Neurofibromatosis Therapeutic Acceleration Program

FALL 2018 - NEWS AND NOTES FROM THE FRANCIS S. COLLINS SCHOLARS PROGRAM

In 2014, the Neurofibromatosis Therapeutic Acceleration Program (NTAP) at Johns Hopkins established the Francis S. Collins Scholars Program in Neurofibromatosis Clinical and Translational Research, with the goal of fostering the next generation of outstanding NF1 focused clinician-scientists that will revolutionize the field of NF1.

NTAP named this award after Francis S. Collins, M.D, Ph.D., who as an exemplary physician-scientist led one of the teams that discovered the NF1 gene in 1990. The Collins Scholars Program is focused on creating a community of exceptionally well-trained clinician-scientists who are ready to make a career long commitment to NF1 research and clinical care, and is intended for applicants who are either in the last stages of their post-doctoral training or within the first seven years of a faculty appointment. Applicants do not need to have any prior NF1 experience, and are selected based on their past accomplishments in addition to their ability to explore new ideas, their commitment to patient-centered research, and their potential to be thought leaders in the field of NF1. There are now nine exceptional clinician-scientists who are Collins Scholars, who each bring a unique skill set and diversity of experience that will position them well as future leaders in the NF field. While some were already involved in NF programs prior to be becoming Collins Scholars, others were 'recruited' to NF. The professional backgrounds of the Collins Scholars are diverse, and include pediatrics, oncology, orthopedic surgery, genetic counseling, and psychology. The projects from each of the Collins Scholars are likewise diverse encompassing translational and clinical research across plexiform neurofibroma, MPNST, cutaneous neurofibroma, and optical pathway glioma, with respect to drug therapy trials, genetic characterization studies, imaging studies, biomarker development, natural history, and neurocognitive studies.

While the Collins Scholars and their projects are varied, what is shared is focus on the clinical translation of excellent science to improve the lives of people living with NF1. In four years, the projects have yielded 25 publications, and have been included in 5 different clinical studies which are helping to accelerate the development of therapies for NF1. To date, four Collins Scholars have 'graduated' from the program. Importantly, each of them are holding roles of leadership in their respective institutions and in the NF field, have generated follow on funding from additional sources, and are actively training other clinicians and scientists in NF. True to the overarching mission of the Collins Scholars program, they represent the next generation of leaders in NF. As commented on by the scholars:

"The Francis S. Collins Scholars program was an early catalyst for my career development. Not only did the FCS program solidify my interest in NF1, it also generated an administrative thrust around me that I've successfully leveraged to establish a translational NF program."

"The protected time provided by this award enabled me to manage patient care responsibilities and devote time to research and professional development in a way that otherwise would not have been possible."

Now seeking applications for Francis Collins Scholars for 2019!

The 2019 call for applications for the Francis S. Collins Scholars Program in Neurofibromatosis Clinical and Translational Research will start on October 22, 2018. To learn more and download the application to apply, click here >> Please encourage any clinician scientists in your program, university or institution that you feel have the characteristics described above to apply! The deadline for application submission is March 15, 2019. We look forward to hearing from you!

NTAP celebrates the Collins Scholars

On August 3, 2018 The Neurofibromatosis Therapeutic Acceleration Program at Johns Hopkins (NTAP) was honored to host an intimate gathering at the Rock Hill Mansion in Bethesda, Maryland, to celebrate the talented clinician-scientists who have received the prestigious Francis S. Collins Scholars Program Award. In attendance were the Collins Scholars, selected mentors, and special guests, including Dr. Francis Collins, Director of the National Institutes of Health, for which the Collins' Scholars Program Award is named. Dr. Collins directly addressed the group, highlighting important events that shaped his scientific and clinical career, with words of advice to guide the scholars as they embark on their NF1 focused careers. This event included brief presentations from each scholar, and lectures from Dr. Dominic Esposito (Director of Protein Expression, Frederick National

Cancer Laboratories), Dr. Sara Gosline (Senior Scientist, Sage Bionetworks), and Dr. Brigitte Widemann (Director of Pediatric Oncology, and the Pharmacology and Experimental Therapeutics Section, NCI). The event concluded with the recognition of Scholars completing their terms, and introduction of the 2018 Collins Scholars.

Two new clinician-scientists chosen as Francis Collins Scholars

The Neurofibromatosis Therapeutic Acceleration Program at Johns Hopkins (NTAP) is proud to announce that Dr. Shruti Garg, of the University of Manchester, and Dr. Ina Ly, of Massachusetts General Hospital, were selected as the 2018 recipients of the Francis S. Collins Scholars Program Award in Neurofibromatosis Clinical and Translational Research. Read More >>

Two Francis Collins Scholars complete terms

This year marked the completion of the Collins Scholars Program for Dr. Miriam Bornhorst (Children's National Medical Center), and Dr. Verena Staedtke (Johns Hopkins), who each started their respective terms as Collins Scholars in 2015. As a Collins scholar, Dr. Bornhorst's research project explored the development of preventative therapy approaches for optic pathway gliomas, where the learnings from this work have provided an improved understanding of how these tumors form and clues for the early detection and management of these tumors in patients. Dr. Bornhorst is continuing her work in NF, now as the recently appointed Clinical Director of the Gilbert Neurofibromatosis Institute at Children's National Medical Center. In 2018, Dr. Bornhorst received a Center for Cancer and Immunology Research (CCIR) Collaborative Pilot Grant Program (CPGP) award as co-PI, and published in Neurooncology. Dr. Staedtke's research project has spearheaded the use of the therapeutic "anti-cancer" agent Clostridium novyi-NT, a genetically modified anaerobic bacterium, for the use in nervous system cancers, particularly glioblastomas and malignant peripheral nerve sheath tumors. Dr. Staedtke has helped advance this treatment to clinical evaluation, where it represents a highly novel approach for the treatment of cancers. Dr. Staedtke is continuing her work in NF as Director of the Pediatric Neurofibromatosis Program in the Johns Hopkins Comprehensive Neurofibromatosis Center. Moreover, Dr. Staedtke has recently been awarded funding to continue her research (through the Developmental Hyperactive Ras Tumors (DHART) SPORE, the Department of Defense (DoD) Neurofibromatosis New Investigator Award, and a NCI K08 award), and has had three publications thus far from her time as a Collins Scholar.

Collins Scholar contributes cutaneous neurofibroma targeting efforts

The targeting of cutaneous neurofibromas represents a major initiative for NTAP, and the Collins Scholars program plays an important role in this area. Dr. Ashley Cannon (FCS, 2016-present, University of Alabama) has been exploring the quantitative natural history of cutaneous neurofibromas. She recently published findings from these studies in Orphanet Journal of Rare Diseases. Dr. Cannon was also a lead author of a manuscript focused on clinical trial design for cutaneous neurofibromas published in Neurology, and is currently co-leading the cutaneous neurofibroma REiNS (Response Evaluation in Neurofibromatosis and Schwannamatosis) committee, focused on developing new standardized response criteria for determining treatment response in patients with NF1 cutaneous neurofibroma.

Promotion of Dr. Ping Chi

In 2018, Dr. Ping Chi (FCS, 2017-present) was promoted to Associate member in the Human Oncology and Pathogenesis Program (HOPP), in the Department of Medicine at Memorial Sloan Kettering Cancer Center (New York City, New York). Dr. Chi has an active translational research program focused on developing therapeutics for targeting MPNSTs. To learn more about Dr. Chi's NTAP project, click here >>

From Washington University to Washington D.C. (and Baltimore)

In the summer of 2018, Dr. Angela Hirbe (FCS, 2017-present, Washington University) spent 3 weeks away from her native St. Louis and home institution Washington University, to perform clinical rotations at Children's National Medical Center, the National Cancer Institute, and Johns Hopkins Hospital. During this time, Dr. Hirbe was able to see NF1 patients alongside fellow NF1 clinicians, meet with a variety of scientific and clinical experts at these institutions in order to develop collaborations, and deliver seminars at these institutions on her group's research.

Collins Scholars Program 'Alumni' focused on NF1

As 'alumni' of the Collins Scholars program, Dr. Peter de Blank (FCS, 2014-2017, Cincinnati Children's Hospital Medical Center) and Dr. Matt Steensma (FCS, 2014-2017, Van Andel Institute, Spectrum

Health) continue to actively grow their careers as leaders in the NF1 field. Dr. de Blank is continuing his work in developing non-invasive biomarkers for NF1 optical pathway glioma, including his project with NTAP. In 2018, Dr. de Blank published recent results in **Experimental Neurology,** and received an extended scholars award from the St. Baldrick's Foundation, and an award from Curing Kid's Cancer. Dr. de Blank has also been serving as the Vice Chair of CONNECT (Collaborative Network for NeuroOncology Clinical Trials). Dr. Steensma has continued an active NF1 research focused program where in 2018, results from his laboratory highlighting potential therapeutic strategies for treating genomically diverse MPNSTs were published in **Cancer Research.** Another key feature of Dr. Steensma's work has included his ongoing oversight of a tissue bank at the Van Andel Institute which includes samples of many types of neurofibromas, which has served as an invaluable tool for NF researchers.