NEUROLOGY

Faculty Profiles

Johns Hopkins UNIVERSITY

Johns Hopkins TECHNOLOGY VENTURES

Novel Treatment Approaches to Specific Psychiatric Symptom Domains, Nociplastic Pain and Symptoms of Dementia/ Neurodegeneration
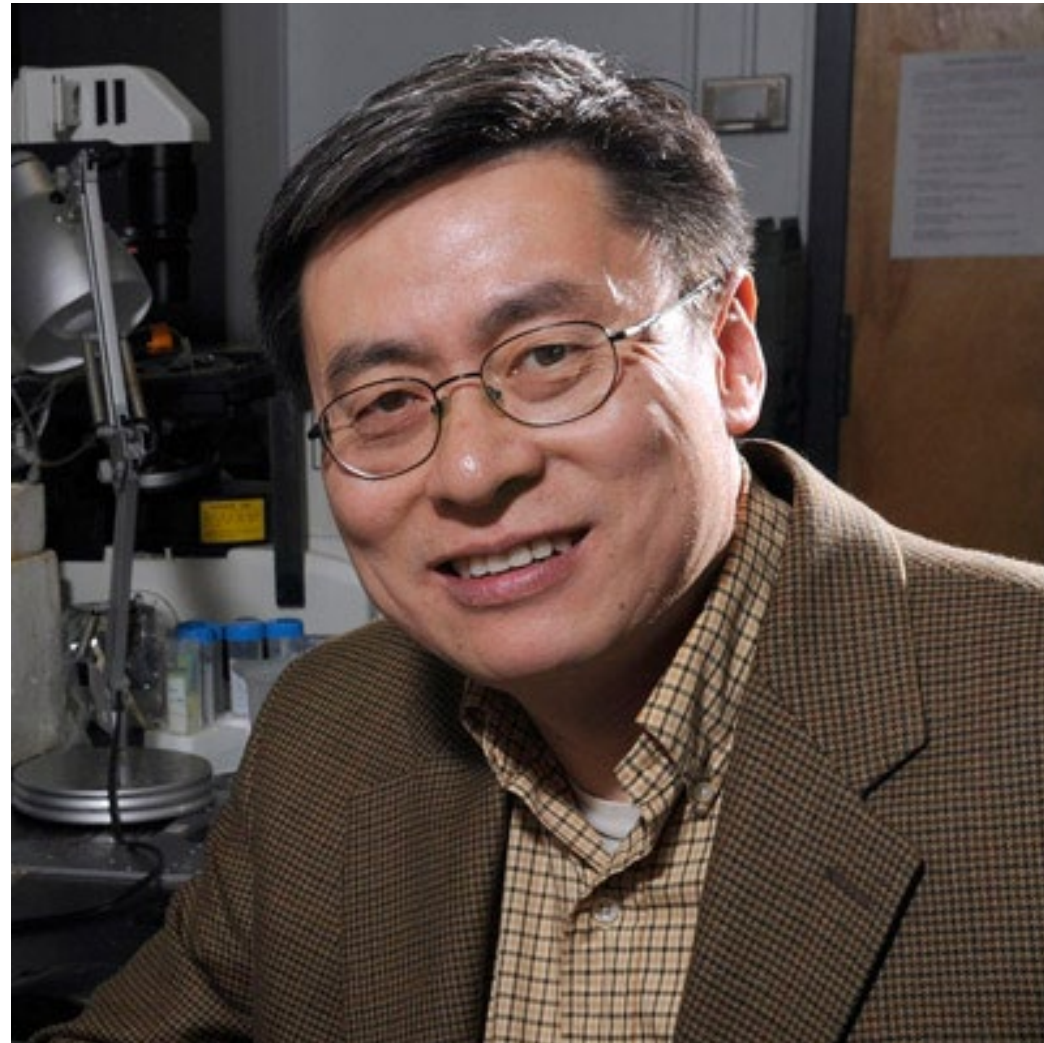
GABSANG LEE, PHD DVM

- Professor of Neurology and Neuroscience, Institute for Cell Engineering
- Disease Areas of Focus: Symptomatic treatment of dementia/neurodegeneration
- Summary of Work: The Lee lab focuses on neural diseases and muscular dystrophies. The lab established novel methodologies for direct derivation and prospective isolation of neurons, glia cell, and skeletal muscle cells from human pluripotent stem cells.

Recent Projects:
- Advanced human iPSC-based preclinical model for Parkinson's disease with optogenetic alpha-synuclein aggregation
- Multi-omic analysis of selectively vulnerable motor neuron subtypes implicates altered lipid metabolism in ALS
- GSK3 inhibition improves skeletal muscle function and whole-body metabolism in male mouse models of Duchenne muscular dystrophy

XINZHONG DONG, PHD

– Professor of Neuroscience, Professor of Neurology
– Disease Areas of Focus: Nociplastic Pain
– Summary of Work: The Dong lab has identified many genes specifically expressed in the pain-sensing neurons of the dorsal root ganglia. The lab uses molecular biology, mouse genetics and behavior, and electrophysiology to examine the function of these genes in pain.

Recent Projects:
– Dissecting functional diversities of human sensory neuron subsets to develop a new humanized platform for pain therapy

JEFFERY ROTHSTEIN, MD PHD

- Professor of Neurology and Neuroscience, Director of the Brain Science Institute
- Disease Areas of Focus: Symptomatic treatment of dementia/neurodegeneration
- Summary of Work: The Rothstein lab focuses on several pathways of cellular dysfunction in ALS, Huntingtons disease, and dementias. In particular, there is a focus on the nuclear pore and astroglia.

Recent Projects
- (C15996) Mitigation of Nuclear Pore defect in Neurodegeneration: ESCRT Pathway Inhibition
- Inhibition of RNA splicing triggers CHMP7 nuclear entry, impacting TDP-43 function and leading to the onset of ALS cellular phenotypes
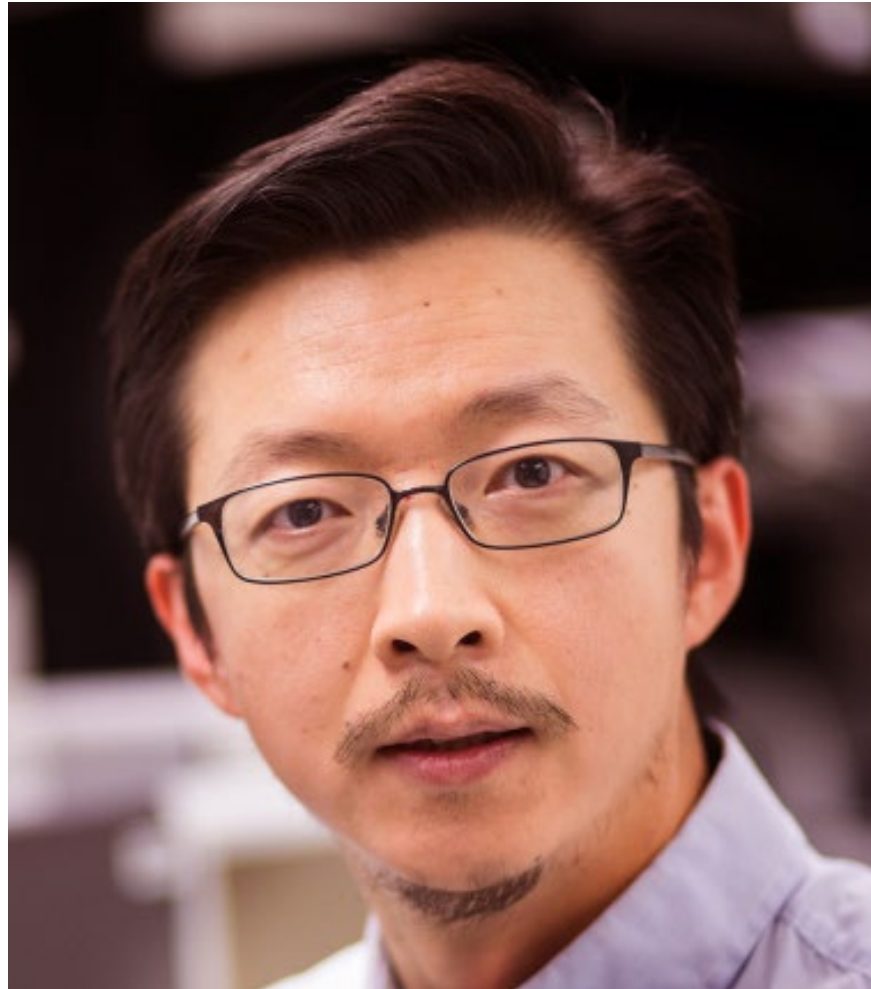
XIAOBO MAO, PHD

- Professor of Neurology and Neuroscience
- Disease Areas of Focus: Symptomatic treatment of dementia/neurodegeneration
- Summary of Work: The Mao lab focuses on protein aggregation and pathogenesis of neurodegenerative disorders. He identified the importance of the C-terminus of α-synuclein and serine129 phosphorylated α-synuclein binding to its receptors (LAG3 and APLP1), and the complex of APLP1-LAG3, exacerbating pathology spread and neurodegeneration.

Recent Projects:
- Nanozyme Scavenging ROS for Prevention of Pathologic α-Synuclein Transmission in Parkinson's Disease.
- Mechanistic basis for receptor-mediated pathological α-synuclein fibril cell-to-cell transmission in Parkinson's disease
- The Efficacy of Nanozyme in Neurodegenerative Diseases

JIOU WANG, MD PHD

– Professor of Biochemistry and Molecular Biology
– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
– Summary of Work: The Wang lab is focused on the biological basis for protein and RNA homeostasis in neurodegeneration

Recent Projects:
– Novel eIF2α kinase target and small molecule inhibitors for modulating translation and the stress response
– G-Quadruplexes as pathogenic drivers in neurodegenerative disorders

JAMES BARROW, PHD

– Professor of Pharmacology and Senior Investigator and Director of the Drug Discovery Division at the Lieber Institute
– Disease Areas of Focus: Schizophrenia, depression & anhedonia, bipolar disorders, symptomatic treatment of dementia/neurodegeneration, nociplastic pain
– Summary of Work: Barrow's lab is focused on medicinal chemistry and drug discovery for diseases of the central nervous system and psychiatric disorders.

Recent Projects:
– Development and characterization of pyridyl carboxamides as potent and highly selective Nav1.8 inhibitors
– Identification and Characterization of a Blood-Brain Barrier Penetrant Inositol Hexakisphosphate Kinase (IP6K) Inhibitor

BARBARA SLUSHER, PHD MAS

– Professor of Neurology, Pharmacology and Molecular Sciences, Psychiatry, Neuroscience, Medicine and Oncology; Director of Johns Hopkins Drug Discovery
– Disease Areas of Focus: Schizophrenia, depression & anhedonia, substance use disorders, Bipolar disorders, Nociplastic pain, dementia, neurodegeneration
– Summary of Work: Dr. Slusher runs the largest drug discovery program at Johns Hopkins and has co-founded four new companies.

Recent Projects:
– Methods to Treat Neurodegenerative Diseases
– Discovery of Small Molecule Inhibitors of Neutral Sphingomyelinase 2 (nSMase2) for the Treatment of Neurodegenerative Diseases
– Glutamine Antagonists for the Treatment of Cognitive Deficits, including NeuroAIDs

TAKASHI TSUKAMOTO, PHD

– Professor of Neurology, Director of Medicinal Chemistry at the Johns Hopkins Drug Discovery Program
– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
– Summary of Work: Dr. Tsukamoto leads a team of medicinal chemists involved in the design and synthesis of small molecules of therapeutic significance for neurological disorders and cancer.

Recent Projects:
– Novel Sigma-1 Receptor Ligand
– Small Molecule Neutral Sphingomyelinase 2 (nSMase2) Inhibitors

VALINA DAWSON, PHD

– Professor of Neurology, Director, Neuroregeneration and Stem Cell Programs, Institute for Cell Engineering
– Disease Areas of Focus: Symptomatic treatment of dementia/neurodegeneration
– Summary of Work: The Dawson lab is focused on discovering and defining cell signaling pathways that lead to either neuronal survival or neuronal death.

Recent Projects:
– PAAN/MIF nuclease inhibition prevents neurodegeneration in Parkinson's disease
– Farnesylation of PARIS Prevents Dopaminergic Neurodegeneration in Models of Parkinson's Disease

CHARLOTTE SUMNER, MD

– Professor of Neurology and Neuroscience, Genetic Medicine; Co-Director of the Johns Hopkins Muscular Dystrophy Association Care Center, the Spinal Muscular Atrophy (SMA), and the Charcot-Marie-Tooth (CMT) clinics
– Disease Areas of Focus: Nociplastic pain, neurodegeneration
– Summary of Work: Dr. Sumner's research focuses on developing treatments for degenerative disorders of motor neurons and peripheral nerves. Her laboratory uses human tissues and induced pluripotent cell lines, mouse models and cultured cells to characterize disease mechanisms and develop treatments.

Recent Projects:
– Disease Progression in Charcot–Marie–Tooth Disease Related to MPZ Mutations: A Longitudinal Study
– Boosting neuregulin 1 type-III expression hastens SMA motor axon maturation

AHMET HOKE, MD PHD

Johns Hopkins University

– Professor of Neurology and Neuroscience, Director of Neuromuscular Division
– Disease Areas of Focus: Neurodegeneration, nociplastic pain
– Summary of Work: The Hoke lab is focused on the mechanisms of axonal degeneration and regeneration.

Recent Projects:

– Ethoxyquin and Derivatives for Treatment of Peripheral Neuropathies and Other Neurodegenerative Disorders

NICHOLAS MARAGAKIS, MD

– Professor of Neurology, Director, ALS Center for Cell Therapy and Regeneration Research
– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
– Summary of Work: The Maragakis lab is focused on creating and characterizing lines of stem cells from patients with ALS using induced pluripotent stem cell (iPSC) methodologies.

Recent Projects:
– Tonabersat as a Neuroprotective Compound for Amyotrophic Lateral Sclerosis

Quantitative Phenotyping of Human Disorders to Stratify Patients, Validate Biomarkers and Identify Circuit and/or System Targets In Psychiatry

KOSTAS LYKETSOS, MD

– Professor of Psychiatry and Behavioral Sciences
– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
– Summary of Work: Dr. Lyketsos is a world expert in the care and treatment of patients with Alzheimer's and related dementias. His team is developing biomarkers to accelerate treatment development for Alzheimer's.

Recent Projects:
– Genetic meta-analysis of diagnosed Alzheimer's disease identifies new risk loci and implicates Aβ, tau, immunity and lipid processing
– iPSC-derived hindbrain organoids to evaluate escitalopram oxalate treatment responses targeting neuropsychiatric symptoms in Alzheimer's disease
– Assessment and management of behavioral and psychological symptoms of dementia

SHUYING SUN, PHD

– Professor of Physiology, Brain Science Institute
– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
– Summary of Work: The Sun lab focuses on the molecular pathways affected by mutations and pathologies associated with neurodegeneration.

Recent Projects:
– Disruption of nuclear speckle integrity dysregulates RNA splicing in C9ORF72-FTD/ALS
– Tyrosine phosphatase PTPomega inhibitors for ALS-FTD

AKIRA SAWA, MD PHD

– Professor of Psychiatry and Behavioral Sciences
– Disease Areas of Focus: Schizophrenia, depression & anhedonia, Impulsivity & substance use disorders, Bipolar disorders, Borderline personality disorder, Post-traumatic stress disorder
– Summary of Work: The Sawa lab focuses on the pathogenesis of major mental illnesses, evaluating molecular targets and neuronal-glial functional interactions.

Recent Projects:
– Nuclear GAPDH in cortical microglia mediates cellular stress-induced cognitive inflexibility
– Big potassium channel activators (Unoprostone) and NS1619 are effective in treating bipolor disorder
– New, Simple and Quick Test to Diagnose, Predict, and Prevent Brain Disorders Using Non-invasive Nasal Brush and Single-Cell Analysis

ANNIE KATHURIA, PHD

- Professor of Biomedical Engineering
- Disease Areas of Focus: Schizophrenia, Symptomatic treatment of dementia/neurodegeneration, Bipolar disorders, Borderline personality disorder
- Summary of Work: The Kathuria lab focuses on organoid tissue engineering to conduct high-throughput drug and toxicological screening for neurological disorders.

Recent Projects:
- Comparison of Model Systems for Emulating Human Tissue and Physiology in Psychiatric Research
- Morphological and transcriptomic analyses of stem cell-derived cortical neurons reveal mechanisms underlying synaptic dysfunction in schizophrenia
- Disease-specific differences in gene expression, mitochondrial function and mitochondria-endoplasmic reticulum interactions in iPSC-derived cerebral organoids and cortical neurons in schizophrenia and bipolar disorder

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JEFFERY ROTHSTEIN, MD PHD

– Professor of Neurology and Neuroscience; Director of the Brain Science Institute
– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
– Summary of Work: The Rothstein lab focuses on the biology of the nuclear pore, nucleoporins, and nuclear transport in ALS and other dementias

Recent Projects:
– Mutant Huntingtin Disrupts the Nuclear Pore Complex.
– Posterior cerebellar Purkinje cells in an SCA5/SPARCA1 mouse model are especially vulnerable to the synergistic effect of loss of β-III spectrin and GLAST.
– Astroglial transcriptome dysregulation in early disease of an ALS mutant SOD1 mouse model

CIPRIAN CRAINICEANU, PHD

- Professor of Biostatistics in the Bloomberg School of Public Health
- Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
- Summary of Work: Dr. Crainiceanu focuses on complex, ultrahigh dimensional data obtained from wearable and implantable computing and neuroimaging studies; signal processing; brain imaging and other high-dimensional functional data

Recent Projects:
- Neuroconductor: An R Platform for Medical Imaging Analysis
- Big Data and Neuroimaging
- Longitudinal High-Dimensional Principal Components Analysis with Application to Diffusion Tensor Imaging of Multiple Sclerosis

PETER ZANDI, PHD

– Professor of Psychiatry and Behavioral Sciences; Co-Director of the Johns Hopkins Precision Medicine Center of Excellence on Mood Disorders
– Disease Areas of Focus: Schizophrenia, Depression & anhedonia, Bipolar disorders, Borderline personality disorder, Dementias
– Summary of Work: The Zandi lab uses statistical genetics and bioinformatics to study the genetic causes of mood disorders and health informatic technologies to support the next generation of large-scale research on mood disorders

Recent Projects:
– Hormone therapy and Alzheimer disease dementia: new findings from the Cache County Study
– Differential responses to lithium in hyperexcitable neurons from patients with bipolar disorder
– Analysis of the influence of microRNAs in Lithium Response in Bipolar Disorder

BIPASHA MUKHERJEE-CLAVIN, MD PHD

- Professor of Neurology and Neuroscience, Co-Director of the Johns Hopkins Charcot-Marie Tooth Disease Clinic
- Disease Areas of Focus: Neurodegeneration, nociplastic pain
- Summary of Work: Dr. Mukherjee-Clavin is focused on the use of patient-derived induced pluripotent stem cells to model genetic Schwann cell and peripheral nerve disorders to undercover novel pathways and potential treatments.

Recent Projects:
- Comparison of three congruent patient-specific cell types for the modelling of a human genetic Schwann-cell disorder
- Large-scale screening using familial dysautonomia induced pluripotent stem cells identifies compounds that rescue IKBKAP expression

PAUL WORLEY, MD

– Professor of Neuroscience
– Disease Areas of Focus: Schizophrenia, Impulsivity & substance use disorders, Symptomatic treatment of dementia/neurodegeneration
– Summary of Work: The Worley lab studies the molecular basis of memory consolidation, and how this process is disrupted in human diseases including drug addiction, schizophrenia, and Alzheimer's disease.

Recent Projects:
– Biomarkers of Alzheimer's and other Diseases of Cognition

YONG DU, PHD

– Professor of Radiology and Radiological Science

– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration

– Summary of Work: Dr. Du's areas of expertise are SPECT and PET imaging physics, image reconstruction, photon transport simulation and modeling, quantitative image biomarker and data analysis, and tracer kinetic modeling for cardiac, tumor, and brain imaging

– Recent Projects:

AI Based Disease Prognosis of Patients with Parkinson's Disease

AI-based Disease Detection from Raw SPECT and PET Data without Image Reconstruction

Digital Therapeutics and Biomarkers, including Digital Biomarkers

KEMAR GREEN, DO

- Professor of Neurology, Neuro-Ophthalmology, Neurotology
- Disease Areas of Focus: Neurodegneration
- Summary of Work: Dr. Green focuses on the application of artificial intelligence, data science and precision medicine to personalize, remotely monitor and treat neurologic diseases by leveraging digital biomarkers (eye movements, etc.), emerging neuro-sensing technologies, and novel human-AI interfaces

Recent Projects:
- aEYE: A Deep Learning System for Video Nystagmus Detection
- Artificial Intelligence-based Ocular Motor Biomarkers for Myasthenia Gravis Diagnosis

BIN WU, PHD

– Professor of Biophysics and Biophysical Chemistry, Neuroscience
– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
– Summary of Work: The Wu Lab is focused on the visualization and quantification of biological events as they happen in real time. The lab is focused on how cells regulate gene expression in developmental and neurodegenerative diseases.

Recent Projects:
– Single-molecule imaging reveals distinct elongation and frameshifting dynamics between frames of expanded RNA repeats in C9ORF72-ALS/FTD
– Translation imaging of single mRNAs in established cell lines and primary cultured neurons
– Spatial organization of single mRNPs at different stages of the gene expression pathway

JENNIFER ERWIN, PHD

– Professor of Neurology and Neuroscience, Investigator Lieber Institute for Brain Development
– Disease Areas of Focus: Schizophrenia, symptomatic treatment of dementia/neurodegeneration
– Summary of Work: The Erwin lab focuses on the contribution of retroelements and somatic mosaicism to human brain function by integrating human stem cell models, postmortem tissue and computational approaches.

Recent Projects:
– Variation in TAF1 expression in female carrier induced pluripotent stem cells and human brain ontogeny has implications for adult neostriatum vulnerability in X-linked Dystonia Parkinsonism
– Recapitulation of Perturbed Striatal Gene Expression Dynamics of Donor's Brains With Ventral Forebrain Organoids Derived From the Same Individuals With Schizophrenia

Innovative CNS delivery and Targeting Approaches

JEFF COLLER, PHD

– Professor of Molecular Biology and Genetics, Bloomberg Distinguished Professor of RNA Biology and Therapeutics
– Disease Areas of Focus: Neurogenetic disorders, neurodegneration
– Summary of Work: Dr. Coller's work focuses developing novel therapeutics for devastating rare diseases, improving gene therapy manufacturing and efficacy, and exploring novel disease diagnostics

Recent Projects:
– Suppression of premature transcription termination leads to reduced mRNA isoform diversity and neurodegeneration.
– Codon optimality-mediated mRNA degradation: Linking translational elongation to mRNA stability.

SETH SHATKIN MARGOLIS, PHD

- Professor of Biological Chemistry, Neuroscience
- Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
- Summary of Work: Dr. Margolis' lab focuses on protein homeostasis machinery (protein translation and protein degradation) that control neuronal function in development and disease.

Recent Projects:
- Neuronal membrane proteasome-derived peptides modulate NMDAR-dependent neuronal signaling to promote changes in gene expression
- Activity-dependent degradation of the nascentome by the neuronal membrane proteasome
- Deleting a UBE3A substrate rescues impaired hippocampal physiology and learning in Angelman syndrome mice

JON LING, PHD

– Professor of Pathology
– Disease Areas of Focus: Symptomatic treatment of dementia/ neurodegeneration
– Summary of Work: The Ling lab employs large-scale computational analyses of next-generation sequencing data to identify novel disease mechanisms and therapeutic strategies for neurodegenerative diseases.

Recent Projects:
– ASCOT identifies key regulators of neuronal subtype-specific RNA splicing.
– Splicing repression is a major function of TDP-43 in motor neurons.
– Identification of TDP-43 Cryptic Exons as Functional Fluid Biomarkers for Alzheimer's Disease and Related Dementia

HAI-QUAN MAO, PHD

– Professor of Materials Science and Engineering; Director, Institute of NanoBioTechnology (INBT)
– Disease Areas of Focus: Neurodegeneration
– Summary of Work: Dr. Mao's work focuses on developing novel biomaterials for therapeutic delivery through therapeutic engineering, regenerative engineering, and immunoengineering.

Recent Projects:
– Compositionally Defined Plasmid DNA/Polycation Nanoparticles and Methods for Making the Same
– Payload distribution and capacity of mRNA lipid nanoparticles.
– Multi-step screening of DNA/lipid nanoparticles and co-delivery with siRNA to enhance and prolong gene expression.
– Sustained IGF-1 delivery ameliorates effects of chronic denervation and improves functional recovery after peripheral nerve injury and repair

KENNETH WITWER, PHD

- Professor of Molecular and Comparative Pathobiology, Neurology
- Disease Areas of Focus: Schizophrenia, symptomatic treatment of dementia/neurodegeneration
- Summary of Work: Dr. Witwer's lab focuses on extracellular vesicles, RNA-mediated regulation, biomarker discovery, and therapeutic modulation of innate and intrinsic defenses in neurodegenerative diseases.

Recent Projects:
- Extracellular vesicles: The next generation in gene therapy delivery
- Extracellular vesicles from human plasma dampen inflammation and promote tissue repair functions in macrophages
- Engineered Extracellular Vehicles as a selective drug delivery platform

JUSTIN HANES, PHD

– Director, Center for Nanomedicine; Professor of Ophthalmology, Neurosurgery, Oncology, Pharmacology and Molecular Sciences
– Disease Areas of Focus: Symptomatic treatment of dementia/neurodegeneration
– Summary of Work: Dr. Hanes is internationally recognized for designing and synthesizing new biodegradable plastics to create nanoscopic, drug/gene-filled particles, capable of targeted delivery to specific sites in the body.

Recent Projects:
– Strategies to enhance the distribution of nanotherapeutics in the brain
– Entirely GRAS Material-based Large Brain-Penetrating Nanoparticles for Widespread Therapeutic Distribution in Healthy and Tumor-bearing Brain Tissues
– Method to Improve Brain Penetration of Nanoparticles