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Press release

Basilea reports on portfolio progress made in 2021

- **Achieved important Cresemba® (isavuconazole) and Zevtera® (ceftobiprole) milestones**
- **Added new clinical candidate BAL0891 to oncology pipeline and advanced clinical programs with derazantinib and lisavanbulin**

Basel, January 06, 2022

Basilea Pharmaceutica Ltd. (SIX: BSLN), a commercial-stage biopharmaceutical company committed to meeting the needs of patients with infectious diseases and cancer, provided today an update on the progress made with both its commercial brands and in its key research and development programs in 2021. The company achieved significant milestones and will continue to focus on delivering on its strategy for continued value creation in 2022.

David Veitch, Chief Executive Officer, commented: “Important milestones for our anti-infective brands included the marketing approval of Cresemba in China for the treatment for adult patients with invasive mucormycosis granted to our partner Pfizer and the filing of a marketing authorization application for isavuconazole in Japan by our partner Asahi Kasei Pharma. For Zevtera, we are expecting to complete patient enrolment into the phase 3 ERADICATE study this January. This will be an important milestone for a potential future U.S. market entry.” Topline results from the ERADICATE study are expected to become available around mid-year 2022.[1]

Significant progress has also been made in Basilea’s oncology pipeline, comprising the FGFR inhibitor derazantinib[2], the tumor checkpoint controller lisavanbulin, as well as the mitotic checkpoint inhibitor BAL0891, which was recently added to the clinical portfolio.

Dr. Marc Engelhardt, Chief Medical Officer, said: “Derazantinib is our most advanced oncology drug candidate. The positive final results for FGFR2 fusion-positive bile duct cancer from the FIDES-01 study with derazantinib published in September 2021 underscored the favorable benefit to risk profile of derazantinib in this indication.”

Anti-infectives key highlights

- In December 2021, Basilea’s partner Pfizer received a Drug Approval License for the oral formulation of Cresemba in China for the treatment of adult patients with invasive mucormycosis. This triggered a USD 10 million milestone payment to Basilea.
- In September 2021, Basilea’s partner Asahi Kasei Pharma filed a New Drug Application (NDA) for the marketing authorization of isavuconazole in Japan for the treatment of the

fungal infections: aspergillosis, mucormycosis and cryptococcosis. The filing is based on a phase 3 study completed in early 2021.[3] It triggered a CHF 5 million milestone payment to Basilea. A decision on the NDA by the Japanese health authorities is expected in H2 2022.

- In July 2021, Basilea entered into an agreement with Moscow-based pharmaceutical company JSC Lancet for the distribution of Zevtera in Russia, as well as in the other countries of the Eurasian Economic Union.
- In May 2021, Basilea was awarded a grant of up to USD 2.7 million from CARB-X (Combating Antibiotic-Resistant Bacteria Biopharmaceutical Accelerator) for the development of selective inhibitors of the bacterial enzyme DXR. This enzyme is essential for the survival of many Gram-negative bacteria and has not yet been exploited as an antibacterial target.

The continued strong commercial performance of Cresemba throughout 2021 triggered several sales milestone payments by its partners to Basilea. By year-end 2021, Cresemba was approved in more than 60 countries and marketed in 55 countries, including the United States, most EU member states and additional countries inside and outside of Europe. In the twelve-month period between October 2020 and September 2021, total in-market sales of Cresemba amounted to more than USD 300 million, a 26.5 percent growth year-on-year.[4]

Oncology key highlights

- In December 2021, the U.S. Food and Drug Administration (FDA) approved the Investigational New Drug (IND) application for starting clinical studies with the novel TTK/PLK1 kinase inhibitor BAL0891. The drug candidate is a first-in-class mitotic checkpoint inhibitor (MCI) that drives aberrant tumor cell division leading to tumor cell death. A phase 1 study in patients with advanced solid tumors is planned to start in the first quarter of 2022.
- In September 2021, the full safety and efficacy data set from the first cohort of the phase 2 study FIDES-01 was presented at the ESMO congress. This cohort explored derazantinib as monotherapy in patients with FGFR2 fusion-positive intrahepatic cholangiocarcinoma (iCCA).[5] The data confirmed the efficacy of derazantinib and supported its differentiation in iCCA to other FGFR inhibitors related to safety and tolerability. Initial topline results from this cohort were reported in February 2021.
- In July 2021, the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to lisavanbulin for the treatment of malignant glioma, including glioblastoma. Orphan Drug Designation qualifies the sponsor of the drug for various incentives, including extended regulatory market exclusivity.
- In March 2021, positive interim results from the second cohort of FIDES-01 were published. In this cohort, derazantinib is explored as monotherapy in iCCA patients with FGFR2 gene mutations and amplifications.

- In February 2021, data on the safety, tolerability and preliminary signals of efficacy of derazantinib in combination with the PD-L1 checkpoint inhibitor atezolizumab in patients with advanced solid tumors from the phase 1b dose-finding cohort in the FIDES-02 study were presented at the ASCO Genitourinary Cancers Symposium. The derazantinib-atezolizumab combination was well tolerated and no dose-limiting toxicities were observed.

Multiple data readouts are expected throughout 2022 that will determine the direction for the further development of Basilea's oncology drug candidates.

Topline derazantinib results for bile duct cancer (iCCA) patients with other FGFR2 genetic aberrations than fusions from the FIDES-01 study are expected in the first half of 2022 and Basilea also expects initial results from the ongoing urothelial and gastric cancer studies, FIDES-02 and FIDES-03, in the first half of 2022.[6, 7]

Interim results from the ongoing lisavanbulin phase 2 study with EB1-positive glioblastoma patients are expected in the first half of 2022.[8] EB1, end-binding protein 1, was previously identified as a potential response-predictive biomarker with long-lasting clinical benefit observed in two patients with recurrent EB1-positive glioblastoma, who participated in the program since the phase 1 portion of the study.

The second ongoing study with lisavanbulin is conducted in the U.S. in collaboration with the Adult Brain Tumor Consortium, ABTC, exploring the combination with radiotherapy in patients with newly diagnosed glioblastoma.[9] Dose-escalation continues based on the combination's tolerability and safety profile observed so far. The maximum tolerated dose has not yet been reached and Basilea is now expecting the recommended phase 2 dose to be established in this patient population in the first half of 2022.

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 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 several 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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