

## PRESS RELEASE

# Basilea expands oncology drug candidate BAL101553 clinical phase 1/2a oral study to include glioblastoma patients

**Basel, Switzerland, December 02, 2016** – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today the expansion of its ongoing oncology drug candidate BAL101553 clinical phase 1/2a oral formulation study. The first patient has been dosed in an additional arm containing adult patients with recurrent or progressive glioblastoma (brain cancer) after prior radiotherapy with or without chemotherapy.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, commented: "We are excited to explore BAL101553 in a separate glioblastoma study arm to our ongoing phase 1/2a clinical study. Glioblastoma is a cancer indication associated with high medical need. There are limited treatment options for glioblastoma patients due in part to the challenge of getting medication into the brain through the blood brain barrier. BAL101553 has been shown to enter the brain and has demonstrated anticancer activity with oral dosing in various preclinical models of glioblastoma, including models refractory to or with reduced sensitivity to standard therapies."

In addition to the activity in glioblastoma tumor lines, BAL101553 was shown to have potent anticancer activity against glioblastoma stem-like cells in a pre-clinical model as reported in a recent publication co-authored by Basilea and the research group of Prof. Diane Braguer of Aix-Marseille University, France. Tumor stem-like cells contribute to glioblastoma regrowth as well as brain invasion, a phenomenon which also occurs in the pre-clinical model used. The publication further reported the observation that BAL101553 promoted the loss of stem-cell properties. These published data further support the potential of BAL101553 to target glioblastoma, a tumor often associated with poor prognosis for patients.<sup>1</sup>

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.<sup>2</sup> Median survival of about 15 months from diagnosis has been reported for adult glioblastoma patients receiving standard-of-care treatment,<sup>3</sup> with a 5-year survival rate of 5%.<sup>2</sup>

The ongoing phase 1/2a study includes patients with advanced or recurrent solid tumors who have failed standard therapy or for whom no effective standard therapy was available. Phase 1 dose escalation to determine the maximum tolerated dose (MTD) of daily oral dosing is currently ongoing. A subsequent phase 2a extension of the study is planned to further evaluate the safety, tolerability and the pharmacokinetic profile of oral BAL101553 at the MTD, and to assess its anti-tumor activity. Furthermore, biomarkers are assessed in both the phase 1 and phase 2a parts of the study to determine their utility in identifying patients who are most likely to respond to treatment, including biomarkers with potential relevance to glioblastoma.

### About BAL101553

Basilea's small molecule oncology drug candidate BAL101553 (the prodrug of BAL27862)<sup>4</sup> is being developed as a potential therapy for diverse cancers. BAL101553 is currently undergoing clinical phase 1/2a evaluation (oral and continuous infusion) in patients with advanced solid tumors. 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.<sup>5, 6, 7</sup> BAL101553 efficiently distributes to the brain, with

anticancer activity in glioblastoma (brain cancer) models.<sup>1, 8, 9</sup> The active moiety BAL27862 binds the colchicine site of tubulin with distinct effects on microtubule organization,<sup>10</sup> resulting in the formation of the "spindle assembly checkpoint" which promotes tumor cell death.<sup>11</sup>

## About Basilea

Basilea Pharmaceutica Ltd. is a biopharmaceutical company developing products that address 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 potentially 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](http://www.basilea.com).

## Disclaimer

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This press release can be downloaded from [www.basilea.com](http://www.basilea.com).

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