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Press release

Basilea reports positive interim results from phase 2 study FIDES-01 for derazantinib in FGFR2 gene mutation- or amplification-positive patients with bile duct cancer (iCCA)

- **Disease control rate of 79% with one complete response**
- **Consistent safety and tolerability profile**
- **Study continues with topline results expected in H1 2022**

Basel, Switzerland, March 24, 2021

Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today positive results of a pre-planned interim analysis in cohort 2 of the phase 2 study FIDES-01 (**F**ibroblast growth factor **I**nhibition with **DE**razantinib in **S**olid tumors), which is assessing the anti-tumor efficacy of the orally administered fibroblast growth factor receptor (FGFR) inhibitor, derazantinib, in patients with inoperable or advanced intrahepatic cholangiocarcinoma (iCCA), a form of bile duct cancer.[1] After cohort 1 of the study provided the clinical proof of concept for derazantinib monotherapy in the treatment of iCCA patients with FGFR2 gene fusions, cohort 2 is enrolling iCCA patients with FGFR2 gene mutations or amplifications.[2] The efficacy data obtained in the interim analysis met the pre-specified threshold so that the study will proceed to the next stage as planned.

The interim analysis of cohort 2 is based on 14 evaluable patients who had at least one post-baseline tumor assessment. The pre-specified criterion that at least 8 patients met the primary endpoint of obtaining progression-free survival (PFS) of at least 3 months was successfully achieved. The positive interim analysis allows the study to advance to its next stage and enrol a total of 43 patients. As a number of patients are still ongoing with treatment the median PFS was not yet mature at the time of the interim analysis and will be defined at a later time point.

The disease control rate (DCR), reflecting the proportion of patients with a complete or partial response or with stable disease, was 79%, including one patient with a confirmed complete response, one patient with an unconfirmed partial response and nine patients with a best response of stable disease at the time when the interim analysis was conducted.

The observed safety and tolerability is consistent with the profile reported for cohort 1.

Dr. Marc Engelhardt, Chief Medical Officer, said: “We are very pleased with the positive interim results for this cohort of iCCA patients with FGFR2 gene mutations or amplifications. The clinical benefit with derazantinib is similar to that reported for iCCA patients with FGFR2 gene

fusions earlier this year. This supports the relevance of derazantinib in a group of patients with iCCA where there has been very limited clinical evidence of successful treatment with other FGFR inhibitors and confirms the broad potential of derazantinib as a monotherapy for the treatment of iCCA patients with diverse FGFR2 genetic aberrations. This outcome is very encouraging and further strengthens the evidence for the differentiation of derazantinib versus other FGFR inhibitors both from the efficacy and safety perspective. We are now progressing the study to the next stage and expect topline results for cohort 2 in the first half of 2022."

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 medical 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 a number of 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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