

PRESS RELEASE

Basilea reports start of clinical phase 1 study in collaboration with the U.S. Adult Brain Tumor Consortium to explore BAL101553 in newly diagnosed glioblastoma

Basel, Switzerland, January 03, 2018 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today the initiation of a phase 1 study conducted under its clinical study agreement with the Adult Brain Tumor Consortium (ABTC) in the U.S. The first patient has been treated in this open-label dose-escalation study to determine the safety and tolerability of the oral formulation of Basilea's novel anticancer drug candidate BAL101553 in combination with standard radiation in patients with newly-diagnosed glioblastoma. Patients participating in the study have a reduced sensitivity to standard chemotherapy with temozolomide due to an unmethylated MGMT promoter.

Ronald Scott, Chief Executive Officer, commented, "Glioblastoma patients have limited therapeutic options and new treatment opportunities are urgently needed. This is especially the case for patients with reduced sensitivity to standard chemotherapy. We are very pleased that this clinical study which includes patients with reduced sensitivity to standard agents has commenced in collaboration with the ABTC."

Glioblastoma is the most common type of primary brain tumor and one of the most lethal types of cancer. MGMT promoter methylation status is an important molecular genetic biomarker in glioblastoma. Median survival of about 22 months from diagnosis has been reported for adult glioblastoma patients with a methylated MGMT promoter receiving standard-of-care chemotherapy/radiation combination treatment.^{1,2} Patients with an unmethylated MGMT promoter receiving the same treatment have a worse prognosis and a reported median survival of only about 13 months.¹ It is estimated that approximately 55% of newly diagnosed glioblastoma patients have an unmethylated MGMT promoter.¹

The study is performed at member sites of the ABTC in the United States, coordinated by the Johns Hopkins University's School of Medicine. The ABTC is funded by the U.S. National Cancer Institute (NCI).

Update on ongoing phase 1 programs with BAL101553

Basilea is already conducting two further open-label phase 1/2a clinical studies to explore different dosing regimens of BAL101553 in patients with advanced solid tumors, one study with weekly 48-hour continuous infusion and the other with once-daily oral dosing. The oral study was amended in late 2016 to also enroll patients with recurrent or progressive glioblastoma. Phase 1 recruitment of patients in the solid tumor part of the oral study and the 48-hour continuous infusion study has been completed and the Maximum Tolerated Doses (MTDs) have been established. Dose-limiting adverse events observed in both studies included reversible hallucinations and reversible hyponatremia (low sodium levels). Basilea plans to present the phase 1 results at upcoming scientific conferences. Basilea expects to complete phase 1 patient recruitment into the separate glioblastoma arm of the oral study in the first half of 2018 and is finalizing its strategy for exploring specific patient populations in an expansion of the 48-hour continuous infusion phase 1/2a study.

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. Two studies are conducted in patients with advanced solid tumors where BAL101553 is given orally or as a 48-hour continuous infusion. The oral study includes a separate glioblastoma (brain cancer) arm. An additional study is evaluating oral BAL101553 in combination with standard radiation in patients with newly-diagnosed glioblastoma. 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. 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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