March 30, 2022
Taiho Pharmaceutical Co., Ltd.
Taiho Oncology, Inc.

U.S. FDA Accepts for Priority Review New Drug Application of Futibatinib for Advanced Cholangiocarcinoma

Taiho Pharmaceutical Co., Ltd. ("Taiho Pharmaceutical") and its U.S. subsidiary Taiho Oncology, Inc. ("Taiho Oncology") announced today that the U.S. Food and Drug Administration (FDA) has accepted and granted priority review of the New Drug Application (NDA) for futibatinib (TAS-120), a covalently-binding FGFR inhibitor, in the treatment of patients with previously treated locally advanced or metastatic cholangiocarcinoma (CCA) harboring *FGFR2* gene rearrangements, including gene fusions. The FDA has provided an anticipated Prescription Drug User Fee Act (PDUFA) action date of September 30 2022.

The NDA submitted in the U.S. is based on the data from the pivotal, Phase 2b FOENIX-CCA2 trial. Results from the trial were presented at the American Association for Cancer Research (AACR) Meeting 2021.[1] Based on these data, the FDA granted Breakthrough Therapy Designation (BTD) to futibatinib for the treatment of patients with previously treated locally advanced or metastatic CCA harboring FGFR2 gene rearrangements, including gene fusions in 2021.[2]

"This is a very important step towards our goal to deliver futibatinib to patients awaiting potential new treatment options," says 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."

"Given the lack of accepted standard chemotherapy following the failure of first-line treatment,[3] futibatinib could represent a significant opportunity for a targeted

therapy in subset of patients with CCA, which has driven our pursuit with this investigational compound," said Volker Wacheck, Vice President, Clinical Development, Taiho Oncology. "We look forward to working with the FDA as they consider the application for futibatinib under priority review."

Through research and development of innovative treatments, the Taiho group aims to contribute to patients and healthcare professionals around the world.

【About Cholangiocarcinoma】

Each year, approximately 8,000 individuals in the U.S.[4] are diagnosed with CCA, a rare cancer of the bile ducts of the liver, and approximately 03.-6 people per 100,000 individuals live with CCA worldwide.[5] CCA is mainly seen in people 65 years of age or older,[6] and treatment options are limited. Within the CCA patient population, approximately 10-16% have *FGFR*2 gene rearrangements,[7,8,9,10,11] including gene fusions, which can form a hybrid gene with tumor-specific expressions. It is this subset of patients with CCA that encompasses the NDA for futibatinib.

【About futibatinib】

Futibatinib (TAS-120) is an investigational, 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 selectively and irreversibly binds to the ATP binding pocket of FGFR1-4 resulting in the inhibition of FGFR-mediated signal transduction pathways, reduced tumor cell proliferation and increased tumor cell death in tumors with *FGFR1-4* genetic aberrations.

Futibatinib has been granted from the FDA, an orphan drug status for the treatment of cholangiocarcinoma in May 2018, also have received Breakthrough Designation for the treatment of patients with previously treated locally advanced or metastatic cholangiocarcinoma in April 2021.

【About FOENIX-CCA2 trial】

FOENIX-CCA2 trial is a Phase 2b 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 objective response rate (ORR).

【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/

【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 about Taiho Oncology, please visit: https://www.taihooncology.com/us/

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