The Friedman Brain Institute
2023 FBI Research Scholars

On behalf of the Philanthropic Leadership Council of The Friedman Brain Institute, we are pleased to announce the 2023 recipients of The FBI Research Scholars Awards.

Zhao Research Scholar Award

Michael S Breen, PhD
Assistant Professor,
Genetics and Genomic Sciences

Makiko Yasuda, MD, PhD
Associate Professor,
Genetics and Genomic Sciences

A novel in vivo reporter model to assess specificity and efficacy of RNA editing therapy and delivery tools

A breakthrough in the field of RNA therapeutics has been the discovery of antisense oligonucleotides (ASOs) that recruit the cell's own machinery to repair disease-causing mutations. Here, rationally designed ASOs can reprogram a unique class of RNA editing enzymes, termed adenosine deaminases acting on RNAs, to target a select RNA molecule and nucleotide of interest and 'edit' to repair nonsense mutations on pre-mRNA. This project is designed to generate a landmark in vivo reporter mouse model containing a disrupted eGFP harboring an in-frame stop codon that will fluoresce only after correction. By coupling our ASO approach with a burgeoning selection of gene delivery tools, this model is poised to accelerate our understanding of the specificity, efficiency, tissue tropism and biodistribution of RNA editing therapeutics across diverse modes of delivery.

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