

PRESS RELEASE

Basilea reports U.S. FDA approval of isavuconazole for the treatment of invasive aspergillosis and invasive mucormycosis

Basel, Switzerland, March 6, 2015 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that the U.S. Food and Drug Administration (FDA) approved Astellas' New Drug Application (NDA) for the use of isavuconazole for patients 18 years of age and older in the treatment of invasive aspergillosis and invasive mucormycosis (also known as zygomycosis). These are life-threatening fungal infections predominantly occurring in immunocompromised patients. Basilea's partner Astellas will market the drug as CRESEMBA® (isavuconazonium sulfate) in the United States.

"We are very pleased with the FDA's approval of isavuconazole, which offers an important new broad-spectrum treatment for patients suffering from life-threatening invasive fungal infections in the United States," said Ronald Scott, Basilea's Chief Executive Officer.

Prof. Andrew J. Ullmann, Head of Infectious Diseases, Julius-Maximilian-University Wurzburg, Germany, said: "We are desperately seeking new antifungal agents that provide broad coverage against fungi including those causing aspergillosis and mucormycosis. Those drugs need to have a good safety profile to be utilized in our severely ill patient populations. The new antifungal agent isavuconazole clearly has the potential to improve patient care by fulfilling these unmet medical needs."

Prof. Achim Kauffhold, Basilea's Chief Medical Officer, added: "We anticipate completion of the review of Basilea's European Marketing Authorization Application for isavuconazole for the treatment of invasive aspergillosis and mucormycosis in adults in the fourth quarter of 2015. In addition, in the second half of 2015 we expect topline results from the isavuconazole ACTIVE phase 3 study in invasive candidiasis, a potential further indication."

Isavuconazole is being co-developed with Astellas Pharma Inc. Basilea holds full global rights to isavuconazole outside the U.S. and Canada where Astellas is the exclusive license holder. A CHF 30 million milestone payment from Astellas is associated with the approval of isavuconazole for the treatment of invasive aspergillosis in the United States.

The safety and efficacy profile of isavuconazole in patients with invasive aspergillosis was demonstrated based on the data from two phase 3 clinical trials in adult patients with invasive fungal infections: SECURE, a randomized, double-blind, active-control study of patients with invasive aspergillosis; and VITAL, an open-label non-comparative study of isavuconazole in patients with invasive aspergillosis and renal impairment or in patients with invasive fungal disease caused by other fungi, including those causing mucormycosis.

In the SECURE study (a study of 516 patients), isavuconazole demonstrated non-inferiority to voriconazole on the primary endpoint of all-cause mortality at Day 42 for the treatment of patients with invasive aspergillosis or other filamentous fungi. All-cause mortality through Day 42 was 18.6 percent in the isavuconazole treatment group and 20.2 percent in the voriconazole treatment group.

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 invasive mucormycosis treated with isavuconazole. All-cause mortality in isavuconazole-treated patients was 38 percent. The efficacy of isavuconazole for the treatment of invasive mucormycosis has not been evaluated in concurrent, controlled clinical studies.

In the SECURE study, the overall safety profile for isavuconazole demonstrated similar rates of mortality and non-fatal adverse events as the comparator, voriconazole. Further, isavuconazole showed statistically fewer study drug-related adverse events relative to voriconazole in invasive aspergillosis patients.¹ In addition, treatment-emergent adverse events for isavuconazole were statistically fewer relative to voriconazole in the system organ classes of hepatobiliary, skin and eye disorders.²

The most frequent adverse events for patients treated with isavuconazole in clinical trials 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 invasive aspergillosis and invasive mucormycosis

Invasive aspergillosis is a life-threatening fungal infection that predominantly affects immunocompromised patients, such as patients with leukemia. Invasive aspergillosis is known for high morbidity and mortality. Invasive mucormycosis (also known as zygomycosis) is a rapidly progressing and life-threatening invasive fungal infection, known for high morbidity and mortality.

About isavuconazole

Isavuconazole is an azole antifungal and the active agent of the prodrug isavuconazonium sulfate. The drug is indicated in the U.S. for use in the treatment of invasive aspergillosis and invasive mucormycosis in adults. Outside the U.S., isavuconazole is an investigational product and currently not approved for commercial use. A European Marketing Authorization Application, submitted by Basilea, for isavuconazole for the treatment of invasive aspergillosis and mucormycosis in adults, is currently under review by the European Medicines Agency.

The following information about isavuconazole is applicable only to the product approved in the United States:

The recommended loading dose of CRESEMBA® is one reconstituted vial or two capsules (372 mg isavuconazonium sulfate equivalent to 200 mg of isavuconazole) every eight hours for six doses (48 hours) via oral or intravenous administration. The recommended maintenance dose is one reconstituted vial or two capsules (372 mg isavuconazonium sulfate equivalent to 200 mg of isavuconazole) once per day via oral or intravenous administration, starting 12 to 24 hours after the last loading dose. Capsules can be taken with or without food. CRESEMBA® for injection must be administered through an in-line filter over a minimum of one hour.

Important Safety Information for CRESEMBA® (isavuconazonium sulfate)

CRESEMBA® is contraindicated in persons with known hypersensitivity to isavuconazole.

Coadministration of strong CYP3A4 inhibitors, such as ketoconazole or high-dose ritonavir (400 mg every 12 hours), with CRESEMBA® is contraindicated because strong CYP3A4 inhibitors can significantly increase the plasma concentration of isavuconazole.

Coadministration of strong CYP3A4 inducers, such as rifampin, carbamazepine, St. John's wort, or long acting barbiturates with CRESEMBA® is contraindicated because strong CYP3A4 inducers can significantly decrease the plasma concentration of isavuconazole.

CRESEMBA® shortened the QTc interval in a concentration-related manner. CRESEMBA® is contraindicated in patients with familial short QT syndrome.

Hepatic Adverse Drug Reactions (e.g., elevations in ALT, AST, alkaline phosphatase, total bilirubin) have been reported in clinical trials and were generally reversible and did not require discontinuation of CRESEMBA®. Cases of severe hepatic adverse drug reactions including hepatitis, cholestasis or hepatic failure including death have been reported in patients with serious underlying medical conditions (e.g., hematologic malignancy) during treatment with azole antifungal agents, including CRESEMBA®. Evaluate liver tests at the start and during therapy. Monitor patients who develop liver abnormalities during CRESEMBA therapy for severe hepatic injury. Discontinue if clinical signs and symptoms consistent with liver disease develop that may be attributable to CRESEMBA®.

Infusion-related reactions including hypotension, dyspnea, chills, dizziness, paresthesia, and hypoesthesia were reported during intravenous administration of CRESEMBA®. Discontinue the infusion of CRESEMBA® if these reactions occur.

Serious hypersensitivity and severe skin reactions, such as anaphylaxis or Stevens Johnson syndrome, have been reported during treatment with other azole antifungal agents. Discontinue CRESEMBA® if a patient develops a severe cutaneous adverse reaction. Caution should be used when prescribing CRESEMBA® to patients with hypersensitivity to other azoles.

During pregnancy, CRESEMBA® may cause fetal harm when administered, and should be used during pregnancy only if the potential benefit to the patient outweighs the risk to the fetus. Women who become pregnant while receiving CRESEMBA® are encouraged to contact their physician.

Following dilution, CRESEMBA® intravenous formulation may form precipitate from the insoluble isavuconazole. Administer CRESEMBA® through an in-line filter.

The most frequent adverse events among CRESEMBA®-treated patients were: nausea (26%), vomiting (25%), diarrhea (22%), headache (17%), elevated liver chemistry tests (16%), hypokalemia (14%), constipation (13%), dyspnea (12%), cough (12%), peripheral edema (11%), and back pain (10%).

The adverse reactions which most often led to permanent discontinuation of CRESEMBA® therapy during the clinical trials were: confusional state (0.7%), acute renal failure (0.7%), increased blood bilirubin (0.5%), convulsion (0.5%), dyspnea (0.5%), epilepsy (0.5%), respiratory failure (0.5%), and vomiting (0.5%).

For full U.S. Prescribing Information, please visit <http://astellas.us/docs/cresemba.pdf>

Conference call

Basilea Pharmaceutica Ltd. invites you to participate in a conference call on Monday, March 9, 2015, 4 p.m. (CET), during which the company will discuss today's press release.

Dial-in numbers are:

+41 (0) 58 310 5000 (Europe and ROW)
+1 (1) 631 570 5613 (USA)
+44 (0) 203 059 5862 (UK)

A playback will be available 1 hour after the conference call until Wednesday, March 11, 2015, 6 p.m. (CET). Participants requesting a digital playback may dial:

+41 (0) 91 612 4330 (Europe and ROW)
+1 (1) 866 416 2558 (USA)
+44 (0) 207 108 6233 (UK)

and will be asked to enter the ID 11656 followed by the # sign.

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 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:

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 D. Kontoyiannis et al. A phase 3, randomized, double-blind, non-inferiority trial to evaluate efficacy and safety of isavuconazole versus voriconazole in patients with invasive mold disease (SECURE): outcomes in invasive aspergillosis patients. IDWeek 2014, oral presentation 1211; <https://idsa.confex.com/idsa/2014/webprogram/Paper46236.html> (accessed March 3, 2015)
- 2 J. Maertens et al. A phase 3 randomised, double-blind trial evaluating isavuconazole vs. voriconazole for the primary treatment of invasive fungal disease caused by *Aspergillus* spp. or other filamentous fungi (SECURE). European Congress of Clinical Microbiology and Infectious Diseases (ECCMID) 2014, oral presentation O230a; https://www.escmid.org/escmid_library/online_lecture_library/material/?mid=12145 (accessed March 3, 2015)