

## PRESS RELEASE

# Basilea starts continuous infusion phase 1/2a clinical study with oncology drug candidate BAL101553

**Basel, Switzerland, September 6, 2016** – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that the first patient has been dosed in a new phase 1/2a continuous infusion study with its tumor checkpoint controller BAL101553 in patients with advanced solid cancers. Continuous infusion with portable pumps is an established mode of drug administration used for the treatment of certain cancers.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, commented: "We are excited to start this phase 1/2a study with our novel tumor checkpoint controller BAL101553. Continuous infusion could provide additional administration flexibility beyond daily oral administration and weekly 2-hour intravenous (i.v.) infusion. In cancer therapy, it is important to offer maximum administration flexibility in order to optimize treatment for specific tumor types and to synchronize treatment schedules in combination with other cancer therapies."

The open-label, multicenter study is being conducted in Switzerland. It includes adult patients with advanced solid tumors who failed standard therapy or for whom no effective standard therapy is available. Study participants receive i.v. BAL101553 administered as 48-hour continuous infusions.

### About BAL101553

Basilea's small molecule oncology drug candidate BAL101553 (the prodrug of BAL27862)<sup>1</sup> is being developed as a potential therapy for diverse cancers, including tumor types unresponsive to standard therapeutics. BAL101553 is currently undergoing clinical phase 1/2a evaluation in patients with advanced solid tumors. It has shown evidence of clinical anti-tumor activity in a phase 1/2a study with weekly 2-hour i.v. dosing, during which the maximum tolerated dose and the recommended phase 2 dose were established. In addition to the newly initiated 48-hour continuous intravenous infusion phase 1/2a study, BAL101553 is also currently being investigated in a dose-escalation phase 1/2a study with an once-daily oral formulation. 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>2, 3, 4</sup> BAL101553 efficiently distributes to tumors and to the brain, with anticancer activity in glioblastoma (brain cancer) models.<sup>5, 6</sup> The active moiety BAL27862 binds the colchicine site of tubulin with distinct effects on microtubule organization<sup>7</sup>, resulting in the formation of the "spindle assembly checkpoint" which promotes tumor cell death.<sup>8</sup> Basilea's approach to oncology includes the early evaluation of potential biomarkers, which are already being tested in phase 1/2a clinical studies in order to optimize dose selection and identify cancer patient groups more likely to respond.

### 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).

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

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