LIBD

LIEBER INSTITUTE *for*
BRAIN DEVELOPMENT
MALTZ RESEARCH LABORATORIES

Global, Collaborative Approach Yields Significant Gains for More Effective Treatments for Mental Illness

Lieber Institute for Brain Development Shares 2014 Progress Report

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BALTIMORE, MD (Dec. 29, 2014)—The Lieber Institute for Brain Development (LIBD) announces significant advances in identifying the causes of schizophrenia and related developmental brain disorders and translating these findings into new treatment strategies:

CRACKING THE CODE OF BRAIN DEVELOPMENT: Two papers published by the LIBD research team report progress identifying specific biological signatures that guide early brain development and can ultimately lead to neurodevelopmental disorders such as autism spectrum disorder (ASD) and schizophrenia. The teams investigated whether prenatal expression of psychiatric disease-associated genes may be responsible for 'kick starting' atypical brain development. The first paper, published in The American Journal of Psychiatry in July, was selected by its editor Robert Freedman, M.D., as "particularly interesting and important" and featured in the editors' selection of noteworthy papers of 2014, noting that it offers an important "puzzle piece" for identifying what causes these disorders to develop. The second paper, published online December 15th in Nature Neuroscience, provides a novel technique for identifying biological markers in brain development that associate with risk for neurodevelopmental disorders. Applying the technique, the team quantified the effect of previously unidentified anomalies in gene expression that determine how the human brain develops from its earliest stages.

DEVELOPING NEW LIBD MEDICATIONS: At LIBD, basic science, clinical research and the development of novel therapeutics are all conducted under one roof. The team filed three patents for new medicines particularly aimed at improving the cognitive impairment that is common in psychiatric and neurologic illness. Cognitive impairment significantly impacts daily functioning skills in patients with brain disorders, limiting their capacity for integration into society. The new LIBD therapies show significant potential to effectively treat these symptoms for which there are currently no medications available; they are fast-tracked for possible Phase I clinical testing in 2015.

CREATING NEW TECHNOLOGIES FOR TREATMENT DEVELOPMENT: A patent was filed for a new technology in stem cell biology that enables a novel approach to identify targets for new treatments of brain disorders. Stem cell technology offers a unique opportunity to translate genetic understanding of mental illness into targeted therapies and the LIBD technology

provides a precise view of how human stem cells act in the first steps that form the human brain. The new technology has enabled a deepening partnership with a major pharmaceutical company in need of a new, more reliable approach for developing treatments across the spectrum of brain disorders.

PARTNERING WITH INDUSTRY TO SPEED RESEARCH TO TREATMENTS: LIBD successfully completed Phase I of an unprecedented precompetitive partnership in a consortium with five major pharmaceutical companies to speed the process of converting basic science findings about the construction and function of the human brain into new treatment options for people living with developmental brain disorders. The consortium is developing a free, online database of in-depth genetic, biological and clinical data that will allow researchers around the world to investigate brain function across lifestages and in schizophrenia. This new approach has brought many of these pharmaceutical companies back to the table to work on developing more targeted and effective treatments for psychiatric illness based on a deeper understanding of the original biological causes.

CONTINUING TO EXPAND THE UNIQUE LIBD BRAIN BANK: LIBD now has more than 1,500 exquisitely curated human brain specimens across lifestages from fetal life to late life, as well as many samples of matched patient skin cells and a bioregistry of extensive clinical and biological information from the individuals. Extensive analytical data from the collection is freely accessible in the LIBD BrainCloud™ database, enabling researchers around the world to study how genes and the environment interact in brain development, and how this may translate to treatments for neurodevelopmental disorders. Hundreds of the brain samples are from individuals who had developmental brain disorders, including the largest collection available of patients who suffered from post-traumatic stress disorder.

WORKING DIRECTLY WITH PATIENT POPULATIONS: With a shared vision of increasing the possibilities for those with mental illness to live full, productive and more joyful lives, LIBD announced a joint venture with Sheppard Pratt Health System, Inc., an internationally renowned behavioral health system that treats more than 70,000 individuals with psychiatric illnesses. Infused with an initial $2 million capitalization from the founding partners, the new Sheppard Pratt-Lieber Research Institute, Inc. (SPL) will develop game-changing new treatments and therapies through research efforts with human patients, including the study of genetics; the use of advanced brain imaging technology; the creation of a patient registry with digital records and an extensive biobank; and clinical trials for both new and repurposed medications.

ESTABLISHING THE FIRST EVER US-CHINA NIH- AND NSF-SPONSORED PARTNERSHIP TO TACKLE MENTAL ILLNESS: Peking University (PKU) and LIBD received support from the National Science Foundation (NSF) in China and the National Institutes of Health (NIH) in the U.S. to develop a joint neuropsychiatric institute (the Peking–Lieber Translational Neuropsychiatry Research Institute) to explore how genes and the environment interact with each other in early development to create risk for and resilience from a range of psychiatric illnesses. The official signing ceremony took place in Beijing in October.

This unique partnership in neuroscience and psychiatry is working with a very large Chinese population to study the effects of starkly different rural and urban childhoods that result from China's unique economic development. An imaging center has been built at PKU, two new MRI scanners have been installed and very detailed data collection began mid-year with LIBD scientists conducting in-depth analyses.

EXPANDING THE JOHNS HOPKINS SCIENCE + TECHNOLOGY PARK: LIBD is the anchor tenant for the research park development of the Forest City-New East Baltimore Partnership. Located on the Johns Hopkins Medical Campus, LIBD expanded another 13,000 square feet to extend its state-of-the-art laboratory space. LIBD attracts scientists from around the world for collaboration, helping boost the research park, economic growth in East Baltimore and the region's high-tech economy as a whole.

About LIBD

The mission of the Lieber Institute for Brain Development is to translate the understanding of basic genetic and molecular mechanisms of schizophrenia and related developmental brain disorders into clinical advances that change the lives of affected individuals. LIBD is an independent, not-for-profit 501(c)(3) organization and a Maryland tax-exempt medical research institution affiliated with the Johns Hopkins University School of Medicine.

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