

## PRESS RELEASE

# Extensive data presented on Basilea's anti-infectives isavuconazole, ceftobiprole and BAL30072 at ICAAC

**Basel, Switzerland, September 5, 2014** – Basilea Pharmaceutica Ltd. (SIX: BSLN) announces today that extensive data will be presented on the anti-fungal isavuconazole and the antibiotics ceftobiprole and BAL30072 at the 54<sup>th</sup> Interscience Conference on Antimicrobial Agents and Chemotherapy (ICAAC), September 5-9, 2014 in Washington, DC (USA).

Basilea's Chief Medical Officer Prof. Achim Kaufhold commented: "Basilea looks forward to the presentation at ICAAC of a wealth and breadth of data on its compounds including Zevtera/Mabelio. The significant data presented underscores the promising profiles of our drug and drug candidates in areas of unmet medical need."

Presentations cover the investigational antifungal isavuconazole, which is co-developed with Astellas Pharma Inc. In particular, data from the SECURE isavuconazole invasive aspergillosis phase 3 study with outcome analyses for subsets of patients with hematologic and other malignancies will be presented. Researchers will also present data from the VITAL phase 3 study on the activity of isavuconazole in patients with invasive fungal disease caused by a variety of emerging fungi that are associated with significant morbidity and mortality.

Basilea's isavuconazole Marketing Authorization Application for the treatment of invasive aspergillosis and mucormycosis (zygomycosis) is under regulatory review in Europe, and a New Drug Application has been filed with the U.S. FDA by Astellas.

In addition, post-hoc efficacy and tolerability analyses from two randomized phase 3 studies for certain Asian populations with ceftobiprole, Basilea's broad-spectrum anti-MRSA antibiotic, will be presented.

Ceftobiprole (ceftobiprole medocartil, brand name Zevtera<sup>®</sup>/Mabelio<sup>®</sup>) is approved in certain European countries<sup>1</sup> for the treatment of hospital-acquired pneumonia (HAP, excluding ventilator-associated pneumonia, VAP) and community-acquired pneumonia (CAP).

Further presentations will include data on the *in-vitro* activity of Basilea's investigational siderophore monosulfactam antibiotic BAL30072 alone and in combination with carbapenem antibiotics against Gram-negative bacteria, and on the role of siderophore receptors for the susceptibility of *Pseudomonas aeruginosa*. BAL30072 is currently in phase 1 clinical testing.

### Posters/Presentations on isavuconazole

*Isavuconazole is Effective for the Treatment of Experimental Cryptococcal Meningitis.*  
L. K. Najvar, N. P. Wiederhold, R. Bocanegra, M. Olivo, W. R. Kirkpatrick, T. F. Patterson; M-427;  
Saturday, September 6, 12:00 PM - 2:00 PM; Exhibit Hall B

*Isavuconazole (Symposium "Old and New Anti-Infective Agents from a Pharmacokinetic/ Pharmacodynamic Perspective").* A. J. Lepak; A-505; Saturday, September 6,  
6:05 PM - 6:45 PM; Room 152 A

*Effect of Multiple Doses of Ketoconazole on the Pharmacokinetics of Isavuconazole in Healthy Subjects.* T. Yamazaki, H. Pearlman, D. L. Kowalski, C. Lademacher, A. V. Desai,  
R. W. Townsend; A-695; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

**Posters/Presentations on isavuconazole (continued)**

*Effect of Multiple Doses of Isavuconazole on the Pharmacokinetics of CYP1A2 Substrate Caffeine and CYP2C8 substrate Repaglinide in Healthy Subjects.* A. Desai, H. Pearlman, T. Yamazaki, C. Lademacher, D. Kowalski, J. Keirns, R. W. Townsend; A-696; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

*Isavuconazole (ISA) Population Pharmacokinetic Modeling from Phase 1 and Phase 3 Clinical Trials and Target Attainment Analysis.* A. Desai, L. Kovanda, D. Kowalski, Q. Lu, R. W. Townsend; A-697; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

*Pharmacodynamics (PD) of Isavuconazole (ISA) For Invasive Pulmonary Aspergillosis (IPA).* H. Box, L. Gregson, J. L. Livermore, T. W. Felton, S. Whalley, J. Goodwin, L. McEntee, A. Johnson, W. W. Hope; A-698; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

*Assessing Isavuconazole Induced Resistance in Aspergillus fumigatus.* C. Jimenez-Ortigosa, S. Matsumoto, M. M. Fouant, L. Kovanda, D. S. Perlin; M-1081; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

*Molecular Epidemiology and Antifungal Susceptibility of Aspergillus terreus Species Complex: A 6-year Surveillance Study at a Tertiary Care Chest Hospital, Delhi, India.* S. Kathuria, C. Sharma, P. K. Singh, F. Hagen, J. F. Meis, A. Chowdhary; M-1084; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

*AFLP and Antifungal Susceptibility of Rhizopus species from Patients with Mucormycosis, Delhi, India.* S. Kathuria, P. K. Singh, F. Hagen, J. F. Meis, A. Chowdhary; M-1086; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

*Isavuconazole and Nine Comparator Antifungal Susceptibility Profiles for Common and Uncommon Opportunistic Fungi Collected in 2013: Application of New Clinical Breakpoints and Epidemiological Cutoff Values.* M. A. Pfaller, S. A. Messer, R. Dietrich, P. R. Rhomberg, R. N. Jones, M. Castanheira; M-1091; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

*In Vitro Combination Therapy with Isavuconazole against Candida spp.* V. Petraitis, M. McCarthy, J. Meletiadis, A. Katragkou, P. W. Moradi, G. E. Strauss, K. L. Myint, K. Hussain, L. L. Kovanda, M. M. Fouant, E. Røilides, R. Petraitiene, T. J. Walsh; M-1106; Sunday, September 7, 11:00 AM - 1:00 PM; Exhibit Hall B

*Resistance Mechanisms to Azoles in Moulds.* J. Chandra, P. Mukherjee, M. A. Ghannoum; M-1278; Monday, September 8, 10:00 AM - 10:15 AM; Room 202 B

*A Phase 3 Randomized, Double-Blind, Non-Inferiority Trial Evaluating Isavuconazole (ISA) vs. Voriconazole (VRC) for the Primary Treatment of Invasive Fungal Disease (IFD) Caused by Aspergillus spp. or other Filamentous Fungi (SECURE): Outcomes by Malignancy Status.* A. J. Ullmann, S. Shoham, W. Huang, S. Mujais; M-1756; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

*A Phase 3 Randomized, Double-Blind, Non-Inferiority Trial Evaluating Isavuconazole (ISA) vs. Voriconazole (VRC) for the Primary Treatment of Invasive Mold Infection (SECURE): Outcomes in Subset of Patients with Hematologic Malignancies (HM).* K. Marr, E. Bow, W. Heinz, M. Lee, R. Maher, B. Zeiher, J. Maertens; M-1757; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

*Outcomes in Patients with Invasive Mold Disease Caused by Fusarium or Scedosporium spp. Treated with Isavuconazole: Experience from the VITAL and SECURE Trials.* O. A. Cornely, L. Ostrosky-Zeichner, G. Rahav, R. Maher, B. Zeiher, M. Lee, J. Perfect; M-1760; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

#### Posters/Presentations on isavuconazole (continued)

Outcomes by Minimum Inhibitory Concentrations from Isavuconazole Phase 3 Trial of Invasive Aspergillosis (SECURE). D. Andes, M. Ghannoum, L. Kovanda, W. Huang, Q. Lu, B. G. Zeiher, M. Jones, W. Hope; M-1761; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

Successful Outcomes in Patients with Invasive Fungal Disease due to *C. gattii* and *C. neoformans* Treated with Isavuconazole: Experience from the VITAL Trial. F. Queiroz-Telles, O. A. Cornely, J. Perfect, L. Kovanda, B. Zeiher, J. Vazquez; M-1773; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

Outcomes in Patients with Invasive Fungal Disease Caused by Dimorphic Fungi Treated with Isavuconazole: Experience from the VITAL Trial. G. R. Thompson, A. Rendon, R. Santos, F. Queiroz-Telles, L. Ostrosky-Zeichner, B. Zeiher, R. Maher, M. Lee, J. Perfect; M-1775; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

#### Poster on ceftobiprole

Efficacy and Tolerability of Ceftobiprole for Treatment of Pneumonia in China, South Korea, and Taiwan: Post-Hoc Analysis of Two Randomized Trials. Y-C. Chuang, M. Saulay, D. Main, A. Kaufhold; L-1742; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

#### Posters on BAL30072

"Two Sides to Every Story": Exploring the functional role of D179 in the  $\Omega$ -loop of KPC-2  $\beta$ -lactamase. M. L. Winkler, M. A. Taracila, K. M. Papp-Wallace, M. G. Page, R. A. Bonomo; C-153; Saturday, September 6, 12:00 PM - 2:00 PM, Exhibit Hall B

Bactericidal activity of BAL30072 Alone And In Combination With Carbapenems Against Gram-negative Bacteria. I. Morrissey, C. Siegmund, E. Genet, M. Neri, S. Hawser, M. Jones, M. Page, A. Santerre Henriksen; C-1371; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

Urinary Concentrations and Antibacterial Activity of BAL30072 against Uropathogens after IV Infusion in Healthy Subjects. F. M. Wagenlehner, B. Blenk, M. Straubinger, C. Wagenlehner, H. Blenk, K. G. Naber; F-1565; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

The PiuA(D)BCE System Affects Activity Of Siderophore-beta-lactam Conjugates In *Pseudomonas aeruginosa*. C. van Delden, M. G. Page, H. Weingart, D. Pletzer, Y. Braun, T. Köhler; C-1453; Monday, September 8, 11:00 AM - 1:00 PM; Exhibit Hall B

#### About isavuconazole

Isavuconazole (drug substance: isavuconazonium sulfate) is an investigational once-daily intravenous and oral broad-spectrum antifungal being developed for the treatment of life-threatening invasive fungal infections which predominantly occur in immunocompromised patients such as cancer patients undergoing chemotherapy.

Isavuconazole demonstrated *in-vitro* and *in-vivo* coverage of a broad range of yeasts (such as *Candida* species) and molds (such as *Aspergillus* species), including emerging and often fatal molds such as those that cause mucormycosis. It has EU and U.S. orphan drug status for the treatment of invasive aspergillosis and mucormycosis. In the U.S., isavuconazole was granted FDA fast-track status and designated a Qualified Infectious Disease Product (QIDP) for invasive aspergillosis, mucormycosis and candidiasis under the U.S. GAIN Act.

In the phase 3 invasive aspergillosis SECURE study, isavuconazole demonstrated non-inferiority to voriconazole on the primary endpoint of all-cause mortality at day 42. The treatment-emergent adverse events for isavuconazole were statistically fewer relative to voriconazole in the system organ classes of hepatobiliary, skin and eye disorders. In addition, isavuconazole showed statistically fewer study drug-related adverse events relative to voriconazole. In both treatment groups, the most common treatment-emergent adverse events were nausea, vomiting, pyrexia (fever) and diarrhea.<sup>2</sup>

Isavuconazole for the treatment of candidiasis is currently being explored in the ongoing phase 3 study ACTIVE, which evaluates the safety and efficacy of intravenously (i.v.) and orally administered isavuconazole versus i.v. caspofungin followed by oral voriconazole in the treatment of invasive *Candida* infections.

Isavuconazole is being co-developed with Astellas Pharma Inc. Basilea holds full rights to isavuconazole in markets outside of the U.S. and Canada, where Astellas is the exclusive license holder.

### About ceftobiprole

Ceftobiprole medocaryl (brand names Zevtera®/Mabelio®) is a bactericidal broad-spectrum intravenous antibiotic from the cephalosporin class, covering Gram-positive and Gram-negative pathogens including methicillin-resistant *Staphylococcus aureus* (MRSA) and *Pseudomonas* spp., which are frequent causes of hospital-acquired pneumonia.<sup>3</sup> It is approved for the treatment of adults with hospital-acquired pneumonia (HAP) (excluding ventilator-associated pneumonia, VAP) and community-acquired pneumonia (CAP) in certain European countries.<sup>1</sup>

### About BAL30072

BAL30072 is an investigational intravenous siderophore monosulfactam antibiotic with bactericidal activity against multidrug-resistant Gram-negative pathogens. It is currently investigated in a phase 1 clinical study evaluating the safety, tolerability, and pharmacokinetics of multiple-ascending doses of intravenously administered BAL30072 in combination with meropenem, an antibiotic of the carbapenem class. *In-vitro* data showed synergistic or additive activity of BAL30072 with antibiotics from this class.<sup>4</sup> The phase 1 study is conducted under a contract with the U.S. Biomedical Advanced Research and Development Authority (BARDA), a division within the U.S. Department of Health and Human Services.

### About Basilea

Basilea Pharmaceutica Ltd. is headquartered in Basel, Switzerland and listed on the SIX Swiss Exchange (SIX: BSLN). Through the integrated research, development and commercial operations of its Swiss subsidiary Basilea Pharmaceutica International Ltd., the company develops and commercializes innovative pharmaceutical products in the therapeutic areas of bacterial infections, fungal infections and oncology, targeting the medical challenge of rising resistance and non-response to current treatment options.

### 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.

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This press release can be downloaded from [www.basilea.com](http://www.basilea.com).

## References

- 1 Following approval under the European decentralized procedure, ceftobiprole has received national licenses in Austria, Belgium, Denmark, Finland, France, Germany, Norway, Spain, Sweden and the United Kingdom; national authorization in Italy and Luxembourg, and reimbursement and pricing authorization in several countries including Spain is ongoing. It is currently under regulatory review in Switzerland.
- 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
- 3 R. N. Jones. Microbial etiologies of hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia. *Clinical Infectious Diseases* 2010 (51), S81-S87
- 4 I. Morissey et al. Activity of BAL30072 alone and in combination with carbapenems against Gram-negative bacteria. European Congress of Clinical Microbiology and Infectious Diseases (ECCMID) 2014, poster P0296