

PRESS RELEASE

Basilea's oncology drug candidate BAL101553 induces significant anti-tumor effect in treatment-refractory tumor models

- **BAL101553 anti-cancer activity in treatment-refractory cancer models, alone and in combination with radiotherapy, presented at EORTC-NCI-AACR Symposium***
- **BAL101553 has anti-tumor activity when administered i.v. or orally in *in-vivo* models of human cancer refractory to taxanes and epothilones**
- **Combination of BAL101553 with radiotherapy led to profound tumor growth delay**

Basel, Switzerland, November 19, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports today that data on its investigational phase 2a anti-cancer drug candidate BAL101553 will be presented at the 26th EORTC-NCI-AACR Symposium* on "Molecular targets and cancer therapeutics" in Barcelona, Spain, November 18-21, 2014.

BAL101553 data alone and in combination with radiotherapy in tumor models resistant to clinically relevant microtubule-targeting agents (MTAs) was generated in collaboration with the group of Prof. Martin Pruschy at the Department of Radiation Oncology, University Hospital Zurich. It shows that intravenously or orally administered BAL101553 in combination with radiotherapy led to profound tumor growth delays as compared to single-agent therapy. The combination of anti-cancer drugs with radiation therapy is an important treatment modality in oncology.

Intravenously or orally administered BAL101553 reduced tumor growth in a taxane-refractory animal model of human cancer, with daily or weekly oral administration eliciting equivalent anti-tumor responses. Moreover, in a tumor model refractory to taxanes and epothilones and with low response to radiotherapy, the combination of intravenously or orally administered BAL101553 and radiotherapy resulted in almost complete tumor stabilization over a prolonged period. Similar effects of BAL101553 were also observed in a second treatment-refractory model alone and in combination with radiotherapy.

In addition, BAL27862, the active moiety of the water-soluble prodrug BAL101553, was tested *in-vitro* for anti-cancer effects in human cancer lines resistant to clinically relevant MTAs. BAL27862 reduced cell proliferation when given alone. The effect was further enhanced in combination with ionizing radiation.

Dr. Laurenz Kellenberger, Chief Scientific Officer of Basilea, said: "Basilea is addressing therapeutic challenges in oncology and infectious diseases by focusing on overcoming resistance to standard treatment regimens and on personalized medicine. BAL101553 showed a profound anti-tumor effect in treatment-refractory models of human cancer after intravenous and oral administration in combination with radiotherapy. This further supports the compound's differentiated profile and its potential to treat patients with limited treatment options."

Presentation on BAL101553 at EORTC-NCI-AACR Symposium 2014

The novel microtubule-destabilizing drug BAL101553 (prodrug of BAL27862) sensitizes a treatment refractory tumor model to ionizing radiation. A. Broggin-Tenzer, F. Bachmann, V. Vuong, A. Messikommer, K. Nytko-Karouzakis, T. O'Reilly, H. A. Lane, M. N. Pruschy. Poster board No. P196 (Abstract No. 202); Wednesday, November 19, 6:00-7:30 p.m., Exhibition Hall

For further information please visit www.ecco-org.eu/Events/EORTC_NCI_AACR_2014.

About BAL101553

BAL101553 is Basilea’s experimental oncology drug currently in phase 2a clinical development. It has shown initial evidence of clinical anti-tumor activity in phase 1, during which the maximum tolerated dose was established. BAL101553 is a novel small-molecule drug candidate with potential as a therapy for diverse cancers, including tumor types unresponsive to standard therapeutics. The active moiety BAL27862 interacts with the microtubule network and has been demonstrated to stop tumor growth by a mechanism different from conventional microtubule-targeting agents such as taxanes. The availability of both intravenous and oral BAL101553 formulations provides dosing flexibility facilitating separate development scenarios depending upon indication and potential treatment combinations. Potential biomarkers are already being tested in early clinical studies with the aim to optimize treatment of cancer patients most likely to respond. Basilea’s oncology operations complement the company’s anti-infectives portfolio, as part of Basilea’s target-driven research and development approach. Immunocompromised cancer patients have an especially high risk of contracting bacterial or fungal infections.

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 Swiss subsidiary Basilea Pharmaceutica International Ltd. to develop and commercialize innovative pharmaceutical products to meet the significant 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 European Organisation for Research and Treatment of Cancer (EORTC), the National Cancer Institute (NCI) and the American Association for Cancer Research (AACR)