PRESS RELEASE

Basilea reports significant revenue growth from marketed products and pipeline progress for full-year 2019

- **Revenue contributions from Cresemba® and Zevtera® increased 39% to CHF 114 million**
- **Operating result improved by 29%**
- **Start of urothelial cancer study for derazantinib with immunotherapy combination and further expansion planned into gastric cancer**
- **Positive topline results reported from ceftobiprole phase 3 skin infection (ABSSSI) study**
- **Year-end cash and investments of CHF 161 million and reduction in net operating cash consumption**
- **In 2020, revenue contributions from Cresemba and Zevtera, excluding deferred revenue, expected to grow 12% to 27% year-on-year**

Basel, Switzerland, February 18, 2020 – Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its financial results for the financial year ended December 31, 2019.

David Veitch, Chief Executive Officer, said: "Basilea made significant progress in 2019. Revenue contributions from our two marketed brands, Cresemba and Zevtera, increased by 39 percent year-on-year. At the same time, we continued to manage closely our expenses, leading to a further reduction of our net cash consumption, and a 29 percent improvement of our operating result. Cresemba sales by our partners continued to grow significantly, both in established markets and new markets, which triggered two sales milestone payments based on sales in Europe. Our commercialization partners doubled the number of launched countries during 2019 and we expect that Cresemba will be available in about 60 countries by the end of 2021."

He continued: "Important progress was also made for lisavanbulin and derazantinib, our most advanced oncology programs. We have identified a potentially response-predictive biomarker approach for lisavanbulin and decided to move the drug candidate forward into a targeted phase 2 study in patients with glioblastoma. For derazantinib, we have started a phase 1/2 study in patients with urothelial cancer and are planning to further expand the clinical development program into gastric cancer. In both studies, derazantinib will be explored as a single drug and in combination with Roche's immune checkpoint inhibitor, atezolizumab. Preclinical profiling of derazantinib indicated that it may make tumors more susceptible to immunotherapy. For our antibiotic ceftobiprole, we have successfully completed the first of the two phase 3 studies which are necessary to support a regulatory filing in the U.S., the commercially most important market for novel branded hospital antibiotics. We expect continued strong global sales uptake for Cresemba while we remain focused on carefully managing our expenses and are convinced that we are very well positioned to take our next value-generating steps."

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Financial summary

Total revenue in 2019 increased to CHF 134.4 million (2018: CHF 132.6 million). Together, product revenue and contract revenue increased to CHF 114.4 million (2018: CHF 105.9 million). Revenue contributions from the two marketed brands Cresemba and Zevtera increased 39% to CHF 114.3 million (2018: CHF 82.0 million), of which CHF 68.7 million (2018: CHF 50.5 million) relate to non-deferred revenue and CHF 45.6 million (2018: CHF 31.5 million) to deferred revenue recognition. Deferred revenue is recognized for upfront, development and regulatory milestone payments received in prior years from partners. The company was able to fully offset the impact from the completion of the non-cash deferred revenue recognition related to Toctino® in 2018 (2018: CHF 23.9 million). Other revenue amounted to CHF 19.7 million (2018: CHF 26.5 million). This included CHF 18.5 million BARDA reimbursements (2018: CHF 25.9 million), which are offsetting a substantial portion of the ceftobiprole phase 3 development expenses. The BARDA reimbursements decreased in line with the reduced development expenses after the successful completion of the ceftobiprole phase 3 skin infection study.

In 2019, investments in Basilea's pipeline resulted in research and development expenses of CHF 102.7 million (2018: CHF 104.9 million). Such expenses were mainly driven by costs for the two phase 3 studies for the antibiotic ceftobiprole, the costs related to the ongoing preclinical and clinical program for derazantinib, the phase 1/2a development of oncology drug candidate lisavanbulin and the ongoing pediatric programs for ceftobiprole and isavuconazole.

Selling, general and administrative expenses remained flat at CHF 30.0 million (2018: CHF 31.4 million). Cost of products sold, which includes manufacturing costs, capacity reservation costs, shipping and handling costs as well as certain one-off items amounted to CHF 18.9 million (2018: CHF 20.3 million).

In 2019, the operating loss amounted to CHF 17.2 million (2018: CHF 24.1 million). Net loss in 2019 was significantly reduced to CHF 22.4 million (2018: CHF 31.4 million), resulting in a basic and diluted loss per share of CHF 2.08 (2018: CHF 2.89).

Net cash consumption from operating activities in 2019 was reduced by 19% to CHF 63.8 million as compared to CHF 79.2 million in 2018. This is a result, on the one hand of the significant increase in cash inflow from Cresemba and Zevtera revenue and, on the other hand, of Basilea's continued focus on managing its operating expenses by continuously optimizing its preclinical and clinical portfolio and targeting its investments into its R&D pipeline. Combined cash and investments amounted to CHF 161.0 million as of December 31, 2019, compared to CHF 223.9 million as of December 31, 2018.

Key financial figures

(In CHF million, except per share data)	**2019**	**2018**
Product revenue	50.9	26.2
Contract revenue[1]	63.5	79.7
Revenue from R&D services	0.3	0.2
Other revenue	19.7	26.5
Total revenue	**134.4**	**132.6**
Cost of products sold	(18.9)	(20.3)
Research & development expenses, net	(102.7)	(104.9)
Selling, general & administrative expenses	(30.0)	(31.4)
Total cost and operating expenses	**(151.6)**	**(156.7)**
Operating loss	**(17.2)**	**(24.1)**
Net loss	**(22.4)**	**(31.4)**
Net cash used in operating activities	(63.8)	(79.2)
Basic and diluted loss per share, in CHF	(2.08)	(2.89)

(In CHF million)	**Dec 31, 2019**	**Dec 31, 2018**
Cash and investments	161.0	223.9

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

[1] Contract revenue in 2019 include none (2018: CHF 23.9 million) related to Toctino® deferred revenue recognition.

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

2020 Outlook

For 2020 Basilea will focus on:

- Through its commercialization partners, continuing to grow significantly in-market sales of Cresemba and Zevtera.
- Advancing its clinical pipeline towards the next value inflection points:
  - Presenting topline results from the registrational phase 2 study with derazantinib in intrahepatic cholangiocarcinoma, in addition to the first interim data of the phase 1/2 study in urothelial cancer.
  - Starting a new phase 1/2 study with derazantinib in gastric cancer.
  - Starting a targeted, biomarker-driven phase 2 study with the oral formulation of lisavanbulin in recurrent glioblastoma.
  - 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 preclinical oncology portfolio through both in-licensing and internal development.

Considering these key priorities, Basilea provides the following financial guidance for 2020:

- Revenue contributions from Cresemba and Zevtera, excluding deferred revenue recognized for payments received in prior years, are expected to grow to CHF 77-87 million (+12% to +27% y-o-y), reflecting the continued significant growth of in-market sales by our partners.
- Total R&D and SG&A expenses are expected to remain at approximately the same level as 2019 but costs of products sold are expected to increase based on higher product deliveries to partners, leading to an anticipated operating loss of CHF 20-30 million.
- Net cash consumption is expected to further decrease as compared to 2019, resulting in anticipated cash and investments of CHF 100-110 million at year-end 2020.
- Deferred revenue recognition relating to one-time upfront, development and regulatory milestones received in prior years is expected to decrease to CHF 33 million (2019: CHF 45.6 million).
- Total revenue is expected to amount to CHF 128-138 million, with reimbursements by BARDA decreasing in line with lower R&D expenses related to the ceftobiprole phase 3 program as compared to 2019.

(In CHF million)	**2020E**	**2019**
Cresemba & Zevtera revenue (deferred)	33	45.6
Cresemba & Zevtera revenue (non-deferred)	77 – 87	68.7
Other (mostly BARDA reimbursements)	18	20.0
Total revenue	128 – 138	134.4
Cost of products sold	25	18.9
R&D and SG&A	133	132.7
Operating loss	20 – 30	17.2
Cash and investments (year-end)	100 – 110	161

Portfolio – Important progress in clinical pipeline and accelerated commercialization of marketed brands throughout 2019

In-market sales of our two marketed brands, Cresemba and Zevtera, continued to increase significantly

In 2019, Basilea's partners doubled the number of countries in which Cresemba is launched. To date, Cresemba is launched in more than 40 countries. According to the latest available public data, global in-market sales of Cresemba reached approximately USD 190 million in the 12 months to the end of September 2019.[1] Zevtera is currently launched in 18 countries. Zevtera sales continued to increase and sales growth is expected to further accelerate through the increasing contributions from the newly launched countries inside and outside of Europe.

Expanding derazantinib data package

In August 2019, we started a phase 1/2 study, FIDES-02, to explore the FGFR kinase inhibitor, derazantinib, in patients with advanced urothelial cancer with FGFR genetic aberrations, alone and in combination with Roche's atezolizumab (Tecentriq®), a PD-L1 checkpoint inhibitor.[2] As announced early in 2020, we are also planning a biomarker-driven multi-cohort phase 1/2 study, FIDES-03, in advanced gastric cancer patients with FGFR genetic aberrations, which is expected to start in the third quarter of 2020. This study, too, will assess derazantinib alone and in combination therapy with atezolizumab. The rationale for the combination is based on the activity of derazantinib to inhibit the colony-stimulating factor-1-receptor (CSF1R), which differentiates derazantinib from other FGFR kinase inhibitors in clinical development. CSF1R kinase inhibition has the potential to enhance the response to atezolizumab's immune-checkpoint inhibition. Roche is providing clinical supply of atezolizumab for the FIDES-02 and FIDES-03 studies. Following the reporting of positive interim results from FIDES-01, a registrational phase 2 study with derazantinib in patients with intrahepatic cholangiocarcinoma (iCCA) and FGFR2 gene fusions, an additional cohort was opened in patients with FGFR2 gene mutations and amplifications in order to further profile derazantinib in this indication.[3]

Tumor checkpoint controller lisavanbulin shows clinical efficacy in glioblastoma

In 2019, we concluded patient enrolment into two phase 1/2 studies with lisavanbulin (formerly BAL101553) with daily oral dosing and weekly 48-hour i.v. infusion, respectively.[4, 5] We observed clinical activity in glioblastoma in both studies, including two patients with profound clinical responses with more than 80% reduction of the tumor area. We decided to advance the development of the oral formulation to a targeted, biomarker-driven, phase 2 study in recurrent glioblastoma and potentially additional tumor types. This study is anticipated to start in glioblastoma patients mid-2020. In addition, a separate phase 1 study with daily oral dosing of lisavanbulin in combination with radiotherapy in patients with newly diagnosed glioblastoma is ongoing.[6] 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.

Positive topline results from phase 3 study with ceftobiprole (Zevtera®) support U.S. strategy

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).[7] Ceftobiprole met primary and secondary efficacy endpoints and was well tolerated with the overall rates of drug-related adverse events being similar between ceftobiprole and the control group. For a future regulatory filing in the U.S., positive results from a second phase 3 study, ERADICATE, would also be required.[8] 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.

Conference call and webcast

Basilea Pharmaceutica Ltd. will host a conference call and webcast today, Tuesday, February 18, 2020, at 4 p.m. (CET), 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 infectious diseases. 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, such as "believe", "assume", "expect", "forecast", "project", "may", "could", "might", "will" or similar expressions concerning Basilea Pharmaceutica Ltd. and its business, including with respect to the progress, timing and completion of research, development and clinical studies for product candidates. 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 IQVIA, September 2019. In-market sales reported as moving annual total (MAT) in U.S. Dollar corrected for currency fluctuations.

2 ClinicalTrials.gov identifier: NCT04045613. Tecentriq® is a registered trademark of Hoffmann-La Roche Ltd.

3 ClinicalTrials.gov identifier: NCT03230318

4 ClinicalTrials.gov identifier: NCT02490800 (glioblastoma, daily oral dosing)

5 ClinicalTrials.gov identifier: NCT02895360 (glioblastoma, ovarian cancer; weekly 48-hour i.v. infusion)

6 ClinicalTrials.gov identifier: NCT03250299

7 ClinicalTrials.gov identifier: NCT03137173

8 ClinicalTrials.gov identifier: NCT03138733

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