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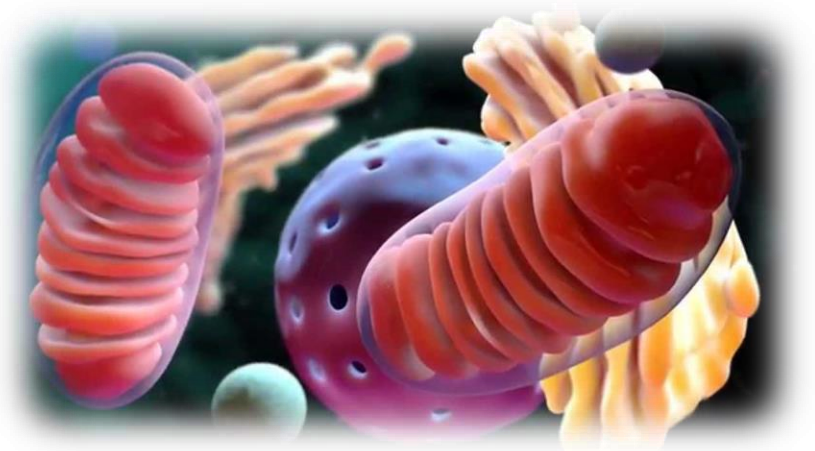
ENERGX Therapeutics



*Energ*XT: A rare diseases biotech providing personalized medicine solutions for disorders of fatty acid oxidation and energy metabolism

ID: *02591, 02669, 03664, 03744, 03786, 03927, 03999, 04061, 04537*

Featured Innovators: Al-Walid Mohsen, PhD, and Jerry Vockley, MD, PhD

Inborn errors in metabolism (IEM) result in diminished function of biochemical pathways that lead to severe, chronic medical conditions with few or no treatment options. Current therapies are restricted to management of diet, limiting physical exertion, and hospitalization in times of metabolic crisis due to infection, fever, or other physiologic stressors. *Energ*XT offers new alternatives for treating fatty acids oxidation (FAO) and energy metabolism disorders through a personalized medicine approach. The individualized testing platform and therapeutic solutions provided by *Energ*XT address the wide variability in disease presentation with a custom treatment plan for each patient.

Technology Description

Using our molecular and cellular testing platform, we have shown that treatment with a reformulation of trimetazidine (TMZ) — an ischemic heart disease generic drug approved outside the US — significantly improves activity of defective mitochondrial fatty acid oxidation enzymes in cells from patients with FAO and metabolic disorders. TMZ causes FAO intermediates to act as chaperones, stabilizing defective enzymes. In combination with other metabolic treatments, our solutions will increase the function of the fatty acid oxidation pathway to dramatically improve energy metabolism. *Energ*XT's ultimate goal is to improve the quality of life for infants, children, and adults with fatty acid oxidation disorders, and to eliminate morbidity and mortality caused by these devastating diseases.

Advantages

- Comprehensive therapeutic solutions to treat patients with fatty acids oxidation disorders
- Custom molecular diagnostic and cellular platform can assess efficacy and customize multi-drug prescription and adjustment for intelligent, personalized therapy
- Eliminates the threat of decompensation
- Improves patients' quality of life and enables them to exercise freely

Applications

- MCAD deficiency
- VLCAD deficiency
- LCHAD deficiency
- Trifunctional protein deficiency
- CPTII deficiency
- Other organic acidemias

Stage of Development

- Clinical trials and preclinical testing of diagnostics and dosage
- Developing and testing combination therapy *in vitro*
- Ongoing animal studies

IP Portfolio

- MCAD treatment with phenylbutyrate:
  US 9,283,200
  US 9,649,285
- Methods of treatment of rhabdomyolysis:
  WO 2017/070445
- Disorders of propionate metabolism and LCHAD with anaplerotic agents:
  WO/2017/184583
- Use of mitochondria-targeting electron, radical to treat fatty acid disorders:
  WO 2017/193000
- Use of Trimetazidine and combinations:
  WO2018/093839
- Acyl-CoA dehydrogenases micro/nano enzyme assay for clinical diagnosis:
  US20180023113

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Innovators

Al-Walid Mohsen, PhD

Research Associate Professor
Department of Pediatrics
University of Pittsburgh

Entrepreneurial Lead

After receiving his PhD, Dr. Mohsen joined Dr. Vockley's research lab at the Mayo Clinic leading discovery of the function of previously unknown enzymes and attended sabbaticals in Konstanz, Germany, and Leicester, UK. He moved with Dr. Vockley to the Department of Pediatrics at Pitt, Division of Medical Genetics in 2004, where he oversees Dr. Vockley's Research program and manages his research lab. In 2009, he was awarded NIH funding for MCAD deficiency therapy. In the last few years, Dr. Mohsen has become the lead innovator in MCAD deficiency and fatty acid oxidation disorders therapy that resulted in numerous patent applications. He is a member of the organizational committee of the International Network on Fatty Acid Oxidation Research and Therapy (INFORM) and is involved in organizing its annual international meetings and its collaborative activities including webinars and a virtual tissue bank.

Education

PhD, Auburn University
BS, University of Ain Shams, Egypt

Jerry Vockley, MD, PhD

Professor
Department of Pediatrics
University of Pittsburgh

Chief of Medical Genetics and
Director of the Center for Rare
Diseases Therapy
Children's Hospital of UPMC

Dr. Vockley is internationally recognized as a leader in the field of inborn errors of metabolism. His current research focuses on mitochondrial energy metabolism, novel therapies for disorders of fatty acid oxidation and amino acid metabolism, and population genetics of the Plain communities in the US. He is the PI on four NIH grants and a co-PI on seven others. Dr. Vockley has served on numerous national and international scientific boards including the Advisory Committee (to the Secretary of Health and Human Services) on Heritable Disorders in Newborns and Children, where he was chair of the technology committee. He is co-chair of the International Network on Fatty Acid Oxidation Research and Therapy (INFORM). He also serves as chair of the PA State Newborn Screening Advisory Committee and the American College of Medical Genetics Therapeutics Committee. He is a past president of the International Organizing Committee for the ICIEM and the SIMD, and co-founder and editor of the North American Metabolic Academy.

Education

PhD, University of Pennsylvania
MD, University of Pennsylvania
BS, Carnegie Mellon University

Relevant Publications

- Mohsen AW, et al. (2018). Novel drug therapies of fatty acid β-oxidation disorders: The future focus and hope. *Molecular Genetics and Metabolism*, 123(3), 252.
- Kormanik, K., et al. (2012). Evidence for involvement of medium chain acyl-CoA dehydrogenase in the metabolism of phenylbutyrate. *Molecular Genetics and Metabolism*, 107(4):684–689.
- Pena L.D., et al. (2016). Outcomes and genotype-phenotype correlations in 52 individuals with VLCAD deficiency diagnosed by NBS and enrolled in the IBEM-IS database. *Molecular Genetics and Metabolism* 118(4):272-81.
- Leipnitz G, et al. (2018). Evaluation of mitochondrial bioenergetics, dynamics, endoplasmic reticulum-mitochondria crosstalk, and reactive oxygen species in fibroblasts from patients with complex I deficiency. *Scientific Reports*, 8(1), 1165.
- Schiff M, et al. (2013). Molecular and cellular pathology of very-long-chain acyl-CoA dehydrogenase deficiency. *Molecular Genetics and Metabolism*, 109(1), 21-27.

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