PRESS RELEASE

Basilea announces positive results of phase 3 TARGET study with antibiotic ceftobiprole in the treatment of acute bacterial skin and skin structure infections (ABSSSI)

- **Ceftobiprole met primary and secondary efficacy endpoints in the treatment of ABSSSI**
- **Ceftobiprole was well tolerated**

Basel, Switzerland, August 06, 2019 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today positive topline results for the phase 3 TARGET study, evaluating ceftobiprole in the treatment of patients with acute bacterial skin and skin structure infections (ABSSSI).[1]

Dr. Marc Engelhardt, Chief Medical Officer, stated: "The successful completion and positive results of our phase 3 study in skin infections marks the achievement of a significant milestone towards a filing of ceftobiprole in the U.S. The second study, in *Staphylococcus aureus* bloodstream infections, is well on track and is expected to deliver topline results in the second half of 2021. In both indications, ceftobiprole addresses unmet medical needs through its broad-spectrum rapid bactericidal activity, with the ability to cover both Gram-positive and Gram-negative pathogens and with the well-established safety profile of a cephalosporin."

If both studies are positive, Basilea plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA). As ceftobiprole was designated a Qualified Infectious Disease Product (QIDP) by the FDA for these indications, if approved, ceftobiprole will be eligible to receive ten years of market exclusivity in the U.S. from the date of approval.

The TARGET study enrolled 679 patients. Ceftobiprole met the pre-specified primary efficacy objective of non-inferiority (within the pre-specified margin of 10%) to vancomycin plus aztreonam in the intent-to-treat (ITT) population. The primary endpoint of early clinical response was based on a 20% or more reduction from baseline in lesion size at 48 to 72 hours after start of study drug administration. In addition, ceftobiprole was also non-inferior to vancomycin plus aztreonam for the pre-specified secondary endpoint of investigator-assessed clinical success, which was based on resolution of baseline signs and symptoms of the infection, in the ITT and the clinically evaluable (CE) patient populations at the test-of-cure (TOC) visit, 15 to 22 days after randomization.

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Key topline study results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary analyses

	Ceftobiprole N=335[a]	**Vancomycin plus aztreonam** N=344[b]	**Difference** (95% CI)[c]
Primary endpoint: Early clinical response at 48-72 hours (ITT population)	91.3%	88.1%	3.3% (-1.2 ; 7.8)
Secondary endpoint: Clinical success at the TOC visit (ITT population)	90.1%	89.0%	1.0% (-3.5 ; 5.6)
Clinical success at the TOC visit (CE population)	97.9%	95.2%	2.7% (-0.3 ; 5.6)
CE population: [a]N=283 , [b]N=294; [c]Difference of ceftobiprole minus vancomycin plus aztreonam			

Ceftobiprole was well tolerated in the TARGET study. The overall rates of drug-related adverse events were similar between the two treatment groups (20% ceftobiprole vs. 18% vancomycin plus aztreonam). The most common drug-related adverse events in both treatment groups were nausea, diarrhoea and headache.

Basilea plans to submit the full data from this study for presentation at an upcoming scientific conference.

About the ceftobiprole phase 3 program

The TARGET study[1] was a randomized, double-blind, multicenter, phase 3 study, which enrolled 679 patients with ABSSSI and compared the safety and efficacy of ceftobiprole medocaril (equivalent to ceftobiprole 500 mg) given intravenously three times daily with intravenous vancomycin 1000 mg (or 15 mg/kg) given twice-daily plus intravenous aztreonam 1000 mg given twice-daily. The study was conducted at more than 30 clinical centers in the U.S. and Europe.

The study was the first of two phase 3 studies conducted under Special Protocol Assessment (SPA) agreements with the U.S. FDA. The second study, ERADICATE, which compares ceftobiprole to daptomycin with or without aztreonam in the treatment of patients with *Staphylococcus aureus* bacteremia (SAB), is ongoing and topline results are expected in the second half of 2021.[2] Both studies are required for an NDA of ceftobiprole in the U.S.

The ceftobiprole phase 3 program is funded in part (up to USD 128 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 medocaril, the prodrug of the active moiety ceftobiprole, is a cephalosporin antibiotic for intravenous administration with rapid bactericidal activity against a wide range of Gram-positive and Gram-negative bacteria. This includes methicillin-susceptible and resistant *Staphylococcus aureus* (MSSA, MRSA) and susceptible *Pseudomonas* spp.[3] The drug is currently approved and marketed in major European countries, Argentina, Canada, Jordan, Peru and Saudi Arabia for the treatment of adult patients with community-acquired pneumonia (CAP) or hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP).[3] 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. Ceftobiprole is currently in phase 3 clinical development for a potential registration in the U.S., with a

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completed study in acute bacterial skin and skin structure infections (ABSSSI) and an ongoing study in *Staphylococcus aureus* bacteremia (SAB).

About acute bacterial skin and skin structure infections (ABSSSI)

Acute bacterial skin and skin structure infections (ABSSSIs) are among the most common infections encountered in both community and hospital settings, and include infections with resistance to previously effective antibacterial treatments.[4] Increasing in incidence, they have become a challenging medical problem associated with high direct and indirect costs to both the medical system and society.[5] Infections due to bacteria with resistance to previously effective antibacterial treatments, such as methicillin-resistant *Staphylococcus aureus* (MRSA), are increasing in incidence and have led to higher rates of complications and hospitalization. MRSA has emerged as the most common cause of pus-forming infections in the United States and many other areas.

About Basilea

Basilea Pharmaceutica Ltd. is a commercial stage biopharmaceutical company, focused on the development of products that address the medical challenges in the therapeutic areas of oncology and anti-infectives. 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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