

PRESS RELEASE

Basilea reports that isavuconazole received QIDP designation from U.S. FDA for the treatment of invasive candidiasis

Basel, Switzerland, July 16, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports today that the U.S. Food and Drug Administration (FDA) designated isavuconazole as a Qualified Infectious Disease Product (QIDP) for the oral and intravenous treatment of invasive candidiasis, a life-threatening invasive fungal infection caused by *Candida* yeasts.

A QIDP designation, granted under the U.S. GAIN Act, provides priority review and a five-year extension of market exclusivity, should the product be approved in the United States. Isavuconazole was previously granted QIDP designation for the treatment of invasive aspergillosis and invasive mucormycosis (also known as zygomycosis), which are severe mold infections. In addition, isavuconazole has FDA fast-track status and received U.S. and European Union orphan drug designations for invasive aspergillosis and mucormycosis.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, commented: "Invasive candidiasis is a nosocomial infection associated with high mortality, frequently occurring in critically ill patients, such as cancer patients undergoing chemotherapy, organ transplantation, and invasive surgical procedures. Moreover, the incidence of *Candida* infections has been increasing over past decades." He added: "We are very pleased that the FDA has granted this third QIDP designation to isavuconazole. QIDP designations are granted to antibacterial or antifungal drugs which are intended to treat serious or life-threatening infections caused by certain qualified pathogens, including *Candida* species, that have the potential to pose a serious threat to public health."

Isavuconazole is being co-developed with Astellas Pharma Inc. Basilea holds full rights to isavuconazole in markets outside of the U.S. and Canada where Astellas is the license holder.

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, predominantly occurring in immunocompromised patients. Isavuconazole demonstrated *in-vitro* and *in-vivo* coverage of a broad range of yeasts (such as *Candida* species) and molds (such as *Aspergillus* species), including emerging and often fatal molds such as those that cause mucormycosis.

Basilea's partner Astellas has submitted a New Drug Application (NDA) to the FDA seeking approval of isavuconazole for the treatment of invasive aspergillosis and invasive mucormycosis. 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 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 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.

The isavuconazole phase 3 program includes a third study, ACTIVE, which 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 candidiasis

Infections by *Candida* yeasts are the most frequent invasive fungal infections in critically ill patients, accounting for 70% to 90% of all invasive mycoses.¹ They are the fourth and seventh most common cause of bloodstream infections in the U.S. and Europe, respectively, and are associated with increased morbidity and mortality.^{2, 3} Estimates of the attributable mortality of *Candida* bloodstream infections (candidemia) range from 15% to 49%.^{4, 5, 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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