

# Accelerating research for a treatment for motor neurone disease (MND)



Setting out the case for UK Government to invest in an innovative solution for this devastating neurological condition



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This brief is the result of a collaboration between the principal MND charities in the UK, leading researchers and members of the MND community. It sets out the case for government investment into a significant unmet need.

There are currently no effective treatments or cure for this devastating terminal disease that is imposing an increasing burden on families and health and social care services globally [1]. To rectify this, we seek your support in order to build on the current exciting developments in research.

Our proposed solution is innovative and consistent with the UK government's aims to improve public health and for the UK to be a "science superpower" [2].



## The humanitarian case for investing in MND research

### *A brutal diagnosis – a relentless killer within the UK population*

A diagnosis of MND is a death sentence. Patients will lose in no set order; the ability to walk, use their arms and hands, speak, eat and ultimately breathe. Some become completely paralysed and horrifically trapped in their own bodies. Almost uniquely, for a terminal disease, from diagnosis to death, MND brings increasingly onerous and costly care requirements, sometimes for several years.

MND is the most common neurodegenerative disease of mid-life, and many younger people are also affected.

#### **Key facts:**

- Progression is rapid with 1/3 of people dying within a year of diagnosis.
- Because people die so quickly, only 5000 live with the disease at any one time.
- But MND is not rare – 1 in every 300 people, across all communities, will develop MND in their lifetime.
- It is the most feared diagnosis among healthcare professionals and the disease for which the highest percentage of patients seek assisted dying [3].
- About 200,000 of the current UK population will die of MND, unless effective treatments are found.



## MND research funding: the challenges and opportunities

### *The Ice Bucket Challenge demonstrates the need to reach a 'threshold' in funding levels to finance groundbreaking advances*

#### **The challenges**

MND research faces significant funding challenges. Among them are the common misconceptions that MND is 'rare' and a 'lost cause' with no hope of a cure. This is combined with a lack of awareness among policy makers in the health sector as to the real nature of the disease.

Such misconceptions create a barrier to reaching the crucial threshold in funding that will attract greater commercial investment to enable an acceleration in finding new treatments.

The 2014 Ice Bucket Challenge illustrates the dramatic impact that improved funding can achieve. The UK public donated over £7m, the majority channeled into research. It kick-started multiple advances which have led directly to potential new treatments. We now face the challenge of keeping the momentum going to ensure recent progress does not go to waste. We cannot realistically expect to achieve such a level of donations again.

A further challenge is that making a precise financial case for improved funding is complex. While it is clear that MND imposes a huge financial burden on health and social care services, on the provision of benefits and on patients' families, it is likely, given the numbers alive at any one time, that the total burden will seem small in comparison to other conditions. Research has shown, however, that keeping patients stable earlier in the disease, through the development of treatments and earlier diagnosis, is economically very attractive [4] as the costs for late stage MND can be more than 9 times as expensive as the early stages.

#### **The opportunities**

The potential upside for neurological disease research in the UK cannot be underestimated. Our scientists are already establishing world-leading drug discovery processes and trial platform foundations. If a funding threshold is established, the UK will consolidate its position and attract greater investment from major global pharmaceutical companies which will also become customers of a new national infrastructure. Diseases such as MND, with their unmet needs, are an important driver for investments in orphan drugs. In 2017 the worldwide market for rare disease drug sales was \$124 bn. By 2022 it is expected to be \$209 bn [5].

Perhaps, most importantly, MND is ready to become the 'Model' for Neurodegenerative disease clinical trials and treatment approaches.

## Current funding environment

UK Government funding for targeted MND research has plateaued at c£3m annually, the vast majority from the BEIS (via the MRC). It includes extremely limited funding from the DHSC (via the NIHR). [6,7] Charitable funding consistently exceeds this by a wide margin.

However, with charitable income likely to be reduced by 20% over the next few years due to the impact of COVID-19, and the Ice Bucket Challenge funds now having been committed, the stark reality is that funding is insufficient to keep the momentum behind current promising projects at a critical time when scientific progress is rapid.

Now is the time for an injection of government funding that will lead not only to accelerated discovery of treatments, a significant reduction in healthcare, social care and benefit costs, but also to the emergence of the UK as the leading player in the extremely valuable global field of neurological disease research.



## This is a pivotal moment for MND research.

### Why invest in MND research now?

### The UK is central to global advances - many building blocks are in place

Despite limited investment, MND is one of the fastest moving sectors in UK health and biomedical research. Current trials hold real promise of a licensed treatment in just 2-3 years for some forms of MND with the appropriate level of investment.

Counterintuitively, MND, with its relatively low prevalence, is incredibly valuable to research into the more common neurodegenerative diseases such as the dementias. The very rapidity of MND progression makes it easier to pioneer and trial treatments in a realistic time frame.

There has never been a better opportunity to harness unprecedented advances in science, medicine and technology that will not only make MND a treatable condition but also accelerate treatment strategies across all neurological diseases including Alzheimer's disease and other dementias.

## The solution - The MND Research Institute (MNDRI)

### A virtual research institute to seize this pivotal moment and catalyse the cure for MND

The MNDRI will provide the efficient and robust infrastructure needed for accelerating treatments to market. It will bring together advances in both clinical and core science research and further develop the already highly collaborative MND research community.

The MNDRI will be laser-focused on MND, taking research from the clinic, to the lab and back to the clinic again. Governance and leadership will be provided through a partnership between people living with MND, government, researchers, MND charities, and the pharmaceutical and biotechnology sectors. The MNDRI will be fully aligned with the UK government's desire to embed personalised medicine within the UK healthcare system.

Many building blocks are already in place, such as the MND Register, MND biobanks, the beginnings of flexible trial techniques, stem cell models and world leading biomarker research. All of these are in the UK.

With new dedicated funding the MNDRI will advance and optimise translational research and clinical trials by:

- **Creating a drug discovery and development programme** which will deliver a continuous stream of new targeted medicines for testing in clinical trials.
- **Developing a sustainable UK MND trials platform** to deliver rapid and efficient clinical trials that will attract pharmaceutical clients with built-in, trusted and proven recruitment processes.
- **Implementing a rigorous clinical research programme** to maximise knowledge generated from each trial resulting in continuous improvement and learning regardless of the trial outcome.

Under the governance of an MNDRI this will lead to more definitive outcomes achieved within considerably reduced time frames and maximise return on invested research funds.

Reflecting the global vision of a world free from MND, the MNDRI will become the flagship of expertise in the UK and globally, enabling it to attract and retain the very best scientists.

## What is needed from Government?

### *A significant cash injection to establish the MND Research Institute and consolidate the UK's position as a global leader in neurological disease research*

We ask the UK government to consider a new investment of £50m over 5 years (£10m a year) dedicated to MND research to fully establish and operate the MNDRI and continue the progress made possible by the combined investment of the MND Association, MND Scotland, and the My Name's Doddie Foundation, together with the world leading expertise of UK scientists.

Funding from the UK government will be combined with ongoing commitments from the MND charities and other supporters.

To put our ask into context...

£50 million over 5 years is the equivalent of just £2000 for each person living with MND per year. The cost of Personal Independence Payment (PIP) alone, just one benefit patients are entitled to, is typically £7000 per year per patient.

We recognise this is a significant request. However, with major breakthroughs in sight, only increased funding will sustain current progress towards the end of our journey – a world free from MND.

*"If you had asked me 25 years ago if there would ever be a cure for MND, I would have said no. But now, in the next few years, I can honestly say that we will probably be able to stop MND in some forms of the disease"*

#### **Ammar Al-Chalabi**

Professor of Neurology and Complex Disease Genetics, Kings College, London



*"Co-ordination of all of the UK's efforts by an MND Research Institute, bringing together and building on the work of all of the academic, NHS, disease charity, SME and biopharma partners, would not only massively accelerate the work towards a cure for MND but would also be a beacon establishing the UK's cutting-edge biomedical science capabilities firmly on the world stage."*

Dr Steve Gardner, CEO  
**PrecisionLife Ltd**

*"Co-ordination of UK MND treatment sites, the MND Register, MND biobanks, academic researchers, patient advocates, and industry partners such as ourselves is a complex and labor-intensive project that requires focused leadership and dedicated resources. We therefore enthusiastically endorse the support for and development of a UK MNDRI."*

Thomas I Cochrane MD, MBA  
Medical Director and Global Medical Lead, ALS/MND,  
**Biogen**

*"Lilly is pleased to support this vitally important initiative in MND research. Lilly is fully committed to the search for treatments and a cure for this devastating disease. The MNDRI will provide a brilliant platform for the whole motor neurone disease research community to enable treatments to come to the NHS as quickly as possible for the benefit of people affected by MND."*

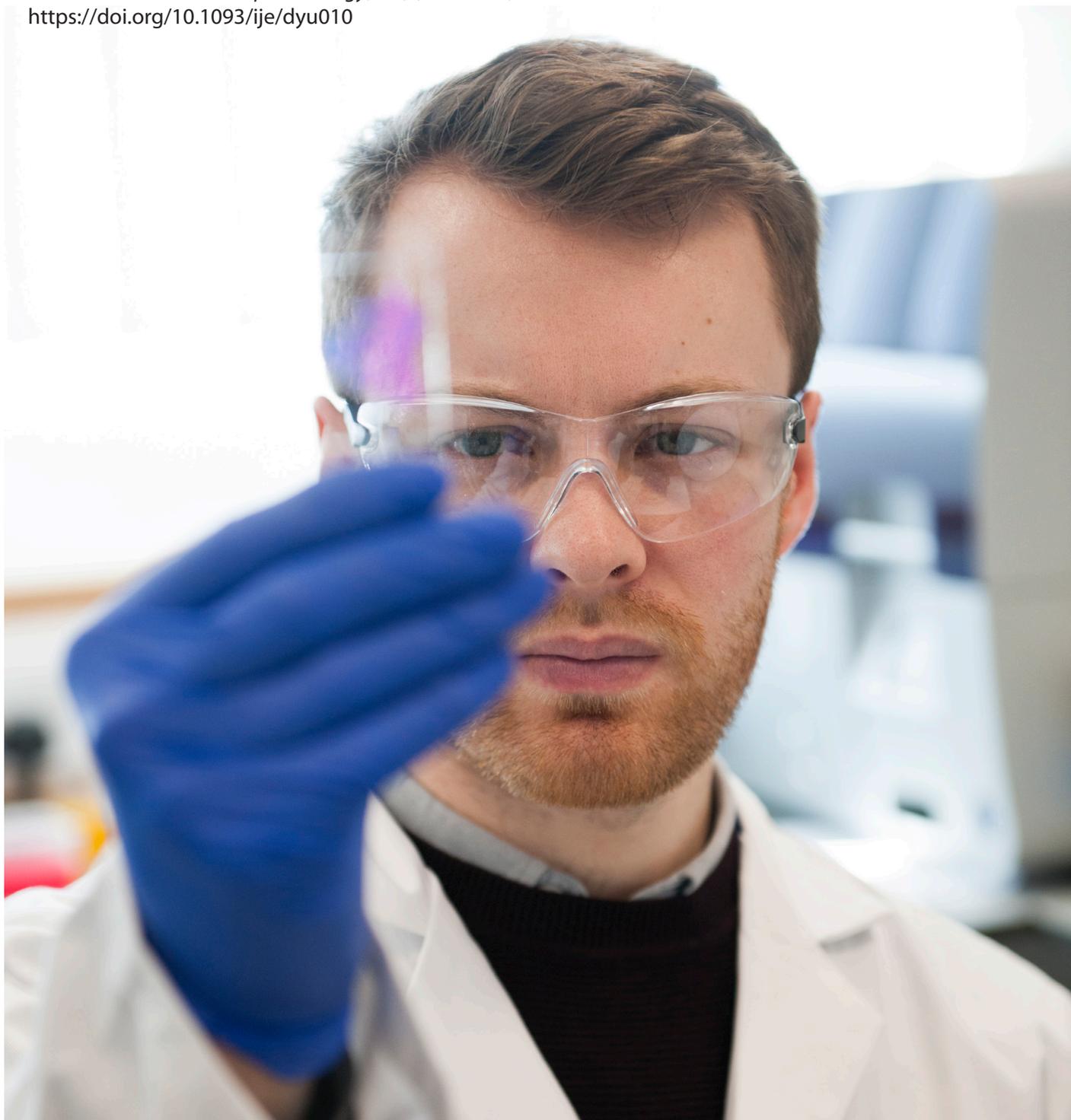
**Lilly UK**

*"We would highly recommend the full implementation of the MNDRI... It will bring together advances in both clinical and core science research and further develop the already highly collaborative MND research community"*

**Novartis Pharma**

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