



Half-year results 2021

August 17, 2021

Webcast presentation



David Veitch

Chief Executive Officer

Introduction



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Participants



David Veitch
CEO

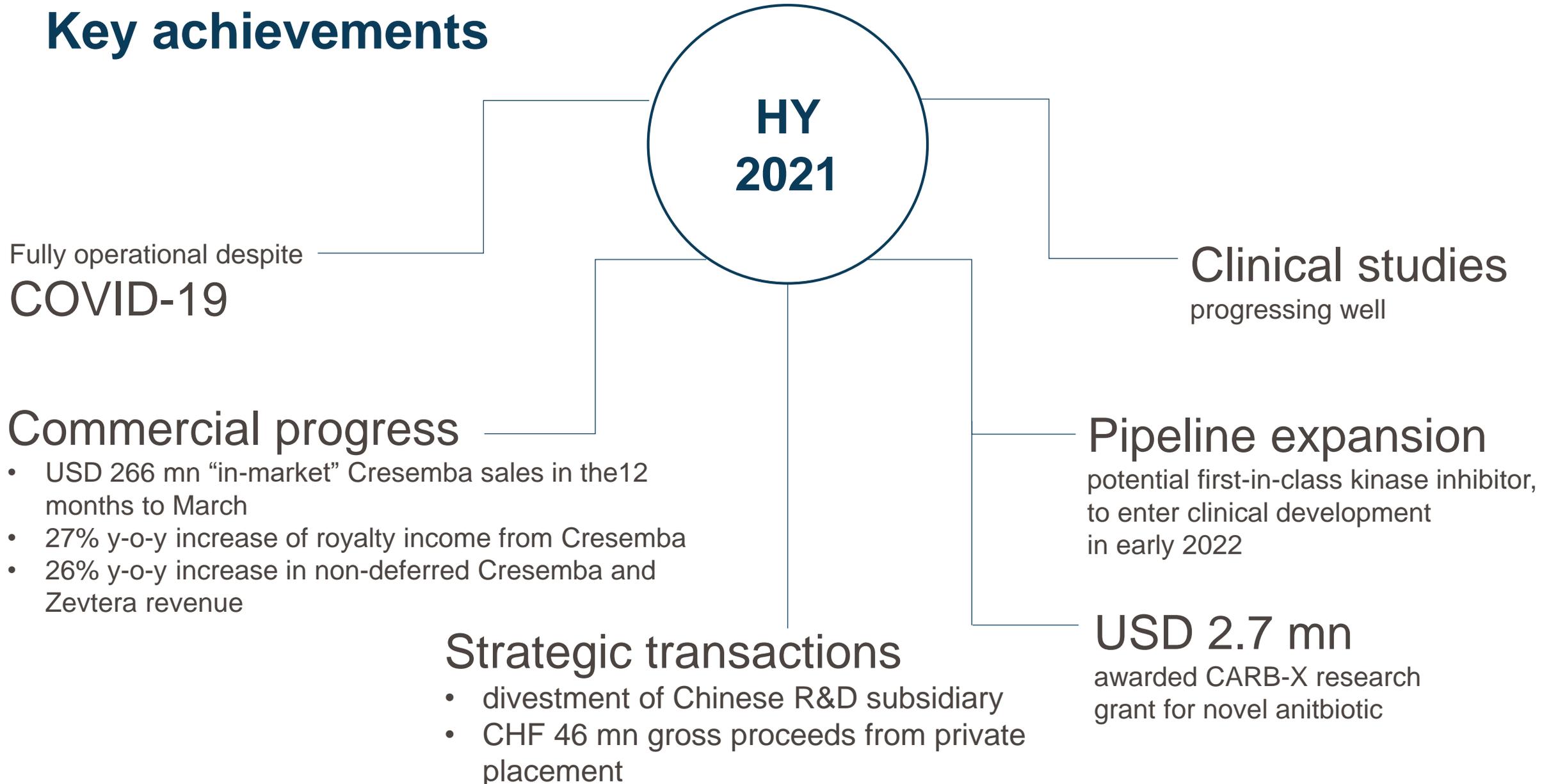


Adesh Kaul
CFO



Dr. Marc Engelhardt
CMO

Key achievements



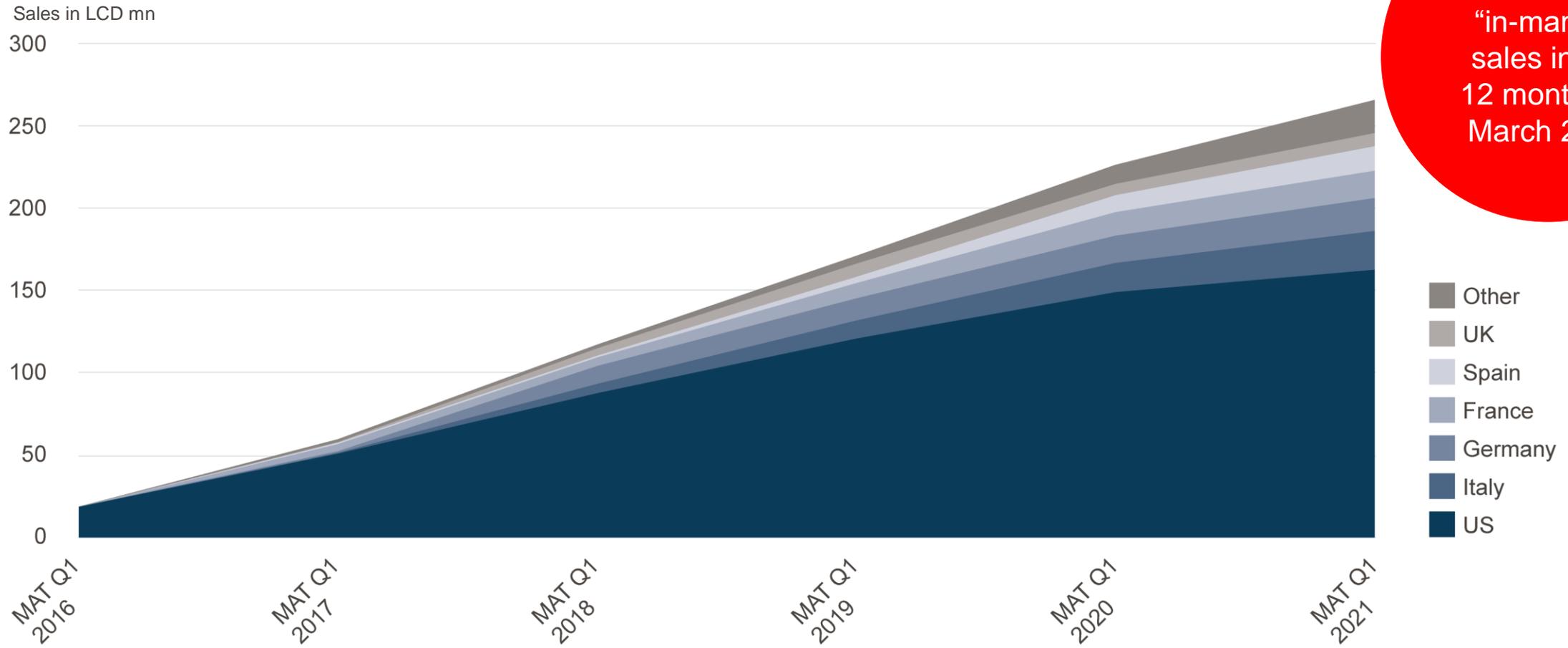
Adesh Kaul

Chief Financial Officer

Commercial &
financial update



Cresemba continues strong in-market sales uptake



USD 266 mn
 “in-market”
 sales in the
 12 months to
 March 2021

LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, March 2021

The company we keep — established strong partnerships

License partners



Europe (excl. Nordics), China
Asia-Pacific, Russia, Turkey
and Israel (Cresemba®)



U.S. (Cresemba®)



Japan (Cresemba®)



China (Zevtera®)

Distribution partners



Europe (excl. Nordics),
Israel (Zevtera®)



MENA region
(Cresemba® and Zevtera®)



LatAm
(Cresemba® and Zevtera®)



Nordics
(Cresemba® and Zevtera®)



Canada
(Cresemba® and Zevtera®)



Russia and the Eurasian
Economic Union
(Zevtera®)

Double-digit
percentage
royalties on
sales by
license
partners

>USD 1 bn
in potential
milestones
remaining

Participation
in sales of
distribution
partners
through
transfer price

>USD 260 mn
upfront and
milestone
payments
received

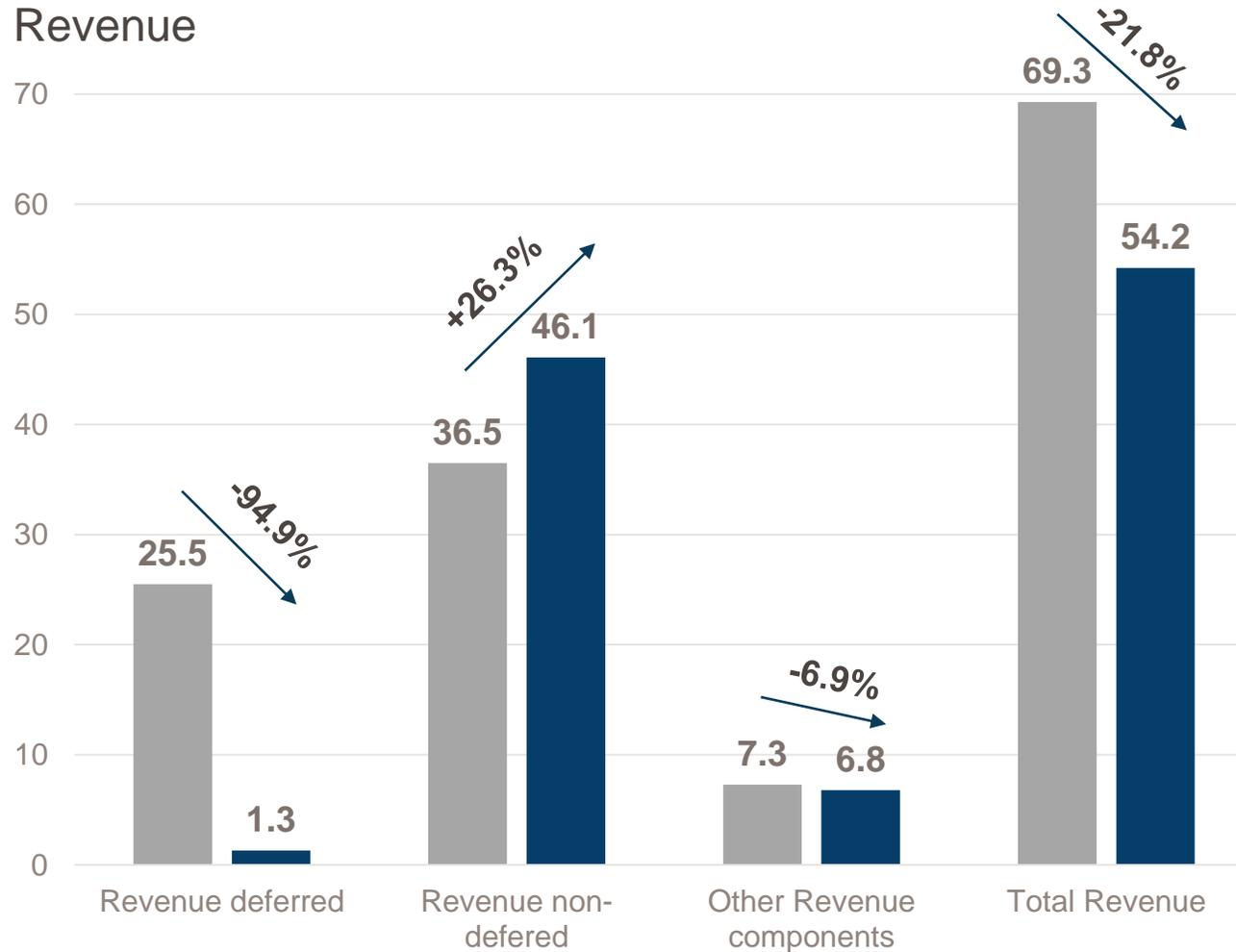


Financials

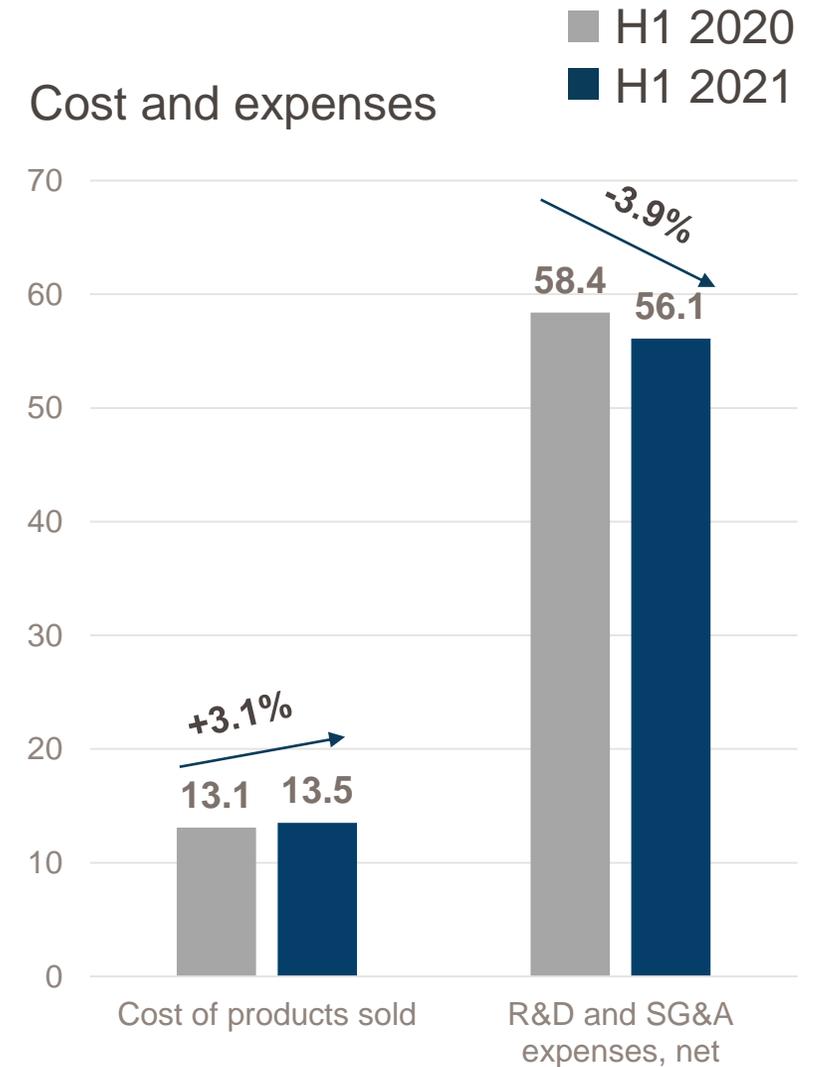


Financial summary, in CHF mn (1/2)

Revenue



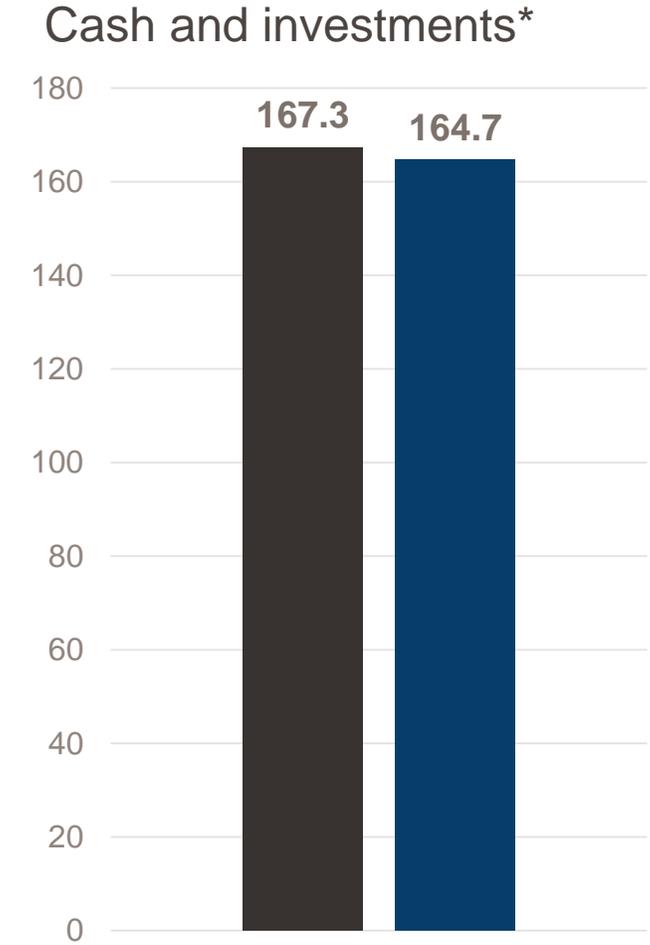
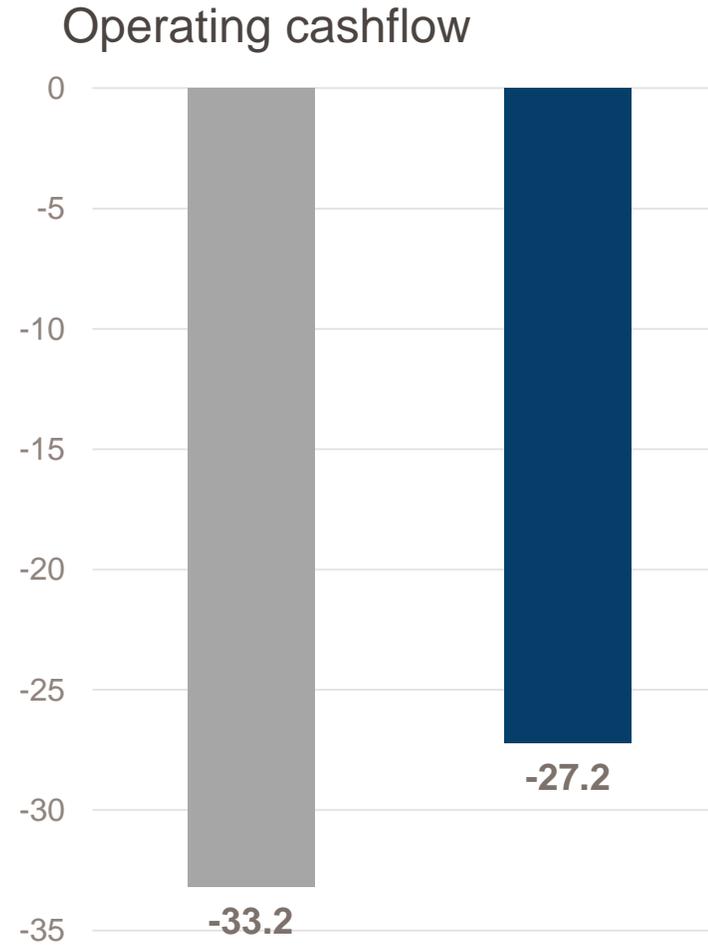
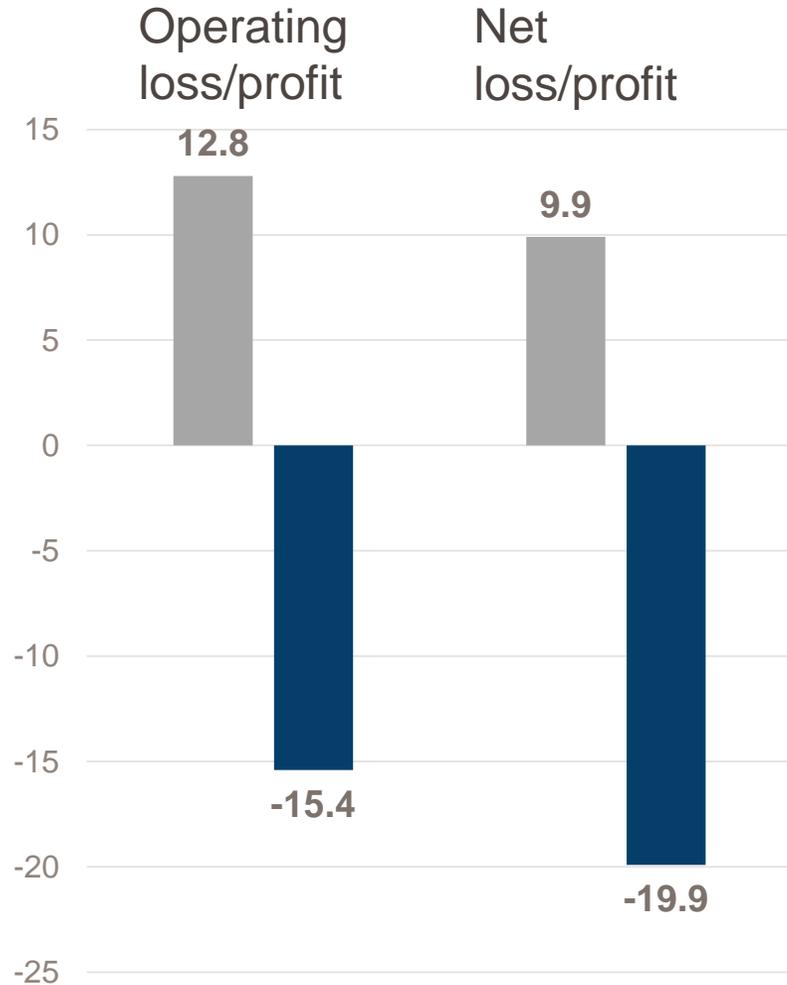
Cost and expenses



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

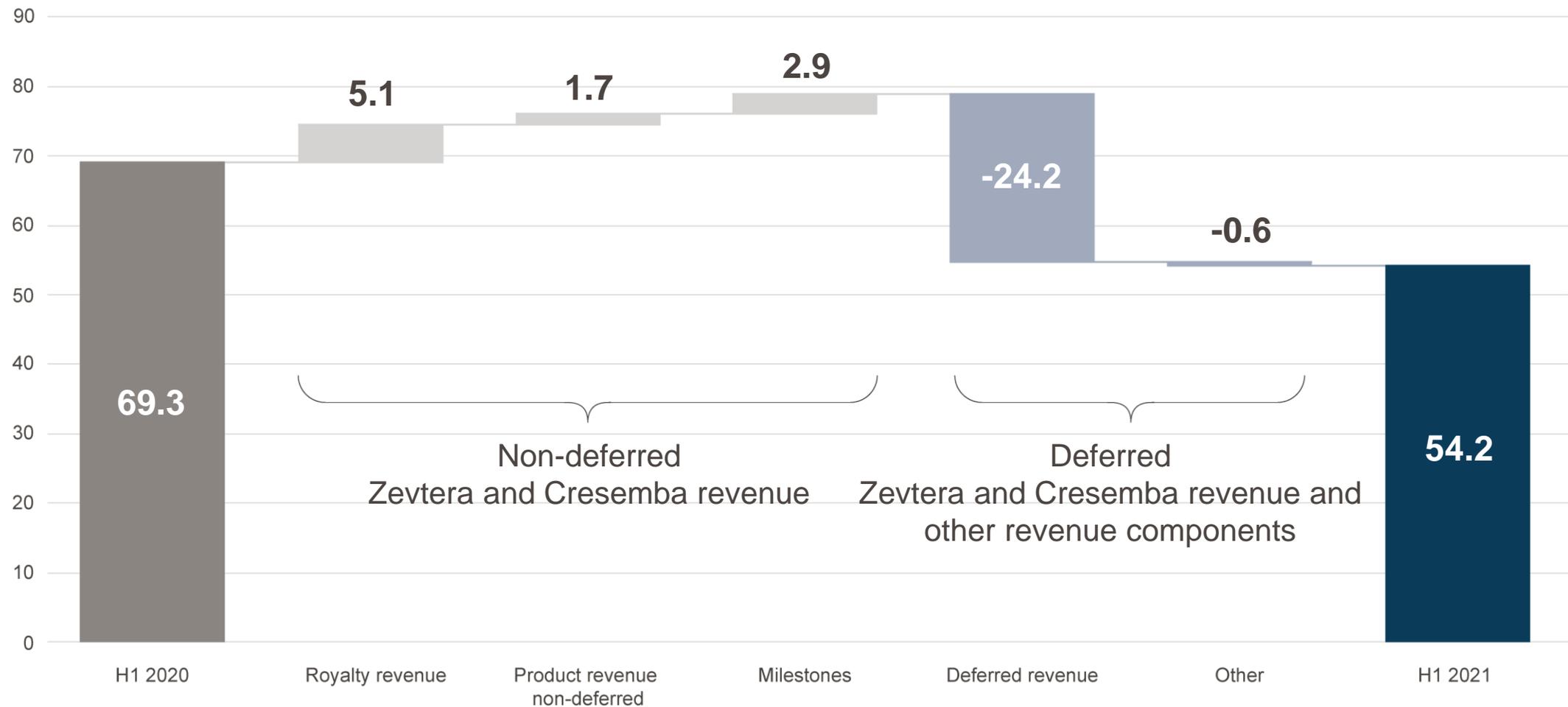
Financial summary, in CHF mn (2/2)

- H1 2020
- H1 2021
- YE 2020



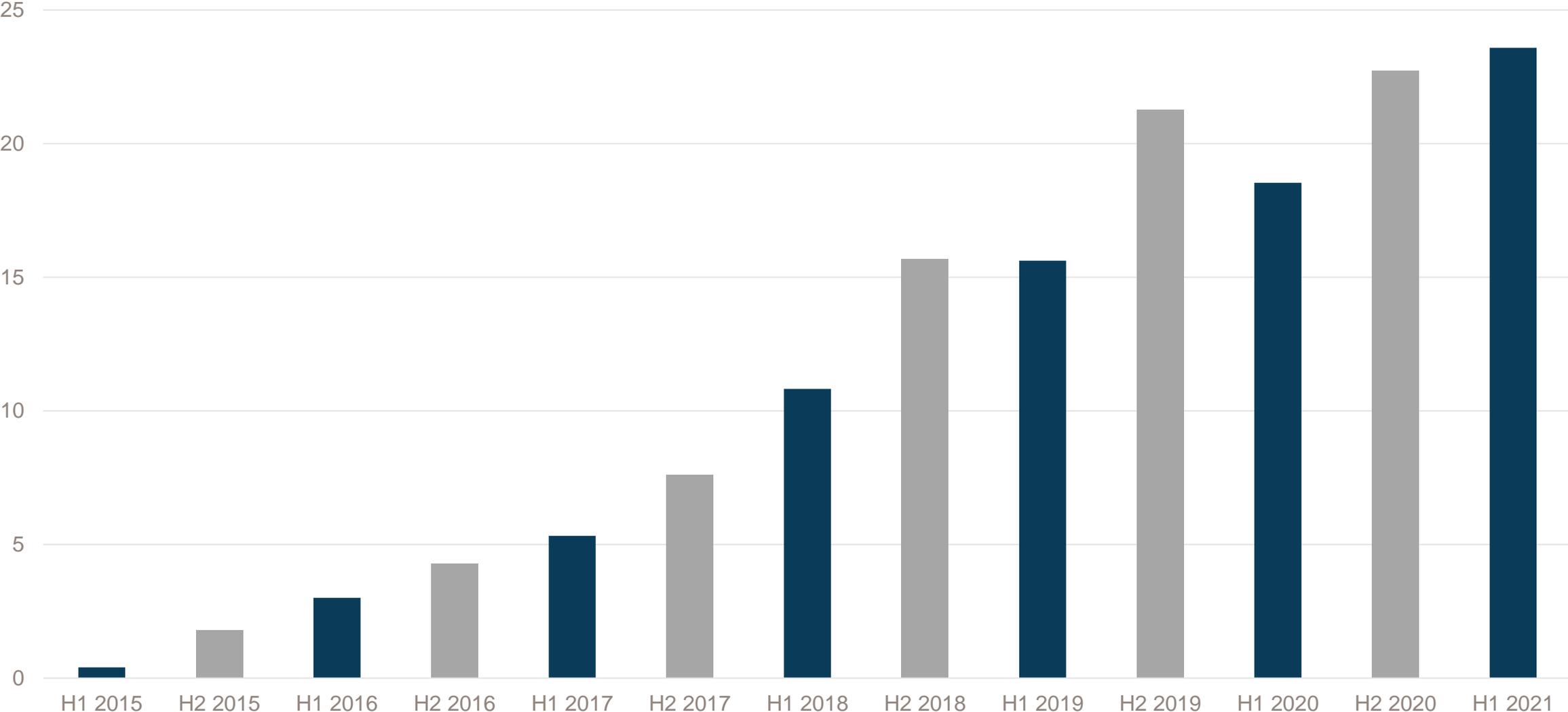
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently, *Cash, cash equivalents, restricted cash and investments

Significant growth in non-deferred revenues based on higher royalties, product revenue and milestones (in CHF mn)



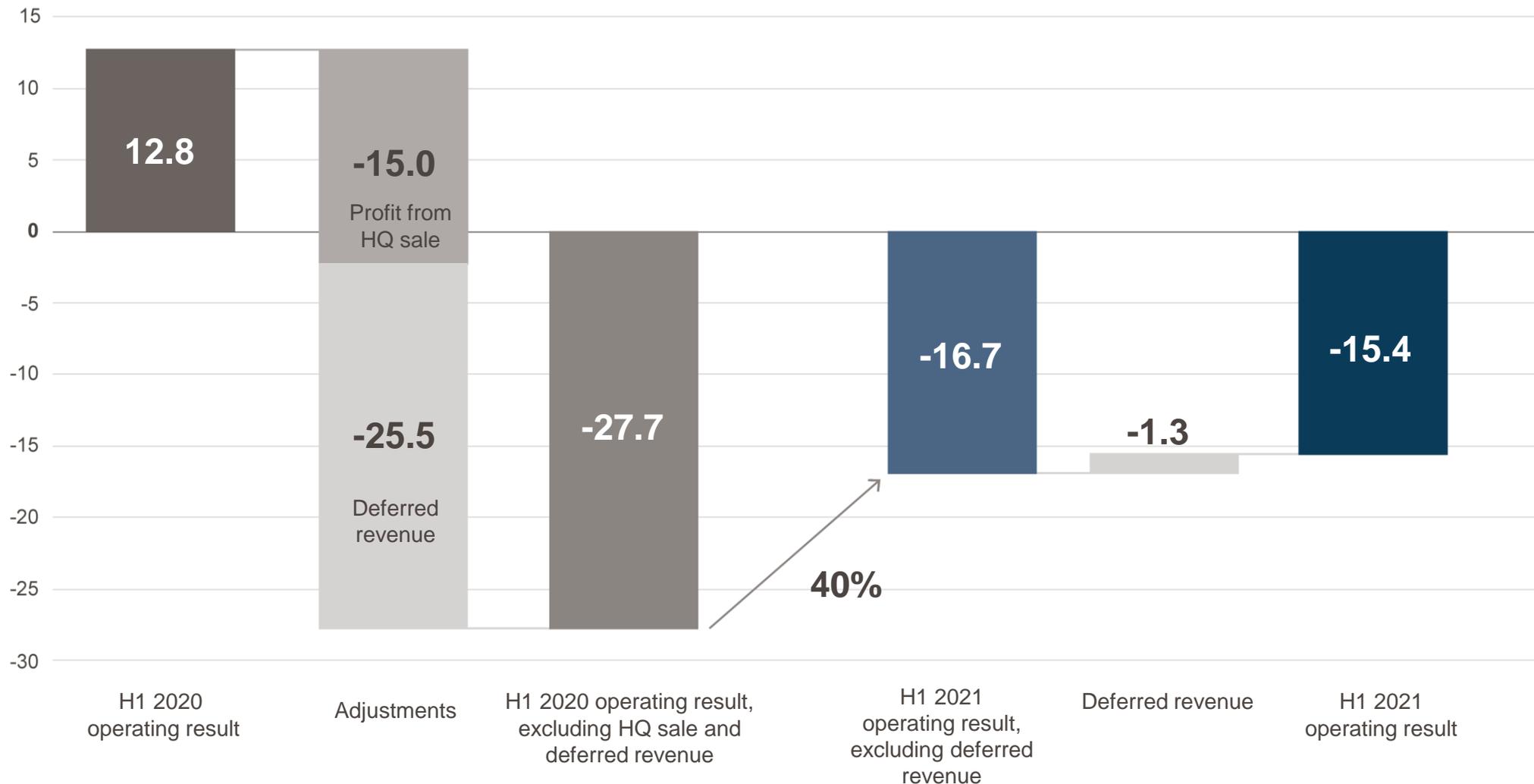
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

Cresemba royalty revenue growth reflects continued commercial success in key territories (in CHF mn)



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

Significant improvement in underlying operating performance (CHF mn)

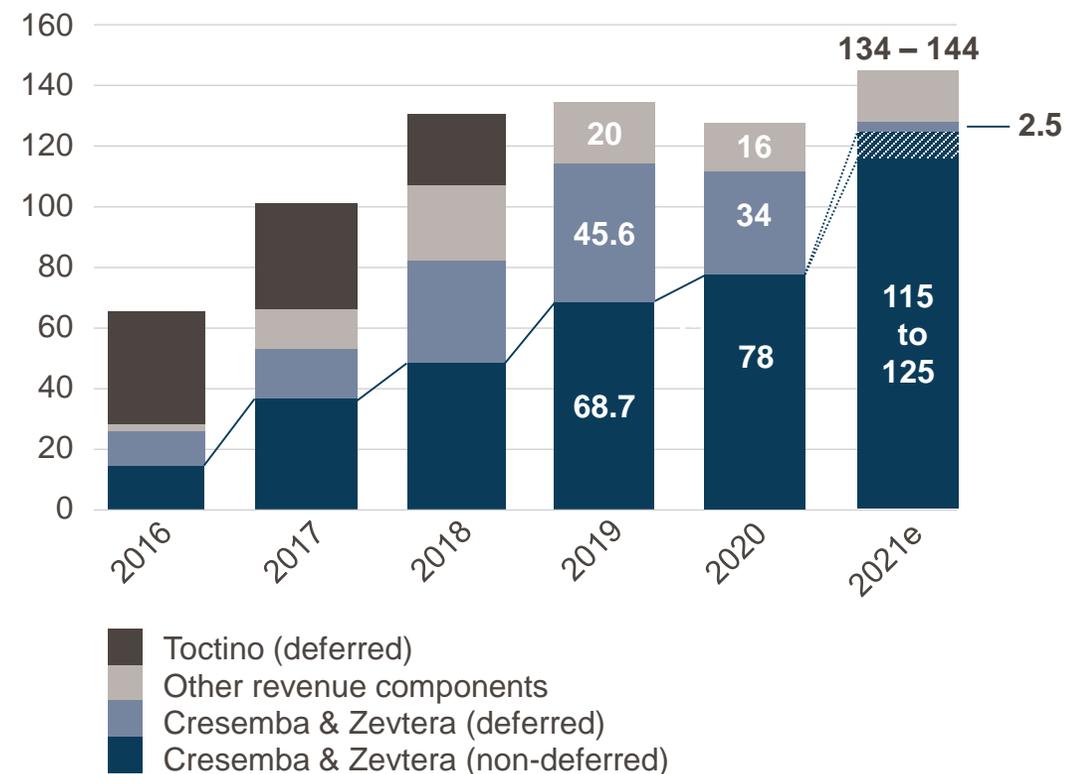


Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

2021 financial guidance - increased revenue and improved operating result

In CHF mn	FY 2021e (updated)	FY 2021e (previous)	FY 2020 (actual)
Total revenue	134 – 144	128 – 138	127.6
thereof: Contributions Cresemba® & Zevtera® non-deferred	115 – 125	108–118	78.2
deferred	2.5	2.5	33.8
Operating loss	7 – 17	13 – 23	8.2
Cash and investments*	165 – 170**	155 – 160**	167.3

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 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)
- Phase 3 program largely funded by BARDA (~70% of total program costs; up to USD ~134 mn)

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



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



- 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 mortality²
- 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

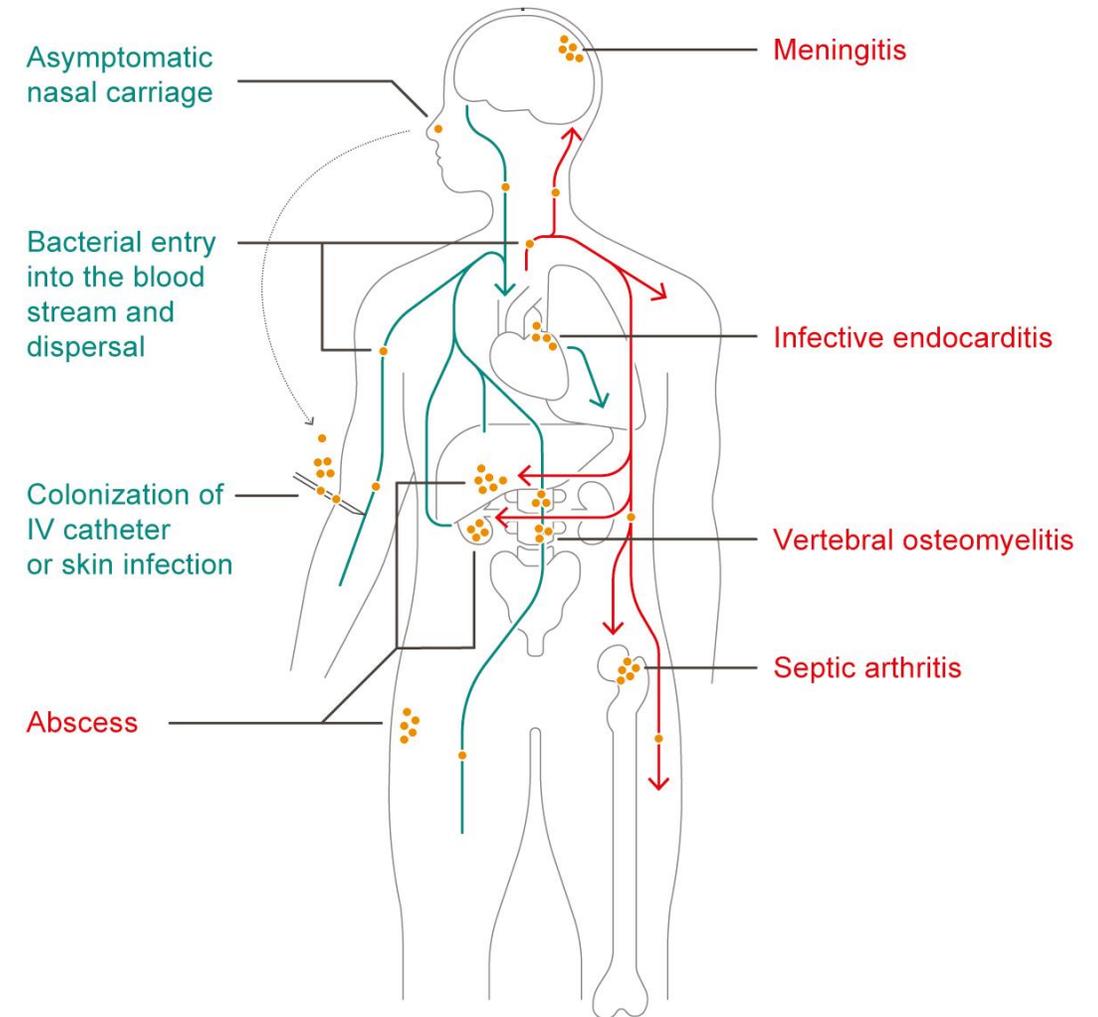
¹ MMWR, 2019;68:214–219.

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.

A microscopic view of cells, likely cancer cells, with an orange tint. The cells are spherical and have a textured surface, with some showing a more granular or fibrous appearance. They are interconnected by thin, filamentous structures. The background is a solid orange color.

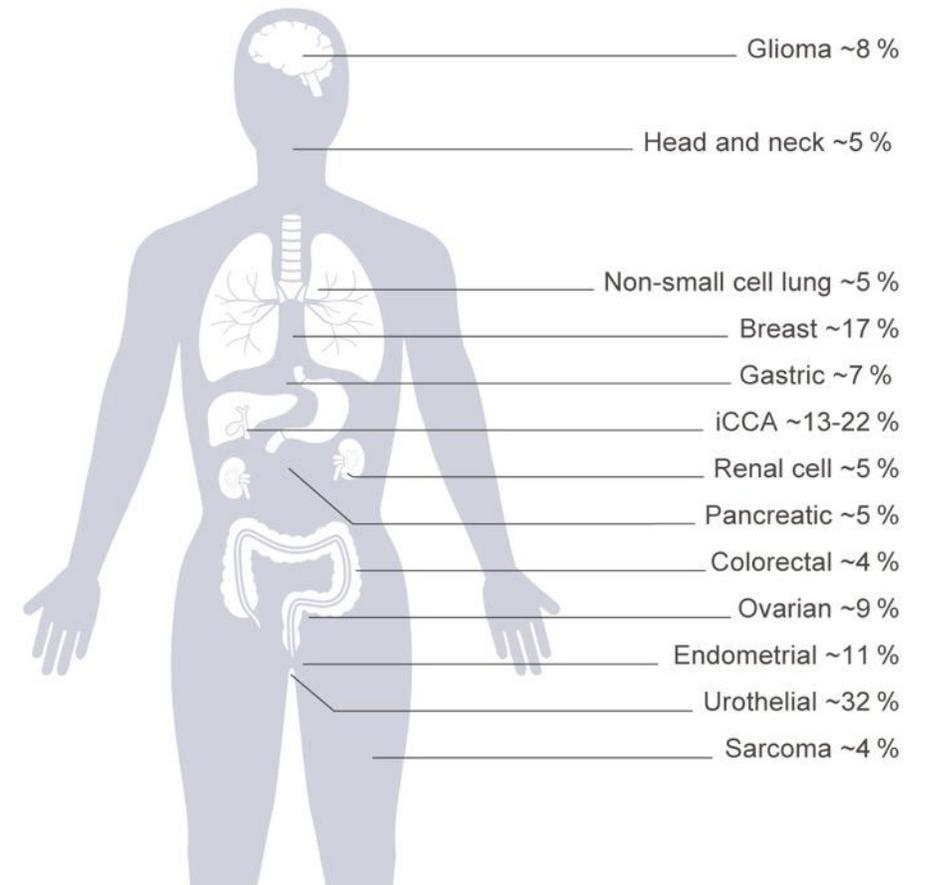
Oncology

Derazantinib

FGFR-driven tumors

Targeting FGFR-driven tumors as single agent and in combinations

- 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 studies with FGFR-inhibitors in iCCA

Variable	Derazantinib ¹ FIDES-01 Cohort 1	Infigratinib ² (QED)	Pemigatinib ³ (Incyte) FIGHT-202	Futibatinib ⁴ (Taiho) FOENIX-CCA2
N	103	108	108	103
Objective response rate	21%	23%	37%	42%
Disease control rate	75 %	84%	82%	83%
Median Progression-free survival	7.8 months	7.3 months	7.0 months	9.0 months

Derazantinib Pooled ⁵	Pemigatinib ⁶ (Incyte) FIGHT-202
23*	20
7%*	0%
79%*	40%
7.2 months	2.1 months

- FGFR2 fusions/rearrangements
- FGF/R non-fusion genetic alterations

- Derazantinib continues to show a well-manageable safety profile, with low rates of retinal side effects, stomatitis, hand-foot syndrome and nail toxicity.
- Overall, these results underscore the favorable benefit to risk profile of derazantinib as a monotherapy in bile duct cancer

*Objective response rate and disease control rate refer to 14 patients from studies ARQ 087-101 and FIDES-01 (Cohort 2), excluding patients from expanded access programs.

1. Basilea, data on file 2021. 2. Javle et al. J Clin Oncol 39, no. 3_suppl (January 20, 2021) 265-265. 3. Abou-Alfa et al. J Clin Oncol 39, no. 15_suppl (May 20, 2021) 4086-4086. 4. Goyal et al. Cancer Res 2021; 81, 13 Supplement, pp. CT010. 5. Droz Dit Busset et al., Annals of Oncology (2020) 31 (suppl_5): S1217-S1239. (Pooled analysis of clinical trials and early access programs). 6 Abou-Alfa et al. Lancet Oncol 2020;21(5):671-684.

Clinical program in urothelial cancer – FIDES-02

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

- Substudies (N≈200) in various treatment settings, including:
 - Post-chemotherapy/immunotherapy recurrence (second-line and post second-line)
 - First-line platinum-ineligible
 - Resistance to prior FGFR-inhibitor treatment
- Clinical supply agreement with Roche for atezolizumab
- Interim results in monotherapy and combination therapy with atezolizumab in patients refractory to prior FGFR-inhibitor treatment expected H2 2021*
- Exploring an intensified dose regimen of derazantinib in two cohorts of the study:
 - Focus on maximizing efficacy by using an intensified dose regimen of 400 mg per day
 - as monotherapy in a second-or post second-line setting in FGFR-inhibitor naïve patients
 - as monotherapy or in combination with atezolizumab in first-line cisplatin-ineligible patients
 - Supported by the observed safety and tolerability profile of derazantinib and by pharmacology data
- Initial results from cohorts utilizing 400 mg per day dose regimen expected H1 2022

*Using a dose regimen of 300 mg per day derazantinib ± 1200 mg atezolizumab every 3 weeks

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
- Exploring an intensified dose regimen of derazantinib 400 mg per day in monotherapy and in combination therapy
- Interim results in derazantinib monotherapy and recommended phase 2 dose of derazantinib in combination with ramucirumab/paclitaxel expected H1 2022
- Clinical supply agreement with Roche for atezolizumab
- Clinical trial collaboration and supply agreement with Lilly for ramucirumab

Oncology

Lisavanbulin (BAL101553)

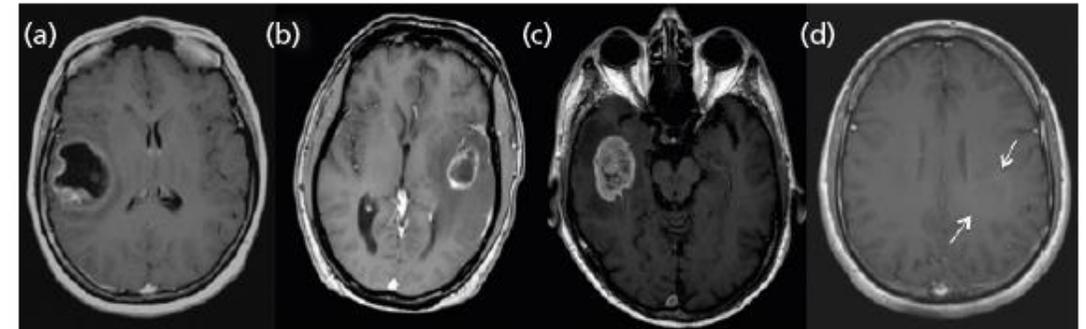
Glioblastoma
and other solid tumors



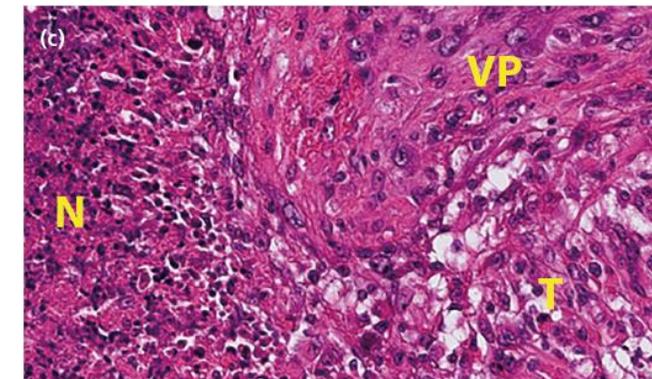
Unmet medical need in glioblastoma

- 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 codeletion³
- No molecular targeted therapy currently approved

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 glioblastoma; H&E stain.

100 μm

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

¹Poon MTC et al. 2020; Sci Rep 10, 11622; ²Hegi et al. NEJM 2005;352:997-1003

³Louis DN et al. Acta Neuropathol. 2016;131:803-820

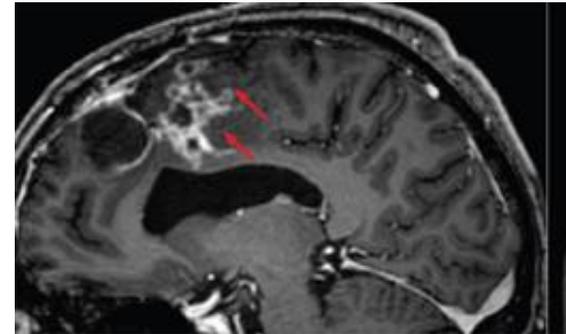
Biomarker-driven phase 2 study ongoing in recurrent glioblastoma

- EB1 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
- Results from phase 1 study with daily oral lisavanbulin in patients with recurrent glioblastoma (n= 20):^{1, 2}
 - Three patients with EB1-positive glioblastoma
 - Two of the EB1-positive patients with long-lasting clinical benefit, ongoing for more than 2 years
 - One exceptional response with >80% reduction in glioblastoma tumor size
 - No clear clinical benefit for EB1-negative patients
- Orphan drug designation granted for the treatment of malignant glioma
- Phase 2 interim results expected H2 2021

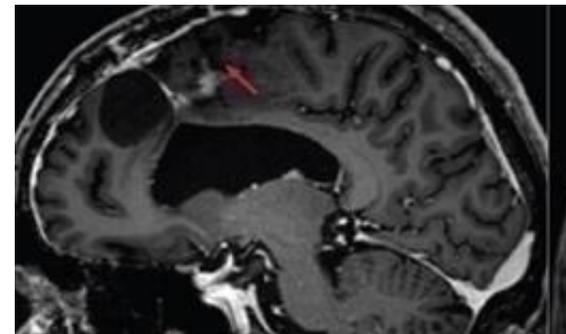
¹ Lopez et al. JCO 2019;37,15 suppl, 2025 (NCT02490800)

² Tiu et al. JCO 2021;39,15 suppl, TPS2068 (NCT02490800)

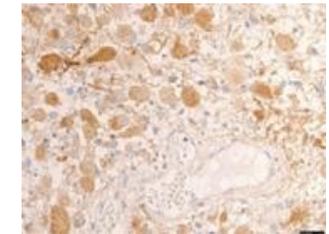
Glioblastoma tumor size reduction in an exceptional responder and EB1 staining of glioblastoma tissue compared to non-responding patients



Baseline (May 2018)



Post Cycle 12 (April 2019)



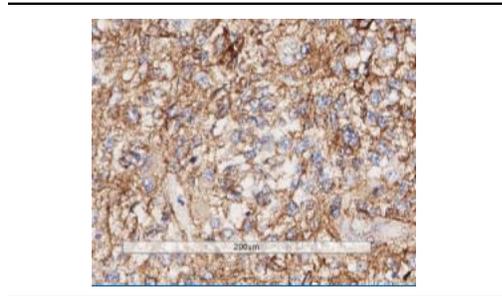
Responder



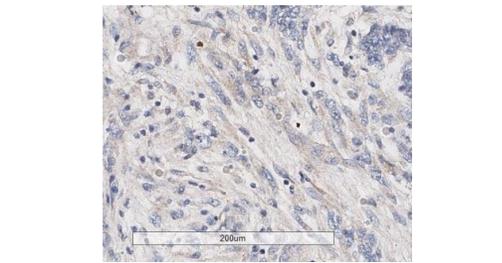
Non-responder

EB1-prevalence in glioblastoma and other cancer types

Example of an EB1-positive and EB1-negative glioblastoma tissue sample¹

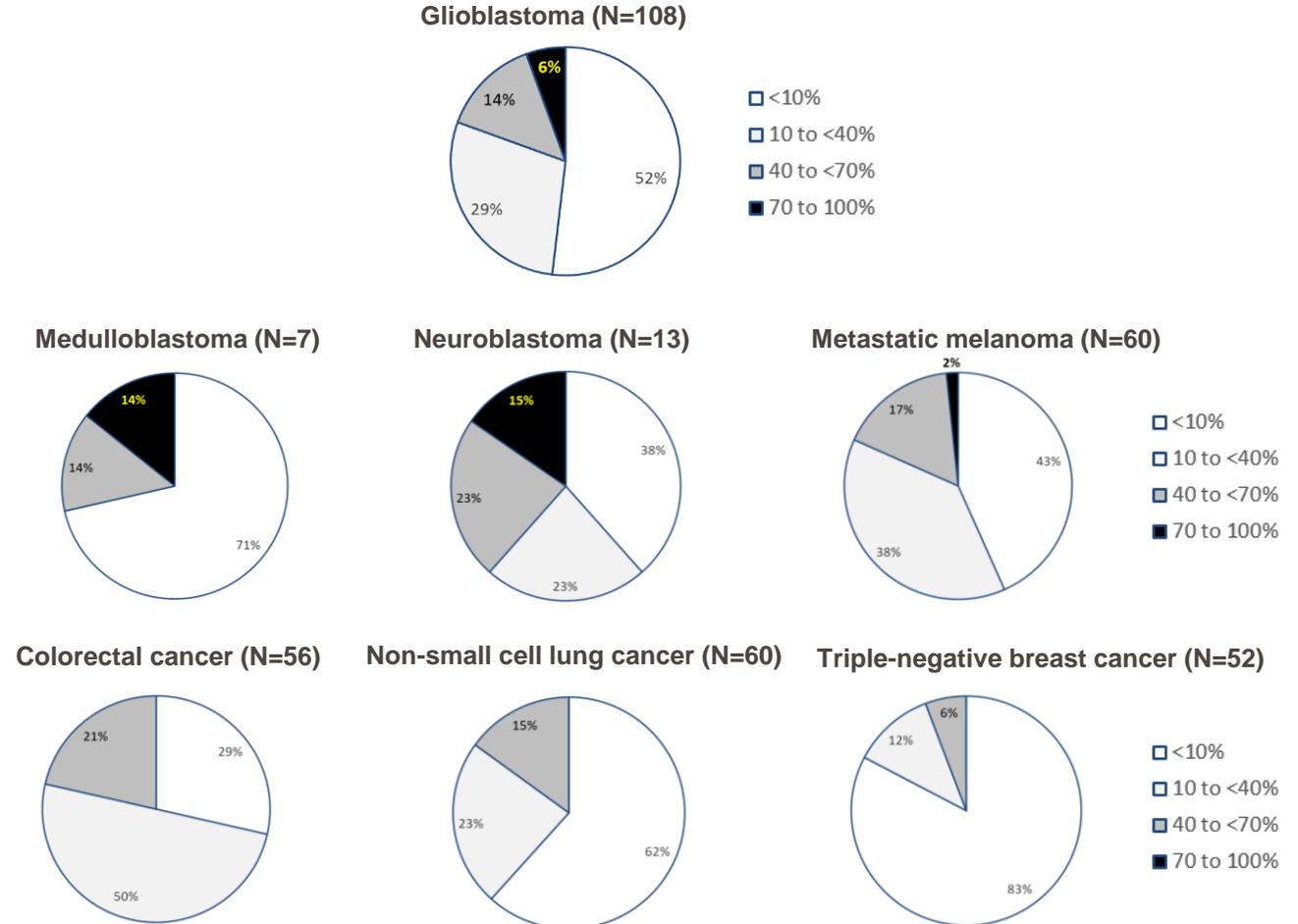


EB1-positive:
Tumor cells show moderate to strong EB1 staining



EB1-negative:
Absence of moderate to strong EB1 staining

Prevalence of moderate/strong EB1 staining in various tumor types¹



1.Skowronska et al. J Clin Oncol 39, no. 15_suppl (May 20, 2021) 3118-3118.

The pie-charts depict the percentages of tissue samples with moderate or strong EB1-staining in the following categories: <10% of tumor cells, 10 to <40% of tumor cells, 40 to <70% of tumor cells, ≥70% of tumor cells.

David Veitch

Chief Executive Officer

Outlook



Outlook 2021 / 2022

Cresemba® & Zevtera® — Increasing cash flows
By the end of 2022, Cresemba to be on the market in ~ 70 countries

		H1 2021	H2 2021	H1 2022	H2 2022
Isavuconazole		✓ Complete patient enrolment in phase 3 study in Japan	File NDA 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 gene fusions)			
		✓ Interim results (other FGFR2 genetic aberrations)		Topline results (other FGFR2 genetic aberrations)	
	FIDES-02 (urothelial cancer)		Interim results in monotherapy and combination therapy with atezolizumab in patients refractory to prior FGFR inhibitors	Interim results in monotherapy (400 mg/day) in 2nd-line FGFR-inhibitor naïve patients and atezolizumab combination in 1st-line cisplatin-ineligible patients	
	FIDES-03 (gastric cancer)			Interim results in monotherapy (400 mg/day) and recommended phase 2 dose with ramucirumab/paclitaxel	Interim efficacy 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		
Novel kinase inhibitor (for cancer therapy)			File IND application	Initiate phase 1 study	



Q & A



Thank you

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
- FGFR: **F**ibroblast **g**rowth **f**actor **r**eceptor
- FIDES: **F**ibroblast growth factor inhibition with **d**erazantinib in **s**olid tumors
- iCCA: **I**ntrahepatic **c**holangiocarcinoma
- IND: **I**nvestigational **n**ew **d**rug
- MSSA: **M**ethicillin-**s**usceptible ***S**ta**h**phylococcus **a**ureus*
- MRSA: **M**ethicillin-**r**esistant ***S**ta**h**phylococcus **a**ureus*
- NDA: **N**ew **d**rug **a**pplication
- ORR: **O**bjective **r**esponse **r**ate
- PFS: **P**rogression-**f**ree **s**urvival
- SAB: ***S**ta**h**phylococcus **a**ureus* **b**acteremia
- VEGFR2: **V**ascular **e**ndothelial **g**rowth **f**actor **r**eceptor **2**



Focused on Growth and Innovation

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