

PRESS RELEASE

Basilea announces clinical study agreement with Adult Brain Tumor Consortium to explore BAL101553 in newly diagnosed glioblastoma

- **The Adult Brain Tumor Consortium (ABTC) is designed to develop more effective treatments for malignant brain tumors. It is funded by the US National Cancer Institute (NCI).**
- **The ABTC will conduct a clinical phase 1 study to determine the safety and tolerability of Basilea's novel tumor checkpoint controller BAL101553 in newly diagnosed glioblastoma patients.**

Basel, Switzerland, June 12, 2017 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that it has entered into a clinical study agreement with the Adult Brain Tumor Consortium (ABTC). The consortium will conduct a clinical phase 1 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 who have a reduced sensitivity to standard chemotherapy due to an unmethylated MGMT promoter. MGMT promoter methylation status is an important molecular genetic biomarker in glioblastoma.

Prof. Achim Kaufhold, Chief Medical Officer of Basilea, said: "There is a critical medical need for new agents in the treatment of patients with glioblastoma with an unmethylated MGMT promoter. Today these patients have fewer therapeutic options than those with a methylated MGMT promoter and a worse disease prognosis. We are very pleased to be working with the ABTC to potentially provide an urgently needed new treatment modality for fighting brain cancer in this patient group."

The ABTC is designed to develop more effective treatments for malignant brain tumors. It has 11 brain tumor centers at leading universities across the United States. It is funded by the US National Cancer Institute (NCI).

Glioblastoma is the most common primary brain tumor and one of the most lethal types of cancer. The incidence of glioblastoma is approximately 3 patients per 100,000 in the United States.¹ Median survival of about 15 months from diagnosis has been reported for adult glioblastoma patients receiving standard-of-care treatment,² with a 5-year survival rate of 5%.¹ It is estimated that approximately 55% of newly diagnosed glioblastoma patients have an unmethylated MGMT promoter, and these patients have a worse prognosis than those with a methylated MGMT promoter.³

The dose escalation phase 1 study will be conducted at ABTC member sites in the United States, coordinated by the Johns Hopkins University's School of Medicine. The majority of the study costs will be borne by the ABTC. The study is anticipated to start in the third quarter of 2017.

About BAL101553

Basilea's small molecule oncology drug candidate BAL101553 (the prodrug of BAL27862)⁴ is being developed as a potential therapy for diverse cancers. BAL101553 is currently undergoing clinical phase 1/2a evaluation in patients with advanced solid tumors or glioblastoma (brain

cancer). 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.^{5, 6, 7} BAL101553 efficiently distributes to the brain, with anticancer activity in glioblastoma models.^{8, 9, 10} The active moiety BAL27862 binds the colchicine site of tubulin with distinct effects on microtubule organization,¹¹ resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.¹²

About Basilea

Basilea Pharmaceutica Ltd. is a 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 uses the integrated research, development and commercial operations of its subsidiary Basilea Pharmaceutica International Ltd. to discover, develop and commercialize 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.

About ABTC

The Adult Brain Tumor Consortium (ABTC) is a multi-institutional consortium, consisting of investigators at renowned institutions across the United States. It is funded by the US National Cancer Institute (NCI). The consortium performs innovative, multidisciplinary phase 1 - 2 clinical trials that focus predominantly on adult patients with grade IV gliomas (glioblastoma multiforme). The ABTC has demonstrated that clinical trials are not only possible in this challenging tumor type, but represent the best hope for making further progress against this devastating disease. For more information visit www.abtconsortium.org.

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For further information, please contact:

Peer Nils Schröder, PhD Head of Corporate Communications & Investor Relations +41 61 606 1102 media_relations@basilea.com investor_relations@basilea.com

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

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