



UCB provides an update on Phase 2b padsevonil safety and efficacy study in epilepsy (ARISE)

- **ARISE did not reach statistical significance for either of the two primary endpoints**
- **UCB will further analyse data to better understand results and their implications**

BRUSSELS, BELGIUM (13 March 2020, 7am CET) – regulated information - Inside Information - UCB, a global biopharmaceutical company, today announced top-line results from ARISE (NCT03373383), the first of two adequate and well-controlled studies, investigating the efficacy and safety of padsevonil for the treatment of observable focal-onset seizures in adults with drug-resistant epilepsy.

This randomized, double-blind, placebo-controlled Phase 2b study did not reach statistical significance for either of the primary endpoints, change from Baseline in observable focal-onset seizure frequency and 75 percent responder rate for padsevonil compared with placebo over the 12-week maintenance period.

Padsevonil was generally well-tolerated and its safety profile was consistent with that seen in earlier studies.

“As many as one third of people living with focal-onset seizures are unable to effectively manage seizures using currently available anti-epileptic drugs which represents a significant unmet need,” explained Prof Dr Iris Loew-Friedrich, Head of Drug Development and Chief Medical Officer, UCB. *“It is disappointing that, with ARISE, we did not achieve statistically significant improvements for these patients. We will further analyse the data over the coming weeks to better understand the results and their implications for the global epilepsy community. Building on our existing heritage in neurology, and the differentiated patient value we already provide to people living with epilepsy around the world, UCB remains committed to leadership in this patient population, and to delivering new and innovative solutions to help them live their lives at their ideal.”*

In addition to padsevonil, UCB has a very robust portfolio with potential to launch multiple, differentiated products by 2025 to create patient value for specific populations now and into the future.

ARISE is the first of two adequate and well-controlled studies which seek to investigate the safety and efficacy of UCB's padsevonil in the treatment of focal-onset seizures in patients with highly treatment-resistant epilepsy.

The impact of the ARISE results on the second, ongoing study, UCB0092 (DUET), is currently being evaluated.

About the ARISE Study

ARISE is a Phase 2b randomized, double-blind, placebo-controlled, dose finding study to evaluate the efficacy and safety of padsevonil as adjunctive treatment of focal-onset seizures in adults with drug-resistant epilepsy. Four hundred and eleven patients were assigned to one of four groups with different dosing regimens. The primary endpoints were the change from Baseline in observable focal-onset seizure frequency and the 75 percent responder rate for padsevonil compared with placebo over the 12-week Maintenance Period. The study enrolled adults with drug-resistant focal epilepsy that had not been controlled with four or more anti-epileptic drugs (AEDs), and who were currently being treated with between one and three AEDs.

About Padsevonil

Padsevonil is a UCB development medicine, discovered at the company's scientific campus in Braine l'Alleud, Belgium.

Padsevonil is a dual-acting antiepileptic drug that has a mechanism of action designed specifically to interact with pre- and post-synaptic targets. Presynaptically, it binds with high affinity to the three isoforms of synaptic vesicle protein 2: SV2A,

SV2B and SV2C.¹ Postsynaptically, it binds with moderate affinity to the benzodiazepine recognition site on the GABAA receptor, where it acts as a partial agonist.^{1,2} Previous results of a Phase II proof-of-concept study demonstrated that treatment with padsevonil may be associated with a clinically meaningful reduction in focal seizure frequency and suggested an acceptable tolerability/safety profile.³

About Epilepsy

Epilepsy is the main symptom of a variety of chronic disorders of the brain. It is the fourth most common neurological condition worldwide and affects approximately 65 million people.⁴ Anyone can develop epilepsy; it occurs across all ages, races and genders, and is defined as one or more unprovoked epileptic seizures with a risk of further seizures.⁵

About UCB in Epilepsy

UCB has a rich heritage in epilepsy with over 30 years of experience in the research and development of anti-epileptic 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.

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 of the immune system or of the central nervous system. With more than 7 600 people in approximately 40 countries, the company generated revenue of € 4.9 billion in 2019. UCB is listed on Euronext Brussels (symbol: UCB). Follow us on Twitter: @UCB_news

For further information, UCB:

Corporate Communications

Laurent Schots
Media Relations, UCB
T+32.2.559.92.64 Laurent.schots@ucb.com

Investor Relations

Antje Witte
Investor Relations, UCB
T +32.2.559.94.14 antje.witte@ucb.com

Isabelle Ghellynck,
Investor Relations, UCB
T+32.2.559.9588, isabelle.ghellynck@ucb.com

Brand Communications

Jim Baxter,
Neurology Communications, UCB
T+32.2.473.78.85.01 jim.baxter@ucb.com

¹ Wood M, et al. *American Epilepsy Society 71st Annual Meeting 2017*; poster 1.271.

² Wolff C, et al. *American Epilepsy Society 71st Annual Meeting 2017*; poster 1.270.

³ Muglia P, et al. *American Epilepsy Society 71st Annual Meeting 2017*; poster 1.283.

⁴ Epilepsy Foundation. Who gets epilepsy? <https://www.epilepsy.com/learn/about-epilepsy-basics/what-epilepsy> date accessed 12th March 2020

⁵ International League Against Epilepsy. Definition of Epilepsy 2014. <https://www.ilae.org/guidelines/definition-and-classification/definition-of-epilepsy-2014> date accessed 12th March 2020