

PRESS RELEASE

Basilea reports half-year financial results reflecting strong in-market product sales growth in addition to progress and expansion of the R&D pipeline

- Total revenue increased by 30% to CHF 59.9 million; 2018 total revenue guidance is increased to CHF 120 – 130 million
- Royalties on Cresemba sales more than doubled to CHF 10.8 million
- Clinical oncology pipeline strengthened through in-licensing of phase 2 panFGFR kinase inhibitor (derazantinib)
- Significant progress made in clinical programs
- Cash position of CHF 247.3 million

Basel, Switzerland, August 14, 2018 – Basilea Pharmaceutica Ltd. (SIX: BSLN) today reported financial results for the first half-year, ended June 30, 2018.

Total revenue increased to CHF 59.9 million (H1 2017: CHF 46.2 million; +30% year-on-year). Revenue contributions from its marketed products Cresemba® and Zevtera® increased to CHF 27.8 million (H1 2017: CHF 22.2 million; +25%) with royalties on Cresemba sales more than doubling to CHF 10.8 million (H1 2017: CHF 5.3 million; +104%). As of June 30, 2018, Basilea reports CHF 247.3 million in cash and financial investments (year-end 2017: CHF 310.7 million) and an operating loss of CHF 20.4 million compared to CHF 19.1 million in H1 2017.

David Veitch, Chief Executive Officer, said: “We are very pleased with the strong sales performance in the first half of 2018. Our commercial partners continued to significantly grow product sales in existing markets and successfully launched our anti-infectives Cresemba and Zevtera in new markets. We have initiated key clinical studies for ceftobiprole and BAL101553 and demonstrated our ability to expand our R&D pipeline.”

In the 12-months to March 2018, “in-market” sales of antifungal Cresemba reached USD 120 million and continued to grow strongly

Since the start of 2018, Pfizer launched Cresemba in Switzerland, Ireland, Greece and the Netherlands. In addition, Grupo Biotoscana was granted regulatory approval for Cresemba in Peru, the first approval of the brand outside the U.S. and Europe, which triggered a regulatory milestone payment of CHF 2 million to Basilea. As of March 31, 2018, the 12-months in-market Cresemba sales amounted to USD 120 million, compared to USD 62 million in the 12 months to March 31, 2017.¹ For the first six months 2018 Astellas Pharma Inc. reported U.S. Cresemba sales of USD 54 million (H1 2017: USD 34 million; +59% year-on-year).

Zevtera sales in Europe by Correvio (formerly Cardiome) increased and sales growth is expected to further accelerate with increasing contributions from new markets outside of Europe, for example, Argentina, Canada and Saudi Arabia.

Basilea expanded its oncology pipeline

In April 2018, Basilea entered into a license agreement with ArQule, Inc., for its late-stage oncology drug candidate derazantinib (BAL087), which targets the fibroblast growth factor receptor (FGFR) family of kinases. The exclusive license grants Basilea worldwide rights to

derazantinib, excluding Greater China. Basilea made an upfront payment of USD 10 million and ArQule is eligible to receive up to USD 326 million upon reaching certain clinical, regulatory and sales milestones, as well as staggered single to double-digit royalties on sales upon commercialization.

Dr. Marc Engelhardt, Basilea's Chief Medical Officer, said: "As part of our strategy, we are continuously exploring opportunities to further strengthen our R&D portfolio through external innovation. The small-molecule drug candidate derazantinib fits with our existing oncology pipeline. It targets an important signal transmission pathway which is considered to be relevant for various tumor types, in patients with limited treatment options. Derazantinib also allows for a clinical development program that builds upon a clear patient selection strategy."

In addition, Basilea has entered into a licensing and research collaboration. The pre-clinical project focuses on the biomarker-driven development of potential first-in-class selective inhibitors of a kinase involved in controlling the process of chromosome segregation during cell division.

Significant progress made in clinical stage programs

During the first half of 2018, Basilea made significant progress in its clinical development programs:

Advanced the ceftobiprole phase 3 program to access the U.S. market

Basilea advanced its phase 3 program comprising two cross-supportive studies aiming to gain regulatory approval for ceftobiprole in the U.S. The U.S. market is an estimated 80% of the global market for branded hospital antibiotics based on value¹ and therefore it plays a critical role in Basilea's commercial strategy for ceftobiprole.

The first study, in acute bacterial skin and skin structure infections (ABSSSI), started in February 2018 and the second study, in *Staphylococcus aureus* bacteremia (SAB), was also recently initiated.² The studies are conducted under Special Protocol Assessments (SPAs) agreed with the FDA.

The phase 3 program is funded in part (up to USD 118 million, which is approximately 70% of the total estimated program costs) with Federal funds from the U.S. Department of Health and Human Services; Office of the Assistant Secretary for Preparedness and Response; Biomedical Advanced Research and Development Authority (BARDA), under Contract No. HHSO100201600002C. For the first half of 2018, Basilea recorded reimbursements for R&D expenses for this phase 3 program from BARDA in the amount of CHF 13.2 million.

Started phase 3 study with isavuconazole in Japan

Asahi Kasei Pharma, Basilea's partner for isavuconazole in Japan, initiated a phase 3 study in April 2018 to support a future regulatory filing in Japan.³ Japan currently represents about eight percent of the global market for best-in-class antifungals.¹

Advanced tumor checkpoint controller BAL101553 into phase 2a expansion study in glioblastoma and ovarian cancer

Basilea continued its activities in the field of glioblastoma, the most common and aggressive form of primary malignant brain tumor and an area of high medical need where very few treatment options are currently available. Basilea is currently running three clinical studies with BAL101553 in this indication.

In the UK, phase 1 dose escalation is ongoing in recurrent or progressive glioblastoma patients with daily oral administration. The study is primarily designed to assess safety at various dose levels. In Switzerland, a phase 2a expansion study in recurrent glioblastoma was started in June using weekly 48-hour infusion. A separate arm in this study includes patients with platinum-resistant ovarian cancer. Finally, Basilea started a phase 1 study in the U.S. in newly diagnosed glioblastoma patients with first-line oral BAL101553 in combination with radiotherapy. This study is

conducted in collaboration with the Adult Brain Tumor Consortium (ABTC), which is funded by the U.S. National Cancer Institute.

These studies will contribute to an assessment of efficacy signals and Basilea expects that an initial assessment of efficacy could become available during 2019.

Completed patient enrollment into phase 1 study with panRAF/SRC inhibitor BAL3833

Basilea's partner and licensor, the Institute of Cancer Research (ICR), completed enrollment into a first-in-human phase 1 dose-escalation study in patients with solid tumors including metastatic melanoma.⁴ 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.

A broad dose range was investigated in the study. A maximum tolerated dose was not defined. The study is currently in the analysis phase, including biomarker data, and results are anticipated to be published at a future scientific conference.

Registrational phase 2 study with derazantinib for intrahepatic cholangiocarcinoma (iCCA) ongoing

Derazantinib has demonstrated favorable clinical data in a biomarker-driven phase 1/2 study in patients with FGFR2 fusion positive iCCA, a form of biliary tract cancer. A registrational phase 2 study with derazantinib in patients with FGFR2 fusion positive iCCA is ongoing in Italy, Canada and the U.S.⁵ An interim analysis of the study is planned in H1 2019. The study may allow to file for U.S. accelerated approval in iCCA, subject to the final outcome of the study.

Basilea plans to initiate clinical phase 2 development of derazantinib in other FGFR-driven solid tumor types in mid-2019.

Basilea continues to implement its strategy for sustainable growth based on its focus areas: hospital antibiotics, hospital antifungals, and oncology

CEO David Veitch stated: "We are well on track in the execution of our strategy, in terms of both growing our revenues and advancing our R&D portfolio. Our partners for Cresemba and Zevtera are making significant progress in the development and commercialization of our two anti-infective brands around the world. We will continue to build on internal and external innovation in the areas of hospital antibiotics, hospital antifungals and oncology to optimize our portfolio and create the basis for sustainable long-term growth."

Key figures

<i>(In CHF million, except per share data)</i>	H1 2018	H1 2017
Product revenue	6.5	9.8
Contract revenue	40.1	31.2
Revenue from R&D services	0.0	0.1
Other revenue	13.3	5.0
Total revenue	59.9	46.2
Costs of products sold	(6.5)	(3.5)
Research & development expenses, net	(57.8)	(27.2)
Selling, general & administrative expenses	(15.9)	(34.6)
Total cost and operating expenses	(80.3)	(65.3)
Operating loss	(20.4)	(19.1)
Net loss	(22.5)	(20.6)
Net cash used for operating activities	(60.4)	(36.6)
Basic and diluted loss per share, in CHF	(2.07)	(1.90)

<i>(In CHF million)</i>	June 30, 2018	Dec 31, 2017
Cash and financial investments	247.3	310.7

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 2018 can be found on the Company's website at <http://interimreport.basilea.com>.

Financial summary

In the first half year of 2018, Basilea substantially increased its revenues and accelerated investments in its pre-clinical and clinical pipeline.

Total revenue in this period increased by 30% to CHF 59.9 million (H1 2017: CHF 46.2 million). As a result of the change in the commercialization model for Europe in 2017, the allocation between product revenue and contract revenue has changed. Together, product and contract revenue continued to grow to CHF 46.6 million (H1 2017: CHF 41.0 million), particularly driven by Cresemba revenue contributions increasing by 30% to CHF 26.3 million (H1 2017: CHF 20.3 million). Other revenue increased to CHF 13.3 million (H1 2017: CHF 5.0 million), including CHF 13.2 million BARDA reimbursements (H1 2017: CHF 4.9 million) compensating for a substantial portion of expenses incurred related to Basilea's ceftobiprole phase 3 program.

In the first half of 2018, Basilea accelerated investments in its pre-clinical and clinical pipeline. Research and development expenses amounted to CHF 57.8 million (H1 2017: CHF 27.2 million), and were mainly driven by costs for the phase 3 program for the antibiotic ceftobiprole for which patient recruitment is ongoing since February, the in-licensing of clinical stage drug candidate derazantinib, including an upfront payment of USD 10 million, as well as in-licensing of

pre-clinical oncology compounds, the phase 1/2a development of oncology drug candidate BAL101553 and activities related to the pediatric programs for ceftobiprole and isavuconazole.

Selling, general and administrative expenses declined significantly to CHF 15.9 million (H1 2017: CHF 34.6 million). This decrease was mainly driven by the transfer of the commercialization of Cresemba and Zevtera from Basilea to its partners following the license agreement with Pfizer and the distribution agreement with Correvio in the second half of 2017.

Costs of product sold, which include manufacturing costs, capacity reservation costs as well as shipping and handling costs, amounted to CHF 6.5 million (H1 2017: CHF 3.5 million) and increased approximately in line with the increased Zevtera and Cresemba demand supplied by Basilea.

In the first half year of 2018, the operating loss amounted to CHF 20.4 million compared to CHF 19.1 million for the first half year of 2017. Net loss in the first half year of 2018 was CHF 22.5 million (H1 2017: CHF 20.6 million), resulting in a basic and diluted loss per share of CHF 2.07 (H1 2017: CHF 1.90).

Operating activities in the first half year of 2018 consumed cash of CHF 60.4 million as compared to a cash consumption of CHF 36.6 million in the first half year of 2017, mainly reflecting the progress in and the expansion of Basilea's R&D pipeline and an increase in working capital. Combined cash and investments amounted to CHF 247.3 million as of June 30, 2018, compared to CHF 310.7 million as of December 31, 2017.

In March 2018, Basilea entered into a market making agreement with Kepler Cheuvreux. Kepler Cheuvreux provides market liquidity supporting trading of the company's shares on the SIX Swiss Exchange.

2018 Outlook

For the second half year of 2018 Basilea will continue to focus on:

- Growing revenues from Cresemba and Zevtera, including preparing for further launches in markets outside of Europe and the U.S.
- Progressing the ceftobiprole phase 3 studies in ABSSEI and SAB under the BARDA contract, for gaining access to the U.S. market
- Advancing the ongoing clinical studies of derazantinib and BAL101553
- Exploring opportunities to strengthen its hospital antibiotic, hospital antifungal and oncology pipeline through internal and external innovation

Reflecting these key priorities and the performance in the first half of 2018 Basilea updates its previous financial guidance for 2018. The company increases its guidance for total revenue from CHF 105-115 million to CHF 120-130 million with contributions from Cresemba and Zevtera expected to increase from CHF 60-65 million to CHF 75-85 million. Considering the upfront payments to license derazantinib and the pre-clinical oncology assets as well as the respective ongoing program costs, the operating loss for full-year 2018 is estimated at CHF 25-35 million.

Portfolio

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

Basilea has entered into license and distribution agreements for isavuconazole covering the United States, Europe, China, Japan, Latin America, Canada, Russia, Turkey, Israel and the Asia-Pacific and Middle East and North Africa regions. In the EU and EEA member states, as well as in Jordan and Peru, 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.⁷ It is also approved in the U.S. and Switzerland.^{6, 8} Isavuconazole has U.S. and European orphan drug designation for its approved indications.

Zevtera/Mabelio (ceftobiprole) – a cephalosporin antibiotic for i.v. administration, for the treatment of severe bacterial infections in the hospital⁹

Ceftobiprole has 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.¹⁰ Ceftobiprole is approved for the treatment of adult patients with community-acquired pneumonia (CAP) and hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP).¹⁰ It is marketed in major European countries, Argentina, Canada and Saudi Arabia. Basilea has entered into license and distribution agreements for the brand in Europe, Latin America, China, Canada, Israel, and the Middle East and North Africa (MENA) region. Ceftobiprole is currently in a phase 3 program for registration of ceftobiprole in the U.S.

Derazantinib (BAL087) – an investigational oral inhibitor of the FGFR family of kinases with potential in a number of cancer types and the opportunity to become the first FGFR kinase inhibitor approved for intrahepatic cholangiocarcinoma (iCCA), an indication with high unmet need and increasing incidence

FGFR kinases are key drivers of cell proliferation, differentiation and migration. Basilea in-licensed derazantinib from ArQule, Inc. The drug candidate has demonstrated favorable clinical data in a biomarker-driven phase 1/2 study in iCCA patients.¹¹ Both the FDA and EMA have granted ArQule orphan drug designation for this disease.

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)¹² is being developed as a potential therapy for diverse cancers. The molecule binds the colchicine site of tubulin with distinct effects on microtubule organization,¹³ resulting in the activation of the "spindle assembly checkpoint" which promotes tumor cell death.¹⁴ It demonstrated *in-vitro* and *in-vivo* activity in diverse treatment-resistant cancer models, including tumors refractory to conventional approved therapeutics and radiotherapy.^{15, 16, 17} BAL101553 efficiently distributes to the brain, with anticancer activity in glioblastoma models.^{18, 19, 20}

BAL3833 – a dual-targeting (panRAF/SRC) kinase inhibitor in clinical phase 1 testing in patients with diverse solid tumors, 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.²¹ 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 and webcast

Basilea Pharmaceutica Ltd. will host a conference call today, Tuesday, August 14, 2018, at 4 p.m. (CEST), to discuss the Company's financial and operating results.

Via audio webcast with presentation:

For the first time Basilea will conduct a live audio webcast of the results presentation, which can be followed [here](#). Please note that there is no function to ask questions via webcast. For questions, please additionally dial-in via phone (see below).

Via phone:

To listen by phone and ask questions, please use the dial-in details below. To ensure prompt access, please call approximately five minutes prior to the scheduled start of the call.

+41 (0) 58 310 5000 (Europe and RoW)
+1 (1) 866 291 4166 (USA)
+44 (0) 207 107 0613 (U.K.)

Replay:

The webcast, along with presentation will be available [online](#) shortly after the event and accessible for three months.

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. With two commercialized drugs, 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.

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