

Behind the Seizure® Program Further Expands Access to Genetic Testing for Children to Speed the Diagnosis of Genetic Epilepsy

*-- Biogen, Encoded Therapeutics, Neurogene, Praxis Precision Medicines and PTC Therapeutics join founders BioMarin and Invitae to expand access for children under eight --
-- Innovative program may shorten the diagnostic odyssey for children who experience unprovoked seizures --*

SAN FRANCISCO, January 8, 2020 -- Today [BioMarin Pharmaceutical Inc.](#) (NASDAQ: BMRN) and [Invitae Corporation](#) (NYSE: NVTX) announced that Biogen (NASDAQ: BIIB), Encoded Therapeutics, Neurogene, Praxis Precision Medicines and PTC Therapeutics joined [Behind the Seizure](#), an innovative, cross-company collaboration that aims to provide faster diagnosis for young children with epilepsy. The program will also be expanded to make no-charge testing available for healthcare providers to order for any child under the age of eight who has an unprovoked seizure.

“Behind the Seizure is one of the longest-running cross-company collaborations aimed at increasing access to genetic testing. It has been shown to decrease time to diagnosis for children experiencing unprovoked seizures by one to two years from reported averages, and as more companies have joined the program, more children have been helped,” said Robert Nussbaum, chief medical officer of Invitae. “Earlier diagnosis enables clinicians to focus on providing disease-specific care sooner, which is particularly important in neurodegenerative diseases. We applaud these companies for their commitment to expanding this unique effort to help children.”

Previously the program was available to children under age five. The newest companies to join Behind the Seizure include:

- Biogen: Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies.
- Encoded: Encoded Therapeutics, Inc. is a biotechnology company developing precision gene therapies for a broad range of severe genetic disorders including epilepsies with a genetic etiology. Our mission is to realize the potential of genomics-driven precision medicine by overcoming key limitations of viral gene therapy. We are advancing our lead asset, ETX101, for the treatment of SCN1A-positive Dravet Syndrome.
- Neurogene: Neurogene is committed to the rare neurological disease community, which includes children living with CLN5 and CLN7, two types of Batten disease involving recurrent seizures, or epilepsy. Expanding the age range of disease identification via Behind the Seizure will provide answers for many more children with unexplained seizures, including those with a later symptom onset, and will help advance important research in pursuit of developing life-changing medicines for patients.
- Praxis: Praxis is leveraging important breakthroughs in the genetics of epilepsy to develop novel precision medicines addressing the underlying cause of multiple pediatric genetic epilepsies.

- PTC: PTC Therapeutics is a global biotech that is working toward bringing to market the first gene therapy indicated for Aromatic L-amino acid decarboxylase (AADC) deficiency, a devastating rare disease that often can be misdiagnosed as epilepsy. AADC deficiency is fatal and there are no approved therapies that address the underlying cause.

Behind the Seizure is supported by eight sponsors in all, including Stoke Therapeutics and Xenon Pharmaceuticals who joined in 2019.

Since the program began, thousands of children have received genetic testing through Behind the Seizure and research has shown that participants in the program were diagnosed one to two years sooner than historic averages.^[1] Companies that participate in the program sponsor the cost of testing using the Invitae Epilepsy Panel, which includes more than 180 genes associated with both syndromic and non-syndromic causes of epilepsy, including neurodegenerative conditions. With the expansion of the program, healthcare providers now can order the test for patients under the age of eight with unprovoked seizures. Test results are available quickly (14 days on average).

More than half of epilepsies are based in genetics. When a child presents with seizures, genetic testing can help identify more than 100 underlying, often rare conditions. Early genetic testing may be the most cost-effective, direct and accurate diagnostic tool for children, shortening years-long diagnostic odysseys. Delays in diagnosis can be devastating for children, as some genetic epilepsies are neurodegenerative and early symptoms may be subtle and easy to misdiagnose.

Participating companies provide financial support for this program, which includes testing and services performed by Invitae. Healthcare professionals must confirm that patients meet certain criteria to use the program. Third parties and commercial organizations may receive de-identified patient data and contact information for healthcare providers who use this program, but at no time do they receive patient identifiable information. Genetic testing and counseling are available in the US and Canada. Healthcare professionals and patients who participate in this program have no obligation to recommend, purchase, order, prescribe, promote, administer, use or support any other products or services from Invitae or from third parties or commercial organizations.

About Behind the Seizure

Behind the Seizure is an innovative, cross-company collaboration designed to increase access to genetic testing for children who experience unprovoked seizures in childhood in the United States and Canada. More than half of epilepsies have some genetic basis, and are often associated with rare, neurodegenerative conditions with non-specific symptoms. Early genetic testing may be the most direct, cost-effective, and accurate diagnostic tool. Participants in the Behind the Seizure program are diagnosed one to two years sooner than reported averages. The program was established by BioMarin and Invitae and now includes: Biogen, Encoded Therapeutics, Neurogene, Praxis Precision Medicines, PTC Therapeutics, Stoke and Xenon. To

learn more about the Behind the Seizure program please visit <https://www.invitae.com/en/behindtheseizure/>.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. The company's portfolio consists of seven commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.biomarin.com.

About Invitae

Invitae Corporation (NYSE: NVTX) is a leading genetics company whose mission is to bring comprehensive genetic information into mainstream medicine to improve healthcare for billions of people. Invitae's goal is to aggregate the world's genetic tests into a single service with higher quality, faster turnaround time, and lower prices. For more information, visit the company's website at invitae.com.

About Encoded Therapeutics

Encoded Therapeutics, Inc. is a biotechnology company developing precision gene therapies for a broad range of severe genetic disorders. Our mission is to realize the potential of genomics-driven precision medicine by overcoming key limitations of viral gene therapy. We focus on delivering life-changing advances that move away from disease management and towards lasting disease modification. For more information, please visit www.Encoded.com.

About Neurogene Inc.

Neurogene was founded to bring life-changing medicines to patients and families affected by rare neurological disorders. We partner with leading academic researchers, patient advocacy organizations and caregivers to bring to patients therapies that address the underlying genetic cause of a broad spectrum of neurological diseases where no effective treatment options exist today. Our lead programs use AAV-based gene therapy technology to deliver a normal gene to patients with a dysfunctional gene. Neurogene is also investing in novel technology to develop treatments for diseases not well served by gene therapy. For more information, visit www.neurogene.com.

About Praxis Precision Medicines

Praxis Precision Medicines is a clinical-stage genetic neuroscience company developing high-impact therapies for patients and families affected by complex and debilitating brain disorders, including rare pediatric epilepsies and neuropsychiatric disorders. These two disease areas share overlapping disease biology and genetic targets, as well as a profound need for new therapeutic options that target the underlying cause of the disease. Praxis is advancing a pipeline of breakthrough medicines with the potential to more precisely treat complex brain disorders. For more information, please visit www.praxismedicines.com.

About PTC Therapeutics

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

[1] Miller, Nicole, et al, "Behind the Seizure: A No-Cost 125-gene Epilepsy Panel for Pediatric Seizure Onset Between 2–4 Years". Presented at the American Society of Human Genetics Meeting: October 16–20, 2018, San Diego, CA.