PRESS RELEASE

Basilea starts clinical phase 2a expansion with BAL101553 in ovarian cancer and glioblastoma

Basel, Switzerland, June 26, 2018 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that the first patient has been dosed in the phase 2a expansion part of Basilea's ongoing phase 1/2a clinical study with its novel tumor checkpoint controller BAL101553, administered as weekly 48-hour intravenous (i.v.) infusion. The study is exploring BAL101553 in patients with recurrent glioblastoma and in patients with platinum-resistant or refractory ovarian cancer to further characterize the safety and tolerability and to obtain efficacy data in these selected cancer types.

Glioblastoma is the most common type of primary brain cancer and one of the most lethal types of cancer.[1] There is also a high medical need in ovarian cancer as recurrence after initial therapy is common and patients with platinum-resistant cancers have limited treatment options.[2]

Dr. Marc Engelhardt, Chief Medical Officer, said: "The start of the phase 2a expansion study marks a significant milestone towards establishing clinical proof-of-concept for our biomarker-driven development strategy with our novel tumor checkpoint controller BAL101553. There are only very limited treatment options available for patients with recurrent glioblastoma and for patients with platinum-resistant or platinum-refractory ovarian cancer. Our decision to explore the potential clinical benefit in these specific patient populations is based on the results from our phase 1 studies and our comprehensive non-clinical profiling."

The phase 2a single-agent, open label study is anticipated to include up to 40 patients with platinum-resistant or platinum-refractory ovarian cancer and recurrent glioblastoma and will be conducted in a number of hospitals in Switzerland. Additional information on this clinical study is available at www.clinicaltrials.gov (identifier: NCT02895360).

In January, Basilea reported the completion of the dose-escalation in advanced solid tumor patients in two phase 1/2a studies with once-daily oral and weekly 48-hour i.v. administration of BAL101553 as a single-agent therapy. The maximum tolerated doses for these dosing regimens in solid tumor patients have been established. Data from these studies were presented at the annual conference of the American Society of Clinical Oncology (ASCO) in June.

Two additional studies with BAL101553 are ongoing: dose-escalation in a separate recurrent glioblastoma arm of the phase 1/2a once-daily oral single-agent study and a phase 1 study in newly diagnosed glioblastoma patients evaluating once-daily oral BAL101553 in combination with standard radiotherapy, which is conducted in collaboration with the Adult Brain Tumor Consortium (ABTC) in the U.S.

About BAL101553

Basilea's oncology drug candidate BAL101553 (the prodrug of BAL27862)[3] is being developed as a potential therapy for diverse cancers. The drug candidate is currently in phase 1/2a clinical evaluation. One study has two separate arms and evaluates once-daily oral BAL101553 in advanced solid tumors (dose-escalation completed) and recurrent glioblastoma (ongoing). A second study (phase 2a expansion) is evaluating BAL101553 as a weekly 48-hour i.v. infusion in recurrent glioblastoma and in platinum-resistant or refractory ovarian cancer. An additional phase 1 study is evaluating oral BAL101553 in combination with standard radiotherapy in

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patients with newly-diagnosed glioblastoma which have a reduced sensitivity to standard chemotherapy. In preclinical studies, the drug candidate demonstrated *in vitro* and *in vivo* activity against diverse treatment-resistant cancer models, including tumors refractory to conventional approved therapeutics and radiotherapy.[4, 5, 6] BAL101553 efficiently distributes to the brain, with anticancer activity in glioblastoma models.[7, 8, 9] The active moiety BAL27862 binds the colchicine site of tubulin with distinct effects on microtubule organization,[10] resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.[11]

About Basilea

Basilea Pharmaceutica Ltd. is a commercial stage biopharmaceutical company developing products that address the medical challenge of increasing resistance and non-response to current treatment options in the therapeutic areas of bacterial infections, fungal infections and cancer. 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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