Delivering Neurodegenerative Diseases Research in the UK

Research in neurodegenerative disease is fully embedded within NHS clinical practice. We have well-established, highly regarded clinical services for patients with neurodegenerative diseases and their carers, with clinical communities having research delivery as a key priority, such as the Motor Neurone Disease Clinical Studies Group, and the Parkinson's Excellence Network. We have a network of study sites across the UK, with study delivery expertise in all three major disease areas – Parkinson's disease, Huntington's disease and Motor neurone disease, encompassing the whole range of study complexity.

The Clinical Research Network provides researchers with the practical support they need to make research happen in our National Health Service (the NHS). As well as providing research delivery staff, we also bring together communities of clinical practice to provide national networks of research expertise. Neurodegeneration, which is part of the Dementia and Neurodegenerative Diseases (DeNDRoN) Specialty, is one of 30 such communities and is made up of leading research-interested clinicians and practitioners at both national and local levels. Our job is to ensure that the studies

### Expertise

All regions in England and the devolved nations have representation in our specialty leadership group, which includes nationally and internationally recognised key opinion leaders who have acted as Chief Investigators for UK and international multi-centre studies. Our industry lead for commercial studies, together with our stratification lead, provides strategic direction as well as an operational link between industry, the NIHR and researchers, ensuring that as a nation we have specialist sites who are trial-ready for the innovative therapeutics being developed.

We take a multidisciplinary approach to developing the next generation of researchers through training and mentorship, building skills amongst a broad range of professionals so that capacity for study delivery sits well within clinical care pathways.

### Access to patients

Our specialty provides access to research for the majority of patients with neurodegenerative conditions attending NHS services in the UK. The cohort study initiatives supported by the major charities and investigators (such as PROBAND and DISCOVERY in Parkinson's disease, PROSPECT for atypical parkinsonism, ENROLL-HD, TRACK-HD and TRACKON-HD in Huntington disease, and the MND National Register) ensure that we have trial-ready potential participants who are well phenotyped, and also, increasingly, genotyped.

Regional research registers also facilitate patient access (such as MotoDeNDRoN in MND and PRO-DeNDRoN in PD). We also work closely with charities to identify access and recruitment needs and solutions, ensuring research access is as inclusive as possible.
High quality study delivery

Year on year, the number of patients recruited into neurodegenerative disease research in the UK grows. In 2017/18, over 30,000 participants were recruited into 250 dementias and neurodegeneration studies in 200 NHS trusts (86 per cent of active trusts).

We have a strong track record of delivering studies to time and target. We have expertise in the delivery of Phase II and III studies including multisite international randomised clinical trials. Our study sites are distributed across the UK, matching the patient population. We undertake feasibility using high quality data analytics, and increasingly sites are implementing data solutions for feasibility assessment. The UK neurodegenerative disease research portfolio is diverse. Expertise exists across all key domains; application of brain imaging; research into pathology and genetics; qualitative research including the development of person-centered care; and epidemiological work.

Collaboration

We have strong links with national charities such as Parkinson's UK, the Huntington's Disease Association and the Motor Neurone Disease Association, including PPI representation on our Specialty Group. These links facilitate patient involvement in research and provide important synergies with providing service excellence across the UK. Several studies on the CRN portfolio have been funded by these charities and, through the efforts of our Specialty Group, have recruited to time and target. We work collaboratively with pharma to deliver IIS, and support trainee networks to ensure development of the next generation of researchers.

Case studies

What the CRN uniquely offers is a collegiate research community, operating for the benefit of our patients, taking on new challenges in study delivery and sharing best practice and solutions.

Parkinson's disease:

Optipark was a commercial study which successfully recruited 43 per cent above the UK targets across 18 sites, with 16 out of the 18 reaching or exceeding their local targets. This study demonstrates the wide network of sites available in the UK to successfully deliver commercial studies.

Motor neurone disease:

233AS101 / SOD-1 This study was investigating a first-in-man novel therapeutic in a genetic MND subtype. It was delivered in a single centre, but relied on our national network to identify patients with the necessary specific genotype. This study over-recruited, demonstrating capability for recruiting to studies based on genotype and ability to deliver novel biological therapies.

Huntington's disease:

ISIS 443139 This ground-breaking study of intrathecally-delivered anti-sense oligonucleotide involved 4 UK sites, delivering 18 of the 46 global recruits. This demonstrates capability to adopt new therapeutic innovations and deliver a unique therapeutic using an unfamiliar delivery system, to share learning between sites across the network to ensure successful study delivery.