PRESS RELEASE

Basilea reports that isavuconazole receives Qualified Infectious Disease Product (QIDP) designation from U.S. FDA for the treatment of invasive aspergillosis

Basel, Switzerland, December 3, 2013 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that the U.S. Food and Drug Administration (FDA) designated isavuconazole as a Qualified Infectious Disease Product (QIDP) for the treatment of invasive aspergillosis.

QIDP status provides priority review and a five-year extension of market exclusivity following product approval in the United States. These incentives were granted under the 2012 U.S. Generating Antibiotic Incentives Now (GAIN) Act as part of the FDA Safety and Innovation Act.

The five-year extension is in addition to the seven-year exclusivity based on isavuconazole's FDA orphan drug designation for the treatment of invasive aspergillosis. In the U.S., isavuconazole also received orphan drug designation for the treatment of zygomycosis, a life-threatening invasive fungal infection (IFI) caused by emerging molds.

Ronald Scott, Basilea's Chief Executive Officer, commented: "We are very pleased that isavuconazole received QIDP designation for the treatment of invasive aspergillosis. Fungal pathogens are a major threat to public health and the inclusion of *Aspergillus* species on the list of qualifying pathogens under the GAIN Act highlights the high medical need of patients suffering from these potentially life-threatening infections. The evidence obtained to date from pre-clinical and clinical data suggests that isavuconazole may have the potential to become an important treatment option for these patients. Analyses of the SECURE and VITAL phase 3 study data are currently being completed to support a potential filing in the first half of 2014."

IFIs are debilitating or life-threatening infections that attack internal tissues or organs and can spread through the bloodstream. Fungi commonly involved in IFIs include *Aspergillus* (molds) and *Candida* (yeasts).[1, 2] Invasive aspergillosis typically affects patients with an impaired or weakened immune system. It is estimated to occur in 5-13% of recipients of bone marrow transplants often associated with leukemia, 5-25% of patients who have received heart or lung transplants, and 10-20% of patients who are receiving intensive chemotherapy for leukemia.[3] Mortality rates for transplant patients with invasive aspergillosis have been reported to be between 34 and 58%.[4] Zygomycosis is an important emerging fungal infection, associated with high morbidity and mortality. Mortality rates have been reported to be as high as 80% in infected transplant patients.[5]

About isavuconazole

Isavuconazole (drug substance: isavuconazonium sulfate) is an investigational once-daily intravenous and oral broad-spectrum antifungal for the potential treatment of severe invasive and life-threatening fungal infections. It is currently in phase 3 of clinical development. Isavuconazole demonstrated *in-vitro* and *in-vivo* coverage of a broad range of yeasts (such as *Candida* species) and molds (such as *Aspergillus* species) as well as activity in *in-vitro* studies and animal models against emerging and often fatal molds including those that cause zygomycosis. Isavuconazole received U.S. FDA fast-track status and U.S. orphan drug designation for invasive aspergillosis and zygomycosis. Isavuconazole is being co-developed with Astellas Pharma Inc.

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About the isavuconazole phase 3 program

The phase 3 program with isavuconazole includes three studies, SECURE, VITAL and ACTIVE. Recently, positive topline results were reported from the SECURE study, a global randomized, double-blind phase 3 study, designed to evaluate 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 certain other filamentous fungi. Isavuconazole demonstrated non-inferiority versus voriconazole. Isavuconazole was effective as determined by the primary endpoint of all-cause mortality through day 42 in the intent-to-treat population (N=516). Study drug-related adverse events were reported in 42.4% of the isavuconazole and 59.8% of the voriconazole treatment group. Overall drug- and non-drug-related adverse events were reported in 96.1% and 98.5% of patients in the isavuconazole and voriconazole treatment groups, respectively.

The VITAL study is an open-label phase 3 study including patients with invasive fungal disease caused by emerging fungal pathogens such as Zygomycetes and patients with aspergillosis and pre-existing renal impairment. Enrollment into VITAL has been completed (N=150). Based on the investigator reported data, approximately 45 patients were enrolled with zygomycosis and a similar number of patients were enrolled with pre-existing renal impairment. Review of diagnosis and outcomes by an Independent Data Review Committee is ongoing.

The phase 3 ACTIVE study is a randomized, double-blind study evaluating the use of isavuconazole i.v. and oral versus caspofungin i.v. followed by oral voriconazole for the treatment of invasive *Candida* infections. The ACTIVE study continues to recruit with anticipated completion of enrollment in the first part of 2015.

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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