Press release

Sciensus partners with Sentynl Therapeutics to become the exclusive distribution partner of NULIBRY® (fosdenopterin) in Europe

Sciensus, a life sciences business, specialising in patient access, engagement and insight solutions is pleased to announce that it has reached an agreement to become the exclusive distribution partner for Sentynl's drug NULIBRY in Europe. NULIBRY is the only medicine currently available for treatment for patients with molybdenum cofactor deficiency (MoCD) Type A, an ultra-rare, life-threatening genetic disorder known to impact fewer than 150 patients globally.

Sciensus will deliver both an early access programme and distribution services to support the market access and reimbursement processes of NULIBRY in Europe. NULIBRY currently holds an exceptional circumstances approval for marketing authorisation in Europe and requires a non-interventional post-authorisation safety study. Sciensus will be providing a digital and clinician-led service to support the development and capture of this critical and voluntary real-world data.

Sciensus has over 30 years of experience in supporting patients getting access to orphan drugs. Its global network is dedicated to accelerating this vital process – from early access all the way to full commercialisation, including patient support programs.

Darryn Gibson, CEO of Sciensus, said: "We are pleased to be partnering with Sentynl to provide programmes which will offer patients in Europe access to potentially-life saving medicines. Our exceptional experience and patient insights to support real world data allows us to deliver tailored solutions, better patient engagement and support, leading to more effective treatments, and elevated health outcomes for patients."

"In partnership with Sciensus, we are proud to extend the reach of this innovative treatment to meet the needs of patients with MoCD Type A in Europe," said Matt Heck, President & Chief Executive Officer of Sentynl. "This partnership marks another step forward in our mission to expand patient access to life-changing rare disease treatment."

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Notes to Editors:

For more information, please contact Sciensus@teneo.com

About Sciensus:

Sciensus is a life sciences business specialising in patient access, engagement and insight solutions. Every day we learn from patients and - together with our partners

like pharmaceutical companies, the NHS and private providers - we use these learnings to develop the best approaches and support to help them get the most out of their therapy.

Whether it's about rare and orphan diseases, cancer or other life changing conditions - we provide bespoke end-to-end services built on exceptional insight and experience in our key markets to connect pharmaceutical companies, insurers and healthcare professionals to patients.

So, whilst medicine is about science, these 70 million patient interactions across the UK and Europe have taught us that, at its best, it must firstly be about the science of people.

About Sentynl Therapeutics

Sentynl Therapeutics is a U.S.-based biopharmaceutical company focused on bringing innovative therapies to patients living with rare diseases. The company was acquired by the Zydus Group in 2017. Sentynl's experienced management team has previously built multiple successful pharmaceutical companies. With a focus on commercialization, Sentynl looks to source effective and well-differentiated products across a broad spectrum of therapeutic areas to address unmet needs. Sentynl is committed to the highest ethical standards and compliance with all applicable laws, regulations and industry guidelines. For more information, visit https://sentynl.com.

About Molybdenum Cofactor Deficiency (MoCD) Type A

MoCD Type A is an autosomal recessive, inborn error of metabolism caused by mutations in the molybdenum cofactor synthesis 1 gene and characterized by a deficiency in molybdenum cofactor production, leading to a lack of molybdenum-dependent enzyme activity.[1,2] The lack of activity leads to decreased sulfite oxidase activity with buildup of sulfite and secondary metabolites (such as S-sulfocysteine) in the brain, which causes irreversible neurological damage.[2]

MoCD Type A is an ultra-rare disease. The estimated prevalence of MoCD Type A in the European Union is 0.005 per 10,000. Based on these estimates, MoCD Type A is likely to be underdiagnosed.

The most common presenting symptoms of MoCD Type A are seizures, feeding difficulties and encephalopathy. Patients with MoCD Type A who survive beyond infancy typically suffer from progressive brain damage, which presents in characteristic patterns on magnetic resonance imaging (MRI). This damage leads to severe psychomotor impairment and an inability to make coordinated movements or communicate with their environment.

References
1 Mechler K et al. Genet Med. 2015;17(12):965-970.
2 Schwarz G. Cur Op in Che Bio. 2016;31:179-187.

About NULIBRY®

NULIBRY (fosdenopterin) for Injection is a substrate replacement therapy that

provides a synthetic source of cPMP, which is converted to molybdopterin. Molybdopterin is then converted to molybdenum cofactor, which is needed for the activation of molybdenum-dependent enzymes, including sulfite oxidase, an enzyme that reduces levels of neurotoxic sulfites. NULIBRY was approved by the U.S. FDA in February 2021, and by the Israel Ministry of Health in July 2022, with an indication to reduce the risk of mortality in patients with MoCD Type A. NULIBRY was also approved by the EMA in September 2022, with an indication for treatment of patients with MoCD Type A. Please see full Prescribing Information for NULIBRY.

Important Safety Information

Warnings and Precautions

Potential for Photosensitivity

NULIBRY can make the patient oversensitive to sunlight. NULIBRY-treated patients or their caregivers are advised to avoid or minimize patient exposure to sunlight and artificial UV light and adopt precautionary measures when exposed to the sun, including wearing protective clothing and sunglasses, and use broad-spectrum sunscreen with high SPF in patients 6 months of age and older. If photosensitivity occurs, caregivers/patients are advised to seek medical attention immediately and consider a dermatological evaluation.

Adverse Reactions

The most common adverse reactions in NULIBRY-treated patients were infusion catheter–related complications (89%), pyrexia (fever) (78%), viral infection (56%), pneumonia (44%), otitis media (ear infection) (44%), vomiting (44%), cough/sneezing (44%), viral upper respiratory infection (33%), gastroenteritis (33%), diarrhea (33%) , and bacteremia (33%). Adverse reactions for rcPMP-treated patients were similar to the NULIBRY-treated patients.

Patient Counseling Information

Please read the FDA-approved NULIBRY Prescribing Information and Instructions for Use and follow the instructions on how to prepare and administer NULIBRY.

NULIBRY has a potential for photosensitivity; see Warnings and Precautions. Seek medical attention immediately if the patient develops a rash or if they notice symptoms of photosensitivity reactions (redness, burning sensation of the skin, blisters).

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

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