# PRESS RELEASE

# Basilea at the 38th Annual J. P. Morgan Healthcare Conference – significant progress in Cresemba commercialization and R&D portfolio in 2019

***Basel, Switzerland, January 08, 2020*** – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that company management, including CEO David Veitch, will be participating in the 38th Annual J. P. Morgan Healthcare Conference in San Francisco, USA, from January 13-16, 2020.

The company made significant progress in the development of its clinical assets during 2019 and will continue to focus on delivering on its strategy for continued value creation in 2020. The key priorities for Basilea will remain on working with its partners to continuously grow "in-market" sales of its commercialized products, the antifungal Cresemba® (isavuconazole) and the antibiotic Zevtera® (ceftobiprole), in addition to advancing and expanding its oncology and infectious diseases R&D pipeline.

David Veitch commented: "In 2019, we have made significant progress with respect to both key pillars of our strategy; generating revenues from our commercialized products and building an R&D portfolio in oncology and infectious diseases to support sustained long-term growth. We are achieving increasing cash flows related to Cresemba and Zevtera. At the same time, we continue to place a major focus on prudently managing our operating expenses, which means that we are highly selective with regard to investments in our R&D portfolio. With respect to our clinical portfolio, we are focusing on clinical studies that have the potential for significant near- and mid-term value creation. The ongoing and planned studies for our key oncology compounds, derazantinib and lisavanbulin, are aiming to provide evidence for clinical differentiation and the ongoing phase 3 bacteremia study for ceftobiprole is the final study required to support a registration in the important US market. With respect to our earlier R&D portfolio, we continue to rely on both internal and external innovation, focusing on innovative small molecules that have the potential for a targeted development, for instance based on biomarker-driven patient selection."

**Product revenues**

Basilea's commercial partners doubled the number of countries where Cresemba is launched to 40. Total global Cresemba "in-market" sales by Basilea's partners reached approximately USD 190 million for the 12 months period ending September 30, 2019 (latest "in-market" data).[1] Basilea participates in the commercial success of its products through royalties, transfer prices and milestone payments; such as the two commercial milestones triggered in 2019 totaling USD 12 million payments from Pfizer Inc. based on sales of Cresemba in Pfizer's European license territory. By the end of 2021, Basilea expects that its partners will have launched Cresemba in about 60 countries.

**Oncology pipeline**

In 2019, Basilea reported positive interim results from the ongoing registrational phase 2 study with derazantinib in intrahepatic cholangiocarcinoma (iCCA), extended this study from FGFR2 gene fusions to gene mutations and amplifications and also started a new study in urothelial cancer as monotherapy and in combination with Roche's immune-checkpoint inhibitor, atezolizumab (Tecentriq®).[2, 3] Based on derazantinib's unique kinase inhibition profile, convincing pre-clinical in vivo data and the high medical need in the indication, Basilea is also planning to start a phase 1/2 study in gastric cancer by Q3 2020.



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Basilea has concluded patient enrolment into two early-stage clinical studies with lisavanbulin (formerly BAL101553). The studies explored once-daily oral lisavanbulin in patients with recurrent glioblastoma or high grade glioma (phase 1 study), and weekly 48-hour infusion in patients with recurrent glioblastoma and platinum-resistant ovarian cancer (phase 2a expansion study).[4, 5] Across the two studies, profound objective responses, with more than 80% reduction of the tumor area, were observed in two patients with glioblastoma, who continue to remain on treatment with lisavanbulin. Basilea therefore decided to advance the clinical development to a targeted, biomarker-driven phase 2 study in recurrent glioblastoma and potentially additional tumor types. The study is anticipated to start mid-2020. Glioblastoma is the most common type of primary brain cancer and one of the most lethal types of cancer.[6]

Finally, a phase 1 study is ongoing in the U.S. in newly diagnosed glioblastoma patients with first-line treatment of oral lisavanbulin in combination with radiotherapy.[7] This study could complete patient enrolment by mid-2020 and is conducted in collaboration with the Adult Brain Tumor Consortium (ABTC), which is funded by the U.S. National Cancer Institute.

The first-in-human phase 1 study with the panRAF/SRC kinase inhibitor BAL3833 in patients with advanced solid tumors did not result in the determination of a maximum tolerated dose.[8] Therefore, Basilea entered into pre-clinical reformulation work with the goal to achieve appropriately high and consistent drug exposure in patients. BAL3833 originates from The Institute of Cancer Research (ICR) in London, where it was developed by scientists funded by Cancer Research UK and the Wellcome Trust.

The company's oncology research portfolio includes several internal as well as externally sourced projects, all focused on the biomarker-driven development of potential first-in-class selective inhibitors of key processes in cancer development and progression.

**Anti-infectives pipeline**

Basilea has successfully completed the TARGET study, the first of the two cross-supportive phase 3 studies with ceftobiprole conducted with the goal to gain regulatory approval for the brand in the U.S.[9] The study explored ceftobiprole in the treatment of acute bacterial skin and skin structure infections (ABSSSI). The second study, ERADICATE, in patients with *Staphylococcus aureus* bacteremia (SAB), is ongoing and on track to report top-line results in the second half of 2021.[10] The U.S. market is estimated to represent more than 70% of the global market for novel, branded hospital antibiotics based on value and therefore it plays a critical role in Basilea's commercial strategy for ceftobiprole. Both studies are conducted under Special Protocol Assessments (SPAs) agreed with the FDA.

The phase 3 program for ceftobiprole is funded in part (up to USD 128 million, which is approximately 70% of the total estimated program costs) with Federal funds from the U.S. Department of Health and Human Services; Office of the Assistant Secretary for Preparedness and Response; Biomedical Advanced Research and Development Authority (BARDA), under Contract No. HHSO100201600002C.

The demand for innovative antibiotics and antifungals continues to be high. In order to address the needs of patients suffering from serious infections, Basilea focuses its research activities and external collaborations on new or unexploited clinically relevant targets and on compounds with the potential to demonstrate clinical superiority against established products.

## 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.

## Disclaimer

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

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