PRESS RELEASE

Basilea announces completion of patient enrolment in phase 1 study with oral BAL101553 in brain cancer

Basel, Switzerland, August 27, 2019 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today the completion of patient enrolment in its phase 1 study with the oral formulation of the novel tumor checkpoint controller BAL101553 in brain cancer patients.[1]

The study included 28 patients with progressive or recurrent glioblastoma (GBM), the most aggressive primary brain tumor,[2] or high-grade glioma. Patients received once-daily oral BAL101553. The maximum tolerated dose (MTD) was determined at 30 mg per day. Dose-limiting adverse events included gait disturbances, hallucinations and confusion, which were all reversible.
Dose levels up to and including 25 mg per day were well tolerated. Clinical antitumor activity of BAL101553 was shown with one exceptional, long-lasting responder still on treatment, whose brain tumor tissue displayed strong expression of the potentially response-predictive biomarker EB1 (end-binding protein 1). In addition, five further patients experienced stable disease as a best response.[3]

Dr. Marc Engelhardt, Basilea's Chief Medical Officer, said: "The results from the phase 1 study in brain cancer patients indicate a manageable safety and tolerability profile, which is consistent with the safety profile observed for BAL101553 in the treatment of other advanced solid tumors. The observation of an exceptional, durable response in a GBM patient whose tissue was strongly positive for EB1 is encouraging, especially as we had previously identified EB1 as a potential response-predictive biomarker for BAL101553 based on comprehensive preclinical studies in GBM models. We are now assessing the potential utility of EB1 to support a biomarker-driven clinical study with BAL101553 in GBM and other cancer types. This would subsequently allow using BAL101553 as a targeted therapy in patients whose tumors show high EB1 expression."

About BAL101553

Basilea's oncology drug candidate BAL101553 (the prodrug of BAL27862)[4] is being developed as a potential therapy for diverse cancers. In addition to the study described in this press release, there are two more clinical studies ongoing. In Switzerland, a phase 2a expansion study is exploring the drug in recurrent glioblastoma and platinum-resistant ovarian cancer patients using weekly 48-hour infusion.[5] In the U.S., a phase 1 study is being conducted in collaboration with the Adult Brain Tumor Consortium (ABTC), in which BAL101553 is explored in combination with radiotherapy in patients with newly diagnosed glioblastoma who have a reduced sensitivity to chemotherapy with the standard-of-care drug temozolomide.[6] In preclinical studies, BAL101553 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] BAL101553 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

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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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