Stanford MCHRI’s Translational Medicine Mission
The goal for this course, along with all of our other efforts in this exciting research area, is to develop a local community of translational medicine (TM) professionals equipped to catalyze the application of discoveries in maternal and child health by leveraging the resources and expertise that are uniquely Stanford.

Overall Educational Objectives
The curriculum will be delivered by Eureka Institute alumni and faculty, and subject matter experts who bring experience from academia, industry, regulatory agencies, venture capital, intellectual property law, etc.

The course will:

1. Facilitate the transfer of knowledge of TM for all learners through diverse teaching approaches
2. Analyze the business, scientific and regulatory aspects of TM
3. Explore the challenges professionals encounter in TM, including accessing mentorship, building successful teams and developing healthy interdisciplinary collaborations
4. Develop critical thinking skills to approach the challenges in TM
5. Develop communication skills for presenting complex scientific ideas to a broad spectrum of people
6. Share with learners information about Stanford resources in Intellectual Property and TM resources

Educational Strategies
The course will feature a number of educational strategies to achieve the overall course objectives and the goals of each individual activity. Key among these is creating an open and safe environment through which participants can navigate, and in which participants may interact.
Thanks to our Support

It is with great appreciation that we thank our strategic partner, the Eureka Institute for Translational Medicine, for all their support that went into the creation of this unique program. Their experience in training in this field for the last 12 years has been a blessing to all those involved as we sought to create a course that is robust and focused on the resources available here at Stanford.

Stanford MCHRI Course Executives

Mary Leonard, MD, MSCE
Director of Stanford Maternal & Child Health Research Institute; Professor and Chair of Pediatrics, Stanford School of Medicine; Physician-In-Chief, Lucile Packard Children’s Hospital Stanford

Anthony Oro, MD, PhD
Co-Director of Stanford Maternal & Child Health Research Institute; Professor of Dermatology, Stanford School of Medicine; Co-Director of Stanford Center for Definitive and Curative Medicine

Maria Grazia Roncarolo, MD
Professor of Pediatrics (Stem Cell Transplantation) and of Medicine (Blood & Marrow Transplantation), Stanford School of Medicine; Director of Stanford Center for Definitive and Curative Medicine; Co-Director of Institute for Stem Cell Biology and Regenerative Medicine, Stanford School of Medicine

Mary Chen, MS, MBA
Assistant Dean of Maternal and Child Health Research; Executive Director of the Stanford Child Health Research Institute

Eureka Institute for Translational Medicine Leadership

Janet Hafler, EdD
Professor of Pediatrics (General Pediatrics), Associate Dean for Educational Scholarship, Director of Teaching and Learning Center, Yale School of Medicine

Vicki Seyfert-Margolis, PhD
Board Chairman, Eureka Institute; CEO & Founder, MyOwnMed, Inc.

Salvatore Albani, MD, PhD
Professor, Duke-NUS Medical School Singapore; Director, Translational Immunology Institute; UCAN-A Chair; President, Eureka Institute

Berent Prakken, MD, PhD
Professor of Paediatric Immunology; Vice Dean for Education, University Medical Centre Utrecht; CEO/Secretary, Eureka Institute

Tanneke Zeeuw, MSc
Chief Operating Officer, Eureka Institute

Stanford Eureka Alumni Contributors

Tamar Green, MD
Mingxia Gu, MD, PhD
David Hong, MD
Manpreet Singh, MD, MS
Marko Jakovljevic, PhD
Melissa Mavers, MD, PhD
Trung Pham, MD, PhD
Thomas Robinson, MD, MPH
Zachary Sellers, MD, PhD
Sriram Vaidyanathan, PhD
Dear Participant,

It is with a mix of pride, great expectations and trepidation that we welcome you to the course “MCHRI Eureka Certificate Course in Translational Medicine”, an initiative which is inspired by and is an integral part of the Eureka Institute for Translational Medicine. Our objective is not to provide you with certainties or burden you with pre-digested knowledge, but rather to inspire you questioning your own notions and hopefully gently shake their foundation. What we seek is what you seek: to shorten the distance between the unmet need and its solution by inspiring and catalyzing a Copernican revolution in medicine, one which puts patients in the center of the process, one which will truly make a difference in the way we conceive and practice in our respective fields.

Translational Medicine faces many challenges and the only way to solve this definitely is to work together and find new solutions for the pressing problems we face. Eureka was founded over a decade ago to do just that, and to empower young translational scientists to overcome the many hurdles between bench and bedside.

You will have the opportunity to forge a new vision of Translational Medicine, one where study design, clinical and scientific questions, even policies, are molded around the patient. This school offers you all possibilities to explore and find your own personal role in this exciting field. A line up of experienced and innovative leaders is ready to help you to take the next steps in your career.

We welcome you to the Eureka community. Our best wish is that your enthusiasm, creativity and energy will help propel all of us, together, toward new horizons, in the sole interest of the advancement of Medicine.

We wish you an inspiring meeting!

Salvatore Albani, MD, PhD
Professor, Duke-NUS Medical School Singapore
Director, Translational Immunology Institute
UCAN-A Chair
President, Eureka Institute

Berent Prakken, MD, PhD
Professor of Paediatric Immunology
Vice Dean for Education, University Medical Centre Utrecht
CEO/Secretary, Eureka Institute
About the Course

In this booklet, you will find:

- A brief introduction to Eureka and translational medicine
- Basic logistical information
- Course materials organized by day
- Faculty and participants bios

This is a living document that will grow and change with you as you move through the course. The program materials are organized chronologically and each session is listed with its core learning objectives. Most days will start at 8:00am with coffee and brief social period, which will segue into the first session of the day (at 8:30am). The course is balanced between didactic sessions, interactive discussion, and practical application. Please make opportunities to synthesize the discussions and explore, in a personal context, how the topics covered can be applied to your own work. Evaluation of the course is an important part of reflecting on the experience and providing feedback aimed at course improvement. You will be provided with an e-based evaluation that can be completed daily throughout the course and should be submitted by the end of the course.

About Translational Medicine

Today, the term “translational medicine” is a buzzword in biomedical sciences with a rapidly increasing number of meetings about it; courses on it; and institutes dedicated to it. A simple Pubmed search on “translational medicine” generates over 139,000 papers, while a Google search yields nearly 36,600,000 hits. Because of its popularity and its increased use, the meaning of the term translational medicine has become progressively ambiguous and is often used synonymously with clinical testing.

In the opinion of those that designed this course, translational medicine encompasses the continuum of activities that extend from the conception of an idea all the way into Phase II/III clinical testing and, ultimately, the development of a tangible product that makes a significant difference in the lives of patients. This itinerary includes multiple and diverse components requiring very different skills and competencies ranging from molecular medicine to pharmacology; from animal testing to clinical trial design; from intellectual property to venture capital.

Translational medicine is therefore the framework needed to ensure the evolution of novel technologies into tangible benefits for patients.
Faculty Roster

Rajni Agarwal-Hashmi, MD, MBBS
Associate Professor of Pediatrics (Stem Cell Transplantation & Regenerative Medicine); Clinical Director for Pediatric Stem Cell Transplantation, Stanford Children’s Health

Crystal Botham, PhD
Director of Research Development in the Department of Pediatrics, Stanford School of Medicine; Director of the Biosciences Grant Writing Academy, Stanford University

Jennifer Swanton Brown, RN
Director of Clinical Research Quality, Stanford School of Medicine Research Office

Molly Bukro
Director of Major Gifts, Lucile Packard Foundation for Children’s Health

Cheryl Cathey, PhD
Licensing Associate, Office of Technology Licensing, Stanford University

Jerel Davis, PhD
Managing Director, Versant Ventures

Grace Gengoux, PhD, BCBA-D
Clinical Associate Professor of Psychiatry & Behavioral Sciences (Child & Adolescent Psychiatry), Stanford School of Medicine

Kevin Grimes, MD, MBA
Professor of Chemical & Systems Biology, Stanford School of Medicine; Co-Director of SPARK, Stanford University

Janet Hafler, EdD
Professor of Pediatrics (General Pediatrics), Associate Dean for Educational Scholarship, Director of Teaching and Learning Center, Yale School of Medicine

Shivaani Kummar, MD
Professor of Medicine (Oncology) and of Radiology (Molecular Imaging Program), Stanford School of Medicine; Director of Phase I Oncology Clinical Research Program

Mary Leonard, MD, MSCE
Director of Stanford Maternal & Child Health Research Institute; Professor and Chair of Pediatrics, Stanford School of Medicine; Physician-In-Chief, Lucile Packard Children’s Hospital Stanford

Margaret A. Neale, MS, PhD
Professor of Management, Stanford Graduate School of Business; Director of the Managing Teams for Innovation and Success Executive Program; Director of the Influence and Negotiation Strategies Executive Program

Anthony Oro, MD, PhD
Co-Director of Stanford Maternal & Child Health Research Institute; Professor of Dermatology, Stanford School of Medicine; Co-Director of Stanford Center for Definitive and Curative Medicine

Thomas Robinson, MD, MPH
Professor of Pediatrics (General Pediatrics) and of Medicine (Stanford Prevention Research Center), Stanford School of Medicine

Maria Grazia Roncarolo, MD
Professor of Pediatrics (Stem Cell Transplantation) and of Medicine (Blood & Marrow Transplantation), Stanford School of Medicine; Director of Stanford Center for Definitive and Curative Medicine; Co-Director of Institute for Stem Cell Biology and Regenerative Medicine, Stanford School of Medicine

Vicki Seyfert-Margolis, PhD
Board Chairman, Eureka Institute; Founder and CEO, My Own Med, Inc.

Manpreet Singh, MD, MS
Associate Professor of Psychiatry and Behavioral Sciences (Child & Adolescent Psychiatry), Stanford School of Medicine; Director of the Pediatric Mood Disorders Program

Michael Snyder, PhD
Stanford W. Ascherman Professor and Chair, Department of Genetics, Stanford School of Medicine; Director, Center for Genomics and Personalized Medicine

James Wall, MD, MS
Associate Professor of Surgery (Pediatric Surgery), Stanford School of Medicine; Director, Program Development, Bodesign Innovation Fellowship, Stanford Byers Center for Biodesign; Director, UCSF-Stanford Pediatric Device Consortium
Ambassadors

Crystal Botham, PhD
Director of Research Development in the Department of Pediatrics, Stanford School of Medicine; Director of the Biosciences Grant Writing Academy, Stanford University

Julie Ledford, PhD
Assistant Professor of Clinical Translational Sciences; Associate Professor of BIO5 Institute, of Cellular and Molecular Medicine, of Immunobiology, and of Medicine, Department of Medicine, University of Arizona College of Medicine

Course Support

Mary Chen, MS, MBA
Assistant Dean of Maternal and Child Health Research; Executive Director of the Stanford Child Health Research Institute

Tamar Green, MD
Assistant Professor of Psychiatry and Behavioral Sciences (Center for Interdisciplinary Brain Sciences), Stanford School of Medicine

Mingxia Gu, MD, PhD
Instructor of Pediatrics (Cardiology), Stanford School of Medicine

David Hong, MD
Assistant Professor of Psychiatry and Behavioral Sciences (Center for Interdisciplinary Brain Sciences), Stanford School of Medicine

Marko Jakovljevic, PhD
Postdoctoral Research Fellow in the Department of Radiology, Stanford School of Medicine

Melissa Mavers, MD, PhD
Instructor of Pediatrics (Stem Cell Transplantation & Regenerative Medicine), Stanford School of Medicine

Trung Pham, MD, PhD
Instructor of Pediatrics (Infectious Diseases), Stanford School of Medicine

Zachary Sellers, MD, PhD
Instructor of Pediatrics (Gastroenterology), Stanford School of Medicine

Sriram Vaidyanathan, PhD
Postdoctoral Research Fellow in the Department of Pediatrics (Stem Cell Transplantation & Regenerative Medicine), Stanford School of Medicine

Grant Wells, MS
Program Manager, Stanford Maternal & Child Health Research Institute
Thank You, Janet!

The course leadership wishes to thank Janet Hafler for all her dedicated time that she took to design this curriculum with our talented Eureka alumni. This was a momentous effort which occurred over several months and many iterations to adapt the Eureka Institute curriculum for our Stanford audience. We have been so fortunate to partner with her on the creation of this course and learn from her experience.

Janet, we are very grateful for your dedication, encouragement, and leadership. We hope you enjoyed working with us as much as we have enjoyed with you!
Eureka Institute
Certificate Course 2020

SCHEDULE
Sunday, February 9

3:00-3:30 PM
Check-in and Group Snack

3:30-4:40 PM
Welcome and Introductions
Speaker: Janet Hafler
Moderators: Anthony Oro, Tamar Green, and David Hong

4:40-5:10 PM
Communication of Course Objectives and Eureka Introduction
Speaker: Maria Grazia Roncarolo
Learning Objectives:
• Define the field of translational medicine
• Analyse the components involved
• Discuss the challenges of translational medicine

5:10-5:15 PM
BREAK

5:15-6:15 PM
From Discovery to Clinical Trial: Mapping the Translational Pathway
Speaker: Maria Grazia Roncarolo
Moderators: Tamar Green and Sriram Vaidyanathan
Learning Objectives:
• Explore the pathway of intellectual property development within an institution
• Discuss the benefits and detriments of technology transfer
• Discuss the options, obligations, and strategies of the investigator

6:15-6:30 PM
BREAK

6:30 PM
Dinner - Informal Q&A with Mary Leonard
Hosts: Tamar Green and David Hong

Monday, February 10

8:00-8:30 AM
Coffee

8:30-9:00 AM
Debriefing and Learning Strategies
Speaker: Janet Hafler
Moderators: Tamar Green and David Hong

9:00-12:00 PM
Building Your Translational Medicine Creativity
Facilitators: Rajni Agarwal-Hashmi, Tamar Green, David Hong, and Thomas Robinson
Learning Objective:
• Design thinking to trigger a creative mindset

12:00-1:00 PM
GROUP LUNCH

1:00-2:00 PM
Negotiate to Get (More of) What You Want
Speaker: Margaret Neale
Moderators: David Hong and Manpreet Singh
Learning Objective:
• Learn about the advances in psychology and behavioral economics to provide new strategies for negotiation

Sunday Social Night

7:30 PM, Nautilus Room
Grab some popcorn and join us for a movie!
2:00-2:15 PM
BREAK

2:15-3:15 PM
How Institutions can Promote and Facilitate Traditional Medicine
Speaker: Mary Leonard
Moderators: David Hong and Manpreet Singh

Learning Objectives:
• Learn how to incorporate the principles of negotiation in the context of one's academic career
• Develop methods and identify resources to ensure that your institution supports your objectives

3:15-3:30 PM
BREAK

3:30-4:00 PM
Introduction to Unfolding Case and Journaling
Speakers: Janet Hafler and Vicki Seyfert-Margolis

4:00-4:15 PM
BREAK

4:15 - 5:30 PM
Unfolding Case - Part I: CARDINALdx
Speaker: Vicki Seyfert-Margolis
Moderators: Mingxia Gu and Sriram Vaidyanathan

Senior Mentors: Janet Hafler, Maria Grazia Roncarolo, Vicki Seyfert-Margolis, and Manpreet Singh

Junior Mentors: Mingxia Gu, Trung Pham, Zachary Sellers, and Sriram Vaidyanathan

*See page 15 for details about Unfolding Case

5:30-5:45 PM
BREAK

5:45-6:15 PM
Journey of a Translational Scientist
Speaker: Manpreet Singh

Learning Objectives:
• Describe the journey of a translational scientist at Stanford
• Appreciate the patient imperative to challenge us to meet clinical unmet needs
• Identify their own inspiration and challenges in implementing TM concepts

Pre-Session Thought Questions for Participants
1. What inspires you to pursue your field of interest?
2. Identify some challenges that you have encountered along the way

6:15-6:30 PM
BREAK

6:30 PM
Dinner: Informal Q&A - Stanford Perspectives on Drug Development with Kevin Grimes
Moderators: Mingxia Gu and Sriram Vaidyanathan

Monday Social Night
7:30 PM, Nautilus Room
Eat, drink, and be merry over wine and cheese!
Tuesday, February 11

8:00-8:30 AM
Coffee

8:30-9:00 AM
Debriefing and Learning Strategies
Speaker: Janet Hafler
Moderators: Tamar Green and David Hong

9:00-10:15 AM
Unfolding Case - Part II: CARDINALdx
Speaker: Vicki Seyfert-Margolis
Moderators: Mingxia Gu and Sriram Vaidyanathan

Senior Mentors: Janet Hafler, Maria Grazia Roncarolo, Vicki Seyfert-Margolis, and Manpreet Singh

Junior Mentors: Mingxia Gu, Trung Pham, Zachary Sellers, and Sriram Vaidyanathan

10:15-10:30 AM
BREAK

10:30-12:00 PM
Translational Medicine Resources
Small Group Sessions
Speakers: Crystal Botham (Grant Writing), Jennifer Brown (Regulatory), Molly Bukro (Philanthropy), and Cheryl Cathey (IP & Tech Transfer)
Moderators: Marko Jakovljevic and Zachary Sellers

12:00-12:15 PM
Small Group Wrap Up

12:15-12:45 PM
GROUP LUNCH

1:15-12:30 PM
Speed Dating I
Moderators: Marko Jakovljevic and Zachary Sellers

Participants will meet 1:1 with faculty on various topics of interest to participants. Individuals available for meetings will be posted on a sign-up sheet.

*See page 15 for details about Speed Dating

2:30-2:45
BREAK

2:45-3:30 PM
Stories in Translational Medicine I
Speaker: Maria Grazia Roncarolo
Moderators: Tamar Green and Sriram Vaidyanathan

Learning Objectives:
• Explore how to develop drugs successfully in an academic setting
• Discuss the pro's & cons of partnering with Big Pharma
• Critically think through cases for drugability

3:30-3:45 PM
BREAK

3:45-4:45 PM
Designing an Innovative Clinical Trial I: Pre-Market Product Development
Speaker: Shivaani Kummar
Moderators: Tamar Green and Sriram Vaidyanathan

Learning Objectives:
• Discuss the principles and steps of drug discovery and development
• Describe the phases of clinical drug development, discussing some of the recent changes
• Discuss some of the design considerations for early phase trials

4:45-5:00 PM
BREAK

5:00-5:15 PM
Introduction to Mentoring
Speaker: Janet Hafler
Moderators: Marko Jakovljevic, Melissa Mavers, and Zachary Sellers
5:15-6:15 PM
Mentoring Session I
Speaker: Janet Hafler
Moderators: Marko Jakovljevic, Melissa Mavers, and Zachary Sellers

Participants will address their own dilemmas in a small group setting as facilitated by expert faculty.

Learning Objectives:
• Discuss effective mentoring
• Explore strategies to facilitate discussion in the groups

*See page 15 for details about Mentoring

6:15-6:30 PM
BREAK

6:30 PM
Dinner Informal Q&A - Wellness in the Competitive Environment with Grace Gengoux
Moderators: Melissa Mavers and Manpreet Singh

Tuesday Social Night
7:30 PM, Firepit
(Next to volleyball court)
Relax, listen to the waves, and enjoy the bonfire!

Wednesday, February 12

8:00-8:30 AM
Coffee

8:30-9:00 AM
Debriefing and Learning Strategies
Speaker: Janet Hafler
Moderators: Tamar Green and David Hong

9:00-10:15 AM
Unfolding Case - Part III: CARDINALdx
Speaker: Vicki Seyfert-Margolis
Moderators: Mingxia Gu and Sriram Vaidyanathan

Senior Mentors: Janet Hafler, Maria Grazia Roncarolo, Vicki Seyfert-Margolis, and Manpreet Singh
Junior Mentors: Mingxia Gu, Trung Pham, Zachary Sellers, and Sriram Vaidyanathan

10:15-10:30 AM
BREAK

10:30-12:00 PM
Stories in Translational Medicine II
Speaker: Anthony Oro

Learning Objectives:
• Identify the essential elements of process development
• Discuss the difference between academic and process development teams
• Identify how to prepare for an IND submission
• Discuss how to interact effectively with the FDA

12:00-1:00 PM
GROUP LUNCH

1:00-2:15 PM
Speed Dating II
Moderators: Marko Jakovljevic and Zachary Sellers

Participants will meet 1:1 with faculty on various topics of interest to participants. Individuals available for meetings will be posted on a sign-up sheet.
Wednesday, February 12 - Cont’d

2:15-2:30 PM
BREAK

2:30-3:30 PM
Stories in Translational Medicine III
Speaker: Michael Snyder
Moderators: David Hong and Manpreet Singh

Learning Objectives:
• Discuss the when & how to spin out a company in an academic setting
• Explore how to interact effectively with VC’s
• Discuss how to protect your research & leverage intellectual property

3:30-3:45 PM
BREAK

3:45-4:45 PM
Designing an Innovative Clinical Trial II: Post-Market Analysis/Real World Evidence
Speaker: Vicki Seyfert-Margolis
Moderators: Tamar Green and Sriram Vaidyanathan

Learning Objectives:
• Explore what the post-market world means for successfully bringing a new product to patients and for continuing to monitor how well the product works in real world medical practices
• Discuss new models of approval that incorporate more real world post-market research into an evolving approval process

4:45-5:00 PM
BREAK

5:00-6:00 PM
Leveraging Venture Capital as a Physician Scientist
Speaker: Jerel Davis
Moderators: David Hong and Manpreet Singh

Learning Objectives:
• Explore alternative career paths for MDs and PhDs
• VC 101: Discuss the basics of venture capital
• Learn how venture capitalists decide what new companies to build or back

Thursday, February 13

8:00-8:30 AM
Coffee

8:30-9:00 AM
Debriefing and Learning Strategies
Speaker: Janet Hafler
Moderators: Tamar Green and David Hong

9:00-10:15 AM
Mentoring Session II
Speaker: Janet Hafler
Moderators: Marko Jakovljevic, Melissa Mavers, and Zachary Sellers

Participants will continue to address their own dilemmas in a small group setting as facilitated by expert faculty.

10:00 - 10:15 AM
BREAK

10:30 -11:00 AM
Closing Reflection
Speakers: Janet Hafler, Mary Leonard, and Maria Grazia Roncarolo

11:00 -12:00 PM
GROUP LUNCH

DEPARTURE
Eureka Dynamic Sessions

A significant element of the program is going to involve YOU talking, either one-to-one or in small groups. The aim of all these sessions is to help you to learn from each other and the faculty directly, and to focus on those parts of translation medicine, and a career in translational medicine, that matter most to you.

For some sessions, you will be allocated to a specific group (Unfolding Case Study; Mentoring); for the others, you will choose who you want to meet and/or which topic you want to discuss (Speed Dating).

Unfolding Case Study: CARDINALdx

This case examines how to effectively develop a diagnostic tool while leveraging the resources & expertise at Stanford. You will work in depth with the case in a pre-allocated small-group setting over three separate sessions.

Through this case, you’ll grapple with the development of the technology based on a clinical use case, explore how intellectual property plays a role in product development and analyze the associated regulatory requirements. You will also discuss and determine research and business strategies necessary to “translate” the product & formulate a potential company. Parallel concepts of collaboration and team will also be explored.

Mentoring

You will each present a dilemma you are currently facing to a small group of peers. You will be allocated to a group, which will be mentored by a faculty member. Your fellow course participants will act as a consultation group. The objective is to advance personal learning while practicing and improving approaches to, and organization of, problem solving.

Reflections using practical real-world problems will anchor the concepts raised in the didactic portion of the program.

As some of you already know, work with or have worked with some of the faculty members, every effort will be made to ensure you have “neutral” tutors guiding the sessions. In addition, unless otherwise specified and agreed to by the group, discussions in the mentoring session are treated as confidential.

You will have your first session on Tuesday and the second session on Thursday.

Speed Dating

“Speed Dating” provides the opportunity for you to have a series of one-on-one discussions with individual faculty for 10 minutes each. Who you talk with and on what topics are your choices!

Please consult the faculty biographies at the end of this booklet prior to completing the signup sheet, which will be prominently displayed and made available from Sunday.

There will be two sessions on Tuesday and Wednesday.
Dr. Agarwal is Associate Professor of pediatrics, section chief and clinical director for the division of Pediatric Stem Cell Transplantation and Regenerative Medicine.

Dr. Agarwal trained extensively in India as a pediatric hematologist-oncologist. Due to a strong interest in pursuing translational research in the field she chose to come to the USA and joined Cincinnati Children’s Hospital Medical Center (CHMC) in Ohio. She received extensive training and expertise in stem cell biology at the hospital for sick children Toronto, Canada and National Institutes of Health (NIH) in Bethesda, MD. At the hospital for sick children, she learned and developed stem cell assays and in vivo models of human hematopoiesis that were critical in understanding of stem cell biology and its clinical applications. At the NIH she spent two years and developed gene transfer assays in Hematopoietic cells. At the NIH, she also worked on developing the mammalian models for in vivo gene transfer in hematopoietic cells. During this time her work was published on chronic myeloid leukemia addressing the role of interaction of stromal cells with hematopoietic cells in the bone marrow. This publication defined conditions to favor the growth of benign hematopoietic cells in patients with chronic myeloid leukemia.

At CHMC, Cincinnati, she established the stem cell biology laboratory to further investigate the field of Hematopoietic stem cells focusing on the umbilical cord blood. She started the clinical Umbilical cord blood transplant program at CHMC. In the laboratory she was able to set up the assays to identify and collect highly purified hematopoietic cells from the cord blood. The engraftment and expansion potential of the cord blood derived hematopoietic cells was studied in the immune deficient mice. These models were then used to develop assays for gene transfer in Fanconi Anemia stem cells.

She has been at Stanford for the past 18 years and currently serves as the Medical Director of the Stem Cell Transplant and Regenerative Medicine program. Most of her work is focused on two projects 1) developing antibody based conditioning for patient’s undergoing stem cell transplantation. Antibody based conditioning is a breakthrough in the field and has the promise to reduce regimen related toxicity and 2) clinical trial using Tr1, regulatory T cell therapy to induce tolerance and reduce the risk of GVHD.
Crystal Botham, Ph.D. is Director of Research Development in the Department of Pediatrics at Stanford University. Dr. Botham provides strategic advice to faculty and others to enable competitive funding applications and productive research programs.

Dr. Botham is also the Director of the Biosciences Grant Writing Academy (https://grantwriting.stanford.edu). The Grant Writing Academy supports graduate and postdoctoral trainees in developing and articulating research strategies to tackle important scientific questions.
Jennifer Swanton Brown, RN, CCRP, has been with Stanford University since 2006, when she joined the IRB as Training Specialist. Jennifer joined the School of Medicine in 2008 as Manager of Regulatory Services and Education for Spectrum. She became the Director of the Clinical Research Quality office at its inception in March 2016, responsible to the Senior Associate Dean for Research. She has 30 years of experience in nursing, writing, training and education, and regulatory affairs, with emphasis in quality improvement and clinical trials. Her education is in Linguistics and Nursing, and she received her Master of in Liberal Arts from Stanford in 2013. Her clinical nursing experience included bone marrow transplant at UCSF, home care and hospice. After clinical nursing, Jennifer worked in the medical device industry for 10 years (Natus Medical) where she worked as a clinical specialist, technical writer, sales trainer, and ultimately Regulatory Affairs Manager.
Molly Bukro is a seasoned development professional currently serving as Director of Major Gifts with Lucile Packard Foundation for Children’s Health, the dedicated fundraising entity for Lucile Packard Children’s Hospital Stanford, and the child and maternal health programs at Stanford University School of Medicine.

With more than 16 years of experience in development including 12 in pediatric medicine at an academic medical center, she has a proven track record of establishing and maintaining trusted relationships with major and principal gift donors, volunteers, and leadership, in addition to soliciting and closing seven figure gift commitments.
Cheryl Cathey, Ph.D., joined the Office of Technology Licensing at Stanford in 2019. Prior to her current position, she spent much of her career as an executive and entrepreneur in the medical diagnostic and life science industries. She has extensive experience in business development, technical leadership, company formation, and product development. She’s worked at a variety of companies including Applied Biosystems, Caliper Life Sciences, Igen, and numerous startups, and was a co-founder and COO of a molecular diagnostic company focused on point-of-care pathogen detection. Cheryl earned a B.S. in Chemical Engineering from the University of Colorado, Boulder and M.S. and Ph.D. degrees in Chemical Engineering from Stanford.
Jerel Davis, Ph.D., is a Managing Director based in Vancouver, Canada. Since joining Versant in 2011, he has been involved in launching and investing in a number of Versant’s portfolio companies including Quanticel (sale), Novira (sale), Crispr (2016 IPO), Inception 4 (sale), Inception 5 (sale), Northern, Turnstone, BlueRock (sale), Repare, VenatoRx and Akero (2019 IPO). He has led Versant’s execution of creative corporate transactions with multiple pharmaceutical partners including Celgene, Roche and Bayer, and was instrumental in establishing Versant’s presence in Canada, including the creation of our company-building infrastructure in Vancouver, Toronto and Montreal. Jerel was promoted to Managing Director at Versant in 2015.

Prior to joining Versant, Jerel was an Associate Principal at McKinsey and Company where he advised healthcare corporations in pharmaceuticals, biotechnology, medical device and molecular diagnostics. He has worked in a number of healthcare markets globally including the U.S., Canada, Europe, China, Russia and India. Jerel was a post-doctoral fellow at Stanford University, where he also completed his Ph.D., and trained at Amgen as a researcher.
Grace Gengoux, Ph.D., BCBA-D, is a Clinical Associate Professor, Director of the Autism Intervention Clinic, and Well-being Director for the Department of Psychiatry and Behavioral Sciences at Stanford University. Dr. Gengoux is a licensed clinical psychologist with expertise in naturalistic developmental behavioral intervention for children with Autism Spectrum Disorder. Dr. Gengoux received her Ph.D. in Clinical Psychology from the University of California Santa Barbara and completed her clinical internship and postdoctoral fellowship at the Yale Child Study Center, before joining the Stanford University School of Medicine clinical faculty in 2010. She has specialized training in Pivotal Response Treatment (PRT) and has completed multiple clinical trials evaluating the effects of PRT on the social-communication competence of young children with autism. Along with her collaborators at Stanford, Dr. Gengoux has published peer-reviewed journal articles and book chapters on treatments for autism and regularly presents her research at professional conferences and community events. Dr. Gengoux serves as Associate Editor for the Journal of Positive Behavior Interventions and serves on the Board of Directors for Gatepath, the largest non-profit serving individuals with developmental disabilities in San Mateo and Santa Clara Counties. In her work as Department Well-being Director, Dr. Gengoux leads a taskforce charged with developing solutions to combat burnout by improving efficiency of practice and cultivating a stronger culture of wellness in the Department of Psychiatry. Dr. Gengoux is particularly passionate about improving professional fulfillment for early career faculty and implementing system-level interventions to support staff, faculty, and trainees to make meaningful contributions in their chosen field.
Kevin Grimes is a Professor of Chemical and Systems Biology and the Co-director of the SPARK Program in Translational Research at the Stanford University School of Medicine. He began his career as a Clinical Assistant Professor of Medicine at Stanford, where his primary duties included the teaching and practice of internal medicine. Grimes received a Hartford Foundation Fellowship to study health economics and obtained an MBA at the Stanford Graduate School of Business. He was subsequently selected as a White House Fellow and assigned to the Department of Defense, where he served as Special Assistant to the Secretary. He spent fifteen years in industry, working in the medical device, life science consulting, and biotechnology sectors prior to returning to Stanford to co-direct SPARK. SPARK's three-fold mission is to advance promising research discoveries into the clinic as new therapeutics and diagnostics; to educate faculty, post-doctoral fellows and students regarding the translational process; and to identify and promote more efficient approaches to bring new therapies to patients. Over 50% of projects completing SPARK have been licensed and/or advanced to clinical trials. The SPARK methodology has been adopted by over 50 academic institutions throughout the world in a SPARK Global network that facilitates collaborative translational research on important medical issues. Grimes also teaches graduate student courses on drug discovery and development and continues to teach and practice internal medicine. He has received the David Rytand Award for Excellence in Clinical Teaching and the Faculty Award for Excellence in Graduate Teaching. She has served as visiting professor internationally and has been invited to present regularly at regional and national professional meetings.
Janet Hafler is a Professor of Pediatrics and is the Associate Dean for Educational Scholarship at Yale University School of Medicine. As the Director of the Teaching and Learning Center her responsibilities include developing and implementing medical education and teaching and learning programs for faculty members, students and residents. Over her career she has nurtured a climate in teaching and learning where faculty and residents have been exposed to the cutting edge literature and ideas in medical education. She has focused on assisting faculty and residents in exploring innovative ways to effectively promote learning in both the classroom and clinical settings.

Promoting, influencing and nurturing a climate in which physicians, residents and students can teach — and learn — has been foremost among her career objectives. She has focused on providing an awareness of context for students, residents and faculty, urging them to be innovative in their many teaching environments and encouraging them to explore ways to understand how they can effectively promote learning in their interactions among themselves.

Dr. Hafler runs an active research program applying qualitative research methods in medical education. She collaborates with and mentors clinicians and faculty on the elements of qualitative research in the field of medical education and medical care. In turn, mentored faculty members have learned to develop and demonstrate the tools necessary to effectively teach and lead others. Dr. Hafler has published over 100 book chapters, curriculum materials and original articles in medical education and
Dr. Kummar’s research interests focus on developing novel therapies for cancer. She specializes in conducting pharmacokinetic and pharmacodynamic driven first-in-human trials tailored to make early, informed decisions regarding the suitability of novel molecular agents for further clinical investigation. Her studies integrate genomics and laboratory correlates into early phase trials. She is interested in alternate trial designs to facilitate rational drug selection based on human data and help expedite drug development timelines. She has published numerous articles in medical journals and serves on a number of national and international scientific committees.
Mary Leonard, MD, MSCE, is the Arline and Pete Harman Professor and Chair of the Department of Pediatrics at Stanford University School of Medicine and the Adalyn Jay Physician in Chief at Lucile Packard Children’s Hospital Stanford. She assumed these positions on July 1, 2016.

Energetic and collaborative, Dr. Leonard is a compassionate clinician and researcher who cares deeply about improving the health and well-being of children everywhere. A graduate of the Stanford University School of Medicine, Mary returned to Stanford Medicine in 2014 after spending 25 years at the Children’s Hospital of Philadelphia and the University of Pennsylvania. At Stanford, her multi-disciplinary research program is focused on the impact of chronic diseases on bone metabolism and nutrition across the life span. Mary directs the innovative and trans-disciplinary child and maternal health research and training initiatives of the Stanford Child Health Research Institute.

Mary is a distinguished investigator, an expert clinician, and a respected mentor who embodies the academic and integrated mission of Stanford Medicine. A member of the Precision Health Committee, she is committed to Stanford Medicine’s vision of proactive and personalized health care and has been at the forefront of efforts to integrate Precision Health approaches and skills into our training programs.
Margaret A. Neale is the Adams Distinguished Professor of Management, Emerita. She was the Graduate School of Business John G. McCoy-Banc One Corporation Professor of Organizations and Dispute Resolution from 2000-2012. Trust Faculty Fellow in 2011-2012 and in 2000-2001. From 1997-2000, she was the Academic Associate Dean of the Graduate School of Business at Stanford University. Prior to joining Stanford’s faculty in 1995, she was the J.L. and Helen Kellogg Distinguished Professor of Dispute Resolution and Organizations at the J.L. Kellogg Graduate School of Management at Northwestern University. She received her Bachelor’s degree in Pharmacy from Northeast Louisiana University, her Master’s degrees from the Medical College of Virginia and Virginia Commonwealth University and her PhD in Business Administration from the University of Texas. She began her academic career as a member of the faculty at the Eller School of Management of the University of Arizona.

Professor Neale’s major research interests include bargaining and negotiation, distributed work groups, and team composition, learning, and performance. She is the author of over 70 articles on these topics and is a coauthor of three books: Organizational Behavior: A Management Challenge (third edition) (with L. Stroh and G. Northcraft) (Erlbaum Press, 2002); Cognition and Rationality in Negotiation (with M.H. Bazerman) (Free Press, 1991); Negotiating Rationally (with M.H. Bazerman) (Free Press, 1992); and one research series Research on Managing in Groups and Teams (with Elizabeth Mannix) (Emerald Press). She is or has served on the editorial boards of the Administrative Science Quarterly, Journal of Applied Psychology, Organizational Behavior and Human Decision Processes, International Journal of Conflict Management, and Human Resource Management Review.

In addition to her teaching and research activities, Professor Neale has conducted executive seminars and management development programs in the United States, United Kingdom, Australia, Holland, Switzerland, Brazil, Thailand, France, Canada, Nicaragua, the People’s Republic of China, Hong Kong, United Arab Emirates, Mexico, Israel, and Jamaica for public agencies, city governments, health care and trade associations, universities, small businesses and Fortune 500 corporations in the area of negotiation skills, managerial decision making, managing teams, and workforce diversity. She is the faculty director of three executive programs at Stanford University: Influence and Negotiation Strategies, Managing Teams for Innovation and Success, and the Executive Program for Women Leaders.
Anthony E. Oro, M.D., Ph.D., is the Eugene and Gloria Bauer Professor of Dermatology, Associate Director of the Center for Definitive and Curative Medicine, and the co-director of the Child Health Research Institute. He is co-founder of the Program in Epithelial Biology, and an active member of the Institute for Stem Cell Biology and Regenerative Medicine, Children’s Health Research Institute, Bio-X, and the Program in Cancer Biology. His research interests encompass cancer genomics and tumor evolution, stem cell biology and hair/skin development and regeneration, and definitive molecular and cellular therapeutics. His clinical interests include hair biology, non-melanoma skin cancer, and stem cell-based therapies for genetic skin diseases.
Thomas Robinson, MD, MPH, is the Irving Schulman, MD Endowed Professor of Child Health and Professor of Pediatrics and of Medicine at Stanford University School of Medicine.

Dr. Robinson designs solutions to improve health and well-being of children, families, and the planet. Dr. Robinson originated the solution-oriented research paradigm and directs the Stanford Solutions Science Lab. He is known for his pioneering obesity prevention and treatment research, including the concept of stealth interventions. His research applies social cognitive models of behavior change to behavioral, social, environmental and policy interventions for children and families in real world settings, making the results relevant for informing clinical and public health practice and policy. His research is largely experimental, conducting rigorous school-, family- and community-based randomized controlled trials. He studies obesity and disordered eating, nutrition, physical activity/inactivity and sedentary behavior, the effects of television and other screen time, adolescent smoking, aggressive behavior, consumerism, and behaviors to promote environmental sustainability. He is published widely in the scientific literature and a frequent appointee to expert and advisory panels for leading national and international scientific and public health agencies and organizations. Dr. Robinson also teaches undergraduate and graduate students at Stanford, and practices Pediatrics at Lucile Packard Children’s Hospital. He received his B.S. and M.D. from Stanford University and his M.P.H. from the University of California, Berkeley. He trained in Pediatrics at Children’s Hospital, Boston and Harvard Medical School.
Maria Grazia Roncarolo, MD

Professor of Pediatrics (Stem Cell Transplantation) and of Medicine (Blood & Marrow Transplantation), Stanford School of Medicine; Director of Stanford Center for Definitive and Curative Medicine; Co-Director of Institute for Stem Cell Biology and Regenerative Medicine, Stanford School of Medicine

Maria Grazia Roncarolo, MD is the George D. Smith Professor in Stem Cell and Regenerative Medicine, Professor of Pediatrics and of Medicine, Director of the Center for Definitive and Curative Medicine, and Co-Director of the Institute for Stem Cell Biology and Regenerative Medicine.

Dr. Roncarolo leads efforts to translate scientific discoveries in genetic diseases and regenerative medicine into novel patient therapies, including treatments based on stem cells and gene therapy.

A pediatric immunologist by training, she earned her medical degree at the University of Turin, Italy. She spent her early career in Lyon, France, where she focused on severe inherited metabolic and immune diseases, including severe combined immunodeficiency (SCID), better known as the “bubble boy disease.” Dr. Roncarolo was a key member of the team that carried out the first stem cell transplants given before birth to treat these genetic diseases.

While studying inherited immune diseases, Dr. Roncarolo discovered a new class of T cells. These cells, called T regulatory type 1 cells, help maintain immune system homeostasis by preventing autoimmune diseases and assisting the immune system in tolerating transplanted cells and organs. Dr. Roncarolo completed the first clinical trial using T regulatory type 1 cells to prevent severe graft-versus-host disease in leukemia patients receiving blood-forming stem-cell transplants from donors who were not genetic matches.

Dr. Roncarolo worked for several years at DNAX Research Institute for Molecular and Cellular Biology in Palo Alto, where she contributed to the discovery of novel cytokines, cell-signaling molecules that are part of the immune response. She studied the role of cytokines in inducing immunological tolerance and in promoting stem cell growth and differentiation.

Dr. Roncarolo developed new gene-therapy approaches, which she pursued as director of the Telethon Institute for Cell and Gene Therapy at the San Raffaele Scientific Institute in Milan. She was the principal investigator leading the successful gene therapy trial for SCID patients who lack an enzyme critical to DNA synthesis, which is a severe life-threatening disorder. Based on the results of this trial, gene therapy for ADA-SCID has obtained Orphan drug status from both the FDA and EMEA and it was licensed to Glaxo Smith Klein, which has received European Commission approval to market under the name of Strimvelis. Under her direction, the San Raffaele Scientific Institute has been seminal in showing the efficacy of gene therapy for otherwise untreatable inherited metabolic diseases and primary immunodeficiencies.

Dr. Roncarolo established the Stanford Center for Definitive and Curative Medicine to cure patients with currently incurable diseases through the development of innovative stem cell-and gene-based therapies.
Vicki Seyfert-Margolis, PhD is the founder and CEO of My Own Med, Inc., a company specializing in using digital technologies to support real world evidence clinical research. Previously, Dr. Seyfert-Margolis was the Senior Advisor for Science Innovation and Policy in the Office of the Commissioner of the US Food and Drug Administration. While at the FDA, she oversaw the development and execution of an agency wide strategic plan for regulatory science. Prior to the FDA, she served as Chief Scientific Officer at the Immune Tolerance Network (ITN), a non-profit consortium of researchers seeking new treatments for diseases of the immune system. At ITN, Dr. Seyfert-Margolis oversaw the development of over 20 leading edge assay development and centralized laboratory facilities, bringing them to GLP and CLIA compliance. She designed and implemented biomarker discovery studies for over 25 Phase II clinical trials across a broad array of immunologically mediated diseases including autoimmune disorders, allergy, and solid organ transplantation. Prior to this, she served as Director of the Office of Innovative Scientific Research Technologies at the National Institute of Allergy and Infectious Diseases at NIH, where she worked to integrate emerging technologies into existing immunology and infectious disease programs. Dr. Seyfert-Margolis completed her PhD in immunology at the University of Pennsylvania’s School of Medicine, and her post-doctoral fellowship work at Harvard University and the National Cancer Institute. Vicki also serves on Board of Directors for the EveryLife Foundation for Rare Diseases, and Eureka Institute for Translational Medicine.
Manpreet Singh, MD, MS

Associate Professor of Psychiatry and Behavioral Sciences (Child & Adolescent Psychiatry), Stanford School of Medicine; Director of the Pediatric Mood Disorders Program

Dr. Singh is Associate Professor of Psychiatry and Behavioral Sciences, and Director of the Pediatric Mood Disorders Program in the Division of Child and Adolescent Psychiatry at Stanford. Her time is divided among the clinical, research, and teaching missions of department. She directs Stanford’s Pediatric Mood Disorders Program, which is an integrated multidisciplinary clinic that aims to treat youth with a spectrum of mood disorders along a developmental continuum. She leads a team of child and adolescent psychiatrists, psychologists, child and adolescent psychiatry fellows, clinical and research postdoctoral fellows, residents, medical students, and research coordinators. Her research focuses on investigating the origins and pathways for developing mood disorders during childhood, as well as methods to protect and preserve function before and after the onset of early mood problems.

Dr. Singh’s research team (Pediatric Emotion And Resilience Lab) conducts innovative research examining the neural, cognitive, and genetic underpinnings of pediatric mood disorders. She has extensive experience with multi-level investigations involving children and families, as well as clinical, neuroimaging, and dimensionally-based behavioral assessments. She completed her NIMH career development award that characterizes emotion regulation in healthy offspring of parents with bipolar disorder, and has been leading three independent NIMH funded studies examining the mechanisms of mood and other psychiatric disorders and their treatments among youth. She is extensively involved in collaborations aimed to investigate methods of treating problems associated with and leading up to mood disorders in youth. Specifically, she is examining the benefits of family focused psychotherapy, mindfulness meditation, and medications in youth with or at risk for mood disorders to reduce mood symptoms and family stress. She has also been reviewing the neural effects of medication and psychotherapy in youth. These areas of research hold considerable promise to impact our understanding of the core mechanisms and early interventions for pediatric onset mood disorders.
Michael Snyder, PhD, is the Stanford W. Ascherman Professor and Chair in the Department of Genetics, and Director of the Center for Genomics and Personalized Medicine at Stanford University.

Dr. Snyder received his Ph.D. training at the California Institute of Technology and carried out postdoctoral training at Stanford University. He is a leader in the field of functional genomics and proteomics, and one of the major participants of the ENCODE project.

His laboratory study was the first to perform a large-scale functional genomics project in any organism, and has developed many technologies in genomics and proteomics. These including the development of proteome chips, high resolution tiling arrays for the entire human genome, methods for global mapping of transcription factor binding sites (ChIP-chip now replaced by ChIP-seq), paired end sequencing for mapping of structural variation in eukaryotes, de novo genome sequencing of genomes using high throughput technologies and RNA-Seq. These technologies have been used for characterizing genomes, proteomes and regulatory networks.

Seminal findings from the Snyder laboratory include the discovery that much more of the human genome is transcribed and contains regulatory information than was previously appreciated, and a high diversity of transcription factor binding occurs both between and within species.

He has also combined different state-of-the-art “omics” technologies to perform the first longitudinal detailed integrative personal omics profile (iPOP) of person and used this to assess disease risk and monitor disease states for personalized medicine. Snyder is a cofounder of several biotechnology companies, including Protometrix (now part of Life Tehcnologies), Affomix (now part of Illumina), Excelix, Personalis and founder of Qbio, and he presently serves on the board of a number of companies.
Dr. James Wall is a pediatric surgeon who focuses on minimally-invasive approaches to children's surgery. He is an alumnus of the Stanford Biodesign Innovation Fellowship. His research focuses on how to educate others to design and develop health technology, as well as on flexible endoscopic surgery in children. He has developed multiple health technologies including a novel epidural needle, a protection device for umbilical catheters, and a wearable leg compression system. James currently holds the roles of director of Program Development for the Stanford Biodesign Innovation Fellowship, chairman of the perioperative value analysis committee for Lucile Packard Children’s Hospital, and co-director of the UCSF-Stanford Pediatric Device Consortium. James graduated from Tulane University with an undergraduate degree in biomedical engineering and has a masters in bioengineering from Stanford. He attended the University of Pennsylvania School of Medicine and completed a general surgery residency training program at the University of California, San Francisco. He completed a fellowship in minimally invasive surgery at the IRCAD institute in France followed by a Pediatric Surgery fellowship at Stanford.
Crystal Botham, PhD

Director of Research Development in the Department of Pediatrics, Stanford School of Medicine; Director of the Biosciences Grant Writing Academy, Stanford University

Crystal Botham, Ph.D. is Director of Research Development in the Department of Pediatrics at Stanford University. Dr. Botham provides strategic advice to faculty and others to enable competitive funding applications and productive research programs.

Dr. Botham is also the Director of the Biosciences Grant Writing Academy (https://grantwriting.stanford.edu). The Grant Writing Academy supports graduate and postdoctoral trainees in developing and articulating research strategies to tackle important scientific questions.

Julie Ledford, PhD

Assistant Professor of Clinical Translational Sciences; Associate Professor of BIOS Institute, of Cellular and Molecular Medicine, of Immunobiology, and of Medicine, Department of Medicine, University of Arizona College of Medicine

Dr. Julie Ledford received her PhD from the University of North Carolina at Chapel Hill. Her lab is focused on determining mechanisms by which endogenous lung proteins mediate various states of lung inflammation, pathogen infection and lung disease progression using a translational research approach between human samples and mouse models. Studies are currently focused around the action of two proteins, surfactant protein-A and club cell secretory protein-16. Based on mechanistic studies of these two endogenous proteins, another aspect of the lab has moved into drug development and testing in pre-clinical animal models. Her research lab is funded by the NIH with 2-R01s and an R21.
I am an Instructor in the Department of Psychiatry and Behavioral Sciences, Division of Interdisciplinary Brain Sciences, in the Stanford University School of Medicine. Since obtaining my Ph.D. in Experimental Psychology in 2013, I have had the great fortune of extending my training as a postdoctoral scholar in the Center for Interdisciplinary Brain Sciences Research, with the express intention of broadening my expertise in clinical neuroscience. My productivity towards this goal has been high; since coming to Stanford, I have amassed 16 peer-reviewed publications (8 as first-author), engaged in multiple international research conferences and other scholarly activities, and have maintained a mentorship role for multiple student and postdoctoral researchers. Notably, my research was recently supported by a highly competitive NIH K99/R00 Pathway to Independence Award, wherein I am investigating the neural and behavioral effects of Turner syndrome on number sense.

As I reflect on my time at Stanford, I am awestruck at the number and quality of career development opportunities that have been presented to me. From a scientific perspective, I am regularly provided with opportunities to collaborate in areas of science that are often new to me, yet which may benefit from my unique background. Indeed, the ability to expand on my training via interdisciplinary collaborations is one of many reasons I love my work. However, I have come to learn that many of the skills that are necessary to develop, operate, and sustain a long lasting and impactful career in translational medical research are more nuanced than the common “publish or perish” idiom would suggest. Namely, it is vitally important that translational scientists possess the skills to communicate with experts from fields other than their own, and that they be exposed to each other in a forum that encourages interdisciplinary collaboration. These represent two key components of the Eureka Certificate Course in Translational Medicine that I look forward to exploring in greater detail.

As I progress in my career as an independent researcher, I look forward to opportunities to pursue translational research that leverages the strength of interdisciplinary collaborations in an effort to provide the most impactful outcome possible for human health and society. Specifically, I look forward to championing these collaborations, and actively seeking out and developing partnerships that are optimally suited to address outstanding problems. To this end, the Eureka Certificate Course in Translational Medicine will be an ideal addition to my training repertoire, and will provide me with the exact skillset and community of like-minded researchers that I need to help make my goals attainable.
I am an interdisciplinary basic scientist seeking to develop in a translational direction. My expertise in human auditory-vocal communication has been developed through neuroscientific, psychological, biological, and acoustic studies of speech, music, and animal vocalization, primarily as they relate to affect and social function. As an instructor in the Department of Psychiatry and Behavioral Sciences at Stanford School of Medicine, I am using a combination of acoustically parallel speech and music stimuli to develop a “gold standard” assessment of auditory-vocal affect perception in individuals with autism spectrum disorder (ASD). This work—which is the subject of two pending grant applications—represents my first attempt to realize the translational potential of my work. It is designed to rigorously define auditory-vocal contributions to core social deficits in ASD, for improved characterization of the disorder, advanced behavioral phenotyping, and objective assessment of clinical outcomes. I am applying to the MCHRI Eureka Certificate Course in Translational Medicine because I desire an intensive, focused experience that will rapidly increase my ability to (1) impact ASD, and (2) develop translational applications for other components of my research program.

My ongoing transition from basic to applied science is motivated by acknowledging, in myself, a driving need to do work that is relevant to society in the here and now. This development has been punctuated by fatherhood (children born in 2016 and 2018), which has opened my eyes to the challenges faced by young families (particularly children and mothers) and the necessity of contributing to a better future. Joining the faculty at Stanford School of Medicine has been a breath of fresh air in this respect. Despite having long thought of myself as a purely basic scientist, I have come to see that my focus on a core component of human social behavior (auditory-vocal communication) has furnished expertise and skills that apply to a broad range of problems in human health. My current work on ASD provides one clear example, but I have a number of other ideas that I am keen to pursue in a translational way. Some ideas extend my analysis of auditory-vocal affect in ASD to other mental disorders characterized by impairments in social communication and/or affect (e.g., major depressive disorder and schizophrenia). Other ideas parallel growing interest in the medical applications of music (e.g., see NIH RFA-NS-19-008, NS-19-009, and AT-19-001). For example, leveraging the relationship between music making and endogenous opioid release for applications in pain management, leveraging the connection between rhythm and movement for applications in motor rehabilitation, and empirically defining the elements of lullaby and parental rocking that work to promote sleep in infants for applications in family health. Finally, I currently pursue health-related applications of music through my role as a scientific advisor for Spiritune, a company that applies insights from basic science to create musical solutions for affect regulation and stress management.

Preliminary signs of success in my work on ASD at Stanford have made me optimistic about my potential to make a meaningful contribution to public health as a translational researcher. But I have much to learn. In particular, I want to improve my understanding of how to formulate and position my new ideas to obtain translational funding, how to accelerate the collection of preliminary data, how to work through the advantages and disadvantages of private vs. federal funding, if and when to take my ideas to industry, and how to reach beyond academia to access business expertise and form teams that can successfully deliver products to market. Finally, I am keen to meet other researchers that work between basic and applied science, to benefit from our collective experience and make connections for the future.
As a pediatric pulmonologist I aspire to improve the lives of children with cystic fibrosis allowing them to live longer, healthier lives. With my clinical research experience in both clinical trials and retrospective reviews prior to specialty training followed by my experience conducting translational research during my fellowship in pediatric pulmonary medicine, I have set myself on a trajectory pursuing a career in translational research.

Early in my training I identified an interest in pursuing hypothesis driven questions but was able to find an area I was passionate about within pediatric pulmonary medicine. I was able to blend my interest in cystic fibrosis, of which I have personal experience with multiple family members, and my interest in the host pathogen interaction. My fellowship project exploring the role of a Pseudomonal bacteriophage in the pathogenesis of lung disease in cystic fibrosis was my first experience with translational research.

While I collected patient samples and reviewed patient data, I also brought my samples to the laboratory where I learned to process them, extract DNA and perform qPCR to identify and study the Pf bacteriophage. Most importantly I became part of a lab and became involved in experimental design, review of data and critiques of other scientific products. I gained an understanding of the real importance of the intersection between basic science and clinical medicine. While it may be more difficult to straddle both these worlds the questions you can address and results you can produce are more meaningful and will often lead to faster implementation or practice change, in comparison to when working in the silos of one or the other. As I had primarily clinical experience prior to fellowship I did not appreciate the importance of having a clinical perspective in the laboratory, nor did I appreciate having an understanding of laboratory science can better inform clinical research. While I have gained experience conducting translational research with elements of patient recruitment, enrollment, sample collection, sample processing and banking, limited laboratory methods, limited biostatistics and data analysis, there are many topics in which further training would augment my ability to carry out translational research and eventually develop my own research program. Specifically training in the business and regulatory aspects, navigating mentorship when working with both clinicians and basic scientists, how to communicate complex scientific ideas and issues relate to intellectual property and design thinking are areas included in the Eureka Certificate Course.

I am currently an instructor in pediatric pulmonary medicine with 3 years (currently starting the 2nd year) of funding support from the department of Pediatrics to provide me with salary support to maintain 75% of my effort in research. I additionally have salary support from the Parker B Francis Fellowship which is a prestigious training grant within the field of research in respiratory disease (currently in year 2 of 3). I was recently awarded a 2-year, mentored grant from Vertex Pharmaceuticals for support of my research on Pseudomonas infection in patients with cystic fibrosis. I am currently in the resubmission phase of a K23 application after receiving generally favorable review from my initial submission. I have published my initial findings in Science Translational Medicine last spring and will be presenting my data at the North American Cystic Fibrosis Conference as an oral presentation. I have thoroughly enjoyed my pursuits in translational research thus far and am excited about continuing these pursuits. I am committed to continuing to pursue a career as a physician scientist and am very interested in obtaining more training to improve my effectiveness.

Elizabeth Burgener, MD
Instructor, Department of Pediatrics
I am an instructor in the laboratory of Dr. Maria Grazia Roncarolo, in Pediatric Division of Stem Cell Transplantation and Regenerative Medicine at Stanford University School of Medicine. I am a Croatian-trained physician and a scientist focused on translational research, human immunology and systems biology. I aim to apply state-of-the-art technology to understand the molecular mechanisms governing human immune responses, and leverage it to cure currently untreatable diseases.

My over-arching career goal is to become a leader in the field of immune tolerance. I want to translate my knowledge into cell and gene therapies for debilitating pediatric disorders, as these are often rare and thus not prioritized in the drug development pipeline of big pharmaceutical companies. At Stanford, I was impressed with the translational mindset of this Division, of the Department of Pediatrics, and of the Center for Definitive and Curative Medicine (CDCM). This mindset is complemented by stellar research faculty with strong collaborative spirit. I am currently applying to become Instructor in Pediatrics; I am eager to continue my work here and acquire skills needed for an independent academic position, while contributing to the Division's, Department's and CDCM mission.

My current project is focused on understanding the molecular regulators of inducible, patient antigen-specific type 1 regulatory T cells (Tr1). These cells are the active ingredient of the T-allo10 cell therapy product, where donor-derived CD4 T cells are made tolerant to patient antigens, and could suppress harmful anti-host responses in allogeneic hematopoietic stem cell transplantation (trial ID NCT03198234, Sponsor Dr. Roncarolo). Understanding Tr1 differentiation during T-allo10 production will allow us to manipulate this process and increase Tr1 frequency within the product, thus increasing the T-allo10 clinical efficacy, and ultimately improving patient outcomes.

To learn about the patient needs and the trial progress, I am regularly participating in the weekly T-allo10 clinical trial updates, together with other basic researchers, clinical investigators and CTO and LCGM staff (Clinical Trial Office and Laboratory for Cell and Gene Medicine, respectively). Therefore, my main research project is already translational in nature, providing me a window into the process of moving a cell therapy through a clinical trial.
I am currently in my 2nd year on the faculty in the Department of Pediatrics, Division of Stem Cell Transplantation and Regenerative Medicine, and have a research focus and clinical interest on expanding stem cell transplantation through the use of innovative conditioning regimens. To this end, I completed my MD degree and PhD in Developmental Biology at the Stanford University School of Medicine and during my graduate studies conducted pioneering work with Prof. Irv Weissman on better understanding the barriers to purified hematopoietic stem cell (HSC) transplantation. Specifically, I showed that host HSCs compete with donor HSCs for engraftment; and identified that engraftment of donor HSCs could be enhanced using antagonistic antibodies to CD117 that deplete host HSCs. I published several high-profile, first-author and middle-author publications during this time, but importantly also identified a clinical agent that could potentially be used as a conditioning therapeutic in patients. This drug, AMG-191, has now entered clinical trials at Lucile Packard Children's Hospital Stanford and is excitingly showing promising early safety and efficacy data in severe combined immunodeficiency patients when used as a single-agent conditioning drug.

Subsequently, I completed my Pediatrics residency training in the Boston Children’s Hospital Combined Residency program and further entered the Pediatric Hematology and Oncology fellowship program at the Dana Farber Cancer Institute to pursue sub-specialty training. During this time I conducted additional innovative research with Prof. Derrick Rossi and Prof. David Scadden, where I showed that CD117 antibody-drug-conjugates could act as even more potent conditioning agents and moreover could also be used in combination with antibody-based immunosuppression to enable complete MHC- mismatched allogeneic transplantation without graft vs. host disease. The intellectual property stemming from this work was subsequently licensed to Magenta Therapeutics which is now developing a similar anti-human CD117 antibody-drug- conjugate that is showing promising results in non-human primates and is likely to be tested in patients as early as 2020.

Given the past exciting translational efforts that have stemmed from my research as a trainee, I subsequently returned to Stanford to complete my fellowship and begin my independent faculty career. This has allowed me to advance my independent research ideas and play a more advanced role in clinical care and translational efforts. Specifically, together with colleagues in the Division of Hematology I have been strengthening our Bone Marrow Failure program. As part of this, we are developing new treatments for Fanconi Anemia (FA) patients, and I am a clinical PI on a first-in-class Phase I FANCA gene therapy trial and have been developing an Investigator Sponsored Antibody conditioning trial for FA patients as well. Furthermore, I have also been running an independent research laboratory that is working to better understand stem cell conditioning, learning how to effectively apply antibody conditioning in a variety of settings, and additionally working to develop new further improved conditioning approaches.

I hope to see many new therapies generated from my future discoveries and I believe developing the formal skills through the Eureka Institute to bring my discoveries efficiently from the bench to the bedside would be invaluable. My past involvement in the advancement of some of my past discoveries, has made me realize the complexity of the translational research process and the importance of acquiring specific training in order to be successful. Although I have had strong formal training to-date, this has primarily been focused on basic science research and clinical training, and I feel there is a gap in my knowledge base which could be further filled by this program.

As an aspiring leader in pediatric stem cell transplantation, I feel that this program will be invaluable and directly aid me in being more effective in translational research now and in the future to further grow the impact of my work. The exposure this program provides to experts from academia, industry, government agencies, and intellectual property law appears truly spectacular and seems critical in equipping participants to truly understand the process of, and engage in, translational research. As a faculty member in Pediatrics on the University Tenure Line, I hope to be a long-term member of our Stanford translational medicine ecosystem and also aspire to teach translational medicine to many trainees.
Richard Frock, PhD  
Assistant Professor, Department of Radiation Oncology

I am a recently appointed Assistant Professor in Radiation Oncology on the UTL and am in the process of building up/developing my laboratory. My research goal is to elucidate mechanisms of DNA double strand break repair pathway choice, in part by using a high-throughput genome-wide chromosome translocation sequencing technology that I co-developed as a postdoctoral fellow (termed HTGTS) in Fred Alt's group at Boston Children's Hospital. I am a co-author on a patent application describing this innovative technology, which has been licensed by Intellia Therapeutics for genome editing.

So, while my scientific training thus far has predominantly focused on basic research and technology development, I do not yet have a clear picture of how best to take advantage of translational medicine resources and opportunities here at Stanford. I believe that participating in the MCHRI Eureka Translational Medicine Course will help to accelerate my professional development and enhance my abilities to more effectively incorporate translational medicine into my research. In this regard, I plan to employ and adapt the HTGTS technology to discover novel drug targets, combinatorial therapeutics, and diagnostics to treat a wide-range of adult and childhood cancers; the basic mechanisms that I uncover from DNA repair research will also have additional translational benefits toward genome editing approaches. While I am already collaborating with a number of investigators here (e.g. Matt Porteus, Karlene Cimprich, Mike Bassik), I believe my skill set will be much better complemented to have taken this course. The acquired skills could be readily applied at a critical juncture in my career to fortify ongoing collaborations and to additionally establish new ones, particularly with other investigators interested in maternal and child health, that could lead to very successful and long-lasting relationships.
Yang Hu, MD, PhD
Assistant Professor, Department of Ophthalmology

After medical school in China I initially trained as an ophthalmologist and later earned a Ph.D. in neuroscience at Cornell. After my postdoctoral training at Harvard, I took a position at Merck and worked there briefly (1.5 yrs) to learn how pharmaceutical companies function. I then established my own lab to pursue my strong long-standing interest in neurodegeneration and axon regeneration, especially in retina and optic nerve. My lab focuses on understanding the mechanisms responsible for retinal ganglion cell degeneration and optic nerve regeneration after injury and in diverse optic nerve diseases including glaucoma, which is the leading cause worldwide of irreversible blindness. The long-term goal is to build on this understanding to develop effective strategies to promote neuroprotection and visual function recovery. Stanford provides a uniquely rich environment for basic and translational vision research. I have established extensive collaborations with Stanford scientific colleagues in the departments of Ophthalmology, Neuroscience, Genetics, Bioengineering, Chemistry, and even Computer Science. I hold one patent “Promoting Axon Regeneration In The Adult CNS Through Control Of Protein Translation, Publication Date: 12-10-2009, Publication Number: 20090305333”; and have filed two provisional patent applications through Stanford OTL. My current awards include 3 NIH R01 grants, one NIH R21 grant and 4 foundation grants as the sole PI and two additional grants as Co-I. These achievements are the best demonstration of my reputation as a leader in the glaucoma and neuroprotection/regeneration fields.

As my research program enters a critical phase with multiple promising therapeutic targets and novel tools, I realize that I need additional practical knowledge about translational medicine, especially the business and regulatory aspects of it. For example, several pharmaceutical and biotech companies have approached me to explore the possibility of commercializing our findings or applying our tools in their research programs. However, while considering these ventures, I have struggled with many unfamiliar legal terms and different thinking processes. Although I now know that CDA means “confidential disclosure agreement”, I do not really understand its purpose or what content I should put into this kind of agreement. I am very enthusiastic about the Eureka Certificate Course in Translational Medicine sponsored by Stanford MCHRI, as I believe that I am exactly the type of trainee that this course designed to target. I attended the introductory webinar for this course and I am impressed by the overwhelmingly positive feedback from the course alumni. I carefully studied the online course agenda. The intensive five-day course appears to cover a host of important issues including communication skills, mentoring, resources for IP and business development, potential challenges, and networking. Although it is hard to incorporate this weeklong course into my busy schedule, I am certain I should take it because it will help to advance my research program and enable me to benefit patients and achieve my career goals. I am sure this course will not answer all my future questions, but it will provide essential information for conducting translational medicine, direct me toward the business models and regulations that I should follow, and, more importantly, teach me where to obtain what I need to push my efforts forward.

In summary, I have employed dramatically different, bold, transformative approaches to achieve significantly high impact in the fields of glaucomatous neurodegeneration and CNS axon regeneration. These achievements have established my credibility as a productive researcher in translational medicine, who will develop novel findings into effective treatments for patients. This course will help me to achieve this goal; it is a timely fit into the current stage of my career and will equip me with essential knowledge, tools and resources to overcome the challenges of the future.
I am a physician scientist in the fields of Pediatric Neurology and Epilepsy. As an Instructor at Lucile Packard Children’s Hospital, I spend >80% of my time conducting research, and the remainder caring for epilepsy patients. My career in translational medicine began as a MSTP student in the lab of Dr. Frank Longo, chair of Neurology at Stanford. My PhD work focused on p75 neurotrophin receptor (p75NTR)-related pathogenic mechanisms and therapeutic strategies for Alzheimer’s disease (AD). I discovered that p75NTR is required for amyloid beta-induced neurodegeneration, and that targeting this receptor with small molecule partial agonists prevented neurodegeneration and cognitive deficits in mouse models of AD. These studies contributed substantially to funding of a NIH U01 grant, and an ongoing clinical Phase 2 trial of a lead small molecule targeting p75NTR for the possible treatment of AD, as featured as a cover story in Time Magazine in February 2016.

Although I later changed the focus of my clinical and research career to pediatric neurological disease, the tools I gained in graduate school are still applicable. A seminal study, published by the laboratory of my post-doctoral research mentor, Dr. Michelle Monje at Stanford, demonstrated that neuronal activity drives myelin plasticity in vivo (Gibson et al, Science, 2014). Subsequently, there has been an extensive emerging literature supporting a critical role for activity-dependent myelination in neuronal network function. The question of how activity-dependent myelination might contribute in disease states where neuronal activity is pathological, such as epilepsy, is largely unexplored. My current research program investigates the relationship between seizures and myelin plasticity using rodent models. My data indicate that recurrent seizures are associated with abnormal myelination of neurons within the seizure network, and that this abnormal activity-dependent myelination further contributes to seizure severity. Ongoing work and future directions are focused upon identifying disease-specific molecular targets and applying advanced MRI methods to determine brain-wide myelin changes in rodents and humans with various forms of epilepsy. This work is funded by a NIH/NINDS K12 award, the CURE Foundation, the American Epilepsy Society and the Stanford MCHRI and Neurology Department. My over-arching career goals are to advance knowledge into the pathogenesis of pediatric epilepsy and, through continued basic and translational approaches, to contribute to development of the first generation of disease-modifying treatments. More broadly, I want to serve as an example and help guide other young researchers at Stanford (and beyond) through the somewhat daunting but, I believe, entirely possible effort of moving basic science in directions that will contribute to advances for patients. In order to accomplish these objectives, I will greatly benefit from additional practical knowledge from the Eureka course: what are the steps to transition a novel therapeutic strategy in the lab, to a clinical trial? What resources at Stanford and beyond (including organizations and individual collaborators) can accelerate this process? When is it appropriate to apply for patents and form a company, and how does industry overlap with translational medicine efforts at Stanford?

For many years in training, I’ve focused on efforts to translate findings from the “wet bench” to patients. I am now at an exciting point in the development of my independent basic and translational research program related to pediatric epilepsy, which has identified a novel disease mechanism. The additional knowledge I will gain from the Eureka course will be critical to my efforts to turn this discovery into tangible benefits for pediatric epilepsy patients during my career.
As a cell biologist with expertise in immune-oncology, I am currently an Instructor at the Department of Obstetrics and Gynecology at Stanford University. I have identified a critical role for adipose-tissue-macrophages in ovarian cancer colonization of the omentum. My research interests are focused on defining the mechanism(s) by which the omental immune microenvironment supports and promotes the progression of precursor cells to high-grade serous ovarian cancers, and ultimately peritoneal metastases. I trained at the University of Chicago and then at Stanford University, where I carried out preclinical validation studies and identified signals that could be used as biomarkers for risk and ultimately targets for prevention. Specifically, I aim to define the function of macrophages in both tissue-and gynecologic-oncology-disease-specific settings, giving us a unique opportunity to redefine the current paradigms on the role of the omental microenvironment in ovarian cancer progression.

My short-term research goal is to determine the cellular and molecular mechanism(s) by which macrophages facilitate ovarian cancer cell lodging in the omentum. My long-term research goal is to work with clinician-scientists to find ways to translate these initial findings into the clinic. I also anticipate investigating the potential role of other omental cells and in malignant growth of ovarian cancers.

I intellectually participate in the guided preclinical study currently conducted by my mentor Dr. Oliver Dorigo where we discuss results regarding important issues such as safety, toxicology, bio-distribution, dose-escalation parameters, and delivery methods to guide clinical trials. However, to improve my hands-on skills in translational research, I have to gain relevant training on management, scientific and regulatory aspects of translational medicine that will be necessary for the independent phase of my career. My career goal is to be in a tenure-track position at an academic medical institution with a multidisciplinary gynecologic oncology program and a comprehensive cancer center.
You Leo Li, PhD, MS
Postdoctoral Scholar, Department of Radiology

I am a Postdoctoral Scholar in Dr. Jeremy Dahl’s lab in the Department of Radiology at the School of Medicine at Stanford University. My research focuses on the sensitive detection and measurement of blood flow in small vessels in human body using medical ultrasound devices.

Trained as a biomedical engineer at Duke University, I consider it as my career goal to be a faculty member at a Tier 1 research institution and to provide engineering solutions to clinical challenges and basic bioscience questions. For example, my current research project is on the detection, visualization, and vector flow velocity measurement of the complex small vessel networks in human placenta. It is a clinical study in collaboration with Drs. Virginia Winn and Deirdre Lyell at the Stanford University Medical Center. The project has to major aims. The first aim is for the scientific study of human placenta. By providing a tool to study human placental blood flow, we hope to make new discoveries and deepen our understanding of the function and development of human placenta. The second aim is to provide potential early detection methods for placental abnormalities, including preeclampsia which accounts for at least 76,000 maternal death worldwide per year. In the past few months, I have worked with Drs. Amen Ness and Jane Chueh at the Sequoia Hospital and collected data from 18 healthy pregnant volunteers.

During the clinical study, I realized that in order to achieve my career goals, I need to gain more training and experience in translational medicine. First of all, in order to work with clinicians, I need to learn how to communicate with them, what they care about the most in clinics, and what the culture is like in clinics. In addition, as a biomedical engineer, I realized that I have an urgent need to learn how to identify clinical challenges, and to properly design clinical studies in order to test the engineering solutions to these challenges. These skills are necessary for me to apply my scientific knowledge and engineering skills to address clinical needs. The courses in the MCHRI Eureka Program will provide me with training in these areas. In addition, by working and learning with a group of clinicians, researchers, and mentors at different seniority levels, I can expand my academic network during the program.

Moreover, in order to achieve my long-term goal of becoming a faculty member at a Tier 1 research institution, I need to learn how to be successful as an independent researcher. I’m thrilled to see that the MCHRI Eureka Program provide courses on how to support my research (“Funding Strategies”), how to support myself (“Wellness in a Competitive Environment”), and how other people have done it (“Stanford Success Stories in Translational Medicine”). I believe that these courses will help me immensely in my career.

Upon finishing the program, I plan to apply for funding in May or August this year using my preliminary results in the placental imaging project. I’m sure that the knowledge and skills in translational medicine that I acquire at the Eureka program will help me in this immediate task to write a better funding proposal. After that, I will move on to apply for faculty positions in medical imaging. I expect that the training and network in the Eureka program will help me as a young faculty to connect and communicate with clinicians, to provide engineering solutions to their problems, and to demonstrate the value of my work through successful clinical trials. Ultimately, the Eureka program will help me build a successful career in translational medicine.
As a Pediatric Neurosurgeon, one of the most challenging clinical problems I am faced with is improving the care of children with shunted hydrocephalus. Unlike many conditions that we treat surgically with a durable effect, hydrocephalus is a condition we treat surgically that requires lifelong maintenance, with regular clinic visits and often many surgical procedures over a lifetime, each with attendance risks. For a child with hydrocephalus, his or her life expectancy ultimately depends on the life expectancy and durability of his/her hydrocephalus treatment. As neonatal care for premature neonates advances and survival rates of extremely premature neonates improve, an increasing proportion of hydrocephalus patients we treat are those with post-hemorrhagic hydrocephalus (PHH) resultant from intraventricular hemorrhage (IVH) of prematurity. Unfortunately, neurosurgical treatment options for hydrocephalus have not advanced and most hydrocephalus patients today are treated the same way they were 50 years ago—with a ventriculoperitoneal shunt. Currently there are no medical treatments to prevent the development of PHH once IVH has occurred. My long-term goal is to develop a medical treatment to prevent the development of PHH in at-risk neonates with severe IVH. Several animal models of IVH and PHH have demonstrated the pathologic role of iron in the development of PHH, as well as a protective effect of iron chelation therapy in preventing PHH. Recently we demonstrated in human CSF the correlation of elevated CSF Hb and ferritin following IVH with the subsequent development of PHH. Translating a therapy for iron chelation delivered to the CNS to prevent development of PHH in neonates with IVH is my priority.

The Eureka Certificate Course in Translational Medicine will provide me with training and resources critical to the next steps in my research career. While I have a strong foundation in clinical research training, many of the aspects unique to translational medicine are unfamiliar to me, yet absolutely essential to my success in the next steps in my career path. I expect this course to help me learn some business and regulatory aspects of translational medicine as well as become familiar with resources available at Stanford. I hope to learn from the experiences of other researchers who have been successful in translating basic science research into clinical trials—to learn from their successes and to hear of pitfalls. I expect this course to be an opportunity for networking as well as for learning from others how they have negotiated successful collaborative relationships with research partners across disciplines.

While my immediate goals in attending the Eureka Certificate Course in Translational Medicine are specific to furthering the development of a medical treatment to prevent PHH in neonates with IVH, I know that the skills I will gain from this course will extend far beyond this specific research goal and throughout my career. I expect that the skills I will gain from this course will transform how I approach advancing treatments in all aspects of Pediatric Neurosurgery and shape my role as a leader in translating basic science discoveries into clinical practice—incorporating medical and surgical treatments for Pediatric Neurosurgical patients.
More than a decade ago, I committed to the training required to become a physician-scientist. However, it wasn’t until 2018—my second year of Pediatric Rheumatology fellowship—that I actually began functioning as both a physician and scientist at the same time. While it has certainly been a challenge to balance simultaneous clinical and research responsibilities, the opportunities within translational medicine have become even further inspiring compared to the more isolated perspectives I’d developed during my clinical-or science-focused training periods. Over this past year, I have applied my basic science training to a translational research project that aims to identify plasma-based biomarkers associated with disease activity and therapy response. Though I’ve always understood the value of rigorous science, more recently I’ve been compelled to consider its importance not just in terms of expanding biomedical knowledge, but also in the context of improving patient care. I increasingly realize how much my clinical training and practice influence my approach to research design and execution, especially with regard to how such studies could impact our understanding of human disease in ways that translate into healthcare advances.

As I work toward the development of a research program that will launch my career as an independent investigator, I am more clearly recognizing the gaps in my knowledge and experience, specifically those related to translational medicine. My training has prepared me well to care for patients in the clinical setting while studying their diseases from a molecular perspective in the lab, but I have limited knowledge on how to translate a scientific concept to a clinical asset. I understand that there are many different building blocks comprising this process, such as clinical trial design and engaging regulatory agencies, but know little detail about each of these many steps. I am also interested in learning more about factors that define a successful translational study and the subsequent actions needed to promote, and sustain, changes in clinical practice. Thus, I am thrilled to have the opportunity to gain a deeper understanding into the entire translational medicine pathway—particularly the steps beyond the discovery phase—through participation in the MCHRI Eureka 2020 Certificate Course in Translational Medicine.

I look forward to learning more about the various processes that pave the pathway of translational medicine. The 5-day intensive course could significantly help shape the focus of my research and funding strategies as a developing physician-scientist who aspires to continue clinical practice and translational research throughout my career. I am currently preparing to apply for the Bridge to K Program/Instructor Support award through the Department of Pediatrics, which will allow me to continue my research at 75% time with 25% clinical practice, and intend to apply for NIH-based support and other career-development awards in the near future.

I am also excited to meet investigators with an interdisciplinary range of translational medicine experience; this will allow me to build my network and find expert mentors with candid insight into the challenges they have faced, as well as the feats that have inspired them to continue their own journeys, along this path. I am especially interested in learning more about the strategies they implement for research design and data analysis to optimize the generation of clinically meaningful findings. It will also be helpful to learn about real-world applications of translational medicine that have ultimately advanced patient care, as these examples serve to inform and further develop the critical-thinking and problem-solving skills I will need to reach my goals.
I recently completed a KL2 Mentored Career Development award through Stanford Spectrum program. During this two-year fellowship, I became a member of the Association for Clinical and Translational Research (ACTS), where I was exposed to the challenges of translational research. I conduct research using computational language analysis of psychotherapy, which straddles clinical psychology, clinical informatics, and computer science. The Eureka program looks especially exciting because of the program’s focus on designing innovative clinical trials and real-world applications of translational medicine.

My work provides a synthesis of the current opportunities and risks for conversational AI in mental health delivery. Prior work has neglected the impact of new technology on the specific roles and duties of clinicians, and my work describes approaches to AI integration in the near future. I am especially excited to expand my professional network of translational researchers, and I look forward to talking with fellow attendees between sessions to both learn and share my experience.
I believe that participating in this course will support my mission to use evidence-based medicine and rigorous scientific methods to promote excellent health and health equity of sexual and gender minority (SGM) people, a historically underserved and understudied community. As an obstetrician and gynecologist with training in public health and clinical research and an interest in sexual and gender minorities, I am particularly focused on understanding and then redefining conceptions of family, family building, and reproductive choice. In working with SGM people, I am compelled that many of our families are not known, visible, or intelligible to much of the research and clinical communities. In this way, there are significant research and clinical gaps that keep people from seeking care and or getting the health care support they need. Frankly, what is seen out in the world is not reflected in our science and medicine. Therefore, I aim to push the boundaries of current “maternal and child health” paradigm to understand and support families that have multiple mothers, no mothers at all, transgender men who are carrying pregnancies, and multiple other family structures to make sure our research and clinical systems reflect the comprehensive and staggeringly beautiful diversity of people’s lives. This type of redefinition naturally crosses boundaries within science, medicine, and even cultural understanding of who people are and what they need. Therefore attending a course that speaks to translation and helps build a network of support for those looking to bridge not only bench to bedside, but bench to bedside to community, is what is compelling me about this course.

As a clinician investigator in the MCL Line at Stanford I am already collaborating with colleagues across the School of Medicine to create a comprehensive Sexual and Gender Minority Health Program to advance health and health care of LGBTQ+ people through clinical care, research, and education. As one of the co-leads on the research arm of this burgeoning SGM Health Program at Stanford, I am hoping to support Stanford in defining and propelling a new investigational domain and create the next generation of SGM health researchers. Currently, my central project is The PRIDE (Population Research in Identities and Disparities for Education) Study (www.pridestudy.org), an online national longitudinal cohort of SGM people, which I co-direct with my Stanford colleague Dr. Mitchell Lunn (Medicine: Nephrology). The PRIDE Study has now recruited over 16,300 people and has 25 sub-studies in our Ancillary Study pipeline across clinical content areas. However, up until now our research has been survey based. To propel The PRIDE Study and the larger SGM Health Program at Stanford forward, I need to understand how to initiate investigational and translational projects to a) utilize our research-ready cohort, b) efficiently partner with researchers, clinicians, and non-traditional agents (industry, community partners), and c) apply our “dry lab” findings clinically.

As a new junior faculty in Stanford, I am eager to learn how to build meaningful and effective teams within and beyond Stanford. I am also, excited to join a community of other investigators at Stanford to maximally use our translational and clinical resources and capitalize on the opportunities for cross-department and cross-school collaborations. I have a strong history of innovation, novel collaborations in the sciences, and I’m hoping that attending this course can help ensure that my research is sustainably deep, impactful, and contemporary.
My career goal is to become a principal investigator, whose ideas and research help to bridge scientific knowledge and its medical application in the fields of child development, neuroscience, language, and technology. I envision a future where I will independently, but collaboratively, conduct interdisciplinary research on the behavioral and neurobiological bases of learning and its differences, stress effects and regulation, educational outcomes and technology, and psychosocial development, parallel to mentoring trainees pursuing careers in basic as well as clinical research.

To achieve my goal, I am currently training as a postdoctoral research fellow at Stanford School of Medicine’s Developmental-Behavioral Pediatrics research division, which is an ideal research environment, given its highly interdisciplinary and cutting-edge scientific community. I am co-advised by Drs. Heidi M. Feldman, Katherine E. Travis, and Ian Gotlib in a cross-disciplinary approach. My research interest is focused on typical and atypical early human development, relevant to child health, learning abilities and differences, and education. I am interested in developing a line of research that will further inform how we can improve child health and support their early experiences to promote positive long-term developmental outcomes.

Previous to my postdoctoral training, I tested questions relevant to typical development. However, in order to grasp a deeper understanding of child brain-behavior relationships, I wanted to also study child health outcomes in the context of atypical development. Therefore, together with my research team, I am currently implementing a randomized clinical trial (RCT) to primarily examine the long-term effects of a Neonatal Intensive Care Unit (NICU) language intervention on language outcomes in preterm infants. In this clinical research, I will be testing whether exposure to mother’s voice ameliorates the neurocognitive disadvantages associated with premature birth, reflected in better language comprehension abilities later in life (18-month-old). The NICU language intervention consists of mother’s voice recording as she reads children’s stories played via speakers placed in the crib and/or incubator at prescribed intervals and durations for a minimum of 2 weeks and a maximum of 9 weeks. Additionally, as a secondary outcome, I will test whether this intervention also improves the brain’s structural connectivity, specifically white matter microstructure, which is known to be altered in children that were born before term. Developing easy-access interventions provided at very early postnatal stages represents a promising clinical “tool”/strategy, as well as a pressing public health need. Therefore, we require more and well-designed neuroimaging studies complemented with behavioral and environmental data to better understand whether early clinical interventions for language exposure improve the developmental trajectory of neural organization and thus neurocognitive outcomes in preterm infants.

In parallel with this RCT, I am currently looking at the effects of Kangaroo Care (KC), or skin-to-skin contact, on white matter organization and neurocognitive outcomes in preterm infants. At the Lucile Packard Children’s Hospital Stanford, a developmental care program was implemented over a year ago; as part of this program, KC between mother/father and infant is strongly encouraged. Although there is a plethora of studies testing whether KC improves clinical outcomes (e.g., increases in breastfeeding, and decreases in newborn sepsis, hypothermia, hypoglycemia, and hospital readmission) in preterm infants, little is known about its role on brain development. Therefore, given that white matter is significantly impacted as a function of premature birth, I am investigating whether there is a relationship between KC, while at the NICU, and water molecules diffusivity and myelination. This project is an example of the bidirectional mission of Translational Medicine (TM), where we are bringing “the bench” to the clinic by producing new knowledge that may benefit medical practice; at the same time, however, we are better designing our research based on the actual needs and practices in the clinic.
By attending the MCHRI Eureka Translational Medicine course, I aspire to become more adept in the analysis of the economic market within medical industry and network with those members of the community who have traversed this field, as strong mentorship is a key component to success. Partaking in the sessions on negotiation, designing an innovative trial, and real-world application sections of this course will allow me to further my education and gain expertise in this research field that I can then apply to clinical neonatology.

The foundation I have in Translational Medicine and the skills I will gain from this course will allow me to continue my academic career in this field. As a graduating clinical neonatology fellow, the critical thinking approach, interdisciplinary collaborations, and knowledge of the business and regulatory aspects of industry in clinical medicine will allow me to pursue this career path as an attending faculty member.
I have been working on the clinical translation of gene engineered regulatory T cells since starting at Stanford School of Medicine in 2016. My current project is focused on preclinical studies of autologous CD4FOXP3 cells, produced by FOXP3 gene transfer into CD4+ T cells. These cells are being developed as a therapy for the rare genetic immune disease, IPEX syndrome, that is caused by genetic mutation of FOXP3. As a postdoctoral fellow, my research focus has always been on the developmental of translational research for the rare genetic immune diseases. But as I progress into a senior scientific role I would like to expand my knowledge to include both the scientific and regulatory aspects of translational research.

As a pediatrician-scientist, I have a broad range of interests all pertaining to translational medicine. These include immune genetic diseases as well as other disorders including hematological malignancy, neural disease and enzyme deficiency. In all these areas, I am very interested in the clinical trial design and management to obtain the transition from basic science to the actual translation into the clinic. At Stanford, I have learned a lot with regards to translational medicine and regulatory science, but it is impossible to cover every field and topic only by single institution. Thus, I still aspire to learn many aspects of translational medicine from basic science to actual clinical translation.

I am originally from Japan and, after my tenure here at Stanford, I now realize that translational medicine in Japan is somewhat delayed comparatively. I believe this is due to several reasons such as conservative nationality but also a lack of human and financial resources. And while Stanford has provided me with an ideal and exceptional opportunity for learning translational medicine, I believe that attending this course will be an ideal opportunity to expand my knowledge. My ultimate goal is to develop the expertise and international collaborations required for me to return to Japan to lead the development of translational research in my home country.
I am a pediatric critical care physician and health services researcher interested in conducting methodologically-innovative and clinically-significant research characterizing the physiological and operational features associated with high-performing health care systems. My goal is to become an academic leader in the improvement of the health care delivery system, working at the intersection of basic science, translational research, informatics, and data science to achieve this goal.

My long-term goal is to become a leader in evidence-based health care delivery reform. By conducting methodologically-sound translational and health services research and interpreting the findings into health policy recommendations, I intend to substantially improve performance and value of pediatric health care delivery. My one-year goal is to obtain funding for a research agenda that will improve objective characterization of physician burnout, using a combination of neuroendocrine biomarkers, bioinformatics, and predictive data analytics. My three-year goal is to complete and publish significant research that will allow objective identification of work settings that place physicians at disproportionately high risk of professional burnout, and allow quantification of the neuroendocrine response to burnout interventions. My five-year goal is to become an independently funded expert investigator focused on improving the prediction and prevention of physician burnout. I specifically plan to seek research funding (NIH R01) for objective identification of risk of burnout across physician disciplines. I also plan to expand this line of research to other health care provider populations, including nurses, respiratory therapists, and physical therapists. My 10-year goal is to be a national leader in evidence-based health care delivery reform, and to complete and publish research that will reduce health care provider burnout and improve the quality of care provided to neonates and children.

In order to achieve the above goals, I require further formal training in translational science. With support from the MCHRI, I have completed a Masters of Science degree in Health Research and Policy and have completed foundational research in predictive data analytics. I have also published research characterizing risk factors for health care provider burnout, and quantifying the relations between burnout and impaired quality of care. In order to incorporate the use of biomarkers in burnout research, I require further training in navigating the intersection between basic science, translational research, and health services research. The Eureka Certificate Course in Translational Medicine will serve as a valuable tool in my career development.
I joined Stanford University 3 years ago as an Assistant Professor and Clinician Scientist. I am dual fellowship trained in both pediatric and adult Interventional Radiology, and hold a joint appointment as an Attending Interventional Radiologist at Stanford University Medical Center and Lucile Packard Children’s Hospital. During my clinical training, I completed 2 doctoral research degrees in fetal cardiovascular physiology and molecular imaging. Currently, I run my own translational research laboratory, which focuses on developing novel therapeutic treatments for pancreatic and kidney regeneration as well as optimizing islet transplantation with mesenchymal stem cells (MSCs).

My current interests are centered on the development and translation of novel therapies which can be used for organ regeneration. Over the past few decades, advances in image guided therapies have enabled Interventional Radiologists to reliably deliver therapies, directly to target tissues, using minimally invasive procedures. As such, a new clinical sub-specialty is starting to emerge called “Interventional Regenerative Medicine (IRM)” in which Interventional Radiologists can deliver stem cells and gene therapy directly to damaged organs. The impetus underpinning IRM parallels that previously observed in Interventional Oncology (IO); traditionally, chemotherapy for several solid cancers was given by IV injection, however, this often resulted in systemic distribution of the drug associated with significant toxicity/side-effects (due to off-target delivery) as well as poor therapeutic effects (from limited concentration of the active drug reaching target tumors as well as first pass metabolism of the drug). To address this, image-guided minimally-invasive procedures were developed by Interventional Radiologists to deliver chemotherapeutic drugs directly to the site of tumors via their arterial blood supply (i.e., transarterial chemomobilization (TACE)) which resulted in significantly improved clinical outcomes and a reduced need for surgical resections and even solid organ transplantation. IRM is therefore perfectly poised to build on this platform given the vast amount of knowledge (i.e. the blood supply of different organs in the body), skills (i.e. the technical ability to access the different vessels that supply these organs or even target these organs directly using delivery needles) and technology (i.e. new microcatheters, needles and imaging equipment with complementary navigation software) developed by Interventional Radiologists over the past few decades.

Recently, Stanford University approved the development of an Islet Transplantation program which I have been building, with the help of my colleagues, since my arrival at this institution. As such, my hope is to one day translate several of the approaches and therapies which we are developing in the lab into select diabetic patients who could benefit from this procedure. As an Interventional Radiologist, I am trained to perform Islet Transplantation and will be the primary operator here at Stanford for this procedure for both adult and pediatric patients.

However, my knowledge of translating therapies from benchtop-to-beside is limited and hence the Eureka program provides a unique opportunity for me to bridge this gap. The ability to learn from, and interact with, academic and industry experts will be invaluable in addition to understanding the potential regulatory hurdles that I will encounter and solutions to overcome these. I am also excited to be able to identify mentors who will help me build translational programs and teams here at Stanford, such that we can be the forefront of translational medicine and provide the best possible treatment for our patients.
I am a hematologist, physician-scientist and Assistant Professor (UTL) in the Department of Pediatrics, Division of Hematopoietic Stem Cell Transplantation and Regenerative Medicine. My clinical scope of practice includes attending on the pediatric hematopoietic stem cell transplantation inpatient service (20%), seeing patients with genetic forms immune dysregulation in my specialty clinic and doing immunology consultations for inpatients with 22q11 Deletion Syndrome (5%). The majority of my time (75%) is devoted to running my laboratory which is entirely devoted to basic and translational research. My group has two distinct research interests which fall within the realm of targeted and precision immune and cell therapy.

(1) My laboratory is in the process of making regenerative thymic epithelial cells from induced pluripotent stem cells. Our ultimate goal is to make a therapeutic thymic epithelial cell organoid for direct transplantation into immunodeficient patients. The thymus is a central immune organ, critical to T-cell differentiation and selection of the T-cell receptor repertoire. Patients with inborn or acquired forms of thymic injury (through 22q11 Deletion Syndrome or stem cell transplantation, graft-versus-host-disease, medications, infection, aging, respectively) suffer from potentially life-threatening and incurable immunodeficiency and autoimmunity. Regenerative thymic epithelial tissues are the only known therapeutic strategy. In addition, appropriately matched regenerative thymic epithelial cells have the potential to induce tolerance after solid organ transplantation. In order to succeed with this equally impactful and challenging mission of making regenerative thymic tissues, I have assembled a group world-renowned (Stanford-based and international) researchers with expertise in stem cell biology, synthetic biology, biomaterials engineering and recombinant cytokine production. Together we are devoted to bringing a therapeutic cell candidate to the clinic in the next 5 years. In order to lead my team to success, I require the knowledge and practical skills to plan and execute the translation of an in-vitro engineered stem cell product into a clinical trial for patients. During the Eureka course, I expect to acquire knowledge on GMP-manufacturing and scaling of a cell therapeutic, learn about regulatory requirements, intellectual property, and partnering with industry sponsors and other funding sources. I also hope to attain practical leadership and presentation/communication skills.

(2) The second focus of my research group is on understanding the metabolic reprogramming process that occurs during human hematopoiesis. While stem cells rely mostly on glycolysis to meet their metabolic needs, differentiated cells switch their metabolism to oxidative phosphorylation (OXPHOS). My laboratory has shown that defects in OXPHOS lead to a differentiation arrest using the disease Reticular Dysgenesis as a model. Other laboratories have shown that the “extra-physiologic oxygen shock” hematopoietic stem cells undergo during hematopoietic stem cell transplantation, reduces their viability and engraftment potential. We therefore reason, that small molecules and procedures that keep hematopoietic stem cells glycolytic and prevent the switch to OXPHOS, will improve survival and engraftment of stem cell products. In order to translate this knowledge into a clinical trial, I hope to gain knowledge about the regulatory requirements of introducing novel drug compounds into the clinic, the design, planning, powering of a clinical trial, and again, as detailed above, gain deeper insights into regulatory requirements, intellectual property, partnering with industry sponsors and other funding sources.

My ultimate career goal is to be an academic physician scientist who is bringing scientific discoveries back to the clinic.
During my fifteen years as a research technician in a basic science laboratory at the Mayo Clinic, I saw first-hand the value of a strong emphasis on translational medicine. I saw how the proverbial “bench to bedside” transition only occurs with a group of individuals committed to translational medicine. Basic scientists, physicians and a team of other skills willing to bridge the gaps between them and focus translating basic science discoveries into clinical practice as efficiently, safely and quickly as possible.

My personal research path began in the pursuit of an understanding of marine biology. This unwavering desire began in early childhood and persisted through my first years of undergraduate studies in Australia. But while my desire to understand was met, it always lacked the sense of human relevance. It lacked translatability. This all changed after some introductory courses in genetics and biochemistry and my newfound interest fostered close personal friendships and mentorships with a number of faculty in the biochemistry department. Since then, my passion to seek understanding of protein biology for medical benefits, has never waned.

I concluded my undergraduate degree with majors in biochemistry and molecular biology. Upon graduating, I took a temporary position in a basic science research lab at Mayo Clinic that was supposed to last one year. However my curiosity was piqued and it took 15 years before I could leave that project behind. Shortly after arriving, I began hypothesizing and designing experiments to address questions within existing projects. This evolved into broader questions leading to new R01 grants for the lab and first authorship manuscripts for myself. One project lead to multiple manuscripts, a phase II multicenter clinical trial and a number of patents. This is when I learned first-hand the value of a strong community committed to translational research.

So, after years of coercion, I finally pursued my PhD in a collaboration between my principle investigator at Mayo Clinic, and the team of researchers at my undergraduate alma mater in Australia. On May 31st, 2016 my PhD was conferred, and June 1st I began my postdoctoral training with a fellowship funded by a nonmalignant hematology T32 grant awarded to Dr. Sakamoto here at Stanford. I feel privileged that I was awarded an MCHRI fellowship in 2017, and was recently appointed to Instructor. It is no coincidence I transitioned from basic science laboratories before and during my PhD, to a translational lab for my postdoctoral training.

I have experienced the strong commitment to translational research with programs such as MCHRI, SPARK and BioX demonstrating the exceptional commitment and understanding Stanford has for the value of a thriving, talented, engaged and collaborative translational research community. As I move forward and develop my own research program, I look forward to developing a niche within a much wider network. Once focused on applying my insights into clinical relevance. Science in isolation is challenging, and with literature expanding too quickly to ensure my findings are getting to the eyes that can apply it, my goal is to position my program as a node within a network focused on rapid (but careful) delivery of new concepts into clinical relevance.
My long-term career goal is to become an independent investigator in the field of headache medicine by utilizing my recent biomolecular observations to determine outcome predictors and to personalize treatment modalities. I am currently working on data-driven approaches to determine links between clinical endophenotypes and metabolomic/proteomic analysis in chronic migraine patients using cross-sectional study. My preliminary results revealed candidate biomarkers based on which I am planning to conduct a longitudinal study to identify cause-and-effect relationships between biomarkers and clinical outcomes. While my scientific trajectory seems to be progressing, I look forward to learning skills needed to capitalize on my research progress and turn it to entrepreneurial venture. By attending this course, I will gain useful tools in making my research findings available for patient care and community health. This course will logically build on my current research and help me in my K award application.

I expect that this course will hone my critical thinking and impart necessary soft skills to tap available resources and facilitate creation of interdisciplinary collaborations. By participating in this course, I aspire to play a leadership role in translational medicine within the Stanford Headache Division by mentoring and advising fellow researchers on how to best navigate their research towards translation. I am also keen to learn the business and regulatory aspects of translational medicine with a focus on intellectual property. I anticipate mutual benefit with the Stanford Headache Division by applying translational research for precision medicine in migraine stratification. In addition, I expect to learn how to best utilize available resources at the Stanford Center for Clinical and Translational Research and Education (Spectrum) funded by the National Center for Advancing Translational Science funded.
Stem cell biology has the potential to influence every field of medicine. In ophthalmology, the ability to create ocular tissue from induced pluripotent stem cells (iPSC) would transform the way we treat eye disease. For the past 8 years, I have focused my research on how to differentiate iPSC into transplantable corneal epithelial stem cells, which could help cure the 23 million patients worldwide afflicted with corneal blindness. With National Eye Institute K08 support, my laboratory has created human iPSC lines and differentiated them into corneal epithelial stem cells. We are currently optimizing our differentiation protocols and have begun in vivo transplantation experiments in a rabbit eye injury model. The timing of this course is ideal because we are still early in the process, having not yet designated a finished product or protocol to submit to the FDA.

As a surgeon-scientist, I have become well-versed in the clinical and surgical management of eye diseases, as well as strategies to investigate their underlying pathogeneses in the laboratory. What is conspicuously missing in my toolbox is the knowledge of how to practically translate my benchtop discoveries, literally to the bedside or operating room of my patients. The desire and anticipation of taking this work to clinical trial has always been there, and spurred visits to multiple investigators worldwide to learn about their experiences overcoming regulatory hurdles to bring therapies to patients. I toured eye centers working on expanded primary corneal stem cells in Antwerp, Belgium, and London, England, as well as with iPSC in Taipei, Taiwan. This year I visited NIH in Bethesda, Maryland, to learn about the first U.S. iPSC clinical trial for macular degeneration. From all these visits, I have learned that early planning and preparation are critical, and that the pathway from pre-clinical data, through clinical trials, to widespread use, is long and arduous. It is my hope that the MCHRI Eureka Course will provide the resources and training to navigate the complexities of a career in translational medicine (TM).

This course will profoundly influence my career development, particularly as a newcomer to TM. With an iPSC corneal clinical trial just underway in Japan and another one slated to begin in Europe, it is paramount that I soon learn about the business, scientific and regulatory aspects of TM, as well as how to build successful teams and interdisciplinary collaborations. Armed with knowledge of TM, I can better anticipate potential pitfalls and challenges during the process. While my laboratory’s goal is to produce transplantable grade corneal stem cells to cure corneal blindness, this is just the beginning. We plan to use a similar pipeline to create other ocular stem cells to treat a variety of eye diseases. I am committed to a career in TM and the skills from this course will support and facilitate all my TM activities for years to come.

At Stanford and in this country, there is currently no group to my knowledge openly planning an iPSC clinical trial to treat corneal blindness. Publishing the first pre-clinical data, overcoming the regulatory hurdles and then completing a clinical trial would help establish my laboratory at Stanford as a leader in this field. I hope to obtain the experience, expertise and ability to help translate iPSC-based ocular discoveries into therapies at Stanford and worldwide.

While attending this course, I have specific goals in mind to help bring iPSC-derived corneal stem cell to clinical trial. In particular, I hope to better understand the FDA review process for iPSC-based therapies, ensure our protocols fit GMP and GLP, and learn the particular requirements needed for our animal and preclinical studies. Additionally, I wish to learn how to initiate clinical trials, troubleshoot potential problems, and successfully analyze the complexity of data. Finally, I hope to learn how to partner with venture capital and industry to bring stem cell therapies to hospitals and clinics. I truly look forward to the opportunity to interact directly with those experts who can share their real-life experiences of how TM pioneers can achieve their goals.

Stem cell research has the potential to revolutionize modern ophthalmology. This course will ultimately help position me to bring the promise of regenerative medicine to patients with sight-threatening illnesses.