biological mechanisms that lead to the disease.**

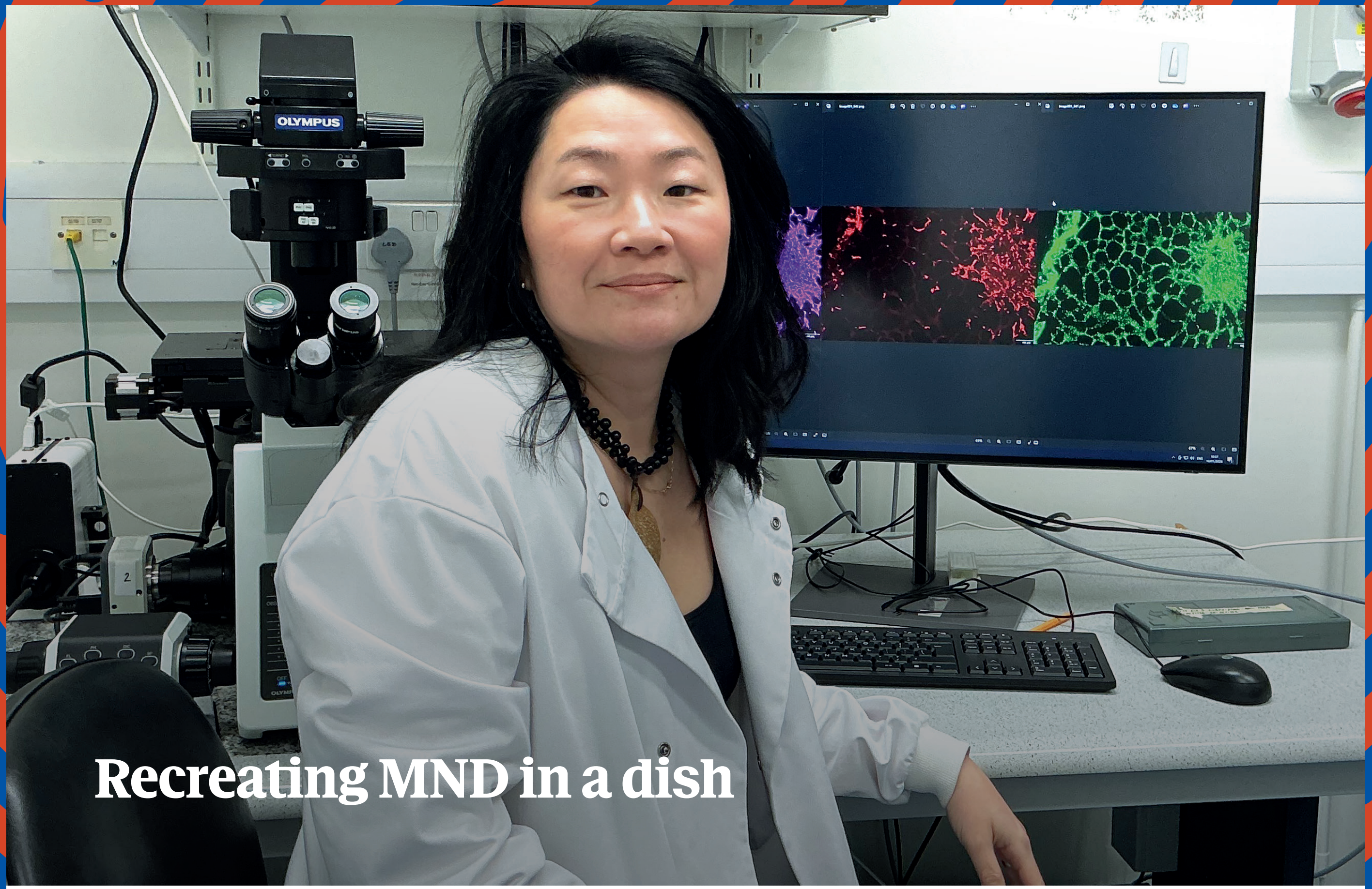
**We must improve understanding of how MND develops, especially in sporadic disease. The more we know, the easier it will be to find new treatments, develop better ways to diagnose MND and even prevent the disease.**

### We will:

- Address a major priority of people with MND by understanding the causes of MND – including the potential role of lifestyle factors and how they interact with people's genetics.
- Improve our understanding of the biological mechanisms that trigger sporadic MND, where there is no clear genetic cause.
- Develop better ways of modelling sporadic MND in the laboratory, so scientists can understand the biological mechanisms that lead to the disease.
- Bring together researchers in MND biology with experts in new therapeutic strategies, to identify the most promising targets for new treatments.

### What this will mean:

- People with MND will have the answers they need about how and why they have developed the disease.
- We will be able to create a new generation of treatments targeting the early biological triggers for MND.
- Knowing the causes of MND will give us vital clues to help us detect the disease earlier or even prevent it.



# Recreating MND in a dish

**Dr Agnes Nishimura is part of a team of researchers who have been recreating MND in the lab so they can understand the disease better and predict whether treatments will work.**

The team has been taking white blood cells from samples collected from people with MND using funding from the MND Association.

They have turned these white blood cells into stem cells, capable of becoming any cell in the body, and then converted them into motor neurones.

These motor neurones share many of the same features of the disease as the person they were taken from, so researchers can use them to study what's triggering the disease and causing the cells to die.

The next step for Agnes is to run clinical trials in a dish using the recreated motor neurones to help predict whether the treatments will work in people.

> **"Stem cell technology is transforming the way we understand and treat motor neurone disease. This approach opens the door for personalised medicine – where therapies are tested on patient specific cells before reaching the clinic."**
>
> **Dr Agnes Nishimura**
> **Senior Lecturer in Stem Cell Biology and Molecular Neuroscience at Queen Mary University of London**

# Detect

## Better and earlier diagnosis

**It can take many months or even years to diagnose someone with MND and even then they may have no idea how quickly symptoms will progress. Improving diagnosis of MND is essential to our goal of finding more effective treatments.**

**We need tests that can tell people what form of MND they have and what their prognosis is likely to be, so their treatment and care can be tailored to what they need.**

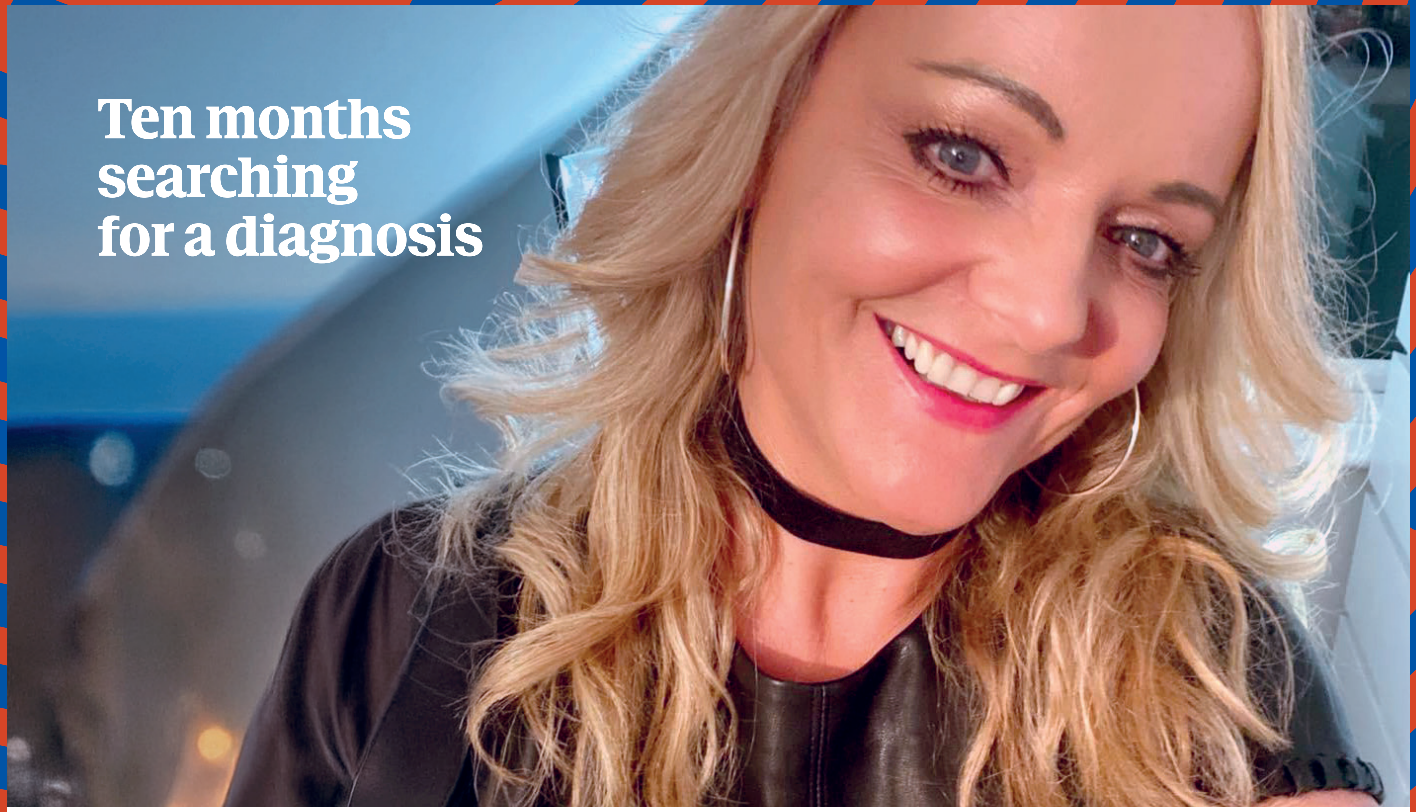
### We will:

- Address a major priority of people with MND, by understanding why some people's symptoms progress so much faster than others and learning to predict how a person's disease will develop.
- Enable large population studies to identify blood markers and clinical signs that can pick up MND in its very earliest stages, so it is possible to create better diagnostic tests.
- Find ways of identifying specific subtypes of sporadic MND and using this to personalise treatment.
- Develop better tests for MND – combining blood and imaging markers and AI tools to diagnose the disease earlier and more precisely, and accurately predict prognosis.

### What this will mean:

- New tests will help to diagnose people with MND faster and earlier, paving the way for more effective treatment, or even prevention.
- People with MND will get more information about their specific disease and how they are likely to progress – helping tailor their treatment and anticipate their future needs.

# Ten months searching for a diagnosis



**Jaki Jackson, like many people who are eventually diagnosed with MND, had to endure many months of invasive testing and confusing messages before doctors could tell her what was causing her symptoms.**

Jaki first noticed something was wrong when her handwriting started looking messier than usual, but her GP thought she had carpal tunnel syndrome. Two months later she got married – and was shocked to realise she could no longer sign her name.

Over the next eight months, she went through numerous tests and scans, as doctors ruled out various other possibilities and narrowed down her diagnosis to MND. Unfortunately, there is no definitive test for MND, so diagnosis is often a process of elimination. But eventually, she was told that had been a false positive, which meant she must have MND.

The MND Association is searching for tests to give people like Jaki a diagnosis much faster – sparing them the mental strain of many months or even years or testing, and allowing treatment to be started earlier.

> **“Nothing was straightforward. It’s crazy to think I was hoping for cancer! Lots of scans and tests were done and they had to come off the fence some time. Being diagnosed with MND had a massive impact on not only me but everyone involved in my life.”**
>
> **Jaki Jackson**

# Discover

## New treatments for everybody

**There have been advances towards treatments for people with some inherited types of MND – now we need to give hope to people with the sporadic form of the disease.**

**We’ve seen promising developments through our existing investments in large-scale programmes including EXPERT-ALS, MND-SMART and the UK MND Research Institute. So we’ll continue to invest in large-scale collaborations to create innovative new treatments. This will also give many more people the opportunity to join cutting-edge clinical trials.**

### We will:

- Find ways of targeting critical underlying mechanisms in MND to create exciting new approaches to treatment – with a particular focus on sporadic disease.
- Invest in the best academic drug discovery – funding researchers to validate drug targets, discover potential treatments and take them further down the pipeline towards people with MND.
- Bring together academic researchers and companies to form partnerships that focus on developing new treatments, potentially including seed funding for biotech companies.
- Give many more people with MND the chance to join a clinical trial – building on our research nurse network to help set up, co-ordinate and recruit people to trials across the UK.
- Support infrastructure for innovative platform trials, to allow new treatments to be tested rapidly against a single control group.

### What this will mean:

- People with all forms of MND will have hope for the future through research to identify the most promising drug targets and discover new treatments.
- A vibrant, collaborative ecosystem of academic researchers, companies, funders and people with MND will be developing exciting new MND treatments and taking them into clinical trials.
- Many more people with MND will have the opportunity to join a clinical trial, wherever they live in the UK.

# Searching for new treatments for sporadic MND

**Professor Pietro Fratta is leading pioneering research to create a new type of treatment.**

Pietro's research aims to target a mechanism found in 97% of cases of MND, in which a critical protein called TDP-43 clumps together in cells. These clumps have damaging effects on the ways other proteins are made. Pietro's team has found one such effect is an RNA processing error reducing the activity of a gene called UNC13A which is needed to keep neurones healthy.

Pietro is now using cutting-edge genetic therapies to correct the errors in gene processing. These therapies have already been used to create new treatments for some inherited forms of MND – researchers now believe rescuing UNC13A could offer hope to people with the sporadic form of the disease.

His work has been supported by the Translational Research Fund – a joint initiative between the MND Association, My Name'5 Doddie Foundation and LifeArc, designed to take new MND treatments into clinical trials as quickly as possible.

> **"It has been exciting to see that we're able to move from identifying a molecular alteration to developing a strategy to correct it and planning clinical trials in less than five years. This is such positive news generally for translational research in MND."**
>
> **Pietro Fratta, Professor of Cellular and Molecular Neuroscience, University College London**

# Innovation

## Innovations in healthcare

**Living with MND is challenging. People with MND experience a wide range of physical symptoms which worsen over time, communication difficulties, emotional challenges, and often behavioural and cognitive change.**

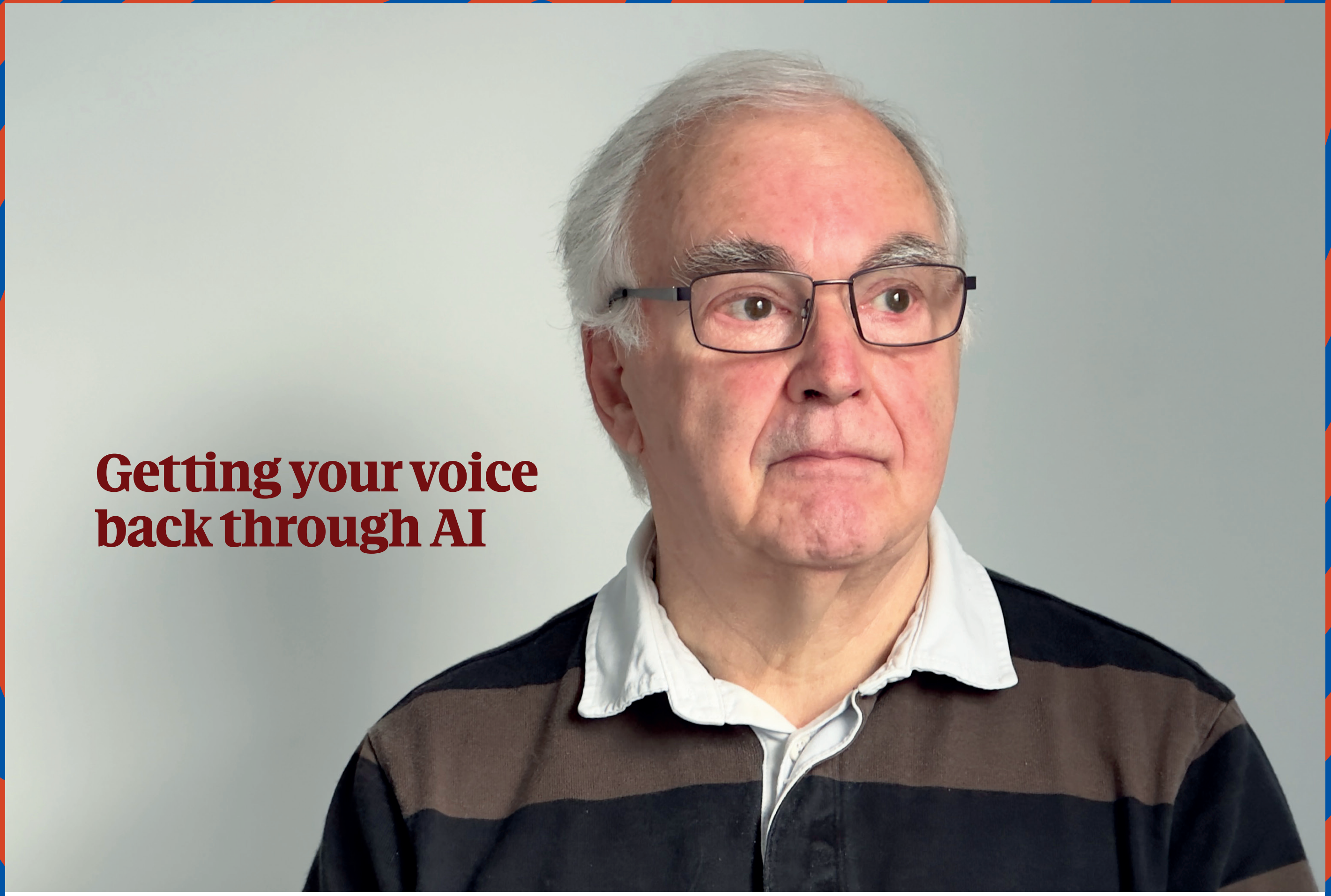
**Through research we must improve the care people with MND receive to give them the best possible quality of life. People with MND must be able to benefit from new technologies that can help maintain their independence, so we'll be assessing what's available and addressing the gaps.**

### We will:

- Use our funding for healthcare research to target the priorities of people with MND – such as fatigue, muscle weakness, psychological care and support for families and carers.
- Bridge the gap between research and healthcare by making sure health research projects are designed with people with MND in mind, and that positive developments are then widely implemented.
- Assess available assistive technologies, provide people with MND and health and social care professionals with evidence for which work best, and identify gaps where new solutions are needed.
- Stimulate development of innovative new solutions by bringing together innovators, people with MND and health professionals, and supporting the most promising ideas with funding.

### What this will mean:

- We'll improve the quality of life of people with MND and their families, by providing stronger, evidence-based care.
- People with MND and health and social care professionals will have better information about the most effective assistive technologies, and the chance to shape new solutions to every day problems.



## Getting your voice back through AI

**Mike Willis has used cutting-edge AI technology to clone his old voice after MND robbed him of the ability to speak.**

Mike, a 71-year-old grandfather from Cambridge, has always been an avid user of technology. He was desperate to find new digital ways to get his voice back.
He started by using voice banking technology that drew from a video he had recorded in the early stages of his disease. He wasn't satisfied with the results though, as he didn't have enough of his voice recorded for the technology to produce a realistic replica.

Instead, Mike turned to an innovative new AI approach pioneered by the company ElevenLabs. This technology was able to clone his voice from just a 90-second video clip, and accurately recapture his tone, accent and intonation. He can now create natural-sounding speech simply by entering text into an app.

> **"I can no longer walk without aids, but the really horrible bit is losing my speech. Even my wife has a hard time understanding me now. As someone who talked for a living it's really hard. But now I have an AI voice clone that sounds just like me, and I'm able to give presentations again!"**
>
> **Mike Willis**

# How we'll drive progress

## Involve people with MND

We will involve people affected by MND in shaping decisions related to our research activities – from understanding people's needs and how they could be addressed through research, to shaping new ideas for research projects and advising on funding applications.

### We will:

- Engage with a diverse range of people with MND – working with care centres and networks, and aiming to reach groups not typically involved in research, including those who are progressing rapidly.
- Develop an inclusive online community of people affected by MND, who can feed their own experiences into our new research initiatives.
- Involve people affected by MND in our committees for reviewing grant applications, to ensure our research funding meets the needs of our community and has real impact.
- Ensure people with MND can shape the wider research agenda, by setting clear expectations around how researchers should involve them in funding applications and delivery of their research, and sharing the views of our community with external organisations.

## Back the brightest minds

To deliver our strategy we need to work with talented scientists and clinicians doing world-leading research into MND. We will nurture a vibrant and diverse research community by attracting and retaining the best scientific minds in MND, and enhancing support for researchers early in their careers.

### We will:

- Fund scientific PhD students and postdoctoral researchers through new institutional training awards to help them develop their careers in MND research, and work with partners to extend support to clinical PhDs.
- Support scientific and clinical fellowships to help the best researchers continue their careers in MND, and work with other charities and public funders to increase the amount of money available.
- Introduce new grant awards to help researchers who are building their careers in MND to gain funding for early-stage, high-risk ideas which have the potential to have a major impact.

## Collaborate and partner

We can't transform the lives of people with MND alone. We will only deliver better diagnosis, treatment and care for people with MND by working in collaboration with the wider research community, in the UK and internationally. We will play a convening role by bringing together academic researchers, companies and funders to focus on exciting areas of science and to develop new partnerships. By pooling resources with other charities and funders, we will accelerate progress and increase our impact.

### We will:

- Build on our global role as organiser of the International Symposium on ALS/MND by facilitating connections and developing platforms for academic researchers and companies, and directing their focus at promising new areas of research.
- Work collaboratively with academia and industry in the UK and internationally to develop new partnerships that address particular unmet needs in MND.
- Explore opportunities to work with other charities in the UK and internationally, to jointly fund research initiatives or training programmes.
- Communicate about research findings with people affected by MND and the research community, so that there is broad awareness of the latest advances.

## Working with the UK MND Research Institute

**We're proud of the role we played in campaigning for and helping to fund the UK MND Research Institute.**

This network of MND centres is bringing together scientists, clinicians and people with MND across the UK to carry out collaborative research and accelerate the search for a cure.

We will continue our commitment to work closely with the UK MND Research Institute. We will draw from the expertise of its world-leading scientists and clinicians, and will work through its leadership structures to co-ordinate large-scale initiatives. Together we will work to identify the most promising drug targets and drive discovery of tomorrow's treatments.

We will collaborate to collect data from people with MND in a register, and on innovative platform trials to give a rapid initial assessment of new drugs.

## Offering a prize for AI-driven innovation

**Over the next five years, an exciting international prize will drive the use of AI to find the most promising new targets for future MND treatments.**

We are the principal funder of the £7.5m Longitude Prize on ALS, working in partnership with a social enterprise called Challenge Works which specialises in the use of prizes to drive innovation. The prize aims to motivate the brightest minds and spark fresh thinking to bring AI to the fight against MND.

Teams will compete over several rounds using AI to identify drug targets, select the most promising for further development and demonstrate how that could lead to treatments.

The teams in each round with the very best ideas will win money to be used for further development work. The team that provides the strongest evidence that it's found a new way to treat MND will scoop the first prize of £1m.

The Longitude Prize on ALS, and other new prizes like it, can stimulate a new wave of innovation driven by AI and the latest data science.

## Harness data and technology

AI and other technologies offer huge opportunities to analyse different sources of data about MND and make sense of the disease's complexity. We will help realise those opportunities by collating information about people with MND within secure platforms, and working with partners to make data easily available to researchers. We want to use technology to connect and analyse huge and complex datasets, so we can understand causes, find ways of diagnosing more precisely, and spot exciting new avenues for treatment.

### We will:

- Encourage researchers to collect more data on people with MND, and work with partners to link it into established studies and datasets.
- Collaborate with academic and commercial partners to expand the MND Register and make data on MND available on secure platforms, so it can be easily accessed by researchers.
- Support early-career researchers to develop data science skills through data-focused PhD studentships or fellowships, or by supporting prizes for innovative use of AI in MND research.

# How we'll fund research

**This strategy must be transformative for people with MND. That means doing science differently. We will fund research on a greater scale and with the needs of people with MND always in mind – driving collaborative efforts to solve the big problems in MND research.**

**MND is a complex and challenging disease to understand and treat, and only by using our funding to stimulate innovation and risk-taking will we make a difference for our community. So we're changing the way we fund research.**

## Larger-scale, collaborative projects

The biggest leaps forward often come at the boundaries between fields, so we will offer large-scale funding to support collaborations between research teams with different skills and perspectives. We will team up discovery scientists with colleagues who have expertise in creating new therapeutic strategies – maximising the chance that scientific discoveries will have an impact for people with MND.

## Challenging researchers to solve big problems

We will be more proactive in the way we fund research – directing the efforts of researchers at overcoming the biggest hurdles we face in MND. We will set researchers specific challenges – such as finding early markers of disease, developing better models of sporadic MND or identifying the most promising treatment strategies – and offer funding for those who propose the most imaginative approaches.

## Training centres to support the brightest minds

We will strengthen our training programmes for early career researchers, to help them to do the best science possible and to stay within MND research. We will support PhD students and new postdoctoral researchers as part of large grants for training centres, rather than through standalone projects. These centres will provide early career researchers with greater strategic direction for their science, support to develop their skills, and options to progress their careers within the MND field.

## Backing the best ideas early

We need to take risks if we're to find innovative new ways of treating MND, and part of that is about supporting pioneering ideas for research early on, to get them off the ground. We will offer researchers small awards to take forward high-risk approaches to research and treatment. Our funding will help researchers produce the data they need to attract larger investments, helping them build innovative research programmes and develop their careers.

## Seed funding for small companies

Small technology and biotech companies are vital innovators. We will give small awards to medical technology companies to help them develop ways of supporting people with MND and attract investment to take them to the market. We will also consider supporting biotech companies which are developing highly innovative ways of testing for or treating MND, to increase the chances that these reach people with the disease.

# Measuring success

**We're introducing new ways to measure the impact our investment in research has over the next five years.**

Our goal is to improve the lives of people affected by MND. And we must do that quickly because people with MND don't have time to wait. At the same time, we must be realistic about the time research can take. While we are committed to making tangible improvements in diagnosis, treatment and care within the five years of our strategy, many of our ambitions will take longer to achieve.

We will use a range of different measures to keep track of our progress – some looking at the difference we are making today to the quality of life of people with MND, and others at milestones on the road to transforming tomorrow's treatment for everyone.

## Examples include:

- The number of new disease mechanisms we discover and validate as targets for future MND treatments.
- The number of disease markers we identify which can form part of diagnostic, predictive and prognostic tests for MND.
- The number of people with MND able to join a cutting-edge clinical trial of a new treatment.
- The number of the MND community's priority questions for healthcare research which we address through research to improve quality of life.

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**Every day we support people affected
by Motor Neurone Disease.
Because with MND, every day matters.**