PRESS RELEASE

Basilea's oncology drug candidate BAL101553 demonstrates broad antitumor activity in treatment-resistant breast cancer models as presented at AACR

Basel, Switzerland, April 7, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reported today that new data demonstrating the broad activity of Basilea's novel oncology drug candidate BAL101553 in pre-clinical models of human breast cancer, including models resistant to standard agents used for the treatment of breast cancer, were presented at the American Association of Cancer Research (AACR) Annual Meeting in San Diego, California, USA.

BAL101553 is an investigational new intravenous and oral small-molecule drug that arrests tumor cell proliferation and induces tumor cell death through a destabilizing effect on microtubules, an intracellular network essential for cell division. In addition, it demonstrates tumor-specific vascular disruption activity, thus depriving tumors of nutrition. Its distinct effect on microtubules, as well as its potent activity across numerous drug-refractory tumor models, differentiates BAL101553 from microtubule-targeting agents marketed today.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, commented: "Resistance to currently available anti-cancer drugs remains a major challenge in the treatment of cancer patients. BAL101553 demonstrated potent antitumor activity in diverse drug-refractory breast cancer models alone and in combination. Specifically, the significant enhancement of antitumor activity of BAL101553 in combination with trastuzumab, a widely-used therapeutic antibody for the treatment of breast cancer, is striking. These data highlight the potential of BAL101553 for the treatment of breast cancer alone and in combination." He added: "Following the recent successful completion of Phase 1 clinical testing determining the maximum tolerated dose, Basilea plans to start a Phase 2 program exploring BAL101553 as monotherapy in patients with advanced solid tumors in the first half of 2014."

The presented data was generated in cooperation between Basilea and the group of Prof. Sikic at Stanford University. BAL27862, the active moiety of the prodrug BAL101553, demonstrated anti-proliferative activity in several breast cancer cell lines, including multidrug-resistant lines that no longer respond to, or are inherently resistant to, paclitaxel and vincristine, two standard microtubule-targeting breast cancer therapies. Intravenous administration of BAL101553 in an animal model of chemotherapy-refractory human breast cancer led to a significantly reduced rate of tumor growth when compared to paclitaxel and vincristine. Antitumor activity was also observed in breast cancer models refractory to treatment with the therapeutic antibody trastuzumab. The combination of BAL101553 with trastuzumab strikingly exhibited enhanced antitumor activity *versus* the single agents in a patient-derived trastuzumab-refractory model. This was associated with a significant delay in tumor growth over an extended time period.

Poster at the AACR Annual Meeting 2014

- *BAL101553 (prodrug of BAL27862): A unique microtubule destabilizer active against drug refractory breast cancers alone and in combination with trastuzumab* – F. Bachmann, K. Burger, G. E. Duran, B. I. Sikic, H. A. Lane; poster/abstract #831

For further information please visit www.aacr.org

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About BAL101553

BAL101553 is a novel small-molecule anti-cancer drug candidate. The agent directly attacks tumor cells by destabilizing microtubules that form an intracellular network essential for cell division. In addition, it disrupts tumor blood vessels depriving the tumor of nutrition.

The investigational drug has shown broad *in-vitro* anti-proliferative activity in a panel of tumor models, including many that are, as a result of diverse resistance mechanisms, not responsive to standard microtubule-targeting agents, such as taxanes or *vinca*-alkaloids. Recently, a dose-escalation Phase 1 was successfully completed and the maximum tolerated dose has been established. In the study, first evidence of clinical antitumor activity in solid tumor patients was observed and reduced tumor cell proliferation and tumor vascularization were shown in patient tumor biopsies post-treatment.

BAL101553 is a highly soluble prodrug of Basilea's BAL27862. The injectable dosage form is formulated without potentially harmful solubilizers. In addition, the prodrug is orally bioavailable.

About Basilea

Basilea Pharmaceutica Ltd. is headquartered in Basel, Switzerland, and listed on the SIX Swiss Exchange (SIX: BSLN). Through the fully integrated research and development operations of its Swiss subsidiary Basilea Pharmaceutica International Ltd., the company focuses on innovative pharmaceutical products in the therapeutic areas of bacterial infections, fungal infections and oncology, targeting the medical challenge of rising resistance and non-response to current treatment options.

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