PRESS RELEASE

Basilea reports 2014 half-year financials – ceftobiprole launch in Germany planned for second half of 2014

- **Solid operational and financial performance**
- **Basilea submitted isavuconazole European MAA and Astellas Pharma Inc. submitted U.S. NDA**
- **Full rights to isavuconazole obtained outside U.S. and Canada**
- **Cash and short-term investments of CHF 246 million**

Basel, Switzerland, August 14, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announces today its financial results for the first half of 2014 with a solid half-year cash position of CHF 245.9 million.

Basilea achieved significant milestones in the first part of the year. In July 2014, Basilea submitted a European Marketing Authorization Application (MAA) and its co-development partner Astellas Pharma Inc. submitted a U.S. New Drug Application (NDA) seeking isavuconazole approval for the treatment of the severe mold infections invasive aspergillosis and mucormycosis. Basilea is eligible for a milestone payment from Astellas upon the U.S. Food and Drug Administration's (FDA) acceptance of the NDA submission. The FDA's NDA review could be completed by the second quarter of 2015 considering isavuconazole's Qualified Infectious Disease Product (QIDP) designation and related priority review. European regulatory review could be completed by the fourth quarter of 2015.

Basilea entered into an agreement with Quintiles in July 2014 for commercialization in key European countries of Zevtera®/Mabelio® (ceftobiprole medocaril) for patients with hospital- and community-acquired pneumonia. Basilea's broad spectrum antibiotic covers Gram-positive and Gram-negative pathogens such as Methicillin-resistant *Staphylococcus aureus* (MRSA) and *Pseudomonas* spp.

Ronald Scott, Basilea's CEO, stated: "Basilea achieved major milestones on time this year. We submitted a Marketing Authorization Application in Europe and our partner Astellas submitted a U.S. New Drug Application for isavuconazole for the treatment of invasive aspergillosis and mucormycosis. We made significant progress on Zevtera/Mabelio national pricing and reimbursement in key European markets, completed an agreement with a commercial services provider and anticipate launching Zevtera in Germany in the second half of the year, followed by launches in other key European markets in 2015." He added: "In the event isavuconazole is approved next year, in addition to Zevtera/Mabelio, Basilea could have two hospital anti-infectives with European market authorization by the end of 2015. This would afford the company significant commercialization synergies and put Basilea in a strong position to optimize the value of these two drugs."

Prof. Achim Kaufhold, Basilea's Chief Medical Officer commented: "We are pleased about the advancements of our clinical pipeline. Basilea successfully completed the phase 1 study of our microtubule-targeting oncology drug candidate BAL101553 and swiftly initiated a phase 2a study including patients with solid tumors refractory to current therapies. In addition, we initiated a phase 1 study of our Gram-negative antibiotic BAL30072 in combination with meropenem. BAL30072 in combination with meropenem has been shown to have synergistic or additive

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activity against a broad range of clinically relevant multidrug-resistant Gram-negative pathogens, for which there are currently few or no treatments available. This program is funded under an agreement with BARDA, the U.S. Biomedical Advanced Research and Development Authority."

Key figures

(In CHF million, except per share data)	**H1 2014**	**H1 2013**
Contract revenue	20.2	20.4
Revenue from R&D services	0.1	0.1
Other income	0.0	0.1
Total operating income	20.3	20.6
Research & development expenses	(27.5)	(26.7)
General & administrative expenses	(12.3)	(11.3)
Total operating expenses	(39.8)	(38.0)
Operating loss	(19.5)	(17.4)
Net loss	(19.4)	(17.3)
Net cash (used for) operating activities	(44.9)	(33.7)
Cash and short-term investments	245.9	262.8
Basic and diluted loss per share, in CHF	(1.87)	(1.80)

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

The unaudited condensed consolidated financial statements of Basilea Pharmaceutica Ltd. for the first half-year 2014 can be found on the company's website at http://interimreport.basilea.com.

Financial summary

Contract revenue in the first six months of the financial year 2014 amounted to CHF 20.2 million (H1 2013: CHF 20.4 million), including CHF 18.5 million related to the global agreement with Stiefel for Toctino® and CHF 1.7 million related to the license agreement with Astellas for isavuconazole. Total operating income in the first half-year 2014 amounted to CHF 20.3 million (H1 2013: CHF 20.6 million).

Research and development expenses were CHF 27.5 million in the first six months of 2014, compared to CHF 26.7 million in the first six months of 2013.

General & administrative expenses increased to CHF 12.3 million (H1 2013: CHF 11.3 million) primarily due to commercial pre-launch activities related to ceftobiprole.

In the first half-year of 2014, operating loss amounted to CHF 19.5 million, compared to CHF 17.4 million in H1 2013. This change is mainly due to higher operating expenses related to pre-launch activities for commercialization and launch material for ceftobiprole. The net loss amounted to CHF 19.4 million (H1 2013: CHF 17.3 million) and the basic and diluted loss per share to CHF 1.87 (H1 2013: CHF 1.80).

The net cash used for operating activities in the first six months of 2014 amounted to CHF 44.9 million as compared to CHF 33.7 million in the first six months of 2013.

Combined cash and short-term investments amounted to CHF 245.9 million as of June 30, 2014, compared to CHF 262.8 million as to June 30, 2013.

Financial outlook

Basilea confirms its unchanged guidance: total operating expenses for 2014 are estimated at CHF 8 to 9 million on average per month. The operating loss in 2014 is estimated at CHF 4 to 5 million on average per month.

Pipeline update

Zevtera®/Mabelio® (ceftobiprole medocaril) – *a bactericidal broad-spectrum intravenous antibiotic from the cephalosporin class, covering Gram-positive and Gram negative pathogens such as MRSA and Pseudomonas spp., which are frequent causes[1] of hospital-acquired pneumonia (HAP). It is approved for the treatment of adults with HAP (excluding ventilator-associated pneumonia, VAP) and community-acquired pneumonia (CAP) in certain European countries[2]* and is *under regulatory review in Switzerland*

In May 2014, further retrospective phase 3 data analyses were presented at the European Congress of Clinical Microbiology and Infectious Diseases (ECCMID) which demonstrated more rapid clinical responses in patients with HAP and CAP treated with ceftobiprole than with the comparator regimen.[3, 4]

Isavuconazole – *an investigational once-daily intravenous and oral broad-spectrum antifungal for the potential treatment of invasive life-threatening fungal infections*

In the first half of 2014, the U.S. FDA designated isavuconazole as a Qualified Infectious Disease Product (QIDP) for the treatment of invasive mucormycosis and invasive candidiasis. QIDP designation for the treatment of invasive aspergillosis was granted last year. The QIDP status provides isavuconazole priority review and a five-year extension of market exclusivity if the drug is approved in the United States.

In July 2014, the European Commission granted isavuconazole Orphan Drug status for invasive aspergillosis and mucormycosis, providing ten years of market exclusivity independent of any existing patent protection, should the drug be approved in the European Union. In the U.S., isavuconazole was granted Orphan Drug status for invasive aspergillosis and mucormycosis in 2013.

BAL30072 – *an investigational intravenous monosulfactam antibiotic with bactericidal activity against multidrug-resistant Gram-negative pathogens*

In June 2014, Basilea initiated 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.[5]

The phase 1 study is conducted under the contract with the U.S. Biomedical Advanced Research and Development Authority (BARDA), a division within the U.S. Department of Health and Human Services, which was concluded in June 2013 and provides development funding of approximately USD 17 million for an initial twenty-two months period, and up to USD 89 million over a total six-year period.

BAL101553 – *an investigational small-molecule microtubule-targeting agent (MTA) with the potential for intravenous and oral administration. Currently available phase 1 data indicate a dual action by inducing tumor cell death and disrupting tumor blood supply[6]*

Preclinical data presented at the American Association of Cancer Research (AACR) annual meeting in April 2014 demonstrated the activity of BAL101553 in models of human breast cancer.[7]

Results of a phase 1 study with intravenous BAL101553 were presented at the American Society of Clinical Oncology (ASCO) annual meeting in June 2014, including the maximum tolerated dose and first evidence of clinical antitumor activity. Among 21 evaluable patients, one patient showed a partial response for more than two years, and five patients showed stable disease. BAL101553 also had an effect on tumor cell proliferation and tumor vascularization as demonstrated by comparison of post to pre-treatment tumor biopsies.[6]

A phase 2a study was initiated in July 2014, assessing the safety and tolerability and obtaining efficacy data of two 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 will continue biomarker testing to further evaluate dose and the patient populations most likely to respond.

Toctino® (oral alitretinoin) – *the only drug licensed 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 in phase 3 and not yet approved by the FDA*

In July 2012, Toctino® was transferred to Stiefel, a GSK company. Stiefel plans to submit a New Drug Application for alitretinoin for the treatment of severe chronic hand eczema to the U.S. FDA in the fourth quarter of 2014. 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 Thursday, August 14, 2014, 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 Saturday, August 16, 2014, 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 10426 followed by the # sign.

About Basilea

Basilea Pharmaceutica Ltd. is headquartered in Basel, Switzerland and listed on the SIX Swiss Exchange (SIX: BSLN). Through the fully integrated research and development operations of its Swiss subsidiary Basilea Pharmaceutica International Ltd., the company focuses on 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,

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

References

1 R. N. Jones. Microbial etiologies of hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia. Clinical Infectious Diseases 2010 (51), S81-S87

2 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

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

7 F. Bachmann et al. BAL101553 (prodrug of BAL27862): A unique microtubule destabilizer active against drug refractory breast cancers alone and in combination with trastuzumab. American Association for Cancer Research (AACR) Annual Meeting 2014, abstract 831