PRESS RELEASE

Basilea reports 2016 half-year results – CRESEMBA® launched in key European markets

- **Antifungal CRESEMBA® (isavuconazole) launched in first European markets**
- **Entered contract with BARDA for the development and potential US registration of the antibiotic ceftobiprole (European trade name Zevtera® or Mabelio®)**
- **Half-year cash and financial investments of CHF 311 million**

Basel, Switzerland, August 15, 2016 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its financial results for the first half of financial year 2016 with a solid half-year cash position of CHF 310.9 million and a net loss of CHF 27.9 million.

Basilea's CEO Ronald Scott stated: "We are proud to have launched CRESEMBA, our antifungal for the treatment of invasive mold infections, in the first European markets. Product sales for CRESEMBA and Zevtera were in line with our guidance in the first-half of this year. We also entered into a new agreement with BARDA that allows us to proceed with the clinical development required for the potential registration of ceftobiprole in the commercially important US market in indications of high unmet medical need such as bacteremia. We intend to expand our oral clinical study for our most advanced cancer compound, the tumor checkpoint controller BAL101553, to include glioblastoma patients later this year."

Anti-infectives: CRESEMBA® and Zevtera®/Mabelio®

Basilea currently commercializes CRESEMBA® (isavuconazole) in Germany, Italy, the United Kingdom (UK) and Austria and receives royalties on product sales in the United States (US) where the drug is marketed by Basilea's license partner Astellas Pharma US.

Zevtera®/Mabelio® (ceftobiprole), Basilea's broad-spectrum antibiotic, is currently available in, Germany, Italy, the UK, France, Austria and Switzerland. In Europe, CRESEMBA® and Zevtera®/Mabelio® are marketed by a single dedicated sales force to leverage the commercial synergies due to the overlapping hospital prescriber base.

Basilea aims to bring ceftobiprole to the US market and in April 2016 signed a contract with the United States Biomedical Advanced Research and Development Authority (BARDA)[1] for a clinical phase 3 program to potentially gain regulatory approval in the US. Under the terms of the contract, BARDA will provide initial funding of approximately USD 20 million. Upon successful completion of pre-defined milestones, the total value of the contract could reach USD 100 million over a period of 4.5 years.

As part of the ceftobiprole program agreed with BARDA, Basilea intends to initially conduct phase 3 studies in acute bacterial skin and skin structure infections (ABSSSI) and *Staphylococcus aureus* bacteremia (SAB). The company is in discussions with the United States Food and Drug Administration (FDA) on the studies with the goal to gain Special Protocol Assessments (SPAs) to support both indications.

After completing a full data review, Basilea decided not to further develop the pre-clinical, inhaled antibiotic BAL30072.

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Oncology: BAL101553 and BAL3833

Overcoming tumor resistance is the second pillar on which Basilea is building its portfolio. Significant progress was made in the first half of 2016 in the development of our anti-cancer drug candidates, the oral/intravenous (i.v.) small-molecule tumor checkpoint controller BAL101553 and the oral panRAF/SRC kinase inhibitor BAL3833.

Data from the completed open-label phase 1/2a study with i.v. BAL101553 were presented at the American Society of Clinical Oncology (ASCO) annual meeting. Overall, the drug candidate was well tolerated at the recommended phase 2 dose for a 2-hour weekly i.v. administration. Signals of clinical activity were observed.

Basilea is currently exploring BAL101553 daily oral dosing in a phase 1/2a study in adult patients with advanced solid tumor cancers. In addition, based on the ability of BAL101553 to penetrate brain tissue and preclinical evidence of its activity in brain cancer, Basilea intends to extend the oral study to adult patients with glioblastoma by year-end. Glioblastoma is the most common and aggressive primary brain tumor, and is often associated with poor prognosis for patients.

Progress was also made in the dose-escalation phase 1 study with BAL3833, which is developed as a potential new treatment option for resistant melanoma and other cancers.

Second half 2016 focus

Basilea's CEO Ronald Scott commented: "In the second half of 2016, we will continue to increase market access and gain formulary adoption for CRESEMBA and Zevtera in key European countries. In addition, we plan to conclude further license and distribution agreements for both drugs to fully leverage their commercial potential in additional territories. We are in active discussion with the FDA regarding the ceftobiprole SPAs and aim in the first half of 2017 to initiate the ceftobiprole phase 3 studies necessary for a potential regulatory approval in the US. We are also focused on further advancing our oncology drug candidates in clinical development."

Key figures

(In CHF million, except per share data)	**H1 2016**	**H1 2015**
Product revenue	1.9	-
Contract revenue	27.8	24.4
Revenue from R&D services	0.0	0.4
Other revenue	0.0	0.2
Total operating income	**29.7**	**25.0**
Costs of products sold	(3.0)	-
Research & development expenses, net	(24.8)	(31.2)
Selling, general & administration expenses	(26.8)	(23.8)
Total operating expenses	**(54.6)**	**(55.0)**
Operating loss	**(24.8)**	**(30.0)**
Net loss	**(27.9)**	**(30.1)**
Net cash (used for) operating activities	(53.7)	(19.3)
Cash and financial investments	310.9	218.4
Basic and diluted loss per share, in CHF	(2.76)	(3.00)

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 2016 can be found on the company's website at http://interimreport.basilea.com.

Financial summary

In H1 2016 product revenue amounted to CHF 1.9 million (H1 2015: CHF 0 million). Contract revenue in the first half-year of 2016 amounted to CHF 27.8 million (H1 2015: CHF 24.4 million), including CHF 18.8 million (H1 2015: CHF 18.8 million) related to the global agreement for Toctino® and CHF 9.1 million (H1 2015: CHF 5.6 million) related to the license agreement with Astellas for isavuconazole. Total operating income in H1 2016 including sales amounted to CHF 29.7 million (H1 2015: CHF 25.0 million).

Research and development net expenses amounted to CHF 24.8 million (H1 2015: CHF 31.2 million) and were mainly related to activities for the phase 1/2a development of oncology drug candidates BAL101533 and BAL3833, costs for running a pediatric program for ceftobiprole, preparing a pediatric program for isavuconazole and the preparation of the ceftobiprole phase 3 US program.

Selling, general and administration expenses amounted to CHF 26.8 million (H1 2015: CHF 23.8 million), and were mainly related to commercial activities for the launch and commercialization of isavuconazole under the trade name CRESEMBA® in Germany, Italy, the UK Austria, and the commercialization of ceftobiprole under the trade name Zevtera®/Mabelio® in Germany, Italy, the UK, France, Austria and Switzerland.

In H1 2016, operating loss amounted to CHF 24.8 million, compared to CHF 30.0 million in H1 2015. The net loss amounted to CHF 27.9 million (H1 2015: CHF 30.1 million) and the basic and diluted loss per share to CHF 2.76 (H1 2015: CHF 3.00).

The net cash used for operating activities in H1 2016 amounted to CHF 53.7 million as compared to CHF 19.3 million in H1 2015. The increase is mainly due to the milestone payment of CHF 30.0 million received from Astellas in H1 2015 upon approval of isavuconazole in the US.

Combined cash and financial investments amounted to CHF 310.9 million as of June 30, 2016, compared to CHF 364.7 million as of December 31, 2015.

Financial outlook

Basilea continues to focus on launching and establishing CRESEMBA® in Germany, Italy, the UK and France in 2016 and on commercializing Zevtera®/Mabelio® in hospitals in major European countries. Basilea confirms guidance for total operating expenses for 2016 of approximately CHF 9 - 10 million on average per month and average operating loss in 2016 of approximately CHF 4 - 5 million per month. The company maintains its total annual product sales guidance of approximately CHF 5 million in 2016.

Portfolio status

CRESEMBA® (isavuconazole) – *an i.v. and oral azole antifungal for the treatment of invasive mold infections*

Isavuconazole is an i.v. and oral azole antifungal and the active agent of the prodrug isavuconazonium sulfate. Isavuconazole was approved in March 2015 by the United States Food and Drug Administration (FDA) for patients 18 years of age and older in the treatment of invasive aspergillosis and invasive mucormycosis. The European Commission granted marketing authorization in October 2015 to isavuconazole for the treatment of adult patients with invasive aspergillosis and for the treatment of adult patients with mucormycosis for whom amphotericin B is inappropriate.[2] The European marketing authorization is valid in all 28 European Union (EU) member states, as well as in Iceland, Liechtenstein and Norway. Isavuconazole has orphan drug designation for the approved indications in Europe and the US. Basilea commercializes isavuconazole as CRESEMBA® in Germany, Italy, the UK and Austria. The drug is commercialized in the US by Basilea's licensee Astellas Pharma US. Outside the US and the EU, isavuconazole is not approved for commercial use.

Zevtera®/Mabelio® (ceftobiprole) – *a broad-spectrum antibiotic from the cephalosporin class for i.v. administration with bactericidal activity against certain Gram-positive and Gram-negative bacteria, including methicillin-resistant Staphylococcus aureus (MRSA) and susceptible Pseudomonas spp.*

Ceftobiprole (European trade name Zevtera® or Mabelio®, depending on the country) is approved for sale in 13 European countries and several non-European countries for the treatment of adult patients with community-acquired pneumonia and hospital-acquired pneumonia (excluding ventilator-associated pneumonia).[3] The drug is currently available in Germany, Italy, the UK, France, Austria and Switzerland. In October 2015 Basilea signed an exclusive distribution and supply agreement with Hikma Pharmaceuticals LLC for Zevtera® for the Middle East and North Africa (MENA) region. Ceftobiprole is not approved in the United States. It received Qualified Infectious Disease Product (QIDP) designation from the US FDA for the potential treatment of community-acquired bacterial pneumonia (CABP) and acute bacterial skin and skin structure infections (ABSSSI). In April 2016, Basilea signed an agreement with the US Biomedical Advanced Research and Development Authority (BARDA) for initial funding of approximately USD 20 million for the phase 3 development of ceftobiprole with the goal to gain regulatory approval in the United States. The total contract value could reach USD 100 million over a period of 4.5 years upon successful completion of pre-defined milestones.[1]

BAL101553 – *a tumor checkpoint controller in phase 1/2a clinical testing in patients with advanced solid tumors*

The small molecule oncology drug candidate BAL101553 (prodrug of BAL27862) is being developed as a potential therapy for diverse cancers, including tumor types unresponsive to standard therapeutics. The drug is currently undergoing clinical phase 1/2a evaluation in patients with advanced solid tumors as a once-daily oral dosage form. It has shown evidence of clinical anti-tumor activity in a phase 1/2a study with weekly 2-hour i.v. infusion, during which the maximum tolerated dose and the recommended phase 2 dose for this administration regimen was established.

BAL3833 – *a phase 1 oral anti-cancer drug candidate (panRAF/SRC kinase inhibitor) targeting tumor growth and therapeutic resistance*

BAL3833 (also known as CCT3833) is an orally available small-molecule panRAF/SRC kinase inhibitor targeting cell proliferation signaling pathways that are associated with tumor growth and resistance development to current therapies. It is the lead compound of a series of kinase inhibitors in-licensed by Basilea in April 2015 under an agreement with The Institute of Cancer Research, London, Cancer Research Technology, the Wellcome Trust, and The University of Manchester. BAL3833 is currently being investigated in a phase 1 study in adult patients with advanced solid tumors including metastatic melanoma. The compound originates from research at The Institute of Cancer Research and the Cancer Research UK Manchester Institute, by scientists funded by Cancer Research UK and the Wellcome Trust.

Conference call

Basilea Pharmaceutica Ltd. invites you to participate in a conference call on Monday, August 15, 2016, 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 Wednesday, August 17, 2016, 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 15275 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 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

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

References

1 Contract No. HSO100201600002C, Biomedical Advanced Research and Development Authority (BARDA), a division within the US Department of Health and Human Services' Office of the Assistant Secretary for Preparedness and Response

2 European Public Assessment Report CRESEMBA® http://www.ema.europa.eu [Accessed: August 11, 2016]

3 UK Summary of Product Characteristics (SPC): http://www.mhra.gov.uk/ [Accessed: August 11, 2016]