PRESS RELEASE

Basilea reports isavuconazole orphan drug designation for treatment of zygomycosis by U.S. FDA

Basel, Switzerland, November 18, 2013 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reported today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to isavuconazole for the treatment of zygomycosis (also known as mucormycosis), a life-threatening fungal infection. Basilea announced on May 28, 2013 that the FDA granted isavuconazole orphan drug designation for the treatment of invasive aspergillosis.

An FDA orphan drug designation provides several benefits to the sponsor including a seven-year market exclusivity in the United States dating from product approval. Isavuconazole also has FDA fast track status, which has the goal of getting important new drugs to patients with life-threatening conditions as quickly as possible.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, commented: "Zygomycosis is a life-threatening infection involving emerging fungi of the Zygomycetes class. Infections with emerging fungi are an increasing healthcare problem. They typically occur in patients with an impaired or weakened immune system such as cancer or transplant patients, or in patients with diabetes. Left untreated, zygomycosis is associated with high mortality rates. In *in-vitro* studies and animal models isavuconazole has demonstrated activity against various Zygomycetes and other emerging fungi. The granting of orphan drug designation for isavuconazole is an important regulatory milestone for Basilea and our partner Astellas." He added: "While focusing on completing the analyses of the SECURE and VITAL studies to support a potential filing in the first half of 2014, the ACTIVE study continues to recruit with anticipated completion of enrollment in the first part of 2015."

Due to their limited activity against Zygomycetes, voriconazole, fluconazole and the echinocandins are not indicated for the treatment of zygomycosis.

Isavuconazole is currently in phase 3 of clinical development. Enrollment in the open-label phase 3 isavuconazole study (VITAL) including patients with invasive fungal disease caused by emerging fungal pathogens such as Zygomycetes and patients with aspergillosis and pre-existing renal impairment is complete (N=150). Based on the investigator reported data, approximately 45 patients were enrolled with mucormycosis (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.

Recently, positive topline results from the isavuconazole phase 3 invasive aspergillosis study (SECURE) were reported. Isavuconazole demonstrated non-inferiority versus voriconazole for the primary treatment of invasive fungal disease caused by *Aspergillus* species or certain other filamentous fungi. 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 randomized, double-blind phase 3 isavuconazole study ACTIVE evaluates the use of isavuconazole i.v. and oral versus caspofungin i.v. followed by oral voriconazole for the treatment of invasive *Candida* infections.

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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 in animal models against emerging and often fatal molds including those that cause zygomycosis (also known as mucormycosis). 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.

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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