



**GW Pharmaceuticals and its U.S. Subsidiary Greenwich Biosciences Announce Publication of Groundbreaking Study of Epidiolex® (cannabidiol) in *The New England Journal of Medicine***

- *Pharmaceutical formulation of cannabidiol significantly reduced convulsive seizure frequency in children on multiple anti-epileptic drugs with poor seizure control –*
- *First well-controlled clinical study of cannabidiol in Dravet syndrome, a rare, severe type of epilepsy with no FDA-approved treatments –*

London, UK, May 24, 2017 – GW Pharmaceuticals plc (Nasdaq: GWPH, “GW,” “the Company” or “the Group”), a biopharmaceutical company focused on discovering, developing and commercializing novel therapeutics from its proprietary cannabinoid product platform, along with its U.S. subsidiary Greenwich Biosciences, announced today that *The New England Journal of Medicine* has published results from a Phase 3 study of Epidiolex® (cannabidiol) in children with Dravet syndrome.<sup>1</sup> Epidiolex, GW’s lead product candidate and the potential first in a new category of anti-epileptic drugs, is a liquid formulation of purified, plant-derived cannabidiol (CBD), a non-psychoactive cannabinoid, which is being studied for the treatment of a number of rare, severe pediatric-onset epilepsy disorders. In the study, Epidiolex significantly reduced monthly convulsive seizure frequency compared to placebo in highly treatment-resistant children when added to existing treatment. Treatment with Epidiolex was generally well tolerated, with a safety profile consistent with prior open label experience.

There are currently no treatments approved by the U.S. Food and Drug Administration (FDA) for Dravet syndrome, a rare form of epilepsy associated with a high mortality rate and significant developmental delays. Results from this study represent the only well-controlled clinical evaluation of a cannabinoid medication for this devastating and drug-resistant condition. The New Drug Application for Epidiolex remains on track for submission to the FDA in the middle of 2017.

"Dravet syndrome is one of the most difficult types of epilepsy to treat and many of the children in this study were experiencing dozens, even hundreds, of seizures per month despite taking multiple concurrent anti-epileptic medications," said Orrin Devinsky, M.D., of NYU Langone

Medical Center's Comprehensive Epilepsy Center and lead author of the study. "These results suggest that Epidiolex can provide clinically meaningful benefits and I look forward to the prospect of an appropriately standardized and tested pharmaceutical formulation of cannabidiol available as a treatment option for these patients."

"The publication of these highly-anticipated positive results represents an important milestone for the Dravet syndrome community in that it provides hope that a new treatment option is within sight for this rare and devastating disease," said Nicole Villas, Scientific Director of the Dravet Syndrome Foundation. "As a foundation dedicated to research and patient assistance, we hope the burden of Dravet syndrome on patients and families is recognized and welcome new, well-researched treatments such as this that might help ease the burden."

The study randomized 120 children ages two to 18 years (mean age 9.8), with Dravet syndrome whose seizures were not controlled by their current anti-epileptic regimen, to receive either Epidiolex (20mg/kg/day) or placebo in addition to standard treatment. Conducted in 23 study centers in the United States and Europe, patients in the study had tried a median of four prior anti-epileptic drugs (range 0-26) and were taking a median of three (range 1-5) during the study. At baseline, patients had a median frequency of 13 convulsive seizures per month, with a wide range of 3.7 to 1,717 seizures per month.

Over the 14-week treatment period, patients taking Epidiolex had a significantly greater median reduction in convulsive seizures (39 percent) compared to placebo (13 percent). The estimated median treatment difference between groups was 23 percent ( $p=0.01$ ). The proportion of patients who had a 50 percent or better reduction in convulsive seizure frequency was 43 percent with Epidiolex versus 27 percent with placebo ( $p=0.08$ ). The study also measured improvement on the Caregiver Global Impression of Change (CGIC) scale. Sixty-two percent of patients treated with Epidiolex were rated as improved in overall condition on the CGIC compared to 34 percent of the placebo group ( $p=0.02$ ). Additionally, more patients became seizure-free on Epidiolex than placebo (5 percent vs 0 percent:  $p=0.08$ ) and total monthly seizure count was significantly reduced with Epidiolex ( $p=0.03$ ).

Epidiolex was generally well tolerated in the trial. The most common adverse events (AEs) (>10 percent) were somnolence, diarrhea, decreased appetite, fatigue, vomiting, pyrexia, lethargy, convulsion, upper respiratory tract infection. Of the 93 percent of patients on Epidiolex that experienced an AE, 84 percent reported it to be mild or moderate. Ten patients on Epidiolex

experienced a serious adverse event compared with three patients on placebo. Eight patients on Epidiolex discontinued treatment due to adverse events compared with one patient on placebo. Elevations in liver enzymes occurred in 12 patients taking Epidiolex and one patient taking placebo, all of whom were on concomitant valproic acid. Four of these patients withdrew from the trial, three on Epidiolex and one on placebo. In the remaining nine patients taking Epidiolex, elevations returned to normal while on treatment.

“The publication of results from this landmark study by *The New England Journal of Medicine* and the accompanying editorial commentary<sup>2</sup> highlight the potential of Epidiolex to address the significant unmet need in Dravet syndrome,” said Justin Gover, GW's Chief Executive Officer. “We remain committed to these patients and their families, and are determined to make this important new medicine available to them as quickly as possible.”

### **About Dravet Syndrome**

Dravet syndrome is a severe infantile-onset and highly treatment-resistant epileptic syndrome frequently associated with a genetic mutation in sodium channels. Onset of Dravet syndrome occurs during the first year of life in previously healthy and developmentally normal infants. Initial seizures are often temperature related, severe, and long-lasting. Over time, people with Dravet syndrome can develop multiple types of seizures, including tonic-clonic, myoclonic, and atypical absences and are prone to bouts of prolonged seizures called status epilepticus, which can be life threatening. Risk of premature death including SUDEP (sudden unexpected death in epilepsy) is elevated in people with Dravet syndrome. Additionally, the majority will develop moderate to severe intellectual and development disabilities and require lifelong supervision and care. There are currently no FDA-approved treatments and nearly all patients continue to have uncontrolled seizures and other medical needs throughout their lifetime.

### **About Epidiolex**

Epidiolex, GW's lead cannabinoid product candidate, is a liquid formulation of plant-derived cannabidiol (CBD), which is in development for the treatment of a number of rare childhood-onset epilepsy disorders. GW has conducted extensive pre-clinical research of CBD in epilepsy since 2007. This research has shown that CBD has significant anti-epileptiform and anticonvulsant activity using a variety of *in-vitro* and *in-vivo* models and efficacy in reducing seizures in acute animal models of epilepsy. To date, GW has received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) for Epidiolex for the treatment of Dravet syndrome, Lennox-Gastaut syndrome (LGS), Tuberous Sclerosis Complex (TSC) and Infantile Spasms (IS), each of which are severe infantile-onset, drug-resistant epilepsy syndromes. Additionally, GW has received Fast Track Designation from the FDA for the treatment of Dravet syndrome and Orphan Designation from the European Medicines Agency, or EMA, for Epidiolex for the treatment of Dravet syndrome and LGS. GW is currently evaluating additional clinical development programs in other orphan seizure disorders.

## **About GW Pharmaceuticals plc**

Founded in 1998, GW is a biopharmaceutical company focused on discovering, developing and commercializing novel therapeutics from its proprietary cannabinoid product platform in a broad range of disease areas. GW is advancing an orphan drug program in the field of childhood-onset epilepsy with a focus on Epidiolex<sup>®</sup> (cannabidiol), which is in Phase 3 clinical development for the treatment of Dravet syndrome, Lennox-Gastaut syndrome, Tuberous Sclerosis Complex and Infantile Spasms. GW commercialized the world's first plant-derived cannabinoid prescription drug, Sativex<sup>®</sup> (nabiximols), which is approved for the treatment of spasticity due to multiple sclerosis in 31 countries outside the United States. The Company has a deep pipeline of additional cannabinoid product candidates which includes compounds in Phase 1 and 2 trials for glioma, schizophrenia and epilepsy. In the United States, GW operates through its subsidiary Greenwich Biosciences, Inc. For further information, please visit [www.gwpharm.com](http://www.gwpharm.com).

## **Forward-looking statements**

*This news release contains forward-looking statements that reflect GW's current expectations regarding future events, including statements regarding financial performance, the timing of clinical trials, the timing and outcomes of regulatory or intellectual property decisions, the relevance of GW products commercially available and in development, the clinical benefits of Epidiolex<sup>®</sup> and the safety profile and commercial potential of Epidiolex. Forward-looking statements involve risks and uncertainties. Actual events could differ materially from those projected herein and depend on a number of factors, including (inter alia), the success of GW's research strategies, the applicability of the discoveries made therein, the successful and timely completion of uncertainties related to the regulatory process, and the acceptance of Epidiolex and other products by consumer and medical professionals. A further list and description of risks and uncertainties associated with an investment in GW can be found in GW's filings with the U.S. Securities and Exchange Commission, including the most recent Form 20-F filed on 5 December 2016. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. GW undertakes no obligation to update or revise the information contained in this press release, whether as a result of new information, future events or circumstances or otherwise.*

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<sup>1</sup> Devinsky O, Cross JH, Laux L, et al. Trial of cannabidiol for drug-resistant seizures in the Dravet syndrome. *N Engl J Med* 2017; 376;2011-20.

<sup>2</sup> Berkovic, SF. Editorial: Cannabinoids for epilepsy – real data, at last. *N Engl J Med* 2017; 376;2075-76.