PRESS RELEASE

Basilea reports improved financial results in half-year 2017 driven by growing product sales; significant progress on partnering and development programs

- **Completed license agreement with Pfizer for Cresemba in Europe (excluding Nordics), Russia, Turkey and Israel; CHF 70 million upfront payment and up to USD 427 million in milestones**
- **56 percent increase in total revenue, amounting to CHF 46.2 million**
- **BARDA committed USD 54.8 million in additional funding to support phase 3 development of ceftobiprole for registration in the U.S.**
- **Agreement with Adult Brain Tumor Consortium for phase 1 clinical study to explore BAL101553 in newly diagnosed glioblastoma**

Basel, Switzerland, August 10, 2017 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its financial results for the first half of financial year 2017 with product sales from Cresemba® (isavuconazole) and Zevtera®/Mabelio® (ceftobiprole) in Europe increased to CHF 9.8 million (H1 2016: CHF 1.9 million; +416% year-on-year) plus royalties on U.S. Cresemba sales of CHF 5.2 million (H1 2016: CHF 3.0 million; +73%). Total revenue amounted to CHF 46.2 million (H1 2016: CHF 29.7 million; +56%). Basilea reported CHF 253.1 million in cash and investments as of June 30, 2017 and a significantly reduced operating loss of CHF 17.7 million in the first half-year (H1 2016: CHF 24.8 million; -29%).

Basilea's Chief Executive Officer Ronald Scott said: "We are very pleased with the strong sales performance of Cresemba in Europe and the U.S., which reflects its increasing recognition as a valuable treatment option for patients suffering from potentially life-threatening invasive fungal infections. Our recent license agreement with Pfizer is another important step toward our goal to maximize the global value of our brands."

Anti-infectives: Significant partnerships executed in important further markets

In June, Basilea announced a license agreement with Pfizer for the antifungal Cresemba in more than 40 countries in Europe (excluding the Nordics), Russia, Turkey and Israel. The transaction was completed on July 19, 2017, providing Basilea an upfront payment of CHF 70 million. Cresemba sales in Europe generated after the completion of the transaction are booked by Pfizer with Basilea receiving royalties in the mid-teen range on Pfizer's sales in its territory. Basilea is also eligible under the license agreement to receive up to USD 427 million in additional payments upon achievement of pre-specified regulatory and sales milestones.

Also in June, Basilea entered into a distribution agreement for isavuconazole and ceftobiprole with Avir Pharma Inc. for Canada, adding another partner to the distribution collaborations already in place for Latin America (Grupo Biotoscana S.L.), the Nordics (Unimedic Pharma AB) and the Middle East and North Africa (MENA) region (Hikma Pharmaceuticals LLC). The first product revenues from distribution partnerships were generated in H1 2017 and Basilea expects sales to steadily increase over the coming years as the drugs gain approval and are launched in additional countries.

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

For Japan, Basilea granted a license to Asahi Kasei Pharma Corporation for the development and commercialization of isavuconazole. Asahi Kasei Pharma is conducting an abbreviated clinical development program which is needed for a potential registration in Japan. Asahi Kasei Pharma successfully completed a phase 1 study in healthy volunteers and now plans to progress towards a phase 3 study, the details of which are currently under discussion with the Japanese regulatory authority.

Additional BARDA funding to support ceftobiprole phase 3 development for the U.S. market

Basilea was awarded additional USD 54.8 million in June 2017 under its contract with the Biomedical Advanced Research and Development Authority (BARDA). The funding is available to reimburse costs for the clinical phase 3 development of ceftobiprole to support a potential regulatory filing in the U.S. BARDA initially provided funding of approximately USD 20 million for the preparation of the phase 3 program. The total value of the BARDA contract could reach approximately USD 108 million over a period of 4.5 years from 2016 if pre-defined milestones are met. Based on the agreement with the U.S. Food and Drug Administration (FDA) on Special Protocol Assessments for two phase 3 studies with ceftobiprole – one in *Staphylococcus aureus* bacteremia (SAB) and one in acute bacterial skin and skin structure infections (ABSSSI) – Basilea is in preparation to initiate these studies.

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

Significant progress has also been achieved in the clinical-stage oncology projects. Basilea continued to explore the safety and tolerability of BAL101553 in two phase 1/2a clinical studies in patients with solid tumors. The first study evaluates the oral dosage form and the second study evaluates continuous infusion as an alternative dosing regimen. Based on promising preclinical data, the oral study was amended in late 2016 by adding a separate arm for patients with recurrent or progressive glioblastoma after prior radiotherapy, with or without chemotherapy. Glioblastoma is the most common primary brain tumor and one of the most lethal types of cancer. Interim data from both solid tumor studies were presented at the American Society of Clinical Oncology (ASCO) meeting this June.

To further explore the potential of BAL101553 in glioblastoma, Basilea will conduct a phase 1 clinical study in collaboration with the Adult Brain Tumor Consortium (ABTC), which is funded by the U.S. National Cancer Institute. The study will determine the safety and tolerability of BAL101553 in combination with standard radiation treatment in patients with newly diagnosed glioblastoma who have a reduced sensitivity to standard chemotherapy due to an unmethylated MGMT promoter. MGMT promoter status is an important prognostic molecular genetic biomarker in glioblastoma. Today, patients with an unmethylated MGMT promoter have fewer therapeutic options than those with a methylated MGMT promoter and also have a worse disease prognosis.

BAL3833 is investigated in an oral phase 1 clinical dose-escalation study in patients with solid tumor cancers including metastatic melanoma. The study is being sponsored by The Royal Marsden NHS Foundation Trust, a partner of The Institute of Cancer Research, London, from which the compound originates. 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.

Near-term focus on growing product revenues and progressing the pipeline

Basilea's CEO Ronald Scott stated: "Going forward, we are focusing on supporting Pfizer in preparing for commercializing Cresemba in major European markets. We expect that Pfizer will be able to further increase product sales across Europe, in which we will participate through royalties and potential sales milestone payments. To date, Basilea's existing partnerships cover more than eighty countries around the world and we are working toward further distribution

agreements for Cresemba and Zevtera in the few remaining key territories such as Asia-Pacific and China. We are also exploring partnering opportunities for Zevtera in Europe. In addition, we anticipate starting the ceftobiprole clinical phase 3 study in skin infections in the fourth quarter of this year and the bloodstream infection study in the first part of 2018, both under the BARDA contract."

In collaboration with the ABTC, Basilea plans to start the phase 1 combination study with BAL101553 in patients with newly diagnosed glioblastoma in the fourth quarter of this year. Furthermore, Basilea anticipates completing patient recruitment into the BAL101553 phase 1/2a solid tumor study with once-daily oral dosing by end of this year. Completion of patient recruitment into the separate glioblastoma study arm and into the continuous infusion study is expected in the first half of 2018. For BAL3833, completion of patient recruitment into the phase 1 study is anticipated in the next six to nine months.

Key figures

(In CHF million, except per share data)	**H1 2017**	**H1 2016**
Product revenue	9.8	1.9
Contract revenue	31.2	27.8
Revenue from R&D services	0.1	0.0
Other revenue	5.0	0.0
Total revenue	**46.2**	**29.7**
Costs of products sold	(3.5)	(3.0)
Research & development expenses, net	(26.4)	(24.8)
Selling, general & administration expenses	(33.9)	(26.8)
Total cost and operating expenses	**(63.9)**	**(54.6)**
Operating loss	**(17.7)**	**(24.8)**
Net loss	**(20.6)**	**(27.9)**
Net cash used for operating activities	(36.6)	(53.7)
Basic and diluted loss per share, in CHF	(1.90)	(2.76)

(In CHF million)	**June 30, 2017**	**Dec. 31, 2016**
Cash and financial investments	253.1	289.0

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

The unaudited condensed consolidated financial statements of Basilea Pharmaceutica Ltd. for the first half-year 2017 can be found on the Company's website at http://interimreport.basilea.com.

Financial summary

In the first half-year of 2017 product revenue increased to CHF 9.8 million (H1 2016: CHF 1.9 million). Contract revenue in this period amounted to CHF 31.2 million (1H 2016: CHF 27.8 million), including CHF 18.8 million (H1 2016: CHF 18.8 million) related to the global agreement for Toctino® and CHF 11.3 million (H1 2016: CHF 9.1 million) related to the license

agreement with Astellas for isavuconazole. Total revenue in H1 2017 including sales increased by 56% to CHF 46.2 million (H1 2016: CHF 29.7 million).

Research and development expenses amounted to CHF 26.4 million (H1 2016: CHF 24.8 million) and were mainly related to activities for the ceftobiprole phase 3 program for the U.S., 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.

Selling, general and administration expenses amounted to CHF 33.9 million (H1 2016: CHF 26.8 million), and included costs related to the commercialization of Cresemba and Zevtera/Mabelio in major European markets. The increase by CHF 7.1 million as compared to H1 2016 is mainly related to increased investments in the commercialization of Cresemba following additional country launches in H2 2016.

In H1 2017, the operating loss was reduced by 28.6% to CHF 17.7 million from CHF 24.8 million in H1 2016 and net loss in H1 2017 was reduced to CHF 20.6 million (H1 2016: CHF 27.9 million), resulting in a lower basic and diluted loss per share of CHF 1.90 (H1 2016: CHF 2.76).

The net cash used in operating activities in H1 2017 amounted to CHF 36.6 million as compared to CHF 53.7 million in H1 2016. This improvement in comparison to H1 2016 is mainly due to higher revenue from product sales and royalties.

Combined cash and investments amounted to CHF 253.1 million as of June 30, 2017*, compared to CHF 289.0 million as of December 31, 2016.

*(pre-Pfizer upfront payment)

Financial outlook

Basilea continues to focus on growing revenues from its two marketed products while at the same time advancing its clinical development pipeline. Basilea updates its financial guidance for 2017. As Cresemba sales are booked by Pfizer following completion of the transaction, Basilea now anticipates its 2017 product sales to be approximately CHF 13 million, and a participation in partner sales through royalties of approximately CHF 15 million. Total operating expenses for 2017, net of anticipated BARDA reimbursements, are estimated at CHF 9-10 million on average per month with an operating loss of approximately CHF 2 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 received marketing authorization in Europe for the treatment of adult patients with invasive aspergillosis and for the treatment of adult patients with mucormycosis for whom amphotericin B is inappropriate.[1] 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] A decision by Swissmedic on Basilea's marketing authorization application for Switzerland is anticipated in 2017. 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. Food and Drug Administration (FDA) under the Generating Antibiotics Incentives Now (GAIN) Act. Basilea has entered into license and distribution agreements for isavuconazole in the U.S., Europe, Japan, Latin America, the Middle East and North Africa (MENA) region, Canada, Russia, Turkey and Israel. The drug is commercialized under the trade name Cresemba. In Europe, it is currently marketed in Germany, Italy, the United Kingdom, France and Austria. Pfizer is anticipated to assume the responsibility for commercializing the drug in Europe (excluding the Nordic countries) by the end of 2017. Basilea's license partner Astellas

Pharma US markets the drug in the U.S. Outside the U.S. and the EU, isavuconazole is currently not approved for commercial use.

Zevtera/Mabelio (ceftobiprole) – *an antibiotic from the cephalosporin class for i.v. administration with 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*.

Ceftobiprole 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).[3] It received Qualified Infectious Disease Product (QIDP) designation from the FDA for the potential treatment of community-acquired bacterial pneumonia (CABP) and acute bacterial skin and skin structure infections (ABSSSI). The drug is currently marketed in Germany, Italy, the United Kingdom, France, Austria and Switzerland under the trade names Zevtera or Mabelio. Basilea has entered into distribution agreements for the drug in Latin America, the Middle East and North Africa (MENA) region, Canada and the Nordics. Basilea is conducting a clinical phase 3 program aiming at the regulatory approval of ceftobiprole in the United States. It consists of two cross-supportive phase 3 studies, one in the treatment of *Staphylococcus aureus* bacteremia (bloodstream infections) and the second one in ABSSSI. Basilea reached agreement with the FDA on Special Protocol Assessments for both studies. The program receives funding from the Biomedical Advanced Research and Development Authority (BARDA), the U.S. Department of Health and Human Services, Office of the Assistant Secretary for Preparedness and Response, under contract number HHSO100201600002C. The total value of the contract, which was signed in 2016, could reach approximately USD 108 million over a period of 4.5 years if pre-defined milestones are met.

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)[4] is being developed as a potential therapy for diverse cancers. Basilea is exploring once-daily oral dosing of BAL101553 in an open-label phase 1/2a study in adult patients with advanced solid tumors. This study was amended in late 2016 to include the enrollment of adult patients with recurrent or progressive glioblastoma (brain cancer) after prior radiotherapy with or without chemotherapy. In another phase 1/2a clinical study, Basilea is exploring weekly 48-hour continuous infusion of BAL101553 as an alternative dosing regimen for the treatment of solid tumors. In June 2017, Basilea entered into a clinical study agreement with the Adult Brain Tumor Consortium (ABTC) in the U.S., to conduct a clinical phase 1 study with BAL101553 to determine the safety and tolerability of BAL101553 in combination with standard radiation. The study will enroll patients with newly diagnosed glioblastoma who have a reduced sensitivity to the standard chemotherapy with temozolomide due to an unmethylated MGMT promoter. MGMT promoter status is an important prognostic molecular genetic biomarker in glioblastoma. In preclinical studies, the active moiety of the prodrug, BAL27862, demonstrated *in-vitro* and *in-vivo* activity against diverse treatment-resistant cancer models, including tumors refractory to conventional approved therapeutics and radiotherapy.[5, 6, 7] BAL101553 efficiently distributed to the brain, with anticancer activity in glioblastoma models.[8, 9, 10] BAL27862 binds the colchicine site of tubulin with distinct effects on microtubule organization,[11] resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.[12]

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 available small-molecule drug candidate. It is a panRAF/SRC kinase inhibitor as it blocks BRAF and CRAF and also inhibits the SRC kinase family. The compound originates from The Institute of Cancer Research in London, where it was developed by scientists funded by Cancer Research UK and the Wellcome Trust. It is currently being explored as a daily oral administration in a clinical phase 1 dose-escalation study in adult patients with advanced solid tumors, including metastatic melanoma. RAF and SRC kinases 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. In particular, melanoma 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,[13] as well as tumor models derived from colorectal, pancreatic and lung cancers associated with genetic changes resulting in activation of the RAF pathway.[14]

Conference call

Basilea Pharmaceutica Ltd. invites you to participate in a conference call on Thursday, August 10, 2017, 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) 866 291 4166 (USA)
+44 (0) 203 059 5862 (U.K.)

A playback will be available 1 hour after the conference call until Monday, August 14, 2017, 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 (U.K.)
and will be asked to enter the ID 15340 followed by the # sign.

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. Basilea 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.

For further information, please contact:

Peer Nils Schröder, PhD
Head Corporate Communications & Investor Relations
+41 61 606 1102
media_relations@basilea.com
investor_relations@basilea.com

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

References

1 European Public Assessment Report (EPAR) Cresemba: http://www.ema.europa.eu [Accessed: August 9, 2017]

2 Cresemba U.S. prescribing information [Accessed: August 9, 2017]

3 U.K. Summary of Product Characteristics (SPC) Zevtera®: http://www.mhra.gov.uk/ [Accessed: August 9, 2017]

4 J. Pohlmann et al. BAL101553: An optimized prodrug of the microtubule destabilizer BAL27862 with superior antitumor activity. American Association for Cancer Research (AACR) annual meeting 2011, abstract 1347; Cancer Research 2011, 71 (8 Supplement)

5 A. Broggini-Tenzer et al. The novel microtubule-destabilizing drug BAL101553 (prodrug of BAL27862) sensitizes a treatment refractory tumor model to ionizing radiation. EORTC-NCI-AACR symposium 2014, abstract 202

6 G. E. Duran et al. In vitro activity of the novel tubulin active agent BAL27862 in MDR1(+) and MDR1(-) human breast and ovarian cancer variants selected for resistance to taxanes. American Association for Cancer Research (AACR) annual meeting 2010, abstract 4412

7 F. Bachmann et al. BAL101553 (prodrug of BAL27862): A unique microtubule destabilizer active against drug refractory breast cancers alone and in combination with trastuzumab. American Association for Cancer Research (AACR) annual meeting 2014, abstract 831

8 R. Bergès et al. The novel tubulin-binding checkpoint activator BAL101553 inhibits EB1-dependent migration and invasion and promotes differentiation of glioblastoma stem-like cells. Molecular Cancer Therapeutics 2016 (15), 2740-2749

9 A. Schmitt-Hoffmann et al. BAL27862: a unique microtubule-targeted agent with a potential for the treatment of human brain tumors. AACR-NCI-EORTC conference 2009, abstract C233; Molecular Cancer Therapeutics 2009, 8 (12 Supplement)

10 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

11 A. E. Prota et al. The novel microtubule-destabilizing drug BAL27862 binds to the colchicine site of tubulin with distinct effects on microtubule organization. Journal of Molecular Biology 2014 (426), 1848-1860

12 F. Bachmann et al. BAL101553 (prodrug of BAL27862): the spindle assembly checkpoint is required for anticancer activity. American Association for Cancer Research (AACR) annual meeting 2015, abstract 3789

13 M. R. Girotti et al. Paradox-breaking RAF inhibitors that also target SRC are effective in drug-resistant BRAF mutant melanoma. Cancer Cell 2015 (27), 85-96

14 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