Game Changer for Future Ataxia Treatments

Hayley McLoughlin, PhD, co-first author of the recently published article "Oligonucleotide therapy mitigates disease in Spinocerebellar Ataxia Type 3 mice" in *Annals of Neurology*.

Dr. Hayley McLoughlin is a Research Investigator in the department of Neurology at the University of Michigan. She received her PhD in Neuroscience from the University of Iowa in 2013 under the mentorship of Dr. Beverly Davidson. From 2014-2016, she completed postdoctoral work with Dr. Henry Paulson at the University of Michigan. Both Dr. Davidson and Dr. Paulson are members of the National Ataxia Foundation's Medical Research Advisory Board.

Dr. McLoughlin's studies focused on gene therapy approaches for Spinocerebellar Ataxia type 3/Machado-Joseph disease (SCA3/MDJ). In the fall of 2016, she joined the faculty of the Neurology Department at the University of Michigan, where she is continuing her research focused on therapeutic strategies and biomarker discovery for neurodegenerative diseases.

Dr. McLoughlin is no stranger to the National Ataxia Foundation. At the 2016 Ataxia Investigators Meeting (AIM), she was an invited speaker in which she presented: *Antisense Oligonucleotide Therapy for SCA3.* And again, at the 2018 AIM, Dr. McLoughlin gave an oral presentation: *Antisense Oligonucleotides provide therapeutic benefit in SCA3.*

When asked about her experience attending the AIMs, Dr. McLoughlin said, "AIM meetings are a unique opportunity to bring the best of the ataxia investigators and their trainees from around the world together to share our progress in the field. It is an exciting event to be a part of and always invigorates my studies with innovative ideas and collaborations. I am especially grateful for the multiple opportunities the AIM provides to interact with patients and families, which helps give both a face and a purpose to my research goals."

The National Ataxia Foundation is committed to bringing in new early-career researchers into the ataxia research field, such as Dr. McLoughlin. Last fall, the research grant application that Dr. McLoughlin submitted to NAF was reviewed and scored very highly. In January of 2018, she was awarded a National Ataxia Foundation SCA Young Investigator grant to seek SCA3 biomarkers through proteomic and transcriptional profiling.

When asked to tell us what led her to go into ataxia research and about her future plans, Dr. McLoughlin replied, "My path to ataxia research was initially based on the research focus of my exceptional thesis mentor, Dr. Davidson. While my graduate studies weren't directly involved in the ground breaking translational work Dr. Davidson is known for, I soaked up gene therapy training in her lab and sought a postdoc opportunity to apply what I had learned. In 2014, I had the fortune to join Dr. Paulson's group and lead studies on a gene silencing approach for SCA3. The exciting results of this preclinical work were recently reported in *Annals of Neurology*. While my future as an early-stage investigator is just beginning, I plan to continue developing the gene silencing technologies in collaboration with Dr. Paulson and our pharmaceutical partners, while leveraging the support of the NAF Young Investigator Award to develop

platforms for my future independent research laboratory, focusing on drug and biomarker discovery for SCAs and molecular mechanisms of neurodegeneration."

In 2018, Dr. McLoughlin was awarded NINDS CREATE Bio U01 funding to lead a collaboration with Ionis Pharmaceuticals in developing antisense oligonucleotide therapy for SCA3. While this collaboration focuses on SCA3, there are other partnerships being developed with Ionis that will explore ASO therapy for other SCAs.

The National Ataxia Foundation congratulates Dr. McLoughlin in this important discovery and we look forward to following her career. To learn more about the study and the publication in the *Annals of Neurology*, click on this link.