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Press release

Basilea provides corporate update

- **Key oncology clinical studies with derazantinib and lisavanbulin remain on track**
- **FDA approves protocol amendment for the phase 3 ERADICATE bacteremia study with ceftobiprole to include a broader spectrum of severely ill patients**
- **No negative COVID-19 impact expected on global prescriptions of Cresemba® and Zevtera®**
- **Early R&D portfolio prioritization**

Basel, Switzerland, May 28, 2020

Basilea Pharmaceutica Ltd. (SIX: BSLN) provided a general corporate update today. The company does not expect a material impact on the timelines of ongoing or planned oncology studies with the FGFR kinase inhibitor derazantinib and the planned phase 2a biomarker driven study with its tumor checkpoint controller, lisavanbulin, due to the coronavirus pandemic. The impact on the ongoing ceftobiprole phase 3 study remains limited, with patient enrolment timelines potentially extended by up to a quarter.

David Veitch, Chief Executive Officer of Basilea, said: “We have assessed the potential impact of the coronavirus pandemic on our business based on the information available to date. We are pleased to report that we do not currently anticipate an impact on our key oncology clinical studies with our most advanced compounds derazantinib and lisavanbulin. As the global healthcare community is prioritizing measures against coronavirus infections, we expect a limited impact of up to a quarter on the timelines for our ceftobiprole phase 3 study. As previously reported, we have no indication from our commercial partners of any negative impact on global prescriptions for both Cresemba and Zevtera, our two marketed brands. The continued strong market demand is also reflected in the 30 percent year-on-year growth in U.S. Cresemba sales as reported by our license partner Astellas, mid-May, with sales of 155 million U.S. dollars for the period April 2019 to March 2020.”

For Basilea’s antibiotic ceftobiprole, the U.S. Food & Drug Administration (FDA) has recently approved a protocol amendment for the phase 3 study ERADICATE, to progress the study to the pre-planned second cohort and extend the maximum treatment duration from four to up to six weeks. ERADICATE explores intravenous ceftobiprole for the treatment of patients with *Staphylococcus aureus* bacteremia (SAB), a type of bacterial bloodstream infection, in comparison to intravenous daptomycin, with or without intravenous aztreonam.[1, 2] The overall target patient enrolment number in the study remains unchanged.

Dr. Marc Engelhardt, Chief Medical Officer of Basilea, said: “We are very satisfied that the study progresses as planned to its next stage. The possibility for an extended treatment duration is important as it enables us now to expand enrolment to patients with more difficult-to-treat infections, including those with complications such as osteomyelitis and epidural or cerebral abscess.”

In its continued effort to optimize resource allocation across its portfolio, Basilea has taken several decisions with respect to its earlier stage R&D portfolio. Basilea has prioritized two potential first-in-class oncology programs, these are expected to potentially enter pre-clinical, IND-enabling studies in the next 12 months. At the same time, it will discontinue the development of the panRAF/SRC kinase inhibitor BAL3833, which was developed by scientists at The Institute of Cancer Research (ICR) in London, funded by Cancer Research UK and the Wellcome Trust, and in-licensed by Basilea in 2015. In 2018, a first-in-human phase 1 dose-escalation study of BAL3833 was completed by the ICR in conjunction with The Christie and Royal Marsden NHS Foundation Trusts and The Cancer Research UK Manchester Institute at The University of Manchester. Basilea had been conducting pre-clinical activities to explore alternative formulations of BAL3833. In addition, it has decided to discontinue one other, externally sourced, pre-clinical oncology project.

About derazantinib

Derazantinib is an investigational orally administered small-molecule FGFR kinase 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 previous clinical studies, including a 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 two clinical studies with derazantinib. The first study, FIDES-01, is a registrational 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.[11] The second study, FIDES-02, is a phase 1/2 study evaluating derazantinib alone and in combination with Roche's PD-L1-blocking immune-checkpoint inhibitor atezolizumab (Tecentriq®) in patients with advanced urothelial cancer, including metastatic, or recurrent surgically unresectable disease, expressing FGFR

genetic aberrations.[12] Basilea in-licensed derazantinib from ArQule Inc, a wholly-owned subsidiary of Merck & Co., Inc., Kenilworth, N.J., U.S.A.

About lisavanbulin (BAL101553)

Basilea's oncology drug candidate lisavanbulin,BAL101553, (the prodrug of BAL27862)[13] is being developed as a potential therapy for diverse cancers.[14, 15, 16] In pre-clinical studies, lisavanbulin demonstrated in-vitro and in-vivo activity against diverse treatment-resistant cancer models, including tumors refractory to conventional approved therapeutics and radiotherapy.[17, 18, 19] Lisavanbulin efficiently distributes to the brain, with anticancer activity in glioblastoma models.[20, 21, 22] In pre-clinical studies, end-binding protein 1 (EB1) was identified as a potential response-predictive biomarker in glioblastoma models.[22] The active moiety BAL27862 binds to the colchicine site of tubulin, with distinct effects on microtubule organization,[23] resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.[24]

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.

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

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