PRESS RELEASE

Basilea reports positive topline phase 3 results for antifungal isavuconazole

- **Isavuconazole phase 3 invasive aspergillosis study (SECURE) meets primary endpoint**

Basel, Switzerland, September 30, 2013 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today positive topline data from the isavuconazole phase 3 invasive aspergillosis study (SECURE). The antifungal agent isavuconazole is being co-developed with Astellas Pharma Inc.

The randomized, double-blind isavuconazole study (SECURE) achieved its primary objective in demonstrating 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). The all-cause-mortality was 18.6% in the isavuconazole treatment group and 20.2% in the voriconazole group. The 95% confidence interval of the treatment difference between isavuconazole and voriconazole was within the pre-specified non-inferiority margin of 10%.

In addition, the key secondary endpoint of overall success rate (composite of clinical, mycological, radiological responses) at the end-of-therapy in patients with proven/probable disease was similar between isavuconazole and voriconazole (35.0% and 36.4%, respectively). This outcome was based on a blinded assessment by the Independent Data Review Committee.

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 most frequent adverse events reported were nausea, vomiting, pyrexia (fever), diarrhoea, and hypokalaemia (deficiency of potassium in the blood) which were reported at similar rates in both treatment groups. Study drug-related adverse events were reported in 42.4% and 59.8% of patients in the isavuconazole and voriconazole treatment groups, respectively.

Basilea's CEO Ronald Scott commented: "The successful completion of this study is a major achievement and an important milestone for our company. Invasive aspergillosis is a life-threatening disease primarily afflicting immunocompromised patients. There is a great medical need for new antifungal agents to treat these critically ill patients. The results from this isavuconazole study (SECURE) represent an important step in the development of this potential new treatment option for patients suffering from invasive aspergillosis."

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, stated: "We are excited about the positive topline results from the SECURE study, which is the largest interventional trial ever conducted in patients with invasive aspergillosis. The data from the SECURE and VITAL studies could form the basis of an initial filing in the first part of 2014. The VITAL study is expected to provide additional important information for the development and evaluation of isavuconazole, particularly in patients with mucormycosis and patients with pre-existing renal impairment."

<u>Update on other ongoing phase 3 isavuconazole studies (VITAL and ACTIVE)</u>

Enrollment in the open-label phase 3 isavuconazole study (VITAL) including patients with invasive fungal disease caused by mucormycetes and other emerging fungal pathogens and patients with aspergillosis and pre-existing renal impairment has been completed (N=150). Based on the

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investigator reported data, approximately 45 patients were enrolled with mucormycosis 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.

Enrollment in the randomized, double-blind phase 3 isavuconazole study (ACTIVE), evaluating the use of isavuconazole i.v. and oral versus caspofungin i.v. followed by oral voriconazole for the treatment of invasive *Candida* infections, is continuing.

Conference call

Basilea Pharmaceutica Ltd. invites you to participate in a conference call on Monday, September 30, 2013, 4 p.m. (CEST), during which the company will discuss today's press release.

Dial-in numbers are:
+41 (0) 58 310 50 00 (Europe and ROW)
+1 (1) 631 570 5613 (USA)
+44 (0) 203 059 5862 (UK)

A playback will be available 1 hour after the conference call until Wednesday, October 02, 2013, 6 p.m. (CEST). Participants requesting a digital playback may dial:
+41 (0) 91 612 4330 (Europe and ROW)
+1 (1) 866 416 2558 (USA)
+44 (0) 207 108 6233 (UK)
and will be asked to enter the ID 12906 followed by the # sign.

About isavuconazole

Isavuconazole 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 *in-vitro* activity against emerging and often fatal molds including those that cause mucormycosis. Isavuconazole received U.S. FDA fast-track and U.S. orphan drug designation for invasive aspergillosis. Isavuconazole is being co-developed with Astellas Pharma Inc.

About the isavuconazole phase 3 program

The phase 3 program with isavuconazole includes three studies, SECURE, VITAL and ACTIVE. The SECURE study is a global double-blind randomized phase 3 study, designed to evaluate 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 is an open-label phase 3 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, yeasts or dimorphic fungi. 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.

About invasive aspergillosis infections

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] Around 47% of solid organ transplant recipients who developed invasive aspergillosis had renal insufficiency and

acute renal failure was reported for 43% of intensive care unit (ICU) patients with invasive aspergillosis, compared to 20.5% in the general ICU population.[2, 3]

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.

Basilea has a robust pipeline of clinical and preclinical programs. For the ceftobiprole program in pneumonia it expects a decision from regulatory authorities in Europe in the fourth quarter of this year. The anti-cancer product BAL101553 in resistant tumors is anticipated to transition into phase 2a of clinical development this year following establishment of the maximum tolerated dose in the ongoing phase 1 study. Phase 1 development is ongoing for the Gram-negative antibiotic BAL30072, which is being developed under a contract with the U.S. Biomedical Advanced Research and Development Authority (BARDA), providing for funding of up to USD 89 million. Basilea continues to focus on bringing these important products addressing resistance to patients, optimizing value and continuing to effectively manage its financial resources.

Disclaimer

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

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