PRESS RELEASE

Basilea reports granting of U.S. orphan drug designation to isavuconazole for the treatment of invasive candidiasis

Basel, Switzerland, November 3, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to isavuconazole for the treatment of invasive candidiasis/candidemia, a potentially life-threatening infection caused by *Candida* yeasts. Isavuconazole has previously been granted orphan drug status in the European Union and the U.S. for the treatment of invasive aspergillosis and mucormycosis.

An FDA orphan drug designation provides several benefits to the sponsor including a seven-year period of market exclusivity in the U.S., should the FDA grant orphan drug exclusivity at approval.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, said: "Invasive candidiasis is a serious bloodstream infection that is associated with high morbidity and mortality. The FDA's grant of orphan drug designation is a further milestone in the development of isavuconazole and underscores the growing need for additional drugs to treat invasive candidiasis."

Previously, the FDA also designated isavuconazole as a Qualified Infectious Disease Product (QIDP) for the treatment of invasive aspergillosis, mucormycosis and candidiasis under the Generating Antibiotic Incentives Now (GAIN) Act. QIDP status provides priority review and a five-year extension of market exclusivity if the drug receives approval in the U.S.

Isavuconazole is currently under regulatory review by the U.S. FDA and the European Medicines Agency (EMA) for the treatment of invasive aspergillosis and mucormycosis in adults. In accordance with the FDA Prescription Drug User Fee Act (PDUFA), the FDA designated the date of March 8, 2015 for the completion of the U.S. New Drug Application review. The regulatory review of the European Marketing Authorization Application is anticipated to be completed by the fourth quarter of 2015.

About invasive candidiasis

Infections by *Candida* yeasts are serious invasive fungal infections and are associated with high morbidity and mortality. Estimates of the attributable mortality of *Candida* bloodstream infections (candidemia) range from 15% to 49%.[1, 2, 3]

About Basilea

Basilea Pharmaceutica Ltd. is headquartered in Basel, Switzerland and listed on the SIX Swiss Exchange (SIX: BSLN). Through the integrated research, development and commercial operations of its Swiss subsidiary Basilea Pharmaceutica International Ltd., the company develops and commercializes 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.

Isavuconazole (active moiety of the prodrug isavuconazonium sulfate) is an investigational once-daily intravenous and oral broad-spectrum antifungal for the potential treatment of life-threatening invasive fungal infections which predominantly occur in immunocompromised patients such as cancer patients undergoing chemotherapy.

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Isavuconazole for the treatment of candidiasis is currently explored in the phase 3 study ACTIVE, which evaluates 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. Enrolment into the ACTIVE study is anticipated to be completed by early 2015.

Information regarding isavuconazole clinical studies is available at www.clinicaltrials.gov.

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 exclusive license holder.

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This press release can be downloaded from www.basilea.com.

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