PRESS RELEASE

Basilea reports positive interim results from registrational phase 2 study with oncology drug candidate derazantinib in intrahepatic cholangiocarcinoma (iCCA)

- **21% objective response rate with six confirmed partial responses from 29 evaluable patients**
- **83% disease control rate**
- **Safety profile and tolerability of continuous dosing schedule confirmed**

Basel, Switzerland, January 09, 2019 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today results from the interim analysis of the registrational phase 2 study with the orally administered pan-fibroblast growth factor receptor (FGFR) kinase inhibitor derazantinib (BAL087). The analysis showed promising efficacy in patients with FGFR2 gene fusion-expressing intrahepatic cholangiocarcinoma (iCCA) and also confirmed the safety profile and tolerability of the drug candidate observed in previous clinical studies.

The interim analysis in the ongoing registrational phase 2 study was conducted after 42 patients have been enrolled in the study, with a subset of 29 evaluable patients who had at least one post-baseline imaging assessment. The objective response rate (ORR) in these 29 patients was 21%. The disease control rate (DCR), reflecting the proportion of patients with a partial response or with stable disease, was 83%. The safety data obtained from all 42 patients enrolled to date was consistent with the results from previous clinical studies with derazantinib.

Dr. Marc Engelhardt, Chief Medical Officer of Basilea, said: "We are very pleased to have achieved this important milestone. The response rate and the safety profile at the time of the interim analysis are promising, especially considering the poor outcomes with chemotherapy in this group of patients reported in the literature. We are looking forward to the final data once the study is completed mid-2020."

He added: "The results of the interim analysis underscore the potential of derazantinib in the treatment of FGFR-driven tumors. As previously communicated, we are planning to extend the clinical development program of derazantinib, by starting a phase 2 study in other types of FGFR-driven solid tumors, mid-2019. We are also planning to expand the ongoing iCCA study by adding a separate cohort of patients with FGFR gene mutations to assess the potential expanded utility of derazantinib in the treatment of iCCA."

The ongoing registrational open-label phase 2 study[1] is expected to enroll up to 100 patients with inoperable or advanced iCCA expressing FGFR2 gene fusions. The patients receive once-daily oral derazantinib, to evaluate its anti-cancer activity with respect to objective response rate, progression free survival, overall survival and duration of response, and to further explore the safety and tolerability of the drug candidate. The additional cohort of iCCA patients whose tumors express FGFR gene mutations is expected to enroll approximately 50 patients.

Basilea Pharmaceutica Ltd. • Grenzacherstrasse 487 • PO Box • 4005 Basel • Switzerland • www.basilea.com

About derazantinib

Derazantinib (BAL087, formerly ARQ 087) is an investigational orally administered small molecule inhibitor of the FGFR family of kinases with strong activity against FGFR 1, 2, and 3. Therefore, it is called a panFGFR kinase inhibitor. FGFR kinases are key drivers of cell proliferation, differentiation and migration. FGFR alterations, e.g. gene fusions, overexpression or mutations, have been identified as potentially important therapeutic targets for various cancers, including iCCA, bladder, breast, gastric and lung cancers.[2] Current scientific literature suggests that FGFR alterations exist in a range of 5% to 30% in these cancers.[3] In iCCA, FGFR2 gene fusions have been reported in 13-22% of the cases[4, 5] and FGFR gene mutations have been reported in up to 5% of the cases.[3] Basilea in-licensed derazantinib from ArQule Inc. in April 2018. The drug candidate has demonstrated favorable clinical data in previous clinical studies, including a biomarker-driven Phase 1/2 study in iCCA patients.[6] Derazantinib has U.S. and EU orphan drug designation for this disease.

About intrahepatic cholangiocarcinoma (iCCA)

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.[7] 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. There is no proven effective treatment for patients who progress on first-line chemotherapy, thus there is a high unmet medical need.[8]

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 anti-infectives. 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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For further information, please contact:

Peer Nils Schröder, PhD
Head of Corporate Communications & Investor Relations
+41 61 606 1102
media_relations@basilea.com
investor_relations@basilea.com

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

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Basilea Pharmaceutica Ltd. • Grenzacherstrasse 487 • PO Box • 4005 Basel • Switzerland • www.basilea.com