PRESS RELEASE

Basilea presents preclinical data on its anticancer drug candidate BAL101553 at EORTC-NCI-AACR symposium

- **Late-breaking abstracts on increased cure rates and synergistic effects of combinations with eribulin and gemcitabine in solid tumor models**
- **Enhanced survival in a brain tumor model after single agent treatment**

Basel, Switzerland, November 14, 2018 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that it is presenting new preclinical data on its clinical stage anticancer drug candidate BAL101553 at the 30th EORTC-NCI-AACR (ENA) Symposium on Molecular Targets and Cancer Therapeutics in Dublin, Ireland, November 13-16, 2018.

Two posters are presented in the late-breaking abstract session. Late-breaking abstracts are selected to highlight novel and potentially practice-changing studies, e.g. ground-breaking and unique data that would not otherwise have been presented at the conference. These posters are showing data on the anticancer effects of BAL101553 in combination with eribulin or gemcitabine, which are approved anticancer drugs for the treatment of advanced metastatic breast cancer and pancreatic cancer, respectively.

Dr. Marc Engelhardt, Chief Medical Officer, said: "We are pleased that our two abstracts have been selected as late-breaking. The preclinical data show well-tolerated profound synergistic anticancer effects of BAL101553 in combination with the well-established cancer drugs eribulin and gemcitabine, suggesting that such combinations could also be advantageous for patients with advanced metastatic breast cancer and pancreatic cancer."

The first poster shows a highly synergistic anticancer effect of the combination of BAL101553 with eribulin in in-vitro cancer models. Moreover, such combinations are shown to be associated with a dose-dependent significant increase of complete regressions, i.e. cures of up to 80% in an animal model of triple-negative breast cancer.

The second poster shows that the combination of BAL101553 with gemcitabine led to complete regression in an animal model of pancreatic cancer (PDAC), compared to tumor size stabilization (stasis) with gemcitabine alone. Upon treatment cessation, tumors in the gemcitabine monotherapy group regrew while 40-80% of the animals in the combination groups remained in complete regression and were confirmed as cures.

A third poster presented at the symposium includes data generated in collaboration with the research group of Prof. Diane Braguer of the Aix-Marseille University, France. The data show that BAL101553 significantly increases the survival duration in an animal model of glioblastoma (brain cancer) after long-term oral administration. The survival benefit was largest in tumors expressing the EB-1 protein, which has previously been identified as a potential predictive biomarker of tumor response to BAL101553.[1] In addition, the data show that BAL101553 counteracts the formation of tumor vessels (angiogenesis), thus cutting off the tumor from blood and nutrition supply and potentially adding to the direct anticancer effect.

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Presentation on BAL101553 at the EORTC-NCI-AACR symposium

BAL101553, a novel microtubule-targeting tumor checkpoint controller, in combination with eribulin leads to increased cures in a TNBC xenograft model. N. Forster-Gros, F. Bachmann, P. McSheehy, H. A. Lane. Tuesday, November 13 to Friday, November 16, 2018, 10:00 a.m. – 5:30 p.m., Exhibition Hall, Poster Board 214, Late-Breaking Abstract 11

BAL101553, a novel microtubule-targeting tumor checkpoint controller, synergizes with gemcitabine providing cures in a PDX-pancreatic model. H. Lane, P. McSheehy, F. Bachmann. Tuesday, November 13 to Friday, November 16, 2018, 10:00 a.m. – 5:30 p.m., Exhibition Hall, Poster Board 216, Late-Breaking Abstract 16

EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after daily oral treatment with the novel Tumor Checkpoint Controller BAL101553. R. Bergès, A. Tchoghandjian, A. Sergé, D. Figarella-Branger, F. Bachmann, H. Lane, D. Braguer. Wednesday, November 14, 2018, 10:00 a.m. – 5:30 p.m.; Exhibition Hall, Poster Board 017

For further information please visit www.ecco-org.eu/Events/ENA2018.

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 Switzerland, a phase 2a expansion study is exploring the drug in recurrent glioblastoma and platinum-resistant ovarian cancer patients using weekly 48-hour infusion. In the UK, phase 1 dose escalation is ongoing in recurrent or progressive glioblastoma patients with daily oral administration. 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.[1, 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 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

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2 J. Pohlmann et al. BAL101553: An optimized prodrug of the microtubule destabilizer BAL27862 with superior antitumor activity. American Association for Cancer Research (AACR) annual meeting 2011, abstract 1347; Cancer Research 2011, 71 (8 supplement)

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

5 F. Bachmann et al. BAL101553 (prodrug of BAL27862): A unique microtubule destabilizer active against drug refractory breast cancers alone and in combination with trastuzumab. American Association for Cancer Research (AACR) annual meeting 2014, abstract 831

6 A. Schmitt-Hoffmann et al. BAL27862: a unique microtubule-targeted agent with a potential for the treatment of human brain tumors. AACR-NCI-EORTC conference 2009, abstract C233; Molecular Cancer Therapeutics 2009, 8 (12 Supplement)

7 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

8 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

9 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