PRESS RELEASE

Basilea reports significantly improved financial results in 2017

- **54% increase in total revenue, amounting to CHF 101.5 million**
- **Operating loss reduced by 68% to CHF 14.1 million**
- **Cash position as of year-end 2017 increased to CHF 310.7 million**
- **License and distribution agreements in 2017 for Cresemba® and Zevtera® cover more than 100 countries with CHF 80 million in upfront payments and up to USD 1.1 billion potential milestone payments**

Basel, Switzerland, February 27, 2018 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its financial results for the financial year 2017 with total revenue increased to CHF 101.5 million (2016: CHF 66.0 million; +54% year-on-year), including product sales from Cresemba® (isavuconazole) and Zevtera® (ceftobiprole) of CHF 16.3 million (2016: CHF 7.1 million; +130%) and royalties on U.S. and European Cresemba sales by Basilea's license partners of CHF 15.0 million (2016: CHF 7.3 million; +105%). Basilea reported CHF 310.7 million (year-end 2016: CHF 289.0 million; +7.5%) in cash and investments as of December 31, 2017 and a significantly reduced operating loss of CHF 14.1 million (2016: CHF 43.9 million; -68%).

Chief Executive Officer Ronald Scott said: "2017 marked a successful year in the implementation of our strategy to maximize the value of our commercial-stage drugs Cresemba and Zevtera through partnerships. At the same time our revenue from sales, royalties and revenue recognition increased to over CHF 100 million while our operating loss reduced by 68% to approximately CHF 14 million bringing Basilea closer to profitability. We received approximately CHF 80 million in upfront payments from our new partners in 2017. Our license and distribution agreements now cover more than 100 countries worldwide. In addition, potential regulatory and sales milestone payments under our licensing agreements amount up to USD 1.1 billion."

Anti-infectives: New partnerships create the basis for accelerated commercial success

During 2017, Basilea entered into major partnerships for its two marketed anti-infective drugs, the antifungal Cresemba (isavuconazole) and the antibiotic Zevtera (ceftobiprole). In June, Basilea announced a license agreement with Pfizer for Cresemba for more than 40 countries in Europe (excluding the Nordics) plus Russia, Turkey and Israel. The transaction completed in July, providing Basilea an upfront payment of CHF 70 million. This agreement was then extended to cover China and sixteen countries in the Asia Pacific region. Basilea receives mid-teen royalties on sales by Pfizer in these territories and is eligible for up to USD 650 million in regulatory and sales milestone payments.

Following the partnering of Cresemba with Pfizer, Basilea entered into a distribution agreement with Cardiome Pharma Corp. for Zevtera for Europe (excluding the Nordics) and Israel. Basilea also entered into a distribution agreement with Avir Pharma Inc. for isavuconazole and ceftobiprole for Canada in June and a license agreement with Shenzhen China Resources Gosun Pharmaceutical Co., Ltd. for ceftobiprole for China in September. Further distribution partnerships for both Cresemba and Zevtera are in place for Latin America with Grupo Biotoscana S.L., the Nordics with Unimedic Pharma AB and the Middle East and North Africa (MENA) region with Hikma Pharmaceuticals LLC.

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Cresemba sales by our partner Astellas Pharma Inc. showed continued strong growth in the second full year on the U.S. market. Astellas reported sales of USD 77 million for the calendar year 2017, which is a 67% increase year-on-year. The strong sales performance in the U.S. triggered a first sales milestone payment to Basilea of CHF 5 million in 2017.

In 2017, Pfizer launched Cresemba in Spain, and Unimedic launched Cresemba and Zevtera in the Nordics. Overall, Basilea expects product sales to grow significantly over the coming years based on both the growing demand in existing markets and contributions from new markets as the drugs gain regulatory approvals and are launched in additional countries.

In Japan, license partner Asahi Kasei Pharma Corporation is conducting an abbreviated clinical development program for isavuconazole to support a potential registration. Asahi Kasei Pharma successfully completed a phase 1 study in healthy volunteers and intends to start a phase 3 clinical study in the first half of 2018.

Working towards accessing the U.S. antibiotics market with increased BARDA funding for the development of ceftobiprole

The U.S. market is an estimated 70% of the global market for branded hospital antibiotics based on value[1] and therefore plays an important role in Basilea's strategy for ceftobiprole. In 2017 Basilea agreed on Special Protocol Assessments (SPAs) with the U.S. Food and Drug Administration (FDA) for two cross-supportive clinical phase 3 studies to support a potential regulatory filing in the U.S.: one in acute bacterial skin and skin structure infections (ABSSSI) and a second in *Staphylococcus aureus* bacteremia (SAB). In 2017, ceftobiprole also received Qualified Infectious Disease Product (QIDP) designation from the FDA for the treatment of SAB, adding to its QIDP designation for ABSSSI and community-acquired pneumonia. QIDP status extends the market exclusivity in the U.S. to ten years after approval.

Following the agreement of the SPAs with the FDA, the Biomedical Advanced Research and Development Authority (BARDA) allocated a second tranche of USD 58 million in June 2017 to support Basilea's clinical phase 3 program under BARDA's existing contract with Basilea.[2] BARDA allocated a first tranche of approximately USD 20 million in 2016. The total value of the BARDA contract could reach approximately USD 108 million if pre-defined milestones are met. In 2017, Basilea received reimbursements from BARDA of CHF 10.5 million. The ABSSSI study has started and Basilea anticipates commencing patient enrollment in the SAB study by mid-2018.

Expanding clinical testing of tumor checkpoint controller BAL101553 into brain cancer

Significant progress has also been achieved in the clinical-stage oncology projects. Basilea entered into a clinical study collaboration with the Adult Brain Tumor Consortium (ABTC), which is funded by the U.S. National Cancer Institute. A phase 1 study with tumor checkpoint controller BAL101553 in combination with radiotherapy in patients with newly diagnosed glioblastoma who have a reduced sensitivity to standard chemotherapy was started at the end of 2017. In addition, Basilea is also exploring BAL101553 as once-daily oral administration in patients with recurrent or progressive glioblastoma in a separate arm of the ongoing phase 1/2a study. Glioblastoma is the most common and aggressive form of primary malignant brain tumor and is an area of high medical need where very few treatment options are currently available.

In 2017, Basilea completed the phase 1 dose-escalation and established the clinical dose ranges for BAL101553 in two clinical phase 1/2a studies in patients with advanced solid tumors with daily oral administration and weekly 48-hour intravenous (i.v.) infusion, respectively. Basilea plans to initiate a phase 2a expansion with weekly 48-hour i.v. infusion in patients with recurrent glioblastoma and ovarian cancer.

BAL3833: potential first-in-class cancer therapy in phase 1 clinical testing

The oral dosage form of BAL3833 is currently being investigated by Basilea's partner and licensor of the drug candidate, the Institute of Cancer Research, in a clinical phase 1 dose-escalation study in patients with solid tumors including metastatic melanoma. The study is being sponsored

by The Royal Marsden NHS Foundation Trust. BAL3833 blocks BRAF and CRAF and also inhibits the SRC kinase family, which play an important role in the transmission of cell growth and proliferation signals. If deregulated, they are associated with tumor growth and the development of resistance to current therapies. BAL3833 is to Basilea's knowledge the only panRAF/SRC kinase inhibitor in clinical testing.

Focus on growing revenue and expanding pipeline

CEO Ronald Scott stated: "We are focused on further growing revenue from our marketed drugs Cresemba and Zevtera. We expect our partners to launch in additional countries in Europe and other parts of the world in 2018, driving sustainable top-line growth. We are expanding our clinical programs in oncology and we have initiated patient recruitment in our ceftobiprole phase 3 program with the goal to enter the U.S. market upon regulatory approval. We are actively exploring opportunities to further strengthen our pipeline in our focus areas of anti-infectives and oncology through internal and external innovation."

Key figures

(In CHF million, except per share data)	**2017**	**2016**
Product revenue	16.3	7.1
Contract revenue	74.0	57.7
Revenue from R&D services	0.3	0.2
Other revenue	10.8	0.9
Total revenue	**101.5**	**66.0**
Costs of products sold	(9.0)	(5.3)
Research & development expenses, net	(53.5)	(48.4)
Selling, general & administration expenses	(53.1)	(56.1)
Total cost and operating expenses	**(115.7)**	**(109.9)**
Operating loss	**(14.1)**	**(43.9)**
Net loss	**(19.4)**	**(51.3)**
Net cash provided by/ (used for) operating activities	19.0	(75.0)
Basic and diluted loss per share, in CHF	(1.79)	(5.07)

(In CHF million)	**Dec 31, 2017**	**Dec. 31, 2016**
Cash and financial investments	310.7	289.0

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

The consolidated financial statements of Basilea Pharmaceutica Ltd. for the financial year 2017 can be found on the Company's website at http://annualreport.basilea.com.

Financial summary

In 2017, Basilea substantially increased its revenues and significantly improved its financial results.

Full-year 2017 total revenue increased by 54% to CHF 101.5 million (2016: CHF 66.0 million) driven by product revenue of CHF 16.3 million (2016: CHF 7.1 million), contract revenue of CHF 74.0 million (2016: CHF 57.7 million), including CHF 37.7 million (2016: CHF 37.7 million) related to the global agreement for Toctino® and CHF 31.9 million (2016: CHF 19.3 million) related to the license agreements with Astellas and Pfizer for isavuconazole, and other revenue of CHF 10.8 million (2016: CHF 0.9 million), including CHF 10.5 million BARDA reimbursements (2016: CHF 0.7 million).

Research and development expenses amounted to CHF 53.5 million (2016: CHF 48.4 million), representing the Company's investment in the U.S. phase 3 program for the antibiotic ceftobiprole (before BARDA reimbursements), the phase 1/2a development of oncology drug candidate BAL101553, the phase 1 clinical development of oncology drug candidate BAL3833, activities for the pediatric program for ceftobiprole, and activities related to isavuconazole as well as other compounds in the Company's research portfolio.

Selling, general and administration expenses amounted to CHF 53.1 million (2016: CHF 56.1 million), and mainly include costs related to the commercialization of Cresemba and Zevtera in major European markets. The license agreement with Pfizer and the distribution agreement with Cardiome executed in 2017 mainly drive the decrease in expenses by CHF 3.0 million as compared to 2016.

The significant increase in revenues in 2017 resulted in a substantial decrease in operating loss by 68% to CHF 14.1 million from CHF 43.9 million in 2016. Net loss in 2017 was reduced to CHF 19.4 million (2016: CHF 51.3 million), resulting in a lower basic and diluted loss per share of CHF 1.79 (2016: CHF 5.07).

Operating activities in 2017 generated net cash of CHF 19.0 million as compared to a cash consumption of CHF 75.0 million in 2016. Combined cash and investments amounted to CHF 310.7 million as of December 31, 2017, compared to CHF 289.0 million as of December 31, 2016. This improvement in comparison to 2016 is mainly due to upfront and milestone payments received in the amount of CHF 86 million from licensing and distribution partners as well as higher revenue from product sales and royalties.

2018 Outlook

In 2018 Basilea will focus on:

- Growing revenues from Cresemba and Zevtera together with its partners including increasing contributions from markets outside of Europe and the U.S.
- Initiating ceftobiprole phase 3 study in bacteremia under BARDA contract
- Expand clinical programs in oncology
- Strengthen anti-infectives and oncology pipeline through internal and external innovation

Reflecting these key priorities, Basilea anticipates total revenue of approximately CHF 105-115 million with contributions from Cresemba and Zevtera increasing to approximately CHF 60-65 million. The operating loss in 2018 is estimated at approximately CHF 10-20 million.

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Portfolio

Cresemba (isavuconazole) – *an i.v. and oral azole antifungal addressing the urgent medical need for new options to treat invasive mold infections*

Isavuconazole is commercialized under the trade name Cresemba. Basilea has entered into license and distribution agreements for isavuconazole covering the U.S., Europe, China, Japan, Latin America, Asia-Pacific, the Middle East and North Africa (MENA) region, Canada, Russia, Turkey and Israel. To date isavuconazole has been granted market authorizations for the treatment of the mold infections invasive aspergillosis and mucormycosis in the U.S., 28 European Union member states, Iceland, Liechtenstein, Norway and Switzerland.[3, 4, 5] Isavuconazole has orphan drug designation for the approved indications in Europe and the U.S. and was designated a Qualified Infectious Disease Product (QIDP) by the U.S. FDA under the Generating Antibiotics Incentives Now (GAIN) Act. Outside European countries and the U.S., isavuconazole is currently not approved for commercial use.

Zevtera (ceftobiprole) – *a cephalosporin antibiotic for i.v. administration, for the treatment of severe bacterial infections in the hospital*

Ceftobiprole is commercialized under the trade name Zevtera in major European countries.[6] It has demonstrated rapid bactericidal activity against a wide range of Gram-positive and Gram-negative bacteria, including methicillin-susceptible and resistant *Staphylococcus aureus* (MSSA, MRSA) and susceptible *Pseudomonas* spp.[7] Ceftobiprole is currently approved for sale in major 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).[7] Basilea has entered into license and distribution agreements for the drug covering Europe, Latin America, China, the Middle East and North Africa (MENA) region, Canada and Israel. Ceftobiprole has QIDP status for the potential treatment of acute bacterial skin and skin structure infections (ABSSSI), *Staphylococcus aureus* bacteremia (SAB) and CAP. Ceftobiprole is not approved for commercial sale in the U.S.

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

The drug candidate BAL101553 (prodrug of BAL27862)[8] is being developed as a potential therapy for diverse cancers. The molecule binds the colchicine site of tubulin with distinct effects on microtubule organization,[9] resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.[10] It demonstrated *in-vitro* and *in-vivo* activity in diverse treatment-resistant cancer models, including tumors refractory to conventional approved therapeutics and radiotherapy.[11, 12, 13] BAL101553 efficiently distributes to the brain, with anticancer activity in glioblastoma models.[14, 15, 16]

BAL3833 – *a potential first-in-class cancer therapy (panRAF/SRC kinase inhibitor) in phase 1 clinical testing, which targets tumor growth and therapeutic resistance across diverse tumor types including melanoma*

BAL3833 (also known as CCT3833) is an orally available small-molecule drug candidate which interferes with the transmission of growth and proliferation signal cascades through so-called kinases. If deregulated, these signaling pathways may lead to uncontrolled growth, i.e. cancer, and also to the development of resistance to current therapies. BAL3833 is called a panRAF kinase inhibitor because it not only blocks the BRAF and CRAF kinases but also inhibits the SRC kinase family. In particular, melanoma, the most aggressive type of skin cancer, is often linked to a mutated BRAF kinase. BAL3833 demonstrated activity in preclinical studies in a range of patient-derived melanoma models with intrinsic or acquired resistance to selective BRAF inhibitors, as well as in tumor models derived from colorectal, pancreatic and lung cancers associated with genetic changes resulting in activation of the RAF pathway.[17] The compound

originates from The Institute of Cancer Research (ICR) in London, 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 Tuesday, February 27, 2018, 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) 866 291 4166 (USA)
+44 (0) 207 107 0613 (U.K.)

A playback will be available 1 hour after the conference call until Thursday, March 1, 2018, 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 (U.K.)
and will be asked to enter the ID 18917 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 2017 will take place on **Wednesday, April 18, 2018 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 10, 2018 will be entitled to participate and exercise their voting rights.

About Basilea

Basilea Pharmaceutica Ltd. is a commercial stage biopharmaceutical company developing products that address the medical challenge of increasing resistance and non-response to current treatment options in the therapeutic areas of bacterial infections, fungal infections and cancer. The company is committed to discovering, developing and commercializing 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 QuintilesIMSHealth SMART MIDAS, May 2017

2 Contract No. HHSO100201600002C, Biomedical Advanced Research and Development Authority (BARDA), the Department of Health and Human Services, Office of the Assistant Secretary for Preparedness and Response

3 Isavuconazole is approved in the U.S. for patients 18 years of age and older in the treatment of invasive aspergillosis and invasive mucormycosis. Cresemba U.S. prescribing information [Accessed: February 26, 2018]

4 In the 28 European Union member states, as well as in Iceland, Liechtenstein and Norway, isavuconazole is approved for the treatment of adult patients with invasive aspergillosis and for the treatment of adult patients with mucormycosis for whom amphotericin B is inappropriate. European Public Assessment Report (EPAR) Cresemba: http://www.ema.europa.eu [Accessed: February 26, 2018]

5 In Switzerland, isavuconazole is approved for the treatment of adult patients with invasive aspergillosis and for the treatment of mucormycosis in adult patients who are resistant to or intolerant of amphotericin B and in adult patients with moderate to severe renal impairment. Full indication in: Swissmedic-approved information for healthcare professionals as of August 2017

6 The trade name for ceftobiprole in Europe is generally Zevtera, except for France and Italy where the trade name is Mabelio, and Ireland, where the trade name is Adaluzis

7 U.K. Summary of Product Characteristics (SPC) Zevtera: http://www.mhra.gov.uk/ [Accessed: February 26, 2018]

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16 A. C. Mladek et al. The novel tubulin-binding 'tumor checkpoint controller' BAL101553 has anti-cancer activity alone and in combination treatments across a panel of GBM patient-derived xenografts. American Association for Cancer Research (AACR) annual meeting 2016, abstract 4781

17 G. Saturno et al. Therapeutic efficacy of the paradox-breaking panRAF and SRC drug CCT3833/BAL3833 in KRAS-driven cancer models. American Association for Cancer Research (AACR) annual meeting 2016, abstract LB-212

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