King's College Hospital NHS Foundation Trust

NIHR | Maudsley Biomedical Research Centre

Motor neuron disease

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All Party Parliamentary Group on MND

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King’s College London

- Professor of Neurology and Complex Disease Genetics at King’s College London
- Consultant Neurologist at King’s College Hospital
- Director, King’s MND Care and Research Centre
  - Supported by MND Association
- Head of Department of Basic and Clinical Neuroscience, KCL

MND Association

- MND Association Biomedical Research Advisory Panel member
- Incoming Programme Lead for International Symposium on ALS/MND
- Lead for 3 national or international MND Association flagship projects
- MND Association clinical adviser for COVID-19 guidance for people with MND
  - Also representing patients, patient associations and MND specialists to CMO's Office regarding Extreme Shielding

Key MND facts

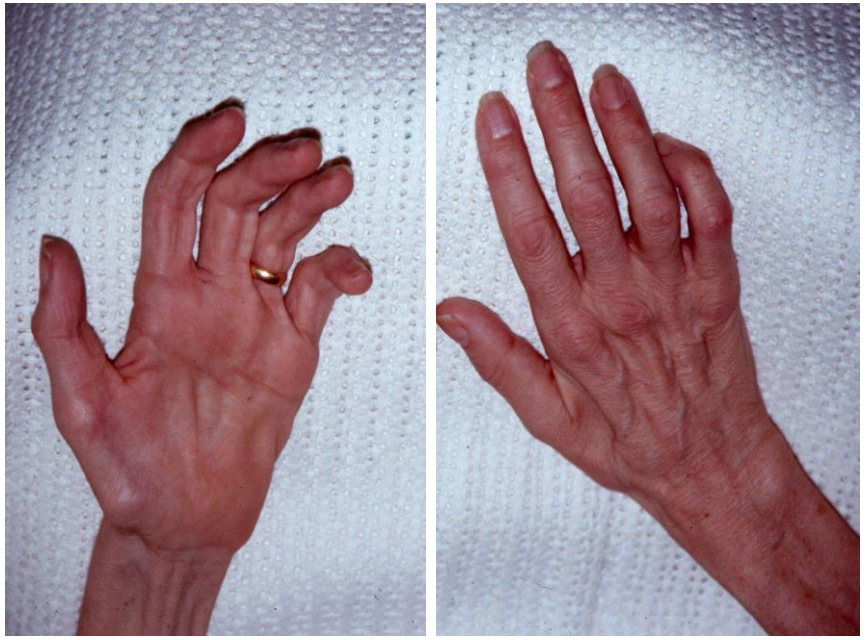
- MND will kill 1 in every 300 people
  - Would be as common as MS
  - Because life expectancy is short, appears rare
- Most common neurodegenerative disease of mid-life
  - Kills people in their prime
- Burden emotionally, socially, economically is huge
  - Most common reason to seek assisted suicide
  - Most feared diagnosis
  - Cost *every 3 months* is
    - £1096 in King's stage 1
    - £3311 in King's stage 4
- We need to find a cure

Doddie Weir, Stephen Darby, Rob Burrow

Clinical trials

- Studies to find new treatments for MND - eg
  - Immune therapy
  - Muscle strengthening
  - Gene therapies
    - Directly modify gene
    - Treat a specific gene product
- International collaborations
  - UK a world-leader

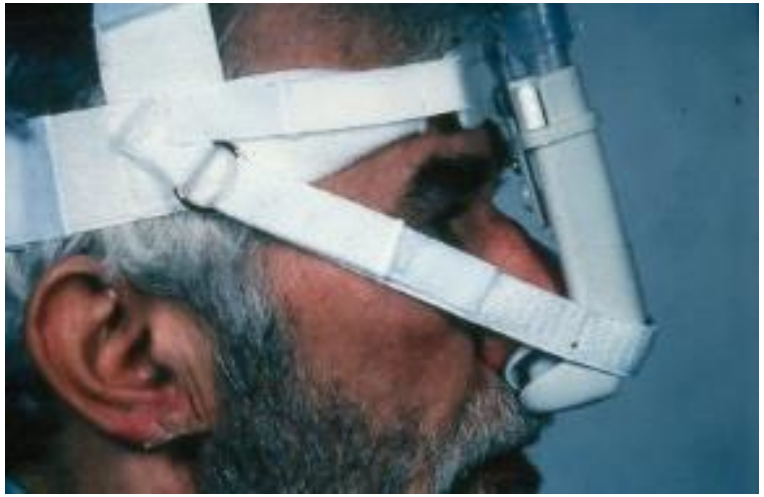
- Essential – MND has no cure and no effective treatment

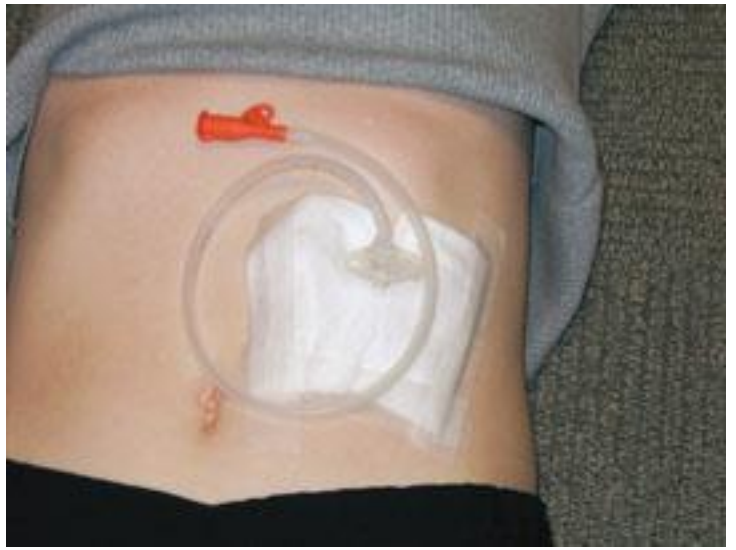


Issues

- Funding climate
  - Funding almost all charitable (MND Association, My Name’5 Doddie Foundation) or commercial

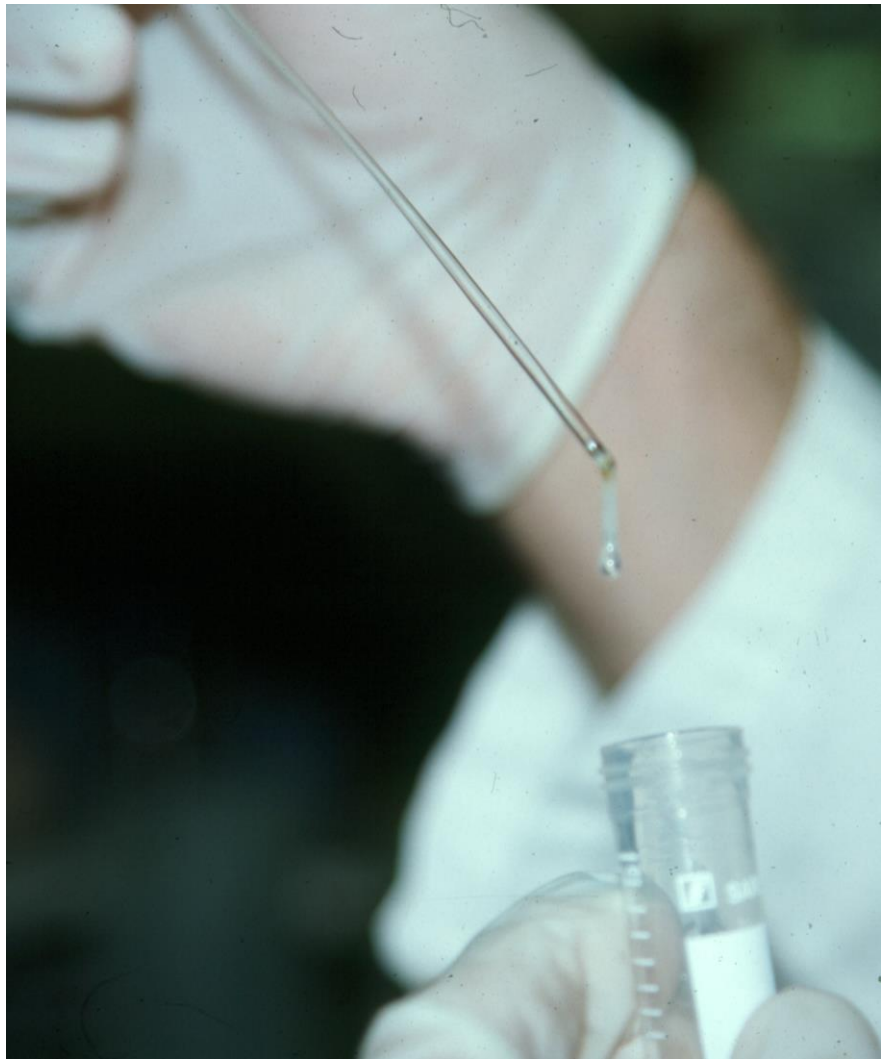
- Pandemic
  - Existing trials paused
    - Safety monitoring difficult
    - Sample collection difficult
    - Breathing assessment impossible
  - New trials
    - Initial setup possible
    - Recruitment paused





Genetics

- Studies to find the cause of MND
  - Genetic contribution to risk
  - 10% have a family history
- International collaboration
  - UK a world leader
- Project MinE
  - Largest single disease sequencing project in the world
  - 12,000 whole genomes so far
  - Cost > £12 million
  - 20 countries
    - Led by UK and NL
- Essential - for new treatment development



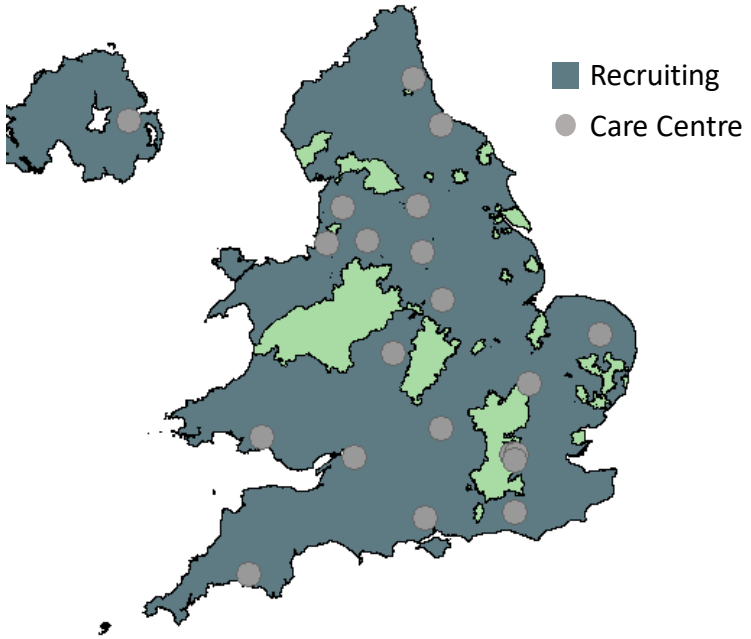
Issues

- Funding climate
  - Funding all charitable (MND Association in UK)
- Pandemic
  - Sample collection paused
  - Sample analysis
    - Laboratory work paused
    - Computer work continues
  - Gene-hunting continues
    - Statistical tests

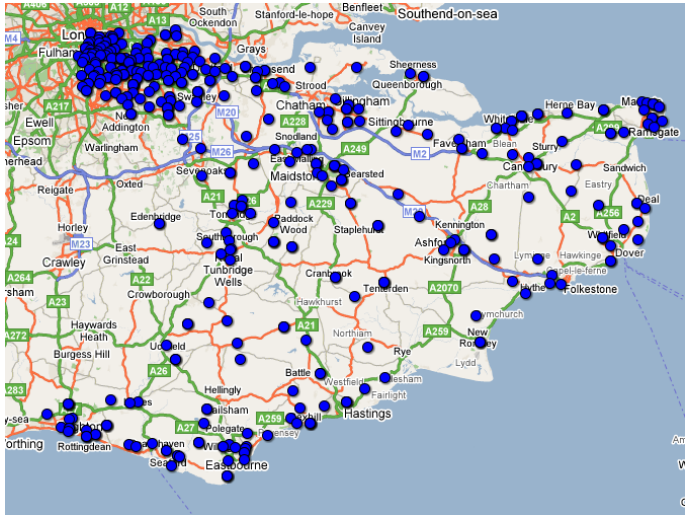
MND Register

- Register of every person with MND in England, Wales and Northern Ireland
  - Currently at about 70% coverage and rising
- Includes clinical information
- Integrates with other research
- Essential - allows mapping of care provision vs care need

Care Centre coverage

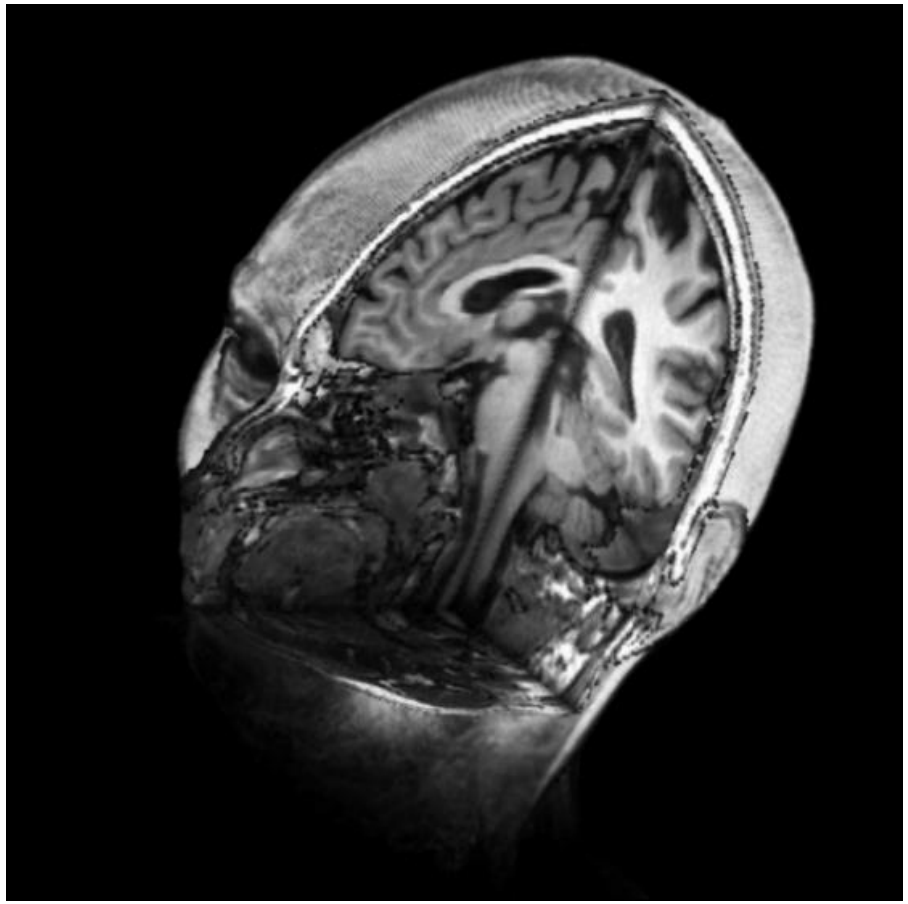


Newly diagnosed people with MND in South East



Issues

- Funding climate
  - Funding all charitable (MND Association, Betty Messenger Foundation)
- Pandemic
  - Collection of information from hospitals paused
    - Research nurses redeployed
    - NHS Trusts focussed on COVID-19 research for approvals
  - Cannot consent patients directly
    - Self-registration is the only method
  - Ethics Committees prioritizing COVID-19 research



How can government help research?

- UK is world-leading in MND research despite funding climate
  - Largely because of effectiveness of MND Association
  - Strong collaborations worldwide
  - Charitable income threatened by pandemic

- Mitigate effects of pandemic on future funding and existing projects
- Generally improve funding landscape
  - Coordinate with charities and international funders
- Reduce bureaucracy

mnda motor neurone disease association

MND Scotland Supporting people affected by Motor Neurone Disease

ALS ASSOCIATION

MY NAME'5 DODDIE foundation

MRC Medical Research Council

NIHR | National Institute for Health Research

THE FRAMEWORK PROGRAMME FOR RESEARCH AND INNOVATION HORIZON 2020

JPND research EU Joint Programme – Neurodegenerative Disease Research

Take home messages

- MND is a devastating diagnosis
  - Effective treatment is urgently needed
- UK is world-leading even with the existing funding barriers
  - Situation worsened by pandemic

- Government support is essential