PRESS RELEASE

Basilea reports 2015 half-year results – Major milestones achieved for CRESEMBA® and Zevtera®

- **Antifungal CRESEMBA® (isavuconazole) received U.S. approval and is recommended for approval in the European Union**
- **Antibiotic Zevtera®/Mabelio® (ceftobiprole medocaril) launched in four European countries**
- **Ceftobiprole designated as a Qualified Infectious Disease Product for the treatment of lung and skin infections by U.S. FDA**
- **Additional oncology program (panRAF kinase inhibitor BAL3833) entered phase 1 of clinical development**
- **Cash and short-term investments of CHF 218.4 million**

Basel, Switzerland, August 14, 2015 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its financial results for the first half of the financial year 2015 with a solid half-year cash position of CHF 218.4 million. Basilea achieved significant milestones in the first part of 2015. The antifungal isavuconazole received U.S. approval and was launched under the trade name of CRESEMBA® in the United States by Basilea's licensing partner Astellas Pharma Inc. for the treatment of the invasive mold infections aspergillosis and mucormycosis in adults. Furthermore, the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) recommended isavuconazole for regulatory approval as a new treatment for the invasive mold infections aspergillosis and mucormycosis. A final decision from the European Commission is expected in the coming months. Basilea has launched its antibiotic Zevtera® (ceftobiprole medocaril)* in four European markets, France, Italy, the United Kingdom and Germany. A possible launch in Spain is planned by end-2015 or the beginning of 2016. To fully access the commercial value of Zevtera®, Basilea is in discussions with potential partners for further territories, including the U.S. The U.S. FDA designated ceftobiprole as a Qualified Infectious Disease Product (QIDP) for the potential treatment of community-acquired bacterial pneumonia and acute bacterial skin and skin structure infections.

Ronald Scott, Basilea's CEO, stated: "We continue to grow the company from a solid base as we focus on our major value drivers. We are now commercializing Zevtera in a number of major European countries. At the same time, we are actively preparing for a potential launch of isavuconazole in Europe. There is high commercial synergy between these two hospital anti-infectives." He added: "Basilea is committed to bring innovative therapies to patients and we look forward to the European Commission's decision regarding a potential approval of isavuconazole in the fourth quarter of 2015."

David Veitch, Chief Commercial Officer of Basilea, commented: "Invasive mold infections represent the most important part of the market in terms of medical need and value. Isavuconazole is clearly positioned in this segment. We are in preparations for a potential launch of isavuconazole in Europe early next year." He added: "We are also currently focused on the market entry of our antibiotic Zevtera. Following the roll-out of the country launches, Zevtera

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sales are expected to accumulate steadily over time as hospitals gain experience with Zevtera and it becomes a routine treatment. Treatment failure rates in pneumonia caused by MRSA are still high and have been attributed in part to inadequate initial antibiotic therapy. Pneumonia patients require effective, fast-acting and well-tolerated treatment, and appropriate initial therapy is very important to decrease mortality, morbidity and length of hospital stay. Here Zevtera addresses an important unmet medical need by offering a new first line antibiotic treatment option."

Prof. Achim Kaufhold, Chief Medical Officer of Basilea, said: "In addition to the important achievements in our anti-infectives portfolio, we have strengthened our oncology portfolio during the first half of the year. The panRAF kinase inhibitor program entered phase 1 clinical development. Substances from this series have shown promising activity in preclinical models of resistant tumors. Furthermore, for the tumor checkpoint controller BAL101553 we are looking forward to the read-out of a phase 2a study in advanced solid tumor patients in the second half of the year."

Key figures

(In CHF million, except per share data)	**H1 2015**	**H1 2014**
Contract revenue	24.4	20.2
Revenue from research & development services	0.4	0.1
Other revenue	0.2	0.0
Total revenue	25.0	20.3
Research & development expenses, net	(31.2)	(27.5)
Selling, general & administrative expenses	(23.8)	(12.3)
Total operating expenses	(55.0)	(39.8)
Operating loss	(30.0)	(19.5)
Net loss	(30.1)	(19.4)
Net cash (used for) operating activities	(19.3)	(44.9)
Cash and short-term investments	218.4	245.9
Basic and diluted loss per share, in CHF	(3.0)	(1.97)

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 2015 can be found on the company's website at http://interimreport.basilea.com.

Financial summary

Contract revenue in the first half of 2015 amounted to CHF 24.4 million (H1 2014: CHF 20.2 million), including CHF 18.8 million (H1 2014: CHF 18.5 million) related to the global agreement with Stiefel for Toctino® and CHF 5.6 million (H1 2014: CHF 1.7 million) related to the license agreement with Astellas for isavuconazole. Total revenues in H1 2015 amounted to CHF 25.0 million (H1 2014: CHF 20.3 million).

Research and development net expenses amounted to CHF 31.2 million in H1 2015 (H1 2014: CHF 27.5 million) and were mainly related to activities to support the launch preparation for isavuconazole in Europe, for maintaining the supply chain for isavuconazole, progressing the

phase 2a development of BAL101553 and strengthening the oncology portfolio by in-licensing panRAF kinase inhibitors as well as to supporting exploratory research programs. The Company recognized CHF 3.6 million in H1 2015 (H1 2014: CHF 3.3 million) under the agreement with BARDA related to the reimbursement of agreed development costs for BAL30072.

Selling, general and administrative expenses increased to CHF 23.8 million (H1 2014: CHF 12.3 million), mainly related to the commercialization of ceftobiprole in Germany, France, Italy and the UK and activities to prepare and support a potential launch of isavuconazole in major European countries.

In H1 2015, operating loss amounted to CHF 30.0 million, compared to CHF 19.5 million in H1 2014. This change is mainly due to higher operating expenses related to commercial activities for ceftobiprole and pre-launch activities for isavuconazole. The net loss amounted to CHF 30.1 million (H1 2014: CHF 19.4 million) and the basic and diluted loss per share to CHF 3.00 (H1 2014: CHF 1.97).

The net cash used for operating activities in H1 2015 amounted to CHF 19.3 million as compared to CHF 44.9 million in 2014. The decrease is mainly due to the milestone payment of CHF 30.0 million received from Astellas in H1 2015 upon approval of isavuconazole in the U.S.

Combined cash and short-term investments amounted to CHF 218.4 million as of June 30, 2015, compared to CHF 226.1 million as of December 31, 2014.

Financial outlook

Basilea remains focused on establishing Zevtera® in hospitals in Germany, France, Italy and the UK and launching the drug in Spain end-2015 or beginning of 2016. The company is also preparing for the potential launch of isavuconazole in Europe. Total operating expenses for 2015 are estimated at approximately CHF 9 million on average per month. Basilea's operating loss in 2015 is estimated at approximately CHF 4-5 million on average per month.

Pipeline update

CRESEMBA® (isavuconazole) – *an intravenous and oral broad-spectrum azole antifungal for the treatment of severe invasive fungal infections — approved in the U.S. and recommended for regulatory approval in the European Union for invasive mold infections*

Isavuconazole is being co-developed with Astellas Pharma Inc. under an agreement granting Astellas license rights in the U.S.; Basilea holds full rights to isavuconazole in markets outside the United States. Astellas launched CRESEMBA® in the United States in April 2015 following FDA's regulatory approval for the treatment of invasive aspergillosis and invasive mucormycosis in adults. Basilea is entitled to receive royalties and sales milestones on Astellas' U.S. sales of isavuconazole. The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) recommended isavuconazole for regulatory approval as a new treatment for invasive aspergillosis and mucormycosis in the European Union.

The isavuconazole phase 3 ACTIVE study in invasive *Candida* infections did not meet its primary endpoint. The key secondary endpoint of the study, overall response rate at two weeks after treatment, was, however, comparable between the two treatment groups. In addition, the secondary endpoint of all-cause mortality was comparable at study day 14 and day 56 in both treatment groups. Additional analyses of the data will be performed to assess the potential role of isavuconazole in invasive *Candida* infections.

Zevtera®/Mabelio® (ceftobiprole medocaril) – *a broad-spectrum intravenous cephalosporin antibiotic — marketed in European countries*

Zevtera®/Mabelio® (European trade names of ceftobiprole, depending on the country) is a new generation broad-spectrum cephalosporin antibiotic with bactericidal activity against Gram-

positive and Gram-negative bacteria associated with pneumonia, including methicillin-resistant *Staphylococcus aureus* (MRSA) and susceptible *Pseudomonas* spp.

Ceftobiprole has received national licenses for the treatment of adult patients with community- and hospital-acquired pneumonia (CAP, HAP), excluding ventilator-associated pneumonia (VAP) in 13 European countries* and has been launched in Germany, France, Italy and the United Kingdom. Ceftobiprole is not registered in the United States.

The FDA designated ceftobiprole in August as a Qualified Infectious Disease Product (QIDP) for the potential treatment of community-acquired bacterial pneumonia and acute bacterial skin and skin structure infections. The QIDP status provides certain incentives for the development of antibiotics, such as priority review if the product is submitted for approval in the U.S., and a five-year extension of certain periods of market exclusivity that may be applicable should it be approved.

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

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, will complete at the end of the initial funding period, as agreed between BARDA and Basilea. Under the contract, a phase 1 study was conducted with intravenous (i.v.) BAL30072 in combination with the carbapenem antibiotic meropenem. Transiently elevated liver enzyme levels were observed as dose-limiting factor during phase 1 development. Basilea is discussing further development options with potential partners and collaborators including additional antibiotic combinations and new dosage forms such as inhaled forms of BAL30072.

BAL101553 – *an anti-cancer drug candidate acting as tumor checkpoint controller — in phase 2a (i.v.) and phase 1 (oral)*

BAL101553 is a microtubule-destabilizing small molecule, acting as tumor checkpoint controller as it promotes tumor cell death through activation of a checkpoint in cell proliferation.

BAL101553 is currently in phase 2a for the i.v. and in phase 1 for the oral dosage form, both in patients with solid cancers. The availability of an oral formulation of BAL101553 offers increased flexibility with regard to both single agent and drug combination treatment strategies across different tumor types.

BAL3833 – *an oral anti-cancer drug candidate (panRAF kinase inhibitor)* targeting *tumor growth and therapeutic resistance — in phase 1*

BAL3833 is the lead compound of a novel class of panRAF kinase inhibitors that are active in tumors which have developed resistance to currently available RAF kinase inhibitors and have the potential to offer new treatment options for melanoma and other cancers. Basilea is developing BAL3833 under a license agreement with a consortium of organizations including The Institute of Cancer Research, London, Cancer Research Technology, the Wellcome Trust and The University of Manchester. A phase 1 in advanced solid tumor patients was initiated in May.

Toctino® (oral alitretinoin) – *a dermatology drug for systemic use in adults with severe chronic hand eczema unresponsive to potent topical corticosteroids — approved and marketed in certain countries; 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. New Drug Application (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 Friday, August 14, 2015, 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 Monday, August 17, 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 19396 followed by the # sign.

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 discover, 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.

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

References

* European trade name Zevtera® or Mabelio®, depending on the country. The drug has received national licenses in 13 European countries for the treatment of adult patients with community- and hospital-acquired pneumonia (CAP, HAP), excluding ventilator-associated pneumonia (VAP): Austria, Belgium, Denmark, Finland, France, Germany, Italy, Luxembourg, Norway, Spain, Sweden, Switzerland and the United Kingdom. Ceftobiprole is not registered in the United States.