PRESS RELEASE

Basilea reports significantly increased cash-generating revenue, flat operating expenses and reduced net loss for half-year 2019

- **Revenue from Cresemba® and Zevtera® increased by 91% to CHF 53 million**
- **Operating result improved by 35%**
- **Expansion of derazantinib clinical program into urothelial cancer with immunotherapy combination**
- **Positive phase 3 topline results from skin infection (ABSSSI) study with ceftobiprole**
- **Half-year cash position of CHF 178 million**

Basel, Switzerland, August 20, 2019 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its financial results for the first six months of its financial year 2019, ended June 30, 2019.

David Veitch, Chief Executive Officer, said: "We have made significant progress both from the operational and financial perspective in the first half of 2019. We are generating increasing cash flow from our two marketed drugs Cresemba and Zevtera. In particular, the sales of the antifungal Cresemba continue to grow impressively, not only in existing markets, but also from a growing number of recently launched countries. At the same time, we have been successful in maintaining our overall expense base at a flat level in spite of the continued selective investment in our R&D portfolio. Key R&D highlights in the first half of 2019 included the expansion of the clinical program for our lead oncology drug candidate derazantinib and the positive topline results reported from the first of the two ceftobiprole phase 3 studies required for a filing in the U.S. We remain focused on increasing our revenues of our two marketed brands and on progressing our development stage assets to the next key value inflection points."

Financial summary

Total revenue in the first half-year 2019 increased by 5.5% to CHF 63.2 million (H1 2018: CHF 59.9 million). Together, product revenue and contract revenue continued to grow to CHF 53.1 million (H1 2018: CHF 46.6 million), with contributions from the two marketed products Cresemba and Zevtera increasing by 91.0% to CHF 52.9 million (H1 2018: CHF 27.7 million). This significant revenue increase fully offset the impact from the completion of the non-cash revenue recognition related to Toctino® in 2018 (H1 2018: CHF 18.8 million; H2 2018: CHF 5.1 million). Other revenue decreased to CHF 10.1 million (H1 2018: CHF 13.3 million), including CHF 9.9 million BARDA reimbursements (H1 2018: CHF 13.2 million) offsetting a substantial portion of the reported research and development expenses related to Basilea's ceftobiprole phase 3 program, and reflecting lower than anticipated total costs for the completion of the ceftobiprole phase 3 skin infection study.

In the first half-year 2019 investments in Basilea's pipeline resulted in research and development expenses of CHF 50.8 million (H1 2018: CHF 57.8 million). Such expenses were mainly driven by costs for the two phase 3 studies for the antibiotic ceftobiprole, the costs related to the ongoing pre-clinical and clinical program for derazantinib, the phase 1/2a development of oncology

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drug candidate BAL101553 and the ongoing pediatric programs for ceftobiprole and isavuconazole.

Selling, general and administrative expenses remained flat at CHF 16.2 million (H1 2018: CHF 16.0 million). Costs of product sold, which include manufacturing costs, capacity reservation costs, shipping and handling costs as well as certain one-off expenses amounted to CHF 9.4 million (H1 2018: CHF 6.5 million).

In the first half-year 2019, the operating loss amounted to CHF 13.2 million (H1 2018: CHF 20.4 million). Net loss in the first half-year 2019 was significantly reduced to CHF 15.4 million (H1 2018: CHF 22.5 million), resulting in a basic and diluted loss per share of CHF 1.44 (H1 2018: CHF 2.07).

Cash consumption from operating activities in the first half-year 2019 was reduced by 25% to CHF 45.4 million as compared to a cash consumption of CHF 60.4 million in the first half-year 2018. The reduction is a result, on the one hand of the significant increase of cash-generating revenue from Cresemba and Zevtera and, on the other hand, of Basilea's continued focus on managing its operating expenses by continuously optimizing its pre-clinical and clinical portfolio and targeting its investments into its R&D pipeline. Combined cash and short-term investments amounted to CHF 177.9 million as of June 30, 2019, compared to CHF 223.0 million as of December 31, 2018.

Key financial figures

(In CHF million, except per share data)	**H1 2019**	**H1 2018**
Product revenue	25.4	6.5
Contract revenue[1]	27.6	40.1
Revenue from R&D services	0.1	0.0
Other revenue	10.1	13.3
Total revenue	**63.2**	**59.9**
Costs of products sold	(9.4)	(6.5)
Research & development expenses, net	(50.8)	(57.8)
Selling, general & administrative expenses	(16.2)	(16.0)
Total cost and operating expenses	**(76.4)**	**(80.3)**
Operating loss	**(13.2)**	**(20.4)**
Net loss	**(15.4)**	**(22.5)**
Net cash used in operating activities	(45.4)	(60.4)
Basic and diluted loss per share, in CHF	(1.44)	(2.07)

(In CHF million)	**June 30, 2019**	**Dec 31, 2018**
Cash and short-term investments	177.9	223.0

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

[1] Contract revenue include CHF 0.0 million (H1 2018: CHF 18.8 million) related to Toctino® deferred revenue recognition.

The unaudited, condensed consolidated financial statements of Basilea Pharmaceutica Ltd. for the first half-year 2019 can be found on the Company's website at https://www.basilea.com/financial-reports.

2019 Outlook

For the second half of 2019 Basilea will focus on:

- Through its commercialization partners, significantly growing in-market sales of Cresemba and Zevtera, including launching in additional markets.
- Advancing the derazantinib registrational phase 2 study in intrahepatic cholangiocarcinoma and the phase 1/2 study in urothelial cancer towards data read-outs in 2020.
- Progressing the ceftobiprole phase 3 study in *Staphylococcus aureus* bacteremia towards top line results in H2 2021.
- Continuing to explore opportunities to selectively expand the clinical and pre-clinical oncology portfolio through both in-licensing and internal development.

Reflecting these key priorities and the performance in the first half-year, Basilea provides the following financial guidance for full-year 2019:

- The company anticipates continued strong revenue growth from Cresemba and Zevtera to CHF 105-110 million (+28% to +34% y-o-y), which is at the upper end of its previous guidance of CHF 100-110 million.
- The company narrows its previous guidance on total revenue to CHF 128-133 million, reflecting lower than previously anticipated BARDA revenue based on lower R&D expenses incurred related to the ceftobiprole phase 3 program. The revenue growth from its two marketed products is expected to compensate for the completion of non-cash revenue recognition from Toctino in 2018.
- Total cost and operating expenses are expected to remain at approximately the same level as 2018, leading to an anticipated operating loss of CHF 22-27 million for 2019.
- Net cash used by operating activities in H2 2019 is expected to further decrease as compared to H1 2019, resulting in an anticipated net cash consumption by operating activities of CHF 60-65 million (-18% to -24% y-o-y) for the full year.

Portfolio – Substantial progress in clinical pipeline and accelerating commercialization of marketed drugs since the beginning of 2019

Accelerated commercialization of Cresemba and Zevtera

Since the beginning of 2019, Cresemba has been launched by Basilea's partners in 14 additional countries. Cresemba is now marketed in 33 countries globally, thus Basilea's partners are well on track to doubling the number of countries in which Cresemba is marketed, to around 40 by end-2019. According to the latest available public data, global in-market sales of Cresemba reached USD 170 million in the 12 months to the end of March 2019.

Zevtera was launched by Basilea's partner Hikma in Jordan. In total, the brand has been launched in 17 countries now. Zevtera sales continued to increase and sales growth is expected to further accelerate with increasing contributions from the newly launched countries inside and outside of Europe.

Expanded clinical development program for derazantinib and early-stage pipeline

In January 2019, positive interim results from FIDES-01, a registrational phase 2 study with derazantinib in intrahepatic cholangiocarcinoma (iCCA) have been reported.[1] The drug candidate, which was in-licensed from U.S. company ArQule Inc. in 2018, targets the fibroblast growth factor receptor (FGFR) family of kinases, namely FGFR1, 2 and 3. Based on the encouraging efficacy demonstrated in iCCA patients with FGFR2 gene fusions, an additional cohort was opened for iCCA patients with FGFR2 gene mutations and amplifications in order to further profile the drug candidate in this indication. Topline data of the FIDES-01 study are expected to be available around mid-2020. Furthermore, a new study, FIDES-02, was initiated to

explore derazantinib in patients with advanced urothelial cancer with FGFR genomic aberrations.[2] The study will assess derazantinib alone and in combination with Roche's immune-checkpoint inhibitor atezolizumab (Tecentriq®)*. The combination rationale is based on the activity of derazantinib to inhibit colony-stimulating factor-1-receptor kinase (CSF1R), which differentiates derazantinib from other FGFR kinase inhibitors in clinical development. CSF1R inhibition has the potential to enhance the response to atezolizumab's immune-checkpoint inhibition. Roche is providing clinical supply of atezolizumab for the study.

As reported previously, a maximum tolerated dose could not be defined for the oral formulation of the panRAF/SRC kinase inhibitor BAL3833 in the first-in-human phase 1 dose-escalation study in patients with solid tumors that was completed last year.[3] Based on the observed pharmacokinetics, Basilea decided not to move forward with the current formulation. Pre-clinical activities to explore alternative formulations are ongoing.

In addition, Basilea has entered into licensing and research collaborations for pre-clinical compounds in its strategic focus areas of oncology and infectious diseases.

Positive topline results from phase 3 study with ceftobiprole (Zevtera®)

In August 2019, Basilea reported positive topline results from the phase 3 TARGET study, which evaluated ceftobiprole in the treatment of patients with acute bacterial skin and skin structure infections (ABSSSI).[4] Ceftobiprole met primary and secondary efficacy endpoints and was well tolerated with the overall rates of drug-related adverse events being similar between the ceftobiprole and control group. For a future regulatory filing in the U.S., positive results from a second phase 3 study, ERADICATE, would also be required.[5] ERADICATE is exploring ceftobiprole in patients with bloodstream infections (bacteremia) caused by *Staphylococcus aureus* bacteria and is on track to report topline results in the second half of 2021.

The ceftobiprole phase 3 program is funded in part (up to USD 128 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.

Ongoing glioblastoma and ovarian cancer studies with tumor checkpoint controller BAL101553

A phase 1 study arm with daily oral administration of BAL101553 in patients with recurrent glioblastoma, an aggressive form of brain cancer, continues towards defining the maximum tolerated dose.[6] Completion is currently expected in 2019. Also a phase 2a expansion study, in which BAL101553 is given as weekly 48-hour infusion in patients with recurrent glioblastoma or platinum-resistant ovarian cancer is ongoing and anticipated to complete around year-end 2019.[7] In addition, a separate phase 1 study with daily oral dosing of BAL101553 in combination with radiotherapy in patients with newly diagnosed glioblastoma is ongoing.[8] This study is conducted in collaboration with the U.S. Adult Brain Tumor Consortium, and patient enrolment into the study could be completed by mid-2020.

** Tecentriq® is a registered trademark of Hoffmann-La Roche Ltd.*

Conference call and webcast

Basilea Pharmaceutica Ltd. will host a conference call and webcast today, Tuesday, August 20, 2019, at 4 p.m. (CEST), to discuss the Company's financial and operating results.

Via audio webcast with presentation:
The live audio webcast of the results presentation 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, focused on the development of products that address the medical challenges in the therapeutic areas of oncology and anti-infectives. 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

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

References

1 ClinicalTrials.gov identifier: NCT03230318
2 ClinicalTrials.gov identifier: NCT04045613
3 ClinicalTrials.gov identifier: NCT02437227
4 ClinicalTrials.gov identifier: NCT03137173
5 ClinicalTrials.gov identifier: NCT03138733
6 ClinicalTrials.gov identifier: NCT02490800
7 ClinicalTrials.gov identifier: NCT02895360
8 ClinicalTrials.gov identifier: NCT03250299