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Press release

Basilea starts phase 1/2 study FIDES-03 with derazantinib in patients with gastric cancer

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Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that it has initiated the phase 1/2 study, FIDES-03, with the FGFR inhibitor derazantinib. The multi-cohort study is evaluating derazantinib in patients with advanced gastric cancer with FGFR genetic aberrations. Derazantinib will be assessed as monotherapy and in combination with other cancer treatments, for instance with Roche's PD-L1 checkpoint inhibitor, atezolizumab.
Basilea will be the sponsor of the study and Roche will provide clinical supply of atezolizumab.

Dr. Marc Engelhardt, Basilea's Chief Medical Officer, said: "Our development strategy for derazantinib is focused on strengthening the clinical evidence on the differentiation versus other FGFR inhibitors. The unique kinase inhibition profile of derazantinib, results from preclinical studies and the safety and tolerability profile observed in clinical studies, provide a strong rationale for evaluating the drug candidate in patients with advanced gastric cancer with FGFR genetic aberrations, both as monotherapy and in combination therapy. Advanced gastric cancer is associated with a very poor prognosis and is an area of high unmet medical need."

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]

About derazantinib

Derazantinib is an investigational orally administered small-molecule FGFR inhibitor with strong activity against FGFR1, 2, and 3.[4] 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.[5] In these cancers, FGFR genetic aberrations are found in a range of 5% to 30%.[6] Derazantinib also inhibits the colony-stimulating-factor-1-receptor kinase (CSF1R).[4, 7] 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.[8] Preclinical 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.[9, 10] Derazantinib has demonstrated antitumor activity and a manageable safety profile in a previous biomarker-driven phase 1/2 study in iCCA patients,[11] 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 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.[12] 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, in patients with advanced urothelial cancer, including metastatic, or recurrent surgically unresectable disease, expressing FGFR genetic aberrations.[13] The third study, FIDES-03, is a phase 1/2 study evaluating derazantinib alone and in combination with other cancer treatments, for instance with Roche's PD-L1 checkpoint inhibitor, atezolizumab, in patients with advanced gastric cancer with FGFR genetic aberrations. 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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12. FIDES-01: ClinicalTrials.gov identifier: NCT03230318
13. FIDES-02: ClinicalTrials.gov identifier: NCT04045613