UCB News

FDA Accepts New Drug Application (NDA) to review Midazolam Nasal Spray, an investigational product for the acute treatment of seizure clusters

- **Midazolam Nasal Spray has been granted orphan drug designation by the United States Food and Drug Administration (US FDA) for the rescue treatment of seizures in patients who require control of intermittent bouts of increased seizure activity (e.g. seizure clusters, acute repetitive seizures)**

- **Midazolam Nasal Spray has also been granted Fast Track designation by the US FDA due to the high unmet need for patients and caregivers**

Brussels (Belgium) & Atlanta Georgia, August 13th 07:00 (CEST): UCB today announced that the U.S. Food and Drug Administration (FDA) has accepted the filing of a New Drug Application (NDA) for midazolam nasal spray*, an investigational product for the acute treatment of seizures in patients who require control of intermittent bouts of increased seizure activity (e.g. seizure clusters, acute repetitive seizures).

The application is supported by data from a Phase 3 clinical study (ARTEMIS 1 -**A**cute **R**escue **T**herapy in **E**pilepsy with **M**idazolam **I**ntranasal **S**pray), which evaluated the safety and efficacy of midazolam nasal spray in 292 patients.[1]

"Managing seizure clusters remains a challenge for thousands of patients and caregivers, in the US and beyond, who live their lives each day with this debilitating condition," explained Jeff Wren, Head of Neurology and Executive Vice-President at UCB*. "There is an unmet need for effective and convenient acute treatment of seizure clusters that can rapidly end ongoing seizures and potentially prevent or delay their reoccurrence.*

* Midazolam Nasal Spray has not been approved by the FDA. These statements solely reflect the opinions of the authors

Seizure Clusters are unpredictable, even when a patient is compliant with their current anti-epileptic drugs. When it comes to managing seizure clusters, it is important that patients have an acute care plan that includes access to a treatment they can take anytime or anywhere.

"With midazolam nasal spray, UCB hopes to expand and diversify the treatment choices we provide to the epilepsy community, complementing our already strong epilepsy portfolio and providing additional solutions to help patients." said Jeff Wren.

UCB estimates that more than 150,000 people in the U.S. with refractory epilepsy also experience seizure clusters.[2,3,4] These types of seizures pose multiple risks to patients, including repeated emergency room visits and related hospitalizations each year.

Midazolam nasal spray has been granted both orphan drug and fast track designations by the FDA, reflecting the significant unmet need which currently exists for acute care of seizure clusters.

The acceptance of this NDA could result in midazolam nasal spray being approved in the U.S. as an acute treatment for increased seizure activities in early 2019. If approved, midazolam nasal spray will be the first new medication approved to treat seizure clusters in more than 17 years.

UCB acquired midazolam nasal spray from Proximagen in June 2018. Both companies have collaborated to quickly progress the NDA filing. This reflects and reinforces the commitment of both companies to making their portfolios of development medicines available to as many patients who could benefit from them as quickly as possible.

About FDA Fast Track Designation:

FDA Fast Track Designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need, with the aim of getting important new medicines to patients more quickly. To be eligible for Fast Track designation, a drug must show some advantage over available therapy.

Fast Track designation provides many favorable elements which can help expedite a medicine's review process. These can include more frequent meetings with FDA to discuss a drug's development plan and ensure collection of appropriate data needed to support drug approval, more frequent written communication from FDA relating to the design of the proposed clinical trials and use of biomarkers, and Rolling Review – allowing a company to submit completed sections of its

NDA for review by FDA, rather than waiting until every section of the NDA is completed before the entire application can be reviewed.

About UCB in Epilepsy:

UCB has a rich heritage in epilepsy with over 20 years of experience in the research and development of antiepileptic drugs. As a company with a long-term commitment to epilepsy research, our goal is to address unmet medical needs. Our scientists are proud to contribute to advances in the understanding of epilepsy and its treatment. We partner and create super-networks with world-leading scientists and clinicians in academic institutions, pharmaceutical companies and other organizations who share our goals. At UCB, we are inspired by patients, and driven by science in our commitment to support patients with epilepsy.

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About UCB

UCB, Brussels, Belgium (www.ucb.com) is a global biopharmaceutical company focused on the discovery and development of innovative medicines and solutions to transform the lives of people living with severe diseases in immunology and neurology. With around 7,500 people operating in 40 countries, the company generated revenue of €4.5 billion in 2017. UCB is listed on Euronext Brussels (symbol: UCB). Follow us on Twitter: @UCB_news

About Proximagen

Proximagen LLC, a member of the ACOVA family of companies located in Plymouth MN (USA), specializes in the development of novel small molecule therapeutics in the areas of CNS, pain and inflammation. Proximagen has a long history of drug development, as part of the Upsher-Smith Laboratories family of companies prior to the sale of its generics business in 2017.

Forward looking statements

This press release contains forward-looking statements based on current plans, estimates and beliefs of management. All statements, other than statements of historical fact, are statements that could be deemed forward-looking statements, including estimates of revenues, operating margins, capital expenditures, cash, other financial information, expected legal, political, regulatory or clinical results and other such estimates and results. By their nature, such forward-looking statements are not guarantees of future performance and are subject to risks, uncertainties and assumptions which could cause actual results to differ materially from those that may be implied by such forward-looking statements contained in this press release. Important factors that could result in such differences include: changes in general economic, business and competitive conditions, the inability to obtain necessary regulatory approvals or to obtain them on acceptable terms, costs associated with research and development, changes in the prospects for products in the pipeline or under development by UCB, effects of future judicial decisions or governmental investigations, product liability claims, challenges to patent protection for products or product candidates, changes in laws or regulations, exchange rate fluctuations, changes or uncertainties in tax laws or the administration of such laws and hiring and retention of its employees.

Additionally, information contained in this document shall not constitute an offer to sell or the solicitation of an offer to buy any securities, nor shall there be any offer, solicitation or sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to the registration or qualification under the securities laws of such jurisdiction. UCB is providing this information as of the date of this document and expressly disclaims any duty to update any information contained in this press release, either to confirm the actual results or to report a change in its expectations.

There is no guarantee that new product candidates in the pipeline will progress to product approval or that new indications for existing products will be developed and approved. Products or potential products which are the subject of partnerships, joint ventures or licensing collaborations may be subject to differences between the partners. Also, UCB or others could discover safety, side effects or manufacturing problems with its products after they are marketed.
Moreover, sales may be impacted by international and domestic trends toward managed care and healthcare cost containment and the reimbursement policies imposed by third-party payers as well as legislation affecting biopharmaceutical pricing and reimbursement.

References:

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2. Jafarpour S et al. (*in press*). Seizure cluster: Definition, prevalence, consequences, and management. Seizure: Eur J Epilepsy (2018), https://doi.org/10.1016/j.seizure.2018.05.013.
3. A Komaragiri et al. Seizure clusters: A common, understudied and undertreated phenomenon in refractory epilepsy. Epilepsy & Behavior 59 (2016) 83–86
4. Acute Repetitive Seizures. Decision Base Report 2015. *Data on file.*