Motor Neurone Disease / Amyotrophic Lateral Sclerosis

Progressive neurodegeneration, predominately motor neurons

A third die within a year of diagnosis, half within two years

Incidence 2/100,000/yr
Prevalence 6/100,000

Lifetime risk 1 in 300 in UK population
Funding Research

Supporting 85 studies as of 31 Dec 2020
Portfolio commitment £14.6M
Facilitating Communication

- 31st International Symposium on ALS/MND (Virtual)
- 9-11 Dec 2020
- 1,800 registered delegates
- 48 countries
Building Partnerships

Guidelines for use of non-invasive ventilation in MND under development

15,000 ALS genomes
20,000 Control genomes
International initiative to sequence the genomes of:
15,000 sporadic ALS cases
7,500 controls
MND Association pledged to sequence up to 2,000 samples
Completion of UK sequencing 2018?
Switch emphasis to data storage and bioinformatics
This may be a turning point in treating neurodegenerative diseases

Success in trials for Huntington’s and Spinal Muscular Atrophy, raises hopes that diseases such as Alzheimer’s and ALS could be tackled using a new class of drugs.
Unprecedented growth in global MND research

Diagram showing relationships between genes:

- VCP
- C9orf72
- SQSTM1
- TBK1
- SOD1
- UBQLN2
- TARDBP
- FUS
- PEN1
The ‘model’ neurodegenerative disease for therapy development

Consistent progression

Prognostic accuracy

Clinical biomarkers

Novel trial design
UK MND Research Institute

Basic Research and Disease Linkage

Discovery – in-lab in vitro/in vivo testing

Pre-clinical Development (regulatory/GLP/GMP) (regulatory tox etc.)

Early Clinical Trials

Late Clinical Trials

Drug Discovery/Development → Sustainable Trials Platform

Clinical Research Programme