PRESS RELEASE

Basilea reports that isavuconazole receives orphan drug designations in Europe for the treatment of invasive mold infections

Basel, Switzerland, July 14, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports today that the European Commission granted isavuconazole orphan drug designations for the treatment of invasive aspergillosis and mucormycosis (zygomycosis). Isavuconazole is an investigational intravenous and oral broad-spectrum antifungal.

An orphan drug designation provides several benefits to the sponsor including ten years of market exclusivity independent of any existing patent protection, should the product be approved in the European Union (EU). As a standard procedure, the maintenance of the European orphan drug status will require confirmation during the review of a future Marketing Authorization Application.

Ronald Scott, Basilea's Chief Executive Officer, commented: "Invasive aspergillosis and mucormycosis are life-threatening mold infections primarily affecting patients with an impaired or weakened immune system. Isavuconazole is the first antifungal to have EU orphan drug status both for the treatment of invasive aspergillosis and mucormycosis. The designation is an important regulatory milestone for Basilea and supports our development strategy for isavuconazole in Europe."

Basilea's co-development partner Astellas recently submitted a U.S. New Drug Application, seeking approval of isavuconazole for the treatment of invasive aspergillosis and invasive mucormycosis. On this basis, Basilea is preparing a European Marketing Authorization Application (MAA). The MAA is planned to be filed, as scheduled, mid-2014.

About the isavuconazole phase 3 program

The phase 3 program with isavuconazole includes three studies, SECURE, VITAL and ACTIVE. The SECURE study was a global double-blind randomized study and evaluated 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 was an open-label 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 study is currently enrolling patients and will evaluate 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 SECURE and VITAL studies are the basis of the regulatory filings in Europe and the U.S.

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

Basilea Pharmaceutica Ltd. • Grenzacherstrasse 487 • PO Box • 4005 Basel • Switzerland • www.basilea.com

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. Invasive 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 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. Isavuconazole demonstrated *in-vitro* and *in-vivo* coverage of a broad range of yeasts (such as *Candida* species) and molds (such as *Aspergillus* species), including emerging and often fatal molds such as 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). In the European Union, the drug received orphan drug designations for the treatment of invasive aspergillosis and mucormycosis.

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 license holder.

Astellas has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA), seeking approval of isavuconazole for the treatment of invasive aspergillosis and invasive mucormycosis.

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.

Disclaimer

This communication expressly or implicitly contains certain forward-looking statements concerning Basilea Pharmaceutica Ltd. and its business. Such statements involve certain known and unknown risks, uncertainties and other factors, which could cause the actual results, financial condition, performance or achievements of Basilea Pharmaceutica Ltd. to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. Basilea Pharmaceutica Ltd. is providing this communication as of this date and does not undertake to update any forward-looking statements contained herein as a result of new information, future events or otherwise.

For further information, please contact:

Media Relations	Investor Relations
Peer Nils Schröder, PhD Head Public Relations & Corporate Communications +41 61 606 1102 media_relations@basilea.com	Barbara Zink, PhD, MBA Head Corporate Development +41 61 606 1233 investor_relations@basilea.com

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

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

5 J. Ambrosioni et al. Emerging invasive zygomycosis in a tertiary care center: epidemiology and associated risk factors. International Journal of Infectious Diseases 2010 (14S), e100-e103

6 J. Wingard. Zygomycosis: Epidemiology and treatment options. Proceedings 2006 (6), S526-S530

Basilea Pharmaceutica Ltd. • Grenzacherstrasse 487 • PO Box • 4005 Basel • Switzerland • www.basilea.com