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

Press release

Basilea reports preliminary 2020 revenue and portfolio progress

- **Significant progress made in the commercialization of Cresemba and Zevtera with 2020 revenue contributions of CHF 112 million in line with guidance**
- **Advancement in the FIDES clinical program with derazantinib**
- **CHF 167 million year-end cash and financial investments, above guidance**

Basel, Switzerland, January 11, 2021

Basilea Pharmaceutica Ltd. (SIX: BSLN) announced today the unaudited preliminary revenue and year-end cash-position for the financial year 2020 as well as provided an update on its key clinical programs. Total revenue is expected to amount to approx. CHF 128 million (FYR 2019: CHF 134.4 million). Total revenue contributions from Basilea's marketed products, the antifungal Cresemba® (isavuconazole) and the antibiotic Zevtera® (ceftobiprole), are expected to amount to approx. CHF 112 million (FYR 2019: CHF 114.3 million) with non-deferred revenue contributions increasing by 13% to approx. CHF 78 million (FYR 2019: CHF 68.7 million). Basilea also reported preliminary CHF 167 million cash and financial investments at year-end 2020. Basilea had guided for total revenue of CHF 128-138 million with total revenue contributions from Cresemba and Zevtera of CHF 110-120 million and a year-end cash position of CHF 150 million.

David Veitch, Chief Executive Officer, commented: "We are very pleased with the significantly increased non-deferred revenue contributions from Cresemba and Zevtera, which are reflecting the continued progress that we, together with our partners, are making in the commercialization of our brands. We expect that the global in-market sales of Cresemba will have exceeded 250 million US dollars in 2020. Both Cresemba and Zevtera have been approved and launched in new markets throughout 2020, which has triggered multiple milestone payments to Basilea."

He added: "We have also made significant progress in the development of our anti-infectives and oncology clinical assets. Specifically, in our FIDES clinical study program with our FGFR inhibitor, derazantinib, we have not only moved closer towards establishing the clinical proof-of-concept as monotherapy in the first indication, intrahepatic cholangiocarcinoma, but we have also taken an important step in our urothelial cancer study towards exploring derazantinib's therapeutic potential in combination with other anti-cancer therapies such as immune checkpoint inhibitors. The initiation of the gastric cancer study, a potential first-to-market opportunity, underscores the broad potential that we see for the compound."

Adesh Kaul, Chief Financial Officer, added: “The continued double-digit growth in our Cresemba and Zevtera non-deferred revenue is indicative of the robust global in-market performance of our brands and the continued commercialization progress made by our partners. The performance is particularly remarkable considering the continued strength of the Swiss franc and the continued impact of the COVID-19 pandemic in the second half of 2020, which has pushed the expected timing of potential commercial milestone events to 2021. Our strong year-end cash position provides us with the required financial flexibility to execute on our strategic priorities.”

The audited full financial statements as well as the annual report 2020 will be published on February 16, 2021. The final audited revenue for 2020 and the cash position as of year-end 2020 may differ from the preliminary reported numbers.

Oncology pipeline
In 2020, Basilea has made significant progress in the clinical development of its FGFR inhibitor derazantinib[2]:

- Enrolment was completed into the first cohort of the phase 2 study FIDES-01, which explores derazantinib as monotherapy in patients with FGFR2 gene fusion positive intrahepatic cholangiocarcinoma (iCCA).[3] The publication of topline results is now projected to be in Q1 2021 because source data verification is taking longer than previously expected due to the COVID-19 pandemic.
- Data from a pooled analysis presented at the ESMO MAP Virtual Congress 2020 show that derazantinib is also active in iCCA patients with FGFR2 gene mutations and amplifications. Basilea is exploring this patient population in a second cohort of the FIDES-01 study, which is expected to report further interim results in H1 2021. To date there is limited clinical evidence from other FGFR inhibitors in this patient population. Confirmed clinical activity in this patient population would underscore the broad therapeutic potential of derazantinib as a monotherapy in FGFR2-positive iCCA.
- A recommended phase 2 dose for the combination with Roche’s PD-L1 checkpoint inhibitor, atezolizumab, in the phase 1/2 FIDES-02 study in patients with advanced urothelial cancer and FGFR genetic aberrations was established.[4] No dose-limiting toxicities were observed. Both therapies can be combined at standard doses, i.e. the derazantinib monotherapy phase 2 dose used in the FIDES-01 study and the approved standard dose for atezolizumab as a single agent in urothelial cancer. This adds further evidence to the manageable safety and tolerability profile of derazantinib.
- The phase 1/2 study FIDES-03 in patients with advanced gastric cancers and FGFR genetic aberrations was initiated.[5] The study assesses derazantinib as monotherapy and in combination with Lilly’s anti-VEGFR2 antibody ramucirumab and paclitaxel or with atezolizumab.

Dr. Marc Engelhardt, Chief Medical Officer, commented: “We expect the publication of a number of interim and topline results across the entire FIDES clinical program throughout 2021 and 2022. In the near-term, we are looking forward to seeing topline results from the FIDES-01 study in FGFR2 gene fusion positive iCCA. If the data is consistent with the interim results published in 2019, this would provide the clinical proof-of-concept for derazantinib as monotherapy in its first indication and confirm the safety and tolerability profile of the compound. Based on its unique kinase inhibition profile, derazantinib has potential for enhanced activity in combination therapy. We are therefore particularly excited 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.”

For lisavanbulin, Basilea has initiated in 2020 a phase 2 study in patients with advanced glioblastoma, whose tumor tested positive for end-binding protein 1 (EB1), a potential response-predictive biomarker.[6] Interim results from this study are expected in H2 2021.

Anti-infectives pipeline

- In January 2021, Basilea’s partner Asahi Kasei Pharma completed patient enrollment into a phase 3 study with isavuconazole in Japan.[7] Topline results from the study are expected in H2 2021.
- We are working towards the completion of patient enrolment into the ceftobiprole phase 3 study ERADICATE, which is the second and last study necessary for a regulatory filing in the U.S. The ERADICATE study explores ceftobiprole in *Staphylococcus aureus* bacteremia (SAB).[8] Completion of patient enrolment is expected in Q4 2021 and topline results in H1 2022.

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.

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. IQVIA, September 2020. In-market sales reported as moving annual total (MAT) in U.S. dollars corrected for currency fluctuations.
2. Basilea in-licensed derazantinib from ArQule Inc., a wholly-owned subsidiary of Merck & Co., Inc., Kenilworth, N.J., U.S.A.
3. FIDES-01: Clinicaltrials.gov identifier NCT03230318
4. FIDES-02: Clinicaltrials.gov identifier NCT04045613
5. FIDES-03: ClinicalTrials.gov identifier NCT04604132
6. ClinicalTrials.gov identifier NCT02490800
7. Clinicaltrials.gov identifier NCT03471988
8. Clinicaltrials.gov identifier NCT03138733