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CHMP ISSUES POSITIVE OPINION FOR FUTIBATINIB FOR THE TREATMENT OF ADULTS WITH CHOLANGIOCARCINOMA

ZUG, Switzerland,27 April 2023– Taiho Oncology Europe GmbH and Taiho Pharmaceutical Co., Ltd. announced today that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has issued a positive opinion recommending the conditional marketing authorization (CMA) of futibatinib for the treatment of adult patients with locally advanced or metastatic cholangiocarcinoma (CCA) with a fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement that have progressed after at least one prior line of systemic therapy.

The CHMP's opinion to recommend the use of futibatinib is now being reviewed by the full EMA. Final marketing authorizations for medical products in the European Union (EU) rest with the European Commission.

If approved, futibatinib will be the first irreversibly binding FGFR-inhibitor in the EU for use in the treatment of patients with CCA and will be commercialized under the brand name LYTGOBI® (pronounced "light-GOH-bee").

Each year, approximately 6.000-8.000 individuals in Europe are diagnosed with CCA[1], a rare cancer of the bile ducts of the liver, and approximately 0,3-6 people per 100.000 individuals live with CCA worldwide[2].

"CCA is an aggressive cancer, and the current five-year survival rate is very poor, which underscores the need for new treatment options," said Peter Foertig, MD, Vice President, Medical Affairs, Taiho Oncology Europe. "We look forward to working with the European Medicines Agency as it reviews the application for marketing authorization."

The positive CHMP opinion on futibatinib is based on data from the pivotal Phase 2 FOENIX*-CCA2 trial in 103 patients with locally advanced or metastatic unresectable intrahepatic (inside the liver) CCA, harboring FGFR2 fusions or rearrangements who had received one or more prior lines of systemic therapy. Patients in the trial received futibatinib 20 mg once daily until disease progression or unacceptable toxicity. The trial's primary endpoint was an objective response rate (ORR), which was 42% as assessed by independent central review. The key secondary endpoint of duration of response (DOR) demonstrated a median of 9.7 months (72% of responses ≥6 months). The most common treatment-related adverse events in the trial were hyperphosphatemia (85%), alopecia (33%), dry mouth (30%), diarrhea (28%), dry skin (27%) and fatigue (25%).

Results from the trial were published in the January 19, 2023, issue of the *New England Journal of Medicine*[3]*.*

“As someone who treats patients with CCA, I am encouraged by these data and the positive news from the CHMP,” said Professor Arndt Vogel, MD, Senior Consultant, Department of Gastroenterology, Hepatology and Endocrinology, Hannover Medical School, Germany. “The study findings underscore the importance of molecular profiling for patients with CCA and other diseases whenever possible to potentially help improve outcomes. Indeed, the ESMO Clinical Practice Guidelines[4] recommend FGFR inhibitors for the treatment of patients with CCA and FGFR2 fusions who have progressed after one or more prior lines of systemic therapy.”

Takeshi Sagara, PhD, Managing Director, Clinical Development and Medical Affairs, Discovery and Preclinical Research at Taiho Pharmaceutical, added, “The global Taiho group will continue its efforts to bring futibatinib as a new treatment option to patients with CCA around the world, and the positive CHMP opinion represents a big step for Taiho in Europe to deliver an innovative drug to help patients with CCA in their journey.”

About Futibatinib
Futibatinib (TAS-120) is an oral, potent, selective, and irreversible tyrosine kinase inhibitor of FGFR1, 2, 3 and 4 being studied as a potential treatment for patients with advanced solid tumors with FGFR1-4 genetic aberrations, including cholangiocarcinoma, who were previously treated with chemotherapy or other therapies. Futibatinib covalently binds to the ATP binding pocket of FGFR1-4 resulting in the inhibition of FGFR-mediated signal transduction pathways, reduced tumor cell proliferation in tumors with FGFR1-4 genetic aberrations.

About Taiho Oncology Europe
The mission of Taiho Oncology Europe is to improve the lives of patients with cancer, their families, and their caregivers. The company specializes in orally administered anti-cancer agents and has a growing pipeline of selectively targeted anti-cancer agents. Taiho Oncology Europe GmbH (Zug, Switzerland) is the European subsidiary of Taiho Pharmaceutical Co., Ltd. (Tokyo, Japan). For more information, visit www.taihooncology.eu

About Taiho Pharmaceutical Co., Ltd.
Taiho Pharmaceutical Co., Ltd., a subsidiary of Otsuka Holdings Co., Ltd., is an R&D-driven specialty pharma company with a focus on oncology. Taiho Pharmaceutical also has development programs in allergy and immunology, urology, and consumer healthcare products. Our corporate philosophy takes the form of a pledge: “We strive to improve human health and contribute to a society enriched by smiles.” For more information about Taiho Pharmaceutical Co., Ltd., please visit: https://www.taiho.co.jp/en/

*The FOENIX-CCA2 trial is a Phase 1 / 2 Study of TAS-120 in Patients With Advanced Solid Tumors Harboring FGF/FGFR Aberrations: ***F***GFR ***O***ral S***E***lective ***N***ovel ***I***nhibitor ***X*** [across] tumors

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