PRESS RELEASE

Basilea provides update on isavuconazole phase 3 program

- **On track for first data from the SECURE and VITAL phase 3 studies in the second half of this year**
- **Primary efficacy assessment in the ACTIVE phase 3 study moved to the end of intravenous treatment**
- **Recruitment into VITAL study completed after enrollment of 150 patients**
- **Pediatric Investigation Plans agreed by EMA**

Basel, Switzerland, July 17, 2013 – Basilea Pharmaceutica Ltd. (SIX: BSLN) provided an update today on the phase 3 program with the novel antifungal isavuconazole that is being developed in collaboration with Astellas Pharma Inc. Topline data from the SECURE and VITAL phase 3 studies are on track to be available in the second half of 2013. The SECURE registration study evaluates the safety and efficacy of once-daily isavuconazole versus twice-daily voriconazole for the primary treatment of life-threatening invasive fungal disease caused by *Aspergillus* species. The VITAL study is an open-label phase 3 study in the treatment of aspergillosis patients with pre-existing renal impairment or with invasive fungal disease caused by emerging and often fatal fungi. With 150 patients enrolled, the VITAL study has now completed recruitment, which was extended beyond its initial target to further expand the database on the use of isavuconazole in the primary treatment of emerging fungal infections for which currently only limited treatment options exist. The data from the SECURE and VITAL studies could form the basis of an initial filing in the first part of 2014.

The ACTIVE phase 3 study is evaluating 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. The study's primary efficacy endpoint of overall response will be moved to the end of intravenous treatment and assessed by a Data Review Committee. The previous assessment at two weeks after i.v. and oral treatment will remain a secondary efficacy endpoint. There are no changes in the overall operational study conduct. The protocol change will facilitate the comparison to data obtained from previous registration trials in invasive *Candida* infections. The study is expected to continue to recruit in 2014.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, commented: "We are on track to provide the first data from the SECURE and VITAL phase 3 studies in the second half of this year. The move of the ACTIVE study outcome assessment time point will facilitate the comparison of the efficacy of isavuconazole to the data from previous registration trials investigating echinocandins. It reflects the most recent thinking of health authorities on the design and analysis of phase 3 clinical trials investigating antifungals for the treatment of invasive yeast infections."

In addition, the European Medicines Agency has agreed to the Pediatric Investigation Plans (PIP) of isavuconazole for the treatment of invasive aspergillosis, mucormycosis and *Candida* infections in children.

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About isavuconazole

Isavuconazole 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 emerging and 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 once-daily intravenous to oral administration. The intravenous formulation of isavuconazole, which is water-soluble, does not contain possibly kidney damaging solubilizers and has the potential to be given to patients with pre-existing renal impairment. In the U.S. isavuconazole has fast-track status and was granted orphan drug designation for the treatment of invasive aspergillosis.

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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