# July Updates: People and Places

### Alzheimer's and aging at JAX

**Catherine Kaczorowski,** an assistant professor at the Jackson Laboratory in Bar Harbor, ME, has been awarded a $2.7 million dollar, five-year grant from the NIH National Institute on Aging to study healthy aging and Alzheimer's disease. Kaczorowski's lab will work with the BXD panel of mice, a set of genetically diverse inbred strains designed to model the genetic complexity of human populations, with the goal of identifying gene variants related to memory function and cognition and ultimately, targets for treating Alzheimer's.

"Identifying novel genetic factors and mechanisms of memory decline will be a critical first step toward developing treatments and personalized gene therapies to maintain cognitive function in elderly humans," Kaczorowski says. "And they would also have the tremendous potential to provide biomarkers for earlier detection of Alzheimer's, which may mean more effective treatment in Alzheimer's patients."

### LNP technology and ALS research at CRISPR Therapeutics

**CRISPR Therapeutics** of Basel, Switzerland, has announced their exclusive license of a family of Lipid Nanoparticle (LNP) Therapies from the Massachusetts Institute of Technology. The LNP technologies were developed in the lab of Daniel Anderson, who is a scientific founder and advisory board member of the company. Anderson said in a press release, "Our laboratory has worked to develop non-viral delivery vectors for nucleic acids, and we are finding that LNP technologies work very well for delivering the CRISPR-Cas9 system. In fact, gene editing applications may be particularly well-suited to LNP delivery given the potential for single dose efficacy. I am excited to see CRISPR-based *in vivo* therapies moving rapidly toward the clinic."

While the primary focus coming from the MIT license will be for treating liver indications, CRISPR Therapeutics is also looking to the central nervous system with a two-year grant from the Target ALS Foundation. The company and academic collaborators Laura Ranum and Eric Wang of the University of Florida will test CRISPR-Cas9 gene-editing strategies in animal models of ALS and frontotemporal dementia. Chad Cowan, the company's head of research, commented, "We are delighted to partner with Dr. Ranum and Dr. Wang to translate our *in vivo* gene-editing platform into potential therapies that address the underlying cause of ALS and FTD. The advances we make on ALS could pave the way for CRISPR-Cas9-based therapies in other CNS indications as well."

### SBIR to Spero for tuberculosis

**Spero Therapeutics** of Cambridge, MA, has received a $564,718 Small Business Innovation Research Program grant from the NIH to continue preclinical studies of SPR720, a novel oral bacterial gyrase inhibitor that may treat tuberculosis (TB). CEO Ankit Mahadevia commented in a press release, "We are pleased with the progress we have made in characterizing the utility of SPR720 in non-tuberculous mycobacteria since acquiring the compound last year and we are excited to expand our research to TB." Spero will collaborate with the Central New York Research Corporation to test safety and efficacy of their candidate in combination with existing TB treatments in drug-susceptible and multidrug resistant animal models.

### On to bigger eyes

**EyeCRO**, headquartered in Oklahoma City, OK, and **MPI Research** of Mattawan, MI, will collaborate to develop and commercialize large mammalian preclinical models of ophthalmic diseases such as Age-Related Macular Degeneration and Diabetic Retinopathy. MPI's Director of Opthalmology Services, Josh Bartoe, praised EyeCRO's preclinical expertise in developing animal models, while EyeCRO CEO Rafal Farjo commented on MPI's track record in preclinical and clinical ophthalmological drug and device development.

In the same press release, EyeCRO announced the opening of a new R&D site in Ann Arbor, MI, 90 minutes from MPI's headquarters. The facility will offer 3500 square feet of wet-research space for model development and translational research with nearby clinicians. The company will seek full AAALAC accreditation for the new laboratory.

## CAREERS UPDATE

**David Sabatini** has been awarded the fifth annual Lurie Prize in Biomedical Sciences from the Foundation for the National Institutes of Health (FNIH) for his discovery of the mTOR cellular pathway and research into its role in mammalian growth, metabolism, and aging. Sabatini, a member of the Whitehead Institute for Biomedical Research, biology professor at the Massachusetts Institute of Technology, and Howard Hughes Medical Institute investigator, received the award and a $100,000 honorarium on May 17.

The Lurie Prize is awarded in recognition of achievements by biomedical scientists under 52 years old. Ann Lurie, a philanthropist, FNIH board member, and namesake of the award, commented in a press release, "The Lurie Prize in Biomedical Sciences was designed to empower young biomedical researchers so they can make further advancements that lead to life changing discoveries. Dr. Sabatini's work has the potential to do just that, by revolutionizing how we treat age-related diseases."