

PRESS RELEASE

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Basilea provides clinical program updates

- **Ceftobiprole U.S. clinical phase 3 program discussed with FDA**
- **Interim data from phase 1/2a study with tumor checkpoint controller BAL101553 show signals of clinical benefit**

Basel, Switzerland, December 28, 2015 – Basilea Pharmaceutica Ltd. (SIX: BSLN) today provided an update on the planned clinical phase 3 development program for its antibiotic ceftobiprole in the United States and reported completion of patient recruitment and interim data from the ongoing phase 1/2a study with the intravenous formulation of its oncology drug candidate BAL101553.

Ceftobiprole U.S. development program

Ceftobiprole (ceftobiprole medocaril) is a broad-spectrum antibiotic for intravenous administration with bactericidal activity against Gram-positive and Gram-negative bacteria associated with pneumonia, including methicillin-resistant *Staphylococcus aureus* (MRSA) and susceptible *Pseudomonas* spp.¹

Following recent discussions with the FDA, Basilea will consider conducting cross-supportive clinical studies for the indications of acute bacterial skin and skin structure infections, community-acquired bacterial pneumonia and *Staphylococcus aureus* bloodstream infections, or bacteremia, including endocarditis, a complication of bacteremia involving the heart valves. Basilea currently expects that it would not initiate phase 3 studies until such time as it has entered into a collaboration agreement with a third party.

Basilea is now preparing phase 3 study protocols for submission to the FDA in the first quarter of 2016, seeking Special Protocol Assessments (SPAs). An SPA provides written guidance by the FDA and documents agreement between the study sponsor and the agency that a clinical study is adequately designed so that it would support a regulatory submission for drug approval, should the study meet its objectives.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, stated: "Infections caused by *Staphylococcus aureus*, and especially by methicillin-resistant strains, are a serious healthcare issue. In preclinical models in endocarditis and clinical studies conducted so far, ceftobiprole has demonstrated its activity against relevant bacterial pathogens, including methicillin-susceptible and resistant *Staphylococcus aureus*. Basilea is now taking a first step to potentially make ceftobiprole available also to patients in the United States and potentially expand the approved label in Europe."

Ceftobiprole has been approved for sale in 13 European countries² and Canada for the treatment of adult patients with community-acquired pneumonia and hospital-acquired pneumonia (excluding ventilator-associated pneumonia) and has been launched in Germany, France, Italy and the United Kingdom. Ceftobiprole received Qualified Infectious Disease Product (QIDP) designation from the U.S. Food and Drug Administration for the potential

treatment of community-acquired bacterial pneumonia and acute bacterial skin and skin structure infections. Ceftobiprole is not approved in the United States.

Phase 1/2a study with i.v. BAL101553: completion of patient recruitment and interim results

BAL101553 is a novel small molecule tumor checkpoint controller (TCC) candidate, promoting tumor cell death through activation of a checkpoint in cell proliferation.^{3,4} Its intravenous formulation (i.v.) is currently being explored in a phase 1/2a clinical study and its oral formulation in a phase 1 study, both with solid tumor patients who have failed standard therapy or for whom no effective standard therapy is available.

In the phase 1 part of the i.v. study (24 patients), the Maximum Tolerated Dose (MTD) was defined as 60 mg/m², when BAL101553 was administered intravenously over 2 hours on day 1, 8 and 15 of 28-day treatment cycles.⁵ The phase 1 study was expanded into an open-label phase 2a study (40 evaluable patients) with the primary objective to further evaluate the safety and tolerability of MTD and sub-MTD doses of BAL101553.

Patient recruitment in this phase 1/2a study has been completed. Of the 72 patients dosed to date in the phase 1/2a study, 52 patients have undergone evaluation for tumor response. One patient with an ampullary pancreatic cancer achieved a partial response and was treated for more than two years with BAL101553 and 12 patients had stable disease lasting between two and eight months. For a 2-hour infusion the recommended phase 2 dose has been defined as 30 mg/m². The majority of patients with signals of clinical benefit, including the partial response, were dosed at the recommended dose or below. Currently, 9 patients are still ongoing with BAL101553 treatment.

BAL101553 demonstrated anti-proliferative and anti-vascular effects as evidenced by tumor biopsies and from circulating vascular cell levels obtained from patients before and after dosing with BAL101553. Pharmacodynamic assessments together with preclinical data support a separation of the anti-tumor cell from the anti-vascular effect at different dose levels, enabling the design of combination studies with other agents or radiotherapy where BAL101553 has shown a combination potential in preclinical models.

Dose-limiting adverse effects in the phase 1 part of the study included transient and reversible grade 2 to grade 3 gait disturbance which occurred together with transient peripheral sensory neuropathy. In addition, cardiac ischemia was observed in the phase 2a portion of the study at dose levels above 30 mg/m².

The recommended 2-hour intravenous dose has been defined based on the observed good tolerability, without myelosuppression, and on pharmacodynamic and clinical response.

Detailed analyses of the study results will be presented at upcoming scientific conferences.

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.

References

- 1 Y. Y. Syed. Ceftobiprole medocaryl: A review of its use in patients with hospital- or community-acquired pneumonia. *Drugs* 2014 (74), 1523-1542
- 2 Ceftobiprole (European trade name Zevtera® or Mabelio®, depending on the country) has received national licenses in 13 European countries for the treatment of adult patients with community- and hospital-acquired pneumonia (CAP, HAP), excluding ventilator-associated pneumonia (VAP): Austria,

Belgium, Denmark, Finland, France, Germany, Italy, Luxembourg, Norway, Spain, Sweden, Switzerland and the United Kingdom.

- 3 F. Bachmann, K. Burger, H. Lane. BAL101553 (prodrug of BAL27862): the spindle assembly checkpoint is required for anticancer activity. American Association for Cancer Research (AACR) annual meeting 2015, abstract 3789
- 4 R. Berges et al. The novel tubulin-binding 'tumor checkpoint controller' BAL101553 exerts EB1 expression-dependent antitumor effects on glioblastoma stem-like cells in vitro and in vivo. AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics, November 2015, abstract A183
- 5 L. R. Molife et al. Phase 1/2a trial of the novel microtubule inhibitor BAL101553 in advanced solid tumors: Phase 1 completed. American Society of Clinical Oncology (ASCO) annual meeting 2014, abstract 2562