PRESS RELEASE

Basilea starts clinical phase 3 study with antibiotic ceftobiprole in *Staphylococcus aureus* bacteremia (SAB)

Basel, Switzerland, August 09, 2018 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today the start of a clinical phase 3 study with its antibiotic ceftobiprole. The registration study evaluates the safety and efficacy of ceftobiprole in the treatment of patients with bacteremia (bloodstream infections) caused by *Staphylococcus aureus*.

David Veitch, Chief Executive Officer, said: "We are very pleased to have started the SAB study. This is the second of our two phase 3 studies that are required to potentially gain a U.S. marketing authorization. SAB and associated complications cause significant morbidity and mortality, in particular if caused by methicillin-resistant *Staphylococcus aureus*, MRSA. With its broad spectrum of activity, including MRSA, ceftobiprole has the potential to become an important treatment option for patients with these serious hospital bacterial infections."

The randomized, double-blind, multi-center study aims to establish the safety and efficacy of intravenously (i.v.) administered ceftobiprole versus i.v. daptomycin (plus optional aztreonam for Gram-negative infections) in the treatment of SAB. The FDA-agreed primary endpoint is to demonstrate non-inferiority of ceftobiprole to the comparator regimen at the post-treatment evaluation visit 70 days after randomization. The study is anticipated to enroll approximately 390 adult patients. Additional information on this clinical study is available at www.clinicaltrials.gov (identifier: NCT03138733).

A first phase 3 study was started in February which evaluates ceftobiprole in the treatment of acute bacterial skin and skin structure infections (ABSSSI). The two phase 3 studies are designed to be cross-supportive for a potential U.S. registration and are conducted under a Special Protocol Assessment (SPA) agreement with the FDA.

Basilea's ceftobiprole phase 3 program is funded in part (up to USD 118 million, which is approximately 70% of the total estimated program costs) with Federal funds from the U.S. Department of Health and Human Services; Office of the Assistant Secretary for Preparedness and Response; Biomedical Advanced Research and Development Authority (BARDA), under Contract No. HHSO100201600002C.

About ceftobiprole

Ceftobiprole is a cephalosporin antibiotic for intravenous administration with rapid bactericidal activity against a wide range of Gram-positive and Gram-negative bacteria, including methicillin-susceptible and resistant *Staphylococcus aureus* (MSSA, MRSA) and susceptible *Pseudomonas* spp.[1] Ceftobiprole is approved in major European countries and several non-European countries for the treatment of adult patients with community-acquired pneumonia (CAP) and hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP).[1] Basilea has entered into license and distribution agreements for the brand in Europe, Latin America, China, Canada, Israel, and the Middle East and North Africa (MENA) region. It is currently marketed in major European countries, Argentina, Canada and Saudi Arabia under the brand names Zevtera® and Mabelio®. The drug received Qualified Infectious Disease Product (QIDP) designation in the U.S. from the FDA for the potential treatment of *Staphylococcus aureus* bacteremia (SAB), acute bacterial skin and skin structure infections (ABSSSI), and community-acquired bacterial pneumonia (CABP). Based on the QIDP

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designation ceftobiprole would be eligible for priority review of a future New Drug Application (NDA) and market exclusivity of ten years upon approval in the U.S. Ceftobiprole is currently in a phase 3 clinical program for registration in the U.S. under an SPA with the FDA. The SPA provides agreement between Basilea and the FDA that the design and planned analysis of a clinical study adequately address the objectives necessary to support a regulatory submission for the approval of a drug in the U.S.

About *Staphylococcus aureus* bacteremia (SAB)

Staphylococcus aureus bacteremia is a leading cause of bloodstream infections, responsible for a broad variety of complications and has been associated with significant morbidity and a mortality of 20 to 40%.[2, 3] Several studies have demonstrated that MRSA bacteremia is associated with a significantly higher mortality rate compared with methicillin-susceptible *Staphylococcus aureus* (MSSA) bacteremia.[4, 5] Infections of the inner lining of the heart or heart valves (infective endocarditis) and bone infections (osteomyelitis) are common complications of SAB.

About Basilea

Basilea Pharmaceutica Ltd. is a commercial stage biopharmaceutical company developing products that address the medical challenge of increasing resistance and non-response to current treatment options in the therapeutic areas of bacterial infections, fungal infections and cancer. With two commercialized drugs, the company is committed to discovering, developing and commercializing innovative pharmaceutical products to meet the medical needs of patients with serious and 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.

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