PRESS RELEASE

Data presented at AACR meeting indicate diverse tumor-targeting properties of Basilea's BAL101553 and show a combination potential with bevacizumab

- **BAL101553 targets tumor cell proliferation, adaptation to low oxygen and vascularization**
- **Switch from weekly to daily oral dosing leads to reduced tumor anti-vascular activity while maintaining tumor growth inhibition**
- **Combination with the anti-VEGF therapy bevacizumab shows enhanced anti-tumor response**

Basel, Switzerland, April 06, 2017 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today the presentation of preclinical data on the anti-tumor activity of its clinical oncology drug candidate BAL101553 as a late-breaking abstract at the annual meeting of the American Association for Cancer Research (AACR) in Washington DC, April 1-5, 2017. The drug candidate was studied both as monotherapy and in combination with bevacizumab (Avastin®).

Basilea's small molecule tumor checkpoint controller BAL101553, currently in clinical phase 1/2a development, binds to tubulin at a site not targeted by any approved microtubule-targeting agent (MTA). This leads to tumor cell death by activation of the spindle assembly checkpoint and promotes disruption of the tumor vasculature. The preclinical data presented at the AACR meeting demonstrated that sub-cytotoxic concentrations of the active drug also directly interfered with the adaptation of tumor cells to oxygen deprivation. This resulted in reduced secretion of Vascular Endothelial Growth Factor (VEGF), a key mediator for the formation of new blood vessels by tumors, which was shown to effect vascular organization. This new data provides evidence of another mechanism that may contribute to BAL101553's anti-cancer activity, in addition to its effect on the spindle assembly checkpoint. Moreover, using a tumor model highly resistant to standard MTAs, the combination of daily oral or weekly intravenous (i.v.) BAL101553 with the monoclonal anti-VEGF antibody bevacizumab showed enhanced anti-tumor effects as compared to either drug alone.

The data also demonstrated that the anti-vascular effect of BAL101553 was more pronounced after administration of high BAL101553 doses, driven by high peak drug concentrations, while its anti-proliferative effect was driven by total exposure. These findings may allow modulation of the overall anti-cancer effect of BAL101553 through the application of low-dose administration schedules (e.g. daily oral), a strategy currently being pursued in ongoing clinical trials.

These presented data were generated in a research collaboration between Basilea and the group of Prof. Martin Pruschy of the Department of Radiation Oncology at the University Hospital Zurich, Switzerland.

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BAL101553 Late-breaking abstract at AACR annual meeting 2017

- *The novel tubulin-binding 'tumor checkpoint controller' BAL101553 has differential effects on tumor vascularization with IV and oral dosing and provides superior anti-tumor activity in combination with bevacizumab – A. Sharma, F. Bachmann, A. Broggini-Tenzer, M. Guckenberger, H. Lane, M. Pruschy; abstract LB-151*

For further information please visit www.aacr.org.

About BAL101553

Basilea's small molecule oncology drug candidate BAL101553 (the prodrug of BAL27862)[1] 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.[2, 3, 4] BAL101553 efficiently distributes to the brain, with anticancer activity in glioblastoma models.[5, 6, 7] The active moiety BAL27862 binds the colchicine site of tubulin with distinct effects on microtubule organization,[8] resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.[9]

About Basilea

Basilea Pharmaceutica Ltd. is a biopharmaceutical company developing products that address the medical problem 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.

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

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