PRESS RELEASE

Basilea reports presentation of phase 1 clinical data with anticancer drug candidate BAL101553 at ASCO meeting

- **Data support expansion into phase 2a**

Basel, Switzerland, June 05, 2018 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reported today that data from the phase 1 dose-escalation parts of two phase 1/2a studies with weekly 48-hour infusion and once-daily oral dosing with its novel tumor checkpoint controller BAL101553 were presented at the annual meeting of the American Society of Clinical Oncology (ASCO) in Chicago on June 4th, 2018. The drug candidate showed initial signals of clinical activity as monotherapy and an acceptable safety profile supporting its advancement into phase 2a clinical development in targeted patient populations.

Dr. Marc Engelhardt, Basilea's Chief Medical Officer, said: "Based on these results, Basilea is in the process of initiating an expansion phase 2a study with BAL101553 administered as a 48-hour infusion in adult patients with glioblastoma, which is a form of brain cancer, or platinum-resistant ovarian cancer. There are limited treatment options available for these cancers with high unmet medical need. We are, therefore, exploring the potential clinical benefit that our novel tumor checkpoint controller may be able to provide in these indications."

Basilea had observed indications of clinical activity in a previously conducted phase 1/2a study with a 2-hour infusion of BAL101553.[1] Dose-limiting effects were vascular toxicities and appeared to be related to the peak plasma concentration (C_{max}), while nonclinical models indicate that the anti-tumor effect of the drug candidate is driven by total drug exposure (AUC, area under the curve).

Phase 1 dose-escalation in the oral and 48-hour infusion studies, which explored BAL101553 in patients with solid tumors, has completed and the maximum tolerated doses were determined. No relevant vascular toxicities were observed in either the 48-hour infusion or the once-daily oral dosing studies. The AUC/C_{max} ratio in the 48-hour infusion study was approximately four times higher compared to the previously investigated 2-hour infusion regimen at the recommended phase 2 dose. A similar pattern was seen in the once-daily oral study. The observed dose limiting toxicities were transient or reversible and included hyponatremia (low sodium levels), hypokalemia (low potassium levels), neutropenia (low levels of white blood cells), hypotension (low blood pressure), and hallucinations. Of the 33 patients evaluable for efficacy from both phase 1 studies, one patient with ovarian cancer in the 48-hour infusion study had a confirmed partial response and six other patients with solid tumors had stable disease for four or more treatment cycles as best response.

Two additional studies with once-daily oral dosing of BAL101553 are ongoing: dose-escalation in a separate recurrent glioblastoma arm of the phase 1/2a study and a phase 1 study in combination with standard radiotherapy in newly diagnosed glioblastoma, which is conducted in collaboration with the Adult Brain Tumor Consortium (ABTC) in the U.S.

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BAL101553 posters at the 2018 ASCO Annual Meeting

- *Phase 1/2a study of BAL101553, a novel tumor checkpoint controller (TCC), administered as 48-hour infusion in adult patients with advanced solid tumors. – M. Joerger, I. Metaxas, A. Stathis, D. Hess, M. T. Mark, F. Hutter, N. Levy, S. Stuedeli, S. Berardi, M. Landau-Salzberg, M. F. Engelhardt, P. Larger, T. Kaindl, P. Hafner, P. McKernan, H. A. Lane, R. A. F. von Moos, C. Sessa; Abstract 2529, Poster Board #355*
- *Phase 1/2a study of once daily oral BAL101553, a novel tumor checkpoint controller (TCC), in adult patients with advanced solid tumors – J. S. Lopez, E. R. Plummer, M.-J. Devlin, R. Rulach, A. H. Ingles Garces, N. R. Md Haris, R. Miller, D. Crawford, M. D'Arcangelo, C. Aversa, A. L. Hannah, S. Anderson, M. F. Engelhardt, T. Kaindl, P. Larger, F. Bachmann, H. A. Lane, P. McKernan, T. R. J. Evans, R. S. Kristeleit; Abstract 2530, Poster Board #356*

For further information please visit am.asco.org.

About BAL101553

Basilea's oncology drug candidate BAL101553 (the prodrug of BAL27862)[2] is being developed as a potential therapy for diverse cancers. The drug candidate is currently in phase 1/2a clinical evaluation. 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.[3, 4, 5] BAL101553 efficiently distributes to the brain, with anticancer activity in glioblastoma models.[6, 7, 8] The active moiety BAL27862 binds the colchicine site of tubulin with distinct effects on microtubule organization,[9] resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.[10]

About Basilea

Basilea Pharmaceutica Ltd. is a commercial stage 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. With two commercialized drugs, the company is committed to discovering, developing and commercializing 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.

References

1 J. Lopez et al. Phase 1/2a trial of intravenous BAL101553, a novel tumor checkpoint controller (TCC), in advanced solid tumors. Journal of Clinical Oncology 2016 (34), suppl., abstract 2525

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3 A. Broggini-Tenzer et al. The novel microtubule-destabilizing drug BAL101553 (prodrug of BAL27862) sensitizes a treatment refractory tumor model to ionizing radiation. EORTC-NCI-AACR symposium 2014, abstract 202

4 G. E. Duran et al. In vitro activity of the novel tubulin active agent BAL27862 in MDR1(+) and MDR1(-) human breast and ovarian cancer variants selected for resistance to taxanes. American Association for Cancer Research (AACR) annual meeting 2010, abstract 4412

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8 A. C. Mladek et al. The novel tubulin-binding 'tumor checkpoint controller' BAL101553 has anti-cancer activity alone and in combination treatments across a panel of GBM patient-derived xenografts. American Association for Cancer Research (AACR) annual meeting 2016, abstract 4781

9 A. E. Prota et al. The novel microtubule-destabilizing drug BAL27862 binds to the colchicine site of tubulin with distinct effects on microtubule organization. Journal of Molecular Biology 2014 (426), 1848-1860

10 F. Bachmann et al. BAL101553 (prodrug of BAL27862): the spindle assembly checkpoint is required for anticancer activity. American Association for Cancer Research (AACR) annual meeting 2015, abstract 3789

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