

PRESS RELEASE

Basilea's oncology drug candidate BAL101553 shows first evidence of antitumor activity in phase 1 study

- **Results of recently completed clinical phase 1 study presented at ASCO**

Basel, Switzerland, June 2, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports that phase 1 study results of its investigational oncology drug, BAL101553, were presented at the American Society of Clinical Oncology (ASCO) annual meeting in Chicago, U.S.A. The maximum tolerated dose (MTD) was determined and the results indicated first evidence of clinical antitumor activity.

BAL101553 is an intravenous and oral microtubule-targeting agent (MTA). Previous preclinical studies demonstrated that the investigational drug has potent anti-cancer activity in tumor models refractory to conventional MTAs. It was shown to arrest tumor cell proliferation and to induce tumor cell death through a destabilizing effect on microtubules which are an intracellular network essential for cell division. In addition, tumor-specific vascular disruption activity was observed in preclinical cancer models.

The currently reported phase 1 open-label, dose-escalation study included adult patients with advanced solid tumors who had failed standard therapy. The study investigated safety and tolerability of intravenous BAL101553 and evaluated pharmacokinetics, pharmacodynamics and antitumor activity.

In total, 24 patients received BAL101553 as a two-hour intravenous infusion of up to 80 mg/m² on day 1, 8 and 15 of a 28-day treatment cycle. BAL101553 was well tolerated up to 60 mg/m². Drug-related events included injection site reactions, nausea, vomiting, diarrhea, peripheral neuropathy (all mild or moderate), and well-manageable, transient hypertension. Gait disturbance together with peripheral sensory neuropathy were dose-limiting events.

One patient demonstrated a confirmed partial response lasting more than two years, and five patients showed stable disease, lasting more than four months in two patients. Comparison of post to pre-treatment tumor biopsies demonstrated a pronounced reduction of tumor cell proliferation and tumor vascularization.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, stated: "The clinical evidence of antitumor activity observed in phase 1 is encouraging and highlights the potential of our novel microtubule-targeting oncology drug candidate. BAL101553 has a distinct effect on microtubules and demonstrated potent activity across numerous drug-refractory tumor models. We will now proceed into phase 2a development for the further investigation of selected solid tumor types. We will continue to assess stratification biomarkers to identify patients most likely to respond to treatment with BAL101553."

BAL101553 poster at ASCO 2014

- *Phase 1/2a trial of the novel microtubule inhibitor BAL101553 in advanced solid tumors: Phase 1 completed* – L. R. Molife, G. Imseeh, M. Capelan, F. El-Khouly, N. Cresti, A. D. Smith, D. Averion, N. Md. Haris, S. J. Stimpson, T. Gumbleton, H. A. Lane, F. Bachmann, A. Schmitt-Hoffmann, A. Tzankov, A. L. Hannah, S. Anderson, U. Bethe, A. H. Calvert, R. Plummer, R. S. Kristeleit; Abstract 2562

For further information please visit <http://am.asco.org>.

About BAL101553

BAL101553 is a novel intravenous small-molecule anti-cancer drug candidate with the potential for oral administration. The agent directly attacks tumor cells by destabilizing microtubules that form an intracellular network essential for cell division.¹ In addition, it disrupts tumor blood vessels.^{1,2} 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.³ 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.¹

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

References

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- 3 F. Bachmann, K. Burger, G. E. Duran, B. I. Sivic, H. A. Lane. BAL101553 (prodrug of BAL27862): A unique microtubule destabilizer active against drug refractory breast cancers alone and in combination with trastuzumab. American Association of Cancer Research (AACR) Annual Meeting 2014, Abstract No. 831