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Press release

Basilea reports strong 2020 financial results and significant progress in the clinical pipeline of its oncology assets

- **Double-digit growth in Cresemba and Zevtera non-deferred revenue contributions**
- **Operating loss reduced by over 50% to CHF 8.2 million**
- **Improved operating cash flow by 15%, CHF 167 million year-end cash and financial investments**
- **Established clinical proof of concept for derazantinib as monotherapy in first cancer indication**
- **2021 guidance: expecting 38% - 51% growth in Cresemba and Zevtera non-deferred revenue contributions, stable expenses and 2021 year-end cash position of CHF 110 - 120 million**

Basel, Switzerland, February 16, 2021

Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today its financial results for the financial year ended December 31, 2020.

David Veitch, Chief Executive Officer, said: “Despite the coronavirus pandemic, we have remained fully operational and have achieved significant milestones throughout 2020, both in the commercialization of our two marketed brands, Cresemba and Zevtera, as well as in the development of our most advanced oncology assets, derazantinib and lisavanbulin, for which we started two new clinical studies.”

Adesh Kaul, Chief Financial Officer, added: “We have once again shown a strong financial performance with improving operating cash flow based on a more than 13% increase in Cresemba and Zevtera non-deferred revenue contributions and diligent management of our operating expenses. We have also successfully completed two strategic financial transactions in 2020, which have further increased our cash position and allowed us to improve our debt maturity profile. Overall, this provides us with sufficient financial flexibility to achieve the value-creating milestones we have set ourselves for the next 24 months.”

Financial summary

Total revenue in 2020 decreased to CHF 127.6 million (2019: CHF 134.4 million), mainly due to the already anticipated decrease in the recognition of deferred revenue to CHF 33.8 million (2019: CHF 45.6 million) related to upfront, development and regulatory milestone payments received in prior years from partners. Non-deferred revenue contribution from the two marketed brands Cresemba and Zevtera increased by more than 13% to CHF 78.2 million (2019: CHF 68.7 million). Other revenue amounted to CHF 15.2 million (2019: CHF 19.7 million). This included CHF 13.2 million BARDA reimbursements (2019: CHF 18.5 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 following the successful completion of the ceftobiprole phase 3 skin infection study and due to the impact of the COVID-19 pandemic on the bacteremia study.

In 2020, investments in the pipeline resulted in research and development expenses of CHF 97.4 million (2019: CHF 102.7 million). Such expenses were mainly driven by costs for the phase 3 program for ceftobiprole, the costs related to the ongoing preclinical and clinical programs for derazantinib and lisavanbulin, as well as to the ongoing pediatric programs for ceftobiprole and isavuconazole.

Selling, general and administrative expenses amounted to CHF 29.4 million (2019: CHF 30.0 million). As a result of increasing sales of products to partners, cost of products sold increased to CHF 24.1 million (2019: CHF 18.9 million).

In 2020, the operating loss was reduced by 52% year-on-year to CHF 8.2 million (2019: CHF 17.2 million) reflecting a CHF 15.0 million (2019: none) positive one-off effect from the sale of the headquarters property. Net loss was also reduced significantly to CHF 14.7 million (2019: CHF 22.4 million), resulting in a basic and diluted loss per share of CHF 1.43 (2019: CHF 2.08).

Net cash used in operating activities in 2020 was reduced by 15% to CHF 54.1 million as compared to CHF 63.8 million in 2019. This is a result, on the one hand of the significant increase in cash inflow, based on the growth of Cresemba and Zevtera non-deferred revenue contributions and on the other hand, of the continued focus on managing operating expenses, by continuously optimizing investments into the preclinical and clinical portfolio. Cash and investments amounted to CHF 167.3 million as of December 31, 2020, compared to CHF 161.0 million as of December 31, 2019. The year-on-year increase is due to the proceeds from the headquarters property sale and the convertible bond transactions.

Key financial figures

(in CHF million, except per share data)	2020	2019
Product revenue	48.7	50.9
Contract revenue	63.3	63.5
Revenue from R&D services	0.4	0.3
Other revenue	15.2	19.7
Total revenue	**127.6**	**134.4**
Cost of products sold	(24.1)	(18.9)
Research & development expenses, net	(97.4)	(102.7)
Selling, general & administrative expenses	(29.4)	(30.0)
Total cost and operating expenses	**(150.9)**	**(151.6)**
Profit from sale of assets	15.0	-
Operating loss	**(8.2)**	**(17.2)**
Net loss	**(14.7)**	**(22.4)**
Net cash used in operating activities	(54.1)	(63.8)
Basic and diluted loss per share, in CHF	(1.43)	(2.08)

(In CHF million)	Dec 31, 2020	Dec. 31, 2019
Cash and investments	167.3	161.0

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

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

FY 2021 financial guidance

Basilea provides the following financial guidance for full-year 2021:

- Non-deferred revenue contributions from Cresemba and Zevtera, excluding deferred revenue recognized for payments received in prior years, are expected to grow to CHF 108 - 118 million (+38% to +51% y-o-y), reflecting the continued significant growth of in-market sales by our partners and several potential milestone events.
- 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.
- The anticipated operating loss amounts to CHF 13 - 23 million, which is below the operating loss reported for 2020, excluding the one-off positive impact from the sale of the headquarters property.
- Cash and investments are expected to be approximately CHF 110 - 120 million at year-end 2021, excluding any potential impact from a reduction of the outstanding convertible bonds.

(in CHF million)	FY 2021e	FY 2020a
Cresemba & Zevtera revenue (non-deferred)	108 - 118	78.2
Cresemba & Zevtera revenue (deferred)	2.5	33.8
Total revenue	128 - 138	127.6
Total cost and operating expenses	149 - 154	150.9
Profit from sale of assets	0	15.0
Operating loss	13 - 23	8.2
Cash and investments (year-end)	110 - 120	167.3

Portfolio – Significant progress made in the commercialization of Cresemba and Zevtera and the clinical programs of our oncology drug candidates

Cresemba launched in important additional markets and "in-market" sales continue to grow

Cresemba has reached USD 244 million "in-market sales" for the 12 months to the end of September 2020, which is a more than 28% growth year-on-year.[2] Based on the additional approvals and launches in territories around the world during 2020, we expect that global "in market" sales for the full year 2020 will have exceeded USD 250 million. The achievements in 2020 include launches in key markets in Asia Pacific and the approval in Russia. These triggered commercial and regulatory milestone payments of approximately CHF 6 million by Pfizer in 2020. In addition, in January 2021, Pfizer's Cresemba sales in their territory passed the threshold that triggered a milestone payment of USD 10 million to Basilea.
To date, Cresemba has been launched in about 50 countries and marketing application processes have been initiated in a number of additional countries, including in China, where the Marketing Authorization Applications for invasive aspergillosis and mucormycosis have been accepted for regulatory review by the health authority. Adding to this, in early January 2021, our partner Asahi Kasei completed patient enrolment in a phase 3 study required for a marketing authorization application in Japan. We therefore remain confident that our partners will have launched Cresemba in 60 countries by the end of 2021.

Preparing for market access of Zevtera (ceftobiprole) in China and advancing phase 3 program for the U.S.

In 2020, our partner CR Gosun obtained regulatory approval for Zevtera in China, which triggered a milestone payment of CHF 3 million to Basilea. Zevtera was approved for the treatment of community- and hospital-acquired pneumonia, and we are currently supporting CR Gosun to prepare for launching the brand in this important market. From a commercial perspective, the most important market for Zevtera is the U.S. market.
In the ongoing phase 3 study called ERADICATE, the U.S. Food and Drug Administration (FDA) in 2020 approved a protocol amendment extending the maximum treatment duration from four to now up to six weeks.[3] This is important, because it 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 patient enrolment into the study will be completed by year-end 2021. If ERADICATE is positive, as was the TARGET phase 3 study in 2019 that evaluated ceftobiprole in the treatment of patients with acute bacterial skin and skin structure infections, we plan 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 two indications, if approved, ceftobiprole will be eligible to receive ten years of market exclusivity in the U.S. from the date of approval.

Five clinical studies ongoing with oncology drug candidates derazantinib and lisavanbulin with many data readouts ahead

We are currently conducting three clinical studies, FIDES-01, -02 and -03, with our most advanced oncology drug candidate, the FGFR inhibitor derazantinib.[5, 6, 7, 8] Positive topline results from the first cohort of the phase 2 study FIDES-01 have just been reported. This first cohort explores derazantinib in patients with FGFR2 gene-fusion positive advanced intrahepatic cholangiocarcinoma (iCCA), a type of bile duct cancer. The topline results are consistent with the interim results reported in 2019 and provide the proof of concept for derazantinib in iCCA as its first indication.

In a second cohort, we are enrolling iCCA patients with other FGFR2 gene aberrations, namely mutations and amplifications. In October 2020, we presented pooled efficacy data from different patient cohorts, at the ESMO MAP Virtual Congress, that demonstrated derazantinib also has antitumor activity in this patient population. If confirmed, these findings may strengthen the clinical evidence on the differentiated profile of derazantinib versus other FGFR inhibitors. We are therefore looking forward to interim results from this second cohort of FIDES-01, which are expected for H1 2021.

The other two FIDES studies, FIDES-02 in urothelial (bladder) cancer and FIDES-03 in gastric (stomach) cancer, which were started in 2019 and 2020, respectively, are phase 1/2 studies focused on exploring derazantinib as a single agent, as well as in combination with other anticancer drugs. In FIDES-02, the combination partner is Roche's immuno-oncology drug atezolizumab, for which we entered into a clinical supply agreement in 2019. This agreement was extended in 2020 to FIDES-03. In addition, we have entered into a clinical trial collaboration and supply agreement with Eli Lilly and Company in 2020 for their anti-angiogenic drug ramucirumab to be explored as combination partner in FIDES-03. Interim results from FIDES-02 monotherapy are expected to become available in H1 2021.

In 2020, we were able to establish the recommended phase 2 dose for the derazantinib/atezolizumab combination and presented these data in February 2021 at the ASCO Genitourinary Cancers Symposium. Interim results for the derazantinib/atezolizumab combination in patients with urothelial cancer are expected to become available in H2 2021. In FIDES-03, interim results for derazantinib monotherapy and the definition of the recommended phase 2 dose for the combination with ramucirumab (plus another anticancer drug, paclitaxel) are anticipated for H2 2021.

Dr. Marc Engelhardt, Chief Medical Officer, said: “We are excited to have established the clinical proof of concept for derazantinib as monotherapy in its first indication in early 2021. We expect the publication of a number of interim and topline results across the entire FIDES clinical program throughout 2021 and 2022. Based on its unique kinase inhibition profile, derazantinib has potential for enhanced activity in combination therapy. We are therefore particularly interested to see the first efficacy data on the combination of derazantinib with other anti-cancer agents in our urothelial and gastric cancer studies, which may allow us to strengthen the evidence for its differentiation versus other FGFR inhibitors, both from the efficacy and safety perspective.”

Two studies are currently ongoing with our second oncology drug candidate, lisavanbulin. Both are exploring orally administered lisavanbulin in patients with glioblastoma, the most common type of primary brain cancer and one of the most lethal types of cancer. In 2020, we started a phase 2 study in recurrent glioblastoma, using EB1 (end-binding protein 1) for patient selection.[9] This is based on the observation of an exceptional long-lasting responder in the phase 1 part of the study, whose tumor was strongly EB1-positive. If we could validate EB1 as a response-predictive biomarker in patients, this would allow a very targeted development of lisavanbulin. Interim results from this study are expected in H2 2021. In parallel, a phase 1 study is exploring lisavanbulin in combination with radiotherapy in patients with newly diagnosed glioblastoma. This study is conducted in collaboration with the Adult Brain Tumor Consortium (ABTC) in the U.S. and we expect that the recommended phase 2 dose for lisavanbulin in this patient population can be determined also in H2 2021.

Conference call and webcast

Basilea Pharmaceutica Ltd. will host a conference call and webcast today, Tuesday, February 16, 2021, 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 50 00 (Europe and RoW)
+1 (1) 866 291 41 66 (USA)
+44 (0) 207 107 06 13 (U.K.)

Replay

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

About Basilea

Basilea is a commercial-stage biopharmaceutical company founded in 2000 and headquartered in Switzerland. We are committed to discovering, developing and commercializing innovative drugs to meet the medical needs of patients with cancer and infectious diseases. We have successfully launched two hospital brands, Cresemba for the treatment of invasive fungal infections and Zevtera for the treatment of severe bacterial infections. We are conducting clinical studies with two targeted drug candidates for the treatment of a range of cancers and have a number of preclinical assets in both cancer and infectious diseases in our portfolio. Basilea is listed on the SIX Swiss Exchange (SIX: BSLN). Please visit 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. The ceftobiprole phase 3 program is funded in part (up to USD 130 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, September 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. Basilea in-licensed derazantinib from ArQule Inc., a wholly-owned subsidiary of Merck & Co., Inc., Kenilworth, N.J., U.S.A.
6. FIDES-01 study: ClinicalTrials.gov identifier NCT03230318
7. FIDES-02 study: ClinicalTrials.gov identifier NCT04045613
8. FIDES-03 study: ClinicalTrials.gov identifier NCT04604132
9. ClinicalTrials.gov identifier NCT02490800