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U.S. Food and Drug Administration (FDA) Accepts for Priority Review Taiho Oncology's New Drug Application for Futibatinib for Cholangiocarcinoma

PRINCETON, N.J., MARCH 30, 2022 – Taiho Oncology, Inc. and Taiho Pharmaceutical Co., Ltd. announced today that the U.S. Food and Drug Administration (FDA) has accepted for priority review the New Drug Application (NDA) for futibatinib in the treatment of patients with previously treated locally advanced or metastatic cholangiocarcinoma (CCA) harboring *FGFR*2 gene rearrangements, including gene fusions. Futibatinib is an investigational, oral, potent, selective and irreversible small-molecule inhibitor of *FGFR*1, 2, 3 and 4. The FDA provided an anticipated Prescription Drug User Fee Act (PDUFA) action date of September 30, 2022.

Each year, approximately 8,000 individuals in the U.S. 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 mainly seen in people 65 years of age or older,[3] and treatment options are limited. Within the CCA patient population, approximately 10-16% have tumors with *FGFR*2 gene rearrangements,[4,5,6,7,8] including gene fusions, which can form a hybrid gene and promote tumor proliferation. It is this subset of patients with CCA that encompasses the NDA for futibatinib.

"Given the lack of an accepted standard chemotherapy following the failure of first-line treatment,[9] futibatinib could represent a significant opportunity for a targeted therapy in this subset of patients with CCA, which has driven our pursuit with this investigational compound," said Volker Wacheck, Vice President, Clinical Development, Taiho Oncology, Inc. "We look forward to working with the FDA as they consider the application for futibatinib under priority review."

The NDA is based on data from the pivotal Phase 2b FOENIX-CCA2 trial in 103 patients with locally advanced or metastatic unresectable intrahepatic (inside the liver) CCA, harboring *FGFR*2 gene rearrangements including fusions 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 41.7% 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). Common treatment-related adverse events (TRAEs) in the trial were hyperphosphatemia (85%), alopecia (33%) and dry mouth (30%). The only serious adverse reaction reported in more than one patient enrolled in the FOENIX-CCA2 trial was migraine (1.9%).

Results from the trial were presented in 2021 at the American Association for Cancer Research (AACR) Meeting. Based on these data, the FDA granted Breakthrough Therapy Designation (BTD) for futibatinib for the treatment of patients with previously treated locally advanced or metastatic CCA in 2021.

"This is a very important step towards our goal to deliver futibatinib to patients awaiting potential new treatment options," said Teruhiro Utsugi, Senior Managing Director at

Taiho Pharmaceutical. “The Taiho group, working as one, will continue to do its utmost to deliver this agent to those in need.”

About Futibatinib
Futibatinib (TAS-120) is an investigational, oral, potent, selective, and irreversible tyrosine kinase inhibitor of *FGFR*1, 2, 3 and 4 being studied as a potential treatment for patients with advanced solid tumors with *FGFR*1-4 genetic aberrations, including cholangiocarcinoma, who were previously treated with chemotherapy or other therapies. Futibatinib selectively and irreversibly binds to the ATP binding pocket of *FGFR*1-4 resulting in the inhibition of *FGFR*-mediated signal transduction pathways, reduced tumor cell proliferation and increased tumor cell death in tumors with *FGFR*1-4 genetic aberrations.

About Taiho Oncology, Inc.
The mission of Taiho Oncology, Inc. is to improve the lives of patients with cancer, their families and their caregivers. The company specializes in the development of orally administered anti-cancer agents and markets these medicines for a range of tumor types in the U.S. Taiho Oncology’s growing pipeline of selectively targeted anti-cancer agents is led by a world-class clinical development organization. Taiho Oncology is a subsidiary of Taiho Pharmaceutical Co., Ltd. which is part of Otsuka Holdings Co., Ltd. Taiho Oncology is headquartered in Princeton, New Jersey and oversees its parent company’s European and Canadian operations, which are located in Zug, Switzerland and Oakville, Ontario, Canada.

For more information, visit http://www.taihooncology.com

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 is simple: “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/

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