# PRESS RELEASE

# Basilea reports that isavuconazole receives Qualified Infectious Disease Product designation from U.S. FDA for the treatment of invasive mucormycosis

***Basel, Switzerland, February 27, 2014*** – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that the U.S. Food and Drug Administration (FDA) designated isavuconazole as a Qualified Infectious Disease Product (QIDP) for the treatment of invasive mucormycosis (also known as zygomycosis), a life-threatening invasive fungal infection caused by certain emerging molds.

QIDP status provides priority review and a five-year extension of market exclusivity in the United States. In 2013, isavuconazole also received QIDP designation for the treatment of invasive aspergillosis, a severe fungal infection caused by widespread molds. These incentives were granted under the 2012 U.S. Generating Antibiotic Incentives Now (GAIN) Act as part of the FDA Safety and Innovation Act.

In the event isavuconazole is approved in the United States, the five years QIDP market exclusivity would be in addition to the seven-year exclusivity based on isavuconazole's orphan drug designation for the treatment of zygomycosis that was granted by the FDA in 2013. In the U.S., isavuconazole also has FDA fast-track status and has received orphan drug designation for the treatment of invasive aspergillosis.

Ronald Scott, Basilea's Chief Executive Officer, commented: "The granting of this second QIDP designation for isavuconazole by the FDA is an important regulatory milestone. It further highlights the important potential role of isavuconazole for the treatment of patients with serious or life-threatening invasive fungal infections such as mucormycosis, which is a devastating infection that typically occurs in severely immunocompromised patients such as cancer patients. There are few treatment options for mucormycosis and the infection is associated with high mortality rates if left untreated."

Isavuconazole is currently in phase 3 clinical development. The results of the two recently completed phase 3 studies (SECURE and VITAL) are planned to form the basis of a potential regulatory filing for the U.S. and Europe mid-2014.

## 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 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 certain emerging and often fatal molds including those that cause mucormycosis. In the U.S., isavuconazole has 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.

Topline results from the randomized, double-blind invasive aspergillosis SECURE phase 3 study showed that isavuconazole was non-inferior to the standard-of-care, voriconazole, as assessed by the primary endpoint of all-cause mortality through day 42. Study drug-related adverse events were significantly lower in the isavuconazole group (42.4%) compared to the voriconazole group (59.8%).



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In addition to a potentially improved safety profile, isavuconazole, through its spectrum of activity against molds causing mucormycosis (zygomycosis) and its predictable drug exposure, has the potential to overcome a number of limitations of the current standard-of-care for the treatment of invasive mold infections.

The open label phase 3 VITAL study enrolled 149 patients. The study included 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. Following completion of the analysis, VITAL study results show that day 42 all-cause mortality in renally-impaired patients with invasive aspergillosis (n = 20) was 15%. In the SECURE study, which due to the comparator did not allow for enrollment of patients with moderate or severe renal impairment, the mortality rate in patients treated with isavuconazole (n = 258) was 18.6%. In addition, day 42 all-cause mortality in VITAL study patients with confirmed mucormycosis (n = 37), which included patients refractory or intolerant to other antifungal therapies, was 37.8%, which is similar to the mortality rates reported in the literature for the treatment of mucormycosis.[1]

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 with anticipated completion of enrollment in the first half of 2015.

## 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

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

## References


1 Lanternier F et al. A global analysis of mucormycosis in France: the RetroZygo study (2005-2007). Clinical Infectious Diseases 2012 (54), Supplement 1, S35-S43