PRESS RELEASE

Basilea 2013 full-year results: strong operational and financial performance

- **Cash and short-term investments of CHF 274 million**
- **Significantly reduced net loss**
- **Approval of antibiotic ceftobiprole in Europe**
- **Positive phase 3 topline data for antifungal isavuconazole**
- **BARDA: Up to 89 million US dollars funding for antibiotic BAL30072**

Basel, Switzerland, February 11, 2014 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its results for the full financial year 2013 with a solid year-end cash position of CHF 273.9 million and a significantly reduced net loss of CHF 33.0 million. Basilea continued its focused investment into its key value-driving assets.

Basilea reported significant achievements in 2013 including the approval of ceftobiprole in pneumonia in Europe, positive results of the isavuconazole phase 3 SECURE study, and a contract for non-dilutive funding of up to USD 89 million for its novel antibiotic BAL30072.

In 2013, Basilea successfully continued to build a competitive hospital drug portfolio focused on breaking resistance against currently available therapies in the areas of invasive life-threatening bacterial infections, fungal infections and oncology. Its portfolio positions Basilea as one of the leading biopharmaceutical companies in the area of anti-bacterials and antifungals. Immunocompromized 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 the oncology drug candidate BAL101553.

Ceftobiprole

The development of new anti-MRSA drugs remains an important goal for improving global health and MRSA continues to be listed as a "serious threat" in the 2013 report on antibiotics resistance by the U.S. Centers for Disease Control and Prevention. Ceftobiprole is the first anti-MRSA cephalosporin to receive approval for both hospital- and community-acquired bacterial pneumonia in Europe and is anticipated to be launched in initial European countries in 2014. Basilea is continuing its discussions with the FDA. To support a timely launch, Basilea has established a supply chain and produced commercial launch material. Basilea has worldwide rights and is continuing its discussions with potential global and regional commercialization partners. Basilea aims to maximize synergy between ceftobiprole and isavuconazole for which it has co-promotion rights. Isavuconazole may possibly be launched next year.

Isavuconazole

Basilea and its partner Astellas Pharma Inc. reported positive topline data from the isavuconazole SECURE phase 3 study for the treatment of invasive fungal disease caused by *Aspergillus* molds. Isavuconazole was compared head-to-head against the standard-of-care, voriconazole, and demonstrated non-inferiority as assessed by the primary endpoint of all-cause mortality through day 42. Study drug-related adverse events were significantly lower in the isavuconazole group (42.4%) compared to the voriconazole group (59.8%). In addition to a potentially improved safety profile, isavuconazole, through its spectrum of activity against molds causing zygomycosis (mucormycosis) and its predictable drug exposure, has the potential to

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overcome a number of limitations of the current standard-of-care for the treatment of invasive mold infections.

The VITAL open label phase 3 isavuconazole study enrolled 149 patients. The study included patients with invasive fungal disease caused by emerging fungal pathogens such as Mucormycetes and patients with aspergillosis who had pre-existing renal impairment for which i.v. voriconazole can only be used with caution. Study results show that day 42 all-cause mortality in renally-impaired patients with invasive aspergillosis (n = 18) was 16.7%. In the SECURE study, which due to the comparator did not allow for enrollment of patients with moderate or severe renal impairment, the mortality rate in patients treated with isavuconazole (n = 258) was 18.6%. These data, together with safety and pharmacokinetic information support the potential use of isavuconazole in the treatment of patients with pre-existing renal impairment. In addition, in the VITAL study, day 42 all-cause mortality in patients with confirmed mucormycosis (n = 35), which included patients refractory or intolerant to other antifungal therapies, was 40% which is similar to the mortality rates reported in the literature for the treatment of mucormycosis. These data will be supportive in the planned filing.

The analyses of the SECURE and VITAL phase 3 study data are currently being completed to support a potential filing for the U.S. and Europe mid-2014 while focusing on completing enrollment into the ACTIVE study in the first half of 2015. Prior to regulatory filing Basilea will carefully assess the value of its co-promote option for isavuconazole in all key markets.

Isavuconazole is the first identified antifungal that was designated Qualified Infectious Disease Product (QIDP) status under the U.S. Generating Antibiotic Incentives Now (GAIN) Act. In combination with the U.S. Orphan Drug status for the treatment of invasive aspergillosis this provides for twelve years market exclusivity, should isavuconazole be approved in the U.S. In addition, isavuconazole received U.S. Orphan Drug status for the treatment of zygomycosis, a life-threatening fungal infection caused by certain emerging molds for which only limited treatment options are available.

BAL30072

The decreasing number of new antibiotics and rising resistance rates have triggered international action to promote the development of new antibiotics. Basilea successfully concluded a contract for the development of its novel antibiotic BAL30072 with the Biomedical Advanced Reseach and Development Authority (BARDA), a division in the U.S. Department of Health and Human Service. The contract provides non-dilutive funding of approximately USD 17 million for the initial twenty-two months period, and up to USD 89 million over a total six-year period. BAL30072 is intended for the treatment of multidrug-resistant Gram-negative bacterial infections, where current antibiotics increasingly fail. Given the synergistic or additive activity of BAL30072 in combination with carbapenems against multidrug-resistant Gram-negative pathogens, further phase 1 development work this year will include a combination study with a carbapenem.

BAL101553

First evidence of clinical anti-tumor activity in solid tumor patients for Basilea's phase 1 oncology compound BAL101553 targeting tumors resistant to current cancer therapies was presented at ASCO 2013. Moreover, reduced tumor cell proliferation and tumor vascularization were observed in patient tumor biopsies post-treatment. Phase 1 was successfully completed and the maximum tolerated dose has been established. This provides the basis for phase 2a i.v. testing in patients with different solid tumor types. The phase 2 program is expected to commence in the first half of 2014 and will also further investigate biomarkers to identify tumor types most likely to respond and to further support the unique mode of action of this novel small-molecule microtubule-targeting anti-cancer compound.

Ronald Scott, Basilea's CEO stated: "We accomplished our 2013 key goals: the approval of ceftobiprole in pneumonia in Europe, positive top-line data in the isavuconazole phase 3 SECURE study and non-dilutive funding for our novel antibiotic BAL30072. Moreover, we recently completed phase1 of our oncology drug BAL101553." He added: "In 2014 we will continue to focus on delivering operational excellence. Basilea has a unique opportunity to optimize the value of two complementary potential anti-infective therapies, ceftobiprole and isavuconazole. While determining the best overall commercialization strategy for ceftobiprole, we are preparing for the entry of ceftobiprole into key European markets once pricing and reimbursement assessments are completed by regulatory agencies. We are also carefully assessing the value of our co-promote option for isavuconazole in all key markets prior to the regulatory filing of the drug."

Key figures

(In CHF million, except per share data)	**2013**	**2012**
Product sales*	-	20.2
Contract revenue	40.5	37.4
Revenue from R&D services	0.4	0.2
Other income	0.4	0.5
Total operating income	**41.4**	58.3
Cost of sales	-	(4.4)
Research & development expenses	(53.3)	(58.9)
Selling*, general & administrative expenses	(21.3)	(45.9)
Total operating expenses	**(74.7)**	(109.2)
Operating loss	**(33.3)**	(50.8)
Net loss	**(33.0)**	(53.0)
Net cash (used for)/provided by operating activities	(59.5)	148.2
Cash and short-term investments	273.9	344.0
Basic and diluted loss per share, in CHF	(3.40)	(5.53)

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

*2012 numbers: Through July.

The consolidated financial statements of Basilea Pharmaceutica Ltd. for 2013 can be found on the company's website at http://annualreport.basilea.com.

Financial summary

Contract revenue in 2013 amounted to CHF 40.5 million (2012: CHF 37.4 million), including CHF 36.9 million related to the global agreement with Stiefel on Toctino® and CHF 3.6 million related to the license agreement with Astellas for isavuconazole. Total operating income in 2013 amounted to CHF 41.4 million (2012: CHF 58.3 million), the reduction compared to 2012 primarily being due to the global agreement with Stiefel on Toctino® entered in July 2012.

Research and development expenses amounted to CHF 53.3 million in 2013, compared to CHF 58.9 million in 2012. This decrease is primarily related to lower expenses for isavuconazole in the context of the cost-sharing mechanism under the license agreement with Astellas.

Selling, general and administrative expenses decreased to CHF 21.3 million in 2013 (2012: CHF 45.9 million) primarily due to the transfer of commercial activities under the global agreement with Stiefel related to Toctino®.

In 2013, the operating loss decreased to CHF 33.3 million from CHF 50.8 million in 2012, mainly due to lower operating expenses. As a result, the average monthly operating loss in 2013 was CHF 2.8 million. The net loss 2013 amounted to CHF 33.0 million, compared to CHF 53.0 million in 2012.

2013 basic and diluted loss per share improved to CHF 3.40, compared to basic and diluted loss per share of CHF 5.53 in 2012.

In 2013, the net cash used by operating activities amounted to CHF 59.5 million as compared to net cash provided by operating activities of CHF 148.2 million in 2012, mainly due to the upfront payment of CHF 224.1 million received from Stiefel related to the Toctino® agreement in 2012. Following the respective approval at the Ordinary General Meeting of Shareholders in April 2013, Basilea distributed CHF 5.00 per share, corresponding to CHF 48.0 million, in June 2013 from capital contribution reserves.

Combined cash and short-term investments amounted to CHF 273.9 million as of December 31, 2013, compared to CHF 344.0 million at year-end 2012.

Financial outlook

Total operating expenses for 2014 are estimated at approximately CHF 7 to 8 million per month. Basilea's average operating loss in 2014 is estimated at approximately CHF 3 to 4 million per month.

Portfolio

Isavuconazole – *an investigational once daily intravenous and oral broad-spectrum antifungal for the potential treatment of severe invasive and life-threatening fungal infections. Positive phase 3 topline results in invasive aspergillosis were reported in September 2013.*

Isavuconazole has demonstrated in-vitro and in-vivo coverage of a broad range of yeasts (such as *Candida* species) and molds (such as *Aspergillus* species) as well as activity in in-vitro studies and animal models against certain emerging and often fatal molds including those that cause zygomycosis (also known as mucormycosis). In the U.S., isavuconazole received FDA fast-track status, QIDP and orphan drug designation for invasive aspergillosis as well as orphan drug status for zygomycosis. Isavuconazole is being co-developed with Astellas Pharma Inc.

Ceftobiprole – *a broad-spectrum intravenous antibiotic from the cephalosporin class for the first-line treatment of severe bacterial infections. It was approved by twelve EU member states for the treatment of hospital-acquired pneumonia (excluding ventilator-associated pneumonia) and community-acquired pneumonia and is currently under regulatory review in Switzerland.*

Ceftobiprole is a bactericidal antibiotic. No other single agent has such broad-spectrum activity that includes methicillin-resistant *Staphylococcus aureus* (MRSA) and *Pseudomonas*. It also covers further Gram-positive bacteria such as vancomycin-resistant *Staphylococcus aureus* (MRSA, VRSA) and penicillin- and ceftriaxone-resistant Streptococcus pneumoniae (PRSP, CRSP) as well as additional Gram-negative pathogens, including Enterobacteriaceae.[1]

BAL30072 – *an intravenous monosulfactam antibiotic with bactericidal activity against multidrug-resistant Gram-negative bacterial infections, currently in phase 1 clinical development.*

BAL30072 has demonstrated *in-vitro* and *in-vivo* coverage of Gram-negative pathogens including multidrug-resistant *Acinetobacter baumannii and Pseudomonas aeruginosa*. It has robust activity against common strains of resistant pathogens, including those that produce antibiotic-inactivating enzymes such as carbapenemases and metallo-beta-lactamases. In addition, BAL30072 has shown synergistic or additive activity with antibiotics from the carbapenem class. Basilea entered a contract with U.S. BARDA for up to USD 89 million in funding for the development of BAL30072.

BAL101553 – *a novel intravenous and oral small-molecule anti-cancer drug with a dual mode of action. A dose-escalation phase 1 study with intravenously administered BAL101553 was recently completed.*

BAL101553 directly attacks tumor cells by destabilizing the intracellular microtubule network that is essential for cell division. In addition, it disrupts tumor blood vessels depriving the tumor from nutrition. The drug has shown potent anti-proliferative activity in a panel of tumor models, including many that are, as a result of diverse resistance mechanisms, not responsive to conventional microtubule-targeting agents, such as taxanes. BAL101553 is a water-soluble prodrug of Basilea's BAL27862, formulated as an injectable dosage form without potentially harmful solubilizers. In addition, it is orally bioavailable.

Toctino® (oral alitretinoin) – *the only licensed drug for systemic use in adults with severe chronic hand eczema unresponsive to potent topical corticosteroid*

Toctino® was developed and successfully brought to market by Basilea. In the U.S., oral alitretinoin is an investigational drug in phase 3 and not yet approved by the FDA. In July 2012, the Toctino® business was transferred to Stiefel, a GSK company. 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 11, 2014, 4 p.m. (CET), during which the company will discuss today's press release.

Dial-in numbers are:
+41 (0) 58 310 50 00 (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 13, 2014, 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 17303 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 9, 2014 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 March 28, 2014 will be entitled to participate and exercise their voting rights.

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, 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

1 Walkty A et al. In vitro activity of ceftobiprole against frequently encountered aerobic and facultative Gram-positive and Gram-negative bacterial pathogens: results of the CANWARD 2007–2009 study. Diagnostic Microbiology and Infectious Disease 2011 (69), 348-355