TAIHO ONCOLOGY

Taiho Oncology Announces Presentation of Phase 2 Data for Futibatinib (TAS-120) in Advanced Intrahepatic Cholangiocarcinoma at Virtual AACR Annual Meeting 2021

PRINCETON, N.J., April 11, 2021 – Taiho Oncology, Inc. today announced efficacy and safety results from the Phase 2 FOENIX-CCA2 trial, a single-arm multicenter Phase 2 study evaluating futibatinib (TAS-120) in patients with intrahepatic cholangiocarcinoma (iCCA) harboring *FGFR*2 gene rearrangements including gene fusions who have failed at least one line of therapy. The data were presented online as an oral presentation at the American Association for Cancer Research (AACR) Annual Meeting 2021 Week 1 Clinical Trials Plenary from 2:00 – 3:45 PM ET on April 11, 2021.

In the FOENIX-CCA2 trial, 103 patients with locally advanced or metastatic unresectable iCCA harboring *FGFR2* gene rearrangements including fusions who had received one or more prior lines of systemic therapy received futibatinib 20 mg once daily until disease progression or unacceptable toxicity. The study met its primary endpoint of a greater than 20% objective response rate (ORR) assessed by independent central review with an ORR of 41.7%. Secondary endpoints of duration of response (DOR) and disease control rate (DCR) were also reported; responses were durable, with a median DOR of 9.7 months and 72% of responses ≥6 months, and a DCR of 82.5%. Median progression-free survival (PFS) was 9.0 months and median overall survival (OS) was 21.7 months, with 72% of patients alive at 12 months.

Common treatment-related adverse events (TRAEs) were hyperphosphatemia (85%), alopecia (33%) and dry mouth (30%). The most frequent grade 3 TRAE was hyperphosphatemia (30%), which resolved in patients with adequate management. One grade 4 TRAE of increased ALT was reported and there were no treatment related deaths.

“The results of FOENIX-CCA2 are significant for patients living with refractory intrahepatic cholangiocarcinoma with *FGFR*2 gene fusions or other rearrangements as futibatinib showed a meaningful ORR of 41.7% and good durability of responses,” said medical oncologist Lipika Goyal, MD, MPhil, Massachusetts General Hospital Cancer Center, and lead investigator on the study. “These results represent another example of the promise of precision medicine in cholangiocarcinoma and indicate that futibatinib could be an option for patients with refractory CCA if approved.”

The U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation (BTD) for futibatinib for the treatment of patients with previously treated locally advanced or metastatic cholangiocarcinoma harboring *FGFR*2 gene rearrangements, including gene fusions in February 2021 based on efficacy and safety results from the FOENIX-CCA2 study. The FDA Office of Orphan Drug Development granted futibatinib orphan drug status for the treatment of cholangiocarcinoma in May 2018.

Additional Data on Futibatinib (TAS-120) in iCCA Presented

Taiho Oncology also presented preclinical and Phase 1 clinical data for futibatinib at AACR as poster presentations. Presentations include:

- **Acquired resistance to ATP-competitive and irreversible FGFR inhibitors (FGFRi's): A library-based approach:** Hiroshi Sootome, MS, Manager, Translational Research Planning & Management group, Taiho Pharmaceutical Co., Ltd. (1117). Results were shared online as a poster presentation from 8:30 AM – 11:59 PM ET on April 10, 2021.
- **Effect of futibatinib on QT/QTc interval: a randomized, controlled, double-blind study:** Ikuo Yamamiya, PhD, Associate Director, Bioanalytics & DMPK, Taiho Oncology, Inc. (CT128). Results were shared online as a poster presentation from 8:30 AM – 11:59 PM ET on April 10, 2021.
- **Evaluation of potential drug-drug interactions (DDIs) between futibatinib and CYP3A inhibitors/inducers, CYP3A substrates, or proton pump inhibitors (PPIs):** Ikuo Yamamiya, PhD, Associate Director, Bioanalytics & DMPK, Taiho Oncology, Inc. (CT125). Results were shared online as a poster presentation from 8:30 AM – 11:59 PM ET on April 10, 2021.

Please visit Taiho Oncology's virtual Medical Booth for more information.

"With the low survival rates typically seen in patients with intrahepatic cholangiocarcinoma, the possibility of a new treatment option with demonstrated efficacy and safety is an important development for the oncology community," said Martin J. Birkhofer, MD, Senior Vice President and Chief Medical Officer, Taiho Oncology, Inc. "Taiho Oncology looks forward to sharing these data with regulatory authorities, with the hope of supporting approval for this important investigational therapy."

About Futibatinib (TAS-120)
Futibatinib (TAS-120) is an investigational, oral, potent, selective, and irreversible small-molecule 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 Cholangiocarcinoma
Cholangiocarcinoma (CCA), also known as bile duct cancer, is not common. About 8,000 people in the U.S. are diagnosed with CCA each year.[1] This includes both intrahepatic (inside the liver) and extrahepatic (outside the liver) cancers. CCA can occur at younger ages, but it is seen mainly in older people. The average age of people

in the U.S. diagnosed with cancer of the intrahepatic bile ducts is 70, and for cancer of the extrahepatic bile ducts it is 72.[1] The five-year survival rate of intrahepatic CCA (all SEER stages combined) is 9%.[2]

The main treatment for CCA is surgery. Radiation therapy and chemotherapy may be used if the cancer cannot be entirely removed with surgery and in cases where the edges of the tissues removed at the operation show cancer cells (also called a positive margin). Both stage III and stage IV cancers cannot be completely removed surgically. Currently, standard treatment options are limited to radiation, palliative therapy, liver transplantation, surgery, chemotherapy and interventional radiology.[3]

About Taiho Oncology, Inc. (U.S.)
Taiho Oncology, Inc., a subsidiary of Taiho Pharmaceutical Co., Ltd. and Otsuka Holdings Co., Ltd., has established a world class clinical development organization that works urgently to develop innovative cancer treatments and has built a commercial business in the U.S. Taiho Oncology has an oral oncology pipeline consisting of selectively targeted agents. Advanced technology, dedicated researchers, and state of the art facilities are helping us to define the way the world treats cancer. It's our work; it's our passion; it's our legacy.

For more information about Taiho Oncology, please visit:
https://www.taihooncology.com/us

For more information about Taiho Pharmaceutical Co., Ltd., please visit:
https://www.taiho.co.jp/en/

For more information about Otsuka Holdings Co., Ltd., please visit:
https://www.otsuka.com/en/

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[1] American Cancer Society. Key statistics for bile duct cancer. https://www.cancer.org/cancer/bile-duct-cancer/about/key-statistics.html#references. Accessed February 2021.

[2] American Cancer Society. Survival rates for bile duct cancer. https://www.cancer.org/cancer/bile-duct-cancer/detection-diagnosis-staging/survival-by-stage.html. Accessed March 2021.

[3] The Cholangiocarcinoma Foundation. Treatment options. https://cholangiocarcinoma.org/the-disease/treatment-options. Accessed February 2021.