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A Rare-Disease Approach to Seizure Treatment

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Overview

Around 50 million people worldwide live with epilepsy making it among the most common neurological disorders[1]. Encompassing a spectrum of complex disorders and characterized by unpredictable seizures that differ in type, cause, and severity[2], epilepsies of all sorts can have profound impacts on both individual quality of life and public health. While epilepsy has been recognized for thousands of years, surprisingly little is understood today about the condition or the mechanisms by which current treatments work.

While epilepsy treatments today are plentiful, they are largely unsatisfactory for patients: often carrying serious side effects and high refractory rates. Further, treatment standards are relatively primitive, with many prescriptions based on trial and error rather than specific patient profiles or differential disease indicators. Yet, despite rising rates of epilepsy, especially among children and older adults[3], drug developers have cut back on research in this area. Many are deterred by the large number of drugs—specifically off-patent drugs—on the market, the challenge of assembling large clinical trials, and unclear financial reward.

With little movement in new epilepsy treatment in the past decade, clinicians are forced to "mix and match" combinations from approximately twenty existing medications, with the realization that current therapies will likely fail for a significant fraction of their patients. And while this process of trial and error results in what may be called personalized medicine, it is hardly the personalization that we'd hope for: using biological knowledge to analyze medical conditions and prescribe treatments with the knowledge that the specific root cause is being addressed.

Polytherapy combinations may work around or attempt to minimize adverse dose related effects, including somnolence, weight gain, feelings of aggression, bone loss, tiredness, memory problems, blurry vision, fatigue and pregnancy risks[4] that can have profound impacts on the patient's quality of life, starting from an early age. Older treatments result in adverse effects in more than half of the patients and newer treatments may offer improved tolerability and safety profiles[5], but higher cost can be a barrier to newer AEDs for many patients.

Along with the challenge of adverse reaction associated with mix-and-match combinations, refractory rates remain the one of the biggest issues of current treatments; an estimated 30 percent of people with epilepsy are refractory to treatment[6].

Given the current state of treatment, biopharmaceutical developers will need to take a different approach to raise the standard of patient care for seizure disorders. Focusing on niche patient populations and using a rare-disease approach could be the answer.

Within the myriad of syndromes within the epilepsies, well-defined subsets present an opportunity for developers to focus on smaller, underserved patient pools with high unmet need. These syndromes are ready for new treatments,

and developing new therapies for these populations follow the tenets that make rare-disease treatment attractive to manufacturers. While the initial beneficiaries of this approach will be those patient subsets, it is expected that the epilepsy community—both patients and manufacturers—may benefit.

Back Bay Life Science Advisors analyzed the evolution of the seizure disorder market to understand how companies develop solutions to long-standing challenges in the treatment of neurologic conditions. We looked closely at the seizure disorders most in need of new development and how companies like GW Pharmaceuticals and Zogenix use a rare-disease approach to help children with specific seizure disorders, and how this approach may benefit the overall patient population.

"Epilepsy is an extremely heterogeneous variable medical condition. There are many different types of epilepsy and many different causes of epilepsy. That is a problem when you're developing drugs. We try to understand how a seizure happens and then intervene at a basic biochemical level to prevent or reduce the risk that a seizure ever starts. But none of that is treating the underlying reason that the seizure is happening in the first place."

Epilepsy Investigator

Seizure Treatment: A Chronic Unmet Need

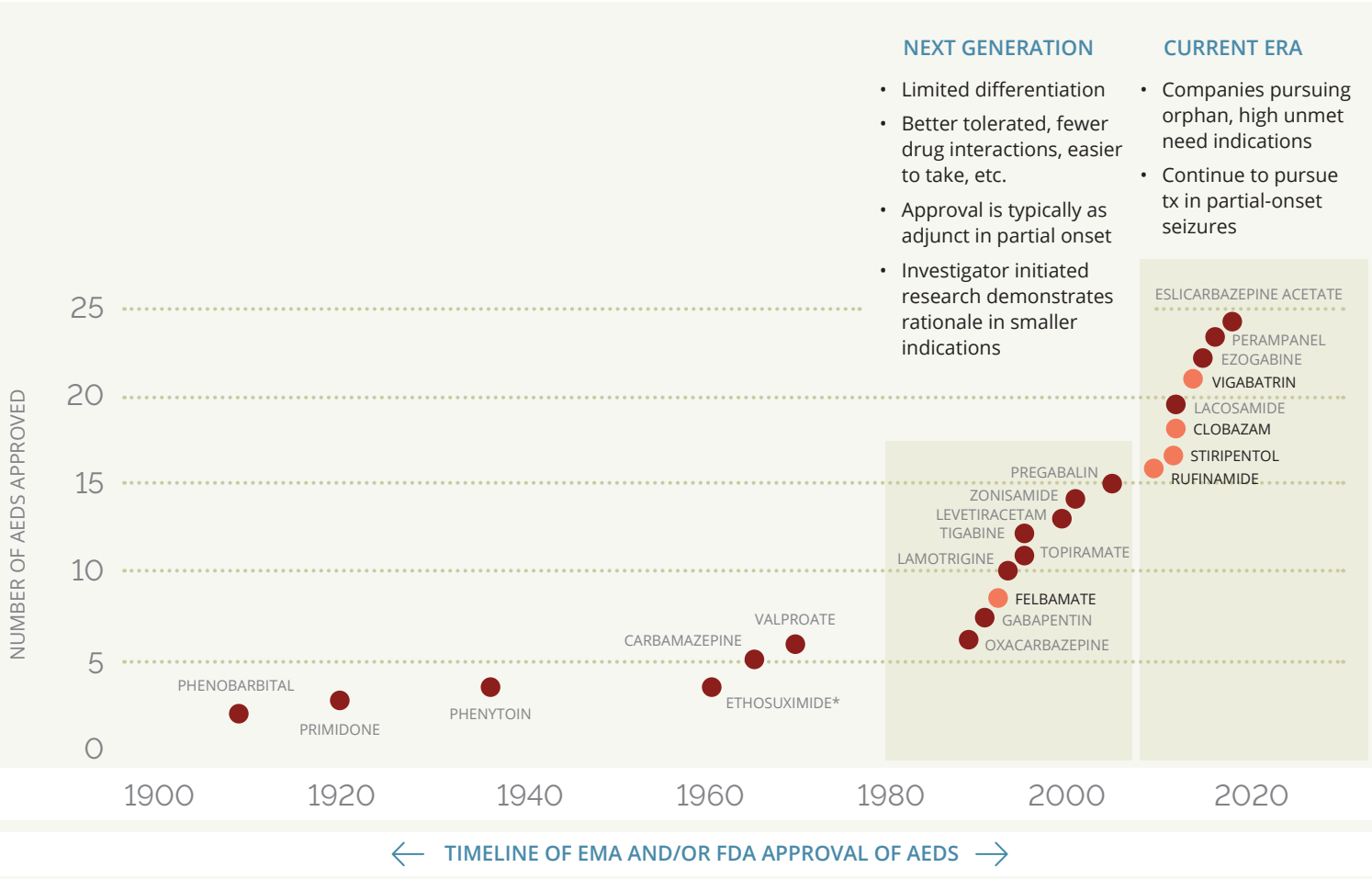
While their causes are poorly understood, and their varieties are many, seizures fall into three main types of seizures: partial (affecting only part of the brain, also called 'focal'), generalized, and unclassified[7]. Most can be described in general terms: when a healthy brain is functioning, a network of neurons constantly carries, responds to, and generates messages as the body interacts with the environment and sustains itself. With the myriad external (sights, sounds, touch, etc.) and internal (hunger, exertion, etc.) stimuli the body is subject to, this typical state can seem almost haphazard—the constant flow of messages makes for a dull roar of brain signals. During a seizure, the flow of information that makes everyday activities work ceases and millions of neurons fire in unison with a hair-trigger threshold. Sometimes this activity manifests itself in the clinical features of a seizure—ranging from momentary non-responsiveness (typical of focal seizures), to full-body muscular limpness (atonic or "drop" seizures) or spasms. In other cases, this activity is clinically subtle and only detected by an electroencephalogram. The origins and causes of this turbulence are often unclear.

The history of seizure medication reflects the neurological community's struggle to understand the human seizure. To date, in the absence of any ways to explore the underlying source of the problem, scientists have developed treatments that simply lowered overall activity of the network to provide a respite for the brain to return to its normal rhythm. Starting in the 1930's, this took the form of barbiturates, trimethadione, and succinicmides; it progressed to the NINDS Anticonvulsant Screening Program (ASP), which was established in 1975 as part of a larger Antiepileptic Drug Development (ADD) program.[8] This program added fifteen more therapies, making the ASP one of the most prolific example of translational medicine in the U.S. medical industrial complex (See Figure 1).

This approach of developing drugs that lower the network activity continues to have merit and some antiepileptic drugs (AEDs) taking this tack have achieved clinical and commercial success. For example, Locosamide, marketed

as Vimpat® by UCB, exemplifies the potential of a new agent with a favorable profile, even in a crowded market. From an efficacy standpoint, locosamide has not shown marked improvement over UCB's flagship drug, levetiracetam (Keppra), but its safety profile is considered much better than its predecessors. In 2008, UCB launched locosamide in the U.S. as an adjunct for focal seizures and received approval for monotherapy in 2014. The drug is expected to generate around $1.3 billion in worldwide sales in 2018[9],based in part on the pressing need for epilepsy treatments that can work when current options have been exhausted.

Figure 1:
Tmeline of Treatment



*Ehtosuxidmide is indicated for generalized absence seizures

However, despite the number of drug options now available, there are still high levels of need for new treatments. Today, seizure control is as much an art as science. There are few indicators predicting which medication or medications will be successful for a given patient. In addition, physicians combine therapies to improve seizure control. Of the approximately 20 medications used in these combinations, most come with wide-ranging side effects, including impacts on mood, appetite, creativity, and thought patterns[10]. Few work consistently for patients. Many patients will not be well-controlled with current therapies[11]—with devastating results throughout their lives, from poor childhood psychosocial interactions and inferior academic achievement, to high levels of adult unemployment and fewer and worse family relationships.[11]

Different—not simply more—drugs are needed.

The Same Path to New Treatments

Despite the high need for new treatments, drug makers have been pulling out of the seizure space even as patients' needs persist. Conversations with industry experts and physicians point to a number of significant barriers including:

- **Heterogeneous Patient Population -** Given that seizures are a symptom of any number of brain disorders, ranging from genetic causes to brain injuries, patient populations are extremely heterogeneous. Their neurological conditions also produce a spectrum of seizure types.
- **Large Refractory Populations -** Most new therapies must be tested in patients who have proven refractory to existing therapies. These patients have often been on many medications and—due to either the nature of their disease or external factors—their seizures are not responsive to treatment. Given the clinical risk and ethical dilemma of taking patients off their current course of anti-epileptic drugs, testing is typically conducted, and approval is typically sought, as an adjunct treatment. Because of this refractory-focused development strategy, patients and physicians often must cycle through several already- approved lines of treatment before trying a developmental agent. This slows both treatment for the individual patient and the testing and approval process for the company.
- **Slow Commercial Uptake -** Commercial uptake is slower in this market, with concomitant longer return on investment due to large and expensive clinical trials. For example, brivaracetam (Briviact®), which received approval from both the EMEA and FDA in 2016 as an adjunctive for people with focal seizures, had been studied in around 3,000 people over eight years before approval. Briviact generated just ~$100M in gross sales in 2017, highlighting the challenges with commercial uptake post-launch.

- **Generics Saturation -** Most major drug developers have seen the challenge of developing new epilepsy treatments where so many exist and most are generic. Subsequently, they have withdrawn from the epilepsy market. As early as 2013, a report by the International League Against Epilepsy (ILAE) and American Epilepsy Society (AES) Working Groups declared: *"...because the marketplace is already awash with anti-epileptic drugs, many pharmaceutical companies now refrain from the expensive enterprise of developing new compounds. Therefore, the ability of the epilepsy research community to convince a limited number of pharmaceutical and biotechnology companies to finance the development of promising new compounds is a growing concern."* [12]

Applying a Rare-Disease Approach

Given these market challenges, some biopharmaceutical companies have pivoted to an orphan-disease approach to developing epilepsy treatments (see Figure 2) seeking to replicate the market success of orphan drugs in other therapeutic areas, such as rare metabolic diseases (e.g., enzyme replacement therapy in lysosomal storage disorders), monogenic diseases (e.g., gene therapies in rare muscular disorders) and mutation-specific cancer indications (e.g., epidermal growth factor receptor [EGFR] in non-small cell lung cancer). The targeted orphan epilepsies typically affect developing brains and are sometimes, as a group, called the "catastrophic generalized epilepsies of childhood." There are several reasons why these conditions are gaining more clinical development now:

- **Better Understanding of Biology –** Basic science progress in neuroscience has recently uncovered genetic causes of some of these syndromes, generating further understanding and awareness in the complex, multifactorial world of epilepsy. For example, several SCN1A disease-causing mutations have been discovered,[13] the causes of a spectrum of seizure disorders ranging from benign febrile seizures to the catastrophic Dravet Syndrome.

- **High Unmet Need -** From a social, economic, and public health perspective, these conditions have high unmet need and, as childhood disorders, can overwhelm families and caregivers. For example:

    - Dravet syndrome (DS), also known as severe myoclonic (twitching or jerking) epilepsy of infancy (SMEI), is rare form of intractable and severe epilepsy that begins in infancy. Sudden unexpected death in epilepsy is the leading reported cause of death in Dravet, accounting for nearly half of all deaths. People typically develop Dravet early in life, and subsequently experience cognitive regression or developmental stagnation that can result in intellectual disability and behavioral disorders.[14]

OVERVIEW of Rare Epilepsy Syndromes

ORPHAN SYNDROME	DISEASE CHARACTERISTICS AND NEED
Dravet / EIFMR	• One of the most severe of the infant epilepsies • High degree of drug resistance • Moderate-severe cognitive disability • 10-20% mortality • Rare compared to LGS and West
Lennox-Gastaut	• 70% identifiable causes, including injury • High degree of drug resistance • Moderate-severe cognitive disability • Need for tx for atonic "drop" seizures • Surgery is a limited option
Tuberous Sclerosis	• Variable drug resistance, some seizure control is possible but tuber removal is not always correlated with seizure control • Severe infantile spasms tx ACTH and vigabatrin • Moderate-severe cognitive disability • Surgery is an option
West/Infantile Spasms	• High unmet need in symptomatic (70%) despite ACTH (significant SEs) and vigabatrin (more sensitive with TS) • Absolute control of seizures is necessary to mitigate developmental delays • Surgery is a limited option
GLUT1 Deficiency Syndrome	• Significant drug resistance • Ataxia and movement problems • Moderate-severe cognitive disability • Ketogenic diet compliance is hard and potential long-term cardio /renal toxicities

- Lennox-Gastaut Syndrome (LGS) is another rare and often debilitating form of childhood-onset epilepsy characterized by a triad of signs including multiple seizure types, cognitive impairment, and an abnormal EEG with slow spike-wave complexes. Because of these complexities, LGS is one of the most difficult forms of epilepsy to treat, according to the LGS Foundation. Frequent falls, injuries and cognitive impairment limit quality of life for LGS patients.[15]

- **Feasible Clinical Development –** In part because of the severity of these conditions, clinical development in these generalized epilepsy syndromes may require fewer trials with fewer patients and shorter duration endpoints than the more traditional partial-seizure (focal-seizure) route (see Figure 2). In addition, given the severity and event rate of some of these conditions, the efficacy of new treatments can be easier to demonstrate. For example, Lundbeck Pharmaceutical's ONFI® (clobazam) was approved as an adjunct

for LGS based on two multicenter controlled trials. The main trial was a placebo-controlled, double-blind and randomized (n=238) and the primary endpoint was a reduction in drop seizures over 15 weeks. Lundbeck's Sabril® (vigabatrin) was approved as a monotherapy for Infantile Spasms (2009 US) based on two multicenter controlled trials (dominant was low-dose, high-dose, partially-blind and randomized (n=221)). The endpoint was the proportion of patients who were spasm-free for seven consecutive days beginning within the first 14 days of therapy.[16]

Figure 2:

Clinical Trial Design Differences Between Partial Seizure Vs Orphan Focus In Epilepsy

PARTIAL Seizures Focus

AED	INDICATION	TRIAL DESIGN	TOTAL POPULATION STUDIED	ENDPOINT DURATION (ENDPOINT)
Vimpat -lacosamide	Adjunct for partial-onset (2008 US)	**Three** randomized, double-blind, placebo-controlled, multicenter trials	1295	20 weeks *(% change in seizure frequency from baseline)*
Fycompa -perampanel	Adjunct for partial-onset (2012 US)	**Three** randomized, double-blind, placebo-controlled, multicenter trials	1037	25 weeks *(% change in seizure frequency from baseline)*
Potiga – ezogabine	Adjunct for partial-onset (2011 US)	**Three** randomized, double-blind, placebo-controlled, multicenter trials	1239	18 weeks *(% change in seizure frequency from baseline)*

ORPHAN Focus (US)

AED	INDICATION	TRIAL DESIGN	TOTAL POPULATION STUDIED	ENDPOINT DURATION (ENDPOINT)
Onfi -clobazam	Adjunct for LGS (2011 US)	**Two** multicenter controlled trials (dominant was placebo-controlled, double-blind and randomized (n=238))	306	15 weeks *(% reduction in weekly frequency of drop seizures)*
Sabril - vigabatrin	Monotherapy for Infantile Spasms (2009 US)	**Two** multicenter controlled trials (dominant was low-dose, high-dose, partially-blind and randomized (n=221)	261	2-3 weeks *(proportion of patients who were spasm-free for 7 consecutive days beginning within the first 14 days of therapy)*

Figure 3:

Current Late Stage Pipeline for Anti-Seizure Drugs

Indicates lead orphan indication

Indicates lead non-orphan indication

COMPANY	THERAPY	MOA	LEAD INDICATION	COMMENTS	PHASE I	PHASE 2	PHASE 3
SK Life Sciences	Cenobamate	Tetrazole derivative	Focal epilepsy	Korean T	●	●	●
Zogenix	ZX008	Serotonergic pathways	Dravet Syndrome	Fenfluramine (of fen-phen)	●	●	●
Aquestive Therapeutics/ MonosolRx	Diazepam Buccal Soluble Film (Diazepam BSF)		Acute Repetitive Seizures	Benzodiazapene with orphan designation	●	●	●
Insys Therapeutics	Cannabidiol	Cannabadiol Receptors -- Cannabinoid-1 (CB1) receptor; Cannabinoid-2 (CB2) receptor; Cannabinoid reuptake/Endocannabinoid system; Serotonin 5-HT1A receptor; GPR55	Infantile Spasms	In Ph 3 for infantile spasms, but in phase 2 for absence seizures and Prader-Willi	●	●	●
Neurelis	NRL-1	intranasal diazepam	Acute Repetitive Seizures	Fast Track Designation / Patented intransal delivery	●	●	●
Sedor Pharmaceuticals	Captisol-enabled™ Fosphenytoin	Phenytoin derivative that does not reconstitution or refrigeration	Status epilepticus seizures occurring during or following neurosurgery or neurologic trauma	Uses Ligand's Captisol formulation	●	●	●
UCB	Midazolam Nasal Spray (USL261)		Acute Repetitive Seizures	Was acquired from Proximagen in April of 2018 / Orphan and Fast Track in US	●	●	●
Biogen	Natalizumab	Intergin antagonist	Focal epilepsy	2010 paper suggests activity	●	●	
Zynerba	ZYN002 Gel	Cannabadiol Receptors	Fragile X Syndrome	First and only pharmaceutically-produced CBD, a non-psychoactive cannabinoid, formulated as a patent-protected permeation-enhanced gel for transdermal delivery through the skin and into the circulatory system	●	●	
Marinus	Ganoxolone	CNS selective GABA A modulator	Fragile X	Synthetic analog of endogenous allopregnanolone, which has been shown to be an effective anticonvulsant	●	●	
UCB	Padsevonil	Dual mechanism of Synaptic Vesicle 2A binding and agonism of GABA-A	Highly drug resistant epilepsy (failed four therapies)	May have been discontinued from earlier development	●	●	
Idorsia Pharmaceuticals	ACT-709478	T-type calcium channel blockers	Photosensitive Epilepsy Patients	Spin out from Actelion	●		
Epygenix	EPX-300	5HT modulator	Dravet Syndrome	ID'd from a zebrafish model of Dravet	●		
Epygenix	EPX-200	5HT modulator	Dravet Syndrome	Weight amnagement therapy	●		
Ovid Therapeutics/ Takeda Pharmaceuticals	TAK-935/ OV935	cholesterol 24-hydroxylase (CH24H) inhibitor	Dravet Syndrome	Global collaboration to develop and commercialise for developmental and epileptic encephalopathies	●		
Cavion Inc	CX-8998	Cav3 (T-Type calcium channel) inhibitor	Generalized Epileptic Syndromes With Absence Seizures	In humans, Cav3 gain-of-function mutants are found in childhood AbsE patients	●		
Biscayne Neurotherapeutics	BIS-001ER	Acetycholinesterase inhibitor	Focal epilepsy (also catastrophic pediatric onset epilepsies such as Dravet and Lennox Gastaut syndromes)	Potent form of huperzine A, a synthetic extract of a traditional Chinese medicine	●		

ANALYSIS OF ORPHAN EPILEPSY FOCUS OVER TIME

Back Bay has analyzed the current late-stage pipeline (See Figure 3) in seizure disorders. As a result of the outlined characteristics and trends, more than half of the therapies in the late-stage pipeline are under development for orphan conditions where seizures are a significant clinical component. More importantly, this charge is being led, not by large biopharma as has traditionally been the case in the epilepsy space, but by smaller, more nimble biotechnology companies.

Taking a look at this pipeline and a deeper look at some of the recently approved and late-stage pipeline candidates (See Figure 4), we can analyze the commercial opportunity (historical peak sales for Sabril® and Onfi®, and analyst-projected peak or 2024 sales for the remaining products) and how it relates to indication focus, efficacy and safety of orphan AEDs. Some clear highlights:

- Eleven of the 17 pipeline candidates are targeting orphan epilepsy indications compared to non-orphan epilepsy indications. Orphan-focus pipeline candidates target the usual suspects such as Dravet (n=4) but also additional orphan white spaces such as Acute Repetitive Seizures and Fragile X Syndrome.
- In some cases, drugs whose safety profiles may be too risky for a larger population can get "new life" in an orphan indication.
- While current era orphan-focus products have generated lower revenues than blockbuster partial seizure products such as Briviact, Fycompa, and Vimpat, some of **the next generation of orphan- focused epilepsy treatments in the pipeline are expected to generate significantly higher revenues (i.e., GW Pharmaceutical's Epidiolex and Zogenix's ZX008).** Both of these compounds have moderate-high efficacy signals with a strong safety profile; although complete data is still pending. Zogenix recently announced clinically significant phase III data for ZX008 in Dravet syndrome with relatively clean safety—albeit in just 43 patients — and GW announced clinically significant and relatively safe data in around 1000 patients across both LGS and Dravet according to a company press release. In the next section, we will dive deeper into how GW Pharmaceuticals and Zogenix are tackling the space, each in their own way, to breathe new life into an old problem.

"We saw four pediatric neurologists in that first year. The fourth doctor told us to stop worrying about stopping the seizures because he could not figure out her EEG [electroencephalogram]. He told us to concentrate on her quality of life. She was 4, not talking, no longer walking, and could not even smile. We were losing everything. What quality of life did she have and where was the bottom of this spiral? We did not want to find out, but we did. We now live at the bottom of the spiral looking up"

Epilepsy Across the Spectrum: Promoting Health and Understanding, Institute of Medicine (US) Committee on the Public Health Dimensions of the Epilepsies

Figure 4:

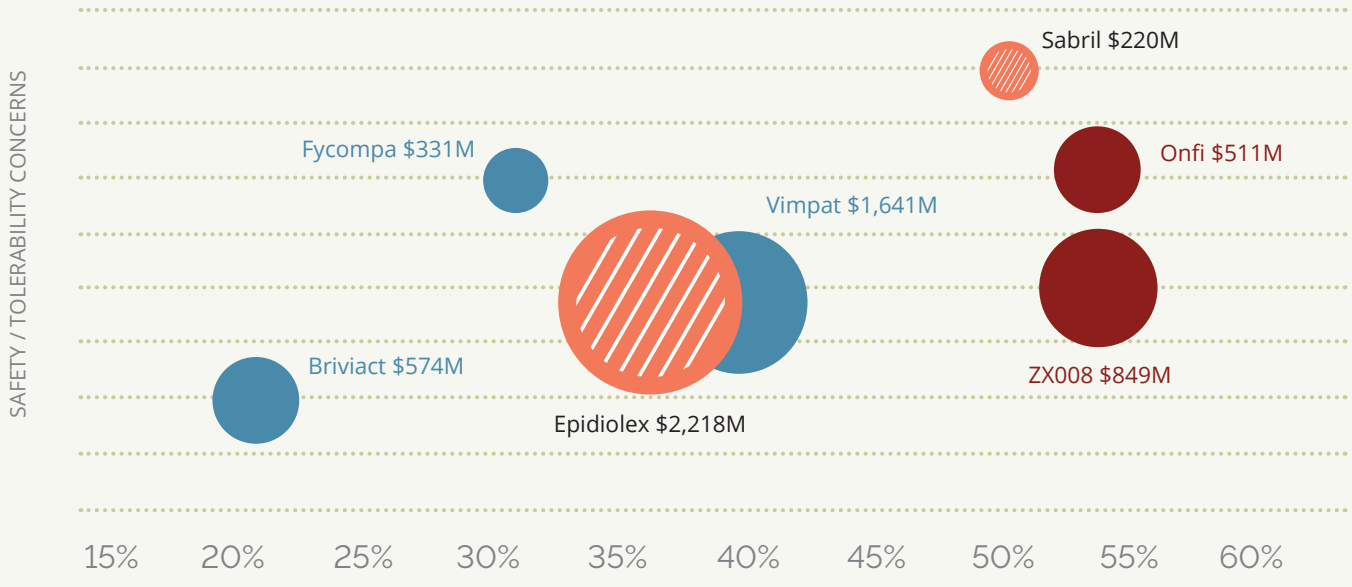
Orphan Vs. Partial Seizure Therapies Peak Sales

PEAK Sales of Select Current-Era AEDs

PRODUCT	GENERIC NAME	MANUFACTURER	APPROVED	PEAK SALES (YEAR)
Briviact	bricaracetam	**UCB**	2016	**$575M (2024)**
Vimpat	lacosamide	**UCB**	2008	**$1643M (2021)**
Epidiolex	cannabidiol	**GW Pharma**	2018	**$2198M (2024)**
ZX008		Zogenix	2019	**$849M (2024)**
Sabril	vigabitrin	Lundbeck	2009	**$230M (2017)**
Onfi	clobazam	Lundbeck	2012	**$510M (2018)**
Fycompa	perampanel	Eisai	2012	**$326M (2024)**

○ Non-orphan Indications ○ Orphan Indications ○ Non-orphan and orphan indications

EFFICACY, SAFETY, AND PEAK SALES of Select Current Era AEDs



● Partial Seizures
● Orphan Indications
● Both

Figure 4 was crafted using reported efficacy data from randomized controlled trials for the x-axis and quantification of safety / tolerability using a blended analysis of (1) proportion of patients experiencing adverse effects defined in randomized controlled trials and (2) FDA-imposed restrictions (i.e., black box warnings) for the y-axis. Then peak sales (or projected peak sales) of specific epilepsy products were recorded and products were colored to depict approval in broad epilepsy indications (green), orphan epilepsy indications (red) or both (checkered red and green).

GW Pharmaceuticals – orphan epilepsy indications as a gateway to broad refractory epilepsy indications

"I've been working with the GW team since they initiated preclinical studies with cannabidiol. Since that time, I've seen tremendous growth in communication and interest from pharma companies, investors, epilepsy communities and physicians."

Physician treating epilepsy patients

Based on randomized, placebo-controlled trials in 1,100 people with Dravet or LGS, GW's Epidiolex has proven capable of reducing median seizure frequency by around 40-50 percent in this patient population.[17] Epidiolex, whose added twist is that it is a plant-based cannabidiol (CBD) – an active ingredient also found in marijuana plants, has also demonstrated long-term safety at 96 weeks. Even though much attention has been focused on the "drug from a drug", these data are compelling and Epidiolex is "safe from a pharmacology perspective, since CBD has no hallucinogenic properties like THC [Tetrahydrocannabinol]," according to an investigator supporting the clinical development.

While GW Pharmaceuticals demonstrated a robust seizure reduction response in this patient population, most of their future revenues are expected to come from the larger drug-resistant epilepsy patient population.

The revenue splits and values are still speculation from the investor class, but interest from the patient and physician community is also very high, in part because the safety profile of Epidiolex shows it well-tolerated as a combination therapy, with few discontinuations in clinical trials. Complementing this interest in broad Epidiolex use, GW Pharmaceuticals announced a price point of $32,500 annually[18] that is significantly lower than comparable orphan drugs developed for similar high-need, niche populations (often priced in the $200,000 – $500,000 range). This deliberate strategy suggests the company expects to expand its market significantly into a larger AED refractory population.

Zogenix – a focused orphan epilepsy indication approach

Unlike GW, Zogenix is focused on developing its therapies exclusively for rare orphan diseases. In July 2018, Zogenix reported an estimated 65 percent reduction in median seizure frequency in 43 patients with Dravet Syndrome treated with their drug, ZX008 (Fenfluramine Hydrochloride) in comparison to placebo patients.[19] Although there are some safety concerns with its active ingredient, Fenfluramine, a serotonergic agent targeting 5-HT2B receptors, similar to the situation with Onfi® and Sabril®, the drug is expected to be used given the severity of Dravet and the paucity of options. Fenfluramine is widely known as part of the discontinued weight-loss combination drug Fen-Phen

"Drug companies are absolutely going for the orphan drug indication. They think that's a business model that's going to work for them, and I've heard rumors that Zogenix, for instance, is going to charge a massive price premium for the product, which would imply to me that they're only putting their eggs in the orphan indication bucket right now." "

Researcher and clinician in epilepsy field

which caused valvulopathy in a broader population using it for weight loss; however, there has been no evidence of valvulopathy during the Dravet trial.

Zogenix has not reported their pricing strategy, but some clinicians interviewed for this analysis anticipate the drug will be costly given its orphan focus and differentiating data.

GW's dual orphan and broad indication focus is projected to generate larger revenues (~$2.5 Billion) than Zogenix's orphan-focus strategy (~$850 Million); based on the assumption that 60% of the revenues are derived from the large partial seizure population. Despite approaching epilepsy markets in different ways, GW Pharmaceuticals and Zogenix both have healthy market caps at $3.4 billion and $1.7 billion, respectively.

Conclusion

These orphan epilepsy-focused companies are tackling an area of unmet need with unique development considerations, historic patient heterogeneity challenges, and an unparalleled basic science uncertainty. Despite advances in our general understanding of the human body and genome, and diverse technologies to prevent, treat and manage many pathophysiologies, epilepsy remains relatively uncharted territory. While many underlying causes are still elusive, our understanding of specific rare variations of epilepsy have recently enabled developers to test novel mechanisms in high-need patient populations like Dravet syndrome and LGS.

New basic and translational research will bring more in-depth understanding of the patients, underlying seizure symptoms, and sources of epilepsy, and as drug developers re-engage with the various epilepsy populations and explore segmented, homogeneous patient populations under the epilepsy umbrella, additional opportunities for clinical and commercial development will emerge in concert.

About
Back Bay Life Science Advisors

Back Bay Life Science Advisors provides integrated strategy consulting and investment banking services to biotech, pharma and medical technology organizations and investors. Our expertise spans stage, sector, and geography, across every therapeutic class, with deep expertise in disorders of the central nervous system, including market and technology analyses of seizure disorders, multiple sclerosis, neurodegenerative diseases and movement disorders, ophthalmological conditions, spinal cord injury, stroke, and neuropsychological disorders.

Combined with our extensive work in rare diseases, Back Bay understands the different clinical issues that determine development feasibility and commercial value. We keep pace as scientific and clinical advances in neuroscience emerge and help our partners understand how cutting-edge technologies such as growth factors, cell therapies, gene editing, and gene therapy can improve outcomes.

Back Bay guides companies through complex financial transactions related to both CNS and rare disease asset development. We excel at gauging the utility and impact of novel therapeutic platforms and helping entrepreneurial companies understand how best to position their platform technology.

Meet our life science experts and sign up for our newsletter at bblsa.com.

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