PRESS RELEASE

Basilea announces start of phase 2a study with oncology drug candidate BAL101553

Basel, Switzerland, June 26, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports today that it initiated a phase 2a study with its investigational oncology drug BAL101553. The study is designed to further characterize safety and tolerability, and to obtain efficacy data in adult patients with advanced or recurrent solid tumors who have failed standard therapy or for whom no effective standard therapy is available. Tumor types were selected based on clinical observations in the phase 1 study and a detailed analysis of potential patient stratification biomarkers across tumor indications. The study will also continue the extensive biomarker testing initiated in Phase 1, to further evaluate dose and patient populations most likely to respond.

BAL101553 is an intravenous and oral small-molecule microtubule-targeting agent (MTA) with a dual action against human tumors, targeting tumor cells refractory to standard MTAs as well as tumor blood supply. In preclinical studies the investigational drug demonstrated potent anti-cancer activity in a broad panel of treatment-resistant tumor models. Tumor cell proliferation was arrested and tumor cell death was induced through a destabilizing effect on microtubules, an intracellular network essential for cell division. In addition, tumor-specific vascular disruption activity was observed in preclinical cancer models. First evidence of clinical antitumor activity has been seen in a recently completed phase 1 study.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, stated: "Following the promising phase 1 results we have swiftly initiated the phase 2a study at leading cancer institutions. Our study will assess BAL101553's safety and efficacy in different solid tumor types in patients refractory to current therapy to facilitate the selection of tumor indications to be included in future expanded phase 2 trials."

The phase 2a open-label multicenter study is scheduled to enroll 40 patients, randomized either to intravenous BAL101553 at the maximum tolerated dose (MTD) determined in the recently completed phase 1 study, or at half of the MTD to determine the optimal dose. Dose selection was supported by preclinical results from a human cancer model, in which similar tumor exposure was achieved for both doses while the lower dose resulted in higher peak intra-tumoral levels, potentially as a result of a less pronounced effect on the vasculature. Anti-vascular effects will be further evaluated in patients through expanded biomarker and functional imaging assessments.

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 not responsive to standard microtubule-targeting agents, such as taxanes or *vinca*-alkaloids, as a result of diverse resistance mechanisms.[3] Recently, the maximum tolerated dose was determined in a phase 1 study where first evidence of clinical antitumor activity was observed.[4] In preclinical studies BAL101553 demonstrated good compatibility with targeted therapeutic antibodies such as trastuzumab.[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]

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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.

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