

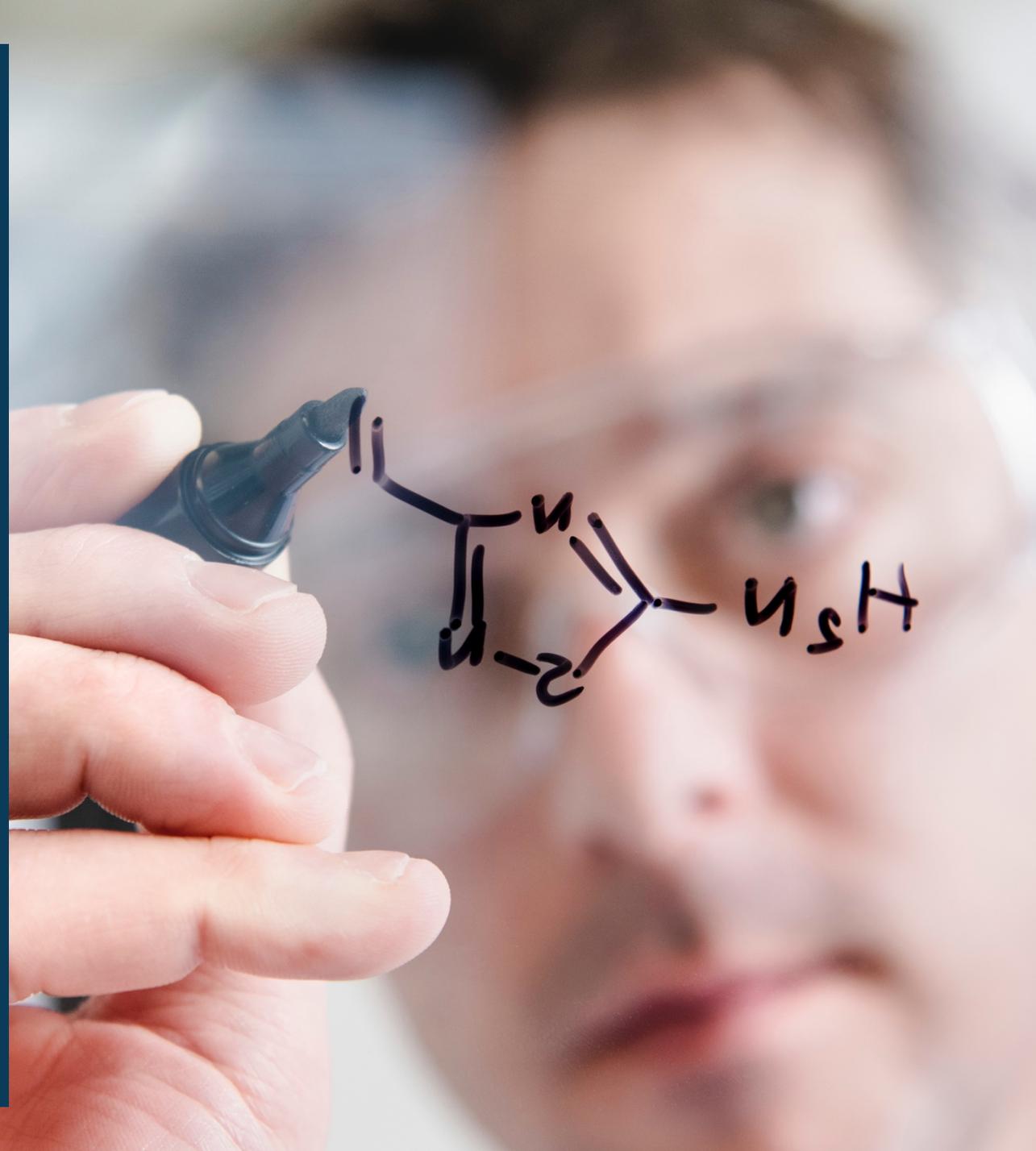


**Focused on  
Growth and Innovation**

**Half-year results 2020**

**August 11, 2020**

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**

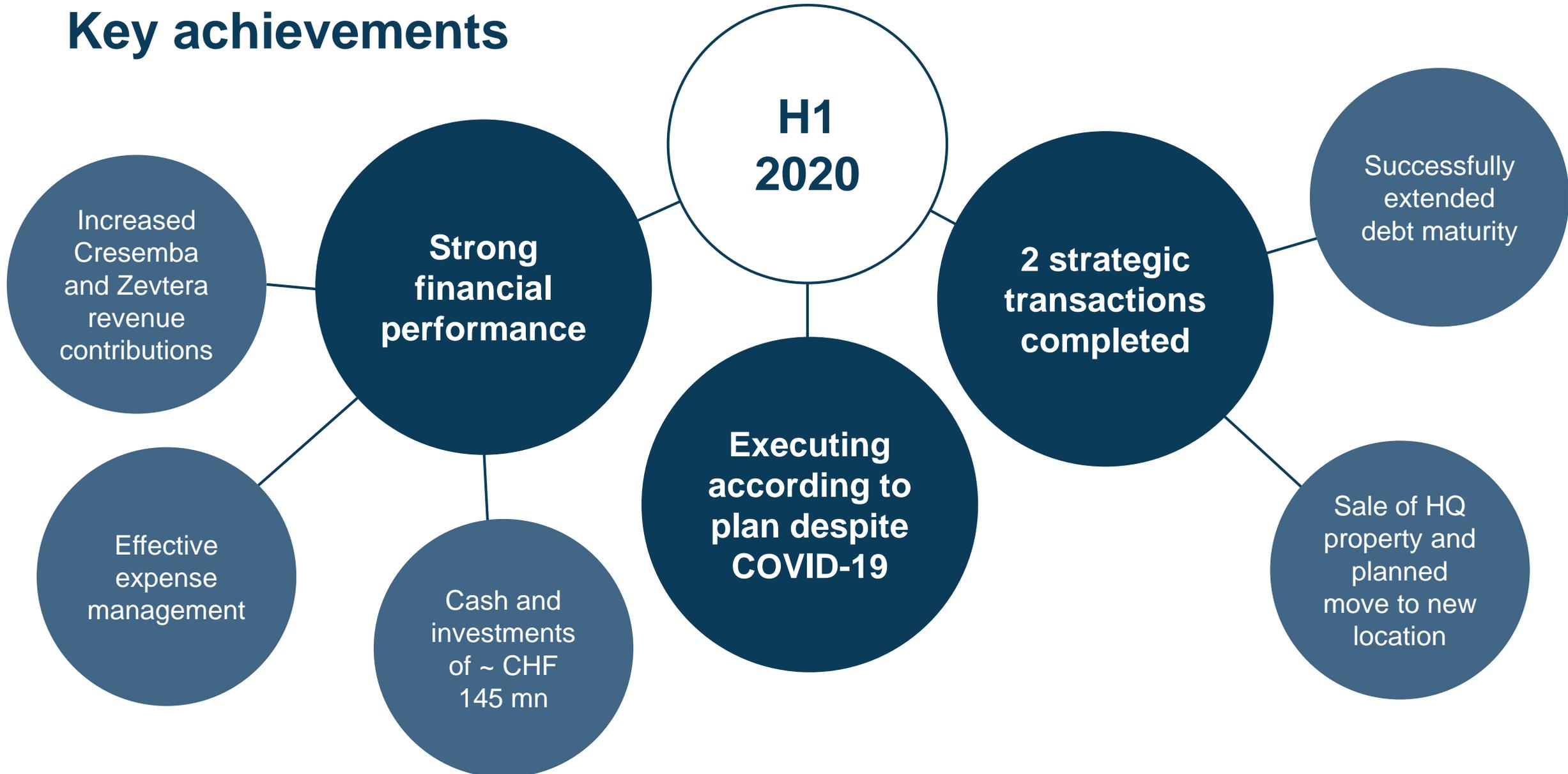


**Adesh Kaul**  
**CFO**

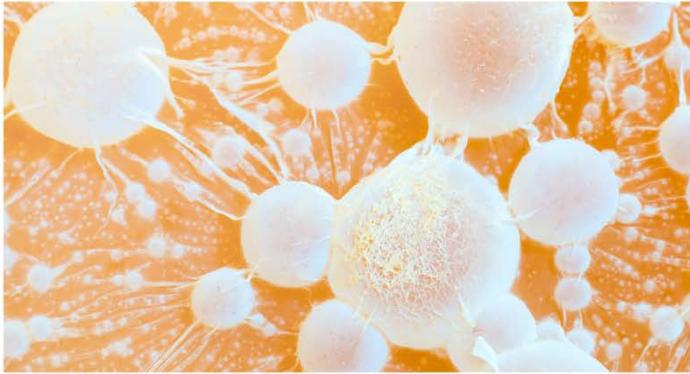


**Dr. Marc Engelhardt**  
**CMO**

# Key achievements



# Pipeline progress



## Derazantinib

- FIDES-01: completed patient enrolment in in cohort with FGFR2 gene fusion-positive iCCA
- FIDES-03: planning for advanced gastric cancer study start in Q3



## Lisavanbulin

- Preparing the start of a biomarker-driven study in patients with glioblastoma multiforme (GBM)



## Ceftobiprole

- FDA approved extension of treatment duration in ERADICATE study

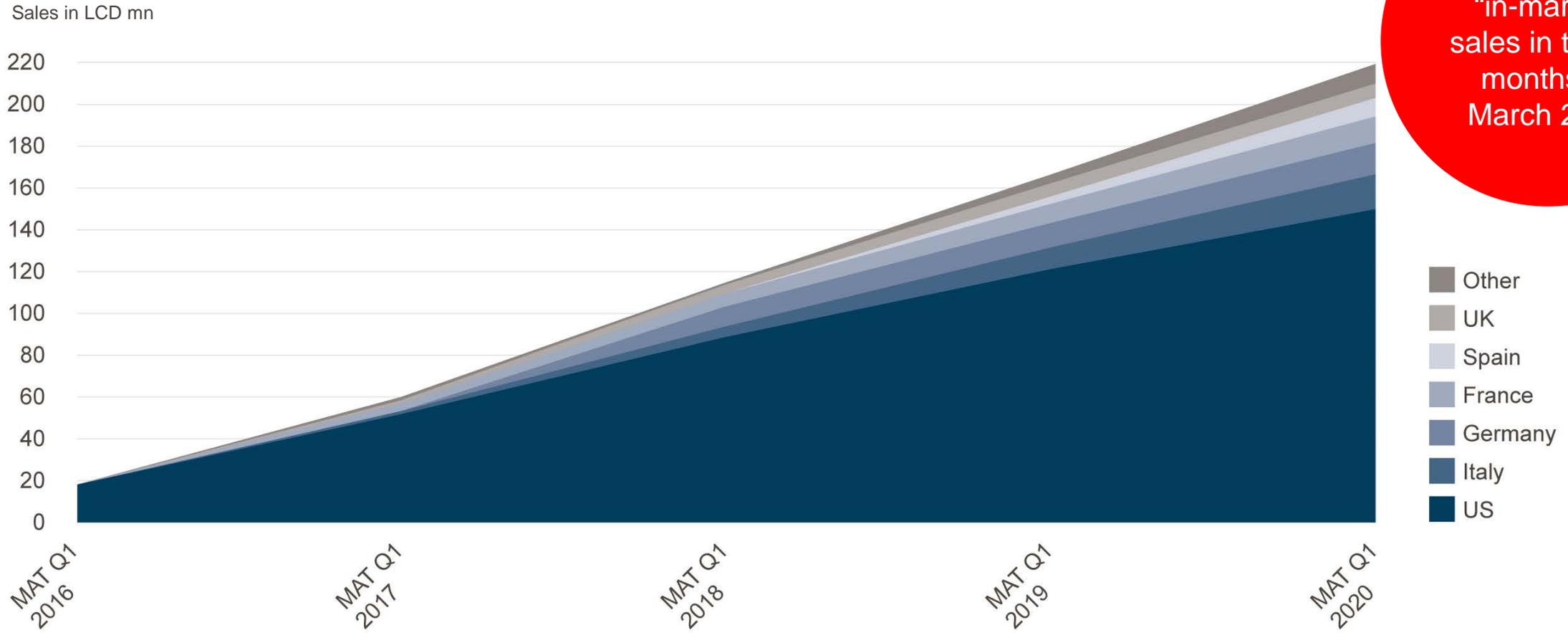
**Adesh Kaul**

Chief Financial Officer

Commercial &  
financial update



# Cresemba continues strong in-market sales uptake



USD 220 mn  
“in-market”  
sales in the 12  
months to  
March 2020

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

# 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®)

Double digit  
percentage  
royalties on  
sales by  
license  
partners

USD 1.1 bn  
in potential  
milestones  
remaining

Participation  
in sales of  
distribution  
partners  
through  
transfer price

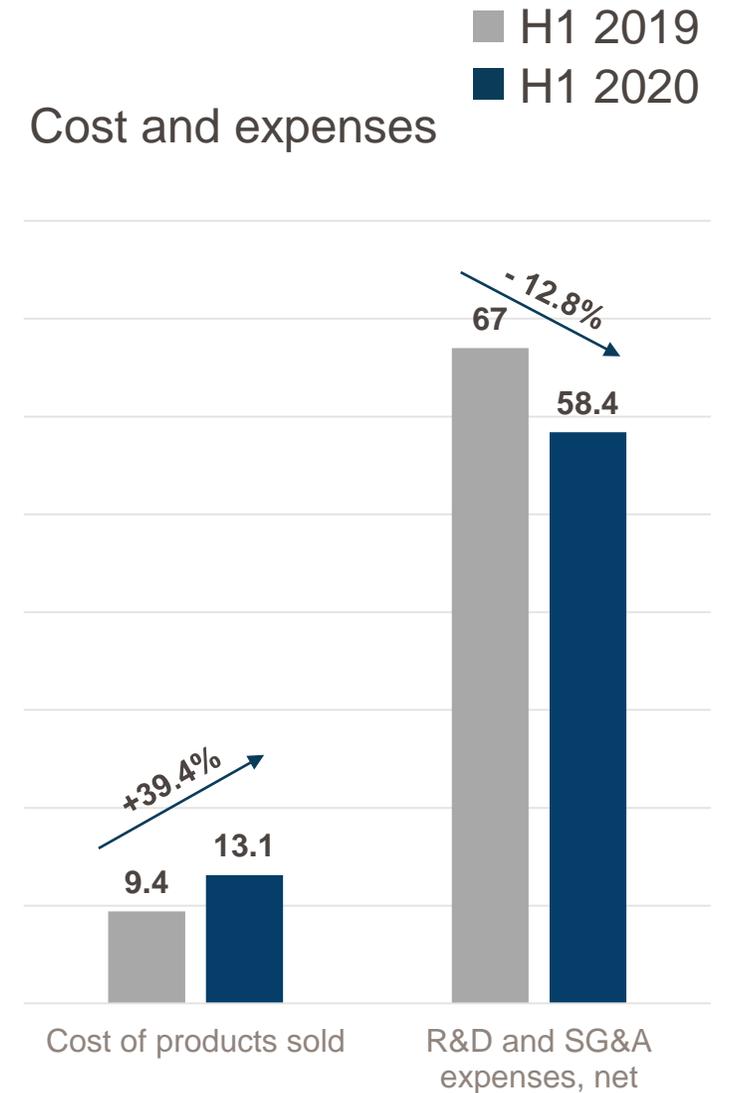
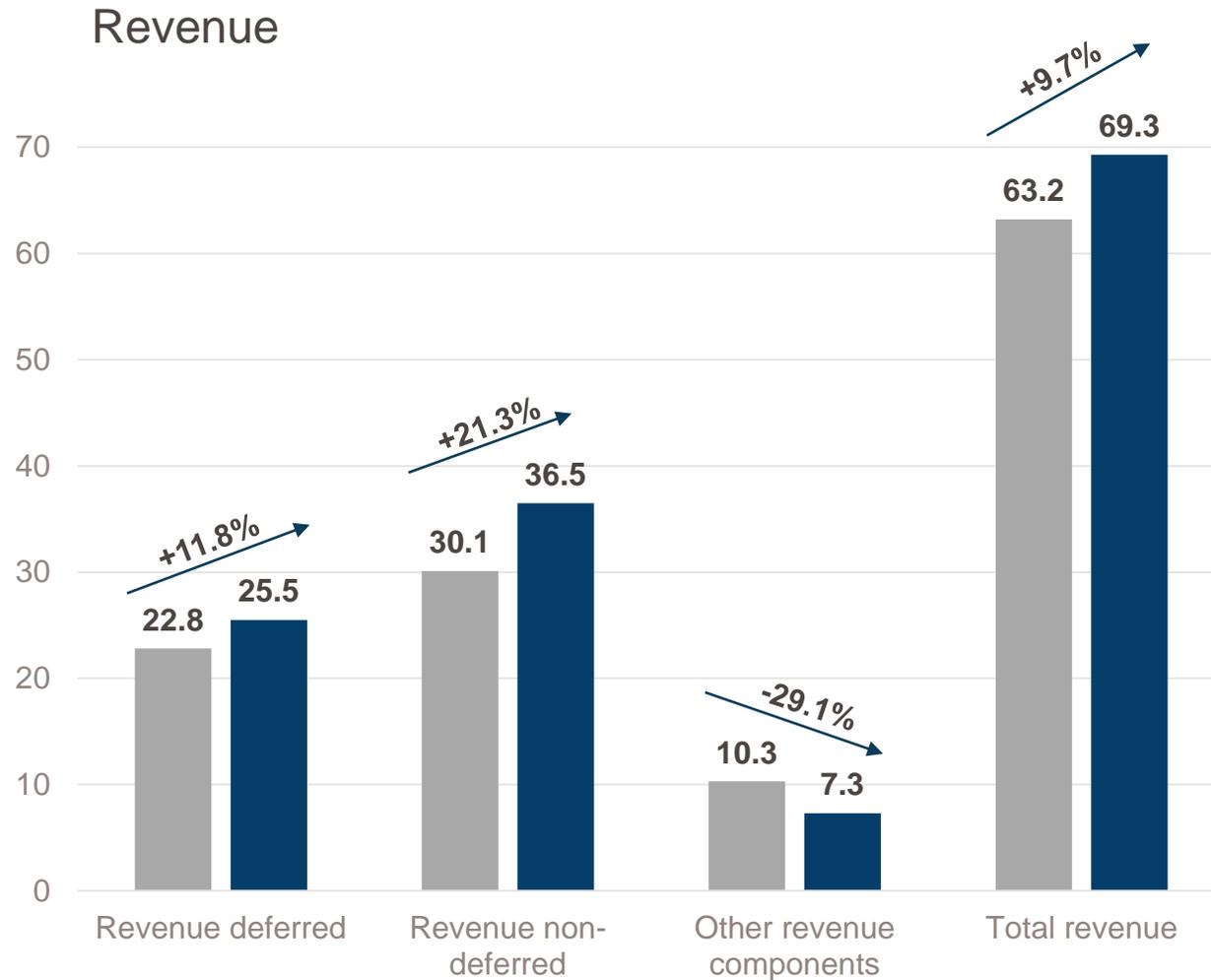
USD ~255 mn  
upfront and  
milestone  
payments  
received



# Financials



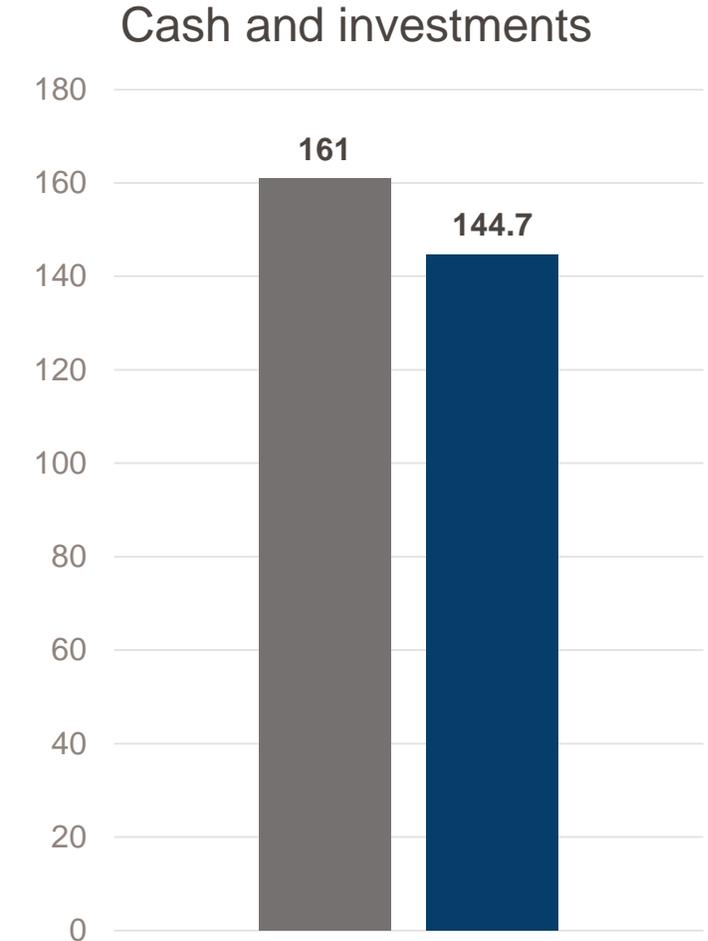
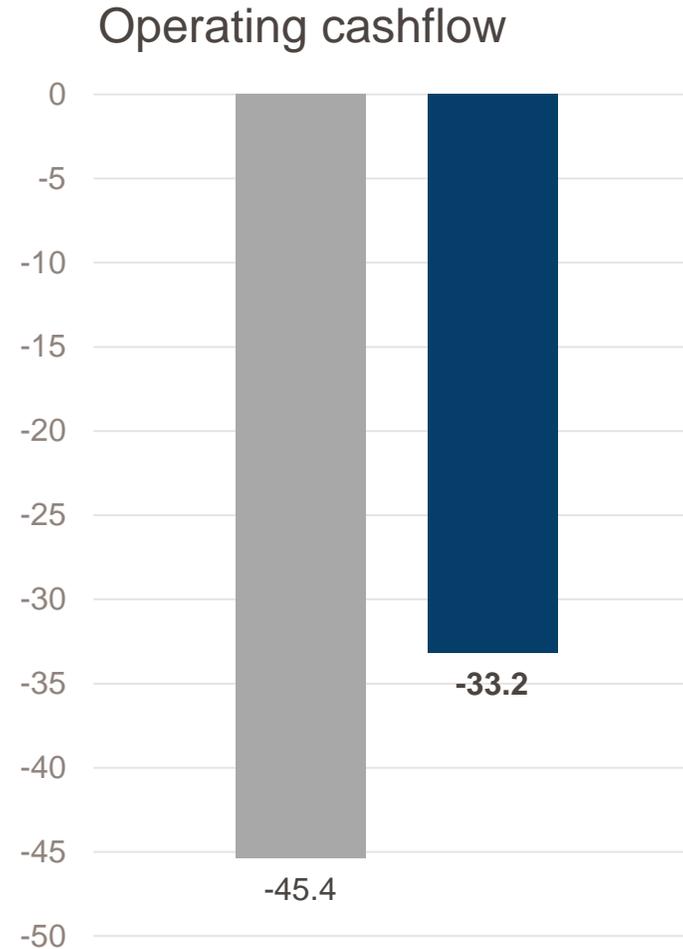
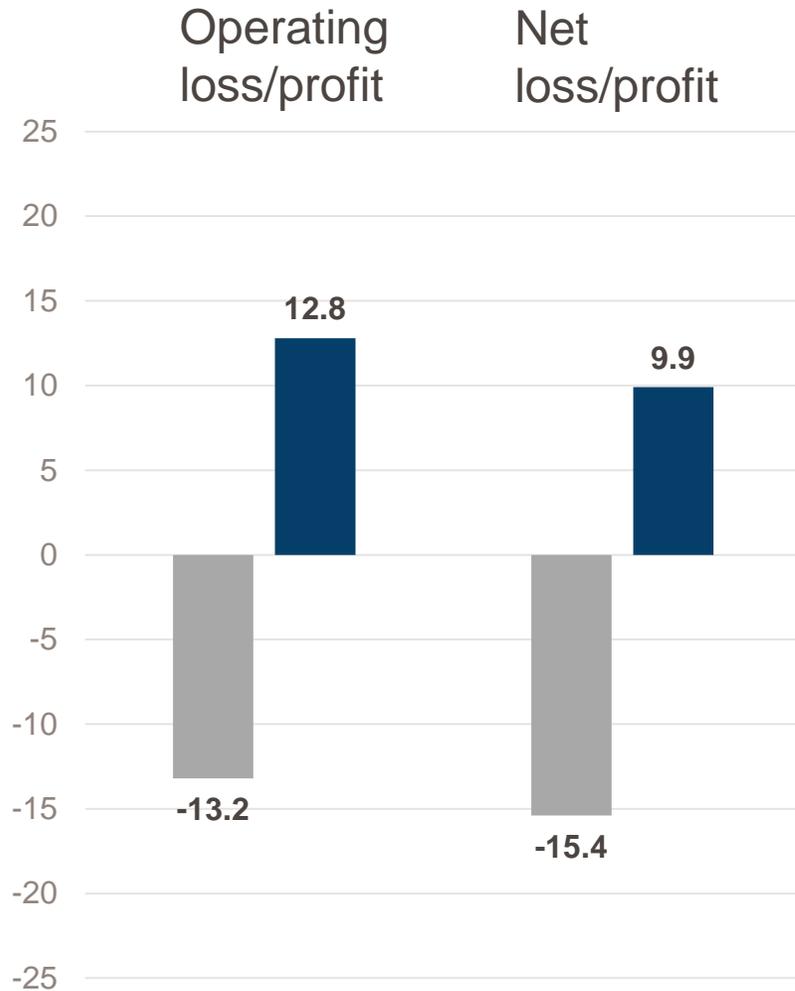
# Financial summary, in CHF mn (1/2)



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

# Financial summary, in CHF mn (2/2)

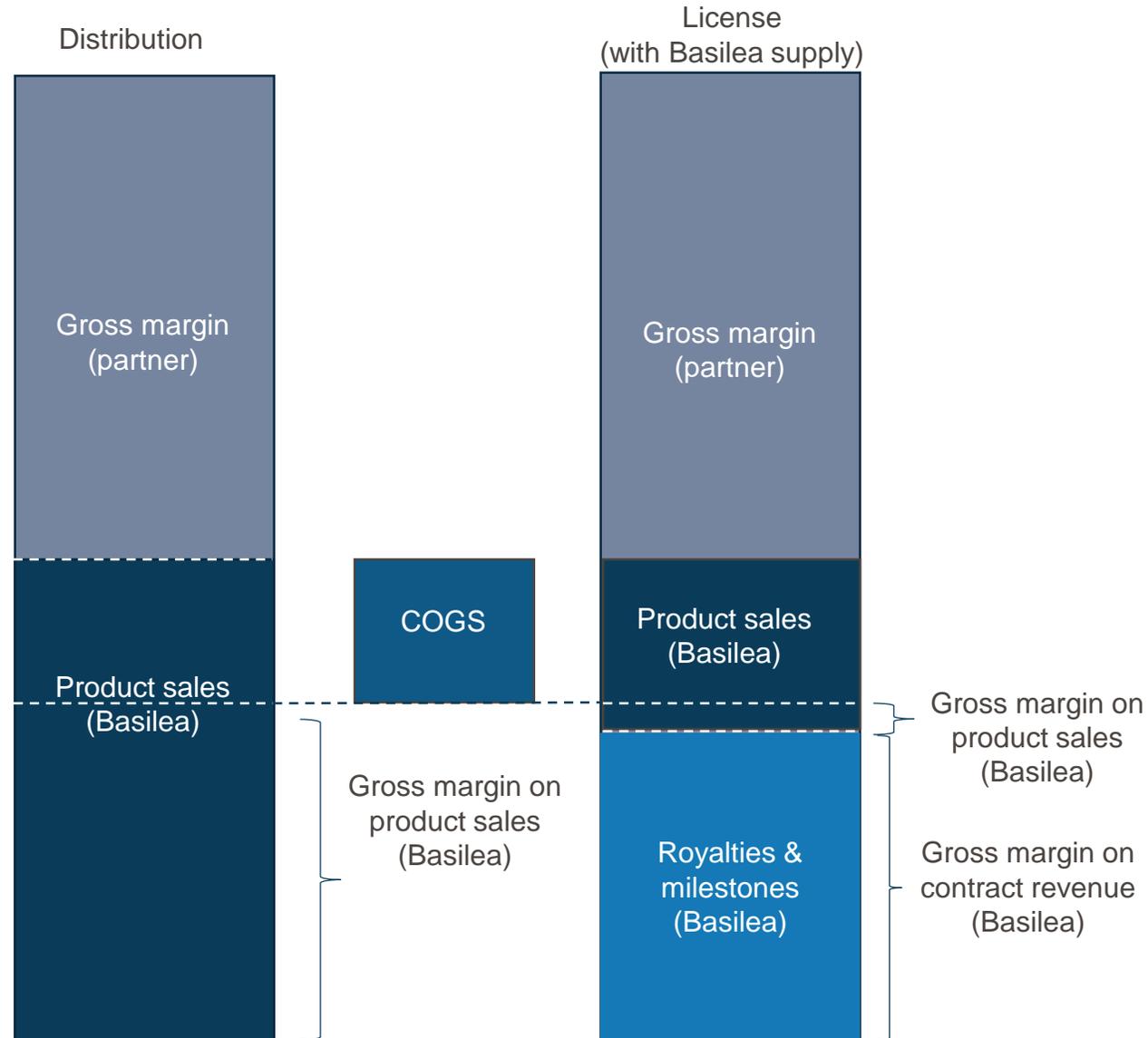
- H1 2019
- H1 2020
- YE 2019



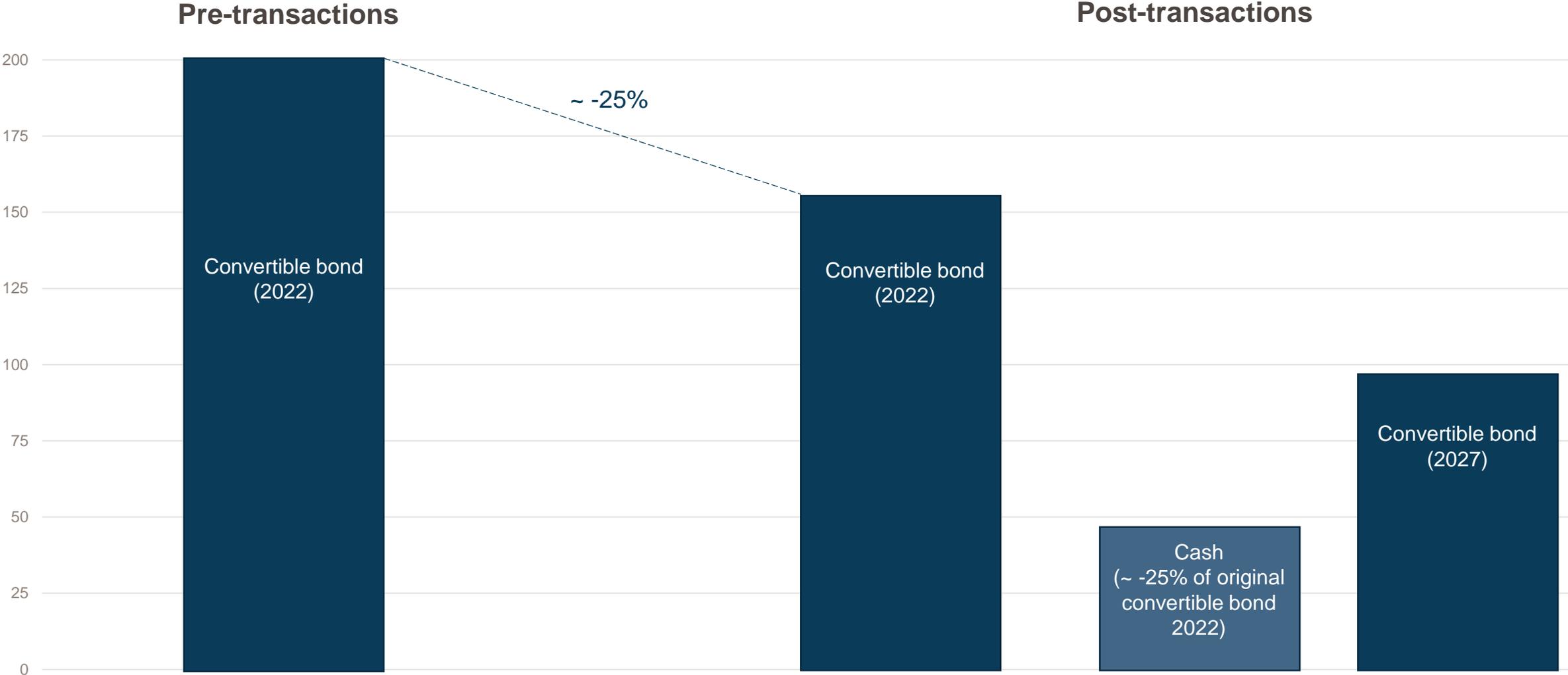
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

# Extension of Pfizer supply period

- Supply API and bulk Cresemba vials 2020/2021
    - Increase in product sales (in CHF)
    - Increase in cost of products sold (in CHF); economies-of-scale in supply to other partners
    - Lower gross margin (in % of product sales)
    - Temporary increase in working capital
- => Net positive cash flow over 2020/2021



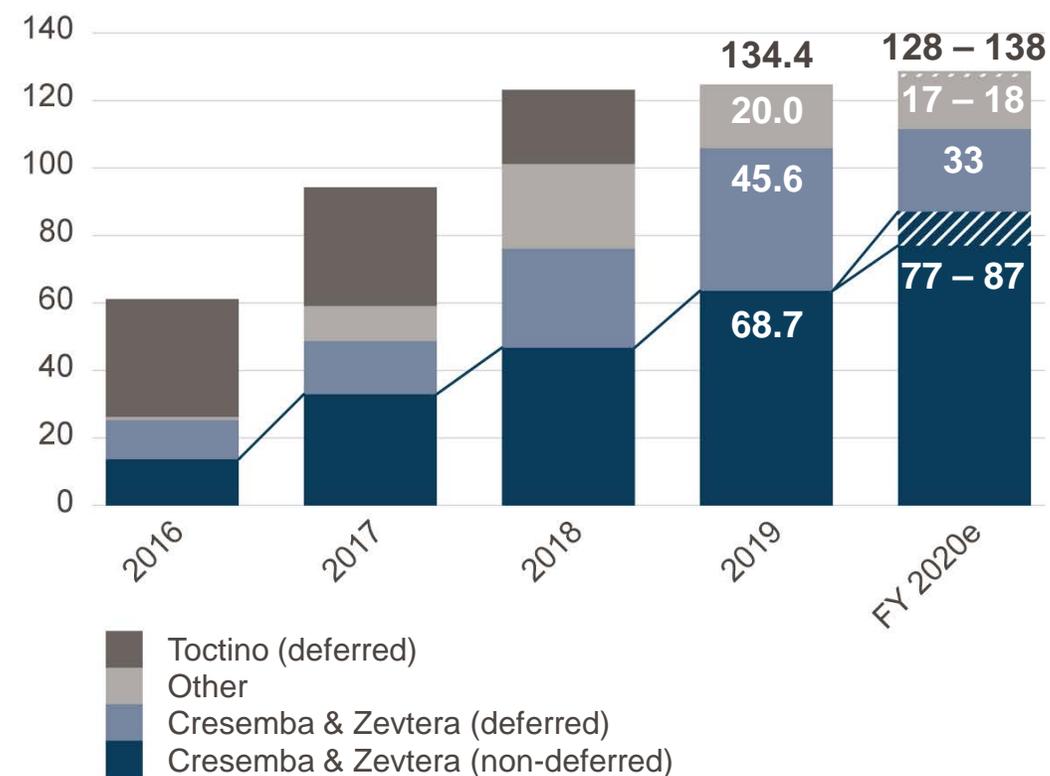
# Convertible bond transactions — successfully improved debt maturity profile (in CHF mn)



# Financial guidance

In CHF mn	FY 2020e (updated)	FY 2020e (initial)	FY 2019 (actual)
Total revenue	128 – 138	128 – 138	134.4
thereof: Contributions Cresemba® & Zevtera® non-deferred deferred	77–87 33	77-87 33	68.7 45.6
Operating loss	5-15	20-30	17.2
Cash and investments	150	100-110	161.0

Strong increase in non-deferred revenue contributions  
Y-o-Y, CHF mn



**Dr. Marc Engelhardt**

Chief Medical Officer

Clinical development  
update



Antibacterial

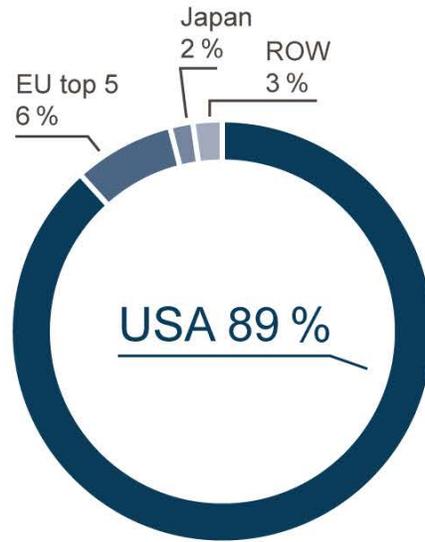
**Zevtera<sup>®</sup> / Mabelio<sup>®</sup>**  
**(Ceftobiprole)**

Severe bacterial infections

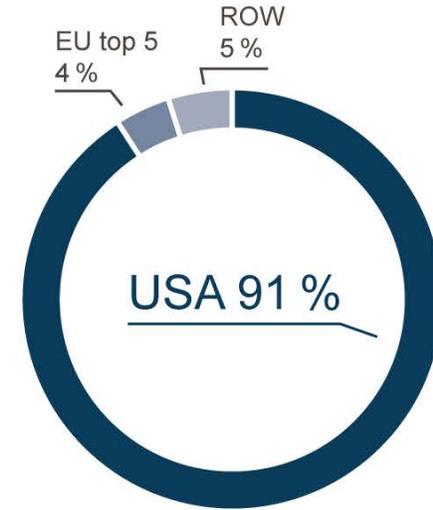


# The hospital anti-MRSA antibiotic market — A USD 3 bn market\* with the U.S. being the most important region

Daptomycin sales by region (2015, before LOE)



Ceftaroline sales by region (MAT Q1 2020)



\* Vancomycin, linezolid, teicoplanin, daptomycin, tigecycline, telavancin, ceftaroline, dalbavancin, oritavancin, and tedizolid

MRSA: Methicillin-resistant *Staphylococcus aureus*; LOE: Loss of exclusivity; ROW: Rest of world  
MAT: Moving annual total; Sales figures in USD, corrected for currency fluctuations; Source: IQVIA, March 2020

# 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 (up to USD 128 mn, ~70% of total program costs)

1. Acute Bacterial Skin and Skin Structure Infections (ABSSSI)<sup>1</sup> successfully completed



2. *Staphylococcus aureus* bacteremia (SAB)<sup>2</sup> ongoing, topline results from phase 3 study expected in Q1 2022



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

<sup>1</sup> Overcash JS et al. ECCMID 2020, abstract 1594. (NCT03137173)

<sup>2</sup> 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)<sup>1</sup>
- 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<sup>2</sup>
- 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

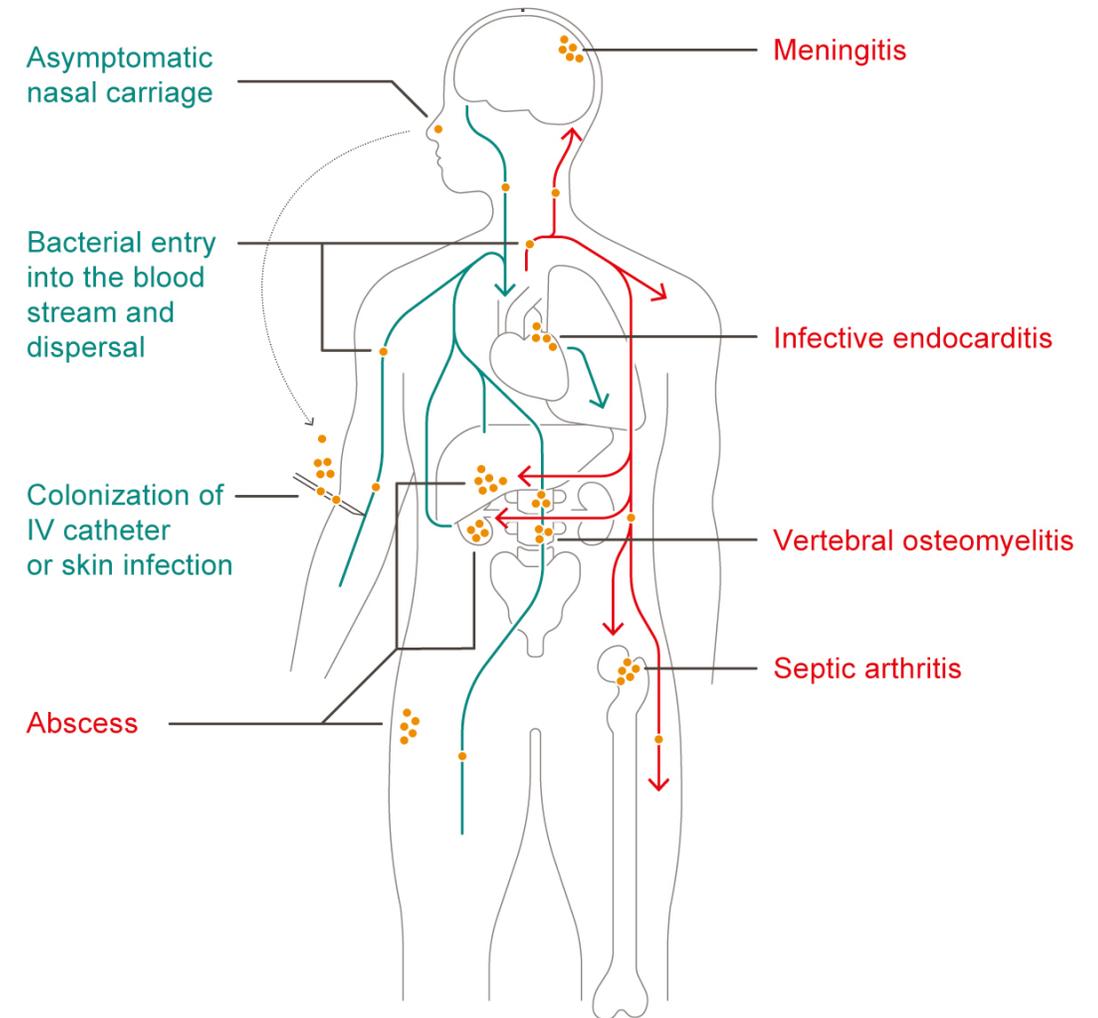
<sup>1</sup> MMWR, 2019;68:214–219.

<sup>2</sup> 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.

# Ceftobiprole key attributes for SAB treatment

- Beta-lactam antibiotic with rapid bactericidal activity against MSSA and MRSA<sup>1</sup>
- Superior activity profile in preclinical models of endocarditis compared to vancomycin and daptomycin<sup>2</sup>
- Low propensity for resistance development<sup>1</sup>
- Gram-negative coverage<sup>1</sup> in cases with polymicrobial infections
- Efficacy demonstrated in Phase 3 clinical trials in pneumonia and complicated skin and soft tissue infections<sup>1,3,4</sup>
- Established safety profile consistent with the cephalosporin class<sup>1,3</sup>

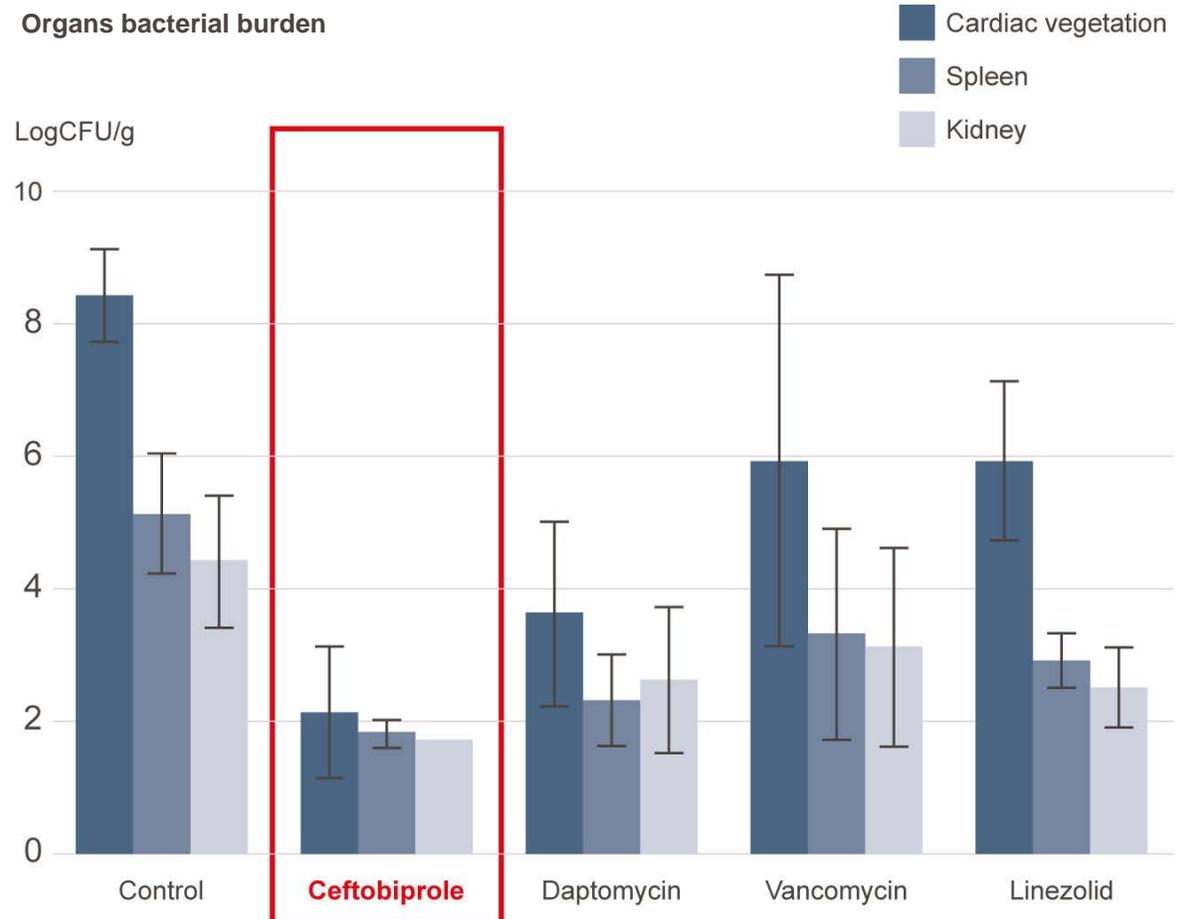
<sup>1</sup>Syed YY. *Drugs*. 2014;74:1523-1542.

<sup>2</sup>Tattevin P et al. *Antimicrob Agents Chemother*. 2010;54:610-613.

<sup>3</sup>Giacobbe DR et al. *Expert Rev Anti Infect Ther*. 2019;17:689-698.

<sup>4</sup>Overcash JS et al. *ECCMID 2020*, abstract 1594

## Comparative efficacy in a rabbit model of endocarditis



Organism titers in cardiac vegetations, spleens and kidneys of untreated and antibiotic treated rabbits infected with MRSA<sup>2</sup>

A microscopic image of cells, likely cancer cells, with an orange overlay. The cells are spherical and have a textured surface. Some cells are larger and more prominent than others. The background is a dense network of fine, fibrous structures. The overall color is a warm, orange-yellow.

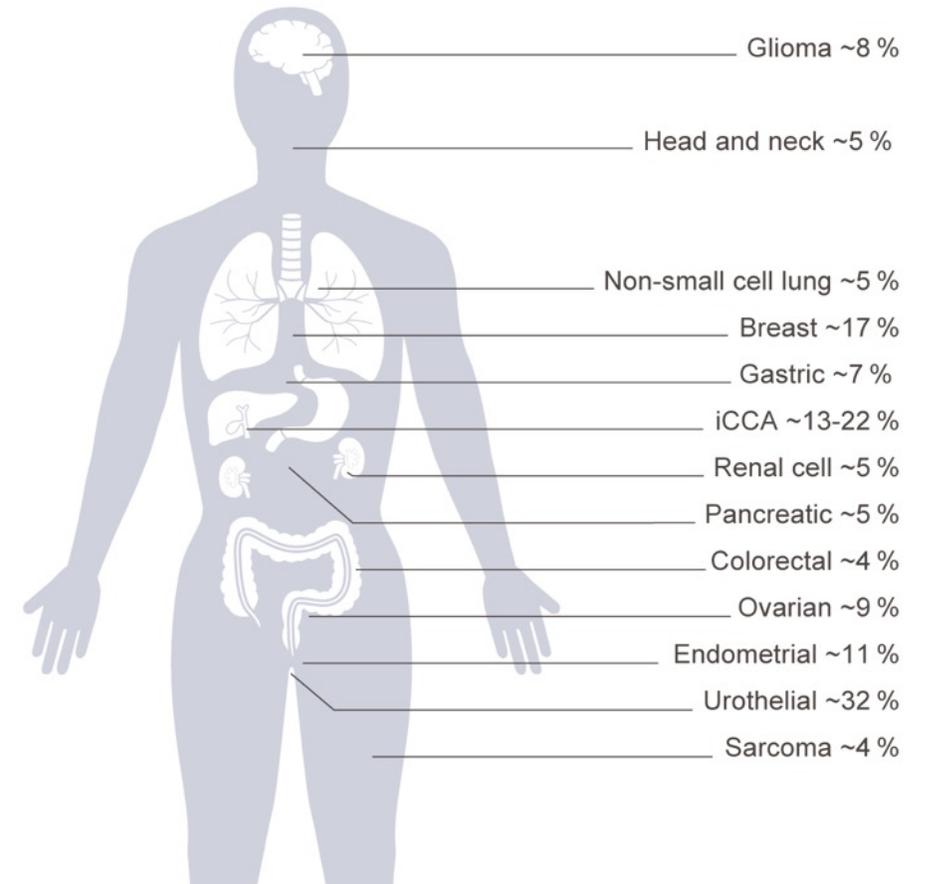
Oncology

# 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
- Two clinical studies ongoing (FIDES-01 in iCCA & FIDES-02 in urothelial cancer)
- Plan to start a multi-cohort phase 1/2 study (FIDES-03) in patients with advanced gastric cancer in Q3 2020



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

# Registrational phase 2 study in iCCA (FIDES-01)<sup>1</sup>

## **Cohort 1:** Patients with FGFR2 gene-fusion expressing iCCA (2nd line)

- Encouraging interim results, consistent with earlier phase 1/2 data<sup>2</sup>
- 21% ORR with six confirmed partial responses from 29 evaluable patients, 83% disease control rate
- Completed patient enrolment in July 2020
- Topline results expected H2 2020

## **Cohort 2:** Patients with FGFR2 gene mutations or amplifications

- Assessing the activity of derazantinib in a broader range of FGFR2-driven tumors
- Clinical benefit observed in a subset of iCCA patients in the phase 1/2 study<sup>2</sup>
- Aim to confirm phase 1/2 study results in a larger cohort of iCCA patients<sup>1</sup>
- Define the full therapeutic potential of derazantinib in iCCA with potential for differentiation
- Interim results expected H2 2020

<sup>1</sup> NCT03230318

<sup>2</sup> Droz Dit Busset et al. Annals of Oncology (2019) 30 (suppl\_5): abstract 3879 (NCT01752920)

# Clinical program in urothelial and gastric cancer

## FIDES-02<sup>1</sup> | Urothelial Cancer

*Multi-cohort Phase 1b/2 study of derazantinib monotherapy or in combination with atezolizumab (Tecentriq<sup>®</sup>) in patients with urothelial cancer expressing activating molecular FGFR 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
- First interim results expected in H2 2020

## FIDES-03 | Gastric Cancer

*Multi-cohort Phase 1b/2 study of derazantinib as monotherapy or in combination therapy with standard of care 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 and standard of care
  - Combination of derazantinib with atezolizumab (Tecentriq<sup>®</sup>)
- Expected start of enrolment in Q3 2020

<sup>1</sup> NCT04045613

Oncology

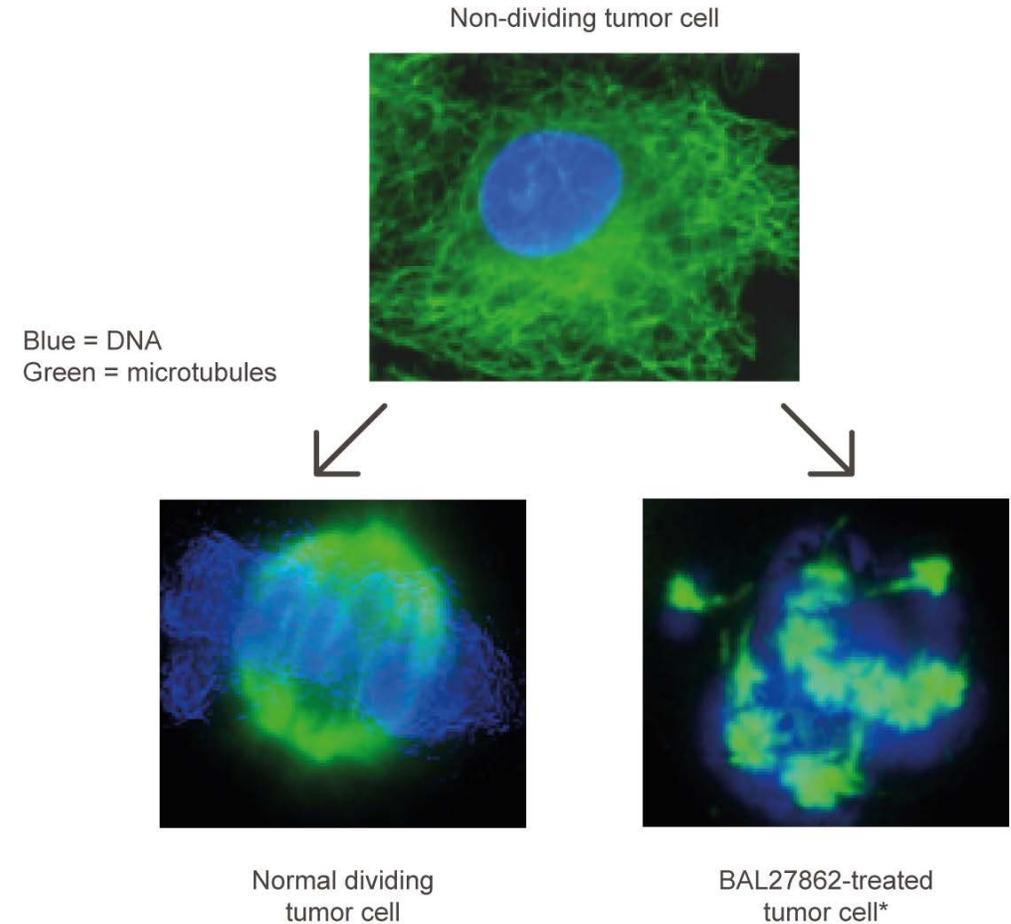
# Lisavanbulin (BAL101553)

Glioblastoma  
and other solid tumors



# Novel tumor checkpoint controller crossing the blood-brain barrier

- Novel compound inducing tumor cell death through spindle assembly checkpoint activation
- Targeting diverse tumor types resistant to standard therapeutic approaches
- Flexible dosing potential, including daily oral dosing
- Comprehensive biomarker program to optimize patient selection
- Crosses the blood-brain barrier with potent activity in brain tumor models alone and in combination
- Clinical program focused on glioblastoma (GBM) using a biomarker-driven approach



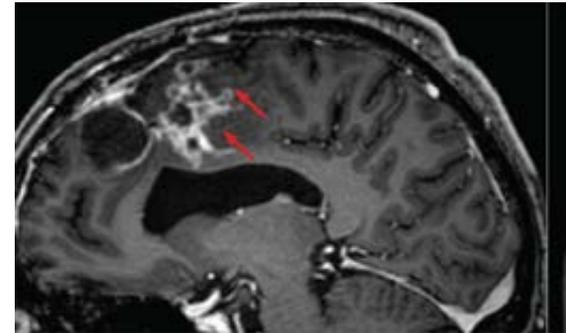
\* Lisavanbulin (BAL101553) is a prodrug of BAL27862

# EB1 — A potential response-predictive clinical biomarker for lisavanbulin

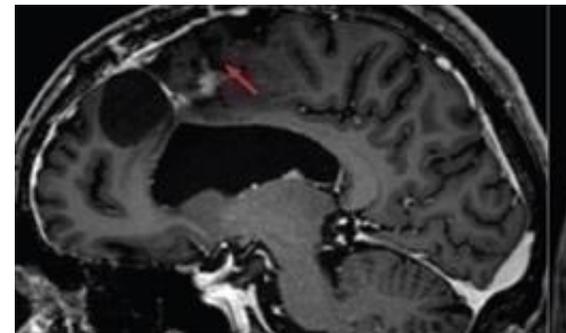
- EB1 (plus-end binding protein) is located on the microtubules and involved in microtubule dynamics and has been shown to be a response predictive marker for lisavanbulin in preclinical studies
- Strong EB1 staining was observed in a patient with an exceptional response to daily oral lisavanbulin in the phase 1 dose-escalation study in recurrent GBM<sup>1</sup>
  - Patient ongoing for more than two years
  - >80% reduction in GBM tumor size
- Biomarker-driven phase 2 study in patients with recurrent GBM using EB1-positivity as patient selection criterion, anticipated to start in the next few months

<sup>1</sup> 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)

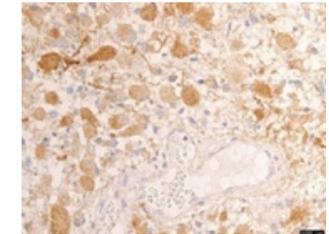
## GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



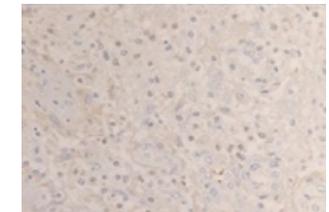
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



Non-responder

**David Veitch**

Chief Executive Officer

Outlook



# Outlook 2020 / 2021

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

	H1 2020	H2 2020	H1 2021	H2 2021
<b>Isavuconazole</b>		Complete patient enrolment in phase 3 study in Japan		Topline results from phase 3 study in Japan
<b>Ceftobiprole</b>				Complete patient enrolment in SAB phase 3 study
<b>Derazantinib</b>	<b>FIDES-01 (iCCA)</b>	✓ Complete patient enrolment in phase 2 registrational study (FGFR2 fusions) Topline results (FGFR2 fusions)		
		Interim results (other FGFR2 gene aberrations)		Topline results (other FGFR2 gene aberrations)
	<b>FIDES-02 (urothelial cancer)</b>	Safety data and recommended phase 2 dose (RP2D) for derazantinib/Tecentriq combination and expansion into phase 2	Interim results in derazantinib monotherapy	Interim results in combination therapy with Tecentriq
	<b>FIDES-03 (gastric cancer)</b>	✓ Clinical supply agreement with Roche in gastric cancer Start of phase 1/2 study		Interim results
<b>Lisavanbulin (Oral)</b>	✓ Full results of phase 1 study in glioblastoma* Start phase 2 biomarker-driven glioblastoma study		Interim results from phase 2 biomarker-driven glioblastoma study	Topline results from phase 2 biomarker-driven glioblastoma study
			Complete patient enrolment in phase 1 study in newly diagnosed glioblastoma	

\* Accepted for ESMO poster presentation (Sept. 2020)



# Q & A

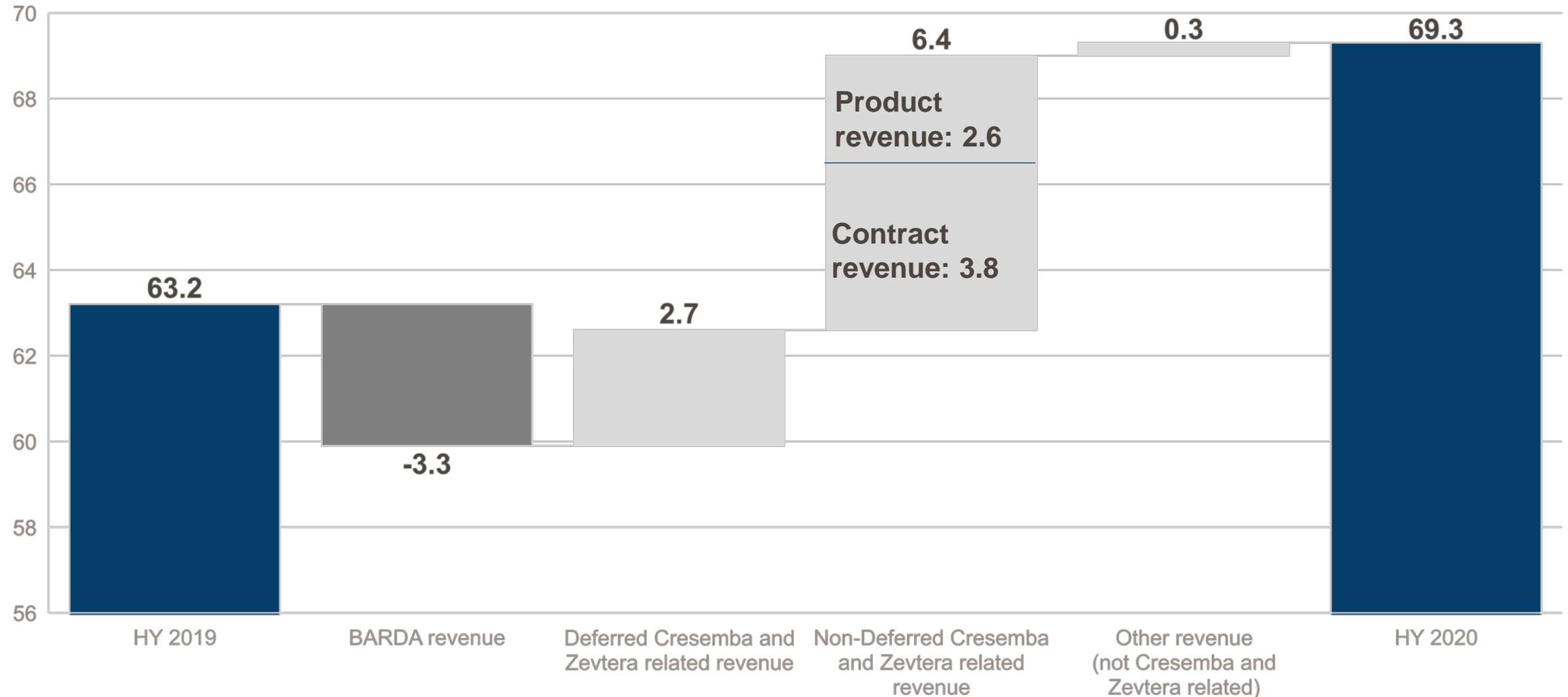


# Thank you

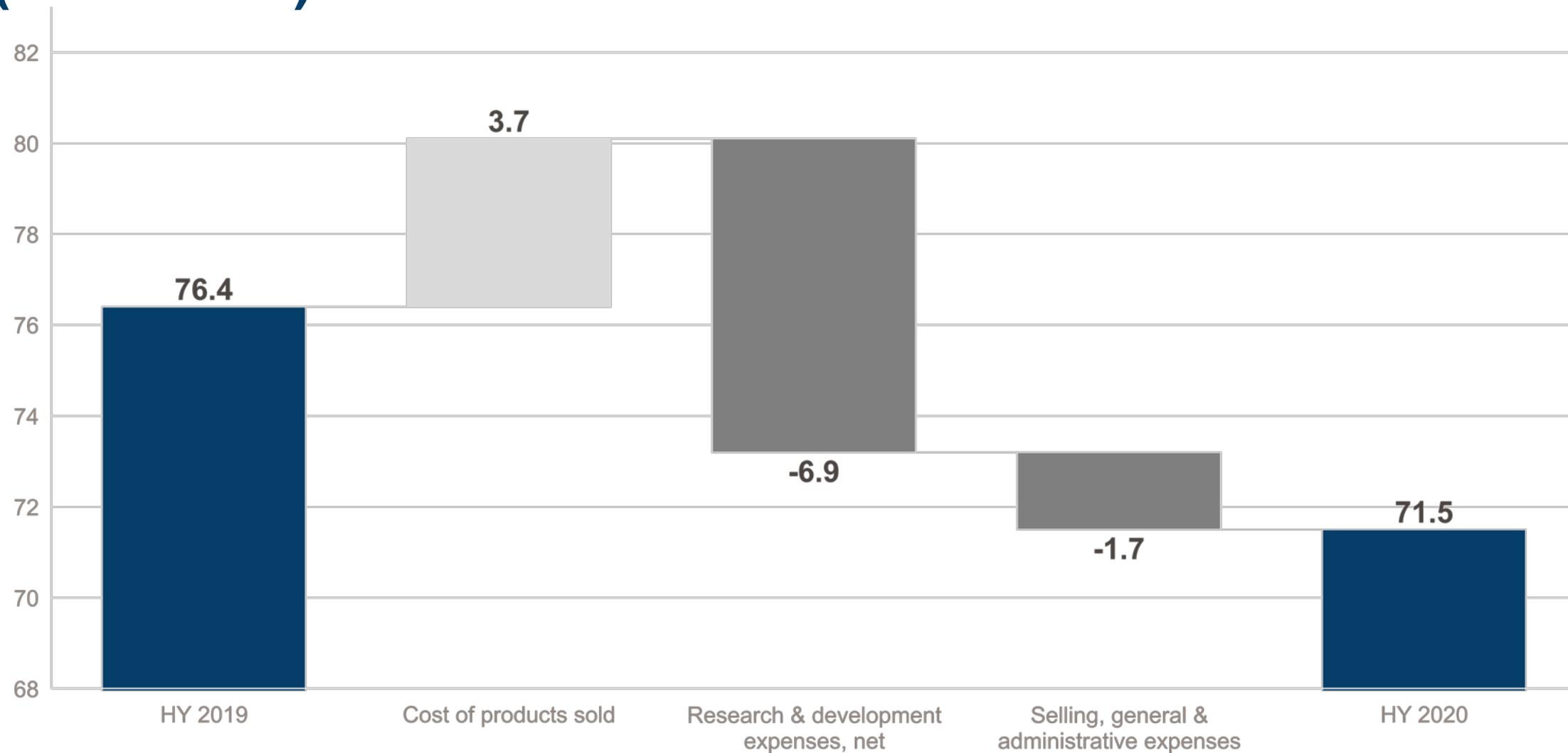


# Appendix

# Revenue bridge H1 2019 to H1 2020 (in CHF mn)



# Cost and operating expenses bridge H1 2019 to H1 2020 (in CHF mn)



# Glossary

- ABSSSI: Acute bacterial skin and skin structure infections
- CSF1R: Colony-stimulating Factor 1 Receptor
- FGFR: Fibroblast Growth Factor Receptor
- GBM: Glioblastoma multiforme
- iCCA: Intrahepatic cholangiocarcinoma
- MRSA: methicillin-resistant *Staphylococcus aureus*
- MSSA: methicillin-susceptible *Staphylococcus aureus*
- SAB: *Staphylococcus aureus* bacteremia
- VEGFR2: Vascular Endothelial Growth Factor Receptor 2



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