# Tutor Biographies

## Professor Gillian Bates

## University College London

Gillian Bates is Professor of Molecular Neuroscience, Co-Director Huntington's Disease Centre and a Group Leader in the UK Dementia Research Institute at UCL. She began her work on Huntington's Disease as a postdoctoral scientist when she joined the international group pf scientists who worked together to clone the Huntington's Disease (HD) gene. After the identification of the HD mutation in 1993, she established her independent research programme and generated the first mouse model of this HD in 1996. Over the course of the last 25 years, her research has been directed toward understanding the molecular basis of HD and validating therapeutic targets. Her group has shown that in addition to the mRNA that is transcribed from the HD gene, a small mRNA is also generated that encodes a highly pathogenic fragment of the huntingtin protein. A major focus of her group is to understand that extent to which this very toxic small protein contributes to the disease and to identify and test novel therapeutic approaches to lower its levels. These include a range of approaches, that include antisense oligonucleotides, siRNAs and small molecules. She has been elected to the Academy of Medical Sciences (1999), EMBO (2002) and the Royal Society (2007). She has been awarded the Royal Society Glaxo Wellcome Award (with Stephen Davies) (1998), the Max Planck Research Award for International Cooperation (1999), the Klaus Joachim Zülch-Preis (2001) and the Leslie Brenner Gehry Prize for Innovation in Science (2011).

## Dr Caroline Benn

## LoQus23 Therapeutics

Current Roles: Caroline is the co-founder and CSO of LoQus23 Therapeutics, a Dementia Discovery Fund-created company. LoQus23 is targeting DNA damage repair processes which are a driving force of the pathophysiology in trinucleotide repeat expansion diseases such asHuntington's disease.

Prior Experience: Caroline obtained a BSc(Hons) in Neuroscience at the University of Sussex, followed by a PhD in Molecular Neurogenetics at Kings College London where she investigated the contribution of subcellular localization to Huntington's disease (HD) pathogenesis in Prof Gill Bates lab. Her first postdoctoral position studying neurotransmitter receptor alterations and mechanisms of transcriptional dysregulation in HD was with Dr Jang-Ho ChaatMassachusetts General Hospital/Harvard Medical School. Subsequently, Caroline worked on the genetic and pharmacological modulation of HDACs in HD at Kings College London. After 12 years in academia, Caroline joined Pfizer as a group leader and drug discovery research project leader with a remit spanning early-stage activities through to the clinic. She has a track record for championing sophisticated molecular and cellular approaches to impact on drug discovery. Caroline then joined Astex Pharmaceuticals as the Associate Director for CNS biology where she was responsible for strategic input, portfolio management and leadership within multidisciplinary project teams for two years prior to joining the Dementia Discovery Fund as an entrepreneur-in-residence.

# Tutor Biographies

## Dr. Frank Bennett

## Ionis Pharmaceuticals

Dr. Bennett is the executive vice president and chief scientific officer at Ionis Pharmaceuticals and one of the founding members of the company. He is responsible for continuing to advance antisense technology and expanding Ionis drug discovery platform. Dr. Bennett is also the franchise leader for neurological programs at Ionis. He has been involved in the development of antisense oligonucleotides as therapeutic agents, including research on the application of oligonucleotides for inflammatory, neurodegenerative diseases and cancer, oligonucleotide delivery, pharmacokinetics and medicinal chemistry.

Dr. Bennett is a co-recipient of the 2019 Breakthrough Prize in Life Sciences for his contributions to the discovery and development of SPINRAZA® (nusinersen) and the 2018 Hereditary Disease Foundation's (HDF) Leslie Gehry Brenner Prize for Innovation in Science for his leadership and continued commitment to developing antisense therapies for Huntington's disease (HD).

Dr. Bennett has published more than 230 papers in the field of antisense research and development, and he is an inventor on more than 175 issued patents.

Prior to joining Ionis, Dr. Bennett was associate senior investigator in the Department of Molecular Pharmacology at SmithKline and French Laboratories (currently, GlaxoSmithKline).

He received his Ph.D. in Pharmacology from Baylor College of Medicine, Houston, Texas and his B.S. degree in Pharmacy from the University of New Mexico, Albuquerque, New Mexico. He performed his postdoctoral research in the Department of Molecular Pharmacology at SmithKline and French Laboratories.

Dr. Bennett serves on the Advisory Board for the Hereditary Disease Foundation.

## Dr. Beverly Davidson

## Children's Hospital of Philadelphia/University of Pennsylvaania

Beverly L. Davidson, PhD is the Director, Raymond G. Perelman Center for Cellular and Molecular Therapeutics, Chief Scientific Strategy Officer, and holds the Arthur V. Meigs Chair in Pediatrics at Children’s Hospital of Philadelphia. She is Professor of Pathology and Laboratory Medicine, Perelman School of Medicine, University of Pennsylvania. She received her Ph.D. in Biological Chemistry from University of Michigan.

The Davidson lab is focused on genetic diseases that affect the brain, including how mutant gene products contribute to disease, and why certain brain regions are more susceptible. The team employs advanced molecular methods, sequencing and imaging modalities in animal models, and uses a variety of molecular tools to test various hypotheses. The lab is also engaged in the development of next generation therapeutics for inherited disorders, including the engineering of novel gene therapy vector capsids and cargo to approach tissue and cell type specific treatments.

Recent honors include election into the American Academy of Arts and Sciences and the National Academy of Medicine, and the Hereditary Disease Foundation’s Leslie Gehry Brenner Prize for Innovation in Science. She is the current president of the American Society of Gene and Cell Therapy, the largest international association of gene and cell therapy research.

# Tutor Biographies

## Professor Alexandra Durr

## Sorbonne University

Alexandra Durr is a University Professor for medical genetics at the Sorbonne University and in the Genetic Department of the Pitié-Salpêtrière University Hospital in Paris, France. She trained in neurology and genetics and has been developing translational neurogenetics for 25 years, based on a thorough clinical expertise that allowed her to identify the molecular bases of many pathologies. Alexandra Durr is a worldwide renowned expert of late-onset hereditary neurological diseases. She worked with premanifest individuals since 1992, i.e. mutation carriers without clinical signs of the disease, pioneering the first presymptomatic structure for neurogenetic diseases in France. She coordinates the National Reference Centre for Rares Diseases (www.brain-team.fr). She has built up national and international collaborations from the beginning of her career. Recognized as a leader in her field, she is a dedicated clinician and scientist. Genetic advances are used by her team at the Paris Brain Institute (ICM: Institut du Cerveau) to understand pathophysiology and to set up innovative therapies. To prepare this new era of genetic therapeutics, Alexandra Durr is taking advantage of the pre-manifest phase of neurodegenerative diseases and is developing progression markers to determine the best therapeutic window, that will ensure that the disease does not develop further.

## Prof Frances Edwards

## UCL

Grew up in Sydney Australia where I completed my undergrad BSc with Honours in Behavioural Pharmacology. Trained as an electrophysiologist at the Australian National University but moved to Goettingen (then west) Germany for most of my PhD with Bert Sakmann where we applied patch clamp techniques to brain slices. I moved as a postdoc to London and then back to Australia for 5 years and finally back to London as a Senior Lecturer in 1996.

All of my research has been on fast synaptic transmission in the rodent brain but over the last decade, I have worked on Alzheimer's disease and expanded to study the interactions of synapses, plaques and microglia including electrophysiology, immunohistochemistry and gene expression.

In my spare time, I love to sail, walk in the forest and to make pottery and wooden bowls.

# Tutor Biographies

## Professor John Hardy

## UCL Queen Square Institute of Neurology

Following his PhD, Hardy did postdoctoral research at the MRC Neuropathogenesis Unit in Newcastle upon Tyne, England and then further postdoctoral work at the Swedish Brain Bank in Umeå, Sweden where he started to work on Alzheimer's disease. He became Assistant Professor of Biochemistry at St. Mary's Hospital, Imperial College London in 1985 and initiated genetic studies of Alzheimer's disease there, before moving to the USA in 1989 then taking the Pfeiffer Endowed Chair of Alzheimer's Research at the University of South Florida, in Tampa in 1992. In 1996 he moved to Mayo Clinic in Jacksonville, Florida, as Consultant and Professor of Neuroscience. He became Chair of Neuroscience in 2000 and moved to National Institute on Aging, Bethesda, Maryland, as Chief of the Laboratory of Neurogenetics in 2001. In 2007 he took up the Chair of Molecular Biology of Neurological Disease at the Reta Lila Weston Institute of Neurological Studies, University College London. November 2015, he was awarded the Breakthrough Prize and in 2018 jointly the Brain Prize from The Lundbeck Foundation in Denmark. In 2022 he was awarded a Knighthood in the New Year's Honours.

## Dr Warren Hirst

## Biogen

Warren Hirst is Senior Director and head of the movement disorders preclinical research efforts in Biogen's Research organization, a role he has led since joining them in 2016. Warren is responsible for developing and driving a diverse preclinical portfolio of programs, using multiple modalities, from antibodies and antisense oligonucleotides, in collaboration in Ionis, to gene therapy and small molecules, targeting mechanisms strongly supported by human genetics and pathology, to deliver novel disease-modifying therapeutics to patients.

Warren received his Ph.D. from Imperial College, London and, after a post-doc at the same institution, he moved to industry. Warren has 23 years of pharma experience, all focused on neurodegeneration research – including Alzheimer's disease, depression, stroke and Parkinson's disease, and drug development, holding positions of increasing responsibility at SmithKline Beecham and GlaxoSmithKline in England, before moving to the USA to join Wyeth and then Pfizer. Warren has directly lead teams that have discovered and advanced 3 molecules into clinical trials and has been on teams that have made similar advances with 12 further molecules. Warren has published over 80 peer-reviewed scientific papers. Warren is actively engaged in the wider scientific community via collaborations with key academic partners, and his service on NINDS study sections, foundation grant review committees, and as an ad-hoc reviewer for multiple journals including Neuron and PNAS. Warren has developed a strong, and long-term, dating back to 2006, relationship with the Michael J Fox Foundation for Parkinson's Research, having received multiple grants in addition to serving as a reviewer and assessor, he served on their Executive Scientific Advisory Board from 2017-2019 and remains a close scientific advisor. In 2019 Warren received the Bill Langston award from the MJFF in recognition of his outstanding contributions to The Michael J. Fox Foundation for Parkinson's Research. Warren is currently on the Parkinson's Foundation Executive Scientific Advisory Board.

Outside of work Warren enjoys snowboarding and travel (looking forward to getting back to both of these post-Covid19).

# Tutor Biographies

**Dr Lorenza Magno**

**University College London**

Lorenza obtained a first-class honours degree in Biotechnology applied to Pharmacy from the University of Milan, Italy, whilst working on characterising peripheral biomarkers for neurodegenerative disorders.

She then received a summa-cum-laude PhD in Medical Neurosciences at Charité University in Berlin, Germany, using genetic and molecular tools, paired with anatomical and behavioural assessments in rodent model to understand molecular pathways and cellular functions underlying rare genetically inherited disorders affecting the brain.

She pursued her interest in neurological diseases by taking a post-doctoral research position at University College London, where she focused on the genetics of forebrain development and contribution of altered developmental trajectories to neurological diseases such as autism.

Lorenza joined the Neurodegeneration Biology group within the Alzheimer Research UK Drug Discovery Institute at UCL in 2017 where she developed microglia model systems to characterise and validate potential drug targets for neurodegenerative disorders. She has successfully led drug discovery projects, working collaboratively and leading cross-site teams within UCL and external partners, with the aim to develop novel therapeutic approaches via harnessing the immune system.

At the DDI, Lorenza now leads a multidisciplinary team of scientists dedicated to identifying new targets and therapies for Alzheimer's disease and other dementia, enabling early drug discovery from target validation through to lead optimisation.

**Mr Vilas Menon**

**Columbia University Irving Medical Center**

Vilas Menon leads a research group at Columbia University with expertise in computational methods applied to large-scale molecular data sets in neuroscience. His lab applies state-of-the-art computational and experimental methods to generate and analyze large-scale molecular data in the context of neurological disease. In particular, his group investigates signatures of differential vulnerability and resistance at both the cell type and individual level in neurodegenerative diseases (including Alzheimer's, Huntington's, ALS, and Parkinson's) and neuroimmune diseases (such as Multiple Sclerosis).

# Tutor Biographies

## Dr Rosa Rademakers

## VIB-UAntwerp

Dr. Rademakers received her BSc degree in Biology in 1997 and MSc degree in Biochemistry in 1999 from the University of Antwerp, Belgium. In 2004, she received a PhD degree from the University of Antwerp where she continued her postdoctoral studies before moving to the Mayo Clinic in Jacksonville, Florida in 2005. Since 2007 she was a faculty member at the Neuroscience Department of the Mayo Clinic Jacksonville, Florida, USA, where she became full Professor in 2014. As of 2019, Dr. Rademakers returned to Belgium where she is currently Scientific Director of the VIB-UA Center for Molecular Neurology and full Professor in the Department of Biomedical Sciences at the University of Antwerp.

Her research is focused on the molecular genetics analyses of neurodegenerative diseases, with a special interest in frontotemporal dementia (FTD), amyotrophic lateral sclerosis (ALS) and early-onset Alzheimer's Disease (AD). Her laboratory has been at the forefront of neurodegenerative disease research since playing a critical role in the discovery of progranulin (GRN) as the first causal gene implicated in FTD. Importantly, in 2011, her laboratory made the discovery that C9ORF72 repeat expansions were the long sought-after cause of ALS and FTD linked to chromosome 9p. Over the last decade her work has expanded into population-based studies to identify FTD genetic risk and modifier factors, including the generation and analyses of whole genome sequences.

Dr. Rademakers leads world-wide consortia to identify causal genes and genetic risk factors for two important pathological subtypes of FTD. She combines genomic, transcriptomic, epigenomic and proteomic analyses on unique collections of patients' brain tissues to identify genes and pathways implicated in disease. She also leads a consortia focused on genetic disease modifiers in FTD patients which are known to carry known causal mutations with the goal to identify factors that could explain the large variability in symptom onset, clinical phenotype and disease penetrance in patients with these mutations.

Dr. Rademakers has published over 400 peer- reviewed original articles and reviews. For her work, she has received the Paolo Gontijo Medicine Award, the Generet Award for Rare Diseases and the Sheila Essey Award for ALS Research from the ALS Association in partnership with the American Academy of Neurology. She is also the recipient of the 2016 Potamkin Prize for Research in Pick's, Alzheimer's and Related Disorders of the American Academy of Neurology.

## Dr Benjamin Ryskeldi-Falcon

## MRC Laboratory of Molecular Biology

Dr Benjamin Ryskeldi-Falcon is a Programme Leader at the MRC Laboratory of Molecular Biology, Cambridge, UK. His focus is on mechanisms of protein self-assembly into amyloid structures in neurodegenerative diseases, using a combination of high-resolution electron microscopy, human tissue samples and cell/ tissue culture models. Benjamin completed his undergraduate degree at University College London and received his PhD from the University of Cambridge in 2016. He took up his current position in October 2019.

# Tutor Biographies

## Dr. Hongjun Song

## University of Pennsylvania

Hongjun Song, Ph.D. is the Perelman Professor of Neuroscience at Perelman School of Medicine of University of Pennsylvania. He received his. B.S from Peking University, M.A. from Columbia University and Ph.D. from University of California at San Diego. The research in Dr. Song's laboratory focuses on plasticity in the adult mammalian nervous system, in particularly, adult neurogenesis and neuro-epigenetics/neuro-epitranscriptomics. His laboratory also uses patient-derived stem cells in 2D and 3D organoids to model human brain development and neurological disorders. He serves on a number of editorial boards and has won several awards, including Young Investigator Award from the Society for Neuroscience, and Jacob Javits Neuroscience Investigator Award from National Institutes of Health. He is a member of the National Academy of Medicine.

## Dr. Dietmar Thal

## KU Leuven

Prof. Dr. Dietmar R. Thal is a clinical neuropathologist at the University Hospital Leuven and professor for Neuropathology at KU-Leuven (Belgium) with his main research focus on Alzheimer's disease. His main interest is the expansion and maturation of protein aggregates in this disorder. He was able to discover phases describing the expansion of amyloid plaque pathology in the human brain. These phases are currently included in the diagnostic criteria for the neuropathological assessment of Alzheimer's disease (known as Thal-Phases).

His group also showed that the current amyloid PET-methods are usually restricted to the detection of advanced phases of amyloid plaques pathology distribution and in so doing represent a valuable tool for diagnosing the symptomatic disease but not for picking up very early stages of the disease. In addition, his group was able to show that not only the anatomical expansion of amyloid plaque aggregates plays a role in the pathogenesis of Alzheimer's disease but also the maturation of the aggregates, meaning that the composition of amyloid plaque/soluble and dispersible amyloid aggregates changes over time with specific proteins becoming detectable only in the symptomatic cases. Aβ also binds to the cellular prion protein which can also bind phosphorylated τ-protein. Dr. Thal's group found that this interaction is associated with an acceleration of the τ-pathology.

Very recently, his group demonstrated the accumulation of the active necrosome in granulovacuolar degeneration in Alzheimer's disease and its association with neuron loss.

# Tutor Biographies

## Dr Selina Wray

## University College London

Selina Wray is a Professor of Molecular Neuroscience and Alzheimer's Research UK Senior Research Fellow in the Department of Neurodegenerative Disease at UCL Queen Square Institute of Neurology. She is also the UCL Queen Square Institute of Neurology Deputy Director, Partnerships and Communications.

Selina received her degree in Biochemistry and Biological Chemistry from the University of Nottingham in 2004, before undertaking PhD training in Dr Diane Hanger's laboratory at the Institute of Psychiatry, Kings College London. Selina was awarded her PhD in 2008 and subsequently joined the laboratory of Professor John Hardy at UCL Institute of Neurology as an Alzheimer's Research UK Junior Research Fellow. Selina spent time as a visiting researcher in the groups of Tilo Kunath at the University of Edinburgh and Rick Livesey at the University of Cambridge before setting up her own lab at UCL.

Selina's work is focussed on the use of induced pluripotent stem cell (iPSC) technology to model dementia, working closely with clinical colleagues to obtain samples from participants with rare, genetic forms of dementia and using these to understand the molecular basis of Alzheimer's Disease and Frontotemporal Dementia.