PRESS RELEASE

Basilea reports additional data on positive isavuconazole phase 3 SECURE study at ECCMID

Basel, Switzerland, May 14, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reported today that detailed efficacy and safety data of the isavuconazole invasive aspergillosis study (SECURE) were presented at the European Congress of Clinical Microbiology and Infectious Diseases (ECCMID) in Barcelona, Spain.

Treatment emergent adverse events for isavuconazole were statistically fewer relative to the study comparator voriconazole in the System Organ Classes of hepatobiliary (8.9% vs. 16.2%), skin (33.5% vs. 42.5%) and eye disorders (15.2% vs. 26.6%). In addition, isavuconazole (42.4%) showed statistically fewer study drug-related adverse events relative to voriconazole (59.8%). In both treatment groups, the most common treatment emergent adverse events for isavuconazole and voriconazole respectively were nausea (27.6% vs. 30.1%), vomiting (24.9% vs 28.2 %), pyrexia (fever) (22.2% vs 30.1%) and diarrhea (23.7% vs 23.2%).

Basilea's antifungal agent isavuconazole is being co-developed with Astellas Pharma Inc.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, stated: "There is an unmet medical need for new antifungals that have a broad spectrum of activity and a favorable safety profile. Based on its activity against both *Aspergillus* spp. and emerging molds, such as mucormycetes, along with its safety profile, isavuconazole may play an important role in the treatment of invasive, potentially life-threatening mold infections." He added: "The results from the SECURE and VITAL phase 3 studies will support regulatory submissions in Europe and in the U.S., which are planned for mid-2014."

Previously announced topline data showed that the randomized, double-blind SECURE study met the primary objective of demonstrating non-inferiority of isavuconazole versus voriconazole for the primary treatment of invasive fungal disease caused by *Aspergillus* species or certain other filamentous fungi. Baseline characteristics of the severely ill patient population enrolled in this trial were balanced between treatment groups and were reflective of patients at risk for invasive fungal disease (mean age 51 years, 84% hematologic malignancies, 66% neutropenic and 20% allogeneic haematopoietic stem-cell transplantation).

The primary endpoint of all-cause mortality through day 42 in the intent-to-treat population (ITT, N=516) was 18.6% in the isavuconazole (ISA) treatment group and 20.2% in the voriconazole (VRC) group. The upper limit of the 95% confidence interval of the adjusted treatment group difference was 5.7% which is below the pre-specified 10% non-inferiority margin. All-cause mortality through day 42 in patients with proven/probable invasive fungal disease (modified intent-to-treat population, mITT) was 19.6% (ISA) and 23.3% (VRC).

Overall response (a composite of clinical, mycological and radiological responses) at end-of-therapy in the mITT population assessed by the independent data review committee was 35.0% for isavuconazole versus 36.4% for the comparator voriconazole.

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Isavuconazole posters and presentations at ECCMID 2014

- *A phase 3 randomised, double-blind trial evaluating isavuconazole vs. voriconazole for the primary treatment of invasive fungal disease caused by Aspergillus spp. or other filamentous fungi (SECURE)* – J. Maertens, T. Patterson, G. Rahav, D. Kontoyiannis, K. Marr, R. Maher, M. Lee, B. Zeiher, A. Ullmann; Oral presentation O230a
- *Pharmacodynamics of the new azole isavuconazole (ISA) in an Aspergillus fumigatus mouse infection model* – S. Seyedmousavi, J. F. Meis, R. J. M. Brüggemann, W. J. G. Melchers, P. E. Verweij, J. W. Mouton; Poster P1698
- *In vivo efficacy of isavuconazole and amphotericin B in a non-neutropenic murine model of disseminated Absidia corymbifera* – P. Warn, A. Sharp; Poster P0106

For further information please visit www.eccmid.org.

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 mucormycosis. In the U.S., isavuconazole was granted FDA fast-track status and received QIDP and orphan drug designation for invasive aspergillosis and mucormycosis (zygomycosis). 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. Topline data from the SECURE study as well as VITAL study results were reported in September 2013 and February 2014, respectively.

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.

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

References

1 E. M. Harman, Medscape Reference, Drugs, Diseases & Procedures, Aspergillosis Clinical Presentation, http://emedicine.medscape.com/article/296052-overview

2 J. W. Baddley et al. Factors associated with mortality in transplant patients with invasive aspergillosis. Clinical Infectious Disease 2010 (50), 1559-1567

3 K. H. Vandewoude et al. Invasive aspergillosis in critically ill patients: attributable mortality and excesses in length of ICU stay and ventilator dependence. Journal of Hospital Infection 2004 (56), 269-276