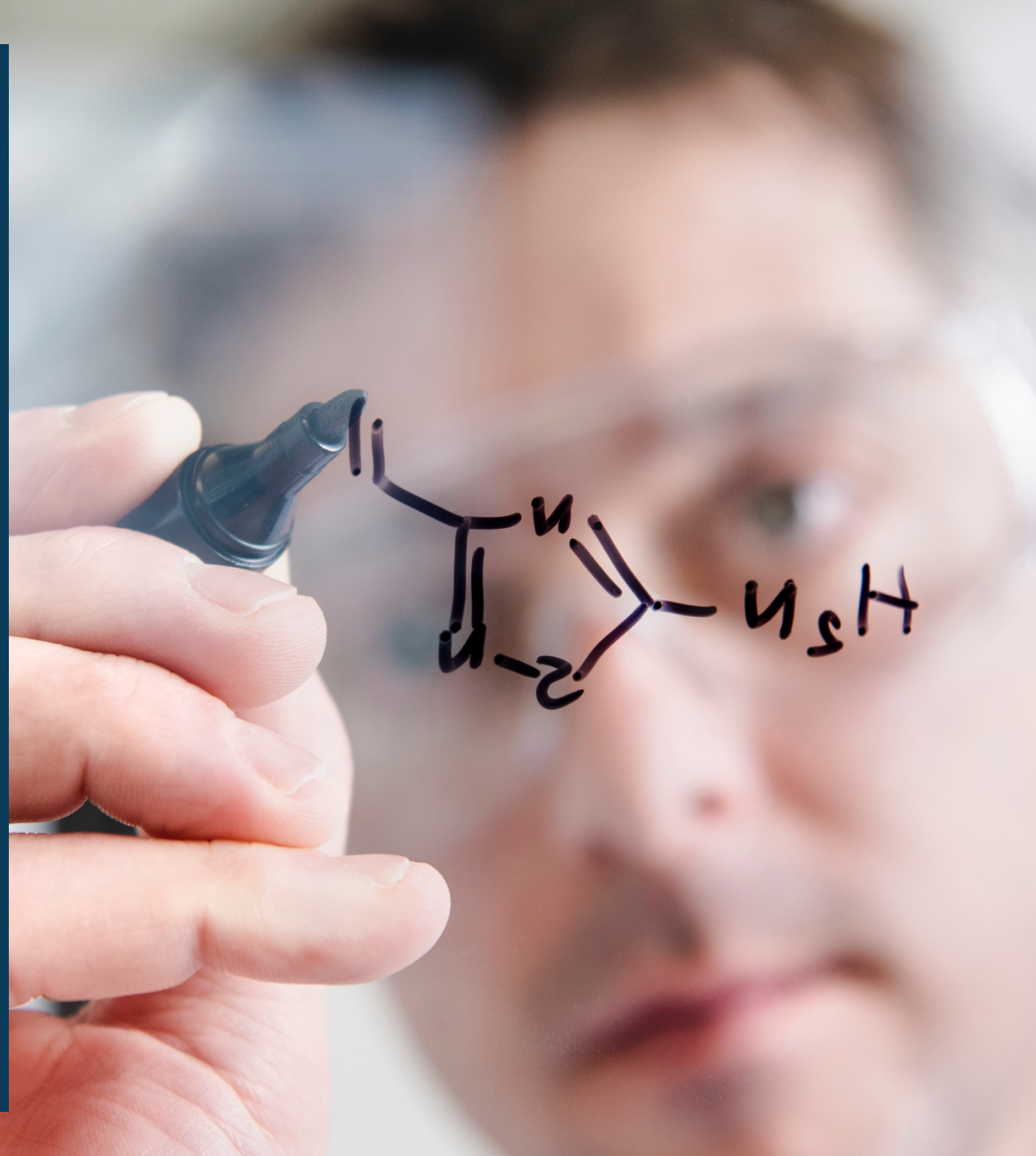
Focus

Full-year results 2020

February 16, 2021

Webcast presentation



David Veitch

Chief Executive Officer

Introduction

Disclaimer and forward-looking statements

This communication, including the accompanying oral presentation, contains certain forward-looking statements, including, without limitation, statements containing the words “believes”, “anticipates”, “expects”, “supposes”, “considers”, and words of similar import, or which can be identified as discussions of strategy, plans or intentions. Such forward-looking statements are based on the current expectations and belief of company management, and are subject to numerous risks and uncertainties, which may cause the actual results, financial condition, performance, or achievements of Basilea, or the industry, to be materially different from any future results, performance, or achievements expressed or implied by such forward-looking statements. Such factors include, among others, the following: the uncertainty of pre-clinical and clinical trials of potential products, limited supplies, future capital needs and the uncertainty of additional funding, compliance with ongoing regulatory obligations and the need for regulatory approval of the company’s operations and potential products, dependence on licenses, patents, and proprietary technology as well as key suppliers and other third parties, including in preclinical and clinical trials, acceptance of Basilea’s products by the market in the event that they obtain regulatory approval, competition from other biotechnology, chemical, and pharmaceutical companies, attraction and retention of skilled employees and dependence on key personnel, and dependence on partners for commercialization of products, limited manufacturing resources, management’s discretion as to the use of proceeds, risks of product liability and limitations on insurance, uncertainties relating to public health care policies, adverse changes in governmental rules and fiscal policies, changes in foreign currency and other factors referenced in this communication. Given these uncertainties, prospective investors are cautioned not to place undue reliance on such forward-looking statements. Basilea disclaims any obligation to update any such forward-looking statements to reflect future events or developments, except as required by applicable law. Derazantinib and its uses are investigational and have not been approved by a regulatory authority for any use. Efficacy and safety have not been established. The information presented should not be construed as a recommendation for use. The relevance of findings in nonclinical/preclinical studies to humans is currently being evaluated.

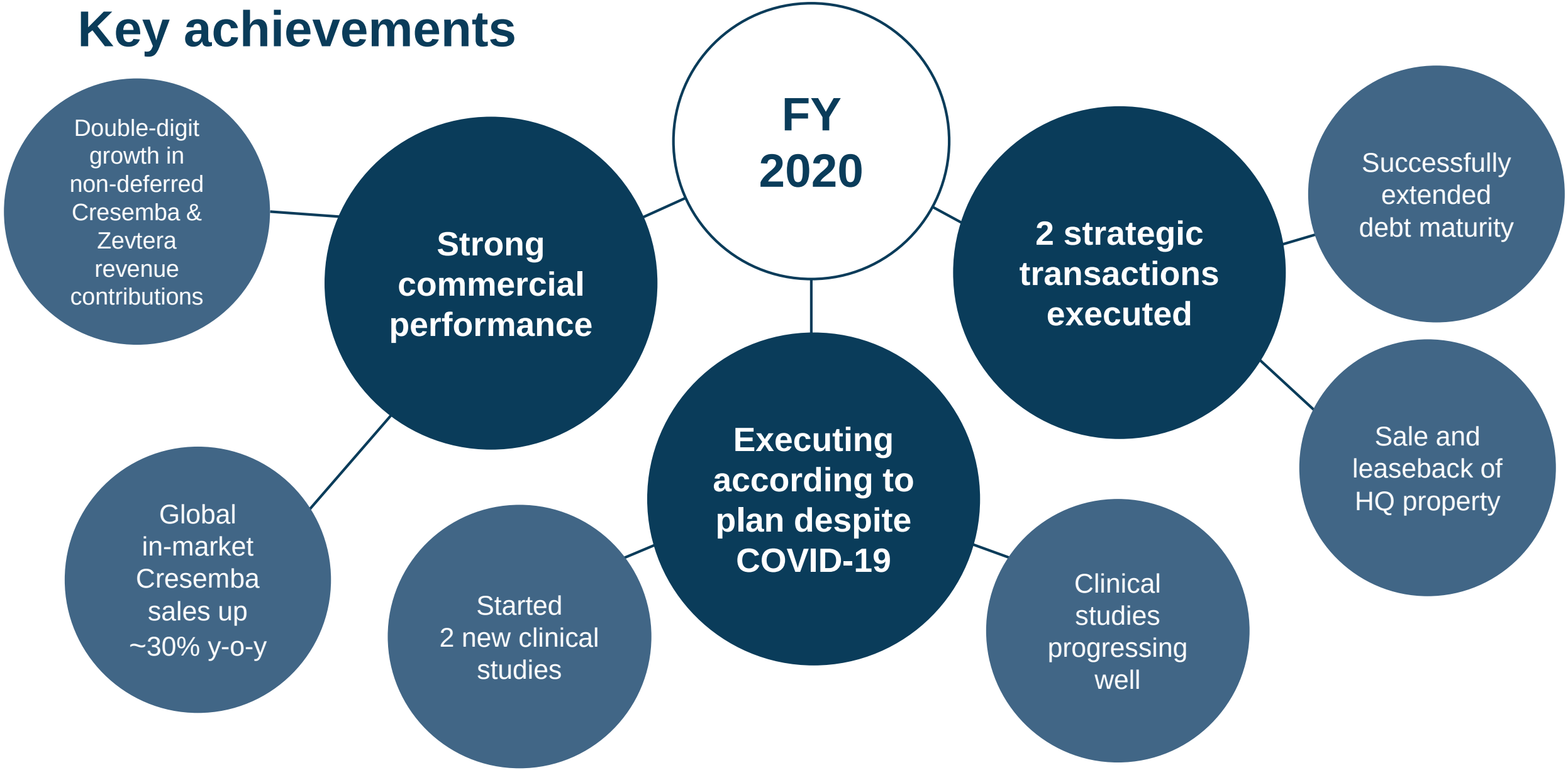
Participants

David Veitch
CEO

Adesh Kaul
CFO

Dr. Marc Engelhardt
CMO

Key achievements



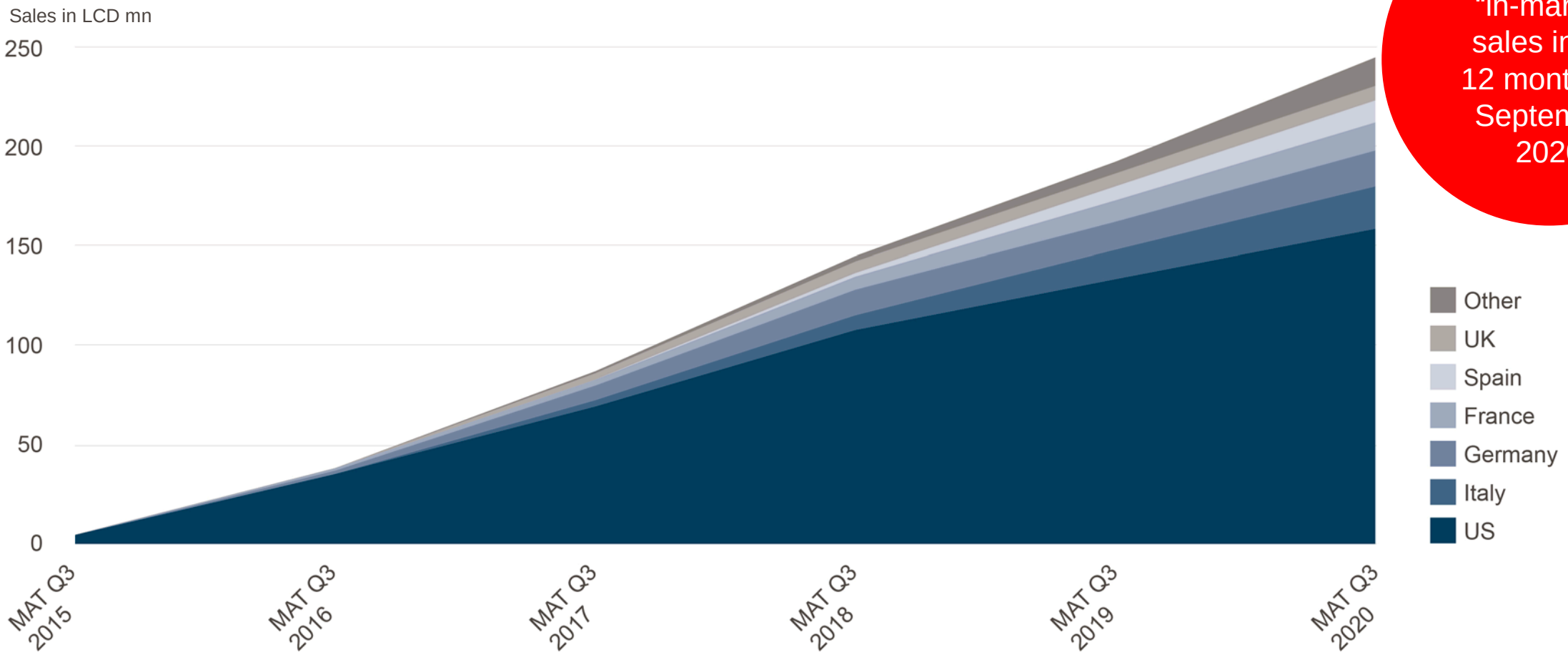
Adesh Kaul

Chief Financial Officer

Commercial & financial update

Cresemba continues strong in-market sales uptake

USD 244 mn "in-market" sales in the 12 months to September 2020



LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, September 2020

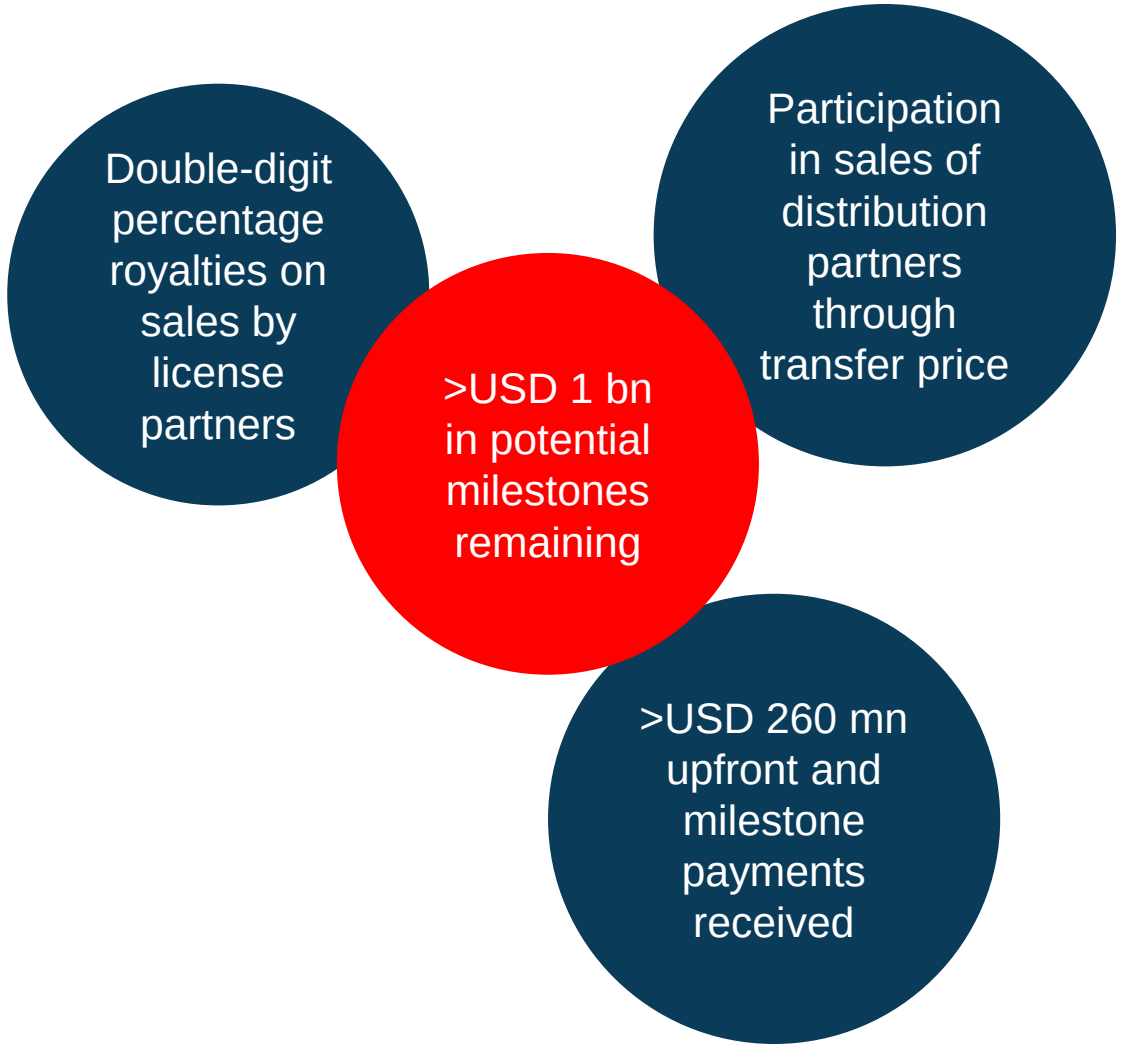
The company we keep — established strong partnerships

License partners

Pfizer Europe (excl. Nordics), China Asia-Pacific, Russia, Turkey and Israel (Cresemba®)	astellas U.S. (Cresemba®)
AsahiKASEI Japan (Cresemba®)	CR Gosun China (Zevtera®)

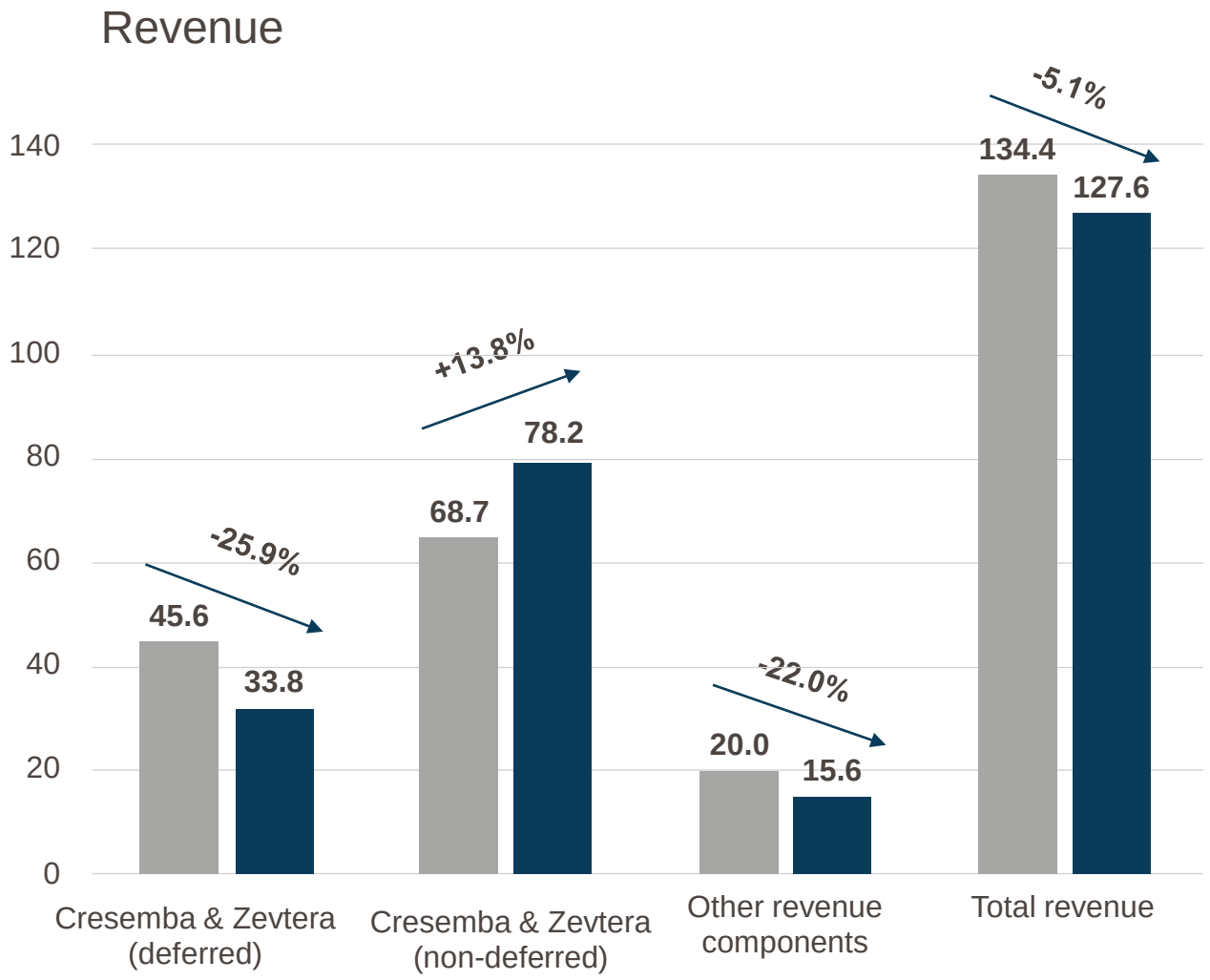
Distribution partners

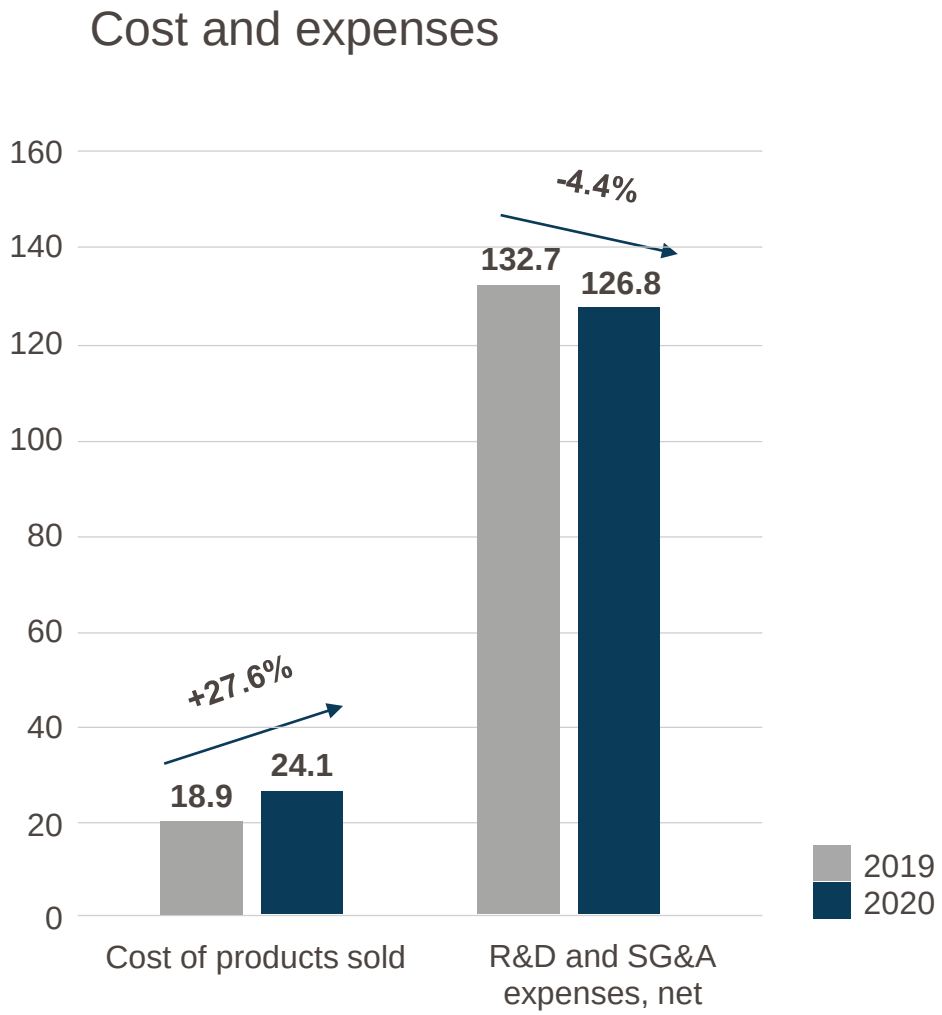
correvio Europe (excl. Nordics), Israel (Zevtera®)	hikma. MENA region (Cresemba® and Zevtera®)	GBT Grupo Biotoscana LatAm (Cresemba® and Zevtera®)
UNIMEDIC PHARMA Nordics (Cresemba® and Zevtera®)	AVIR PHARMA Canada (Cresemba® and Zevtera®)	



Financials

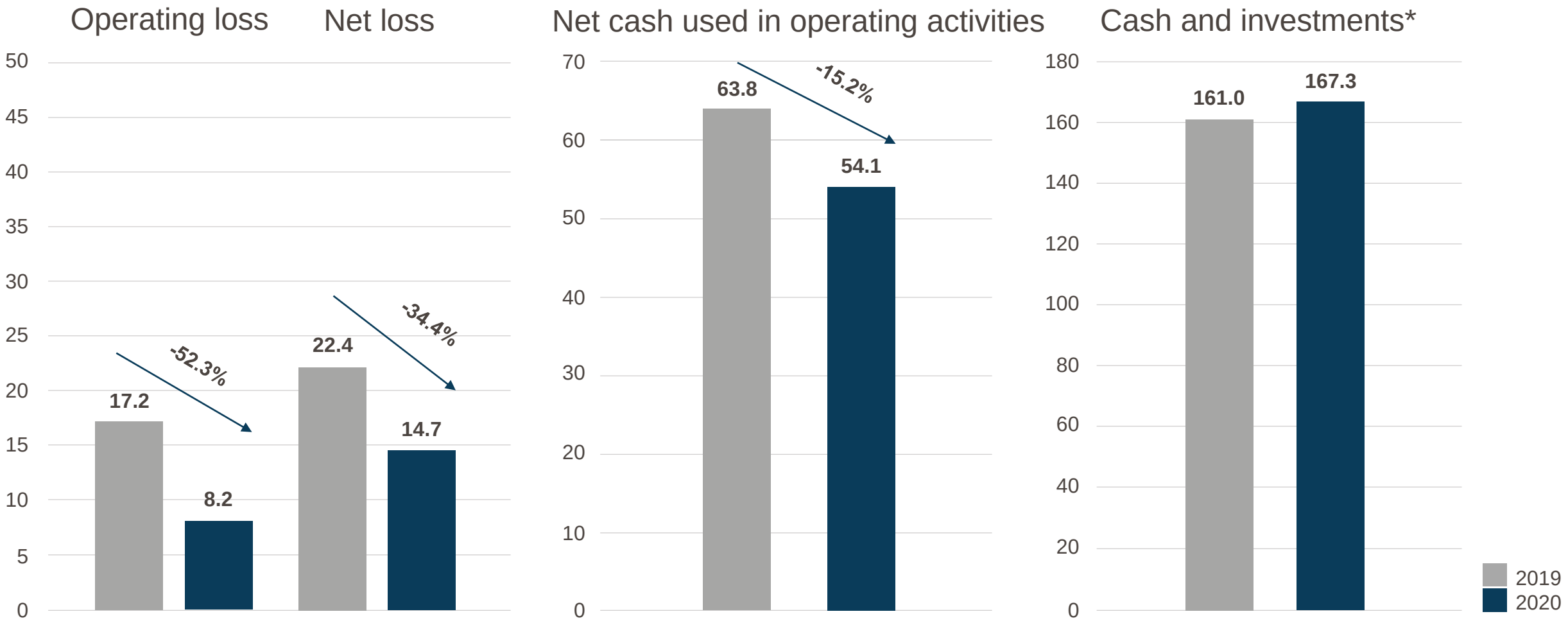
Financial summary, in CHF mn (1/2)





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

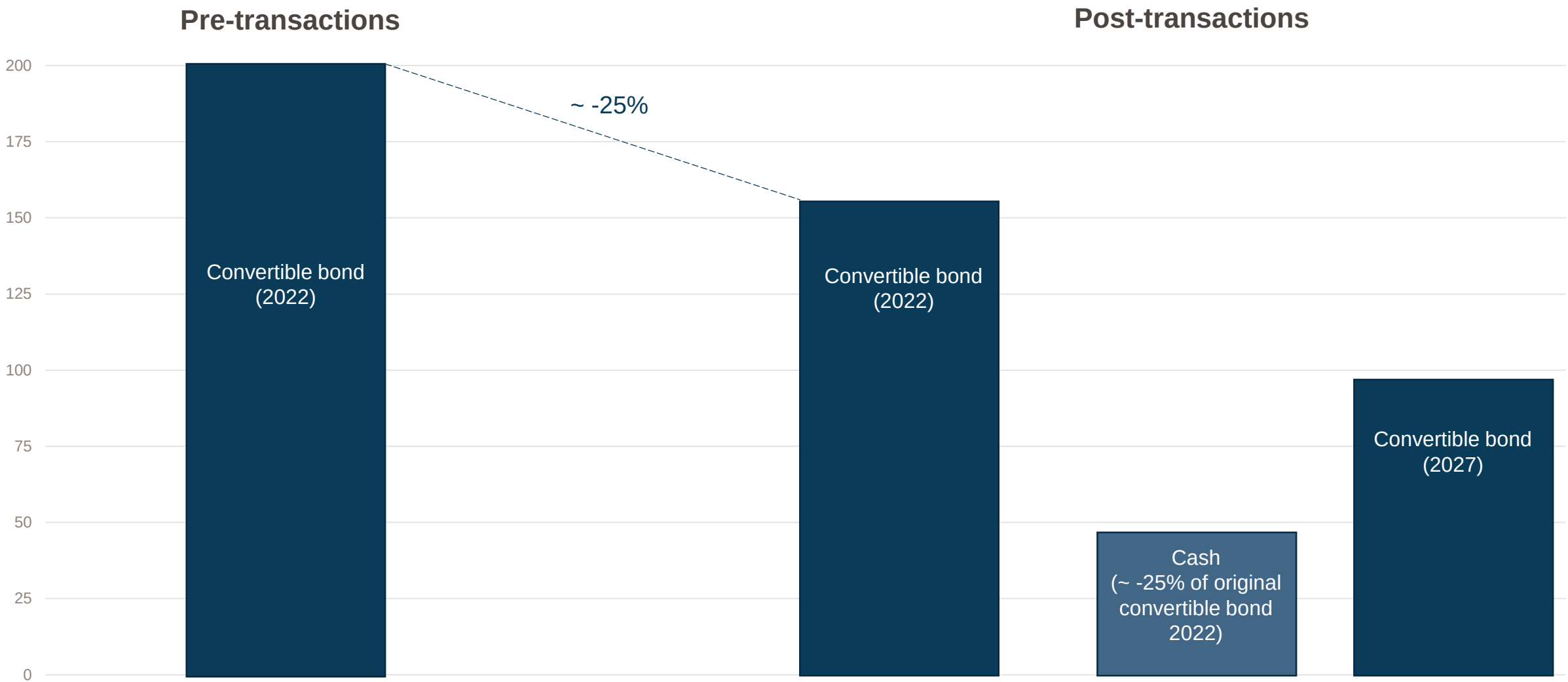
Financial summary, in CHF mn (2/2)



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

*Cash, cash equivalents, restricted cash and investments

Convertible bond transactions — successfully improved debt maturity profile (in CHF mn)



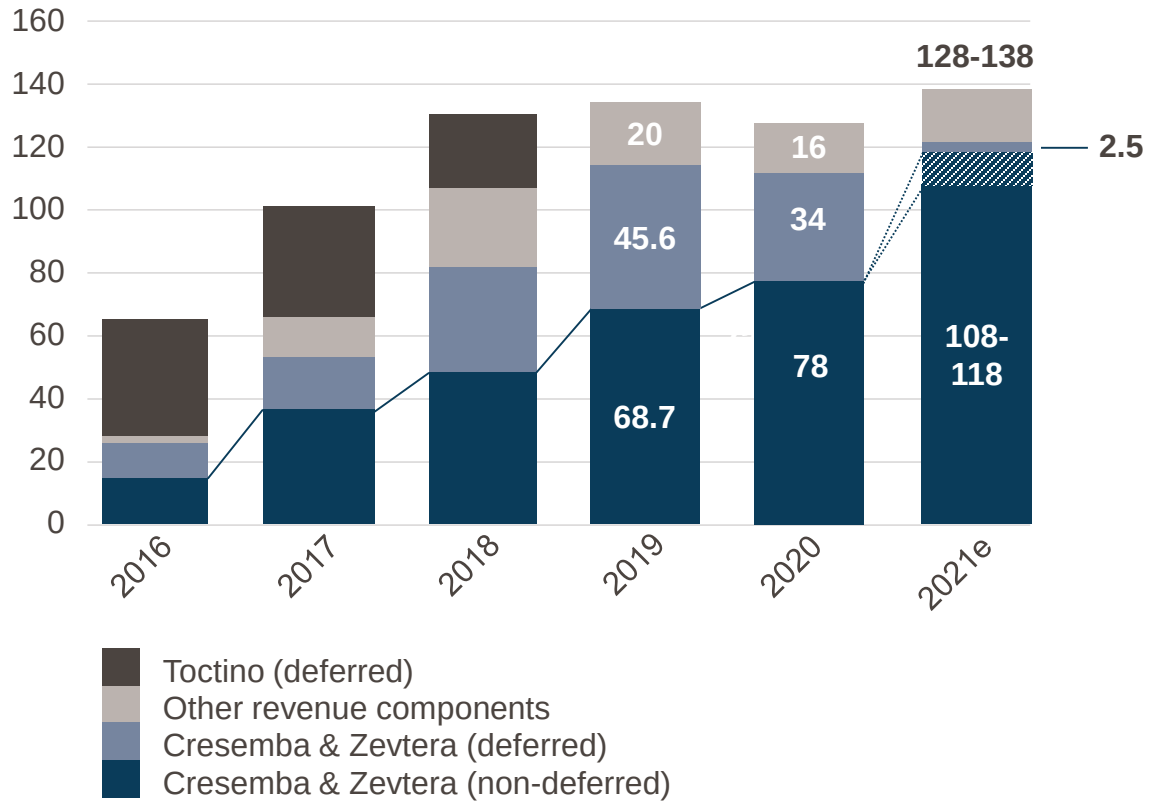
Divestment of Chinese R&D subsidiary to U.S.-based custom manufacturing organization PHT International Inc. (“PHT”)

- Total purchase price of USD 6.3 million
  - USD 2.5 million upon closing, USD 3.8 million over the course of the next three years
- Basilea has entered into an agreement with PHT for continued R&D service provision
  - All 72 employees and the facilities will be transferred to PHT
  - Ensuring continuity on R&D projects
  - Providing sufficient time to optimize external sourcing of R&D services
- Closing of the transaction is expected in Q2 2021
- Financial impact
  - Annual operating expenses related to Chinese subsidiary mid-single digit million
  - Small positive P/L impact on sale in 2021
  - Limited (positive) impact in 2021/2022 on operating expenses due to transition period
  - Subsequently a greater potential impact on operating expenses

Financial guidance

In CHF mn	FY 2020	FY 2021 guidance
Total revenue	127.6	128 - 138
thereof: Contributions Cresemba® & Zevtera® non-deferred deferred	 78.2 33.8	 108 - 118 2.5
Operating loss	8.2	13 - 23
Cash and investments*	167.3	110 – 120**

Continued strong double-digit growth in Cresemba & Zevtera non-deferred revenue contributions Y-o-Y, CHF mn



*Cash, cash equivalents, restricted cash and investments / **Excluding any potential impact from a reduction of the outstanding convertible bonds

Dr. Marc Engelhardt

Chief Medical Officer

Clinical development update

Antibacterial

Zevtera® (Ceftobiprole)

Severe bacterial infections

Strategy for accessing the U.S. market

- Two cross-supportive phase 3 studies under FDA Special Protocol Assessment (SPA)

1. Acute Bacterial Skin and Skin Structure Infections (ABSSSI)[1] successfully completed

TARGET
ABSSSI Study

2. *Staphylococcus aureus* bacteremia (SAB)[2] ongoing, topline results from phase 3 study expected in H1 2022

ERADICATE
SAB Study

- Phase 3 program largely funded by BARDA (up to USD ~130 mn, ~70% of total program costs)

BIOMEDICAL ADVANCED RESEARCH AND DEVELOPMENT AUTHORITY
BARDA

- Qualified Infectious Disease Product (QIDP) designation extends U.S. market exclusivity to 10 years from approval

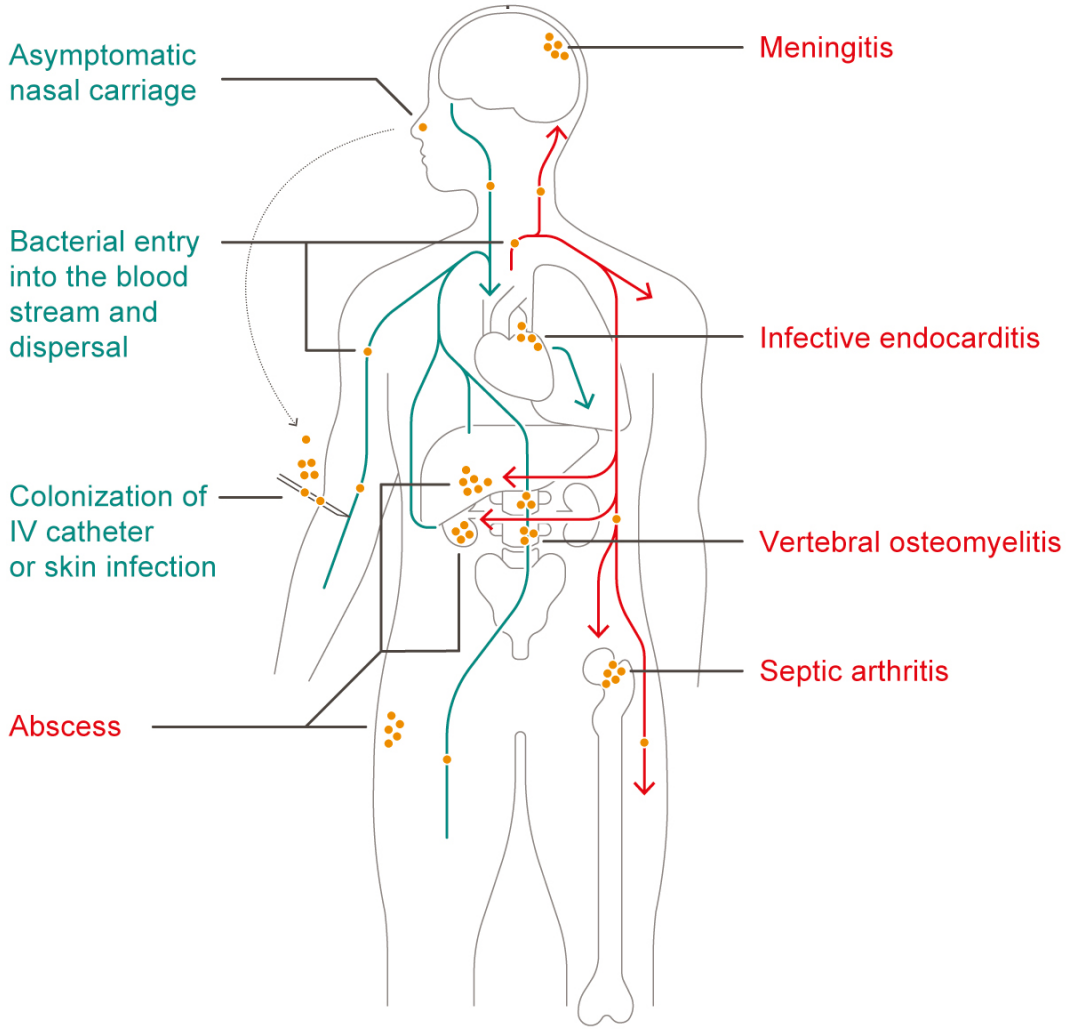
[1] Overcash JS et al. ECCMID 2020, abstract 1594. (NCT03137173)
[2] Hamed K et al. Future Microbiol. 2020;15:35-48. (NCT03138733)

SAB – an area with high medical need

- Nearly 120,000 *S. aureus* bloodstream infections in the US (in 2017)[1]
- ERADICATE targets complicated SAB, characterized by concomitant or metastatic infections such as bone, joint or heart valve infections; persistent bacteremia; or bacteremia in patients on dialysis
- Substantial morbidity and approximately 20% 30-day mortality[2]
- Limited antibiotic treatment options with only two approved treatments for SAB in the U.S. that cover both MSSA and MRSA, i.e. vancomycin and daptomycin

[1] MMWR, 2019;68:214–219.
[2] Hamed K et al. Future Microbiol. 2020;15:35-48.
MRSA: methicillin-resistant *Staphylococcus aureus*
MSSA: methicillin-susceptible *Staphylococcus aureus*

Causes and consequences of SAB



Adapted from Edwards AM et al. Trends Microbiol. 2011;19:184-190.

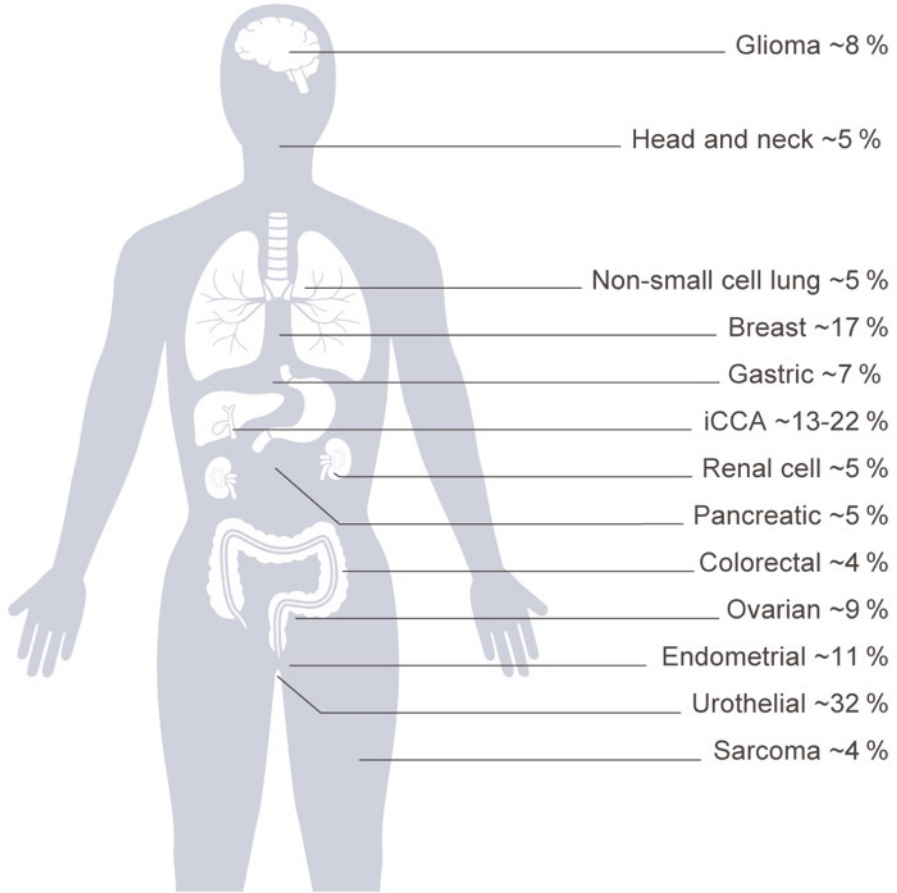
Oncology

Derazantinib

FGFR-driven tumors

Targeting FGFR-driven tumors as single agent and in combination with immunotherapy

- Small molecule, oral inhibitor of FGFR family of kinases
- Development strategy focused on achieving differentiation by leveraging unique properties of derazantinib
  - Kinase inhibition profile: exploring therapeutic potential of additional targets of derazantinib such as CSF1R and VEGFR2 kinase
  - Safety profile: exploring relevance for potential combination therapies
- Three clinical studies ongoing
  - FIDES-01 (Ph 2) in intrahepatic cholangiocarcinoma (iCCA)
  - FIDES-02 (Ph 1/2) in urothelial cancer
  - FIDES-03 (Ph 1/2) in gastric cancer



Sources: Helsten et al., Clin Cancer Res 2016 (22), 257-267; FGFR2 fusions in iCCA: Graham et al. Hum Pathol 2014 (45), 1630-1638; Jain et al. JCO Precis Oncol 2018 (2) 1-12

Phase 2 study in iCCA* – established clinical proof of concept in FIDES-01

FIDES-01 Cohort 1 (N=103)	FIDES-01 Cohort 2 (N=43) - ongoing
FGFR2 fusions (~15% of iCCA) **Topline results:** ORR: 20.4% Median PFS: 6.6 months	**FGFR2 mutations/amplifications** (~5% of iCCA) Pooled data from 23 patients (clinical studies/EAP)[2] Median PFS 7.2 months
• Consistent with earlier Phase1/2 data[1] and with interim analysis from FIDES-01 • Clinical proof of concept for derazantinib as monotherapy in its first indication established	• Encouraging progression-free survival in pooled analysis consistent with outcome in patients with FGFR2 gene fusions • Interim results expected H1 2021

Manageable safety profile with low incidence of nail toxicity, retinal events, hand-foot syndrome and stomatitis

FIDES-01: NCT03230318

[1]Mazzaferro et al. Br J Cancer. 2019

[2]Droz Dit Busset et al. Annals of Oncology (2020) 31 (suppl_5): abstract 45P (NCT01752920, NCT03230318)

*in patients who progressed after at least one prior systemic chemotherapy regimen

Clinical program in urothelial cancer – FIDES-02

Multi-cohort phase 1b/2 study of derazantinib monotherapy or in combination with atezolizumab in patients with urothelial cancer harboring FGFR genetic aberrations

- Substudies (N≈300) in various treatment settings, including:
  - Post-chemotherapy/immunotherapy recurrence (second-line and post second-line)
  - First-line platinum-ineligible, PD-L1-low
  - Resistance to prior FGFR-inhibitor treatment
- Successful completion of phase 1b cohort
  - Recommended phase 2 dose for the combination established
  - No dose-limiting toxicities observed
- Clinical supply agreement with Roche for atezolizumab
- Plan to amend the study protocol to explore a higher dose of derazantinib in two cohorts of this study
  - Supported by the observed safety and tolerability profile of derazantinib at the current dose of 300 mg per day
  - May provide additional benefits in monotherapy and combination to patients with FGFR-positive urothelial cancer
  - Considers the evolving treatment and competitive landscape in urothelial cancer in patients both with and without FGFR genetic aberrations
- Interim results in derazantinib monotherapy expected H1 2021
- Interim results in combination therapy with atezolizumab expected H2 2021

FIDES-02: NCT04045613; Chaudhry A et al. Journal of Clinical Oncology 2020; 38, no. 6_suppl. TPS590

Clinical program in gastric cancer – FIDES-03

Multi-cohort Phase 1b/2 study of derazantinib as monotherapy or in combination therapy with standard of care (ramucirumab/paclitaxel) or atezolizumab in patients with advanced HER2-negative gastric adenocarcinoma harboring FGFR genetic aberrations

- Substudies using derazantinib monotherapy or combination treatment, including:
  - Derazantinib monotherapy in various molecular subtypes
  - Combination of derazantinib with ramucirumab/paclitaxel
  - Combination of derazantinib with atezolizumab
- Clinical supply agreement with Roche for atezolizumab
- Clinical trial collaboration and supply agreement with Lilly for ramucirumab
- Interim results in derazantinib monotherapy and recommended phase 2 dose of derazantinib in combination with ramucirumab/paclitaxel expected H2 2021

FIDES-03: NCT04604132

Oncology

Lisavanbulin (BAL101553)

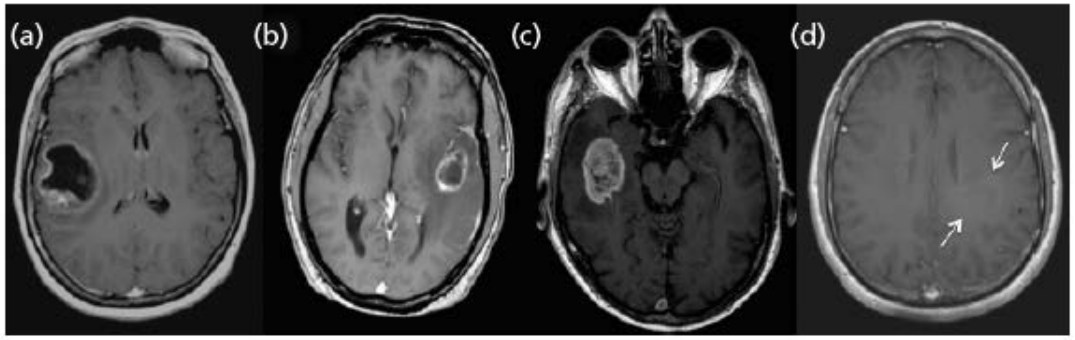
Glioblastoma
and other solid tumors

Unmet medical need in glioblastoma

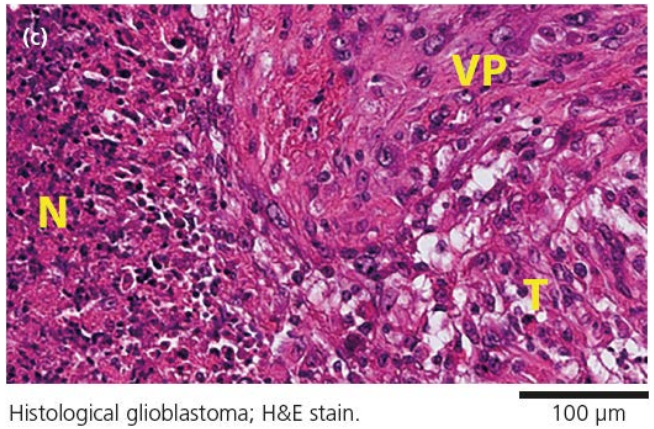
- The most common primary brain cancer in adults with an incidence of 3-4 per 100,000 people, (though geographic variation exists) and a median age at onset of > 60 years
- Associated with poor prognosis, high morbidity and healthcare burden
- 5-year survival is below 5% with current standard of care (multimodality treatment including surgery, radiotherapy, chemotherapy)[1]
- *MGMT*-promoter methylation status has been demonstrated as a predictor for the response to (radio)chemotherapy (temozolomide)[2]
- Established molecular markers used for classification include *IDH* mutations and/or 1p/19q codeletion[3]
- No molecular targeted therapy currently approved

[1]Poon MTC et al. 2020; Sci Rep 10, 11622; [2]Hegi et al. NEJM 2005;352:997-1003
[3]Louis DN et al. Acta Neuropathol. 2016;131:803-820

Radiological and tissue presentations of glioblastoma



Variable glioblastoma appearances on post-gadolinium T1-weighted MRI: central necrotic mass with nodular rim enhancement (a,b), predominantly solid enhancement (c), lack of contrast uptake (d)



Histological features of glioblastoma include marked hypercellularity, nuclear atypia, microvascular proliferation, and necrosis (N: necrosis, VP: vascular proliferations, T: tumor)

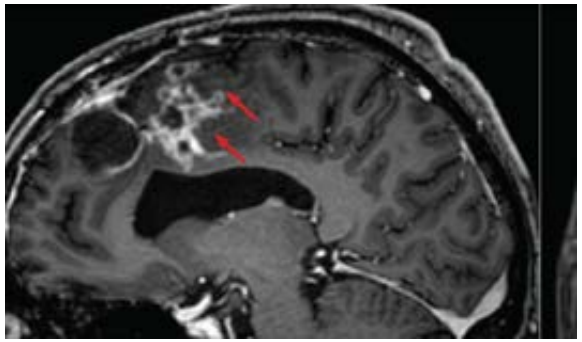
McNamara MG, Brandner S, Thust SC. Fast facts: Glioblastoma. 2020. S Karger Publishers Ltd.

Biomarker-driven phase 2 study ongoing in recurrent glioblastoma

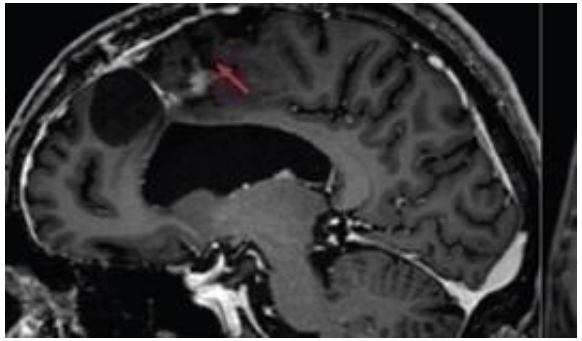
- EB1 (end-binding protein 1) is located on the microtubules and involved in microtubule dynamics and has been shown to be a response predictive marker for lisavanbulin in preclinical studies
- Strong EB1 staining was observed in a patient with an exceptional response to daily oral lisavanbulin in the phase 1 dose-escalation study in recurrent glioblastoma[1]
  - Patient ongoing for more than two years
  - >80% reduction in glioblastoma tumor size
- Interim results expected H2 2021

[1] Lopez et al. Phase 1/2a study of once daily oral BAL101553, a novel tumor checkpoint controller, in adult patients with progressive or recurrent glioblastoma or high-grade glioma. JCO 2019;37:15 suppl, 2025 (NCT02490800)

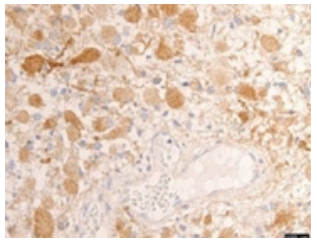
Glioblastoma tumor size reduction in an exceptional responder and EB1 staining of glioblastoma tissue compared to non-responding patients



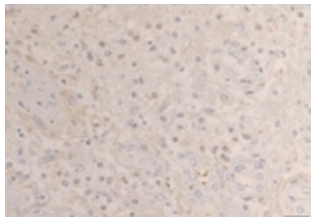
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



Non-responder

David Veitch

Chief Executive Officer

Outlook

Milestones & Outlook 2021 / 2022

Cresemba® & Zevtera® — Increasing cash flows
By the end of 2021, Cresemba to be on the market in 60 countries

		H1 2021	H2 2021	H1 2022	H2 2022
Isavuconazole		✓ Complete patient enrolment in phase 3 study in Japan	Topline results from phase 3 study in Japan		
Ceftobiprole			Complete patient enrolment in SAB phase 3 study	Topline results from SAB phase 3 study	
Derazantinib	**FIDES-01 (iCCA)**	✓ Topline results (FGFR2 fusions)			
		Interim results (other FGFR2 gene aberrations)		Topline results (other FGFR2 gene aberrations)	
	FIDES-02 (urothelial cancer)	Interim results in derazantinib monotherapy	Interim results in combination therapy with atezolizumab		Topline results in combination therapy with atezolizumab
	FIDES-03 (gastric cancer)		Interim results in monotherapy and recommended phase 2 dose with ramucirumab/paclitaxel		Interim results in combination with ramucirumab/paclitaxel
Lisavanbulin			Interim results from phase 2 biomarker-driven glioblastoma study	Topline results from phase 2 biomarker-driven glioblastoma study	
			Recommended phase 2 dose in phase 1 study in newly-diagnosed glioblastoma in combination with radiotherapy		

Q & A

Thank you

Appendix

Glossary

- ABSSSI: **A**cute **b**acterial **s**kin and **s**kin **s**tructure **i**nfections
- CSF1R: **C**olony-**s**timulating **f**actor **1** **r**eceptor
- EAP: **E**xpanded **a**ccess **p**rogram
- FGFR: **F**ibroblast **g**rowth **f**actor **r**eceptor
- FIDES: **F**ibroblast growth factor **i**nhibition with **de**razantinib in **s**olid tumors
- iCCA: **I**ntrahepatic **c**holangio**ca**rcinoma
- MSSA: **M**ethicillin-**s**usceptible ***S**taphylococcus **a**ureus*
- MRSA: **M**ethicillin-**r**esistant ***S**taphylococcus **a**ureus*
- ORR: **O**bjective **r**esponse **r**ate
- PFS: **P**rogression-**f**ree **s**urvival
- SAB: ***S**taphylococcus **a**ureus* **b**acteremia
- VEGFR2: **V**ascular **e**ndothelial **g**rowth **f**actor **r**eceptor **2**

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