

PRESS RELEASE

Basilea launching antifungal CRESEMBA® (isavuconazole) in Germany

Basel, Switzerland, March 04, 2016 – Basilea Pharmaceutica Ltd. (SIX: BSLN) hosts a symposium to support the launch of the new azole antifungal CRESEMBA® (isavuconazole) in Germany. The symposium will be held on March 5 and 6, 2016 in Berlin. The symposium is co-chaired by Prof. Oliver A. Cornely, Department of Internal Medicine I, University of Cologne, Germany; and Prof. Andrew J. Ullmann, Julius-Maximilians-University, Department of Internal Medicine II, Infectious Diseases, Würzburg, Germany. The focus of the symposium will be on current challenges and recent developments in the management of invasive mold infections.

David Veitch, Basilea's Chief Commercial Officer, commented: "We are excited to be launching CRESEMBA in Germany. The symposium provides an opportunity for clinicians to discuss important clinical data and share their experiences in relation to the treatment of potentially life-threatening invasive mold infections. CRESEMBA addresses an important medical need of immunocompromised patients. We are focused on rolling out CRESEMBA in our core European markets this year."

CRESEMBA® (isavuconazole) was approved by the European Commission in October 2015 for the treatment of adults with invasive aspergillosis and the treatment of adults with mucormycosis for whom amphotericin B is inappropriate. Invasive aspergillosis and mucormycosis are life-threatening fungal infections that often affect immunocompromised patients, such as patients with cancer and after transplantation. Invasive aspergillosis is often fatal. Mucormycosis (also known as zygomycosis) is a rapidly progressive and life-threatening invasive fungal infection, often affecting the nose and sinuses with high mortality.

Prof. Andrew J. Ullmann stated: "Clinicians have with isavuconazole a new antifungal agent with a broad spectrum activity against invasive fungal diseases and an improved safety profile. The combination of these features demonstrates clearly that isavuconazole is an improvement for the care of our patients with severe invasive fungal diseases."

About CRESEMBA® (isavuconazole)

Isavuconazole is an intravenous and oral azole antifungal and the active agent of the prodrug isavuconazonium sulfate. Isavuconazole was co-developed with Astellas Pharma Inc. under an agreement granting Astellas a license to commercialize isavuconazole in the U.S. Basilea holds full isavuconazole rights in markets outside the United States. Isavuconazole is marketed under the trade name CRESEMBA®. The drug was approved in March 2015 by the U.S. FDA for the use for patients 18 years of age and older in the treatment of invasive aspergillosis and invasive mucormycosis. The European Commission granted marketing authorization in October 2015 to isavuconazole for the treatment of adult patients with invasive aspergillosis and for the treatment of adult patients with mucormycosis for whom amphotericin B is inappropriate. The European marketing authorization is valid in all 28 European Union (EU) member states, as well as in Iceland, Liechtenstein and Norway. Isavuconazole has orphan drug designation for the treatment of invasive aspergillosis and mucormycosis in Europe and the U.S.

About the isavuconazole invasive aspergillosis and mucormycosis studies

The approval of CRESEMBA® is based on results from the isavuconazole development program. The safety and efficacy profile of isavuconazole in adult patients with invasive aspergillosis was demonstrated based on data from two phase 3 clinical studies: SECURE, a randomized, double-blind, active-control study in 516 patients (intent-to-treat population, ITT) with invasive aspergillosis, and VITAL, an open-label non-comparative 146-patient study (ITT) of isavuconazole in the treatment of invasive aspergillosis patients with renal impairment, or invasive fungal disease (IFD) caused by rare molds, yeasts or dimorphic fungi, including invasive mucormycosis.

In the SECURE study, isavuconazole was non-inferior to voriconazole based on the primary endpoint of all-cause mortality at Day 42 in the intent-to-treat population. All-cause mortality through Day 42 was 19% in the isavuconazole treatment group and 20% in the voriconazole treatment group.¹

In the SECURE study, similar rates of non-fatal adverse events were observed for isavuconazole and the comparator, voriconazole. Further, the percentage of study drug-related adverse events in invasive aspergillosis patients was 42% for isavuconazole and 60% for voriconazole. In addition, the percentage of treatment-emergent adverse events in the system organ classes of hepatobiliary disorders was 9% for isavuconazole versus 16% for voriconazole; skin or subcutaneous tissue disorders was 33% for isavuconazole versus 42% for voriconazole; and eye disorders was 15% for isavuconazole versus 27% for voriconazole.¹

The safety and efficacy profile of isavuconazole in patients with mucormycosis was demonstrated based on data from the VITAL study, which included a subpopulation of 37 patients with proven or probable mucormycosis, of whom 21 received isavuconazole as primary treatment for their infection. All-cause mortality at Day 42 was 38% which is similar to mortality rates reported in literature for the treatment of mucormycosis. In this trial the rate of overall response against mucormycosis at the end of therapy was 31%, with an additional 29% exhibiting a stable response. For patients receiving isavuconazole as primary therapy, this number was 32%, with an additional 32% having stable disease. The efficacy of isavuconazole for the treatment of mucormycosis has not been evaluated in concurrent, controlled clinical trials.

The most frequent adverse events for patients treated with isavuconazole in clinical phase 3 studies were nausea (26%), vomiting (25%), diarrhea (22%), headache (17%), elevated liver chemistry tests (17%), hypokalemia (14%), constipation (13%), dyspnea (12%), cough (12%), peripheral edema (11%), and back pain (10%).

About aspergillosis and mucormycosis

Aspergillosis is the name given to a wide variety of diseases caused by the airborne fungus *Aspergillus*. Common *Aspergillus* infections include invasive aspergillosis, allergic bronchopulmonary aspergillosis, chronic pulmonary aspergillosis and aspergilloma. Mucormycosis is the name for fungal infections caused by numerous fungi found in soil and mouldy bread.

About Basilea

Basilea Pharmaceutica Ltd. is a biopharmaceutical company developing products that address increasing resistance and non-response to current treatment options in the therapeutic areas of bacterial infections, fungal infections and cancer. The company uses the integrated research, development and commercial operations of its subsidiary Basilea Pharmaceutica International Ltd. to discover, develop and commercialize innovative pharmaceutical products to meet the medical needs of patients with serious and potentially life-threatening conditions. Basilea Pharmaceutica Ltd. is headquartered in Basel, Switzerland and listed on the SIX Swiss Exchange (SIX: BSLN). Additional information can be found at Basilea's website www.basilea.com.

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

References

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