PRESS RELEASE

Basilea reports activity of derazantinib in preclinical models of gastric cancer at ASCO Gastrointestinal Cancers Symposium

Basel, Switzerland, January 23, 2020 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reported today that its oncology drug candidate derazantinib showed convincing activity in preclinical models of gastric cancer with FGFR genetic aberrations. The data will be presented at the Gastrointestinal Cancers Symposium of the American Society of Clinical Oncology (ASCO), which takes place January 23-25, 2020, in San Francisco, USA.

Dr. Laurenz Kellenberger, Basilea's Chief Scientific Officer, said: "The preclinical data presented at the ASCO symposium show that derazantinib has the potential to provide clinical benefit in gastric cancer with FGFR genetic aberrations. Based on this convincing data we recently announced our intention to start a phase 1/2 study with derazantinib in patients with advanced gastric cancer in the third quarter of 2020."

The preclinical data were generated in patient-derived mouse models of biliary, gastric and colorectal cancer with FGFR gene fusions, mutations and amplifications. Derazantinib was well tolerated and led to strong responses in several animal models, particularly in gastric cancer models with FGFR2 gene fusions, where responses included complete tumor regression.

Gastric cancer is the fifth most common cancer worldwide and the third most lethal cancer type.[1] Median survival rarely exceeds twelve months and the five-year-survival is less than 10%.[2] Basilea estimates that there are approximately 190,000 new cases of gastric cancer per year in total across the EU top 5 countries, Japan and the U.S. FGFR genetic aberrations have been observed in about 10% of gastric cancers.[3]

A second abstract at the symposium provides an update on the ongoing registrational phase 2 study FIDES-01 (**F**ibroblast growth factor **I**nhibition with **DE**razantinib in **S**olid tumors) with derazantinib in intrahepatic cholangiocarcinoma (iCCA) patients. The multicenter, multicohort open-label study is planned to enroll 43 patients with mutations or amplifications of the FGFR2 gene confirmed by next-generation sequencing. In addition, the study includes a cohort of approximately 100 patients with FGFR2 gene fusion positive iCCA.
To date, FGFR inhibitors have demonstrated clinical activity in FGFR2 gene fusion-driven iCCA. Therefore, assessing the activity of derazantinib in FGFR2 gene mutations or amplifications is important to further define the full therapeutic potential of derazantinib in iCCA. Topline data for the cohort with FGFR2 gene fusion-positive patients and interim data for the cohort with FGFR2 gene mutations or amplifications are expected to become available in the second half of 2020.

Intrahepatic cholangiocarcinoma 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.[4] Patients are often diagnosed with advanced or metastatic disease that cannot be surgically removed. FGFR2 gene fusions have been reported in 13-22% of iCCA cases.[5, 6] 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 with chemotherapy.[7] There is no proven effective treatment for patients who progress on first-line chemotherapy, thus there is a high unmet medical need.[8]

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Derazantinib abstracts at the 2020 ASCO GI Cancers Symposium

Thursday, 23 January 2020 – 12:00-1:30 p.m. PST

Poster Session A

- The FGFR-inhibitor, derazantinib (DZB), is active in PDX-models of GI-cancer with specific aberrations in FGFR – Paul McSheehy, Felix Bachmann, Nicole Forster-Gross, Mahmoud El Shemerly, Mila Roceri, Laurenz Kellenberger, Heidi Lane; abstract 421, poster G10

Friday, 24 January 2020 – 12:00-1:30 p.m.PST

Trial in Progress Poster Session B

- FIDES-01, a phase 2 study of derazantinib in patients with unresectable intrahepatic cholangiocarcinoma (iCCA) and FGFR2 fusions and mutations or amplifications (M/A) – Walid L. Shaib, Christoph Gahlemann, Andrea Boncompagni, Silke Friedmann, Stephan Braun, Marc Engelhardt, Ghassan K. Abou-Alfa, Mitexh J. Borad; abstract TPS597, poster P10 (ClinicalTrial.gov identifier: NCT03230318)

For further information, please visit https://meetings.asco.org/gi/abstracts-posters.

About derazantinib

Derazantinib (formerly ARQ 087) is an investigational orally administered small-molecule panFGFR kinase inhibitor with strong activity against FGFR1, 2, and 3.[9] 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.[10] In these cancers, FGFR genetic aberrations are found in a range of 5% to 30%.[11] Derazantinib also inhibits the colony-stimulating-factor-1-receptor kinase (CSF1R).[9, 12] 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.[13] 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.[14, 15] Derazantinib has demonstrated antitumor activity and a manageable safety profile in previous clinical studies, including a biomarker-driven phase 1/2 study in iCCA patients,[16] and has received U.S. and EU orphan drug designation for iCCA. Basilea is currently conducting two clinical studies with derazantinib. The first study, FIDES-01, is a registrational 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.[17] The second study, FIDES-02, is a phase 1/2 study evaluating derazantinib alone and in combination with Roche's PD-L1-blocking immune-checkpoint inhibitor atezolizumab (Tecentriq®) in patients with advanced urothelial cancer, including metastatic, or recurrent surgically unresectable disease, expressing FGFR genetic aberrations.[18] Basilea in-licensed derazantinib from ArQule Inc, a wholly-owned subsidiary of Merck & Co., Inc., Kenilworth, N.J., U.S.A.

About Basilea

Basilea Pharmaceutica Ltd. is a commercial stage biopharmaceutical company, focused on the development of products that address the medical challenges in the therapeutic areas of oncology and infectious diseases. With two commercialized drugs, the company is committed to discovering, developing and commercializing innovative pharmaceutical products to meet the medical needs of patients with serious and life-threatening conditions. Basilea Pharmaceutica Ltd. is headquartered in Basel, Switzerland and listed on the SIX Swiss Exchange (SIX: BSLN). Additional information can be found at Basilea's website www.basilea.com.

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

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18 ClinicalTrials.gov identifier: NCT04045613. Tecentriq® is a registered trademark of Hoffmann-La Roche Ltd.

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