**IoN Scientific Advisory Board Jan 2017**

**Chair Hugh Perry**

<table>
<thead>
<tr>
<th>Name</th>
<th>Institution</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hugh Willison</td>
<td>(Glasgow)</td>
</tr>
<tr>
<td>Michael Sendtner</td>
<td>(University Würzburg)</td>
</tr>
<tr>
<td>Mark van Buchem</td>
<td>(Leiden University)</td>
</tr>
<tr>
<td>Phil Harding</td>
<td>(UCL Finance Director)</td>
</tr>
<tr>
<td>Tim Quine</td>
<td>(Exeter-education)</td>
</tr>
<tr>
<td>David Reynolds</td>
<td>(Alzheimer’s research UK)</td>
</tr>
</tbody>
</table>
Academic Vision - IoN aligned with UCL 2034
UCL Translational Neuroscience

- **Leading translational neuroscience centre in the world** translating discovery into experimental studies in patients to deliver new diagnostics and new therapies

- **Number one student destination** for educational programmes embedded in translational neuroscience research

- **Overcome fragmentation**; centre for connecting industry, commercial partners, academics and patients to accelerate translate research, internationally
DEPT REORGANISATION PART OF ION OPERATIONAL PLAN

Science  

Discovery science and bi-directional translational research

Patients

8 Departments, 1200 staff and students, £268m active grants

1. Neurodegenerative Diseases  
   Sarah Tabrizi & Nick Fox

2. UK Dementia Research Institute at UCL  
   Karen Duff

3. Clinical and Experimental Epilepsy  
   Ley Sander

4. Neuromuscular Diseases  
   Linda Greensmith

5. Neuroinflammation  
   Ken Smith

6. Clinical and Motor Neuroscience  
   Tony Schapira

7. Imaging Neuroscience  
   Cathy Price

8. Brain Repair and Rehabilitation  
   Dave Werring
<table>
<thead>
<tr>
<th>Funding Body</th>
<th>Principal Investigator(s)</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Medical Research Council</td>
<td>Pitceathly</td>
<td>£1.1m</td>
</tr>
<tr>
<td></td>
<td>Hanna, Reilly, Houlden</td>
<td>£3.66m</td>
</tr>
<tr>
<td></td>
<td>Lambert</td>
<td>£1.2m</td>
</tr>
<tr>
<td></td>
<td>Tabrizi, Bates</td>
<td>£1.5m</td>
</tr>
<tr>
<td></td>
<td>Busche</td>
<td>£1.6m</td>
</tr>
<tr>
<td></td>
<td>Hong</td>
<td>£1.8m</td>
</tr>
<tr>
<td></td>
<td>Fratta</td>
<td>£1.9m</td>
</tr>
<tr>
<td></td>
<td>Sleigh</td>
<td>£1.2m</td>
</tr>
<tr>
<td></td>
<td>Patani</td>
<td>£1.9m</td>
</tr>
<tr>
<td>British Heart Foundation</td>
<td>Werring</td>
<td>£2.1m</td>
</tr>
<tr>
<td>MDA (USA)</td>
<td>Reilly</td>
<td>$1.1m</td>
</tr>
<tr>
<td>NIHR</td>
<td>Ciccarelli</td>
<td>£1.8m</td>
</tr>
<tr>
<td></td>
<td>Schrag</td>
<td>£1.5m</td>
</tr>
<tr>
<td>Wellcome</td>
<td>Kullmann</td>
<td>£1.6m</td>
</tr>
<tr>
<td></td>
<td>Macguire</td>
<td>£2.3m</td>
</tr>
<tr>
<td></td>
<td>Rusakov</td>
<td>£2.7m</td>
</tr>
<tr>
<td></td>
<td>Parashkev</td>
<td>£4.16m</td>
</tr>
</tbody>
</table>
Outputs in 2018:
- 1613 papers (1 every 5h!)
- 22 book chapters
- 1 book

ISI world ranking for highly cited authors in Neuroscience and Behaviour: 5 in the top 100 / 4 in the top 50

68 publications in the top 50 highest impact journals:
Successful Quinquennial Review
£15M funding for 2020-25

- £6.5M investment from Dementia Discovery Fund into new biotech start-up
- Modulation of astrocyte function
(Some) research highlights 2018-19
Department of Imaging Neuroscience

Head Professor Cathy Price

Arrival of 7T scanner

May 31st, 2019
Scanning human brains, September 12th, 2019

Dominik Bach
New PI

Ray Dolan
Royal society Ferrier Medal

Steve Fleming
BPS Spearman Medal
Professor Tony Schapira- Head of Department

50th Anniversary of Reta Lila Weston Institute symposium, Attended by trustees and UCL Provost – an artwork from QSBB was presented by Provost

DCMN one of 5 centres in the world selected to train movement disorders Fellows by the Michael J. Fox Foundation and Edmond J. Safra Foundation

Prof Tom Warner
Director
Reta Lila Weston Institute

85 brains in 2018-19
Neuropathological progression of clinical Parkinson disease subtypes

Eduardo De Pablo-Fernández, Andrew J. Lees, Janice L. Holton and Thomas E. Warrer
Biallelic expansion of an intronic repeat in RFC1 is a common cause of late-onset ataxia

Andrea Cortese 1, 2, Roberto Simone 2, Roisin Sullivan 1, 11, Jana Vandrovova 1, 11, Huma Tariq 1, Wai Yan Yau 1, Jack Humphrey 1, Zane Jaunmuktane 2, Prasanth Sivakumar 1, James Polko 3, Muhammad Illyas 4, Eloise Tribollet 1, Pedro J. Tomaselli 5, Grazia Devigili 6, Ilaria Callegari 7, Maurizio Versino 7, 8, Vincenzo Salpietro 1, Stephanie Ethymiou 1, Diego Kaski 1, Nick W. Wood 1, Nadja S. Andrade 9, Elena Buglo 10, Adriana Rebelo 10, Alexander M. Rossor 1, Adolfo Bronstein 2, Pietro Fratta 1, Wilson J. Marques 6, Stephan Zühner 10, Mary M. Reilly 1, 12, Henry Houlden 1, 3, 12, *
Neurology Families per region  ION/NHNN >> rest of England!
INAUGURAL SYMPOSIUM SEPT 2019

Over 200 Attendees
(>30 waiting list)
- Representatives from Charities, MRC;
  Journal Editors; MHRA; NHS; 16 Industry Reps

- Across UK: London, Cambridge, Sheffield, Reading, Oxford, Midlands

- International: USA, Italy, Denmark, Netherlands

18 Speakers; 11 Sponsors

Organisers:
Lizzy Fisher
Debbie Hadley

Plenary Lecture:
J Paul Taylor
Howard Hughes Medical Institute; St Judes Hospital USA

Symposium Dinner:
Academy of Medical Sciences
Expressing worm receptors to cure epilepsy

Mechanisms underlying epilepsy related mortality

Biochemical autoregulatory gene therapy for focal epilepsy

The ventrolateral medulla and medullary raphe in sudden unexpected death in epilepsy
Gene therapy for refractory epilepsy

RESEARCH ARTICLE

Optogenetic and Potassium Channel Gene Therapy in a Rodent Model of Focal Neocortical Epilepsy

Robert C. Wykes, Joost H. Heeroma, Laura Mantoan, Kaiyu Zheng, Douglas C. MacDonald, Karl Deisseroth, Kevan S.Hashemi, Matthew C. Walker, Stephanie Schorge, Dimitri M. Kullmann

£2.2m MRC DPFS grant to initiate Phase 1/2a clinical trial

Kullmann, Matthews. Schorge
First measurements of extracellular glutamate and GABA during seizures

Marvin et al (Nature Methods 2019)
Information that defines an individual disease

Prof Ken Smith-Head

Major trial of statins to treat multiple sclerosis begins

The biggest ever trial for secondary progressive multiple sclerosis (SPMS) in the UK has begun under UCL research leadership.

Olga Ciccarelli
NIHR Professorship
Early imaging predictors of long-term outcomes in relapse-onset multiple sclerosis

Wallace J. Brownlee,1 Dan R. Altmann,1 Ferran Prados,1,3,4 Katherine A. Miszkiel,1 Arman Eshaghi,1 Claudia A.M. Gandini Wheeler-Kingshott,1,4,7 Frederik Barkhof,1,3,8,9 and Olga Ciccarelli1,3

Figure 1  Representative images of patients classified as CIS, relapsing-remitting multiple sclerosis (RRMS) and secondary progressive multiple sclerosis (SPMS) at 15 years. MRI scans obtained at baseline (the time of CIS), 1 year and 3 years.
Dept Brain Repair and Rehabilitation

Dave Werring Head

Timing of anticoagulation after recent ischaemic stroke in patients with atrial fibrillation

Lancet Neurol 2019

Cerebral microbleeds and stroke risk after ischaemic stroke or transient ischaemic attack: a pooled analysis of individual patient data from cohort studies

Lancet Neurol 2019

Early Onset Cerebral Amyloid Angiopathy following Childhood Exposure to Cadaveric Dura

ANN NEUROL 2019;85:284–290
Parashkev Nachev: £4.5 million Wellcome Innovations Grant: Programme for High-Dimensional Translation in Neurology (Stroke) Commenced 2019

Alex Leff and Jenny Crinion awarded £600,000 (National Brain Appeal) to set up a high-dose aphasia service Commenced 2019

Prof Leff talked about language after stroke on BBC Radio 4
QS Genetic Therapies Centre

- Antisense therapy, gene therapy, genome editing
- Dedicated Preclinical Genetic Therapy Centre
- Seamless with LWENC for clinical trials
- Positions UCL as “go-to” partner for therapeutic gene therapy programmes
HTT ASO Global Phase 3 trial (n=660) now started -Roche

5-year Senior Fellowship from CHDI Foundation to Ed Wild: £900,000

Sarah Tabrizi awarded 2018 Cotzias Prize from Spanish Society of Neurology
UCL research into genetic therapies for neurodegeneration boosted by £5m donation from The Sigrid Rausing Trust

New Neurogenetic Therapies Programme to test promising genetic therapies for neurodegenerative and dementia-causing diseases launched at QS ION. UCL thanks to a £5m donation by Sigrid Rausing Trust, one of the UK’s largest philanthropic foundations.
Harnessing Seven Decades Of Prospective Data Collection To Understand The Causes Of Dementia – A Deep Phenotyping Study Of The British 1946 Birth Cohort

Schott, Fox, Zetterberg, Crutch, Hardy, Richards, Chaturvedi

$10,559,333
UK DRI at UCL ION

UK Dementia Research Institute

£250m investment from 3 founding funders

Head Professor Karen Duff

Adrian Ivinson  Bart De Strooper

Partners are key to the success of the UK DRI

Founding funders

MRC Medical Research Council

Alzheimer's Society United Against Dementia

Alzheimer's Research UK The Power to Defeat Dementia
The Leonard Wolfson Experimental Neurology Centre (LWENC)

Vincenzo Libri

Active studies

- Phase II/III
- Proof-of-concept/Phase 2a
- FTIM/Phase I
- Experimental medicine

Other neurological disorder
Neurodegerative

HD
AD
ALS
PD

Antisense oligonucleotide studies

6 4 3 5
5 7 3 18
Plans commenced to expand the capacity of the Centre during 2020-21
... September 2019

- 30-40 active research studies active at any one time
- 10-20 research studies in set up at any one time
8 Antisense Oligonucleotide Trials

- Huntington’s x 4 (Tabrizi-Wild)
- Alzheimer’s FIH x 1 (Mummery)
- Parkinson’s FIH x 1 (Morris)
- Centronuclear Myopathies FIH x 1 (Quinlivan)
- ALS FIH x 1 (Libri)
Numerous honours and awards

Royal Society Ferrier Medal
Professor Ray Dolan

Professor Jim Rothman
Fellow of the Royal Society

Professor Rob Brownstone
Fellow Academy of Medical Sciences
Senior Promotions 100% success rate

Emma Matthews
Principal Research Fellow

Helene Plun-Favreau
Professor of Cellular Neurodegeneration

James Jepson
Principal Research Fellow

Michael Lunn,
Professor of Clinical Neurology

Parashkev Nachev
Professor of Neurology

Pedro Machado
Associate Professor

Robert Pitceathly
Principal Research Fellow

Rickie Patani
Professor of Human Stem Cells and Regenerative Neurology

Rohan De Silva
Professor of Molecular Neuroscience

Rosaline Quinlivan
Professor of Neuromuscular Disease
Retirements

Professor Simon Shorvon
Department CE Epilepsy

Professor Martin Brown
Department of Brain Repair & Rehabilitation

Shihan Jayasuriya
Institute Executive Officer, IoN
30 years at UCL!

Professor Janice Holton
Neuropathology
Department NDD
World-leading Queen Square clinician scientists

Professor Gerald Stern
October 9 1930 to September 9 2019

Professor Michael Harrison
August 24, 1936 to July 9, 2019
Charity / Philanthropy Partners

UCL OVPD

Brain Research UK

National Brain Appeal

Many charities and patient organisations e.g. Alzheimer’s Society, ARUK, MDUK, UCLH charity
Key Partnerships to deliver Translational Neuroscience

Hospital / Trust

UCL Partners

Faculty of Brain Sciences and School

NIHR Biomedical Research Centre

Sainsbury Wellcome Centre

UCL Neuroscience Domain

Crick Institute
A single cohesive translational research community across different locations
New Translational Neuroscience Building EDH 2023

Major investment in the future of IoN

~£281M

- Philanthropy | £~50M
- Dementia Retail Partnership | £20M
- MRC Dementia hub (awarded) | £40M
- HEFCE RPIF | £28.8M
- UCL Capital Investment | £120M

GAP | £23.8M

Full business case approved by UCL Council March 2019

Camden planning approval granted Sept 2019
Where is the Site?

The site on Gray’s Inn Road is formed of three historic phases of development. The former Royal Free Hospital courtyard building to the north, Eastman Dental Hospital to the south and a cluster of buildings which make up the Levy Wing to the east of the site.
Thanks to
Michael Schuitevorder
Building Project Programme Director

Roger Tuke
UCL Estates
Queen Square House

- Refurbished Lecture Theatre
- Consultation and Infusion suite
- Remodelled entrance with cafe, meeting and seminar facilities
Linking Translational Neuroscience Community
Total students
October 2019
MSc: 182
PhD: 291
Total: 473
Teaching awards 2018/19

Djamshidian-Chinthapalli award for outstanding contribution to clinical teaching
• Dr Etienne Laverse

Clinical Tutor of the year
• Dr Sam Shribman

• Dr Tony Pullen Lecturer of the Year Award
• Prof Nick Fox
ION LIFE-LONG LEARNING STRATEGY

MASTER CLASS (2 days)
NEUROLOGY 2015, 2016, 2017, 2018, 2019

250-300 delegates trainees and consultants

Prof Simon Shorvon

Dr Tabish Saifee
Student Prizes 2019
2019

MSc Clinical Neurology

Hugo James Ross Prize

Dr Sabhat Iqbal
Queen Square Essay Prize 2019

Dr Karen Sutterlin

MRC Clinical Training fellow
Annual Address

Dr C. Frank Bennett, PhD, Senior Vice President, Research & Neurology Franchise Leader, IONIS Pharmaceuticals Inc.
Annual Address

Dr C. Frank Bennett, PhD

• BS Pharmacy Univ New Mexico
• PhD Pharmacology, Baylor College Medicine, Houston, Texas
• Associate Senior Investigator, Molecular Pharmacology, GlaxoSmithKline
• Currently Senior Vice President Ionis Pharmaceuticals – founding member of company
• Leads development of preclinical antisense drug discovery
• Published >200 paper on antisense research; 175 issued US patents
• 2018 Hereditary Disease Foundation’s Leslie Gehry Brenner Prize– HD

• 2019 Co-recipient of Breakthrough Prize in Life Sciences for discovery and development SPINRAZA (nusinersen) for SMA
Annual Address

Dr C. Frank Bennett, PhD, Senior Vice President, Research & Neurology Franchise Leader, IONIS Pharmaceuticals Inc.