PRESS RELEASE

Basilea reports solid 2016 results and expects to double product sales in 2017

- **Cresemba® launched by Basilea in first European countries and marketed together with Zevtera®/Mabelio®**
- **Entered into distribution and license agreements covering more than 40 countries for both Cresemba and Zevtera/Mabelio**
- **Product sales of CHF 7.1 million in Europe and CHF 7.3 million royalties received on 2016 Cresemba US sales**
- **CHF 289 million cash and financial investments at year-end**
- **Initiation of ceftobiprole clinical phase 3 development program under BARDA agreement anticipated mid-2017 to support potential future US regulatory filing**

Basel, Switzerland, February 20, 2017 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced its financial results for 2016 today with product sales from Cresemba® (isavuconazole) and Zevtera®/Mabelio® (ceftobiprole) of CHF 7.1 million in Europe, royalties on US Cresemba sales of CHF 7.3 million, total revenue of CHF 66.0 million, a year-end cash and financial investment position of CHF 289.0 million and a reduced operating loss of CHF 43.9 million.

Basilea's CEO Ronald Scott said: "We've launched Cresemba addressing severe fungal infections in the first European markets and made substantial progress in the commercialization of both Cresemba and our antibiotic Zevtera/Mabelio. We are pleased to announce that we achieved sales for the full year of CHF 7.1 million in Europe. The Cresemba launch is also going well in the USA, where our license partner Astellas Pharma US reported 2016 sales of USD 46 million, on which we received CHF 7.3 million in royalties."

He continued: "We plan to initiate a ceftobiprole clinical phase 3 development program under our agreement with BARDA in mid-2017 to support a potential future US regulatory filing and we continue to make good progress on our oncology development programs addressing tumor resistance. We were able to expand our BAL101553 oral phase 1/2a study to include brain cancer patients according to plan."

Anti-infectives: Cresemba and Zevtera/Mabelio marketed by Basilea in first European countries with partnerships in place for important additional markets

Basilea is marketing Cresemba and Zevtera/Mabelio in Germany, Italy, the UK, France and Austria; Zevtera is also marketed in Switzerland. Basilea's licensing partner Astellas Pharma US markets Cresemba in the United States. In 2016, Basilea entered into distribution agreements for isavuconazole and ceftobiprole with Grupo Biotoscana S.L. in nineteen Latin American countries and with Unimedic Pharma AB for the Nordics. The distribution agreement with Hikma Pharmaceuticals LLC for the Middle East and North Africa (MENA) region was extended to include isavuconazole in addition to ceftobiprole. In addition, Basilea concluded a license agreement with Asahi Kasei Pharma Corporation for the development and commercialization of isavuconazole in Japan. Basilea's existing partnerships cover more than forty countries around the world in addition to the countries that Basilea is directly serving.

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The latest guideline issued by the European Conference on Infections in Leukaemia (ECIL) recommends Cresemba for the first-line treatment of invasive aspergillosis in leukemia and hematopoietic stem cell transplant patients. The guideline states that isavuconazole is as effective as voriconazole with a better safety profile.[1] This recommendation in one of the most relevant treatment guidelines in Europe underscores the potentially important clinical role of Cresemba in the treatment of patients with these life-threatening infections.

Contract with BARDA to support ceftobiprole phase 3 development for US market

Basilea entered into a contract in 2016 with the Biomedical Advanced Research and Development Authority (BARDA) for the clinical phase 3 development of ceftobiprole to support a potential regulatory filing in the US, the largest market value-wise for branded hospital antibiotics. BARDA provides initial funding of approximately USD 20 million for the preparation of the phase 3 program. The total value of the BARDA contract could reach USD 100 million over a period of 4.5 years if pre-defined milestones are met. Basilea submitted clinical study protocols to the US Food and Drug Administration (FDA) for two phase 3 studies, one in *Staphylococcus aureus* bacteremia (SAB) and one in acute bacterial skin and skin structure infections (ABSSSI). Basilea will initiate the phase 3 clinical development program once it completes the FDA Special Protocol Assessment (SPA) process.

Two oncology drug candidates in clinical development: tumor checkpoint controller BAL101553 and panRAF/SRC kinase inhibitor BAL3833

In 2016 Basilea further strengthened its oncology pipeline, the second pillar of its hospital-focused strategy, by broadening BAL101553's clinical development program. A separate study arm for patients with glioblastoma was added to the ongoing phase 1/2a clinical study with oral BAL101553, based data in preclinical glioblastoma tumor models demonstrating activity of the drug candidate in this often lethal brain cancer. Potential patient-selection biomarkers have also been identified and will be assessed in BAL101553-treated glioblastoma patients. In addition, a further phase 1/2a clinical study was initiated to explore continuous intravenous infusion.

Dose-escalation in the phase 1 study with orally administered BAL3833 in patients with solid tumor cancers, including metastatic melanoma, is continuing with the aim to determine the maximum tolerated dose. Preclinical data on BAL3833 presented at the American Association for Cancer Research (AACR) annual meeting showed that the drug candidate has anti-cancer activity in KRAS-driven *in vitro* and *in vivo* tumor models via inhibition of the RAF and SRC family kinases. This indicates that BAL3833 may also be effective in non-melanoma KRAS-mutant cancers such as pancreatic, colorectal and non-small-cell lung cancer, potentially providing a new therapeutic option in these indications.

Focus 2017 on growing product sales and progress in pipeline

Basilea's CEO Ronald Scott stated: "In 2017, we expect to further grow our product sales as we continue to execute on our commercialization and partnering strategy. We anticipate seeing initial contributions from our current distributors as their first marketing authorizations are granted. We are also working towards further agreements with potential partners to cover remaining commercially relevant markets including Asia Pacific, Russia/CIS, and certain European countries. In addition, we anticipate that Swissmedic will complete its review of our isavuconazole marketing authorization application in 2017."

He added: "An important goal for us this year is to finalize the Special Protocol Assessment process with the US FDA in order to start the clinical phase 3 program for ceftobiprole under our BARDA contract. Our initial focus will be on skin and bloodstream infections, two areas of high medical need."

Ceftobiprole will have a total of ten years of market exclusivity in the US from potential approval based on its Qualified Infectious Disease Product designation granted by the FDA. Upon

successful completion of the studies, the phase 3 data could be used to support supplemental marketing authorization applications for ceftobiprole in Europe and other territories, potentially resulting in label extensions for ceftobiprole.

In 2017, Basilea will further advance the clinical development of its oncology drug candidates and expects to complete dose-escalation in BAL101553's phase 1/2a studies and BAL 3833's phase 1 study.

Key figures

(In CHF million, except per share data)	**2016**	**2015**
Product revenue	7.1	-
Contract revenue	57.7	51.2
Revenue from R&D services	0.2	0.5
Other revenue	0.9	1.2
Total operating income	**66.0**	**52.8**
Costs of products sold	(5.3)	-
Research & development expenses, net	(48.4)	(60.1)
Selling, general & administration expenses	(56.1)	(54.2)
Total operating expenses	**(109.9)**	**(114.3)**
Operating loss	**(43.9)**	**(61.5)**
Net loss	**(51.3)**	**(61.6)**
Net cash (used in) operating activities	(75.0)	(67.8)
Cash and financial investments	289.0	364.7
Basic and diluted loss per share, in CHF	(5.07)	(6.09)

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

The consolidated financial statements of Basilea Pharmaceutica Ltd. for the financial year 2016 can be found on the company's website at http://annualreport.basilea.com.

Financial summary

Full-year product revenue 2016 amounted to CHF 7.1 million (2015: none). Contract revenue 2016 amounted to CHF 57.7 million (2015: CHF 51.2 million), including CHF 37.7 million (2015: CHF 37.6 million) related to the global agreement for Toctino® and CHF 19.3 million (2015: CHF 13.6 million) related to the license agreement with Astellas for isavuconazole. Total operating income in 2016 including sales amounted to CHF 66.0 million (2015: CHF 52.8 million).

Research and development net expenses in 2016 amounted to CHF 48.4 million (2015: CHF 60.1 million) and were mainly related to activities for the phase 1/2a development of oncology drug candidate BAL101533, phase 1 clinical development of oncology drug candidate BAL3833, costs for the pediatric program for ceftobiprole and activities related to isavuconazole. The decrease of CHF 11.7 million as compared to 2015 is mainly due to 2015 isavuconazole pre-launch activities.

Selling, general and administration expenses in 2016 amounted to CHF 56.1 million (2015: CHF 54.2 million), and included costs related to the commercialization of Cresemba and Zevtera/Mabelio and stock-based compensation of CHF 4.2 million (2015: CHF 4.6 million).

In 2016, the operating loss was reduced by 29% to CHF 43.9 million from CHF 61.5 million in 2015 and net loss 2016 was reduced to CHF 51.3 million (2015: CHF 61.6 million), resulting in a lower basic and diluted loss per share of CHF 5.07 (2015: CHF 6.09).

The net cash used for operating activities in 2016 amounted to CHF 75.0 million as compared to CHF 67.8 million in 2015. The increase in comparison to 2015 is mainly due to the milestone payment from Astellas in 2015 upon approval of isavuconazole in the US, which reduced the net cash used in the previous period.

Combined cash and financial investments amounted to CHF 289.0 million as of December 31, 2016, compared to CHF 364.7 million as of December 31, 2015.

Financial outlook

Basilea continues to focus on growing sales of its two marketed products while at the same time advancing its clinical development pipeline. Basilea anticipates total annual product sales of approximately CHF 15 million in 2017, a more than 100% increase over 2016, and a participation in US sales through royalties of approximately CHF 14 million. Total operating expenses after anticipated BARDA reimbursements for 2017 are estimated at approximately CHF 10 million on average per month with an operating loss of approximately CHF 3 million on average per month.

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. It is approved in the United States for patients 18 years of age and older in the treatment of invasive aspergillosis and invasive mucormycosis.[2] In Europe, isavuconazole received marketing authorization for the treatment of adult patients with invasive aspergillosis and for the treatment of adult patients with mucormycosis for whom amphotericin B is inappropriate.[3] 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 is marketing isavuconazole as Cresemba in Germany, Italy, the UK, France and Austria. In the United States Cresemba is marketed by Basilea's licensee Astellas Pharma US. Outside the US and the EU, isavuconazole is not approved for commercial use. The European Conference on Infections in Leukaemia (ECIL) recommends isavuconazole in its current guideline for the first-line treatment of invasive aspergillosis in leukemia and hematopoietic stem cell transplant patients.[1]

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 (CAP) and hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP).[4] Basilea is currently marketing the drug in Germany, Italy, the UK, France, Austria and Switzerland. Ceftobiprole 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). The drug is not approved in the United States. In 2016, Basilea entered into a contract with the Biomedical Advanced Research and Development Authority (BARDA) for the clinical phase 3 development of ceftobiprole to support a potential regulatory filing in the US. The total value of the BARDA contract could reach USD 100 million over a period of 4.5 years if

pre-defined milestones are met. Initial studies are planned in acute bacterial skin structure infections (ABSSSI) and *Staphylococcus aureus* bacteremia (SAB).

BAL101553 – *a tumor checkpoint controller in phase 1/2a clinical testing in patients with advanced solid tumors including recurrent or progressive glioblastoma*

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 (oral and continuous infusion) in patients with advanced solid tumors. In December 2016, the oral study was extended by adding a separate arm for patients with recurrent or progressive glioblastoma after prior radiotherapy. The drug candidate 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.[5]

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

BAL3833 (also known as CCT3833) is an orally administered 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, 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 the renowned UK cancer research institution, The Institute of Cancer Research, where it was developed 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, February 20, 2017, 4 p.m. (CET), 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, February 22, 2017, 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 19991 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. for the business year 2016 will take place on **Thursday, April 27, 2017 at 2 p.m. at the Radisson Blu 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 April 13, 2017 will be entitled to participate and exercise their voting rights.

About Basilea

Basilea Pharmaceutica Ltd. is a biopharmaceutical company developing products that address the medical problem of 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.

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 F. Tissot et al. ECIL-6 guidelines for the treatment of invasive candidiasis, aspergillosis and mucormycosis in leukemia and hematopoietic stem cell transplant patients. Haematologica 2016 (101), published online ahead of print; www.haematologica.org/content/early/2016/12/20/haematol.2016.152900 [Accessed February 17, 2017]

2 Cresemba US prescribing information [Accessed: February 17, 2017]

3 European Public Assessment Report (EPAR) Cresemba: http://www.ema.europa.eu [Accessed: February 17, 2017]

4 UK Summary of Product Characteristics (SPC) Zevtera®: http://www.mhra.gov.uk/ [Accessed: February 17, 2017]

5 J. Lopez et al. Phase 1/2a trial of intravenous BAL101553, a novel tumor checkpoint controller (TCC), in advanced solid tumors. American Society of Clinical Oncology (ASCO) annual meeting 2016, abstract 2525, poster board #225