Winter 2020 | News & Updates from our Research Center

**Center Updates**

Check out our new logo!

Our new website is set to launch in June!

**NEW LOCATION**

The IDDRC team will be moving to 1 Autumn Street in May 2020.

**New Staff Member**

Eva Economou, Program Manager, joined the IDD Research Center in February, and she will be serving as the administrative point person for our center.

(eva.economou@childrens.harvard.edu)

**Research News**

Congratulations to Drs. Engle and Stevens!

Last fall Dr. Elizabeth Engle and Dr. Beth Stevens were elected to the National Academy of Medicine. Membership in the NAM is considered one of the highest honors in the fields of health and medicine.

Dr. Ann Poduri’s work was featured in Boston Globe’s Meet the researchers working to curb childhood cancer, repair your ACL, stop epileptic seizures, and more, and in the Wall Street Journal’s Unfulfilled Promise of DNA Testing.

MIT Technology Review announced this year’s 10 Breakthrough Technologies, and one of the topic areas is hyper-personalized medicine, which includes the work of Dr. Tim Yu to treat his patient, Mila Makovec. Read more

**Current Trials in our Center**

**Rett Syndrome:** Rett syndrome is a neurodevelopmental disorder that primarily affects females. The disorder is characterized by normal development for the first 6 to 18 months of life,
followed by a period of regression when many children lose their ability to speak, walk, and use their hands. Breathing problems, seizures, anxiety, orthopedic, and gastrointestinal issues are also common. Rett syndrome is caused by pathogenic (disease causing) variants in the MECP2 gene on the X chromosome. The Rett Syndrome Research Program at Boston Children's Hospital, led by David Lieberman, MD, PhD and Mustafa Sahin, MD, PhD, is committed to advancing our understanding of Rett syndrome and MECP2-related disorders.

ARCH Study: This trial is sponsored by GW Pharmaceuticals. The goal of this randomized, double-blind, placebo-controlled trial is to investigate the safety and efficacy of cannabidiol oral solution (GWP42003-P) compared with placebo, at the end of up to 24 weeks' treatment in reducing symptom severity in patients with Rett syndrome.

For more information about the trial, please contact: Michelle DeLeo at RettResearch@childrens.harvard.edu.

PTEN Hamartoma Tumor Syndrome: Phosphatase and tensin homolog hamartoma tumor syndrome (PHTS) is a rare genetic condition caused by germline heterozygous (PTEN) gene mutations. PHTS is associated with a spectrum of clinical disorders characterized by neurocognitive deficits, autism symptomatology, skin lesions, macrocephaly, hamartomatous overgrowth of tissues, and an increased risk of cancers.

PTEN Study: This study is sponsored by NIH, PTEN Research, and Novartis Pharmaceuticals. The goal of this randomized, double-blind, placebo-controlled study is to evaluate the safety and efficacy of the medication known as RAD001 to affect neurocognition and behavior in children and adolescence with PTEN mutations as measured by standardized, direct and indirect neurocognitive tools and behavioral measures (processing speed/working memory).

For more information about the trial, please contact: Greg Geisel at gregory.geisel@childrens.harvard.edu.

Click here for more projects.

Events and Seminars

Find below our upcoming Neurobiology and Translational Neuroscience seminars. Hope to see you there!

**Neurobiology seminars**

3/9: Ivan Soltesz, PhD
Stanford School of Medicine
Title: 'Organization and control of hippocampal circuits'

3/16: Sergiu Pasca, PhD
Stanford University
Title: 'Assembly of Tridimensional Brain Organoids to Study Development and Disease'

3/23: Kelly Monk, PhD
Vollum Institute, OHSU
Title: TBA

3/30: Mark Harnett, PhD
McGovern Institute, MIT
Title: TBA

4/6: Fred Rieke, PhD
University of Washington
Title: TBA

4/13: Ryohei Yasuda, PhD
Max Planck Florida Institute for Neuroscience
Title: TBA

4/27: Adam Carter, PhD
New York University
Title: TBA

Location: Folkman Auditorium, Enders Bldg
Time: 12.15pm - 1.15pm

**Translational Neuroscience seminars**

3/10: Jeremy Schmahmann, MD
Massachusetts General Hospital
Title: TBA

4/14: Christos Papadelis, PhD
Boston Children's Hospital
Title: TBA

5/12: Chris Dulla, PhD
Tufts University
Title: TBA

6/9: David Glahn, PhD
Boston Children's Hospital
Title: TBA

Location: Center for Life Science, 12th floor
Time: 1pm - 2pm

Click here for more TNC seminars/dates

Click here for more Neurobiology seminars/dates
Featured Publications

Congratulations on your excellent work, Investigators!


