PRESS RELEASE

Basilea reports that further subgroup and health economic analyses of isavuconazole phase 3 studies will be presented at IDWeek conference

Basel, Switzerland, October 08, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announces today that further subgroup and health economic analyses of data from the phase 3 studies SECURE and VITAL with the investigational antifungal isavuconazole will be presented at the infectious disease conference IDWeek 2014, October 8-12 in Philadelphia (PA), USA.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, said: "The analyses presented at IDWeek provide further support for the safety profile of isavuconazole, which has demonstrated activity against life-threatening invasive fungal infections in a variety of patient populations in two pivotal studies. In addition, the results of a health economic outcome analysis suggest that treatment with isavuconazole may be associated with shorter length of hospital stay as compared to the current standard of care for invasive aspergillosis."

Presentations include analyses of efficacy and safety data for patient subgroups from the SECURE phase 3 study. The SECURE study assessed the efficacy and safety of isavuconazole versus voriconazole as primary treatment of patients suffering from invasive mold disease such as aspergillosis, a severe infection caused by *Aspergillus* or other mold species. Isavuconazole demonstrated comparable efficacy with a lower rate of drug-related adverse events versus the comparator voriconazole in the pre-specified subgroups of patients with proven or probable invasive aspergillosis, patients with and without neutropenia, and patients suffering from pulmonary mold infections. For invasive aspergillosis patients treated with isavuconazole, the difference in fewer drug-related adverse events reached statistical significance.

In addition, a health economic outcome analysis of patients from the SECURE study will be presented. The results favor isavuconazole compared to voriconazole with numerically fewer days of hospital stay (13 vs. 15 days) and lower 30-day hospital readmission rates (18.3% vs. 24.4%).

A further presentation analyses overall response, survival and safety in the subgroup of immunocompromised patients with proven or probable invasive mucormycosis, a high-mortality mold disease, who were treated with isavuconazole in the open-label phase 3 VITAL study. The analysis suggests that in this high-risk immunocompromised population isavuconazole has the potential for becoming a new option for the treatment of mucormycosis. The VITAL study assessed the efficacy and safety of isavuconazole in patients with invasive fungal disease caused by certain emerging fungal pathogens such as Mucormycetes and patients with aspergillosis who had pre-existing renal impairment for which i.v. voriconazole can only be used with caution.

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Presentations on isavuconazole at IDWeek 2014

A Phase 3, Randomized, Double-Blind, Non-Inferiority Trial to Evaluate Efficacy and Safety of Isavuconazole versus Voriconazole in Patients with Invasive Mold Disease (SECURE): Outcomes in Neutropenic Patients. T. Patterson, D. Selleslag, K. Mullane, O. Cornely, W. Hope, O. Lortholary, B. Zeiher, R. Maher, M. Lee, W. Huang, D. Kontoyiannis; Oral #1210, Friday, October 10, 2:00 p.m., Room 109-AB

A Phase 3, Randomized, Double-Blind, Non-Inferiority Trial to Evaluate Efficacy and Safety of Isavuconazole versus Voriconazole in Patients with Invasive Mold Disease (SECURE): Outcomes in Invasive Aspergillosis Patients. D. Kontoyiannis, M. Giladi, M. Lee, M. Nucci, I. Raad, E. Bow, V. A. Morrison, J. Baddley, B. Zeiher, R. Maher, W. Huang, K. A. Marr; Oral #1211, Friday, October 10, 2:15 p.m., Room 109-AB

A Phase 3, Randomized, Double-Blind, Non-Inferiority Trial to Evaluate Efficacy and Safety of Isavuconazole versus Voriconazole in Patients with Invasive Mold Disease (IMD): Outcomes in Patients with Pulmonary Infections. I. Raad, K. M. Mullane, D. Selleslag, G. Thompson, D. Neofytos, S. Shoham, M. Lee, R. Maher, B. Zeiher, F. M. Marty; Poster #825, Friday, October 10, 12:30-2:00 p.m., Hall BC

Health Economic Outcome Analysis of Patients Randomized in the SECURE Phase III Trial Comparing Isavuconazole to Voriconazole for Primary Treatment of Invasive Fungal Disease Caused by Aspergillus Species or Other Filamentous Fungi. N. Khandelwal, B. Franks, F. Shi, J. Spalding, N. Azie; Poster #826, Friday, October 10, 12:30-2:00 p.m., Hall BC

An Open-Label Phase 3 Study of Isavuconazole (VITAL): Focus on Mucormycosis. F. M. Marty, J. R. Perfect, O. A. Cornely, K. M. Mullane, G. Rahav, M. Lee, M. Ito, R. Maher, B. Zeiher, L. Ostrosky-Zeichner; Poster #824, Friday, October 10, 12:30-2:00 p.m., Hall BC

For further information please visit www.idweek.org.

About isavuconazole

Isavuconazole (drug substance: 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.

Isavuconazole is currently under regulatory review by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency 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. It has European Union and U.S. orphan drug status for the treatment of invasive aspergillosis and mucormycosis. In addition, it was granted FDA fast-track status and designated a Qualified Infectious Disease Product (QIDP) for the treatment of invasive aspergillosis, mucormycosis and candidiasis under the U.S. GAIN Act.

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.

About invasive aspergillosis and mucormycosis

Invasive aspergillosis is estimated to occur in 5-13% of bone marrow transplant recipients, 5-25% of patients who have received heart or lung transplants, and 10-20% of patients who have received 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% in the general ICU population.[2, 3]

Mucormycosis (also known as zygomycosis) is an often lethal fungal infection caused by certain emerging molds. Mucormycosis is associated with high morbidity and mortality rates in immunocompromised patients such as patients undergoing chemotherapy or bone marrow transplantation.[4, 5] Left untreated, mucormycosis is almost always lethal, and even with appropriate medical management, mortality rates remain high.[6]

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.

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

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4 F. Lanternier et al. A global analysis of mucormycosis in France: the RetroZygo study (2005-2007). Clinical Infectious Diseases 2012 (54), S35-S43

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