

## PRESS RELEASE

# Basilea extends phase 2 study with derazantinib in intrahepatic bile duct cancer (iCCA) to include patients with FGFR2 gene mutations or amplifications

**Basel, Switzerland, July 01, 2019** – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that the first patient was enrolled in a newly opened cohort in the ongoing FIDES-01 phase 2 registrational study with the panFGFR kinase inhibitor derazantinib in intrahepatic cholangiocarcinoma (iCCA).<sup>1</sup> iCCA is affecting the part of the bile duct inside the liver and patients with advanced iCCA have very limited treatment options and a poor prognosis. The additional cohort is open for enrollment of approximately 40 patients with FGFR2 gene mutations or amplifications in their tumors. 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.

Dr. Marc Engelhardt, Chief Medical Officer, stated: "Based on preclinical models and clinical data, derazantinib may provide clinical benefit to patients with iCCA harboring a broad range of different FGFR2 aberrations, including gene fusions, mutations and amplifications, which are considered to be relevant oncogenic drivers. To date FGFR inhibitors have demonstrated clinical activity in FGFR2-fusion driven iCCA. Assessing the activity of derazantinib in a broader range of FGFR2-driven tumors is therefore important to further define the full therapeutic potential of derazantinib in iCCA."

The FIDES-01 (**F**ibroblast growth factor **I**nhibition with **D**erazantinib in **S**olid tumors) study is a multi-center, open-label phase 2 registrational study of once-daily oral derazantinib for the treatment of patients with inoperable or advanced iCCA and FGFR2 gene fusions or FGFR2 gene mutations or amplifications. In January 2019, a pre-planned interim analysis of the FGFR2 fusion-positive cohort of the study showed promising efficacy in this patient population and also confirmed the safety profile and tolerability of the drug candidate observed in previous clinical studies.<sup>2</sup> Topline data for the cohort of FGFR2 fusion-positive patients are expected to be available around mid-2020.

### About derazantinib

Derazantinib (formerly ARQ 087) is an investigational orally administered small molecule panFGFR kinase inhibitor with strong activity against FGFR1, 2, and 3. FGFR kinases are key drivers of cell proliferation, differentiation and migration. FGFR gene alterations, e.g. gene fusions, amplifications or mutations, have been identified as potentially important therapeutic targets for various cancers, including intrahepatic cholangiocarcinoma (iCCA), urothelial, breast, gastric and lung cancers.<sup>3</sup> In these cancers, FGFR gene alterations are found in a range of 5% to 30%.<sup>4</sup> FGFR2 gene fusions have been reported in 13-22% of iCCA cases.<sup>5, 6</sup> FGFR2 gene mutations and amplifications are less frequent and account for about 10% of FGFR2 genomic alterations in iCCA.<sup>6</sup> Overall, iCCA is accounting for 10-20% of all primary liver cancers.<sup>7</sup> Basilea in-licensed derazantinib from ArQule Inc. in April 2018. 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,<sup>2</sup> and has received U.S. and EU orphan drug designation for iCCA.

## 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.<sup>8</sup> 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.<sup>9</sup> There is no proven effective treatment for patients who progress on first-line chemotherapy, thus there is a high unmet medical need.<sup>10</sup>

## 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](http://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 <a href="mailto:media_relations@basilea.com">media_relations@basilea.com</a> <a href="mailto:investor_relations@basilea.com">investor_relations@basilea.com</a>
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This press release can be downloaded from [www.basilea.com](http://www.basilea.com).

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