

PRESS RELEASE

Basilea reports protocol amendment resulting in earlier completion of isavuconazole phase 3 invasive candidiasis study

Basel, Switzerland, October 16, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announces today that its partner Astellas Pharma Inc. has filed an amendment to the phase 3 ACTIVE study protocol with the relevant health authorities and ethics committees. The study assesses the investigational antifungal isavuconazole in the treatment of candidemia and other invasive *Candida* infections.

In the protocol amendment, the targeted patient number will be reduced from 526 to 438 which will allow an earlier finalization of the study. A blinded assessment of the overall subject evaluability rate based on the available study data estimates a higher rate of evaluable patients than originally expected. This allows for a reduction in patient numbers while preserving sufficient statistical power for the primary objective of demonstrating non-inferiority in overall response rates at the end intravenous treatment between isavuconazole and caspofungin.

The protocol amendment is subject to regulatory and ethic committee approval.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, stated: "We are pleased that enrolment into the isavuconazole ACTIVE study may now be completed earlier than expected by early 2015 and topline data are anticipated for the second half of 2015, following completion of treatment and follow-up periods."

The phase 3 ACTIVE study is evaluating the safety and efficacy of intravenously (i.v.) and orally administered isavuconazole versus i.v. administered caspofungin followed by oral voriconazole in the treatment of candidemia and other invasive yeast infections caused by *Candida* spp.¹

Isavuconazole is currently under regulatory review by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) 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.

About invasive candidiasis

Infections by *Candida* yeasts are serious invasive fungal infections and are associated with high morbidity and mortality. Estimates of the attributable mortality of *Candida* bloodstream infections (candidemia) range from 15% to 49%.^{2, 3, 4}

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 FDA and the EMA for the treatment of invasive aspergillosis and mucormycosis in adults. 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. Generating Antibiotics Incentives Now (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 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.

References

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