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TAIHO ONCOLOGY

Taiho Oncology Presents Data on Key Investigational Compound TAS-120 at ESMO 20th World Congress on Gastrointestinal Cancer 2018

Update of Clinical Data for TAS-120 in Cholangiocarcinoma, a Rare Cancer with Limited Treatment Options; Investigational Therapy Granted Orphan Drug Designation by U.S. Food and Drug Administration

PRINCETON, N.J., June 20, 2018 – Taiho Oncology, Inc. today announced a Phase I study to examine the efficacy of TAS-120, an investigational irreversible pan-fibroblast growth factor receptor (FGFR) inhibitor as a potential treatment for patients with advanced solid tumors, including cholangiocarcinoma (CCA), who were previously treated with chemotherapy or other therapies including other FGFR inhibitors. This is a presentation of updated clinical data for TAS-120 in cholangiocarcinoma, a rare cancer with limited treatment options. These data were presented as oral and poster presentations on Wednesday, June 20 at 2:50 PM CEST at the ESMO 20th World Congress on Gastrointestinal Cancer 2018 (ESMO-GI) in Barcelona, Spain, June 20 to 23.

“We’re very pleased to see that TAS-120 has shown great promise among CCA patients in this study,” said Robert Winkler, MD, senior vice president and head of Clinical Development, Taiho Oncology, Inc. “With such a broad range of tumor types with high unmet medical need, Taiho Oncology has remained committed to pioneering innovative research in specific areas that we hope will ultimately lead to an expanded range of potential therapies.”

In this study of 45 patients with CCA harboring FGF/FGFR aberrations (e.g., FGFR2 gene fusions, FGF/FGFR mutations, amplifications and re-arrangements), TAS-120 demonstrated clinical activity in patients with CCA with FGFR2 gene fusions, and showed efficacy in patients who progressed on prior FGFR inhibitors. In 28 patients with FGFR2 gene fusions, 71 percent experienced tumor shrinkage and seven achieved confirmed partial response (cPR). The objective response rate in these patients was 25 percent, and 15 patients (54%) experienced stable disease as their best response, with seven still on treatment. The overall disease control rate was 79 percent. In 17 patients with other FGF/FGFR aberrations who received TAS-120, 18 percent achieved cPR. In 13 patients who had received prior FGFR inhibitors, 31 percent achieved cPR.

“These results are encouraging for this rare and difficult-to-treat cancer,” said Milind Javle, MD, professor, Gastrointestinal Medical Oncology, The University of Texas MD Anderson Cancer Center, Houston, and investigator on the Phase I TAS-120 study. “Given the limited number of patients diagnosed each year with cholangiocarcinoma in

the U.S., we recognize the sense of urgency for and importance of ongoing research to advance potential new therapies."

The most common treatment-related adverse events (AEs) of all grades in all patients were hyperphosphatemia (78%), increased aspartate aminotransferase (29%), dry skin (29%), diarrhea (27%) and dry mouth (27%). Grade ≥3 treatment-related AEs were reported in 51 percent of patients (51%); the most common was hyperphosphatemia in 22 percent of patients.

A Phase II study of TAS-120 in CCA patients has been initiated. The abstract for this presentation is available on the ESMO-GI website at http://sched.co/Drky.

In May 2018, the U.S. Food and Drug Administration Office of Orphan Drug Development granted Taiho's investigational TAS-120 orphan drug status for the treatment of cholangiocarcinoma.

About FDA Orphan Drug Designation
The mission of the FDA Office of Orphan Products Development (OOPD) is to advance the valuation and development of products (drugs, biologics, devices, or medical foods) that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions. The Orphan Drug Designation program provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or preventive of rare diseases/disorders that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug. The program has successfully enabled the development and marketing of more than 350 drugs and biologic products for rare diseases since 1983. In contrast, the decade prior to 1983 saw fewer than 10 such products come to market.

About Cholangiocarcinoma
Cholangiocarcinoma (CCA), also known as bile duct cancer, is not common. About 8,000 people in the United States are diagnosed with CCA each year. 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 United States diagnosed with cancer of the intrahepatic bile ducts is 70, and for cancer of the extrahepatic bile ducts it is 72. The chances of survival for patients with CCA depend to a large extent on its location and how advanced it is when it is found.[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 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 with a strong commercial business in the U.S. dedicated to bringing the company's approved medical innovations to patients. 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.

About Taiho Pharmaceutical Co., Ltd. (Japan)
Taiho Pharmaceutical, a subsidiary of Otsuka Holdings Co., Ltd., is an R&D-driven specialty pharma focusing on the three fields of oncology, allergy and immunology, and urology. Its corporate philosophy takes the form of a pledge: "We strive to improve human health and contribute to a society enriched by smiles." In the field of oncology, in particular, Taiho Pharmaceutical is known as a leading company in Japan for developing innovative medicines for the treatment of cancer, a reputation that is rapidly expanding through their extensive global R&D efforts. In areas other than oncology, as well, the company creates and markets quality products that effectively treat medical conditions and can help improve people's quality of life. Always putting customers first, Taiho Pharmaceutical also aims to offer consumer healthcare products that support people's efforts to lead fulfilling and rewarding lives.

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

About Otsuka Holdings Co., Ltd. (Japan)
The Otsuka group of companies is a total-healthcare enterprise that aims to contribute to the health of people around world under the corporate philosophy, "Otsuka-people creating new products for better health worldwide."

Healthcare is broadly and holistically addressed through the two main pillars – the pharmaceutical business for the diagnosis and treatment of diseases and the nutraceutical[1] business to support the maintenance and promotion of everyday health. Our 46,000[2] employees across 183 companies in 28 countries and regions take on challenges across various fields and themes to help fulfill the universal wish of people to be healthy. Our pursuit of these challenges is motivated by the Otsuka' s corporate culture, articulated as "Ryukan-godo" (by sweat we recognize the way), "Jissho" (actualization) and "Sozosei" (creativity), and fostered by successive generations of Otsuka leaders. By striving to provide unique products and services, we seek to achieve sustainable growth and be an indispensable contributor to the world.

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[1] Nutraceuticals: nutrition + pharmaceuticals
[2] As of end of December 2017

For more information, please visit the company's website at 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 May 2018.
[2] The Cholangiocarcinoma Foundation.Treatment Options. https://cholangiocarcinoma.org/the-disease/treatment-options Accessed May 2018.