

## PRESS RELEASE

# Basilea initiates phase 1/2a oncology study with oral formulation of tumor checkpoint controller BAL101553

**Basel, Switzerland, June 25, 2015** – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today the start of a clinical phase 1/2a study with an oral formulation of its anti-cancer drug candidate BAL101553. BAL101553 is a microtubule-destabilizing small molecule, acting as tumor checkpoint controller (TCC) as it promotes tumor cell death through activation of an important checkpoint in cell proliferation.

The primary objective of the phase 1 study is to determine the maximum tolerated dose (MTD) in adult patients with advanced or recurrent solid tumors who have failed standard therapy or for whom no effective standard therapy is available. BAL101553 capsules will be administered once daily. A subsequent phase 2a extension of the study is planned to further evaluate the safety, tolerability and the pharmacokinetic profile of daily oral BAL101553 at the MTD, and to assess its anti-tumor activity. Furthermore, biomarkers will be explored in both the phase 1 and phase 2a parts of the study to determine their potential utility in identifying patients who are most likely to respond to treatment.

An intravenous (i.v.) formulation of BAL101553 is currently being investigated in a separate phase 2a clinical study in cancer patients with advanced solid tumors.

“Oral bioavailability of our anti-cancer compound BAL101553 may provide advantages complementary to the i.v. formulation,” said Prof. Achim Kaufhold, Basilea’s Chief Medical Officer. “The availability of an oral formulation of BAL101553 offers increased flexibility with regard to both single agent and drug combination treatment strategies across different tumor types and thus could potentially broaden the options for the clinical development of this anti-cancer drug candidate.”

### About tumor checkpoint controller BAL101553

BAL101553 is a water-soluble prodrug of Basilea’s small molecule oncology drug candidate BAL27862. The prodrug is available in oral and intravenous (i.v.) formulations. The investigational drug is being developed for the treatment of diverse cancers, including tumor types unresponsive or resistant to standard therapeutics. BAL101553 showed first evidence of clinical anti-tumor activity in the phase 1 part of an ongoing phase 1/2a i.v. study in solid tumor cancer patients.<sup>1</sup>

BAL101553 destabilizes the microtubule network involved in cell proliferation and has demonstrated its ability to stop tumor growth by a dual action on tumor cell proliferation and tumor vascularization through a mechanism different from conventional microtubule-targeting agents such as taxanes.

Data presented at the 2015 American Association of Cancer Research (AACR) Annual Meeting in Philadelphia (USA) show that BAL101553 induces the formation of the spindle assembly checkpoint (SAC), which promotes tumor cell death when the microtubule spindle is not properly formed.<sup>2</sup> Furthermore, the presence of SAC components, including BubR1, was shown to be associated with response to BAL101553 in a range of tumor models. The data presented at AACR confirmed that the sensitivity of human cancer cell lines to BAL101553 correlates with the presence of an intact SAC, supporting the hypothesis that prediction of cancer response to BAL101553 may lay in its tumor checkpoint control function. The utility of SAC components as

predictive biomarkers to identify patients likely to respond to BAL101553 is being explored in the clinical program.

## 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 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).

## References

- 1 L. R. Molife et al. Phase 1/2a trial of the novel microtubule inhibitor BAL101553 in advanced solid tumors: Phase 1 completed. American Society of Clinical Oncology (ASCO) annual meeting 2014, abstract 2562
- 2 F. Bachmann, K. Burger, H. Lane. BAL101553 (prodrug of BAL27862): the spindle assembly checkpoint is required for anticancer activity. American Association for Cancer Research (AACR) annual meeting 2015, abstract 3789