

INVESTIGATOR SPOTLIGHT: Steven Gray, PhD, University of North Carolina at Chapel Hill

This month's installment would like to move the Investigator spotlight to Dr. Steven Gray a young faculty member of the University of North Carolina at Chapel Hill. Dr. Gray is a Research Assistant Professor in the Department of Ophthalmology. Dr. Gray's main interests lie in gene therapy approaches for CNS disorders, and his core expertise is in Adeno-Associated Virus (AAV) gene therapy vector engineering and finding ways to optimize gene delivery to the central and peripheral nervous system.

Dr. Gray received his PhD in Molecular Biology from Vanderbilt University in 2006. He then trained as a postdoctoral fellow and was promoted to research associate in the laboratory of Dr. Jude Samulski of the Gene Therapy Center prior to his new faculty appointment at UNC. In the Samulski lab, he became an independent

investigator working on gene therapy approaches for Rett syndrome. He has long been supported by IRSF (and previously RSRF). He had received a Postdoctoral Fellowship in 2008 and was able to secure several translational research HeART awards for his work in generating better AAV vectors to safely deliver the normal MECP2 gene to the brain.

This year Dr. Gray continues to expand avenues of gene therapy for Rett syndrome, and he was awarded a 2012 HeART grant for the project entitled "MeCP2 gene transfer using novel RTT-specific rAAV vectors". IRSF is proud that our Training Program was able to recruit such a talented young investigator to work on Rett syndrome research who remains dedicated to finding new treatments and a cure as he moves forward in his career.

What prompted you to begin a career in research?

I had a strong interest in research and molecular biology since high school. At the beginning of my freshman year of college I started working in a laboratory, and after running my first gel to see DNA I was hooked.

Provide a brief outline of your training and the work you have conducted that has led to this proposal.

I got a B.S. in molecular biology at Auburn University, where I worked sequentially in 2 labs for 4 straight years while taking classes. After that I got a PhD in molecular biology at Vanderbilt University studying DNA replication and chromosome structure/function. My postdoctoral training was with Jude Samulski in the Gene Therapy Center at the University of North Carolina at Chapel Hill. My training and research as part of Dr. Samulski's group, and also as an independent investigator, has been to optimize gene delivery to the central nervous system using AAV vectors. Part of my work involves the engineering of better AAV vectors, and the other part of my work involves developing and testing therapeutic approaches for neurological disorders. My current project is a focused marriage of these 2 general avenues of research, to use novel AAV vectors tailored towards Rett syndrome and to deliver a therapeutic gene for Rett Syndrome.



What is the single most rewarding aspect of conducting Rett syndrome research?

The possibility of developing a therapy which could change the lives of these patients and their families.

Identify a potential positive outcome of the research you are conducting that is specific to this proposal.

This proposal has the potential to globally treat many aspects of Rett syndrome. The ultimate goal of these and future studies is to provide functional MeCP2 to the entire CNS, which has the potential to impact any number of Rett symptoms.

If you could pick any one symptom of Rett syndrome to prevent or to provide relief for, what would it be?

I would leave this to the families to answer. My simple answer for practical reasons is that we'll test global therapies that could restore MeCP2 function, and it is an open question as to what effect this will have.

What other diseases does your research focus on?

Giant Axonal Neuropathy, Batten Disease, Krabbe Disease, Epilepsy, AGU, and Tay-Sachs Disease.

Besides your role as principal investigator on this project and as a Rett syndrome investigator, what other roles do you currently hold that are specific to the field of Rett syndrome research?

IRSF Grant Reviewer (on Scientific Review Board)

Provide any other interesting information about yourself or your work that you would like the Rett syndrome community to know about you.

I have a strong connection to some of the affected families that I have developed through the course of my research, in particular with Giant Axonal Neuropathy. Since several of the projects I'm working on are preclinical in nature, getting involved with these families and sharing the hope of clinical translation with them has changed my life. My passions are my family and my research (and usually Auburn football in the fall but not this year), and long-distance running allows me to meditate and stay sane.

Read more about Dr. Steven Gray and his project:

<http://www.med.unc.edu/opth/meet-our-faculty/research-faculty/steven-gray-phd>

<http://rettsyndrome.org/research-programs/funded-projects/research-awardees-2012#Gray>

For a list of Dr. Gray's publications, please visit:

<http://www.ncbi.nlm.nih.gov/pubmed/?term=gray+sj>