Epilepsy Research: Accelerating New Therapies

Epilepsy Foundation Research and New Therapies Program Overview

Dr. Kathleen Farrell, MB BCh BAO
Director of Clinical Research
UNMET NEED

30% of people living with epilepsy **still** do not have seizure control.
RESEARCH INVESTMENT IN EPILEPSY IS LOW

Compared to other neurological diseases, epilepsy nonprofits contribute minimally to research

Top Nonprofits: $74.5 Million

Top Nonprofits: $5.5 Million

$161 Million

$157.9 Million

Parkinson’s Disease

Epilepsy
RESEARCH OVERVIEW

Our Purpose:
To develop an epilepsy research ecosystem for excellence, innovation, radical thinking and exciting discoveries to end epilepsy.

Innovation
Pushing new areas and ideas for epilepsy care and cures
- Epilepsy Innovation Institute
- Epilepsy Therapy Project
- Targeted Research Programs

Engagement
Building collaborative networks between people with epilepsy, their families, healthcare providers, researchers and investors
- Rare Epilepsy Network
- Research Roundtable
- Clinical Trials Portal

Next Generation Scientists
Supporting our developing workforce in partnership with the American Epilepsy Society
- Clinical Research Fellowships
- Junior Investigator Research Awards
- Clinical Research Apprenticeships
Aside from the funding, validation by ETP experts who vetted our project, and found it to be a worthwhile pursuit, has been a significant factor in our rapid progress and expansion into new centers.

— Ashok Gowda, PhD, Founder of Visualase, Inc, acquired by Medtronic in 2014, President & CEO BioTex, Inc.
ACCELERATING LATE STAGE DEVELOPMENT THERAPIES ONTO THE MARKET

Assessing our impact in the Epilepsy Therapy Project Program

"The origin of the Epidiolex trial began with a pivotal meeting of researchers from across the US and representatives of the Epilepsy Foundation. This was followed the next year by the Epilepsy Foundation’s Innovation Seal of Excellence research award, that provided critical seed funding for the open label study. Positive results from that study convinced GW Pharmaceuticals to proceed with the randomized controlled trials."

— Orrin Devinsky, MD, Director of the NYU Comprehensive Epilepsy Center and the Saint Barnabas Institute of Neurology and Neurosurgery
SAVE THE DATE

EPILEPSY FOUNDATION SUDEP CHALLENGE

The Fourth Challenge: Validating Biomarkers of Epilepsy Seizures

Launched 08/08/2016

Milestone 1: Deadline 10/10/2016
Award Pool: $100,000

Milestone 2: Deadline 10/10/2017
Award Pool: $100,000

Milestone 3: Deadline 10/10/2020
Award Pool: $800,000

Awardees will demonstrate the validity of human biomarkers that predict the risk for SUDEP or life-threatening seizures that compromise cardiac or respiratory function, and can be used for intervention.

Please visit https://www.innocentive.com/ar/challenge/9933784

Contact Dr. Kathleen Farrell at kfarrell@efa.org
**EPILEPSY INNOVATION INSTITUTE**

*Our Platform*

1. **SELECT TOPIC**
   Through surveys of people with epilepsy and caregivers, we define which area to tackle first.

2. **DEFINE CHALLENGE**
   We invite experts across disciplines to Innovation Workshops where we assess the major scientific roadblocks to addressing unmet needs of people living with epilepsy.

3. **FUND SOLUTION**
   We support multi-disciplinary teams to tackle the defined scientific roadblocks investing $1 million per year for up to 3 years.

4. **PROVIDE MENTORSHIP**
   We provide our Solution Teams with support and specialized expertise throughout the process.
COMMUNITY SURVEY 2016

- What aspects of epilepsy impact people the most?
- Where should researchers focus?
- What is your biggest frustration with epilepsy?
- What is your biggest hope for epilepsy in next 5 years?

Unpredictability
2017 Topic
My Seizure Gauge
Early Warning Seizure-Risk Assessment System
RARE EPILEPSY NETWORK
Creating a learning network and database

Over 1340 people enrolled and growing!
Welcome to the Rare Epilepsy Network. We need your help.

The Rare Epilepsy Network, or REN for short, is a collaboration between the Epilepsy Foundation, RTI International, Columbia University, New York University, and many different organizations that represent patients with a rare syndrome or disorder that is associated with epilepsy or seizures. These organizations include Aaron’s Onahama Foundation, Alcardi Syndrome Foundation, International Foundation for CDKL5 Research, Dravet Syndrome Foundation, Dup15q Alliance, Hope for Hypothalamic Hamartoma, Lennox-Gastaut Syndrome Foundation, PCDH19 Alliance, Phelan-McDermid Syndrome Foundation, and Tuberous Sclerosis Alliance. The REN will establish a registry of these patients which includes patient or caregiver-reported data in order to conduct patient-focused research. Learn how you can help with this important research.

What You Can Do

**Learn**
Learn about the REN study and whether you or your child is eligible to participate.

**Enroll**
Enrollment is easy and can be done online.

**Participate**
Enrolled users login and participate here.

Research Project Summary

The Rare Epilepsy Network (REN) PPRN is an initiative created by and for patients with catastrophic rare epilepsies. The REN’s goal is to build a patient-centered and-driven database designed to provide the patients and their families an opportunity to participate in research that will improve the lives and quality of care for people with rare epilepsies.

Our PPRN is led by the Epilepsy Foundation (EF), a patient advocacy organization dedicated to the welfare of the almost 3 million people with epilepsy living in the United States.
RESEARCH ROUNDTABLE

Convening Stakeholders to Address Roadblocks to Research and Development

- 2016: Reducing Placebo Exposure in Clinical Trials
- 2017: Pediatric Drug Development
- 2018: Populations and Endpoints in Epilepsy Trials
Several large epilepsy trials funded by the National Institutes of Health had to be terminated due to insufficient enrollment.

84% of volunteers for epilepsy clinical trials would be willing to participate again.
NEXTGEN SCIENTISTS
Since 2007, investing over $12.65 Million in our developing workforce

- 69 Junior Investigators
- 99 Postdoctoral Fellows
- 36 Clinical Fellowships
- 69 Graduate Students
- 30 College Students
SINCE 2007, OVER $24.6 MILLION INVESTED IN ALL RESEARCH GRANTS

Our research has no boundaries. We go where the science is.

We also have invested over $1.4 Million abroad:
- Africa
- Asia
- Australia
- Canada
- Europe
- South America
### OUR GLOBAL AND LOCAL RESEARCH IMPACT

**GLOBAL**

- Since 2007, the national office has invested over $24.6M worldwide to support
  - The developing workforce
  - Investments in new therapies, and
  - Developing new diagnostic tests for epilepsy care
- We have invested in ~40% of pipeline therapies
- We supported 239 researchers/clinical fellows
- We are expanding our programs to focus on higher-risk projects

**Local**

- Since 2007, the national office has invested $510,000 in Michigan to support
  - The developing workforce, and
  - Developing diagnostic tools
SUPPORTING RESEARCHERS IN MICHIGAN

This project has led to the development of a number of important experimental tools that are now being used to understand changes... that result in defective “wiring in the brain.” The award support I received was incredibly helpful... and had greatly improved the likelihood that I will be able to continue to perform epilepsy-related research”

– Jeffrey Calhoun, PhD
(2012 Predoctoral Fellowship at University of Michigan. Upon getting his PhD, is continuing to research Epilepsy as a postdoctoral fellow at Northwestern University)

“Our [EF Supported] study highlights the multifaceted pathophysiology of a severe form of epilepsy [SCN1A associated Dravet Syndrome]... that includes brain, heart and autonomic disturbances, and suggests novel therapeutic strategies for interventions to reduce sudden death...”

– David Scott Auerbach, Ph.D.
(2013 Postdoctoral Fellowship at University of Michigan. Dr. Auerbach is now part of the Center for SUDEP research)
HOW TO GET INVOLVED

• **Research**
  • Attend [2018 Pipeline Conference and Community Day](#)
  • Enroll in the [Rare Epilepsy Network](#)
  • Consider a clinical trial or observational study via EF’s [Clinical Trials Portal](#)

• **EF Advocacy and Awareness**
  • Make your voice heard in [Public Policy](#) matters
  • [2018 National Walk](#) (SAVE THE DATE 4/14/18)
  • [Community Action Networks](#)
  • [Lemonade for Livy](#)
  • [Kids Crew](#), and many more!
Thank You