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Press release

Basilea reports updated interim results for iCCA patients with FGFR2 mutations and amplifications from phase 2 study FIDES-01 at ASCO GI Cancers Symposium

Basel, Switzerland, January 24, 2022

Basilea Pharmaceutica Ltd. (SIX: BSLN), a commercial-stage biopharmaceutical company committed to meeting the needs of patients with cancer and infectious diseases, announced today that updated interim results from cohort 2 of the phase 2 study FIDES-01 (**F**ibroblast growth factor **I**nhibition with **DE**razantinib in **S**olid tumors) were presented at the American Society of Clinical Oncology (ASCO) Gastrointestinal (GI) Cancers Symposium 2022, held from January 20 to 22, in San Francisco, USA. Cohort 2 of FIDES-01 is assessing the safety and anti-tumor activity of Basilea's orally administered fibroblast growth factor receptor (FGFR) inhibitor, derazantinib, in patients with locally advanced or metastatic intrahepatic cholangiocarcinoma (iCCA), a form of bile duct cancer, harboring FGFR2 mutations or amplifications.[1]

At the cut-off date for the interim analysis, August 31, 2021, 28 patients had been dosed, of which 23 patients were eligible for the efficacy assessment with a median follow-up period of 5.2 months. Based on investigator assessments, the disease control rate (DCR) was 74%, including two patients with a confirmed objective response and 15 patients with stable disease, where tumor shrinkage was observed in the majority of patients. The median progression-free survival (PFS) was 7.3 months. The DCR and median PFS are similar to those reported for the group of iCCA patients with FGFR2 fusions from cohort 1 of the FIDES-01 study.[2] The observed safety and tolerability was consistent with the profile reported for cohort 1, with low incidences of nail toxicities, stomatitis, hand-foot syndrome and retinal effects, which have been associated with the FGFR inhibitor class.

Dr. Marc Engelhardt, Chief Medical Officer, said: "The updated interim results in iCCA patients with FGFR2 mutations and amplifications remain encouraging and support the therapeutic relevance of derazantinib in this group of patients where there has been limited clinical evidence of successful treatment with other FGFR inhibitors. Derazantinib has demonstrated clinically meaningful efficacy across all types of FGFR2 genetic aberrations, which confirms the broad potential of derazantinib as a monotherapy for the treatment of iCCA patients. FIDES-01 continues to enroll and we expect topline results for cohort 2 in the first half of 2022."

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Derazantinib poster presented at the 2022 ASCO GI Cancers Symposium

Abstract #	Authors/title
427	M. M. Javle, G. K. Abou-Alfa, T. Macarulla, N. Personeni, F. Bergamo, D. Malka, A. Vogel, J. Adeva, J. Knox, J. Evans, W. P. Harris, M. Dimova-Dobreva, M. Saulay, M. Engelhardt, S. Braun, M. Droz dit Busset, M. J. Borad Efficacy of derazantinib in intrahepatic cholangiocarcinoma patients with FGFR2 mutations or amplifications: Interim results from the phase 2 study FIDES-01.

For further information please visit https://conferences.asco.org/gi/abstracts.

About derazantinib

Derazantinib is an investigational orally administered small-molecule FGFR inhibitor with strong activity against FGFR1, 2, and 3.[3] FGFR kinases are key drivers of cell proliferation, differentiation and migration. FGFR genetic aberrations, e.g. gene fusions, mutations or amplifications, have been identified as potentially important therapeutic targets for various cancers, including intrahepatic cholangiocarcinoma (iCCA), urothelial, breast, gastric and lung cancers.[4] In these cancers, FGFR genetic aberrations are found in a range of 5% to 30%.[5] Derazantinib also inhibits the colony-stimulating-factor-1-receptor kinase (CSF1R).[3, 6] CSF1R-mediated signaling is important for the maintenance of tumor-promoting macrophages and therefore has been identified as a potential target for anti-cancer drugs.[7] Pre-clinical data has shown that tumor macrophage depletion through CSF1R blockade renders tumors more responsive to T-cell checkpoint immunotherapy, including approaches targeting PD-L1/PD-1.[8, 9] Derazantinib has demonstrated antitumor activity and a manageable safety profile in a previous biomarker-driven phase 1/2 study in iCCA patients,[10] and has received U.S. and EU orphan drug designation for iCCA. Basilea is currently conducting three clinical studies with derazantinib. The first study, FIDES-01, is a phase 2 study in patients with inoperable or advanced iCCA. It comprises one cohort of patients with FGFR2 gene fusions and another cohort of patients with mutations or amplifications.[1] The second study, FIDES-02, is a phase 1/2 study evaluating derazantinib alone and in combination with Roche's PD-L1 checkpoint inhibitor, atezolizumab, in patients with advanced urothelial cancer, including metastatic, or recurrent surgically unresectable disease, expressing FGFR genetic aberrations.[11] The third study, FIDES-03, is a phase 1/2 study evaluating derazantinib alone and in combination with Lilly's anti-VEGFR2 antibody ramucirumab and paclitaxel, or with Roche's PD-L1 checkpoint inhibitor atezolizumab, in patients with advanced gastric cancer with FGFR genetic aberrations.[12] Basilea has in-licensed derazantinib from ArQule Inc., a wholly-owned subsidiary of Merck & Co., Inc., Kenilworth, N.J., U.S.A.

About intrahepatic cholangiocarcinoma

Intrahepatic cholangiocarcinoma (iCCA) is a cancer originating from the biliary system. The age-adjusted incidence rate of iCCA in the United States has been increasing over the past decade and is currently estimated to be approximately 1.2 per 100,000.[13] Patients are often diagnosed with advanced or metastatic disease that cannot be surgically removed. Current first-line standard of care is the chemotherapy combination of gemcitabine and platinum-derived agents. The prognosis for patients with advanced disease is poor, with a median survival of less than one year.[14]

About Basilea

Basilea is a commercial-stage biopharmaceutical company founded in 2000 and headquartered in Switzerland. We are committed to discovering, developing and commercializing innovative drugs to meet the needs of patients with cancer and infectious diseases. We have successfully launched two hospital brands, Cresemba for the treatment of invasive fungal infections and Zevtera for the treatment of severe bacterial infections. We are conducting clinical studies with two targeted drug candidates for the treatment of a range of cancers and have several preclinical assets in both cancer and infectious diseases in our portfolio. Basilea is listed on the SIX Swiss Exchange (SIX: BSLN). Please visit basilea.com.

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This press release can be downloaded from www.basilea.com.

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