



Center for Clinical and Translational Science e-NEWSLETTER

Center News

Clinical Scholars Program Celebrates New Graduates

By Editorial Staff

Nine graduating Clinical Scholars received Masters' of Clinical and Translational Science degrees at a dinner celebrating them and their mentors on June 6, 2019.

Dr. Chibuzo Enemchukwu

Mentors: Dr. Charles Rice & Sarit Golub

Dr. Enemchukwu's research focuses on improving access and delivery of evidence-based HIV prevention services in health disparity populations. She is currently leading an NIH funded Einstein-Rockefeller-CUNY Center for AIDS Research supplement project that works in collaboration with the New York City Department of Health to examine strategies for engaging justice-involved women into HIV testing, prevention, and care services.

Dr. Enemchukwu is a Senior Research Scientist in the Hunter Alliance for Research and Translation (HART) laboratory at Hunter College; and she is a Senior Consultant at Nelu Diversified Consulting Solutions LLC., a company she



Front row: Drs. Barry Collier, Michel Nussenzweig, Ching-Lan Lu, Moonjung Jung, Kathrine Myers, Youngmin Lee, Sarit Golub, and Chibuzo Enemchukwu **Back row:** Drs. James Krueger, Agata Smogorzewska, Sarah Schlesinger, Ethan Ravetch, Norihiro Yamaguchi, Scott Drutman, Jason Hawkes, Scott Friedman, and Charles Rice

co-founded that works with employers to strategize and create inclusive health and financial benefits programs that reach an increasingly diverse workforce.

Dr. Scott Drutman

Mentor: Dr. Jean-Laurent Casanova

Dr. Drutman's research focused on the investigation of the genetics of susceptibilities to influenza virus, cytomegalovirus, and human papillomavirus. Dr. Drutman is Associate Director, Clinical Sciences, Oncology at Regeneron.

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Dinner with Dr. Scott Gottlieb, Former 23rd Commissioner of the FDA

By Tobias Becher, MD

Clinical Scholars and Faculty had the unique opportunity to meet Dr. Scott Gottlieb, former Commissioner of the FDA on June 26th for a dinner event. Dr. Gottlieb received his bachelor's degree in economics from Wesleyan University and his M.D. from the Icahn School of Medicine in 1999. After completing his residency in Internal Medicine at Mount Sinai Medical Center in New York, he joined the Food and Drug Administration (FDA) as senior advisor to the Commissioner and then as Director of Medical Policy Development from 2002 to 2003. He



Members of the Center for Clinical and Translational Science leadership, Scholars, and Rockefeller University Early Phase Physicians Scientists with Dr. Gottlieb

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Cerner Millennium Electronic Infrastructure and Electronic Health Record (EHR) Being Installed in The Rockefeller University Hospital

By Prasanth Manukonda, MS, MA and Maija Williams, MPH

The Rockefeller University Hospital is in the process of implementing Cerner Millennium as our electronic infrastructure and Electronic Health Record (EHR). The hospital devoted more than three years to assessing different systems in order to identify one that is the very best fit for Rockefeller's unique structure. One of the key features that made Cerner Millennium the unanimous choice of the review team is its HIPPA-compliant clinical support application termed PowerTrials, which is fully integrated into Cerner's EHR (Power Chart). PowerTrials efficiently organizes and maintains protocol enrollment data on each research participant according to the study in which they are enrolled, facilitating oversight of Rockefeller's entire research portfolio. PowerTrials also has a prescreening function that can be used to search PowerChart for potentially eligible research participants using pre-established recruitment

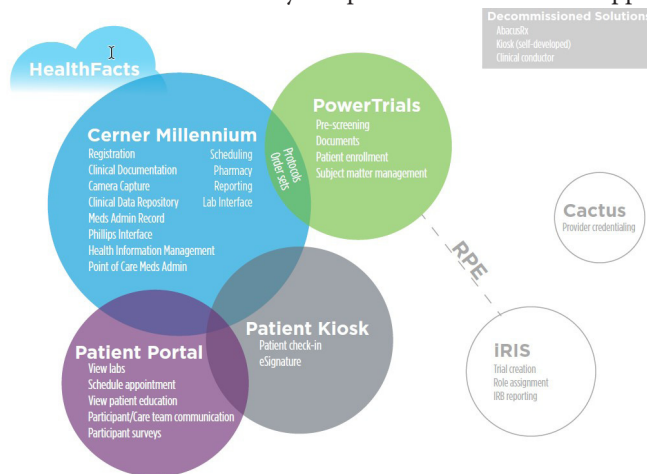
criteria. In addition, other extremely valuable components of Cerner Millennium include: 1. Sophisticated research participant scheduling system, which is applicable to both inpatients in the Rockefeller University Hospital and outpatients in the Heilbrunn Outpatient Center; 2) Support of bionutritional studies, and 3. Advanced pharmacy system, including physician order entry and formulary control. Other features of the Cerner system that impressed the review team were superior functionality, ease of use, and ease of customization.

Most importantly, it will provide a secure patient portal for bidirectional exchange of information between research participants and investigators. One of the highest priorities of our research program is to engage patients in all phases of the research process, from setting priorities and designing and executing studies, to returning data about the study to participants and

understanding of the broad implications of the results of the studies. The Patient Portal will enhance our ability to achieve all of these goals.

Implementation of the Cerner system began in September and is scheduled to take approximately 9 months, with a "go live" scheduled for April 2020. Roll out will include intense training of staff, which has already begun, and extensive testing prior to implementation. Once Cerner Millennium is fully implemented it will provide a truly state-of-the-art electronic infrastructure to support clinical investigation at Rockefeller far into the future. We are confident that it will improve quality and efficiency, streamline operations, and promote patient safety, as well as increase our ability to participate in activities of the Clinical and Translational Science Award (CTSA) program.

Future State of Rockefeller University Hospital EHR and Clinical Application Framework



Cerner EHR implementation - Timeline and Major Milestones

Date	Event
Sept 25, 2019	Project Kick Off
Oct 14, 2019	Workflow and Integration
Dec 09, 2019	Train the trainer
Jan 20, 2020	Integration Testing 1
Mar 16, 2020	Integration Testing 2
Apr 20, 2020	Physician Concierge
Apr 27, 2020	Go Live

New Clinical Scholars Join the Center for Clinical and Translational Science

By Editorial Staff

On July 1, 2019, four new Clinical Scholars joined the Rockefeller University Clinical Scholars Program. They are Drs. Dana Bielopolski, Rachel Niec, Yael Renert-Yuval, and Jeffrey Wong.



Dana Bielopolski, MD, PhD

Mentors: Drs. Rhonda Kost and Jonathan Tobin

Laboratory: Allen and Frances Adler Laboratory of Blood and Vascular Biology

Research Interest: Dr. Bielopolski's research focuses on characterizing the mechanisms by which nutritional interventions impact blood pressure. She will conduct an inpatient clinical trial enrolling otherwise healthy participants with early hypertension; participants will switch from eating their typical Western diet to consuming the DASH diet, which has been proven to lower blood pressure. Measures of the urine electrolyte ratio, serum hormones, and urine exosomes will be analyzed.

Bio: Dr. Dana Bielopolski received her MD and PhD from the Sackler School of Medicine, Tel Aviv University in Israel. She completed her internal medicine residency and nephrology fellowship at the Rabin Medical Center in Israel.



Rachel Niec, MD, PhD

Mentor: Dr. Elaine Fuchs

Laboratory: Robin Chemers Neustein Laboratory of Mammalian Cell Biology and Development

Research Interest: Dr. Niec's research focuses on understanding how immune homeostasis is achieved at barrier surfaces, including in the intestinal tract and skin; specifically how immune cells, epithelial cells, and other tissue resident cells interact throughout development and during inflammation to set appropriate thresholds for immune reactivity and maintain homeostasis in these tissues.

Bio: Dr. Rachel Niec received her MD and PhD from the Cornell Medical College and Tri-Institutional MD PhD Program. She completed her internal medicine residency research track at Weill Cornell Medical College. She is in her gastroenterology fellowship at Weill Cornell Medical College/New York Presbyterian Hospital.



Yael Renert-Yuval, MD

Mentor: Dr. James Krueger

Laboratory: Laboratory of Investigative Dermatology

Research Interest: Dr. Renert-Yuval's research focuses on the immune dysregulations of various inflammatory dermatologic diseases, allowing better understanding of the underlying pathogenesis of these diseases, with the ultimate goal of developing novel therapies. Diseases of interest include scarring and non-scarring hair-loss and atopic dermatitis.

Bio: Dr. Yael Renert-Yuval received her MD and completed her dermatology residency from the Hadassah-Hebrew University Medical School in Israel, and spent a year as a research fellow at the Laboratory for Inflammatory Skin Diseases at the Mount Sinai Medical Center in New York.



Jeffrey Wong, MD, PhD

Mentor: Dr. Jeffrey Ravetch

Laboratory: Leonard Wagner Laboratory of Molecular Genetics and Immunology

Research Interest: Dr. Wong's research focuses immunobiology of therapeutic antibodies for the treatment of cancer with the goal of developing antibodies with enhanced anti-cancer activity.

Bio: Dr. Wong received his MD and PhD from the University of Pittsburgh. He completed his internal medicine residency at Brigham and Women's Hospital/Dana-Farber Cancer Institute. He is currently completing a fellowship in medical oncology at the Memorial Sloan Kettering Cancer Center.

Meet the Scholar: John W. Frew, MD

By Editorial Staff



John Frew

Dr. John Frew joined the Clinical Scholars program at the Rockefeller University in 2018. Dr. Frew received his MD from the University of South Wales, Australia. He completed his dermatology residency with the Australasian College of Dermatologists, Australia.

As a medical student, Dr. Frew was not initially interested in research or lab-based work. He thought it was too abstract and not directly applicable to patient care. In Australia, medical students have a compulsory research rotation, which he performed with a dermatologist doing translational research in congenital blistering disorders. This rotation was the turning point for him, leading to his passionate interest in research.

During his dermatology residency, Dr. Frew participated in clinical studies in Hidradenitis Suppurativa

(HS), a debilitating inflammatory dermatological condition, and one of his attending physicians mentioned that an Australian dermatologist who had moved to New York City was currently working on HS. The dermatologist was Dr. Michelle Lowes, a Clinical Scholar program graduate. Dr. Frew contacted Dr. Lowes to discuss HS, and she mentioned the program to him as an opportunity to conduct research in the United States. Dr. Lowes introduced him to Dr. Krueger who invited Dr. Frew to join his lab as a Clinical Scholar.

Dr. Frew's current research focuses on investigating the immunological mechanisms underlying HS in order to identify new targets for therapy and then perform clinical trials with novel agents to test their safety and efficacy in patients with HS.

Dr. James Krueger, Dr. Frew's mentor, stated, "John's work in unravelling the pathogenesis of Hidradenitis Suppurativa is generating opportunities for new therapies to help the many individuals suffering from this debilitating disease. His drive and passion are noteworthy and have placed him on the path to becoming a successful physician scientist."

When asked about his expectation about the Clinical Scholars Program, Dr. Frew responded,

"I knew that the potential opportunities in the US, and NYC in particular, were much greater than anything back in Australia, but the sheer level of support, encouragement, and research opportunities still have me pinching myself almost every day - even more than a year after arriving here. My expectations have been greatly surpassed. Every day has been a learning opportunity in the Clinical Scholars program.

The Clinical Scholars program has allowed me to begin to understand the concept of translational science and translational medicine and start to understand both worlds- the basic science and the clinical world. It is a challenge to live in both worlds but it is an important task. The Clinical Scholars program is an amazing opportunity, translating knowledge into action."

Dr. Frew has already authored 18 publications (7 in press) and developed 6 HS study protocols since joining the Clinical Scholars program.

Dr. Frew plans to return to Australia to apply the knowledge and skills he develops at Rockefeller to continue investigating novel therapies for HS, particularly their modes of action in modulating inflammatory pathways.

Inaugural Tri-State Nursing Translational Research Consortium Meeting Held at Rockefeller University

By Bernandette (Candy) Capili PhD, NP-C

On September 17, 2019, Bernandette (Candy) Capili PhD, NP-C hosted the inaugural Tri-State Nursing Translational Research Consortium Group at Rockefeller University. The Consortium is composed of nurse leaders from the Tri-State CTSA's. The members include Dr. Christine Kovner, New York University, Dr. Elizabeth Cohn, Hunter College, Dr. Margaret Barton-Burke, Memorial Sloan Kettering Cancer Center, Dr. Elizabeth Walker, Albert Einstein, Dr. Elizabeth Corwin, Columbia University, Dr. Olga Jarrin Montaner, Rutgers University, Dr. David Vlahov, Yale University, and Dr. Marilyn Hammer, Dana-Farber Cancer Institute-Harvard University. Members attend meetings at Rockefeller University or take part via conference call. Dr. Barry Collier, Physician-in-Chief, joined the meeting to discuss the Consortium's goals

and the important role of clinical research nursing in translational research.

The Consortium's goals are to foster collaborations, and communication, and to discuss opportunities and needs that nurses involved with CTSA's can address. Methods to reduce silos among disciplines is another goal the group hopes to undertake by having each Consortium member invite a colleague from another discipline from their respective institution to future meetings. The term "colleague themed topic" was coined to identify which scientific team member is invited to a meeting. Thus, future meeting will focus on clinical research coordinators, physician-scientists, post-doctoral fellows, biostatisticians, or clinical research nurses (CRN). The goal is to listen and to learn from each guest and to generate understanding.

Other highlights from the meeting included discussions of the need to introduce clinical research nursing to local nursing schools because it was clear from the meeting that some of the Consortium members did not know about the specialty of clinical research nursing and how vital the clinical research nurse role is to clinical research. The Consortium also discussed how best to help each other, and it was agreed that sharing success stories of initiatives that worked will be a good place to start. The concept behind using success stories is to understand the conditions and the measures that allowed the achievement of a positive outcome as a way of helping others replicate the success. The process and procedures to carry out these plans are on the agenda for the next meeting in December.

Meet the Graduate: Chibuzo Enemchukwu, MD, MS

By Editorial Staff



Chibuzo Enemchukwu

Dr. Chibuzo Enemchukwu graduated from the Clinical Scholars program and received a Master's degree in Clinical and Translational Research in June 2019. She received her MD from the University of North Carolina-Chapel Hill, completed her internal medicine residency at Mount Sinai Hospital in New York City, and her fellowship in infectious diseases at the Albert Einstein College of Medicine-Montefiore Medical Center in the Bronx, NY.

Dr. Enemchukwu commented that she was not always interested in research, because she did not fully understand the power of research, and how it can effect change in the lives of individuals and entire communities. As an infectious diseases clinical trainee, Dr. Enemchukwu cared for people living with HIV in a public clinic in the Bronx and in the southeastern African country of Malawi. In both settings, she experienced similar frustrations and had the same question, "How do we get effective evidence-based treatments and interventions to the people who need them most?" She consistently witnessed families and communities devastated by HIV/AIDS and other infections. Although effective prevention strategies and treatments were available, she could not understand how to overcome the structural, societal, and cultural factors that affected access to life saving interventions. After completing her clinical training, she had the opportunity to join the Clinical Scholars program mentored by leading experts in the field of implementation science. The Clinical Scholars program opened up the pathway for her to start answering how to get treatment to those who need them the most. Dr. Enemchukwu's interest in research then flourished as

she appreciated that it is one of the most effective ways to impact disparities in health outcomes.

Dr. Enemchukwu's current research centers on using implementation science to address disparities in HIV outcomes among minority women. African-American/Black and Latino (AAL) women make up over 90% of new HIV infections in New York City (NYC), yet they are less likely to be engaged in HIV treatment and prevention programs, and they suffer worse clinical outcomes. As a Clinical Scholar, she received NIH funding through an Einstein-Rockefeller-CUNY Center for AIDS Research (ERC-CFAR) supplement that allowed her to partner with the NYC Department of Health and Mental Hygiene and local community-based organizations (CBO) to directly address engagement in HIV prevention and treatment programming among this population. Specifically, she examining the feasibility of bringing HIV prevention and treatment interventions directly to CBOs that provide social services to AAL women.

When asked what were her expectations for the Clinical Scholars program, Dr. Enemchukwu's responded:

"To be honest, my expectations were that I would learn the "fundamentals" of research. I thought I would improve my grant writing, biostatistics skills, and participate in research projects that met my interests. Well, I did all of that! But I think the more relevant question for me now is, "What did I NOT expect?" I did not expect to have a complete life and career changing experience. I did not expect to finish the program with skills and knowledge that would translate into city-wide research collaborations, entrepreneurship, and starting my own consulting firm.

The biggest learning point as a Scholar was the importance of not setting limits on what you are capable of achieving. I learned this through mentorship from our program leaders as well as from my own research mentors. As clinicians, we are trained to care for patients and to work within a system, whether it be in a hospital, clinic, or other patient care area. The Clinical Scholars program

emphasized the value of our clinical training and the ability apply these skills across other endeavors such as research, administration, and entrepreneurship.

I think one of the best aspects of the Clinical Scholars program was the diversity of thought, skill, and research background among the Scholars. Every interaction with the other Scholars was an opportunity to learn or teach someone something new!

The most surprising aspect of the program was the exposure to accomplished policy makers, industry leaders, and entrepreneurs. This is an aspect of science and research to which I had not been previously exposed and it really help guide my current career trajectory."

We asked Dr. Enemchukwu to describe the Clinical Scholar program in one sentence, and she responded, "A career and life steering program that provides time, space, and mentorship as you discover your full potential."

In addition to continuing her current research work, Dr. Enemchukwu co-founded Nelu Diversified Consulting Solutions. Nelu is a consulting firm that appreciates the increasing diversity of the US workforce, and the importance of employer-sponsored health and financial benefits offerings that reach all segments of employee populations. Its mission is help employers strategize and create inclusive health and financial benefits programs that align with the unique needs of the diverse workforce.

Dr. Enemchukwu emphasized that her work with Nelu is an extension of her commitment to use implementation science to ensure that essential and effective programming and interventions are successfully engaging diverse populations.

Heilbrunn Family Center for Research Nursing Appoints Five Nurse Scholars 2019

By Bernadette (Candy) Capili PhD, NP-C

Five nurses from around the country were selected to receive the Heilbrunn Nurse Scholar Award, given by The Rockefeller University's Heilbrunn Family Center For Research Nursing to support nurses while they pursue independent research projects that will make a significant contribution to the discipline of nursing. Each award provides a maximum of \$25,000 for one or two years. Funding for the awards, now in the sixth year, is from an endowment established by sisters Helaine Lerner and Joan Rechnitz in honor of their parents, Harriet and Robert Heilbrunn.

This year's recipients will study topics ranging from self-management in left-ventricular assist devices (LVADS) to identifying neurobiologically informed psychosis and schizophrenia symptom profiles. A senior group of scientists reviewed the applications. The applications with the highest scientific and technical merit were selected to receive the Heilbrunn Nurse Scholar Award. For the 2019 award cycle, applications were submitted by doctoral and postdoctoral nurses from across the United States from 32 different institutions.



Jesus Casida, PhD, RN, APN-C. Dr. Casida is nationally and internationally known for his groundbreaking work on self-management in left-ventricular assist devices (LVADs). He built and advanced the LVAD self-management science through theoretical and empirical work. The Heilbrunn Award will support the expansion of his work focusing

on building patient and caregiver self-efficacy skills and adherence to LVAD care regimen. For this purpose, he invented a mobile phone application (VAD Care App®, 2014) as a tool for LVAD self-management process. He plans to evaluate the effect of VAD Care App-directed and nurse-supported self-management intervention on healthcare utilization (e.g., hospital readmission) outcomes. His interdisciplinary team envisions that the findings of this randomized control trial, supported by the Heilbrunn Award, will inform future studies to further understand the mechanism(s) of the effect of LVAD self-management on healthcare utilization and its economic impact as well as overall health and quality of life. Dr. Casida's long-term goal is to expand the intervention to other implantable artificial organs (whole heart, lung, kidney) and complex conditions requiring intensive self-management supported by nurses.

Dr. Casida recently joined Johns Hopkins University School of Nursing as a Faculty Associate and a Robert Wood Johnson Foundation Nurse Faculty Scholar Alumnus. He also has extensive clinical and leadership experience in cardiac surgery and cardiology critical-care including a pioneering an advanced practice nursing role for LVAD in heart failure and transplant treatment program. Dr. Casida obtained his MS degree in Critical Care from Columbia University School of Nursing and his PhD in Health Sciences from Seton Hall University. He has served as chair for several nursing research committees at regional and national levels. Currently, he is the founding leader for the Nursing, Health Science, and Allied Health Council Research Workforce within the International Society for Heart and Lung Transplantation.



Karen M. Jennings Mathis, PhD, RN, PMHNP-BC. Dr. Karen Jennings Mathis will examine relations among early life adversity, adipokine status, dietary composition, and physical health outcomes among adults. Dr. Jennings Mathis' primary research interests

include biopsychosocial mechanisms underlying the development and maintenance of mental disorders and the synergistic relationship between research and practice. Dr. Jennings Mathis is a Jonas Nurse Leaders Scholar, a Fellow of the Robert Wood Johnson New Careers in Nursing Scholarship, and a recipient of the 40 Under 40 Emerging Nurse Leader Awards.

Dr. Jennings Mathis is an Assistant Professor at the University of Rhode Island College of Nursing. She recently completed an NIMH T32 Postdoctoral Research Fellowship in the Department of Psychiatry and Behavioral Neuroscience at The University of Chicago and was adjunct Clinical Faculty at Rush University College of Nursing. She received her B.A. in Psychology from Amherst College, and her M.S. and Ph.D. in Nursing from Boston College.

She is certified as an Advanced Practice Registered Nurse in the specialty of Family Psychiatric-Mental Health Nursing. Dr. Jennings Mathis currently serves on the Editorial Board for the Journal of the American Psychiatric Nurses Association and is a Section Editor for the Journal of Psychosocial Nursing and Mental Health Services. She also serves as the co-chair for the Research-Practice Committee and is a member for the Diversity, Equity, and Inclusion Advisory Committee to the Board for the Academy for Eating Disorders and serves on the American Psychiatric Nurses Association's Research Council Steering Committee.



Mitchell Knisley PhD, RN-BC, ACNS-BC. Dr. Knisely’s research seeks to optimize pain assessment and management through a better

understanding of the biopsychosocial determinants of pain in individuals with Sickle Cell Disease (SCD). This study will primarily focus on characterizing pain profiles in adults with SCD and identifying genetic polymorphisms associated with the pain profiles. Findings will provide a foundation for identifying patients at risk for high pain burden and potential new targets for interventions to prevent or manage pain in this population.

Dr. Knisely is an Assistant Professor at Duke University School of Nursing. He earned his BSN from Purdue University, MSN, and his PhD from Indiana University. He completed a postdoctoral

fellowship in molecular genetics at the University of Pittsburgh School of Nursing. Additionally, he trained at the NIH National Institute of Nursing Research’s 2015 Summer Genetics Institute. Dr. Knisely is board certified as an Adult Health Clinical Nurse Specialist and in Pain Management Nursing.



Maura McCall MSN, RN. Ms. McCall’s dissertation research will explore the complex relationships among the symptom experience, genomic variation, and medication adherence in women with breast cancer. Her proposed study will

examine aromatase inhibitor adherence and symptom patterns and trajectories and will explore the role of genomics in these patterns and trajectories.

Ms. McCall is a doctoral student at the University of Pittsburgh School of Nursing (T32NR009759 Targeted Research and Academic Training Program for Nurses in Genomics). She earned her MSN at the University of Pittsburgh and BSN at Duquesne University. She has many years of experience in chronic disease medication adherence research. Ms. McCall has published articles on study recruitment, mentoring students, and the biological underpinnings of symptoms. She has co-authored book

chapters on adherence, an online educational module for rheumatology practitioners, and a continuing education video on patient adherence to hospital discharge instructions. Ms. McCall attended the NINR’s Summer Genetics Institute, and is a recipient of the American Cancer Society’s Doctoral Degree Scholarship in Cancer Nursing and the Oncology Nursing Foundation Research Doctoral Scholarship. She is a member of the International Society of Nurses in Genetics, the Oncology Nursing Society, the Sigma, National Council on Undergraduate Research, and the Pharmacogenomics Research Network.



Rose Mary Xavier, PhD, MS, RN, PMHNP-BC. Using novel tools from network science and bioinformatics and by integrating multiplex high-

dimensional data including genetic, neuroimaging, cognitive, and symptom data, Dr. Xavier aims to (1) identify neurobiologically informed psychosis and schizophrenia symptom profiles and (2) examine potential functional mechanisms of risk for psychosis and schizophrenia spectrum in a community sample of youth.

Dr. Xavier’s program of research broadly focuses on understanding neurobiological mechanisms of psychiatric symptoms that cross traditional diagnostic boundaries for clinical translation. A second but equally important interest is in research methodology and analysis guided by the philosophy and principles of open science. A Nurse Scientist and Psychiatric

Nurse Practitioner, she completed a Post-Doctoral Research Fellowship in Neuropsychiatry at the University of Pennsylvania, Perelman School of Medicine. In July 2019, she transitioned to the University of North Carolina-Chapel Hill, where she started her research lab as a tenure track Assistant Professor. Dr. Xavier has a PhD in Nursing and a doctoral certificate in Cognitive Neuroscience from Duke University with interdisciplinary training in Genomics.

Dinner with Dr. Scott Gottlieb, Former 23rd Commissioner of the FDA

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served at the FDA again from 2005 until 2007 as Deputy Commissioner for Medical and Scientific Affairs. Dr. Gottlieb then returned to the private sector as an attending at NYU Langone's Tisch Hospital. He became a partner at the venture capital firm New Enterprise Associates (NEA) and held directorships and board positions at Tolero, Daiichi Sankyo, and GlaxoSmithKline, all while still maintaining his clinical skills as an attending physician at NYU. Dr. Gottlieb received a number of awards including being ranked 6th among "The World's 50 Greatest Leaders" by Fortune magazine, one of the "50 People Transforming Healthcare in 2018" by Time magazine and the Nathan Davis Award for outstanding government service in 2018 by the American Medical Association. In October 2018, Dr. Gottlieb was elected a member of the National Academy of Medicine.

Dr. Gottlieb was appointed as the 23rd Commissioner of the FDA in May 2017 and served in this role until April 2019. During his tenure, the FDA implemented the 21st Century Cures Act (2016), improved the display of nutritional information in restaurants, opened the FDA Oncology Center, expedited the clinical trials approval process, and addressed the dangers of e-cigarette advertisements targeted toward children.

In his introduction, Dr. Gottlieb emphasized the importance of leadership and impartiality in heading an organization such as the FDA. Attendees at the dinner had the opportunity to ask Dr. Gottlieb a number of questions addressing the agency's relationship with congress, his perspectives on the future of drug pricing and the regulation of pharmaceutical development and

testing. Dr. Gottlieb also discussed the reasoning behind the FDA's position on genome editing as well as how and why the agency decided to initiate and fund research on the risks of e-cigarettes.

After engaging and stimulating discussions, the evening concluded on a high note with Dr. Gottlieb and participants discussing the potential for clinician-scientists and translational researchers to contribute to several facets of the biomedical enterprise.

New Pilot Grants Awarded

The Rockefeller University Center for Clinical and Translational Science (CCTS), along with the Center for Basic and Translational Research on Disorders of the Digestive System (CDDS), and the Shapiro-Silverberg Fund for the Advancement of Translational Research supported 30 pilot projects out of a total of 56 applications that were submitted this year. CCTS Clinical Scholars received 10 pilot awards. This year's total of \$1,020,784 awarded brings the grand total of pilot project funding to \$9,948,712 since the program began under the initial CTSA grant in 2006. A total of 480 different pilots have been funded to 46 different laboratories.

Support from the Center for Clinical and Translational Science

Pilots Projects Led by CCTS Clinical Scholars

Tobias Becher, MD (Cohen Lab): *Role for QSOX1 in the Regulation of Blood Pressure.* Obesity is associated with cardiovascular disease, the leading cause of death in the United States. In preliminary studies, the Cohen Lab have identified the fat-secreted protein quiescinsulfhydryl oxidase 1 (QSOX1) as a potential mediator of obesity-associated hypertension. This pilot project aims to understand how QSOX1 may regulate vascular function and contribute to the development of hypertension.

Dana Bielopolski, MD, PhD (Coller Lab): *Translational Characterization of Blood Pressure Changes Following Dietary Modification – From Nutrition through Electrolytes to Exosomes.* Uncontrolled hypertension is a significant cause of morbidity and mortality, which can be modified by specific diets, such as the DASH diet, which reduces sodium and increases potassium intake. The response to nutritional changes is inconsistent and the mechanism is poorly understood. This pilot project's goal is to understand the mechanism by hospitalizing volunteers with mild hypertension, feeding them a DASH diet menu and examining and analyzing their clinical and laboratory data, including urinary exosomes.

John Frew, MD (Krueger Lab): *Assessment of Inflammatory Mediators in Hidradenitis Suppurativa.* This pilot project aims to establish baseline knowledge of immune status (i.e., histological cell infiltrate, tissue cytokine milieu, mRNA gene expression, tissue microbiome metabolomic profile, and adipose tissue analysis) from skin samples of patients with Hidradenitis Suppurativa (HS) and their relation to disease activity and comorbidities compared to healthy control patients. This pilot project builds on results from a 2018 pilot award which led to the identification of IL-17C as a novel cytokine secreted from epithelial keratinocytes of the epidermis and dermal tunnels, as well as the identification of psuedo-psoriasiform epidermal hyperplasia and epidermal recapitulation in the lining of epithelialized tunnels of HS.

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Dr. Jason Hawkes

Mentor: Dr. James Krueger

Dr. Hawkes's research focused on the global gene expression profile and predominant signaling pathways of guttate psoriasis (a self-limited, eruptive form of the disease) versus chronic plaque psoriasis in an effort to better define the molecular biology of psoriasis and identify novel disease and therapeutic biomarkers. Dr. Hawkes is in private practice at UC Davis Health at Rocklin, California.

Dr. Moonjung Jung

Mentor: Dr. Agata Smogorzewska

Dr. Jung is studying the disease modifiers in Fanconi anemia, especially endogenous metabolic by-product such as reactive aldehydes, as potentially important sources of DNA damage. Her study is expected to provide valuable insights into leukemogenesis and hematopoietic stem cell protection both in Fanconi anemia patients and in the general population. Dr. Jung is continuing her research at Rockefeller University in the Smogorzewska Laboratory.

Dr. Youngmin Lee

Mentor: Dr. Thomas Tuschl

Dr. Lee's research focused on liver diseases with specific interest in the biology of fibrogenic cells and identifying determinants that impede liver regeneration. Dr. Lee is Assistant Professor in the Departments of Surgery and Medicine at Vanderbilt University.

Dr. Ching-Lan Lu

Mentor: Dr. Michel Nussenzweig

Dr. Lu's research focused on the impact of HIV broadly neutralizing antibodies on the latent reservoir of HIV, with the goal of eliminating the reservoir so as to effect a true cure of the disease. Dr. Lu is a resident in Internal Medicine at New York Presbyterian Hospital/Columbia University Medical Center.

Dr. Kathrine Meyers

Mentor: Dr. Martin Markowitz

Dr. Meyers's research focused on Pre-exposure prophylaxis (PrEP), a key biomedical HIV prevention strategy that can significantly reduce HIV incidence in real-world settings. Despite its demonstrable benefits, introduction and global scale-up of PrEP has been slow. The Yunnan-ADARC HIV Prevention Program will study the process of integrating PrEP into an existing health system and generate an implementation blueprint that can be used by other health systems planning to introduce PrEP to decrease the incidence of HIV in priority populations. Dr. Meyers is Social Scientist and Program Director, China Programs at the Aaron Diamond AIDS Research Center and Associate Professor of Medical Sciences in the Division of Medicine at Columbia University Medical Center.

Dr. Ethan Ravetch

Mentor: Dr. Sohail Tavazoie

Dr. Ravetch's research focused on pancreatic cancer metastasis and the development of novel diagnostic biomarkers of the disease. A neuronal protein was identified that is implicated as a key driver of highly metastatic pancreatic ductal adenocarcinoma. Dr. Ravetch's analysis of this protein's downstream effects identified interaction with the MAPK signaling pathway and the transcription of hypoxia response elements. Dr. Ravetch is in the General Surgery training program at Montefiore Medical Center in New York as a third year resident.

Dr. Norihiro Yamaguchi

Mentor: Dr. Sohail Tavazoie

Dr. Yamaguchi's research focuses on providing novel therapeutics targeting cancer metastasis. A key gene was identified that promotes pancreatic cancer liver metastasis and he developed a number of potentially therapeutic monoclonal antibodies targeting the protein coded by the gene. Pre-clinical experiments showed that one of the monoclonal antibodies drastically reduced pancreatic cancer liver metastasis. Dr. Yamaguchi is continuing his research to translate the discovery to clinic at Rockefeller University in the Tavazoie Laboratory.

David Knorr, MD, PhD (Ravetch Lab): *Vaccine immunity in Chronic Lymphocytic Leukemia) and Small Lymphocytic Lymphoma (CLL/SLL) and the Effect of Bruton's Tyrosine Kinase Inhibition.* The cellular mediators of human immunity to infection remains poorly defined as the majority of our knowledge in this field has come from inherited germline deficiencies or murine models. Patients with cancer are particularly susceptible to infections both intrinsically from their disease as well as due to immunosuppressive drugs they may receive as part of their anti-cancer therapy. More recently, targeted therapies for chronic leukemias (e.g. CLL/SLL) have led to deep remissions and improved survival in this patient population. However, infection remains a significant cause of morbidity and mortality. In CLL/SLL, targeting Bruton's tyrosine kinase is an effective therapy against malignant B cells, but the mechanisms by which it alters normal B cell immunity are less clear. Thus, this proposal aims to define the intrinsic immune defects in patients with CLL/SLL as well as determine the mechanisms by which BTK inhibition alters the normal humoral response to vaccination.

Rochelle Maxwell, MD (Smogorzewska Lab): *Characterizing the Role of the Fanconi Anemia DNA Repair Pathway in the Pathogenesis of Squamous Cell Carcinoma.* Persons with Fanconi anemia (FA) have a higher incidence of squamous cell carcinoma (SCC) than the general population. They develop SCC at a younger age, their tumors are more aggressive, and they have higher morbidity and mortality from the disease. This project aims to investigate the specific mechanisms that underlie the development and progression of SCC in the setting of FA, with the long term goal of developing interventions that may improve clinical outcomes.

Rachel Niec, MD, PhD (Fuchs Lab): *Exploring Lymphatic Capillaries as Regulators of the Intestinal Stem Cell Niche.* Maintenance of the intestinal barrier epithelium is achieved by long-lived tissue stem cells that have the remarkable capacity to self-renew and give rise to differentiated progeny to fuel this rapidly dividing tissue. These stem cells rely on localized niche signals for their maintenance, differentiation, and environmental responsiveness. Using this pilot award, we will employ three dimensional deep imaging and transcriptional profiling to examine the colonic stem cell niche in health and in inflammatory bowel disease with the goal of identifying new therapeutic targets for regenerative medicine and inflammatory disease.

Yael Renert Youval, MD (Krueger Lab): *A Pilot Study of Safety and Biomarkers of Ustekinumab for Cicatricial Alopecia.* Primary cicatricial alopecias (CA) are an increasingly common, progressive, scarring disease, resulting in permanent hair-loss, severely affecting quality-of-life. The mechanisms of CA are poorly understood. There are no approved therapies for these conditions and treatment options are unsatisfactory. In a setting of an open-label clinical trial, we will assess samples of skin and blood from CA patients by molecular techniques to investigate the mechanisms of inflammation and scarring at baseline and during treatment with ustekinumab. These findings will help to identify new treatment targets and direct further investigation for the development of new therapies for these disfiguring diseases.

Tukisa Smith, MD, MS (Breslow Lab): *Contact Activation in Common Variable Immunodeficiency (CVID).* The contact system, a well-characterized serine protease cascade, generates pro-inflammatory mediators and is implicated in key host innate and adaptive immune responses. Although activation of this pathway has been observed in various inflammatory diseases, the contact system is understudied in the most common primary immunodeficiency known as combined variable immunodeficiency (CVID), in which 25-30% of patients develop inflammatory complications despite standard care. This pilot project involves the study of contact system biomarkers in CVID patients, with and without inflammatory complications, with the goal of identifying patients that might benefit most from therapeutically targeting this pathway.

Ying Wang, MD, PhD (Birsoy Lab): *Regulation of Erythropoiesis by Mitochondrial Carrier Protein SLC25A39.* Anemia is a common disease of red blood cell (RBC) deficiency and is a major cause of morbidity; thus understanding how RBCs mature is important. This pilot award aims to determine how SLC25A39, the orphan mitochondrial solute carrier protein, coordinates cellular metabolism to promote RBC maturation. This study could provide new insights into the cellular processes of RBC maturation and thus will define new therapeutic strategies to ameliorate erythroid marrow failure and anemia.

Jeffrey Wong, MD, PhD (Ravetch Lab): *CD40 Agonism for the Treatment of Bladder Cancer.* Non-muscle invasive bladder cancer (NMIBC) is an area of significant unmet clinical need, with a large affected patient population, high rates of recurrence and progression, and limited salvage therapy options aside from radical cystectomy. A novel immunotherapy approach is proposed based on the agonism of CD40, an immune-stimulatory receptor centrally involved in activating antigen-presenting cells and downstream anti-tumor immunity to investigate locally delivered CD40 agonist therapy in a clinically relevant and highly translatable approach. This pilot study will help define the therapeutic potential of CD40 agonism and support an IND amendment for intravesical treatment of NMIBC with our antibody candidate, which recently began phase I evaluation at Rockefeller University for intratumoral treatment of patients with solid tumor cutaneous metastases.

Pilots Projects Led by Faculty and Postdoctoral Fellows

David Buchholz, PhD (Hatten Lab): *Modeling Ataxia-Telangiectasia with Induced Pluripotent Stem Cell-Derived Purkinje Cells.* Ataxia-telangiectasia (A-T), caused by mutations in the ATM gene, results in cerebellar degeneration, increased susceptibility to cancer and immune deficiency. Mice with *Atm* mutations do not have cerebellar degeneration, making this aspect of the disorder difficult to study. This pilot project aims to generate a model system to study human cerebellar Purkinje cells with the ATM mutation, differentiated from induced pluripotent stem cells derived from A-T patients.

Zu-Lin Chen, MD, PhD (Strickland Lab): *Specific Blocking Antibodies against High Molecular Weight Kininogen as a Potential Novel Therapy for Hereditary Angioedema.* Hereditary angioedema (HAE) is a disease characterized by recurrent tissue swelling that can be life-threatening. It is mainly caused by the release of bradykinin, a peptide cleaved out of high molecular weight kininogen (HK). We generated antibodies specific to cleaved and uncleaved forms of HK, and we will use these to block bradykinin release from HK in vivo. We will determine whether these specific antibodies have the potential to be developed for treatment of HAE and other bradykinin-induced pathologies.

Maria De Obaldia, PhD (Vosshall Lab): *Discovery of Human Skin Volatiles that Promote Mosquito Attraction to Humans.* Dr. Obaldia is studying why the yellow fever/Zika vector mosquito, *Aedes aegypti*, prefers to bite some people over others. We have identified human subjects who are uniquely attractive or unattractive to mosquitoes in laboratory assays. The goal of my pilot award is to perform chemical analysis to identify specific skin odors that make some humans “mosquito magnets.”

Yu-Ling Lee, PhD and Keiichi Ito, PhD (Roeder Lab): *Functional Study of E2A-PBX1 Driven Leukemogenesis and Potential Clinical Significance for Targeting PD-L1.* Based on our biochemical and cell-based assays implicating the importance of MED1/Mediator for the maintenance of E2A-PBX1 fusion driven leukemia, our aim is to address the functional significance of this insight in vivo. We will (i) employ a mouse genetic approach to test the role of MED1 in E2A-PBX1 driven leukemia and (ii) pharmacologically inhibit genes that are directly controlled by E2A-PBX1-MED1 pathway. The goal of our study is to extend mechanistic and functional understanding of E2A-PBX1 driven leukemia and to provide therapeutic approaches against this disease.

Isaac Marin-Valencia, MD, MS (Hatten Lab): *Mechanisms and Therapies for Cerebellar Maldevelopment in Pyruvate Dehydrogenase Deficiency.* Mitochondrial disorders are the most common inborn errors of metabolism. These conditions frequently involve the developing brain, in particular, the cerebellum that mediates major disability in patients. This proposal aims to identify mechanisms that underlie cerebellar disruption in the context of defective mitochondria and apply effective therapies accordingly. This project will focus on the prototypical mitochondrial disease with cerebellar involvement, pyruvate dehydrogenase deficiency (PDHD). Preliminary data from a mouse model of PDHD reveal that glucose metabolism in the cerebellum is impaired, and that proliferation and migration of granule cells (GC) is compromised, leading to cerebellar hypoplasia. The central hypothesis is that PDHD disrupts cerebellar formation by limiting GC development due to impaired glucose metabolism. Two aims are proposed: 1) To elucidate developmental processes that underlie the cerebellar disruption, and 2) To identify metabolic mechanisms relevant to cerebellar disease. This proposal is significant because it will advance the understanding of how mitochondrial disorders cause cerebellar disease. It is innovative because it combines advanced techniques to tackle previously unanswerable questions. Long-term, this project will set the basis to potentially approach any neurodevelopmental manifestation of any mitochondrial disease.

Erin Norris, PhD (Strickland Lab): *Development of ELISA for Cleaved High Molecular Weight Kininogen as a Diagnostic Tool for Alzheimer's Disease.* The Alzheimer's disease (AD) peptide, beta-amyloid, can activate the contact system, launching thrombotic and inflammatory pathways. The inflammatory arm leads to cleavage of high molecular weight kininogen (HK), which is found at higher levels in the plasma of AD patients compared to non-demented individuals. We generated HK-specific antibodies to develop a sandwich ELISA that will quantify the levels of intact and cleaved HK in plasma, serving as a way to stratify AD patients with vascular pathology. This tool may also lead to specific therapeutic approaches to delay disease progression.

Jean-Pierre Roussaire, PhD (Greengard Lab): *Single-cell Profiling of Preclinical Alzheimer's Disease For Identification Of Drivers of Neurodegeneration.* The aim of the study is to generate a high-quality reference dataset of gene expression in the neurons most vulnerable to Alzheimer's Disease, at very early stages of their pathology. For that, we are performing state-of-the-art single-nucleus sequencing on human postmortem tissue. This dataset, in addition to previous system-level analysis we made of these neurons, will allow us to identify therapeutic targets for treatments to block neurodegeneration in Alzheimer's Disease.

Sanford Simon, PhD (Simon Lab): *Patient Partnership to Identify Cause of Hyperammonemic Encephalopathy in Fibrolamellar Hepatocellular Carcinoma.* Fibrolamellar hepatocellular carcinoma is a rare, usually lethal, liver tumor that afflicts adolescents and young adults. A frequent cause of death is hyperammonemic encephalopathy. Given that fibrolamellar is a liver tumor, most treatments involve lactulose, the standard treatment for such tumors. While this works for other liver pathologies, it inevitably leads, in the case of fibrolamellar, to coma and death. Our analysis of the blood and urine levels of the patients suggests a disruption of the urea cycle in fibrolamellar, or in a subset of these patients, which can be treated by sodium benzoate. Our analysis of the transcriptome and proteome of the tumor tissue suggest that we can identify the presence of this disruption and its molecular basis. Success in doing so would inform other therapeutics and save lives.

Support from Center for Basic and Translational Research on Disorders of the Digestive System

Jingyi Chi, BS (Cohen Lab): *Molecular regulation of the crosstalk between adipocytes and the sympathetic nervous system in adipose tissue by S100B*. The goal of this pilot project is to clarify the molecular mechanism underlying the interaction between beige adipocytes and sympathetic nerves. Insights from this project may suggest novel approaches to prevent and treat obesity.

Gregory Donaldson, PhD (Mucida Lab) *Immunoglobulin A Regulation of the Gut Microbiota During the Emergence of Inflammation-Driven Gastrointestinal Cancer*. The project investigates how intestinal B cell responses are involved in maintaining a healthy mucosal microbiome, and how this affects downstream immune responses in the initiation and progression of colorectal cancer. This may open up new avenues of treatment or prevention through manipulations of the gut bacterial community for anti-inflammatory or anti-tumor functionality.

Dennis Hsu, MD (Tavazoie Lab) *Metabolism-Directed Treatments Using Codon-Based Mutational Signatures in Colorectal Cancer*. Our project proposes to use codon-based mutational signatures to identify metabolic vulnerabilities in colon cancer. The goal of this research would be to find a way to use data that is already commonly collected from cancer patients (i.e. next generation sequencing of tumor DNA) and use these data to find patients who might benefit from metabolism-directed therapies. We will test our predictions using mouse models and cell lines that are predicted to be sensitive and resistant, with the hopes that a positive result can be translated into clinical studies down the road.

Eleftherios Michailidis, PhD (Rice Lab) *Single-cell Transcriptomics of Human Hepatocytes in Response to Hepatitis B Virus Infection and Interferon Treatment*. Chronic carriers of hepatitis B virus (HBV) are at high risk of developing liver cirrhosis and hepatocellular carcinoma. Our proposed work will characterize the hepatocyte transcriptome in response to HBV infection and will provide insights into the curative potential of interferon α treatment. These studies will reveal suitable targets for novel and improved anti-HBV therapies.

Ryan Moy, MD, PhD (Tavazoie Lab) *Unraveling the Mechanisms Regulating Gastric Cancer Metastasis to the Liver*. Metastatic progression is the primary cause of death in gastric cancer, and the liver is one of the most common sites of metastasis. Using in vivo selection, we have generated human and mouse gastric cancer cells with enhanced ability to metastasize to the liver. We have identified a set of genes that are up-regulated or down-regulated in highly liver metastatic gastric cancer cells, and we will use CRISPR/Cas9 to test the function of these genes in in vivo metastasis assays using cell lines and patient-derived xenograft models.

Bernardo Sgarbi Reis, PhD (Mucida Lab) *Characterizing tumor-infiltrating gd T cells in Colon Cancer*. Colorectal cancer (CRC) is one of the most common and deadly cancers in the US. The role of intraepithelial lymphocytes (IELs) in the development and progression of CRC is still unclear. My proposal will apply novel tools and concepts from the field of mucosal immunology to elucidate the role of TCRgd+ IELs in CRC."

Support from the Shapiro-Silverberg Fund for the Advancement of Translational Research

Jose Aléman, MD, PhD (Breslow Lab): *The Time-Restricted-feeding Effects on Inflammation and Obesity (TRIO)*. Study Subtitle: The Impact of Early Time Restricted Feeding on Metabolism And Inflammation in Obesity. Time-Restricted Feeding (TRF), a form of intermittent fasting, shows great promise as a novel intervention for addressing obesity and its complications. We propose to conduct a randomized 7 day study in which 10 prediabetic subjects eat all their calories within their first 6 waking hours, followed by 7 days with their usual eating pattern and vice versa. Specifically, we will study how TRF improves glucose metabolism in comparison to systemic inflammation.

Claudia Lorena Buitrago, PhD (Coller Lab): *Decoding the Unique Interaction Between Platelet Integrin α IIB β 3 and Cross-Linked Fibrin*. The formation of a stable thrombus is accompanied by the generation of thrombin and the conversion of fibrinogen to cross-linked fibrin. While the interaction of platelets with fibrinogen has been well established the interaction with fibrin is less understood despite its clinical relevance. Using human platelets we aim to elucidate the binding kinetics and affinity of α IIB β 3 interaction with fibrin and to make monoclonal antibodies that specifically block fibrin- α IIB β 3 interactions and explore their therapeutic potential.

Jonatan Ersching, PhD (Victora Lab): *B Cell Clonal Evolution in Long-Lasting Germinal Centers During Chronic Hepacivirus Infection*. The main goal of this research proposal is to characterize the dynamics of B cell clones in long-lived GCs during chronic viral infection with a newly described hepatitis C virus-related hepacivirus. I will test the hypothesis that new B cells invade ongoing GCs over time and eventually replace the initial pool of clones recruited. This will be assessed with fate-mapping strategies combined with parabiosis and single-cell sequencing. Two novel genetic mouse models will also be developed to determine mechanisms of GC clonal replacement and the specific contribution of newly recruited clones to the generation of protective antibodies in serum.

New Pilot Grants Awarded

Support from the Shapiro–Silverberg Fund for the Advancement of Translational Research

Seon-Hui Hong, PhD (Rice lab): *Interferons Response and Production of Proinflammatory Cytokines and Chemokines from Inherited IFNAR1 Deficient Patient with Vaccine-associated Viscerotropic Disease (YEL-AVD)*. Although YFV-17D have been safe and effective vaccine to prevent yellow fever virus infection, rare individuals suffer from life-threatening disease including YEL-AVD after vaccination. Using patient-derived induced pluripotent stem cells (iPSCs) from those who have suffered from YEL-AVD, this pilot project will study the underlying mechanism of how the vaccination of YFV-17D can cause YEL-AVD. This research might be helpful to develop new treatment of YEL-AVD and evaluate individual's risk of YEL-AVD before vaccination.

Simon Pelham, MD PhD (Casanova Lab): *Identifying the Genetic Susceptibility Underlying Cryptococcosis*. S. Pelham's pilot study will focus on cryptococcus, the fungus that causes severe infections in HIV-immunodeficient and immunosuppressed individuals. In addition to these individuals, there exists subsets of patients who are otherwise healthy, yet suffer from cryptococcosis. We hypothesize that these patients are prone to cryptococcosis due to an inborn error of immunity that selectively disrupts the host defense against cryptococcus. The potential discovery of inherited defect(s) responsible for cryptococcosis could provide insight into the general pathogenic mechanism of cryptococcosis, paving the way for novel treatment strategies, while also revealing novel mechanisms of anti-fungal immunity in natural conditions, a hallmark of human genetic studies.

Andr s Spaan, MD, PhD (Casanova Lab): *Human Genetics of Hyper-Inflammatory Responses to Bacterial Infections*. Most humans contract minor infections, while only a small minority develops severe disease. Dr. Spaan recently identified a number of patients that suffer from life-threatening hyper-inflammatory responses to bacterial infections due to the same underlying genetic defect. The funding of the pilot award and the facilities of the Hospital will be used to recruit additional patients. The project will provide proof of principle, and will allow for the exploration of innovative therapeutic avenues to treat infectious diseases.



Enhancing Scientific Rigor, Reproducibility, and Reporting

CCTS Methodology Series 2019 – 2020

Thursdays

2:00 pm – 3:00 pm

Date	Title	Speaker	Room
October 24, 2019	<i>Just Tell Me Three Mice Are Enough – Research Reproducibility in Translational Science</i>	Roger Vaughn, MS, DrPH Director of Biostatistics	Nurse's Residence 110B
November 14, 2019	<i>Essentials of Data management</i>	Matt Covey, PhD University Librarian	Nurse's Residence 110B
December 12, 2019	<i>Overview on RNA-seq Analysis: Design, Quality Control and Report</i>	Yupu Liang, PhD, Computer Science Director of Research Bioinformatics	A Level Training Room, Welch Hall
January 9, 2020	Principles of Good Data-Sharing Hygiene	Rie Goto, MSLIS Assistant University Librarian	Nurse's Residence 110B
February 13, 2020	<i>Hands on Elements of Statistical Power Analysis</i>	Neha Singh, MS Statistical Programmer	A Level Training Room, Welch Hall
March 12, 2020	<i>GMP Drug Formulation: secrets revealed</i>	Robert MacArthur, PharmD, MS Director of Pharmacy Services	Nurse's Residence 110B

Rockefeller Historical Vignette: Hematopoietic Stem Cells, and Unraveling the Immune Response to Tuberculosis

By Elizabeth (Betsy) Hanson



Sabin, Florence
Courtesy of the Rockefeller Archive Center

When *Mycobacterium tuberculosis* infects tissue such as the lungs, tubercles typically form—lumps made up of layers of immune system cells as they surround and attempt to destroy the bacteria. Since the 1890s, researchers had known that even dead *Mycobacterium tuberculosis* could stimulate this response. In the 1920s, Florence Sabin (1871-1953) set out to identify the chemical components of the bacterium that provoke the activity of different immune system cells. In addition, during the course of this work, Sabin discovered how monocytes evolve into the multinucleated "giant cells" found in tubercular lesions. These studies, carried out between 1925 and 1938, made important contributions toward understanding the immune response to TB infection.

Sabin's interest in immune system cells, also known as white blood cells, stemmed from her pioneering work on the origin of blood cells in developing embryos. Studying chick embryos with the new tissue culture methods of the day, she observed both red and white blood cells emerging from the endothelium of the blood vessels. Sabin also perfected cell staining techniques with which

to differentiate the living blood cells. In addition, her investigations of the embryological origins of the lymphatic system established that its structures develop from the embryo's veins. These areas of research, as well as her widely used textbook, *Atlas of the Medulla and Midbrain* (1901), garnered Sabin a national reputation as an anatomist.

Florence R. Sabin received the BS from Smith College (1893) and the MD from The Johns Hopkins University Medical School (1900). After an internship at the Johns Hopkins Hospital, she joined the medical school faculty, advancing to professor of histology in 1917. She was the first woman to become a full professor at The Johns Hopkins Medical School. In 1924 she became the first female president of the American Association of Anatomists. The next year she moved to the Rockefeller Institute for Medical Research, where she was the first woman to be appointed member (full professor) of the Institute. She

retired from Rockefeller in 1938, and in 1944 she embarked on a new career in her home state, Colorado, investigating health services and campaigning for public health legislation in a series of government posts. For this work she received a Lasker Award for Public Service (1951). In addition to 15 honorary degrees, Sabin's scientific achievements were recognized with election to the U.S. National Academy of Sciences (1925), of which she was the first female member, and the Trudeau Medal of the National Tuberculosis Association. She also published a biography of her Johns Hopkins mentor: *Franklin Paine Mall: The Story of a Mind* (1934). Arguably the best known woman scientist of her generation, Sabin was named one of America's twelve most eminent living women in a *Good Housekeeping* magazine poll (1931). The State of Colorado honored Sabin by selecting a statue of her as its contribution to the National Statuary Hall of the U.S. Capitol.



Sabin, Florence
Courtesy of the Rockefeller Archive Center