# RNA AND SPATIAL TRANSCRIPTOMICS

## Faculty Profiles

JOHNS HOPKINS UNIVERSITY

JOHNS HOPKINS TECHNOLOGY VENTURES

# BETHANY POWELL GRAY, PHD

*Assistant Professor, Pharmacology and Molecular Sciences; Assistant Professor, Oncology*

## AREAS OF SPECIALIZATION

Aptamers, RNA therapeutics, pharmacology, nucleic acid chemistry

## SUMMARY OF WORK

Dr. Gray's work in RNA therapeutics focuses on:

– Novel and stable RNA aptamers against disease targets

– E3 RNA aptamer with selective uptake in prostate cancer

– Tunable cytotoxic aptamer-drug conjugates in prostate cancer

# RANJAN PERERA, MS, PHD

*Assistant Professor, Pharmacology and Molecular Sciences; Assistant Professor, Oncology*

## AREAS OF SPECIALIZATION

lncRNA, miRNA, melanoma, medullablastoma.

## SUMMARY OF WORK

Dr. Perera's work in RNA therapeutics focuses on:

- LncRNAs and their endogenous binding partners: SPRIGHTLY-PTBP1
- miRNAs as metabolic switch and therapeutic target in melanoma: MIR211 in melanoma
- miRNA as regulators of disease-specific expression: MIR211 in tumor microenvironment and MIR196 in adipose tissue
- lncRNAs as therapeutic modality: Lnc-HLX-2-7 in pediatric medulloblastoma
- Circular RNAs as therapeutic target: circRNA Edis

# SHUYING SUN, PHD

*Associate Professor, Physiology; Associate Professor, Neuroscience; Associate Professor, Pathology*

## AREAS OF SPECIALIZATION

RNA biology, RNA metabolism, RNA-mediated toxicity, neurodegeneration.

## SUMMARY OF WORK

Dr. Sun's work in RNA therapeutics focuses on:

- Antisense oligonucleotides against repeat-containing RNAs: ALS, frontotemporal degeneration (FTD)
- Proteins that modulate RNA phenotypes: DDX3x, RNA helicase
- Binding and mechanisms of splicing modulators (proteins) for therapeutic development: SRSF1, RBFOX1/2

# SEYED FATEMI, MD, MBA

*Professor, Neurology; Professor, Pediatrics; Chief Medical Officer, Kennedy Krieger Institute; Director, Division of Neurogenetics*

## AREAS OF SPECIALIZATION

Neurogenetics, cerebral palsy, therapeutic development.

## SUMMARY OF WORK

Dr. Fatemi work in RNA therapeutics focuses on:

– Antisense oligonucleotides against rare diseases

# SHAWN LUPOLD, PHD

*Catherine Iola and J. Smith Michael Distinguished Professor of Urology; Co-Director, The Sidney Kimmel Comprehensive Cancer Center Prostate Cancer Program; Professor of Urology; Assistant Professor of Radiation Oncology and Molecular Radiation Sciences; Professor of Oncology*

## AREAS OF SPECIALIZATION

Urological oncology, microRNAs, siRNAs, gene/drug delivery, nanoparticles.

## SUMMARY OF WORK

Dr. Lupold's work in RNA therapeutics focuses on:

- Modulating RNA via microRNAs in the prostate cancer phenotype
- Modulating RNA via targeted siRNAs
- Modulating cancer ligands through RNA aptamers

# TED DEWEESE, MD, PHD

*Dean of the Medical Faculty and CEO, Johns Hopkins Medicine*

## AFFLIATIONS

The Sidney Kimmel Professor of Radiation Oncology and Molecular Radiation Sciences; Vice President for Interdisciplinary Patient Care, Johns Hopkins Medicine; Radiation Oncologist -in-Chief; Professor of Radiation Oncology and Molecular Radiation Sciences; Professor of Oncology; Professor of Urology

## AREAS OF SPECIALIZATION

Bladder cancer, prostate cancer, urological oncology, radiation oncology, cancer-targeted RNA, androgen receptor signaling

## SUMMARY OF WORK

Dr. DeWeese's work in RNA therapeutics focuses on:

- siRNA-targeting DNA repair protein is combined with an aptamer targeting the prostate-unique protein, prostate specific membrane antigen (PSMA).
- The chimeric siRNA-aptamer that is a potent, targeted radiosensitizer with potential for translation into prostate cancer therapy.

# JEFF COLLER, PHD

*Bloomberg Distinguished Professor RNA Biology and Therapeutics; Professor, Molecular Biology and Genetics*

## AREAS OF SPECIALIZATION

RNA therapeutics, mRNA translation, mRNA stability.

## SUMMARY OF WORK

Dr. Coller's work in RNA therapeutics focuses on:

- Modulating RNA levels: Tethered mRNA amplifier
- Modulating RNA stability: Codon optimization
- Modulating RNA translation efficiency: Acetylation of cytidine and mRNA deadenylation via CCR4

# BIN WU, PHD, MPHIL

*Assistant Professor of Biophysics and Biophysical Chemistry; Assistant Professor of Neuroscience*

## AREAS OF SPECIALIZATION

RNA biology and RNA life-cycle.

## SUMMARY OF WORK

Dr. Wu's work in RNA therapeutics focuses on:

- A rapid inducible RNA decay system (RIRD)
  - Works ~30min vs 2-3h using siRNA (in collaboration with JHU's Takanari Inoue)

# JIOU WANG, MD, PHD

*Walder Distinguished Professor, Biochemistry and Molecular Biology*

## AREAS OF SPECIALIZATION

RNA biology, RNA homeostasis, neurodegeneration.

## SUMMARY OF WORK

Dr. Wang's work in RNA therapeutics focuses on:

- RNA binding proteins involved in miRNA gene silencing
  - Fused in sarcoma (FUS) regulates microRNA mediated gene silencing

# REBECCA SHULMAN, PHD

*Associate Professor, Chemical and Biomolecular Engineering and Computer Science; Kent Gordon Croft Investment Management Faculty Scholar*

## AREAS OF SPECIALIZATION

Biomedical engineering, transcription, nanobiology, synthetic biology.

## SUMMARY OF WORK

Dr. Shulman's work in RNA therapeutics focuses on:

- RNA aptamer-regulated transcription
- Assembly or disassembly of DNA/RNA components

# ALEKSANDER POPEL, PHD

*Director, Systems Biology Laboratory; Professor of Biomedical Engineering, Professor of Medicine; Professor of Oncology*

## AREAS OF SPECIALIZATION

Immunoengineering, controlled drug delivery, autoimmune and cancer disease models.

## SUMMARY OF WORK

Dr. Popel's work in RNA therapeutics focuses on:

- Design and development of therapeutic peptides
  - Biomimetic peptides for treatment of angiogenesis-dependent diseases
- Integrating transcriptional and proteomic data
  - Reduced invasion after GFPT2 shRNA knockdown in hepatic cellular carcinoma (in collaboration with P Tran, E Fertig, A Ewald at JHU)

# JOSH DOLOFF, PHD

*Assistant Professor, Biomedical Engineering; Assistant Professor, Materials Science and Engineering*

## AREAS OF SPECIALIZATION

Therapeutic peptides, systems pharmacology, angiogenesis.

## SUMMARY OF WORK

Dr. Doloff's work in RNA therapeutics focuses on nanoparticle delivery and includes methods for:

- Endothelial delivery of siRNA
- Reprogramming tumors through targeted mRNA delivery
- Self-amplifying mRNA (SAM) as vaccine strategy

# SCOTT WILSON, PHD

*Assistant Professor, Biomedical Engineering*

## AREAS OF SPECIALIZATION

Immunoengineering, biomedical engineering, biomaterials.

## SUMMARY OF WORK

Dr. Wilson's work in RNA therapeutics focuses on:

- Programmable cytokine-targeted siRNA localizing to sites of inflammation for gene regulation
  - TNFalpha-siRNA to inhibit inflammation in the intestines

# RELEVANT JHU INSTITUTES AND CENTERS

## JOHNS HOPKINS DRUG DISCOVERY

The Program works with researchers across Johns Hopkins Medicine to research and develop tomorrow's therapeutics for a wide range of human disorders including drug discovery projects in Oncology, Immunology, Neurology, Psychiatry, Ophthalmology, and Gastrointestinal disorders.

## SIDNEY KIMMEL COMPREHENSIVE CANCER CENTER

Within SKCCC, clinicians and research scientists work closely together to develop new drugs and therapies to provide patients with cutting edge options for cancer care.

## CHEMICAL AND BIOMOLECULAR ENGINEERING

The department is known for making an impact through innovative engineering research across a variety of disciplines including drug development and delivery.

## INSTITUTE OF NANOBIOTECHNOLOGY (INBT)

The INBT is a multidisciplinary team of faculty, researchers, and student experts uncovering new knowledge and creating innovative technologies at the interface of nanoscience, engineering, biology, and medicine