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Genkyotex's setanaxib granted Orphan Drug Designation by the US FDA for the treatment of PBC

Genkyotex (Euronext Paris & Brussels: FR0013399474 – GKTIX), a biopharmaceutical company and leader in NOX therapies, today announced that its lead drug candidate, setanaxib, has been granted orphan drug designation (ODD) by the US Food and Drug Administration (FDA) for the treatment of primary biliary cholangitis (PBC).

Elias Papatheodorou, CEO of Genkyotex, comments: *“Obtaining the ODD from the FDA is of strategic importance for the development of setanaxib in PBC. The FDA and the European Medicines Agency (EMA) have also granted ODD for setanaxib in the treatment of idiopathic pulmonary fibrosis and systemic sclerosis, highlighting the compound’s therapeutic potential in multiple fibrotic disorders with high unmet needs.”*

Orphan Drug Designation is granted by the FDA to drugs or biological products intended for the safe and effective treatment of rare diseases with an unmet medical need, affecting fewer than 200,000 people in the United States. Among other benefits beyond the clinical development phases of the product, ODD provides 7 years of market exclusivity after obtaining marketing approval in the United States. In addition, in certain cases, it may provide tax credits on clinical research expenses, an accelerated registration procedure, technical assistance to complete the registration file as well as exemptions from filing fees with the regulatory agencies.

The company is currently discussing its registration strategy for setanaxib in PBC with the FDA and the EMA. Genkyotex will provide an outline of its late stage development plan once final approval of a common registration strategy has been obtained from these regulatory agencies.

Next financial press release:

Q3 2020 business update and cash position: October 22, 2020 (after market)

About Genkyotex

Genkyotex is the leading biopharmaceutical company in NOX therapies, listed on the Euronext Paris and Euronext Brussels markets. Its unique platform enables the identification of orally available small-molecules which selectively inhibit specific NOX enzymes that amplify multiple disease processes such as fibrosis, inflammation, pain processing, cancer development, and neurodegeneration. Genkyotex is developing a pipeline of first-in-class product candidates targeting one or multiple NOX enzymes. The lead product candidate, setanaxib (GKT831), a NOX1 and NOX4 inhibitor has shown evidence of anti-fibrotic activity in a Phase II clinical trial in primary biliary cholangitis (PBC, a fibrotic orphan disease). Based on its positive Phase II results, a phase 3 trial with setanaxib in PBC is being planned. Setanaxib is also being evaluated in an investigator-initiated Phase II clinical trial in Type 1 Diabetes and Kidney Disease (DKD). A grant from the United States National Institutes of Health (NIH) of \$8.9 million was awarded to Professor Victor Thannickal at the University of Alabama at Birmingham (UAB) to fund a multi-year research program evaluating the role of NOX enzymes in idiopathic pulmonary fibrosis (IPF), a chronic lung disease that results in fibrosis of the lungs. The core component of this program is a Phase 2 trial with setanaxib in patients suffering from IPF for which the first patient has been enrolled in September 2020. This product candidate may also be active in other fibrotic indications.

Genkyotex also has a versatile platform well-suited to the development of various immunotherapies (Vaxiclase). A partnership covering the use of Vaxiclase as an antigen per se (GTL003) has been established with Serum Institute of India Private Ltd (Serum Institute), the world's largest producer of vaccine doses, for the development by Serum Institute of cellular multivalent combination vaccines against a variety of infectious diseases.

For further information, please go to www.genkyotex.com

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