On Monday, November 21, 2016 Massachusetts General Hospital, in collaboration with the Translational Neuroscience Center (TNC) and Massachusetts Institute of Technology, will host the second Boston-wide Neurodevelopmental Disorders Symposium in the Joseph B. Martin Conference Center at Harvard Medical School.

This symposium, entitled New Directions in Neurodevelopmental Disorders, will focus on new frontiers in the genetics of neurodevelopmental disorders, developmental considerations and early detection, new approaches to the neurobiology of neurodevelopmental disorders, and current clinical controversies.

Sponsored by the Anne and Paul Marcus Family foundation, the symposium is open to scientists and physicians from academia, the pharmaceutical and biotechnology industries, disease-based foundations, and the government. The day-long conference will include guest speakers and a poster session for graduate students and post-doctoral fellows.

Confirmed Speakers Include:
- Michael Talkowski, PhD - Massachusetts General Hospital
- Helen Tager-Flusberg, PhD - Boston University
- Sarah Spence, MD, PhD - Boston Children's Hospital
- Mark Daly, PhD - Massachusetts General Hospital and Broad Institute
- Christopher Walsh, MD, PhD - Boston Children's Hospital
- Takao Hensch, PhD - Boston Children's Hospital
- Charles Nelson, PhD - Boston Children's Hospital
- Beth Stevens, PhD - Boston Children's Hospital
- Guoping Feng, PhD - Massachusetts Institute of Technology
- Caroline Robertson, PhD - Massachusetts Institute of Technology
- Jean Frazier, MD - University of Massachusetts Medical School
- Ann Neumeyer, MD - Massachusetts General Hospital
- Sourav Choudry, PhD – Massachusetts Institute of Technology

To Register for the symposium, please follow the link below: https://www.eventbrite.com/e/2016-neurodevelopmental-disorders-symposium-tickets-25703962243

We also invite you to submit poster abstracts on clinical and translational neuroscience research and neurodevelopment disorder resources for this
Symposium to be held at 12:30pm on Monday, November 21, 2016. Please complete a poster abstract submission form and submit to tnc@childrens.harvard.edu by Friday, September 30, 2016. Please also visit the event webpage to RSVP.

### Human Neuron Core Expansion

The TNC’s Human Neuron Core has been expanding staff, services and equipment, thanks to an award from the Massachusetts Life Sciences Center Capital Program. We were fortunate to recruit 2 expert supervisors to oversee new services. Ivy Pin-Fang Chen joined the Human Neuron Core as the laboratory supervisor for Human Neuron Differentiation Service. She received a B.A. degree in Molecular Biology and Biochemistry from Wesleyan University, and a Ph.D. degree in Biomedical Science from University of Connecticut Health Center. During her Ph.D. training, Dr. Chen studied genomic imprinting and modeled rare neurodevelopmental disorders using patient-specific iPSCs and iPSC-derived neurons. Ivy’s career goal is to help develop treatments for patients with neurological disorders.

Liz Buttermore also joined the Human Neuron Core in July to lead the Human Neuron Phenotyping Service. Dr. Buttermore received her B.S. in Biochemistry and Molecular Biology from the University of Richmond, VA, and her PhD in Neurobiology from the University of North Carolina, where she studied the organization and maintenance of molecular domains in myelinated axons. Dr. Buttermore recently completed her postdoctoral training in the lab of Clifford Woolf at BCH, where she developed protocols for the differentiation of sensory neurons from human fibroblasts and iPSCs, and used these neurons to develop phenotypic screening platforms to identify novel therapeutic targets for chemotherapy-induced peripheral neuropathy. Dr. Buttermore is excited to be a part of the HNC team, working at the interface of the clinic, academia, and industry to advance therapeutic options for patients with rare and untreated neurological disorders.

John Micozzi is a Research Assistant for the Human Neuron Differentiation Service. He received his B.S. in Biomedical Science with a concentration in Medical Microbiology from the University of New Hampshire. He is currently enrolled at Northeastern University, where he is in the process of receiving his Master’s degree in Biotechnology with a focus in Bioprocessing. Syed Mohammad Adil Wafa is also a Research Assistant in the Human Neuron Differentiation Service. He received a B.A. (Hons.) degree in Natural Sciences Tripos from University of Cambridge, and a M.Sc. degree in Developmental Neuroscience and Psychopathology from University College London and Yale University. The new team is very excited about working with local investigators on their iPSC derived neuron projects.

**New equipment** in the core includes an Axion Maestro, which has the capability to measure multi-electrode array plates in 12well, 48well, or 96well formats. The Axion Maestro is available...
for sign-up via iLabs after users have been trained. Alternatively, core personnel can conduct recordings for users for an assisted use fee. For training on the Axion Maestro, please contact elizabeth.buttermore@childrens.harvard.edu.

For questions about ilabs access, cost, or neuron provision, please contact pin-fang.chen@childrens.harvard.edu.

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**Epilepsy Reciprocal Translation Unit (RTU) meeting held at BCH**

More than 25 scientists representing academic, industrial and foundation research communities from across the Boston area gathered at Boston Children’s Hospital on July 15th to discuss roadblocks in research into new treatments for epilepsy. The discussion-oriented meeting featured investigators from Boston Children’s Hospital, Mass General Hospital, Brigham and Women’s Hospital, Brandeis University, Tufts University, Harvard Stem Cell Institute, Broad Institute, MIT, Amgen, Clarus Ventures, Novartis, Pfizer, Sunovion and the Epilepsy Foundation.

The TNC organized event provided a forum for a Reciprocal Translation Unit (RTU) meeting of experts to address key translational challenges for epilepsy drug development. The event featured a continuum of presentations that began with Dr. Ann Poduri, who addressed “Monogenic epilepsies—what determines if they are ready for translation?” and was followed by Dr. Paola Arlotta, who highlighted “Emerging models of epilepsy: cerebral organoids.” Next, an overview from Dr. Kevin Staley on “Key features of preclinical models to support drug trials,” and a wrap-up discussion led by Dr. Page Pennell on “Clinical Trials in Epilepsy: maximizing speed and minimizing trial size” provided a solid platform for ranging discussion on how to enable more clinical trials for epilepsy. Feedback from the session was overwhelmingly positive and identified a set of follow up topics and actions to address some of the bottlenecks identified.

“The RTU team put together an incredibly well-organized and productive meeting. All the key stakeholder groups in the epilepsy therapy development process were part of the conversation, including those who don’t normally interact, like basic scientists, family caregivers and venture capitalists. What we learned was that each had valuable contributions to make when discussing how to move more effectively from discovery to therapy development.” – Dr. Brandy Fureman, Vice President of Research and New Therapies, Epilepsy Foundation

Based on responses from the feedback provided, TNC will be organizing subsequent meetings to dive deeper into specific questions and the translational path for bringing new medication to the clinic. Among the follow up topics to be included are: 1) Individual sessions on monogenic epilepsies (e.g, SCN1A, PCDH19); 2) Acquired epilepsy and 3) Biomarkers and outcome measures for epilepsy clinical trials. The next 2 upcoming sessions will focus on 1) SCN1A and family members and 2) injury-induced epilepsy. These sessions are being scheduled for December and January. We are currently coordinating dates and speakers for these follow up meetings. If you have suggestions for speakers or participants for these topics please send them to tnc@childrens.harvard.edu.

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TNC Newsletter
Kleefstra Syndrome Day event sponsored by TNC

The GeneSpark Foundation organized the first International Conference on Kleefstra Syndrome in Boston on June 25, 2016 with support from the TNC at Boston Children’s Hospital. Kleefstra Syndrome is a rare neurodevelopmental condition caused by heterozygous loss of function mutations or deletions in the *EHMT1* gene, which encodes a protein required for epigenetic suppression of gene expression programs. The conference brought together researchers, patients and families from around the world to learn about the latest science surrounding Kleefstra Syndrome and GeneSpark’s plans to support research into identifying potential drug treatments and learn from other patient families about their experiences. Scientific presentations included:

- Dr. Tjitske Kleefstra, Radboud UMC Nijmegen, Netherlands: *Kleefstra Syndrome Update*
- Dr. Harvey Lodish, MIT: *Drug Development Overview*
- Dr. Hans van Bokhoven, Radboud UMC Nijmegen, Netherlands: *Radboud’s Path Forward on Kleefstra Syndrome Drug Development*
- Dr. Hans Bjornsson, Johns Hopkins University: *Learnings From Kabuki Syndrome and Relevance To Kleefstra Syndrome*
- Dr. Robin Kleiman, Boston Children’s Hospital: *How iPS Cells Can Speed A Potential Therapeutic Treatment For Kleefstra Syndrome*
- Dr. Carl Ernst, McGill University, Canada: *Increasing Gene Product In Haploinsufficiency Syndromes As A Pharmacological Strategy*

Save the Date! TNC Symposium on April 6, 2017

On April 6, 2017 the TNC will host its symposium at the Joseph B. Martin Conference Center at Harvard Medical School. The Symposium is open to scientists and physicians from academia, the pharmaceutical and biotechnology industries, disease-based foundations, and government. Confirmed Speakers Include:

- **Joseph Buxbaum, PhD**
  G. Harold and Leila Y. Mathers Professor
  Vice Chair for Research, Department of Psychiatry
  Director, Seaver Autism Center for Research and Treatment
  Icahn School of Medicine at Mount Sinai
• **Adam Gazzaley, MD, PhD**  
  Professor of Neurology, Physiology and Psychiatry  
  Kavli Institute for Fundamental Neuroscience  
  Director, Neuroscience Imaging Center and Neuroscape Lab  
  University of California San Francisco

• **Sally Temple, PhD**  
  Scientific Director, Principle Investigator and Co-Founder  
  Neural Stem Cell Institute, Rensselaer, NY

• **Steve Goldman, MD, PhD**  
  URMC Distinguished Professor of Neurology and Neuroscience  
  Co-Director, Center for Translational Neuromedicine  
  University of Rochester Medical Center

• **Beth Stevens, PhD**  
  Assistant Professor of Neurology,  
  FM Kirby Neurobiology Program  
  Boston Children’s Hospital  
  Harvard Medical School

Stay tuned to our website [www.childrenshospital.org/tnc](http://www.childrenshospital.org/tnc) for more details!

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**TNC Seminar Series**

The TNC Seminar Series takes place from 12 pm to 1 pm in the 12th floor conference room of the Center for Life Science (CLS) building at 3 Blackfan Circle. The seminars are accessible to all faculty and staff at Boston Children’s Hospital and other Harvard-affiliated institutions who have access privileges to the CLS building. All others who wish to attend may contact the TNC office tnc@childrens.harvard.edu, so that we can make arrangements with CLS security for you to enter the building.

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**TNC Seminars 2016-2017**

- September 13, 2016: Florian S. Eichler, MD – Massachusetts General Hospital
- October 11, 2016: Nicole J. Ullrich, MD, PhD – Boston Children’s Hospital
- November 8, 2016: Sandeep Robert Datta, MD, PhD – Harvard Medical School
- December 13, 2016: Dost Öngür, MD, PhD – McLean Hospital
- January 10, 2017: Suzanne Paradis, PhD – Brandeis University
- February 14, 2017: Susan Faja, PhD – Boston Children’s Hospital
- March 14, 2017: Nadine Gaab, PhD – Boston Children’s Hospital
NEW TNC Request for pilot project proposals!

The TNC is Requesting Applications that support the development of new preclinical models for Autism Spectrum Disorders (ASD), Arteriovenous Malformations (AVM) and Sturge-Weber Syndrome (SWS). High priority will be given to development of new iPSC lines that model autism spectrum disorders or the development of new animal model of ASD, AVM or SWS. See the TNC website for details. Applications due December 23rd, 2016.

Expansion of the Clinical Research and Regulatory Affairs Service

The TNC Clinical Research and Regulatory Affairs Service has expanded. Two new staff members were hired this year allowing for the extension of services. Zaira Miles-Diaz joined us in February as an Administrative Associate. Zaira ensures administrative tasks within the Service run smoothly and efficiently. She is excited to be a member of the TNC team. Evi Abada, MS joined us in March as a Clinical Research Specialist. She received her Master of Science degree in International Health Policy and Management from Brandeis University, Waltham Massachusetts. Evi has a background and wealth of experience in running clinical trials and brings that experience to the TNC. She is currently involved with several clinical trials at BCH, providing regulatory and project management assistance. Evi looks forward to supporting the successful implementation of clinical studies through the TNC. The entire TNC is excited to add Evi and Zaira to the team to help further the TNC’s provision of services.

First Neuroscience Clinical Research Cluster Program Managers Meeting Held

The TNC Clinical Research and Regulatory Affairs Service organized its first Neuroscience Clinical Research Cluster Program Managers Meeting on September 15, 2016. The meeting was attended by project and program managers representing Developmental Medicine, Emergency Medicine, Genetics and Genomics, Neurology, Neurosurgery, Newborn Medicine and Psychiatry. The meeting included discussion on a variety of topics around conducting clinical research studies/trials including developing best practices. Issues surrounding study staff training, reviewing and centralizing research templates/guidance documents and clinical trial/grant budgeting were discussed. These meetings are scheduled to be held monthly and we look forward working with this diverse group of experienced managers.