PRESS RELEASE

Basilea reports 2013 half-year financials with improved operating results

- **Continued improvement in financial performance**
- **Cash and short-term investments of CHF 262.8 million**
- **Guidance on isavuconazole phase 3 topline data and ceftobiprole regulatory decision in Europe maintained for H2 2013**
- **Significant agreement with BARDA of up to USD 89 million funding for novel antibiotic BAL30072 announced in H1 2013**

Basel, Switzerland, August 21, 2013 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today financial results for the first half-year 2013 with improved operating results, a net loss of CHF 17.3 million and cash and short-term investments of CHF 262.8 million at the end of the period.

In the first six months of 2013, Basilea made substantial progress toward the company's major milestones: the reporting of the isavuconazole phase 3 data and a potential approval of ceftobiprole. In addition, the company continued to advance its earlier-stage clinical programs.

For the antifungal isavuconazole, Basilea and its partner Astellas Pharma Inc. are currently preparing the phase 3 data analyses of the SECURE study in aspergillosis and the VITAL study in aspergillosis patients with kidney impairment or with invasive fungal disease caused by emerging and often fatal fungi. Topline data continues to be expected in H2 2013.

In Europe, the regulatory procedure of the marketing authorization application for Basilea's broad-spectrum, anti-MRSA antibiotic ceftobiprole for the treatment of pneumonia in the hospital is moving forward according to plan. If the regulatory decision anticipated in H2 2013 is positive, first approvals of ceftobiprole in Europe would be possible this year. Regarding the U.S., the FDA indicated that their current guidelines mandating two pivotal trials per indication are still valid. Basilea is continuing its discussions with the agency.

Basilea's continued commitment to address the medical challenges of resistance is demonstrated by the progress made on Basilea's two phase 1 compounds from its research. Both drug candidates are unique and highly differentiated from current therapies or drugs in development. Due to the broad activity of BAL30072 against multidrug-resistant Gram-negative pathogens including those that pose a biothreat, the Biomedical Advanced Research and Development Authority (BARDA), a division within the U.S. Department of Health and Human Services, entered a contract with Basilea for up to USD 89 million for the development of BAL30072. The phase 1 development of BAL30072, which will include combination studies with antibiotics from the carbapenem class is ongoing.

First evidence of anti-tumor activity from phase 1 study data was presented in June at the Annual Meeting of the American Society of Clinical Oncology (ASCO) for Basilea's novel microtubule-destabilizing and vascular disrupting anti-cancer drug, BAL101553. The program is anticipated to transition into phase 2a of clinical development this year following establishment of the maximum tolerated dose in the ongoing phase 1 study.

Stiefel is in the process of preparing for a U.S. filing of alitretinoin for the treatment of severe refractory chronic hand eczema planned for 2014. Basilea participates in the U.S. alitretinoin

Basilea Pharmaceutica Ltd. • Grenzacherstrasse 487 • PO Box • 4005 Basel • Switzerland • www.basilea.com

opportunity through a milestone payment related to the launch of the product and royalties on future U.S. sales.

Ronald Scott, Basilea's CEO stated: "Basilea is committed to fight the growing global threat of resistance against established therapies. We continue to focus on achieving our key milestones for this year including a potential approval of our antibiotic ceftobiprole in Europe, reporting phase 3 results for our antifungal isavuconazole and continued improvement of our financial performance. We remain open to explore innovative collaboration and transaction structures and we were very pleased to announce a significant agreement with BARDA allowing for non-dilutive funding of up to USD 89 million for our drug candidate BAL30072, which addresses Gram-negative bacterial resistance."

Key figures

(In CHF million, except per share data)	**H1 2013**	**H1 2012**
Product sales	-	17.7
Contract revenue	20.4	13.4
Revenue from R&D services	0.1	0.1
Other income	0.1	0.0
Total operating income	20.6	31.2
Cost of sales	-	(5.1)
Research & development expenses	(26.7)	(31.6)
General & administrative/Selling, general & administrative expenses	(11.3)	(28.7)
Total operating expenses	(38.0)	(65.5)
Operating loss	(17.4)	(34.2)
Net loss	(17.3)	(34.6)
Net cash used for operating activities	(33.7)	(47.6)
Cash and short-term investments	262.8	149.0
Basic and diluted loss per share, in CHF	(1.80)	(3.61)

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 2013 can be found on the company's website at http://interimreport.basilea.com.

Financial summary

In the first six months of the financial year 2013, contract revenues increased to CHF 20.4 million (H1 2012: CHF 13.4 million), mainly driven by the revenue recognition associated with the agreement for Toctino® concluded with Stiefel in July 2012. Following the transaction, Toctino® sales were recorded by Stiefel, leading to a decrease in total operating income for the first six months of 2013 to CHF 20.6 million (H1 2012: CHF 31.2 million).

Research and development expenses were CHF 26.7 million in the first six months of 2013, compared to CHF 31.6 million in the first six months of 2012. This decrease is mainly due to Basilea's fulfillment of its commitment to contribute to isavuconazole development costs in the first half of 2012.

As a consequence of the transfer of the Toctino® business to Stiefel, selling, general and administrative expenses were significantly reduced from CHF 28.7 million in the first half of 2012 to CHF 11.3 million in the first half of 2013. Total operating expenses in the first six months of 2013 were CHF 38 million compared to CHF 65.5 million of the corresponding 2012 period.

As a result of these factors, and driven by Basilea's prudent financial management, operating loss in the first six months of 2013 was reduced by approximately 50 percent to CHF 17.4 million (H1 2012: CHF 34.2 million) and the net loss of the period decreased to CHF 17.3 million, compared to CHF 34.6 million in the corresponding period in 2012. The basic and diluted loss per share was reduced to CHF 1.80 for the first six months of 2013 compared to CHF 3.61 for the respective 2012 period. In the first six months of 2013, net cash used for operating activities was CHF 33.7 million as compared to CHF 47.6 million in the first six months of 2012.

At the Ordinary General Meeting of Shareholders in April, Basilea's shareholders approved the distribution of CHF 5.00 per share, corresponding to CHF 48.0 million, from capital contribution reserves. The payment was made in June. As of June 30, 2013, Basilea's combined cash and short-term investments were CHF 262.8 million, compared to CHF 149.0 million as of June 30, 2012, and to CHF 344.0 million (including the upfront payment of CHF 224.1 million from Stiefel following the Toctino® agreement) as of December 31, 2012.

Financial outlook

Total average operating expenses for 2013 are estimated to decrease to approximately CHF 7 million per month primarily due to the agreement with BARDA and continued prudent expense management. Basilea's average operating loss in 2013 is estimated to improve to approximately CHF 4 million per month.

Portfolio

Isavuconazole – *an investigational intravenous and oral broad-spectrum antifungal, partnered with Astellas Pharma Inc., for the potential treatment of severe invasive and life-threatening fungal infections (phase 3 clinical development)*

Isavuconazole 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 *in-vitro* activity against emerging and often fatal molds including those that cause mucormycosis. In clinical studies isavuconazole achieved predictable drug levels in patients, supporting reliable once-daily dosing and a switch from intravenous to oral administration. Isavuconazole received U.S. FDA fast-track and U.S. orphan drug designation.

Ceftobiprole – *an investigational broad-spectrum intravenous antibiotic for the potential first-line empiric treatment of severe bacterial infections (under regulatory review in Europe for the potential treatment of pneumonia in the hospital)*

Ceftobiprole has demonstrated broad-spectrum activity against Gram-positive bacteria including methicillin-resistant and vancomycin-resistant *Staphylococcus aureus* (MRSA, VRSA) and penicillin-resistant *Streptococcus pneumoniae* (PRSP) as well as Gram-negative pathogens, including Enterobacteriaceae and *Pseudomonas aeruginosa*.

BAL30072 – *an intravenous bactericidal sulfactam antibiotic against multidrug-resistant Gram-negative bacterial infections (phase 1 clinical development)*

BAL30072 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 bacteria that produce antibiotic-inactivating enzymes including carbapenemases and metallo-beta-lactamases such as the New Delhi metallo-beta-lactamase 1 (NDM-1). In addition, BAL30072 has shown additive or synergistic

activity with antibiotics from the carbapenem class. Basilea entered a contract with BARDA for up to USD 89 million in funding for the development of BAL30072.

BAL101553 – *an intravenous and oral small-molecule anti-cancer drug with a dual mode of action (phase 1 clinical development)*

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 not responsive to conventional microtubule-targeting agents, such as taxanes, as a result of diverse resistance mechanisms. 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 corticosteroids*

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 Wednesday, August 21, 2013, 4 p.m. (CEST), 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 Friday, August 23, 2013, 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 17234 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, 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.

For further information, please contact:

Media Relations	Investor Relations
Peer Nils Schröder, PhD Head Public Relations & Corporate Communications +41 61 606 1102 media_relations@basilea.com	Barbara Zink, PhD, MBA Head Corporate Development +41 61 606 1233 investor_relations@basilea.com

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