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**Press release**

# Basilea presents full safety and efficacy data set on derazantinib in patients with FGFR2 fusion-positive iCCA at ESMO congress

– **Progression-free survival (PFS) of derazantinib monotherapy increased to eight months**

**Basel, Switzerland, September 17, 2021**

Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today the reporting of the updated efficacy and safety results from cohort 1 of the phase 2 study FIDES-01, which evaluated its fibroblast growth factor receptor (FGFR) inhibitor, derazantinib, in patients with FGFR2 fusion-positive advanced or metastatic intrahepatic cholangiocarcinoma (iCCA), a type of bile duct cancer, at the Congress of the European Society for Medical Oncology (ESMO), taking place as a virtual meeting from 16 to 21 September 2021.

Patients with advanced iCCA have a poor prognosis. With the current chemotherapy standard-of-care, the median overall survival is less than one year.[1]

Cohort 1 of FIDES-01 enrolled 103 iCCA patients with confirmed FGFR2 fusions.[2] Since the reporting of first topline results in early February 2021, more patient follow-up data has been obtained, showing improvements in efficacy outcomes over time. At the cut-off date in early August, for the data presented at the ESMO congress, the disease control rate (DCR) was 75.7%, including 22 patients with a partial response as the best objective response, corresponding to an objective response rate (ORR) of 21.4%. Importantly, the progression-free survival (PFS) further increased to 8.0 months (previously: 7.8 months). The time to progression (TTP) with derazantinib was 8.1 months and thus markedly longer when compared to a TTP of only 4.5 months with the previous anti-cancer treatment the patients had received prior to entering the study.
Median overall survival was 15.9 months, with follow-up ongoing. As reported at ESMO, derazantinib had a notably well manageable adverse event profile, with a low incidence of class effects such as nail toxicities, stomatitis, hand-foot syndrome and retinal effects.

Dr. Marc Engelhardt, Chief Medical Officer, said: “The further improved efficacy data and confirmed good safety and tolerability profile presented at ESMO are very encouraging and further strengthen the evidence for the efficacy of derazantinib and its differentiation in iCCA to other FGFR inhibitors from a safety perspective.”

**Derazantinib ePoster at ESMO Congress 2021, published on September 16**

– Derazantinib for patients with intrahepatic cholangiocarcinoma harboring *FGFR2* fusions/rearrangements: Primary results from the Phase 2 study FIDES-01 – M. Droz dit Busset, W. L. Shaib, K. Mody, N. Personeni, N. Damjanov, W. P. Harris, F. Bergamo, G. Brandi, G. Masi, T. Halfdanarson, V. Tam, L. W. Goff, J. Knox, A. Hollebecque, T. Macarulla Mercade, F. Cantero, M. Saulay, S. Braun, M. Javle, M. Borad; abstract 47P

For further information please visit esmo.org/meetings/esmo-congress-2021.

## 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.[2] 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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