PRESS RELEASE

Basilea to present preclinical brain tumor stem cell data on its oncology drug candidate BAL101553

- **Tumor check point controller BAL101553 demonstrates inhibitory activity on tumor stem cell self-renewal and invasion in models of brain cancer**
- **Loss of glioblastoma stem cell properties induced by BAL101553 correlates with EB1 expression levels *in vitro* and in animal models**

Basel, Switzerland, November 05, 2015 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reported today that preclinical data on its investigational clinical stage anti-cancer drug candidate BAL101553 will be presented at the AACR-NCI-EORTC International Conference* on Molecular Targets and Cancer Therapeutics in Boston, USA, November 5-9, 2015.

At the conference, data will be presented that were generated in a collaboration between Basilea and the research group of Prof. Diane Braguer of the Aix-Marseille University, France. The data demonstrate that BAL27862 (the active moiety of the prodrug BAL101553) has anti-proliferative activity against glioblastoma stem-like cells. Furthermore, short-term treatment of mice bearing glioblastoma brain tumors with BAL101553 was shown to provide a survival benefit.

BAL27862 stem cell activity, including inhibition of their self-renewal and invasive capacity, was dependent on the expression level of the End-binding 1 protein (EB1), a protein previously shown to be involved in tumor cell migration and overexpressed in glioblastoma stem-like cells that display a high tumorigenicity.[1] The activity of BAL27862 on stem-like cell characteristics was more pronounced in cells with high EB1 expression levels, indicating that EB1 may be a potential biomarker to aid selection of glioblastoma patients more likely to benefit from BAL101553 treatment.

Presentation on BAL101553 at the AACR-NCI-EORTC conference

The novel tubulin-binding 'tumor checkpoint controller' BAL101553 exerts EB1 expression-dependent antitumor effects on glioblastoma stem-like cells in vitro and in vivo. R. Berges, A. Tchoghandjian, S. Honore, D. Figarella-Branger, F. Bachmann, H. Lane, D. Braguer; poster A183; Friday, November 6, 2015 12:15 PM – 3:15 PM; Session A, Hall C-D

For further information please visit www.aacr.org.

About BAL101553

Basilea's oncology drug candidateBAL101553 is currently undergoing clinical evaluation in patients with advanced solid tumors as an i.v. (phase 2a) and oral (phase 1) formulation. It has shown evidence of clinical anti-tumor activity in a phase 1 study during which the maximum tolerated dose was established.[2] In previous pre-clinical 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 tumor and to brain, with cytotoxic effects in glioblastoma (brain tumor) cell lines.[6]

BAL101553 is the prodrug of the small-molecule BAL27862, developed as a potential therapy for diverse cancers, including tumor types unresponsive to standard therapeutics.

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The active moiety BAL27862 binds the colchicine site of tubulin with distinct effects on microtubule organization, resulting in the formation of the "spindle assembly checkpoint" which promotes tumor cell death.[7] Potential biomarkers are being tested in early clinical studies in order to optimize dose selection and treat cancer patients 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.

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

* Hosted by the American Association for Cancer Research (AACR), the National Cancer Institute (NCI), and the European Organisation for Research and Treatment of Cancer (EORTC)

References

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3 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

4 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

5 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

6 A. Schmitt-Hoffmann et al. BAL27862: a unique microtubule-targeted agent with a potential for the treatment of brain tumors. AACR-NCI-EORTC symposium 2009, abstract C233

7 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