

PRESS RELEASE

Basilea launching antifungal CRESEMBA® (isavuconazole) in the United Kingdom

Basel, Switzerland, March 03, 2016 – Basilea Pharmaceutica Ltd. (SIX: BSLN) hosts a symposium to support the launch of the new azole antifungal CRESEMBA® (isavuconazole) in the UK. The symposium will be held on March 3, 2016 as part of the 7th *Advances Against Aspergillosis* conference in Manchester, UK. It will be chaired by Prof. David W. Denning, Professor of Infectious Diseases, National Aspergillosis Centre, University of Manchester and will focus on current challenges and recent developments in the management of invasive mold infections.

David Veitch, Basilea's Chief Commercial Officer, commented, "We are proud to be launching CRESEMBA in the UK and to share important data at this symposium. The symposium provides an opportunity for clinicians to discuss clinical data and share their experiences in relation to the treatment of potentially life-threatening invasive mold infections. CRESEMBA fills an important medical need and we are focused on rolling out its launch across our core European markets throughout 2016."

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 all too often fatal. Mucormycosis (also known as zygomycosis) is a rapidly progressive and life-threatening invasive fungal infection, often affecting the nose and sinuses with a high mortality.

Prof. David W. Denning, stated: "It's been almost a decade since the last new antifungal drug became available to treat invasive mold infections. I really welcome the introduction of isavuconazole, a new once daily treatment option, with a favourable adverse event and drug interaction profile. A new treatment option will be of great benefit to those affected by these severe fungal infections."

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 the National Aspergillosis Centre

The UK National Aspergillosis Centre (NAC) is based at the University Hospital of South Manchester. The NAC is commissioned by the Department of Health to provide long term care for patients with chronic pulmonary aspergillosis, and provides diagnostic services for patients with all fungal infections, including invasive 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.

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:

Peer Nils Schröder, PhD Head Public Relations & Corporate Communications +41 61 606 1102 media_relations@basilea.com investor_relations@basilea.com
--

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

References

- 1 J. A. Maertens, I. I. Raad, K. A. Marr et al. Isavuconazole versus voriconazole for primary treatment of invasive mould disease caused by *Aspergillus* and other filamentous fungi (SECURE): a phase 3, randomised-controlled, non-inferiority trial. *The Lancet* 2016 (387), 760-769