Genoscience Pharma receives FDA Orphan Drug Designation for ezurpimtrostat to treat HepatoCellular Carcinoma (HCC)

Ezurpimtrostat could meet important unmet need in HCC, bringing new treatment options to patients worldwide

FDA designation significant milestone following launch of phase 2b ABE-Liver trial

Marseille, France, February 1, 2023 – Genoscience Pharma, a clinical-stage biotech company developing unique lysosomotropic drug candidates for the treatment of cancer, fibrosis and auto-immune diseases through autophagy modulation, today announces that its lead candidate, ezurpimtrostat, a PPT-1 (Palmitoyl Protein Thioesterase-1) inhibitor, has been granted Orphan Drug Designation (ODD) by the US Food and Drug Administration (FDA) for the treatment of HepatoCellular Carcinoma (HCC). This is an important milestone in the development of ezurpimtrostat, as well as for patients. ODD qualifies ezurpimtrostat for a potential seven years of market exclusivity after approval.

The FDA's ODD program provides orphan status to drugs and biologics intended for the treatment, prevention or diagnosis of a rare disease or condition; those affecting less than 200,000 people in the US. 35,563 new cases of primary liver cancer were reported in 2019 and 27,958 people died. Liver cancer is the sixth most common cancer worldwide and the third leading cause of cancer-related death.[1]

Without treatment, HCC is rapidly fatal. In the disease's untreated progression, the median survival time for patients with advanced HCC ranges from four to eight months. The approved combination of atezolizumab and bevacizumab has more than doubled this life expectancy and improved the patient-reported outcome. However, progression-free survival remains short and new treatment options are needed.

Ezurpimtrostat (GNS561) is a first-in-class, first-in-human autophagy inhibitor whose anticancer activity is linked to PPT-1 inhibition. It displayed high liver tropism and potent anti-tumor activity against a panel of human cancer cell lines and in HCC *in vivo* models – alone and in combination with immune checkpoint inhibitors. Recent investigations show that autophagy inhibitors in combination with immune checkpoint inhibitors provide opportunities for enhancing anti-tumor activity. Preliminary data from a phase 1b trial on primary and secondary liver tumors has confirmed that administration of ezurpimtrostat as a monotherapy is both feasible and well tolerated.

The drug candidate is currently being trialed, as a first-line treatment in combination with an anti-PDL1 and an anti-angiogenic, in ABE-Liver, a phase 2b clinical trial sponsored by Grenoble University Hospital (France), which will enroll up to 196 patients.

[1] Centers for Disease Control and Prevention: United States Cancer Statistics, 2019.

"FDA Orphan Drug Designation is a significant milestone for both Genoscience and for our product, ezurpimtrostat. It recognizes that our treatment has the potential to improve the lives of individuals living with HCC," said Professor Philippe Halfon, CEO of Genoscience Pharma. "We have recently launched our phase 2b clinical trial using ezurpimtrostat in conjunction with the standard atezolizumab/bevacizumab treatment. We are looking forward to sharing the intermediate results in 2024."

About Orphan Drug Designation
The FDA's Orphan Drug Designation program provides orphan status to drugs and biologics intended for the treatment, prevention or diagnosis of a rare disease or condition; those that affect less than 200,000 people in the US or meet the cost recovery provisions of the act. Orphan designation qualifies the sponsor of the drug for the various development incentives in the Orphan Drug Act, including tax credits for qualified clinical testing. In addition, it provides seven years of marketing exclusivity upon regulatory approval of the drug in the orphan designation indication.
www.fda.gov

About Genoscience Pharma
Genoscience Pharma is a French clinical-stage biotechnology company developing novel lysosomotropic treatments to establish a new standard of care in cancer, fibrosis and autoimmune diseases. Its lead candidate, GNS561/ezurpimtrostat, is a best-in-class drug candidate that has entered phase 2b clinical trials. It works by attacking cancer cells through autophagy modulation. Genoscience Pharma is developing other molecules in its portfolio for use in oncology and fibrosis indications.
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