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Ad hoc announcement pursuant to Art. 53 LR

Basilea to become a leading anti-infectives company backed by strong financial results 2021

- **65% year-on-year growth in non-deferred revenue from Cresemba and Zevtera**
- **Operating profit of CHF 1.2 million**
- **Future strategic focus on anti-infectives**
- **Exploring strategic options to maximize the value of oncology assets**
- **Sustainable profitability from FY 2023 expected**

Basel, Switzerland, February 15, 2022

Basilea Pharmaceutica Ltd. (SIX: BSLN), a commercial-stage biopharmaceutical company, announced today its results for the financial year ended December 31, 2021, and strategic decisions to optimize the long-term value of its two business pillars, anti-infectives and oncology.

David Veitch, Chief Executive Officer, stated: “Following a strategic review, we have decided to separate our activities in anti-infectives from oncology. Our two businesses are at different stages of development, requiring different approaches. For our oncology assets, we aim to optimize the value through either portfolio or individual asset transactions, with partners specialized in oncology. We will focus in the future on the research, development and commercialization of innovative treatments for severe bacterial and fungal infections. Basilea is uniquely positioned to benefit from the improving business environment for anti-infectives and to become a leading company in this space, based on its proven expertise in advancing anti-infectives through research and development to the market.”

Adesh Kaul, Chief Financial Officer, said: “We have delivered very strong financial results in 2021. The performance and progress of our anti-infectives business is reflected by the 29% increase in royalty income year-on-year and the more than five-fold increase of regulatory and commercial milestone payments to CHF 49 million in 2021. Also maintaining a focus on our cost structure enabled us to further improve our operating cash flow. Our strategic decision to focus on anti-infectives, will accelerate our path to sustained profitability from 2023 and provides us with the financial flexibility to both invest in our internal pipeline and access external assets.”

In 2022, Basilea will continue its activities in oncology in order to ensure project continuity and progression. For derazantinib, the company will focus on continuing the FIDES-01 study in intrahepatic cholangiocarcinoma (iCCA) and the FIDES-03 study in gastric cancer, but de-prioritize the FIDES-02 program in advanced urothelial cancer. This entails stopping enrolment of patients in the substudies in first-line treatment of cisplatin-ineligible patients and

in the treatment of patients refractory to other FGFR inhibitors. Patient enrolment has been challenging in these substudies, due to the evolving competitive environment in urothelial cancer treatment. For the remaining substudy, in the second-line treatment of patients with advanced urothelial cancer, patient enrolment into the first stage has been completed and patients will be followed-up through to data maturity.

Financial summary

Total revenue in the financial year (FY) 2021 increased 16% to CHF 148.1 million (FY 2020: CHF 127.6 million), reflecting the regulatory and commercial progress made in particular with Cresemba. Non-deferred revenue contributions from Cresemba and Zevtera increased by 64.7% to CHF 128.8 million (FY 2020: CHF 78.2 million). This included royalty income from Cresemba, which increased by 29.1% to CHF 53.2 million (FY 2020: CHF 41.2 million), and upfront and milestone payments of CHF 49.4 million (FY 2020: CHF 9.0 million). Other revenue amounted to CHF 16.6 million (FY 2020: CHF 15.2 million). This included CHF 14.0 million BARDA reimbursements (FY 2020: CHF 13.2 million), which are offsetting a substantial portion of the ceftobiprole phase 3 development expenses.[1]

In 2021, research and development expenses remained stable at CHF 93.2 million (FY 2020: CHF 97.4 million). The expenses mainly included the costs for the phase 3 program for ceftobiprole, the costs related to the ongoing preclinical and clinical programs for derazantinib and lisavanbulin, the ongoing pediatric programs for ceftobiprole and isavuconazole, as well as the completion of the preclinical program for BAL0891.

Selling, general and administrative expenses amounted to CHF 29.7 million (FY 2020: CHF 29.4 million). Cost of products sold remained stable at CHF 24.1 million (FY 2020: CHF 24.1 million).

In 2021, an operating profit of CHF 1.2 million was recorded (FY 2020: operating loss of CHF 8.2 million). The net loss was reduced to CHF 6.8 million (FY 2020: CHF 14.7 million), resulting in a basic and diluted loss per share of CHF 0.58 (FY 2020: CHF 1.43).

Net cash used in operating activities in 2021 was reduced by 40.9% to CHF 32.0 million (FY 2020: CHF 54.1 million). This improvement 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 Basilea’s continued focus on managing operating expenses, by optimizing investments into the R&D portfolio and improving the cost base through strategic transactions. Cash, restricted cash and investments amounted to CHF 150.0 million as of December 31, 2021, compared to CHF 167.3 million as of December 31, 2020. The convertible bond maturing in December 2022 (ISIN CH0305398148) was reduced by CHF 22.7 million in 2021.

Key financial figures

(in CHF million, except per share data)	FY 2021	FY 2020
Product revenue	26.2	48.7
Contract revenue	105.2	63.3
Revenue from R&D services	0.2	0.4
Other revenue	16.6	15.2
Total revenue	**148.1**	**127.6**
Cost of products sold	(24.1)	(24.1)
Research & development expenses, net	(93.2)	(97.4)
Selling, general & administrative expenses	(29.7)	(29.4)
Total cost and operating expenses	**(147.0)**	**(150.9)**
Profit from sale of assets	-	15.0
Operating profit/loss	**1.2**	**(8.2)**
Net loss	**(6.8)**	**(14.7)**
Net cash used in operating activities	(32.0)	(54.1)
Basic loss per share, in CHF	(0.58)	(1.43)
Diluted loss per share, in CHF	(0.58)	(1.43)

(in CHF million)	Dec 31, 2021	Dec. 31, 2020
Cash, restricted cash and investments	150.0	167.3

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 2021 can be found on the Company's website at https://www.basilea.com/financial-reports.

Financial guidance

Based on further increasing revenue from Cresemba and Zevtera and an expected reduction of around 30% in operating expenses in 2023 versus 2022, Basilea expects to reach sustainable profitability and generate positive cash flow from operating activities in 2023.

For 2022, Basilea expects continued strong in-market sales growth of its key brand Cresemba, even accounting for an expected decrease in one-off sales related to COVID-19. The company provides the following financial guidance, which does not take into consideration the potential effect of any strategic transactions related to its oncology business:

– In line with the expected continued strong growth of Cresemba in-market sales, royalty income is expected to increase double-digit to approximately CHF 59 million, reflecting the underlying health of the commercial business.
– Cresemba & Zevtera related revenue is expected to amount to CHF 98 – 104 million. The decrease versus 2021 is only due to lower expected milestone payments from partners. 2022 milestone payments are expected to be more in line with previous years.
– Net cash used in operating activities is expected to improve further to CHF 10 – 15 million.

(in CHF million)	FY 2022e	FY 2021a
Cresemba & Zevtera related revenue	98 to 104	131.4
Royalty income	~59	53.2
Total revenue	106 to 112	148.1
Cost of products sold	21 - 24	24.1
Operating expenses	~110	122.9
Operating loss/profit	-20 to -25	1.2
Net cash used in operating activities	10 to 15	32.0

Refinancing of convertible bond and Board nominations

Adesh Kaul, Chief Financial Officer, said: “At the end of 2022, our convertible bond with the nominal amount of approximately CHF 125 million outstanding, will mature. Our aim is to manage the convertible bond in a way that further reduces our debt level and minimizes dilution, reflecting the confidence that we have in our financial strength in 2023 and beyond. We will be able to make an informed decision on how precisely to manage the upcoming bond maturity once we have more visibility on the strategic transactions on our oncology assets and the outcome of the ceftobiprole ERADICATE phase 3 study. If ERADICATE is positive, this would pave the way to accessing the U.S. market, which would have a significant positive impact on our cash flow starting from 2023. In order to account for different scenarios, we intend to request conditional capital at the upcoming annual shareholder meeting, with the sole purpose of refinancing the outstanding bond, in case it is required.”

The Board of Directors has nominated Domenico Scala (Chairman), Dr. Martin Nicklasson, Dr. Nicole Onetto, Steven D. Skolsky and Dr. Thomas Werner for re-election as Board members. The Board has nominated Leonard Kruimer for election as a new Board member. The Board of Directors will continue to be composed of 6 members.

Portfolio progress 2021

Cresemba (isavuconazole): global in-market sales continue to show double-digit growth – approvals in China and NDA filed in Japan

Cresemba has reached more than USD 300 million global in-market sales for the 12 months to the end of September 2021.[2] This is an increase of 26.5% year-on-year. The excellent commercial performance triggered several milestone payments from our partners with a total of approximately CHF 35 million in 2021.

In China, our partner Pfizer received Drug Approval Licenses from the National Medical Products Administration (NMPA), for the oral formulation of isavuconazole for the treatment of adult patients with invasive aspergillosis and mucormycosis. The first approval in December

2021 triggered a USD 10 million milestone payment to Basilea. Separate marketing authorization applications for the intravenous formulation for the treatment of invasive aspergillosis and mucormycosis are under regulatory review by the Center for Drug Evaluation at the NMPA.

At the end of September 2021, our partner, Asahi Kasei Pharma, filed a New Drug Application (NDA) for the marketing authorization of isavuconazole in Japan for the treatment of the fungal infections: aspergillosis, mucormycosis and cryptococcosis. The filing triggered a CHF 5 million milestone payment to Basilea. A regulatory decision on the NDA is expected in the second half of 2022.

Zevtera (ceftobiprole): ERADICATE phase 3 study on track for topline results mid-2022

In early January 2022, the last patient was enrolled into the phase 3 ERADICATE study, which is investigating ceftobiprole in the treatment of patients with *Staphylococcus aureus* bacteremia (SAB).[3] Topline results are expected to become available around mid-year 2022. If the study is positive, we intend to submit a New Drug Application to the U.S. FDA.

Innovative anti-infectives: received CARB-X grant for development of novel antibiotic

In May 2021, Basilea was awarded a research grant of up to USD 2.7 million from CARB-X (Combating Antibiotic-Resistant Bacteria Biopharmaceutical Accelerator), a global non-profit partnership dedicated to supporting the early development of antibacterial products.[4] The funding supports the development of DXR inhibitors, a novel class of antibiotics targeting drug-resistant Gram-negative bacteria such as carbapenem-resistant Enterobacterales, *Acinetobacter baumannii* and multidrug-resistant *Pseudomonas aeruginosa.*

FGFR inhibitor derazantinib[5]: Clinical proof-of-concept provided for FGFR2 fusion-positive bile duct cancer (iCCA)

Topline results have been reported for cohort 1 (FGFR2 fusion-positive iCCA) of the FIDES-01 phase 2 study, which provided the clinical proof-of-concept for derazantinib monotherapy in this patient population. The results show an objective response rate (ORR) of 21%, a disease control rate (DCR) of 76% and a median progression-free survival (PFS) of 8.0 months.[6] In January 2022, the updated interim results for FIDES-01 cohort 2 (iCCA patients with FGFR2 mutations or amplifications) were reported and show similar clinical benefit as in cohort 1.[7] At the cut-off date of August 31, 2021, the DCR was 74% and the median PFS 7.3 months, which is encouraging, as there has been only limited evidence so far for the successful treatment of this patient group with other FGFR inhibitors. FIDES-01 continues to enroll and topline results for cohort 2 are expected mid-2022.

Ongoing biomarker-driven phase 2 study with lisavanbulin:

Basilea is currently investigating its tumor checkpoint controller lisavanbulin in patients with glioblastoma. A phase 2 study, enrolling patients with recurrent glioblastoma, which have tested

positive for the potential response-predictive biomarker, EB1 (end-binding protein 1), is expected to report interim results in the first half of 2022.[8] In July 2021, the U.S. Food and Drug Administration (FDA) granted lisavanbulin Orphan Drug Designation for the treatment of malignant glioma, which includes glioblastoma.

BAL0891 added to oncology clinical pipeline

In December 2021, the U.S. FDA approved the Investigational New Drug (IND) application for BAL0891, a dual inhibitor of TTK (threonine tyrosine kinase) and PLK1 (polo-like kinase 1) and first-in-class mitotic checkpoint inhibitor (MCI). Preparations are ongoing to enable the start of a phase 1 study in patients with advanced solid tumors mid-2022.

Conference call and webcast

Basilea Pharmaceutica Ltd. will host a conference call and webcast today, Tuesday, February 15, 2022, at 4 p.m. (CET), to discuss the Company's financial and operating results and to provide an outlook.

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 the 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 needs of patients with bacterial and fungal infections and cancer. 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 several preclinical assets in both anti-infectives and cancer 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 ad hoc announcement can be downloaded from www.basilea.com.

References

1. The phase 3 program is funded in part (up to USD 134.2 million, which is approximately 70% of the total potential 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 number HHSO100201600002C.
2. IQVIA, September 2021. In-market sales reported as moving annual total (MAT) in U.S. dollar.
3. ERADICATE: ClinicalTrials.gov identifier NCT03138733
K. Hamed, M. Engelhardt, M. E. Jones et al. Ceftobiprole versus daptomycin in *Staphylococcus aureus* bacteremia: a novel protocol for a double-blind, Phase III trial. Future Microbiology. 2020 (1), 35-48
4. CARB-X's funding for this project is sponsored by Cooperative Agreement Number IDSEP160030 from ASPR/BARDA and by awards from Wellcome Trust and Germany's Federal Ministry of Education and Research. The content is solely the responsibility of the authors and does not necessarily represent the official views of CARB-X or any of its funders.
5. Basilea has in-licensed derazantinib from ArQule Inc., a wholly-owned subsidiary of Merck & Co., Inc., Kenilworth, N.J., U.S.A.
6. FIDES-01: ClinicalTrials.gov identifier: NCT03230318
M. Droz dit Busset, W. L. Shaib, K. Mody et al. Derazantinib for patients with intrahepatic cholangiocarcinoma harboring FGFR2 fusions / re-arrangements: Primary results from the Phase 2 study FIDES-01. Annals of Oncology 2021 (32), supplement 5, S376-S381; https://doi.org/10.1016/j.annonc.2021.08.326 and Basilea data on file

7. M. M. Javle, G. K. Abou-Alfa, T. Macarulla et al. Efficacy of derazantinib in intrahepatic cholangiocarcinoma patients with FGFR2 mutations or amplifications: Interim results from the phase 2 study FIDES-01; Journal of Clinical Oncology 40, no. 4_suppl (February 01, 2022) 427-427
8. ClinicalTrials.gov identifier NCT02490800