PRESS RELEASE

Basilea plans progression of oncology candidate lisavanbulin to targeted, biomarker-driven phase 2 study

- **Confirmed signals of efficacy with profound responses in two patients with glioblastoma across two different clinical studies**
- **Manageable, well-characterized safety profile**
- **Plan to proceed with targeted, biomarker-driven oral expansion study in mid-2020**

Basel, Switzerland, December 16, 2019 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that it plans to advance the clinical development of its novel tumor checkpoint controller lisavanbulin (BAL101553) by focusing on a targeted, biomarker-driven approach based on the initial results from the two recent phase 1/2 clinical studies.

The phase 1 study (NCT02490800) with daily oral lisavanbulin with recurrent glioblastoma (GBM), or high grade glioma, was completed in August 2019. Basilea has now also completed an interim data review of the ongoing open-label phase 2a expansion study using weekly 48-hour intravenous (i.v.) administration in twelve patients with recurrent GBM and nine patients with platinum-resistant ovarian cancer (NCT02895360).

Glioblastoma is the most common type of primary brain cancer and one of the most lethal types of cancer.[1]

Across the two studies, profound objective responses, with more than 80% reduction of the GBM tumor area, were observed in two patients with glioblastoma, who continue to remain on treatment with lisavanbulin. In the ovarian cancer group, four patients showed reduction in target lesion size but did not meet the formal response criteria of the study protocol. The safety profile observed with daily oral or weekly 48-hour i.v. lisavanbulin was consistent with previous studies. Basilea plans to submit the data for presentation at upcoming scientific conferences.

Dr. Marc Engelhardt, Basilea's Chief Medical Officer, said: "We have observed clinical activity of lisavanbulin in recurrent glioblastoma, with profound clinical responses seen in a subset of patients. Based on this observation, we are investigating a panel of biomarkers, including end-binding protein 1, EB1, which could be useful for identifying cancer patients that may benefit most from the treatment with lisavanbulin. This allows us to take a targeted approach for advancing the clinical development of lisavanbulin, moving from the evaluation of an unselected patient population to a biomarker-driven phase 2 study in recurrent glioblastoma and potentially additional tumor types. Additionally, we currently intend to focus on the oral formulation in the next stage of clinical development of lisavanbulin given the consistent outcome observed with both formulations."

There will be no additional patients enrolled in the weekly 48-hour i.v. infusion study. All ongoing patients will remain on treatment as long as they continue to benefit from treatment.

EB1 was previously identified by Basilea as a potential response-predictive biomarker for lisavanbulin, based on comprehensive preclinical studies in glioblastoma models. Initial clinical

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evidence was then provided in August 2019, when Basilea announced the completion of patient enrolment into the phase 1 study with daily oral lisavanbulin in recurrent glioblastoma or high-grade glioma.[2] One glioblastoma patient in that study, whose tumor tissue was strongly positive for EB1, was reported as an exceptional long-lasting responder.[3]

In the U.S., a phase 1 study is being conducted in collaboration with the Adult Brain Tumor Consortium (ABTC), in which oral lisavanbulin is evaluated in combination with radiotherapy in patients with newly diagnosed glioblastoma and a reduced sensitivity to chemotherapy with the standard-of-care drug temozolomide.[4]

About lisavanbulin (BAL101553)

Basilea's oncology drug candidate lisavanbulin (BAL101553, the prodrug of BAL27862)[5] is being developed as a potential therapy for diverse cancers.[2, 4, 6] In preclinical studies, lisavanbulin demonstrated in-vitro and in-vivo activity against diverse treatment-resistant cancer models, including tumors refractory to conventional approved therapeutics and radiotherapy.[7, 8, 9] Lisavanbulin efficiently distributes to the brain, with anticancer activity in glioblastoma models.[10, 11, 12] In preclinical studies, end-binding protein 1 (EB1) was identified as a potential response-predictive biomarker in glioblastoma models.[12] The active moiety BAL27862 binds to the colchicine site of tubulin, with distinct effects on microtubule organization,[13] resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.[14]

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

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