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

Press release

Basilea reports strong financial results for half-year 2020, progress in R&D portfolio and successful completion of strategic projects, despite COVID-19

- **Financial results reflecting strong Cresemba and Zevtera performance and continued focus on expense management**
- **Operating profit of CHF 12.8 million and net profit of CHF 9.9 million, including positive one-time effect from the sale of headquarters property**
- **Solid cash and investments of approximately CHF 145 million, excluding cash proceeds from July bond transactions for improving debt maturity profile**
- **No material COVID-19-related impact on operating performance and key studies for derazantinib and lisavanbulin and limited delay in phase 3 study with Zevtera**
- **FY 2020 guidance on revenues and operating expenses confirmed; strong year-end cash and investments position expected**

Basel, Switzerland, August 11, 2020

Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its financial results for the first six months ended June 30, 2020.

David Veitch, Chief Executive Officer, said: “We have achieved significant milestones in a highly volatile and challenging environment in the first half year of 2020. Despite the COVID-19 pandemic, the global in-market sales of our anti-infective brands, Cresemba and Zevtera, continued to show strong growth. We have also seen no material COVID-19-related impact on the key studies for our most advanced oncology drug candidates, derazantinib and lisavanbulin, and expect as previously announced, only a limited delay of potentially up to one quarter in the patient enrolment timelines for our phase 3 study with Zevtera in patients with *Staphylococcus aureus* bacteremia. Importantly, the U.S. FDA approved the extension to the maximum treatment duration in the study from four to up to six weeks, which will be beneficial for positioning, should the drug be approved, as it would allow for the treatment of more difficult-to-treat infections.”

He added: “We have also taken a major step forward in the optimization of our debt maturity profile, by the successful placement of new senior convertible bonds of approximately CHF 97 million and the repurchase of approximately CHF 47 million of our outstanding convertible bonds. Through this, we extended in an initial step the maturity of about 25 percent of our mid-term debt to 2027. In addition, we sold our headquarters property in preparation to moving to a new location in the Basel area in 2022. Based on both a strong underlying financial performance and the one-time effect from this sale, we are pleased to report an operating profit

of approximately CHF 13 million and a net profit of almost CHF 10 million for the first half-year 2020."

He continued: "We are looking forward to a number of important milestones in our oncology portfolio in the second half of the year and beyond. Following the recently announced completion of patient enrolment into the first cohort of the FIDES-01 derazantinib study in patients with bile duct cancer and FGFR2 gene fusions, we are expecting to present topline results in the second half of 2020. Interim results from patients with other FGFR2 genetic aberrations, who are included in the second cohort of this study, are also anticipated in the second half of the year. We are on track with patient enrolment in the urothelial cancer, FIDES-02 derazantinib study and are planning to start the FIDES-03 derazantinib study, in gastric cancer, in the third quarter of 2020. In addition, we are preparing the start of the phase 2 study with our tumor checkpoint controller, lisavanbulin, in patients with brain cancer within the next few months."

Financial summary

Total revenue in the first half-year 2020 increased to CHF 69.3 million (H1 2019: CHF 63.2 million). Revenue contributions from the two marketed brands Cresemba and Zevtera increased by 17.2% to CHF 62.0 million (H1 2019: CHF 52.9 million), of which CHF 36.5 million (H1 2019: CHF 30.1 million) relate to non-deferred revenue and CHF 25.5 million (H1 2019: CHF 22.8 million) to recognition of deferred revenue. Deferred revenue is recognized for upfront, development and regulatory milestone payments received in prior years from partners. Other revenue amounted to CHF 7.2 million (H1 2019: CHF 10.1 million). This included CHF 6.6 million BARDA reimbursements (H1 2019: CHF 9.9 million), which are offsetting a substantial portion of the ceftobiprole phase 3 development expenses.[1] The BARDA reimbursements year-on-year decreased in line with the reduced development expenses after the successful completion of the ceftobiprole phase 3 skin infection study and due to the COVID-19 related slower enrolment into the bacteremia study.

In the first half-year 2020, investments in Basilea's pipeline resulted in research and development expenses of CHF 43.9 million (H1 2019: CHF 50.8 million). Such expenses were mainly driven by costs for the phase 3 program for ceftobiprole, the costs related to the ongoing preclinical and clinical program for derazantinib and the ongoing pediatric programs for ceftobiprole and isavuconazole.

Selling, general and administrative expenses decreased to CHF 14.4 million (H1 2019: CHF 16.2 million). As a result of increasing sales of products to partners, cost of products sold increased to CHF 13.1 million (H1 2019: CHF 9.4 million).

In the first half-year of 2020, the operating profit amounted to CHF 12.8 million (H1 2019: operating loss of CHF 13.2 million), leading to a net profit of CHF 9.9 million (H1 2019: net loss

CHF 15.4 million), which resulted in a basic earnings and diluted earnings per share of CHF 0.92 and CHF 0.91, respectively (H1 2019: basic and diluted loss per share CHF 1.44).

Net cash consumption from operating activities (excluding cash inflow from the sale of the headquarters property) in the first half-year 2020 was reduced by 27% to CHF 33.2 million as compared to CHF 45.4 million in H1 2019. This is a result, on the one hand of the significant increase in cash inflow based on Cresemba and Zevtera performance and, on the other hand, of Basilea's continued focus on managing its operating expenses by continuously optimizing its investments into its preclinical and clinical portfolio. Combined cash and short- and long-term investments amounted to CHF 144.7 million as of June 30, 2020, compared to CHF 161.0 million as of December 31, 2019.

Key financial figures

(In CHF million, except per share data)	**H1 2020**	**H1 2019**
Product revenue	30.5	25.4
Contract revenue	31.5	27.6
Revenue from R&D services	0.2	0.1
Other revenue	7.1	10.1
Total revenue	**69.3**	**63.2**
Cost of products sold	(13.1)	(9.4)
Research & development expenses, net	(43.9)	(50.8)
Selling, general & administrative expenses	(14.5)	(16.2)
Total cost and operating expenses	**(71.5)**	**(76.4)**
Profit from sale of assets	15.0	-
Operating profit/(loss)	**12.8**	**(13.2)**
Net profit/(loss)	**9.9**	**(15.4)**
Net cash used in operating activities	(33.1)	(45.4)
Basic earnings/(loss) per share, in CHF	0.92	(1.44)
Diluted earnings/(loss) per share, in CHF	0.91	(1.44)

(In CHF million)	**June 30, 2020**	**Dec. 31, 2019**
Cash and short- and long-term investments	144.7	161.0

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

The unaudited, condensed consolidated interim financial statements of Basilea Pharmaceutica Ltd. for the first half-year 2020 can be found on the Company's website at https://www.basilea.com/financial-reports.

FY 2020 financial guidance

Basilea provides the following updated financial guidance for full-year 2020 based on an assumed average USD/CHF exchange rate of approximately 0.94 in H2 2020 and a gradual easing of COVID-19 lock-down measures over the remainder of 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 approximately stable. Cost of products sold is expected to increase based on higher product deliveries to partners. An anticipated extension of the supply period to Pfizer is expected to positively affect revenue and increase cost of products sold, primarily in 2021.
- The anticipated operating loss amounts to CHF 5-15 million.
- Cash and short- and long-term investments are expected to be approximately CHF 150 million at year-end 2020. This represents a significant increase compared to the initial guidance, due to the proceeds from the headquarters property sale and the convertible bond transactions and being partially offset by a temporary increase in working capital, which is anticipated to be reversed in early-2021.

(In CHF million)	FY 2020e (updated)	FY 2020e (initial)	FY 2019a
Cresemba & Zevtera revenue (non-deferred)	77-87	77-87	68.7
Cresemba & Zevtera revenue (deferred)	33	33	45.6
Other (mostly BARDA reimbursements)	17-18	18	20.0
Total revenue	128-138	128-138	134.4
Cost of products sold	23	25	18.9
R&D and SG&A	135	133	132.7
Profit from sale of assets	15	0	0
Operating loss	5-15	20-30	17.2
Cash and short- and long-term investments (year-end)	150	100-110	161.0

Portfolio – Continued commercial success of our anti-infective brands and important upcoming milestones for our oncology drug candidates

Cresemba in-market sales continue to grow

Cresemba has reached USD 220 million “in-market sales” for the 12 months to the end of March 2020, which is a more than 30% growth year-on-year.[2] Our partners have reported a number of additional approvals and launches in territories around the world since the beginning of the year. This includes launches in key markets in Asia Pacific and approval in Russia. These

triggered commercial and regulatory milestone payments of approximately CHF 6 million from Pfizer to Basilea. To date, Cresemba has been launched in 45 countries and marketing application processes have been initiated in a number of additional countries, including in China, where the Marketing Authorization Application for mucormycosis was recently accepted for regulatory review by the health authority. We therefore remain confident that our partners will have launched Cresemba in 60 countries by the end of 2021.

Zevtera (ceftobiprole) SAB phase 3 study now expanded to enrol more difficult-to-treat patients

The U.S. Food and Drug Administration (FDA) approved the amendment of the protocol for the phase 3 study ERADICATE, which is conducted in patients with *Staphylococcus aureus* bacteremia (SAB).[3] This amendment extends the maximum treatment duration from four, to now up to six weeks, which allows for the inclusion of patients with more difficult-to-treat infections, including those with complications such as osteomyelitis and epidural or cerebral abscess. We expect that the recruitment of the ERADICATE study can be completed in H2 2021. If the study is positive, as reported for the TARGET phase 3 study last year, which evaluated ceftobiprole in the treatment of patients with acute bacterial skin and skin structure infections, Basilea plans to submit a New Drug Application (NDA) to the FDA.[4] As ceftobiprole was designated a Qualified Infectious Disease Product (QIDP) by the FDA for these indications, if approved, ceftobiprole will be eligible to receive ten years of market exclusivity in the U.S. from the date of approval.

Key oncology clinical studies with derazantinib and lisavanbulin remain on track

In July, we announced that the patient enrolment into the first cohort of the FIDES-01 study with the FRGR kinase inhibitor, derazantinib, has been completed.[5] This first cohort enroled patients with advanced intrahepatic cholangiocarcinoma (iCCA), a type of bile duct cancer, whose tumors expressed fusions of the FGFR2 gene, a genetic aberration which has previously been identified as driving this type of cancer. A second cohort, which enrols iCCA patients with other FGFR2 genetic aberrations, namely mutations and amplifications, is ongoing with interim results expected during H2 2020. The results from the second cohort will support defining the full therapeutic potential of derazantinib in iCCA and potentially further strengthen the differentiation of derazantinib from other FGFR kinase inhibitors. In addition, the clinical development program for derazantinib includes two further Phase 1b/2 studies, the ongoing FIDES-02 in patients with urothelial cancer and the planned FIDES-03, in patients with advanced gastric cancer, which is expected to start in Q3 2020.[6] In FIDES-02 and FIDES-03, we are not only exploring derazantinib as a single agent, but also in combination with an immuno-oncology drug, atezolizumab (Tecentriq®), with the rationale that derazantinib may enhance the response to immuno-oncology treatment.[7]

In 2019, initial results of a phase 1 study with daily oral lisavanbulin in patients with glioblastoma multiforme (GBM), the most common type of primary brain cancer and one of the

most lethal types of cancer, have been reported.[8] These results showed clinical activity of lisavanbulin in GBM with one exceptional, long-lasting responder, whose brain tumor tissue displayed strong expression of the protein EB1. Full results of the study as well as data on the potential utility of EB1 as a response-predictive biomarker for lisavanbulin have been accepted for presentation at this year's annual congress of the European Society of Medical Oncology (ESMO) which is taking place in a virtual format in September 2020. Based on the results of this and other studies, we have decided to progress lisavanbulin to the next clinical stage and are preparing a biomarker-driven phase 2 study with lisavanbulin in patients with GBM. The start of the study is anticipated in the next few months. We expect interim results for this study in H1 2021 and top line results in H2 2021. This approach allows us to efficiently test our hypothesis that lisavanbulin can be developed in a targeted patient population, initially in GBM and then potentially in other tumor types.

Conference call and webcast

Basilea Pharmaceutica Ltd. will host a conference call and webcast today, Tuesday, August 11, 2020, 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 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

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For further information, please contact:

Peer Nils Schröder, PhD

Head of Corporate Communications & Investor Relations

Phone +41 61 606 1102

E-mail media_relations@basilea.com
investor_relations@basilea.com

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

References

1. 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.
2. IQVIA, March 2020. In-market sales reported as moving annual total (MAT) in U.S. Dollar corrected for currency fluctuations.
3. ERADICATE study: Clinicaltrials.gov identifier NCT03138733
4. TARGET study: Clinicaltrials.gov identifier NCT03137173
5. FIDES-01 study: Clinicaltrials.gov identifier NCT03230318
6. FIDES-02 study: Clinicaltrials.gov identifier NCT04045613
7. Tecentriq® is a registered trademark of Hoffmann-La Roche Ltd.
8. Clinicaltrials.gov identifier NCT02490800