FDA approves DIACOMIT (Stiripentol) for the treatment of seizures associated with Dravet syndrome (DS) in patients 2 years of age and older taking clobazam

BIOCODEX SAS (Gentilly – France) is pleased to announce that on August 20th 2018, the Food and Drug Administration (FDA) has approved DIACOMIT (stiripentol) for the treatment of seizures associated with Dravet syndrome (DS) in patients 2 years of age and older taking clobazam. DIACOMIT is expected to be available in U.S. pharmacies in early January 2019.

"The FDA's approval of DIACOMIT is a major milestone for Biocodex. It recognizes our R&D excellence in developing therapies for Dravet syndrome and brings a new treatment option to young patients in the USA," said Jean-Marie Lefèvre, President and CEO of Biocodex. "Diacomit is the result of Biocodex's own research and has been the subject of substantial investment. Our research work, along with the pre-clinical and clinical studies carried out in close collaboration with teams of university experts from around the world, have enabled us to obtain approval in Europe, Japan, Canada and, as of now, in the United States. Our efforts will not stop there. We are continuing our research on stiripentol in order to do everything in our power to improve treatment for children and adults suffering from pharmacoresistant epilepsy."

The FDA approval of DIACOMIT was based on two multicenter placebo-controlled trials similar in terms of disease characteristics and prior treatment of patients, STICLO France and STICLO Italy The primary efficacy endpoint in both trials was the responder rate, with a responder defined as a patient who experienced a greater than 50% decrease in the frequency (per 30 days) of generalized clonic or tonic-clonic seizures during the double-blind treatment period compared to the 4-week baseline period. In STICLO France, the responder rate for patients receiving DIACOMIT was 71% [95% CI: 52% – 91%], compared to 5% [95% CI: 0% – 15%] for patients receiving placebo. In STICLO Italy, the responder rate for patients receiving DIACOMIT was 67% [95% CI: 40% – 93%], compared to 9.1% [95% CI: 0% – 26%] for patients receiving placebo. The most common adverse reactions, occurring in at least 10% of DIACOMIT-treated patients and more frequently than on placebo, included somnolence (67%), decreased appetite (45%), agitation (27%), ataxia (27%), weight decreased (27%), hypotonia (24%), nausea (15%), tremor (15%), dysarthria (12%), and insomnia (12%). There were 2 patients in whom adverse reactions led to discontinuation of DIACOMIT treatment: one patient had an adverse reaction of status epilepticus; the second patient had drowsiness, balance impaired and sialorrhea.

About Dravet Syndrome

Dravet syndrome, also known as severe myoclonic epilepsy in infancy (SMEI), is a catastrophic early onset epileptic syndrome that is thought to affect approximately 2,000 to 8,000 patients in the U.S. Dravet syndrome is characterized by severe epilepsy, psychomotor retardation, and often ataxia. In most cases, the first seizures occur during the first year of life. Status epilepticus is frequent and is thought to be in part responsible for the high mortality rate reported in these patients, ranging from 15.9 to 18%.

About DIACOMIT (stiripentol)

DIACOMIT is a new molecular entity developed by Laboratories Biocodex. Possible mechanisms of action of its anticonvulsant effect in humans include direct effects mediated through the $GABA_A$ receptor and indirect effects involving inhibition of cytochrome P450 activity with resulting increase in blood levels of clobazam and its active metabolite. DIACOMIT is now approved by FDA in two formulations (capsules and powder for oral suspension).

DIACOMIT is approved for adjunctive treatment with clobazam and valproate in Dravet syndrome in 27 countries in the EU (January 2007), Canada (December 2012), and Japan (September 2012).

Biocodex received orphan-drug designation for DIACOMIT from the FDA in 2008.

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About Biocodex

Biocodex is an independent multinational pharmaceutical company founded in France in 1953. It is headquartered in Gentilly (France) with its American subsidiary in Redwood City (CA). The company is focused on discovering, developing and commercializing innovative therapeutics relating to the central nervous system (epilepsy, pain management, psychiatry) and gastroenterology (microbiota).

Biocodex has its own research and development center with a diverse team of highly experienced scientific researchers. Stiripentol is a new antiepileptic drug developed at the Biocodex research center.

The Biocodex scientific teams also work in partnership with leading universities and research organizations around the world and are consistently involved in some of the most forward-looking aspects of medical research. Biocodex has been working with healthcare professionals for more than 60 years to fulfil its mission of developing effective long-term solutions to health problems. Biocodex is now a worldwide company which is engaged in research and development, which manufactures and markets its own products, and operates in more than 115 countries through 18 fully owned subsidiaries and a network of distributors and local partners. The company employs more than 1,200 people worldwide.

Its manufacturing site is a state-of-the-art facility located in Beauvais, 50 miles north of Paris.