PRESS RELEASE

Basilea announces that *The Lancet Infectious Diseases* published results from the CRESEMBA® (isavuconazole) open-label study for the treatment of mucormycosis

Basel, Switzerland, March 09, 2016 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today that results from the open-label phase 3 VITAL study evaluating CRESEMBA® (isavuconazole) in adult patients with mucormycosis were published in *The Lancet Infectious Diseases*. The article, titled "Isavuconazole treatment for mucormycosis: a single-arm open-label trial and case-control analysis", was published online on March 8 and will also appear in a future print issue of the journal.[1]

Data on a sub-group of mucormycosis patients from the VITAL study supported the mucormycosis registrations for CRESEMBA in the U.S. and Europe, in addition to the invasive aspergillosis indication.

Prof. Achim Kaufhold, Basilea's Chief Medical Officer, said: "CRESEMBA addresses an important medical need. Mucormycosis is an emerging fungal infection that occurs predominantly in immunocompromised patients, such as cancer patients. Without prompt diagnosis and treatment, mucormycosis has a mortality rate that may exceed 90 percent."

CRESEMBA was co-developed with Astellas Pharma Inc. The drug was launched in the United States by Astellas in 2015 following the U.S. approval for the treatment of adults with invasive mucormycosis and invasive aspergillosis. Outside the United States, Basilea has full rights for CRESEMBA. Basilea has launched the drug in the UK and Germany and is planning launches in further European countries throughout 2016. CRESEMBA was approved in Europe by the European Commission in October 2015 for the treatment of adult patients with invasive aspergillosis and the treatment of adult patients with mucormycosis for whom amphotericin B is inappropriate.

Prof. Oliver A. Cornely, University of Cologne, Germany, corresponding author of the publication and investigator in the VITAL and SECURE clinical studies, stated: "Isavuconazole is a welcome new treatment option in the management of mucormycosis. The VITAL and SECURE studies together support the use of isavuconazole as an effective new antifungal treatment for patients with the life-threatening fungal infections of invasive aspergillosis and mucormycosis. Furthermore, isavuconazole is a new once-daily treatment option with a favorable adverse event and drug interaction profile."

Results from the pivotal phase 3 SECURE study, on which the registration for the invasive aspergillosis indication for CRESEMBA was based, were also recently published in *The Lancet*.[2] In the publication isavuconazole was reported as being non-inferior to voriconazole for the primary treatment of suspected invasive mold disease, with fewer study-drug-related adverse events relative to voriconazole. The authors concluded that the results support the use of isavuconazole for the primary treatment of patients with invasive mold disease.

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About the VITAL study

VITAL was a global, open-label non-comparative clinical study that evaluated the efficacy and safety of isavuconazole for the treatment of invasive aspergillosis in patients with renal impairment, and for invasive fungal disease caused by mucormycetes and other emerging fungal pathogens. The primary study endpoint was overall response at Day 42 as assessed by an independent data-review committee (DRC); secondary endpoints included assessments of all-cause mortality at Days 42 and 84. The trial evaluated CRESEMBA at an oral or intravenous (i.v.) loading regimen of 200 mg of isavuconazole every eight hours for six doses, followed by 200 mg of isavuconazole once-daily. 37 mucormycosis patients received isavuconazole: 21 patients for primary therapy, 11 for refractory disease, and five after intolerance to other antifungals. Day 42 all-cause mortality was 38 percent, whilst all-cause mortality was 43 percent by Day 84. A case-control analysis comparing VITAL study patients, who received primary isavuconazole treatment, with controls from the international FungiScope Registry (a database on emerging invasive fungal diseases) demonstrated that mortality did not differ for patients treated with either isavuconazole or amphotericin B formulations. These results provide evidence that isavuconazole is effective for the treatment of mucormycosis, in light of the mortality rates of untreated mucormycosis, which may exceed 90%.

35 of 37 patients (95%) experienced at least one adverse event (AE) during treatment and 28 patients had serious AEs. The most common AEs reported (>10% of patients) were pyrexia (37%), vomiting (32%), diarrhea (27%), nausea (27%), constipation (22%), decreased appetite (16%), headache (16%), peripheral oedema (16%), abdominal pain (14%), dyspnea (14%), pneumonia (14%), back pain (11%), cough (11%), hypoglycemia (11%), insomnia (11%) and restlessness (11%).

About mucormycosis

Mucormycosis is a rapidly progressing and devastating fungal infection. Mucormycosis is known for high morbidity and mortality.

About CRESEMBA® (isavuconazole)

Isavuconazole is an intravenous and oral azole antifungal and the active agent of the prodrug isavuconazonium sulfate. The drug 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 was approved in March 2015 by the United States Food and Drug Administration (FDA) for the use for patients 18 years of age and older in the treatment of invasive aspergillosis and invasive mucormycosis, and was launched in the U.S. by Astellas in April 2015. Isavuconazole is marketed under the trade name CRESEMBA®. 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 member states, as well as in Iceland, Liechtenstein and Norway. Basilea has launched CRESEMBA in the UK and Germany, and launches in further European countries are planned throughout 2016. Isavuconazole has orphan drug designation for the treatment of invasive aspergillosis and mucormycosis in Europe and the U.S.

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

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needs of patients with serious and 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.

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