PRESS RELEASE

Basilea submits isavuconazole European Marketing Authorization Application for the treatment of invasive mold infections

Basel, Switzerland, July 17, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports today that it submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) seeking approval of isavuconazole for the treatment of invasive aspergillosis and mucormycosis (zygomycosis). Basilea's co-development partner Astellas Pharma Inc. also recently submitted a New Drug Application (NDA) for isavuconazole to the U.S. Food and Drug Administration (FDA).

Ronald Scott, Basilea's Chief Executive Officer, stated: "New antifungal therapies are urgently needed due to the increasing number of immunocompromised patients who are at risk for developing invasive fungal infections such as cancer patients undergoing chemotherapy. The isavuconazole regulatory submission in the EU is a major achievement for Basilea and complements the U.S. submission by our partner Astellas."

Isavuconazole (drug substance: isavuconazonium sulfate) is an investigational once-daily intravenous and oral broad-spectrum antifungal for the potential treatment of life-threatening invasive fungal infections which predominantly occur in immunocompromised patients. In the European Union (EU), isavuconazole was recently granted orphan drug status for the treatment of invasive aspergillosis and mucormycosis, providing ten years of market exclusivity independent of any existing patent protection should the product be approved in the EU. In the U.S., isavuconazole was granted FDA fast-track status and designated a Qualified Infectious Disease Product (QIDP) for invasive aspergillosis, mucormycosis and candidiasis under the U.S. GAIN Act. QIDP status provides priority review and, if the product is approved, a five-year extension of market exclusivity in the United States. In addition, isavuconazole received U.S. orphan drug designations for invasive aspergillosis and mucormycosis.

Basilea holds full rights to isavuconazole in markets outside of the U.S. and Canada where Astellas is the license holder. Basilea will be eligible to a milestone payment upon FDA acceptance of the U.S. NDA submission.

The MAA is supported by data from the SECURE and VITAL phase 3 studies. The SECURE study was a global double-blind randomized study that enrolled 516 patients (intent-to-treat population) and evaluated the safety and efficacy of once-daily isavuconazole versus twice-daily voriconazole in the primary treatment of invasive fungal disease caused by *Aspergillus* species or other filamentous fungi. The VITAL study was an open-label study of isavuconazole (N=149 patients) in the treatment of aspergillosis patients with pre-existing renal impairment or patients with invasive fungal disease caused by emerging and often fatal molds such as *Mucorales*, yeasts, or dimorphic fungi.

In the invasive aspergillosis SECURE study, isavuconazole demonstrated non-inferiority to voriconazole on the primary endpoint of all-cause mortality at day 42. The treatment-emergent adverse events for isavuconazole were statistically fewer relative to voriconazole in the system organ classes of hepatobiliary, skin and eye disorders. In addition, isavuconazole showed statistically fewer study drug-related adverse events relative to voriconazole. In both treatment

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groups, the most common treatment-emergent adverse events were nausea, vomiting, pyrexia (fever) and diarrhea.[1]

The isavuconazole phase 3 program includes a third study, ACTIVE. It is currently enrolling patients and will evaluate the safety and efficacy of intravenously (i.v.) and orally administered isavuconazole versus i.v. caspofungin followed by oral voriconazole in the treatment of invasive *Candida* infections.

About invasive aspergillosis and mucormycosis

Invasive aspergillosis is estimated to occur in 5-13% of bone marrow transplant recipients, 5-25% of patients who have received heart or lung transplants, and 10-20% of patients who have received intensive chemotherapy for leukemia.[2] Mortality rates for transplant patients with invasive aspergillosis have been reported to be between 34% and 58%.[3] Around 47% of solid organ transplant recipients who developed invasive aspergillosis had renal insufficiency and acute renal failure was reported for 43% of intensive care unit (ICU) patients with invasive aspergillosis, compared to 20% in the general ICU population.[3, 4]

Mucormycosis (also known as zygomycosis) is an often lethal fungal infection caused by certain emerging molds. Mucormycosis is associated with high morbidity and mortality rates in immunocompromised patients such as patients undergoing chemotherapy or bone marrow transplantation.[5, 6] Left untreated, mucormycosis is almost always lethal, and even with appropriate medical management, mortality rates remain high.[7]

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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