PRESS RELEASE

Basilea's partner Astellas submits isavuconazole U.S. NDA for the treatment of invasive aspergillosis and invasive mucormycosis

Basel, Switzerland, July 9, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports today that its co-development partner Astellas Pharma Inc. submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) seeking approval of isavuconazole for the treatment of invasive aspergillosis and invasive mucormycosis (zygomycosis).

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 predominantly occurring in immunocompromised patients. In the U.S., isavuconazole was granted FDA fast-track status, designated as a Qualified Infectious Disease Product (QIDP) under the U.S. GAIN Act, and received orphan drug designations for the treatment of invasive aspergillosis and mucormycosis.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, stated: "The pathogens *Aspergillus* and *Mucorales* molds cause serious invasive fungal infections in the growing number of immunocompromised patients such as cancer patients. These infections are associated with high mortality. This first regulatory submission for isavuconazole marks an important milestone towards potentially providing a new therapeutic option for the treatment of these life-threatening fungal infections."

Ronald Scott, Basilea's Chief Executive Officer, added: "We are pleased that the NDA has been filed in the U.S. by our partner, Astellas, potentially bringing isavuconazole one step closer to patients. On this basis, Basilea is preparing the European Marketing Authorization Application or MAA in parallel to the U.S. application. We plan to file the MAA as scheduled, mid-2014. Basilea remains committed to address the need for new drugs to treat life-threatening infections."

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 NDA 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 phase 3 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 groups, the most common treatment-emergent adverse events were nausea, vomiting, pyrexia (fever) and diarrhea.

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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.[1] Mortality rates for transplant patients with invasive aspergillosis have been reported to be between 34% and 58%.[2] 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.[2, 3]

Mucormycosis (also known as zygomycosis) is a serious and often lethal fungal infection caused by certain emerging molds. Invasive mucormycosis is associated with high morbidity and mortality rates in immunocompromised patients such as patients undergoing chemotherapy or bone marrow transplantation.[4, 5] Left untreated, mucormycosis is almost always lethal, and even with appropriate medical management mortality rates remain high.[6]

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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