European Commission Grants Conditional Marketing Authorization for Taiho's LYTGOBI® Tablets for the Treatment of Adults With Cholangiocarcinoma

LYTGOBI is the first irreversibly binding FGFR inhibitor in the European Union for use in the treatment of patients with cholangiocarcinoma

ZUG, Switzerland, 4 July 2023 - Taiho Oncology Europe GmbH and Taiho Pharmaceutical Co., Ltd., announced today that the European Commission has granted conditional marketing authorization for LYTGOBI® (futibatinib) monotherapy for the treatment of adult patients with locally advanced or metastatic cholangiocarcinoma (CCA) with a fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement that have progressed after at least one prior line of systemic therapy.

CCA is an aggressive cancer of the bile ducts of the liver. While rare – in Europe, approximately 6.000-8.000 people are diagnosed with CCA[1] – this disease is associated with poor outcomes and is growing in prevalence worldwide[2], underscoring the need for new treatment options.

"Today is an important day for current and future patients with CCA as well as the healthcare providers who treat them," said Peter Foertig, MD, Vice President, Medical Affairs, Taiho Oncology Europe. "LYTGOBI is an oral molecularly targeted medication that may provide clinically meaningful outcomes for patients undergoing treatment for CCA."

Added John Bridgewater, MD, PhD, Investigator and Senior Author of the recently published FOENIX*-CCA2 pivotal trial in the *New England Journal of Medicine*: "FGFR2 fusions/rearrangements are one of the most frequent actionable alterations in CCA. As an irreversibly binding FGFR inhibitor, LYTGOBI targets FGFR in a unique way and offers new hope in a disease that, for me, has been among the most challenging to treat in my career." Professor Bridgewater is a Clinical Researcher and Medical Oncologist at University College London Cancer Institute and University College London Hospitals NHS Foundation Trust.

The European Commission's conditional marketing authorization for LYTGOBI is based on data from the aforementioned FOENIX-CCA2 trial, a global open label trial evaluating 103 patients with unresectable, locally advanced or metastatic intrahepatic (inside the bile ducts of the liver) CCA harboring FGFR2 gene rearrangements, including fusions.

In this trial, patients received LYTGOBI orally once daily at a dose of 20mg until disease progression or unacceptable toxicity. Within Europe, patients were enrolled from France, Germany, Italy, the Netherlands, Spain and the United Kingdom.

Results of FOENIX-CCA2 trial showed:

- LYTGOBI demonstrated an objective response rate (ORR) of 42% as assessed by independent review (primary endpoint met).[3]

- LYTGOBI demonstrated a median duration of response (DOR) – a key secondary endpoint – of 9.7 months. A total of 72% of the responses lasted ≥6 months.[3]

“I believe that LYTGOBI may be part of a new era in the treatment of CCA, one in which the power of personalised medicine may touch the lives of patients in ways we haven’t seen before with traditional chemotherapy,” said Helen Morement, CEO of the AMMF – The Cholangiocarcinoma Charity and the UK’s only charity dedicated to this cause.

“We thank the many patients and healthcare professionals who participated in the FOENIX-CCA2 trial,” said Atsushi Azuma, Managing Director of Taiho Pharmaceutical and Chairman of Taiho Oncology Europe. “Patients with CCA are often diagnosed at an advanced stage when surgery is not an option. We are pleased that LYTGOBI now will be a new treatment option for patients with CCA.”

A conditional marketing authorization in Europe is granted for medicines that fulfill an unmet medical need to treat serious diseases, and the benefits of having them available earlier outweighs any risks associated with using the medicines while waiting for further evidence. Under the specific obligation to complete post-authorization measures for the conditional marketing authorization, Taiho has until October 2027 to provide additional clinical data on LYTGOBI.

Safety Information From the EU Summary of Product Characteristics (SmPC)[3]

LYTGOBI may cause serious adverse reactions. The most common serious adverse reactions were intestinal obstruction and migraine.

The most common adverse reactions were hyperphosphatemia, nail disorders, constipation, alopecia, diarrhoea, dry mouth, fatigue, nausea, dry skin, increased AST, abdominal pain, stomatitis, vomiting, palmar-plantar erythrodysaesthesia syndrome, arthralgia, and decreased appetite.

Prolonged hyperphosphatemia may cause soft tissue mineralization, including cutaneous calcification, vascular calcification, and myocardial calcification, anaemia, hyperparathyroidism, and hypocalcemia that may cause muscle cramps, QT interval prolongation, and arrythmias. Hyperphosphatemia is a pharmacodynamic effect expected with LYTGOBI administration. Recommendations for management of hyperphosphatemia include dietary phosphate restriction, administration of phosphate-lowering therapy, and dose modification when required.

LYTGOBI can cause serous retinal detachment, which may present with symptoms such as blurred vision, visual floaters or photopsia. Ophthalmological examination should be performed prior to initiation of therapy, 6 weeks thereafter, and urgently at any time for visual symptoms. For serous retinal detachment reactions, the dose modification guidelines should be followed.

LYTGOBI should not be used during pregnancy unless the clinical condition of the women requires treatment with LYTGOBI.

About Taiho Oncology Europe
The mission of Taiho Oncology Europe is to improve the lives of patients with cancer, their families, and their caregivers. The company specializes in orally administered anti-cancer agents and has a growing pipeline of selectively targeted anti-cancer agents. Taiho Oncology Europe GmbH (Zug, Switzerland) is the European subsidiary of Taiho Pharmaceutical Co., Ltd. (Tokyo, Japan). For more information, visit www.taihooncology.eu

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 takes the form of a pledge: “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/

*The FOENIX-CCA2 trial is a Phase 1 / 2 Study of TAS-120 in Patients With Advanced Solid Tumors Harboring FGF/FGFR Aberrations: FGFR Oral SElective Novel Inhibitor X [across] tumors

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[3] Taiho Oncology Europe GmbH. LYTGOBI® (futibatinib): Summary of Product Characteristics.