Taiho Oncology Announces Presentation of Data for Futibatinib in Advanced Intrahepatic Cholangiocarcinoma at 2020 ASCO Meeting

PRINCETON, N.J., May 31, 2020 – Taiho Oncology, Inc. today announced efficacy and safety results of an interim analysis of FOENIX-CCA2, a single-arm multicenter Phase 2 study evaluating futibatinib (TAS-120) in patients with intrahepatic cholangiocarcinoma (iCCA) harboring *FGFR2* gene fusions or other rearrangements, who have failed at least one line of therapy. The data were presented online at the 2020 American Society of Clinical Oncology (ASCO) Annual Meeting from 10:30 a.m.-12:00 p.m. ET on Sunday, May 31, 2020.

“The interim analysis demonstrated that treatment with the covalently-binding *FGFR* inhibitor futibatinib may lead to meaningful clinical benefit in patients with refractory iCCA with *FGFR*2 gene fusions or other rearrangements,” said medical oncologist Lipika Goyal, MD , MPhil of the Massachusetts General Hospital Cancer Center. “In a disease with limited treatments, this drug could be an effective and well-tolerated option for patients and the oncologists that care for them.”

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 primary endpoint of the trial is independent central radiology reviewed objective response rate (ORR). Secondary endpoints include disease control rate (DCR), duration of response (DOR) and safety. The interim analysis reported data for 67 patients (65%) with a minimum of 6 months of follow up and found the ORR was 37.3% (1 CR=1.5%; 24 PR=35.8%). Median duration of response was 8.31 months. The most common treatment-related adverse events (all grades, grade 3) at the time of analysis were hyperphosphatemia (80.6%; 26.9%), diarrhea (37.3%; 0%), and dry mouth (32.8%;0%). There were no grade 4 treatment related adverse events. Final results from the trial will be presented at a future medical meeting.

“FOENIX-CCA2 adds to the body of evidence supporting futibatinib as a potential treatment option for patients living with intrahepatic cholangiocarcinoma,” said Martin J. Birkhofer, MD, Senior Vice President and Chief Medical Officer, Taiho Oncology, Inc. “We are pleased to see the interim results of the FOENIX-CCA2 trial, which point to the efficacy and tolerability of futibatinib in these patients, and we look forward to sharing the final results and progressing this investigational compound.”

In May 2018, the U.S. Food and Drug Administration Office of Orphan Drug Development granted futibatinib orphan drug status for the treatment of cholangiocarcinoma.

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.[2] The five-year survival rates of localized iCCA is 24%.[1]

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.[2]

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 *FGFR1-4* 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. (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 has an oral oncology pipeline consisting of both novel antimetabolic agents and 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. What are the key statistics about bile duct cancer? https://www.cancer.org/cancer/bile-duct-cancer/about/key-statistics.html#references. Accessed April 2020.
[2] The Cholangiocarcinoma Foundation. Treatment Options. https://cholangiocarcinoma.org/the-disease/treatment-options. Accessed April 2020.