DELIVERY AND SPATIAL OMICS

Faculty Profiles

JOHNS HOPKINS UNIVERSITY

JOHNS HOPKINS TECHNOLOGY VENTURES

JOHNS HOPKINS: A HUB FOR RNA INNOVATION

With world-renowned faculty specializing in gene therapy delivery and editing technologies, Johns Hopkins is uniquely positioned as a hub for RNA innovation.

JHU INSTITUTES & DEPARTMENTS

- Institute of NanoBioTechnology (INBT)
- Biomaterials and Drug Delivery Laboratory
- Department of Genetic Medicine

Source: https://hub.jhu.edu/2022/08/23/gene-medicine-delivery-faster-affordable/

HAI-QUON MAO, PHD

Professor, Department of Materials Science and Engineering; Director, Institute of NanoBioTechnology (INBT)

AREAS OF SPECIALIZATION

Biomaterials, therapeutic delivery, regenerative, engineering, and immunoengineering.

SUMMARY OF WORK

Dr. Mao's work focuses on developing novel biomaterials for therapeutic delivery through therapeutic engineering, regenerative engineering, and immunoengineering.

Mao Lab Website

Mao Lab Publications

Mao's Patents

CURRENT PROJECTS

- Kinetically controlled polyelectrolyte nanoparticle assembly and its scalable manufacturing for delivery of biologic therapeutics
- Engineering polycation- and lipid-based non-viral nanoparticles for delivery of nucleic acid therapeutics via systemic, local, or oral administration

HAI-QUON MAO, PHD

PUBLICATIONS

- 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.
- Quaternary nanoparticles enable sustained release of bortezomib for hepatocellular carcinoma.
- Size-Controlled and Shelf-Stable DNA Particles for Production of Lentiviral Vectors.
- Scalable Purification of Plasmid DNA Nanoparticles by Tangential Flow Filtration for Systemic Delivery.
- Flash technology-based self-assembly in nanoformulation: from fabrication to biomedical applications.
- Surface-Functionalized PEGylated Nanoparticles Deliver Messenger RNA to Pulmonary Immune Cells.

JOHN LING, PHD

Assistant Professor of Pathology

AREAS OF SPECIALIZATION

RNA splicing, therapeutics, neuropathology, genetic engineering, gene therapy, bioinformatics.

SUMMARY OF WORK

Dr. Ling's research employs large-scale computational analyses of next-generation sequencing data to identify novel disease mechanisms, including splicing-based mechanisms, with the goal of translating these discoveries to the clinic.

Ling's Website

Ling's Publications

Ling's Patents

LICENSABLE TECHNOLOGIES

- Method for using alternative splicing to control specificity of gene therapy
- Target Validation of Splicing Repression, a Major Function of TDP-43 in the Motor Neuron

JOHN LING, PHD

PUBLICATIONS

- Payload distribution and capacity of mRNA lipid nanoparticles.
- Cell-specific regulation of gene expression using splicing-dependent frameshifting.
- ASCOT identifies key regulators of neuronal subtype-specific RNA splicing.
- Splicing repression is a major function of TDP-43 in motor neurons.
- Recount3: summaries and queries for large-scale RNA-seq expression and splicing.

SARAH WOODSON, PHD

T.C. Jenkins Professor of Biophysics at Johns Hopkins University

AREAS OF SPECIALIZATION

RNA folding, RNA dynamics, small regulatory RNAs, and ribosome assembly.

SUMMARY OF WORK

Dr. Woodson's research studies how RNA molecules fold into specific three-dimensional structures and how the RNA and proteins components of cellular complexes come together.

CURRENT PROJECTS - INVESTIGATING

- How conditions inside the cell alter the way the RNA folds.
- How new structures arise as the RNA sequence evolves.
- How small, non-coding RNAs (sRNA) and RNA chaperones act by base pairing with a target.
- Messenger RNA, decreasing or increasing translation of the target gene.

Woodson Lab Website

Woodson Lab Publications

SARAH WOODSON, PHD

PUBLICATIONS

- Small RNAs and Hfq capture unfolded RNA target sites during transcription.
- Direct observation of RNA structure and dynamics in repeat-RNA assemblies using single molecule fluorescence microscopy.
- Ribosomes clear the way for siRNA targeting.
- RNA toxicity and perturbation of rRNA processing in spinocerebellar ataxia type 2.
- Single-molecule FRET studies of RNA structural rearrangements and RNA-RNA interactions.
- Intrinsically disordered interaction network in an RNA chaperone revealed by native mass spectrometry.

GREGORY NEWBY, PHD

Assistant Professor of Genetic Medicine

AREAS OF SPECIALIZATION

Genome editing, base editing, molecular medicine, CRISPR-Cas9

SUMMARY OF WORK

Dr. Newby's research has been foundational to developing CRISPR-Cas9 base editing and prime editing. His current work focuses on using efficient genome editing tools to re-wire regulatory elements surrounding disease-associated genes and form the basis of new therapeutics.

Newby Lab Website

Newby Lab Publications

GREGORY NEWBY, PHD

PUBLICATIONS

- Efficient prime editing in mouse brain, liver and heart with dual AAVs.
- Shuttle Peptide Delivers Base Editor RNPs to Rhesus Monkey Airway Epithelial Cells In Vivo.
- Protospacer modification improves base editing of a canonical splice site variant and recovery of CFTR function in human airway epithelial cells.
- Cas9-based diagnostic assay and methods of using.
- Nonviral base editing of *KCNJ13* mutation preserves vision in a model of inherited retinal channelopathy.
- Adenine base editors and uses thereof.

JOSHUA MODELL, PHD

Assistant Professor of Molecular Biology and Genetics

AREAS OF SPECIALIZATION

CRISPR-Cas9 systems

SUMMARY OF WORK

Dr. Modell's work synthesizes genetic, genomic and cellular analyses to better understand the basic biology of CRISPR systems and to inform the next generation of CRISPR-based technologies.

Modell Lab Website
Modell Lab Publications

JOSHUA MODELL, PHD

PUBLICATIONS

- A natural single-guide RNA repurposes Cas9 to autoregulate CRISPR-Cas expression.
- CRISPR–Cas systems exploit viral DNA injection to establish and maintain adaptive immunity.
- Viral recombination systems limit CRISPR-Cas targeting through the generation of escape mutations.