PRESS RELEASE

Basilea reports topline results of isavuconazole phase 3 study in candidemia and other invasive *Candida* infections

Basel, Switzerland, July 30, 2015 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today topline results from the phase 3 ACTIVE study. The randomized double-blind study evaluated the efficacy and safety of intravenously (i.v.) and orally administered isavuconazole versus a regimen of i.v. caspofungin followed by oral voriconazole, as a potential treatment for adults with candidemia and other invasive *Candida* infections.

The results showed that the study did not meet the primary objective of demonstrating non-inferior efficacy of isavuconazole versus the study comparator at the end of i.v. therapy within the pre-specified non-inferiority margin.

The overall response rates at two weeks after treatment were, however, comparable between the two treatment groups. Overall response at two weeks after treatment was the key secondary endpoint of the study. In addition, the secondary endpoint of all-cause mortality was comparable at study day 14 and day 56 in both treatment groups. The overall safety profile of isavuconazole was similar to caspofungin and consistent with safety data seen in the previously reported phase 3 studies.

Prof. Achim Kaufhold, Chief Medical Officer of Basilea, said: "We are currently reviewing the results of the study in more detail, in order to understand the totality of data to evaluate potential options for isavuconazole related to invasive candidiasis. Detailed results of the study will be submitted for presentation at scientific meetings and publications. We are also focusing on the recent positive recommendation by the European CHMP to approve isavuconazole in the area of highest medical need: the treatment of invasive aspergillosis and mucormycosis."

The overall response at the end of i.v. treatment in the modified intent-to-treat population (mITT; N=400)* was 60.3% in the isavuconazole treatment group and 71.1% in the caspofungin group with an adjusted treatment difference of -10.8% (95% CI; -19.9%, -1.8%). The lower bound of the 95% confidence interval (CI) of the treatment difference between isavuconazole and caspofungin exceeded the pre-specified non-inferiority margin of -15%.

The key secondary endpoint of overall treatment response at two weeks after the end of treatment was 54.8% in the isavuconazole treatment group and 57.2% in the caspofungin/voriconazole treatment group with an adjusted treatment difference of -2.7% (95% CI; -12.2%, 6.8%).

About the ACTIVE study

ACTIVE is a phase 3, double-blind, randomized study of 440 adult patients (ITT population)* with candidemia and other invasive *Candida* infections at multiple sites globally. The primary endpoint of the study was to compare the overall treatment response of isavuconazole versus caspofungin at the end of i.v. therapy as determined by the independent, blinded Data Review Committee (DRC). After study day 10, patients had the option to continue i.v. therapy or switch to oral therapy. The key secondary endpoint was to assess the success rate of overall treatment response at the first follow-up visit (two weeks after the end of therapy) for i.v./oral isavuconazole-treatment versus the comparator regimen of caspofungin i.v. or caspofungin i.v. followed by oral voriconazole.[1]

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About invasive *Candida* infections

Invasive infections by *Candida* yeasts 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 is an azole antifungal and the active agent of the prodrug isavuconazonium sulfate, which is indicated in the U.S. for use in the treatment of adult patients with invasive aspergillosis and invasive mucormycosis. Basilea is co-developing the drug with Astellas Pharma Inc., who is marketing the drug in the United States. Outside the U.S., isavuconazole is an investigational product and currently not approved for commercial use.

Isavuconazole was 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. It has European Union orphan drug status for the treatment of invasive aspergillosis and mucormycosis.

On July 23, 2015, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending approval of isavuconazole for adults as treatment for invasive aspergillosis, and for the treatment of patients with invasive mucormycosis for whom amphotericin B is inappropriate.

Conference call

Basilea Pharmaceutica Ltd. invites you to participate in a conference call today, Thursday, July 30, 2015, 4 p.m. (CEST), 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 Monday, August 03, 2015, 6 p.m. (CEST). 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 12429 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 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 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

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

References

1 Clinicaltrials.gov identifier: NCT00413218

2 J. Morgan et al. Excess mortality, hospital stay, and cost due to candidemia: a case-control study using data from population-based candidemia surveillance. Infection Control and Hospital Epidemiology 2005 (26), 540-547

3 T. E. Zaoutis et al. The epidemiology and attributable outcomes of candidemia in adults and children hospitalized in the United States: a propensity analysis. Clinical Infectious Diseases 2005 (41), 1232-1239

4 O. Gudlaugsson et al. Attributable mortality of nosocomial candidemia, revisited. Clinical Infectious Diseases 2003 (37), 1172-1177

* ITT: intent-to-treat population; all randomized patients who received at least one dose of study medication. mITT: modified intent-to-treat population; the subset of the ITT population of those who have documented invasive candidiasis or candidemia at baseline (based on the assessment by the Data Review Committee)