

## **RRE Advisory Committee**

The Epilepsy Foundation deeply appreciates the partnership of four esteemed organizations that make up the Research Roundtable Advisory Committee: the American Epilepsy Society, Citizens United for Research in Epilepsy, the Epilepsy Leadership Council and the National Institute for Neurological Disorders and Stroke. This outstanding group, in addition to two members nominated from within the RRE, played a major role in organizing the agenda for the meeting around the chosen topic, confirming the most innovative speakers, and ensuring this years' Roundtable is a success for individuals with epilepsy, their families, and all who are dedicate their professional lives to treating epilepsy patients and finding a cure. Thank you!

# **American Epilepsy Society**

2017 RRE representative: Dr. Dennis Dlugos, MD

## Additional attendees:

- 1. Ms. Eileen Murray, MM, CAE, Executive Director
- 2. Dr. Cynthia Harden, MD

The American Epilepsy Society (AES) is a medical and scientific society whose members are professionals engaged in both research and clinical care for people with epilepsy. For more than 75 years, AES has provided a dynamic global forum where professionals from academia, private practice, not-for-profit, government and industry participate in interdisciplinary communication and information sharing. AES champions sound science and clinical care through the exchange of knowledge, by providing education, supporting research, and by furthering the advancement of the profession. AES is a chapter under the North American Commission of the International League Against Epilepsy.

# Citizens United for Research in Epilepsy

2017 RRE representative: Dr. Laura Lubbers, PhD, Chief Scientific Officer



# **Epilepsy Leadership Council**

2017 RRE representative: Dr. Christianne Heck, MD, MMM

## **National Institute of Neurological Disorders and Stroke**

2017 RRE representative: Dr. Adam Hartman, MD, Program Director

#### Additional attendees:

- 1. Dr. John Kehne, PhD, Program Director; Director, Epilepsy Therapy Screening Program
- 2. Dr. Vicky Whittemore, PhD, Program Director, Epilepsy

The National Institute of Neurological Disorders and Stroke (NINDS) is one of the 27 Institutes and Centers at the National Institute of Health (NIH), and since 1950 has been conducting and funding research for brain and nervous system disorders. NINDS promotes scientific discovery through basic and translational research, as well as through clinical trials that benefit patients. Initiatives like the Epilepsy Therapy Screening Program seek to pioneer new therapies for those living with seizures.

# **Research Roundtable Advisory Members**

Each year, members of the Research Roundtable elect two representatives to participate on the Advisory Committee. The 2017 representatives are:

- 1. Dr. Gail Farfel, PhD, Executive Vice President and Chief Development Officer, Zogenix, Inc.
- 2. Dr. Lynn Kramer, MD, Corporate Vice President, Chief Clinical Officer and Chief Medical Officer, Neurology Business Group, Eisai Co. Ltd.



## **2017 RRE Member Companies**

The Epilepsy Foundation is honored to include eighteen highly innovative companies in the second year of the Research Roundtable for Epilepsy (RRE) initiative. Participating companies, designated representatives, and a brief summary of each member company's priorities for new and improved epilepsy therapies are listed below. On behalf of the community of individuals and families living with epilepsy, the Foundation commends the Research Roundtable member companies for contributing to this altruistic and collaborative partnership to advance new therapies for the epilepsies. Thank you!

## **Adamas Pharmaceuticals**

Adamas Pharmaceuticals, Inc. is driven to improve the lives of those affected by chronic disorders of the central nervous system. The company seeks to achieve this by modifying the pharmacokinetic profiles of approved drugs to create novel therapeutics for use alone and in fixed-dose combination products. Adamas is currently developing ADS-5102, its lead wholly owned product candidate, for the treatment of levodopa-induced dyskinesia associated with Parkinson's disease and for the treatment of major symptoms associated with multiple sclerosis in patients with walking impairment. The company is also evaluating ADS-4101, an extended-release version of an FDA-approved single-agent compound for the treatment of epilepsy. In addition, under a license agreement with Forest Laboratories Holdings Limited, an indirect wholly owned subsidiary of Allergan plc., the company is eligible to receive royalties from Forest on sales of Namenda XR® and Namzaric™ beginning in June of 2018 and May of 2020, respectively. For more information, please visit www.adamaspharma.com.

(Namzaric<sup>™</sup> is a trademark of Merz Pharma GmbH & Co. KGaA; Namenda XR® is a registered trademark of Merz Pharma GmbH & Co. KGaA.)

# **Anavex Life Sciences Corporation**

2017 RRE representative: Christopher Missling, PhD, Chief Executive Officer and President

## Additional attendees:

1. Ms. Kristina Capiak, CCRP, Vice President of Regulatory Affairs

Anavex Life Sciences Corp. is a biopharmaceutical company dedicated to the development of differentiated therapeutics for the treatment of neurodegenerative and neurodevelopmental diseases





including Alzheimer's disease, Parkinson's disease and rare, orphan indications such as Rett syndrome, for which the FDA has awarded orphan designation for ANAVEX 2-73. Anavex's lead drug candidate, ANAVEX 2-73, is an orally available, small-molecule activator of the sigma-1 receptor restoring cellular homeostasis by targeting protein misfolding, oxidative stress, mitochondrial dysfunction, inflammation and cellular stress, factors in both neurodegenerative and neurodevelopmental diseases.

ANAVEX 2-73 Phase 2a clinical trial in Alzheimer's disease met both primary and secondary endpoints. ANAVEX 2-73 demonstrate a favorable safety, bioavailability, dose-response curve and cognitive and functional benefits. ANAVEX 2-73 demonstrated also efficacy in the following preclinical animal models: Rett syndrome (data from the Rett Syndrome Foundation), epileptic seizures (data from the NIH), Parkinson's disease (data from The Michael J. Fox Foundation for Parkinson's Research), Fragile X-autism-related disorders (data from the Fraxa Foundation), depression, anxiety and multiple sclerosis (MS), indicating its potential to treat additional CNS disorders. Anavex was awarded a grant from the Rett syndrome Foundation to commence a Phase 2 trial with ANAVEX 2-73.

## Axcella Health

#### Eisai

<u>2017 RRE representative:</u> Dr. Lynn Kramer, MD, Corporate Vice President, Chief Clinical Officer and Chief Medical Officer, Neurology Business Group, Eisai Co. Ltd.

#### Additional Attendees:

1. Dr. Antonio Laurenza, MD, Executive Director, Clinical Development, Neurology Business Group

A fully integrated pharmaceutical business, Eisai operates in two global business groups, oncology and neurology, of which epilepsy is a key area of therapeutic focus. The company strives to discover and develop innovative therapies based on an understanding of the emotions and realities of patients, in order to effectively address unmet medical needs. Its epilepsy products include Fycompa®, an AMPA receptor antagonist discovered in-house; Inovelon®/BANZEL®, a treatment for the rare disease Lennox-Gastaut syndrome; Zonegran®, a treatment for partial-onset seizures that Eisai owns the rights to in Europe, the Middle East, Africa and Russia under license from Sumitomo Dainippon Pharma Co., Ltd.; and Zebinix®, a treatment for partial-onset seizures that Eisai co-markets in Europe, the Middle East and Africa through a licensing agreement with the BIAL Group. To learn more about Eisai, please visit us at www.eisai.com.





## **GW Pharmaceuticals**

<u>2017 RRE representative:</u> Dr. Stephen Wright, MD, MBBS, Chief Medical Officer, GW Pharmaceuticals

#### Additional attendees:

- 1. Dr. Kelly Simontacchi, PhD, Senior Advisor, Greenwich Biosciences
- 2. Dr. Catherine Maher, PhD, Head, US Regulatory Affairs, Greenwich Biosciences

Greenwich Biosciences is the US subsidiary of GW Pharmaceuticals plc. The company has established a world leading position in the development of plant-derived cannabinoid prescription medicines to meet patient needs in a wide range of therapeutic indications. Their current epilepsy pipeline of investigational products includes Epidiolex® (cannabidiol oral solution) as a treatment for various orphan pediatric epilepsy syndromes and GWP42006, which features cannabidivarin (CBDV) as the primary cannabinoid and which has shown antiepileptic properties across a range of preclinical models of epilepsy.

## Lundbeck

2017 RRE representative: Dr. Krai-rith Chatamra, PhD, Head of Neurology, Medical Affairs-US

#### Additional attendees:

1. Mr. David Tworek, MS, MBA, Regional Director, Medical Affairs

Lundbeck is a global pharmaceutical company based in Demark and founded in 1915 that strives for global leadership in psychiatry and neurology. One of the world's leading companies specializing in brain disorders, Lundbeck is focused on developing innovative treatments. Their epilepsy products include Onfi (clobazam; approved in 2011 for use in Lennox-Gastaut syndrome), Sabril (vigabatrin; approved in 2009 for use in Infantile Spasms and refractory complex-partial seizures), and I.V. Carbamazepine, recently approved by the FDA.





## **MonoSol**

2017 RRE representative: Mr. Tracy J. Stalvey, MPH, MBA, Director of Clinical Operations

### Additional attendees:

- 1. Mr. A. Mark Schobel, Chief Innovation and Strategy Officer
- 2. Dr. Alfred Schweikert, PhD, VP and Head of Regulatory Affairs

MonoSol Rx is a specialty pharmaceutical company leveraging its proprietary PharmFilm drug delivery technology to develop products that improve patient outcomes and address unmet needs. PharmFilm can benefit patients by improving the efficacy, safety, convenience, and compliance of pharmaceutical products. MonoSol Rx's leadership in film drug technology is supported by strong IP protection, a robust pipeline of prescription drug formulations, and two FDA-approved products — Suboxone® (buprenorphine and naloxone) sublingual film and Zuplenz® (ondansetron) oral soluble film. Their epilepsy pipeline includes Diazepam Buccal Soluble Film and Clobazam Oral Soluble Film.

#### **Neurelis**

2017 RRE representative: Mr. Craig Chambliss, Chief Executive Officer and Co-founder

Neurelis is a specialty pharmaceutical company organized to license, develop, and commercialize product candidates for the treatment of central nervous system disorders. Neurelis' lead product, NRL-1 (intranasal diazepam), is being developed worldwide for pediatric, adolescent, and adult patients with epilepsy who require intermittent use of diazepam to control cluster seizures. In clinical trials, the NRL-1 nasal spray has shown high bioavailability, low patient-to-patient and dose-to-dose variability, and minimal adverse events. In November 2015, Neurelis received Orphan Drug Designation for NRL-1 in the treatment of Acute Repetitive Seizures, and it is in the final stage of clinical testing prior to submitting an NDA.





# **Ovid Therapeutics**

2017 RRE representative: Dr. Matthew During, MD, President/Founder, CSO

### Additional attendees:

- 1. Dr. Joe Grieco, MD Associate Director of Clinical Development
- 2. Dr. Jeannie Visootsak, MD, Early Clinical Development

Ovid Therapeutics is a biopharmaceutical company focused on developing therapies for rare and orphan diseases of the brain. Ovid is pursuing the development of OV101 (gaboxadol) in Angelman syndrome and Fragile X syndrome, two orphan neurological disease indications with no available treatment options. Currently, Ovid has two active studies (Phase 2 safety/exploratory efficacy in adults with Angelman syndrome and Phase 1 PK Study in adolescent subjects with Angelman syndrome and Fragile X syndrome). Ovid is also partnering with Takeda to initiate a Phase 1b/2a study in 2017 in patients with rare epileptic encephalopathies including Dravet syndrome, Lennox-Gastaut syndrome and Tuberous Sclerosis Complex.

### **Pfizer**

<u>2016 RRE representative:</u> Dr. Lloyd Knapp, PharmD, Executive Director, Pfizer Global Product Development, Neurosciences and Pain, Pfizer Innovative Health

#### Additional attendees:

- 1. Ms. Diane Shoda, Senior Director, Pfizer Regulatory Affairs
- 2. Dr. Bruce Parsons, MD, Senior Director Pfizer Global Medical Affairs, Pfizer Innovative Health

Pfizer continues to explore neuroscience diseases with researchers exploring Precision Medicine approaches, rooted in human biology, neuroimaging, novel biomarkers, and a deeper understanding of brain circuitry. Neuroscience researchers are exploring the origins of central nervous system (CNS) diseases using genetics, neurophysiology, and functional brain imaging to design next generation therapeutics. Their epilepsy pipeline includes work with Lyrica (pregabalin; initially approved as adjunctive treatment for partial onset seizures in 2004 in EU), including an ongoing, comprehensive pediatric epilepsy program, as well as PF-06372865 being investigated in a photo-sensitivity epilepsy study.





## **SAGE Therapeutics**

<u>2017 RRE representative:</u> Dr. Amy Schacterle, PhD, Vice President Regulatory Affairs and Quality Assurance

Sage Therapeutics is a clinical-stage biopharmaceutical company committed to developing novel medicines to transform the lives of patients with life-altering central nervous system (CNS) disorders. Sage has a portfolio of novel product candidates targeting critical CNS receptor systems. Sage's lead program, SAGE-547, is in Phase 3 clinical development for super-refractory status epilepticus, a rare and severe seizure disorder. Sage is developing its next generation modulators with a focus on acute and chronic CNS disorders, including orphan epilepsies.

## Sunovion Pharmaceuticals Inc.

2017 RRE representative: Mr. Todd Grinnell, Director, Clinical Development and Medical Affairs

## Additional attendees:

1. Dr. David Blum MD, Executive Medical Director

Sunovion's contribution to epilepsy therapeutics is Aptiom (eslicarbazepine acetate). This agent is approved by FDA for the treatment of partial-onset seizures as monotherapy or adjunctive therapy. Other marketed agents include Latuda (for treatment of schizophrenia and bipolar depression), Brovana (for controlling symptoms of COPD), and Lunesta (for insomnia). They are developing new therapies in the areas of attention deficit disorder, schizophrenia, binge-eating disorder, motor deficit from chronic stroke, and COPD.

# **Supernus Pharmaceuticals**

<u>2017 RRE representative:</u> Dr. Stefan Schwabe, MD, PhD, Chief Medical Officer, EVP of Research and Development

#### Additional attendees:

1. Dr. Welton O'Neal, PharmD, Head of Medical Affairs

Supernus has brought two exciting medications to the epilepsy field in recent years: Oxtellar XR (oxcarbazepine) and Trokendi XR (topiramate). They also have a dynamic pipeline in psychiatry, which





is peaking interest among the neurology community due to the question of links between the two specialties.

#### Takeda

2017 RRE representative: Dr. Mahnaz Asgharnejad, PharmD, VP Global Program Leader

## Additional attendees:

1. Dr. Jonathon Parker, RPh, MS, PhD, VP Global Regulatory Affiars

Takeda Pharmaceutical Company Limited is a global, research and development-driven pharmaceutical company committed to bringing better health and a brighter future to patients by translating science into life-changing medicines. Takeda focuses its R&D efforts on oncology, gastroenterology and central nervous system therapeutic areas plus vaccines. Takeda conducts R&D both internally and with partners to stay at the leading edge of innovation. New innovative products, especially in oncology and gastroenterology, as well as our presence in Emerging Markets, fuel the growth of Takeda. More than 30,000 Takeda employees are committed to improving quality of life for patients, working with our partners in health care in more than 70 countries. Among the several compounds in development in the CNS area is TAK-935; a potent, highly-selective, first-in-class inhibitor of the enzyme cholesterol 24-hydroxylase (CH24H); which is being studied in rare pediatric epilepsies. Additional information about Takeda is available through its corporate website, www.Takeda.com.

## **UCB**

<u>2017 RRE representative:</u> Dr. Konrad Werhahn, MD, PhD, Head of Clinical Strategy, Seizure Freedom Patient Value Mission

#### Additional attendees:

- 1. Dr. Elena Cleary, PhD, Head of Regulatory Pathways, Seizure Freedom Patient Value Mission
- 2. Dr. Ali Bozorg, MD, Medical Director, Seizure Freedom Patient Value Mission

UCB has a rich heritage in epilepsy with more than 20 years of experience in the research and development of anti-epileptic drugs. As a company with a long-term commitment to epilepsy research, UCB's goal is to create more value for patients by addressing their unmet medical needs and continuously advancing science to be able to deliver the right drug at the right time to the right patient. In 2016, there were several significant achievements and pipeline updates to the epilepsy portfolio:





- Vimpat<sup>®</sup> (lacosamide) was approved by the European authorities for use as monotherapy in the treatment of adults with partial-onset seizures, and was approved for use in Japan as adjunctive therapy for partial-onset seizures in patients 16 years and older.
- Briviact® (brivaracetam) was approved in the US and the EU as adjunctive therapy in adults (16 years and older) with partial onset seizures.
- UCB0942 (PPSI) has ongoing Phase II studies in highly drug resistant epilepsy.
- UCB3491 (Radiprodil), an investigational treatment for epilepsy, started clinical Phase I.

## **Upsher-Smith Laboratories**

<u>2017 RRE representative:</u> Dr. Mark B. Halvorsen, PharmD, Medical & Scientific Affiars Associate Vice President

Upsher-Smith Laboratories, Inc. is a family-owned, privately-held pharmaceutical company. Since 1919, we've been a partner in healthcare, striving to be a trusted source for quality, affordable generic and branded medications that bring confidence to patients and families, create better health outcomes and lower the cost of care. Our goal is to continually expand our portfolio with therapies and solutions that measurably improve lives. Historically known for our strong industry relationships and consistent supply, we also view ourselves as a company that can see, pursue and do things differently. We aspire to push boundaries, make an impact and deliver value—not only for our wide variety of customers, but also for the healthcare system at large. For more information, visit <a href="https://www.upsher-smith.com">www.upsher-smith.com</a>.

# **Z**ogenix

<u>2017 RRE representative:</u> Dr. Gail Farfel PhD, Executive Vice President and Chief Development Officer

## Additional attendees:

- 1. Dr. Arnold Gammaitoni, PharmD, Vice President of Medical and Scientific Affairs
- 2. Mr. Alan J. (AJ) Acker, Vice President, Global Regulatory Affairs

Zogenix, Inc. (Nasdaq: ZGNX) is a pharmaceutical company committed to developing and commercializing CNS therapies that address unmet medical needs of people living with orphan and CNS disorders who need innovative treatment alternatives to improve their daily functioning. Zogenix's lead investigational product candidate is ZX008, a fenfluramine HCl oral solution. ZX008 is currently being evaluated in a multicenter, double-blind, parallel-group, placebo-controlled Phase 3 clinical program, FAiRE (Fenfluramine Assessment in Rare Epilepsy), to assess the efficacy, safety, and PK





of ZX008 when used as adjunctive therapy for uncontrolled seizures in pediatric and young adult subjects with Dravet syndrome. For more information, www.zogenix.com

## **Zynerba**

2017 RRE representative: Ms. Terri Sebree, President

## Additional attendees:

- 1. Dr. John Messenheimer, MD, Medical Monitor
- 2. Dr. Donna Gutterman, PharmD, VP Medical

At Zynerba Pharmaceuticals, we are dedicated to developing next-generation synthetic cannabinoid therapeutics for transdermal delivery for patients with high unmet medical needs. Our mission is to improve the lives of patients battling severe health conditions including epilepsy, Fragile X syndrome, osteoarthritis, fibromyalgia and peripheral neuropathic pain through these therapeutics. Our development pipeline includes patent-protected synthetic transdermal cannabinoid products, which we believe will provide new treatment options for patients as well as additional treatment options for patients not currently receiving adequate relief or otherwise experiencing intolerable side effects from current treatment regimens. ZYN002 is the first and only synthetic CBD, a non-psychoactive cannabinoid, formulated as a patent-protected permeation-enhanced gel for transdermal delivery through the skin and into the circulatory system. ZYN001 is a pro-drug of THC that enables effective transdermal delivery via a patch.