PRESS RELEASE

Basilea reports solid 2014 full-year results, preparing the ground for commercialization of Zevtera® in Europe

- **Antifungal isavuconazole U.S. NDA and European MAA submissions under regulatory review – decisions expected in Q1 2015 in the U.S. and Q4 2015 in the EU**
- **First commercial launch of antibiotic Zevtera® (ceftobiprole medocaril)**
- **Cash and short-term investments of CHF 226 million**

Basel, Switzerland, February 17, 2015 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announces its financial results for the financial year 2014 with a solid year-end cash position of CHF 226.1 million and a net loss of CHF 41.5 million. Basilea achieved important milestones in 2014 including its first launch of Zevtera® (ceftobiprole medocaril).

Regulatory submissions for the antifungal isavuconazole for the treatment of severe mold infections invasive aspergillosis and mucormycosis are currently under review in the U.S. and the European Union (EU). On January 22, 2015, the U.S. Food and Drug Administration's (FDA) Anti-Infective Drugs Advisory Committee recommended the approval of the U.S. isavuconazole New Drug Application (NDA), which was filed by Basilea's partner Astellas. The target date for the FDA to complete its NDA review is March 8, 2015 (Prescription Drug User Fee Act/PDUFA date). Regulatory review of Basilea's EU Marketing Authorization Application (MAA) by the European Medicines Agency (EMA) is anticipated to be completed in the fourth quarter of 2015.

Treatment failure rates in pneumonia, especially in pneumonia caused by methicillin-resistant *Staphylococcus aureus* (MRSA), are still high and have been attributed in part to inadequate initial antibiotic therapy.[1] Basilea announced the launch of its antibiotic Zevtera® (ceftobiprole medocaril) in Germany in December 2014. Ceftobiprole is currently approved in 13 European countries for the treatment of community-acquired pneumonia (CAP) and hospital-acquired pneumonia (HAP) in adults, excluding ventilator-associated pneumonia (VAP), under the trade name of Zevtera® or Mabelio®.* Ceftobiprole provides physicians with a simplified first-line empiric treatment option with its broad-spectrum activity, including MRSA and *Pseudomonas*, thus reducing the need for using combinations of antibiotics.

In addition, Basilea made significant progress on its early-stage development programs in 2014. Basilea initiated a phase 2a study in solid tumor patients for its oncology drug candidate BAL101553, an i.v. and orally available, dual-action microtubule-destabilizing small molecule. Immunocompromised cancer patients are the largest patient group suffering from invasive bacterial and fungal infections, creating potential synergy between Basilea's anti-infective drug portfolio and BAL101553. The company also initiated a phase 1 combination study with Basilea's Gram-negative antibiotic BAL30072 and meropenem, an antibiotic of the carbapenem class. The study is being conducted under the development agreement with the Biomedical Advanced Research and Development Authority (BARDA), a division within the U.S. Department of Health and Human Services.

Ronald Scott, Basilea's CEO, stated: "We met our major milestones again in 2014. Isavuconazole was filed in the U.S. and the EU as planned. We were very pleased by the FDA Advisory Committee's recent recommendation for the approval of isavuconazole in the United States for

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the treatment of the severe mold infections invasive aspergillosis and mucormycosis. A regulatory decision by the FDA is anticipated around the PDUFA date of March 8, 2015 in the U.S. A regulatory decision by the European authorities related to isavuconazole is expected in the fourth quarter this year. Our research and development capabilities and portfolio position Basilea as one of the leading biopharmaceutical companies focused on the high medical need of overcoming drug resistance." He added: "We announced the launch of Zevtera in Germany in December 2014 and anticipate further launches in additional key European markets in 2015. In the event isavuconazole is approved, Basilea would have the unique opportunity of bringing two hospital focused anti-infectives to patients in Europe."

Key figures

(In CHF million, except per share data)	**2014**	**2013**
Contract revenue	42.1	40.5
Revenue from R&D services	0.4	0.4
Other income	0.1	0.4
Total operating income	**42.6**	**41.4**
Research & development expenses	(54.4)	(53.3)
Selling, general & administrative expenses/ General & administrative expenses	(30.1)	(21.3)
Total operating expenses	**(84.5)**	**(74.7)**
Operating loss	**(41.8)**	**(33.3)**
Net loss	**(41.5)**	**(33.0)**
Net cash used for operating activities	(71.5)	(59.5)
Cash and short-term investments	226.1	273.9
Basic and diluted loss per share, in CHF	(4.17)	(3.40)

Notes: Consolidated figures in conformity with US GAAP; rounding was consistently applied.

The consolidated financial statements of Basilea Pharmaceutica Ltd. for 2014 can be found on the company's website at http://annualreport.basilea.com.

Financial summary

Contract revenue in the financial year 2014 amounted to CHF 42.1 million (2013: CHF 40.5 million), including CHF 36.9 million (2013: CHF 36.9 million) related to the global agreement with Stiefel for Toctino® and CHF 3.9 million (2013: CHF 1.9 million) related to the license agreement with Astellas for isavuconazole. Total operating income in 2014 amounted to CHF 42.6 million (2013: CHF 41.4 million).

Research and development expenses amounted to CHF 54.4 million in 2014 (2013: CHF 53.3 million) and were mainly related to activities for the preparation and support of the regulatory filing in the European Union of isavuconazole, for maintaining the supply chain for isavuconazole as well as ceftobiprole, for the phase 1 development of BAL30072 and the phase 2a development of BAL101553. The Company recognized CHF 9.5 million in 2014 (2013: CHF 0.0 million) under the agreement with BARDA related to reimbursement of agreed development costs for BAL30072.

Selling, general and administrative expenses increased to CHF 30.1 million (2013: CHF 21.3 million), mainly related to commercial activities to prepare and support the launch of ceftobiprole in Germany and additional major European countries.

In 2014, operating loss amounted to CHF 41.8 million, compared to CHF 33.3 million in 2013. This change is mainly due to higher operating expenses related to commercial pre-launch and launch activities for ceftobiprole. The net loss amounted to CHF 41.5 million (2013: CHF 33.0 million) and the basic and diluted loss per share to CHF 4.17 (2013: CHF 3.40).

The net cash used for operating activities in 2014 amounted to CHF 71.5 million as compared to CHF 59.5 million in 2013.

Combined cash and short-term investments amounted to CHF 226.1 million as of December 31, 2014, compared to CHF 273.9 million as of December 31, 2013.

Financial outlook

Basilea is focused on launching and establishing ceftobiprole in hospitals in Germany, France, Italy and the UK in 2015. Total operating expenses for 2015 are estimated at approximately CHF 9 million on average per month. Basilea's average operating loss in 2015 is estimated at approximately CHF 4 million per month, with the vast majority of operating income driven by revenue recognition from upfront and milestone payments.

Pipeline update

Ceftobiprole (ceftobiprole medocaril) – *a new-generation broad-spectrum intravenous cephalosporin antibiotic with rapid bactericidal activity against Gram-positive and Gram-negative bacteria, including methicillin-resistant Staphylococcus aureus (MRSA) and susceptible Pseudomonas spp.*[2]

Ceftobiprole is approved in thirteen European countries for the treatment of community-acquired pneumonia (CAP) and hospital-acquired pneumonia (HAP) in adults, excluding ventilator-associated pneumonia (VAP).*

In July 2014, Basilea entered into an agreement with Quintiles to commercialize Zevtera®/Mabelio® (ceftobiprole medocaril) in key European countries. In May 2014, ceftobiprole early clinical benefit data in pneumonia, based on post-hoc analyses of phase 3 data were presented at the European Congress of Clinical Microbiology and Infectious Diseases (ECCMID).[3, 4]

Basilea owns the worldwide rights to ceftobiprole and is in discussions with potential regional partners. Ceftobiprole is currently not approved by the U.S. FDA and is not registered in the USA.

Isavuconazole (isavuconazonium sulfate) – *an investigational once-daily intravenous and oral broad-spectrum antifungal for the treatment of severe and life-threatening invasive fungal infections, which predominantly occur in immunocompromised patients such as cancer patients undergoing chemotherapy*

Isavuconazole was designated as a Qualified Infectious Disease Product (QIDP) by the U.S. FDA under the U.S. Generating Antibiotics Incentives Now (GAIN) Act for the treatment of invasive aspergillosis, mucormycosis, and candidiasis. In addition, it has EU and U.S. orphan drug status for invasive aspergillosis and mucormycosis, and U.S. orphan drug designation for invasive candidiasis. QIDP and orphan drug designations provide certain benefits including extended market exclusivity in the event of approval.

Patient enrolment into the phase 3 ACTIVE study, assessing isavuconazole in the treatment of candidemia and other invasive *Candida* infections in adults, was completed in January 2015. Topline data are anticipated for the second half of 2015, following completion of treatment and follow-up periods.

Isavuconazole is being co-developed with Astellas Pharma Inc. Basilea holds full global rights to isavuconazole except for the U.S. and Canada where Astellas is the exclusive license holder. In 2014, Basilea received a CHF 12 million milestone payment from Astellas related to the U.S. FDA's acceptance of the NDA submission for the treatment of invasive aspergillosis and mucormycosis. Basilea is entitled to further milestone payments totaling up to CHF 362 million.

BAL30072 – *an investigational phase 1 intravenous monosulfactam antibiotic with activity against many clinically relevant multidrug-resistant Gram-negative bacteria*

In June 2014, Basilea initiated a phase 1 clinical study to evaluate the safety, tolerability, and pharmacokinetics of multiple-ascending doses of intravenously administered BAL30072 alone and in combination with meropenem, a carbapenem antibiotic. *In-vitro* data showed synergistic or additive activity of BAL30072 with antibiotics from this class.[5] The phase 1 study is being conducted under a contract with BARDA. Based on milestone achievement, the contract provides development funding of approximately USD 17 million for an initial 22-month period, with potential funding extending to up to USD 89 million over a six-year period in total.

BAL101553 – *an investigational phase 2a intravenous and oral dual-action microtubule-destabilizing small-molecule anti-cancer drug*

BAL101553, the water soluble prodrug of the active moiety BAL27862, has shown initial evidence of clinical anti-tumor activity in phase 1, during which the maximum tolerated dose was established. Currently available phase 1 data indicate a dual-action effect on tumor cell-proliferation and tumor vascularization.[6]

A phase 2a study was initiated in July 2014, assessing the safety and tolerability and obtaining efficacy data of two different doses of BAL101553 in different solid tumor types in adult patients refractory to current therapy in order to facilitate the selection of tumor indications to be included in future expanded phase 2 studies. The study continues biomarker testing to further evaluate dose and the patient populations most likely to respond to treatment.

Toctino® (oral alitretinoin) – *the only drug approved in certain countries for systemic use in adults with severe chronic hand eczema unresponsive to potent topical corticosteroids; in the U.S., oral alitretinoin is an investigational drug and not approved by the FDA*

Global rights to Toctino® were transferred to Stiefel, a GlaxoSmithKline company, in July 2012. Stiefel's preparations of a U.S. NDA for alitretinoin for the treatment of severe chronic hand eczema are ongoing. Basilea is eligible for a milestone payment related to the U.S. launch of alitretinoin and participation in future U.S. product sales.

Conference call

Basilea Pharmaceutica Ltd. invites you to participate in a conference call on Tuesday, February 17, 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 Thursday, February 19, 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 11378 followed by the # sign.

Note to shareholders

The shareholders of Basilea Pharmaceutica Ltd. are informed that the Ordinary General Meeting of Shareholders of Basilea Pharmaceutica Ltd. will take place on **Wednesday, April 29, 2015 at 2 p.m. at the Hilton Hotel in Basel, Switzerland**. The invitation will be published in the Swiss Official Gazette of Commerce (*Schweizerisches Handelsamtsblatt*, SHAB). Shareholders who are recorded in the share register with voting rights on April 17, 2015 will be entitled to participate and exercise their voting rights.

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 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.

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

References

* Ceftobiprole (European trade name Zevtera® or Mabelio®, depending on the country) has received national licenses for the treatment of CAP and HAP (excluding VAP) in adults in Austria, Belgium, Denmark, Finland, France, Germany, Italy, Luxembourg, Norway, Spain, Sweden, Switzerland and the United Kingdom. Reimbursement and pricing authorization in several countries including Spain is ongoing

1 C. Woods, G. Colice. Methicillin-resistant *Staphylococcus aureus* pneumonia in adults. Expert Review of Respiratory Medicine 2014 (8), 641-651

2 Y. Y. Syed. Ceftobiprole medocaril: A review of its use in patients with hospital- or community-acquired pneumonia. Drugs 2014 (74), 1523-1542

3 T. Scheeren et al. Early clinical improvement and clinical cure in a randomised controlled phase 3 study of ceftobiprole versus ceftazidime/linezolid in patients with hospital-acquired pneumonia. European Congress of Clinical Microbiology and Infectious Diseases (ECCMID) 2014, presentation O151

4 T. Welte et al. Early clinical response in a randomised controlled phase 3 study of ceftobiprole versus ceftriaxone with or without linezolid in patients with community-acquired pneumonia requiring

hospitalisation. European Congress of Clinical Microbiology and Infectious Diseases (ECCMID) 2014, poster eP431

5 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

6 L. R. Molife et al. Phase 1/2a trial of the novel microtubule inhibitor BAL101553 in advanced solid tumors: Phase 1 completed. American Society of Clinical Oncology (ASCO) annual meeting 2014, abstract 2562

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