PRESS RELEASE

Basilea reports isavuconazole orphan drug designation by U.S. FDA

Basel, Switzerland, May 28, 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 invasive aspergillosis.

An FDA orphan drug designation provides several benefits to the sponsor including a seven-year market exclusivity from product approval in the U.S. Isavuconazole was previously granted FDA fast track status that is designed to facilitate development and expedite the review of drugs to treat serious diseases and that fill an unmet medical need in order to get important new drugs to patients earlier.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, commented: "Invasive aspergillosis is a life-threatening infection caused by *Aspergillus* molds. It typically affects patients with an impaired or weakened immune system such as transplant and cancer patients and is associated with high mortality. The granting of orphan designation for isavuconazole in the U.S. reflects the high medical need and is an important regulatory milestone for Basilea and our partner Astellas."

Topline data from two isavuconazole phase 3 studies are expected in the second half of 2013. These include the SECURE phase 3 registration study, evaluating safety and efficacy of once-daily isavuconazole versus twice-daily voriconazole for the primary treatment of invasive fungal disease caused by *Aspergillus* species and from the open-label VITAL study investigating isavuconazole for the treatment of patients with invasive life-threatening fungal disease caused by emerging fungi and the treatment of aspergillosis patients with pre-existing renal impairment.

The isavuconazole ACTIVE phase 3 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, will continue to recruit into 2014.

Invasive aspergillosis is estimated to occur in 5-13 % of recipients of bone marrow transplants, 5-25 % of patients who have received heart or lung transplants, and 10-20 % of patients who are receiving intensive chemotherapy for leukemia.[1] Mortality rates for transplant patients with invasive aspergillosis have been reported to be between 34 and 58 %.[2]

About isavuconazole

Isavuconazole (drug substance: isavuconazonium sulfate) is an investigational intravenous and oral broad-spectrum antifungal. In collaboration with Astellas Pharma Inc., isavuconazole is being investigated in phase 3 clinical studies for the treatment of severe invasive fungal infections. The drug 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 *in-vitro* activity against less prevalent but often fatal molds including those that cause mucormycosis. In clinical studies to date, isavuconazole achieved predictable drug levels supporting reliable dosing and a switch from intravenous administration to a once-daily oral dose. The intravenous formulation of isavuconazole, which is water-soluble, does not contain potentially kidney damaging solubilizers and has the potential to be given also to patients with pre-existing renal impairment.

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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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2 Baddley JW et al. Factors Associated with Mortality in Transplant Patients with Invasive Aspergillosis. Clinical Infectious Disease 2010 (50), 1559-1567