



Pediatric Epilepsy Research Foundation to Fund Research on Treatment for Infantile Spasms Pediatric Epilepsy Research Consortium to Conduct Study

DALLAS (Nov. 7, 2017) – [The Pediatric Epilepsy Research Foundation](#) (PERF), a not-for-profit foundation established in 2004, announced that the research study "A Novel Approach to Infantile Spasms: Combined ACTH and Vigabatrin Induction Therapy" has met its scientific criteria for funding. To fund this research, PERF will issue a \$3,150,000 grant to study the combined synthetic adrenocorticotrophic hormone (ACTH) and vigabatrin (VGB) as a first-line treatment of infantile spasms (IS), a form of epilepsy affecting infants usually under one-year of age. The study will be conducted by [The Pediatric Epilepsy Research Consortium](#) (PERC) and led by principal investigator Kelly Knupp, M.D., from Children's Hospital Colorado and the University of Colorado School of Medicine.

"A Novel Approach to Infantile Spasms: Combined ACTH and Vigabatrin Induction Therapy" is the first study of its kind to be conducted in the United States and is funded collaboratively by PERF and West Therapeutic Development. The study has satisfied requirements for funding including receipt of approvals from the Food and Drug Administration to proceed with the Investigational New Drug (IND) clinical study and from the Institutional Review Board to initiate the clinical study. Investigators from 15 academic medical centers across the United States will convene in January for an investigators meeting to begin the trial.

This FDA-approved research project will focus on determining:

1. the efficacy of the investigational drug synthetic (1,24) ACTH Depot (cosyntropin), which consists of the first 24 amino acids occurring in the natural corticotropic hormone (ACTH) sequence and displays the same physiological properties as ACTH compared to vigabatrin in children with new onset infantile spasms;
2. the efficacy of combination therapy of synthetic (1,24) ACTH and vigabatrin to monotherapy synthetic (1,24) ACTH;
3. the outcome of development in children with infantile spasms at 18 months of age; and
4. seizure outcomes in children with infantile spasms at 18 months of age.



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Synthetic ACTH Depot is used in virtually every country around the world for the treatment of infantile spasms, except the United States where it is not currently approved.

"These are the type of studies we like to fund because the findings have the potential to dramatically change lives. We are excited to see the results of this test, which are based on a similar European study that discovered a 71 percent effective rate of the combination therapies (syn-ACTH or oral steroids plus

vigabatrin) versus a 54 percent effective rate of syn-ACTH or oral steroids alone,” said Roy Elterman, M.D., co-founder of PERF. “While infantile spasms are relatively rare, (around 1,000-2,000 infants are diagnosed each year in the US), the treatment options are limited. We are hopeful this revolutionary combined drug therapy has an opportunity to significantly increase the number of spasm free patients with West syndrome.”

According to the National Institute of Neurological Disorders and Stroke, an infantile spasm is defined as a specific type of seizure seen in an epilepsy syndrome of infancy and childhood known as West Syndrome. West Syndrome is characterized by infantile spasms, developmental regression and a specific pattern on electroencephalography (EEG) testing called hypsarrhythmia (chaotic brain waves). The onset of infantile spasms is usually in the first year of life, typically between 4-8 months. The seizures primarily consist of a sudden bending forward of the body with stiffening of the arms and legs; some children arch their backs as they extend their arms and legs. Spasms tend to occur upon awakening or after feeding, and often occur in clusters of up to 100 spasms at a time. Infants may have dozens of clusters and several hundred spasms per day. Infantile spasms usually stop by age five, but may be replaced by other seizure types. Many underlying disorders, such as birth injury, metabolic disorders and genetic disorders can give rise to spasms, making it important to identify the underlying cause. In some children, no cause can be found.

West Therapeutic Development and Depomed will have exclusive rights to utilize the data collected for regulatory filing purposes to obtain approval for an infantile spasms indication. The investigators have full academic freedom to publish and report on the trial results.

The study is expected to start in early 2018 and last three years. Approximately 400 patients will participate in the study.

More information on “A Novel Approach to Infantile Spasms: Combined ACTH and Vigabatrin Induction Therapy” has been submitted to clinicaltrials.gov and is expected to be posted shortly. For more information on The Pediatric Epilepsy Research Foundation go to <http://pediatricpilepsyresearchfoundation.org>.

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ABOUT PEDIATRIC EPILEPSY RESEARCH FOUNDATION

The Pediatric Epilepsy Research Foundation provides grant funding to child neurologists in the U.S./Canada for meritorious clinical, translational, basic science, comparative-effectiveness, implementation research, etc. related to all neurologic conditions in infants, children and adolescents. Epilepsy projects are of particular interest. <http://pediatricpilepsyresearchfoundation.org>

ABOUT THE PEDIATRIC EPILEPSY RESEARCH CONSORTIUM

The Pediatric Epilepsy Research Consortium (PERC) is a group of US pediatric epilepsy centers, researchers and professionals in field of epilepsy whose goal is to provide a network and infrastructure to facilitate collegial, collaborative practice-changing research that will provide answers needed to improve the care of children with epilepsy. Many severe childhood epilepsies are relatively rare, and it is only through a large collaborative effort involving multiple centers that progress in their treatment and outcomes will be made. <http://www.pediatricerc.com>

ABOUT DEPOMED

Depomed is a leading specialty pharmaceutical company focused on enhancing the lives of the patients, families, physicians, providers and payors we serve through commercializing innovative products for pain and neurology related disorders. Depomed markets five medicines with areas of focus that include mild to severe acute pain, moderate to severe chronic pain, neuropathic pain and migraine. Depomed is headquartered in Newark, California. To learn more about Depomed, visit www.depomed.com.

ABOUT WEST THERAPEUTIC DEVELOPMENT

U.S. based West Therapeutic Development and Galway, Ireland based sister company, Eolas Pharma Teoranta, are biopharmaceutical companies focused on improving patient health and addressing unmet medical needs by acquiring, developing and commercializing specialty pharmaceutical products in niche orphan disease and specialty market segments. Eolas Pharma Teoranta is developing synthetic ACTH Depot (cosyntropin) for approval in the UK, EMEA and other markets around the world and is the exclusive supplier of synthetic ACTH Depot (cosyntropin) to Depomed. Depomed Inc. is the exclusive licensee for synthetic ACTH Depot (cosyntropin) in the United States and Canada.