



**Focused on  
Growth and Innovation**

**“Patients are at the heart  
of what we do”**

Investor presentation  
January 20, 2021



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## Executive summary



# Experienced leadership team



**David Veitch** CEO

Joined 2014

Previous roles:



**Adesh Kaul** CFO

2009



**Marc Engelhardt** MD, Ph.D. CMO

2010



**Gerrit Hauck** Ph.D. CTO

2018



**Laurenz Kellenberger** Ph.D. CSO

2000

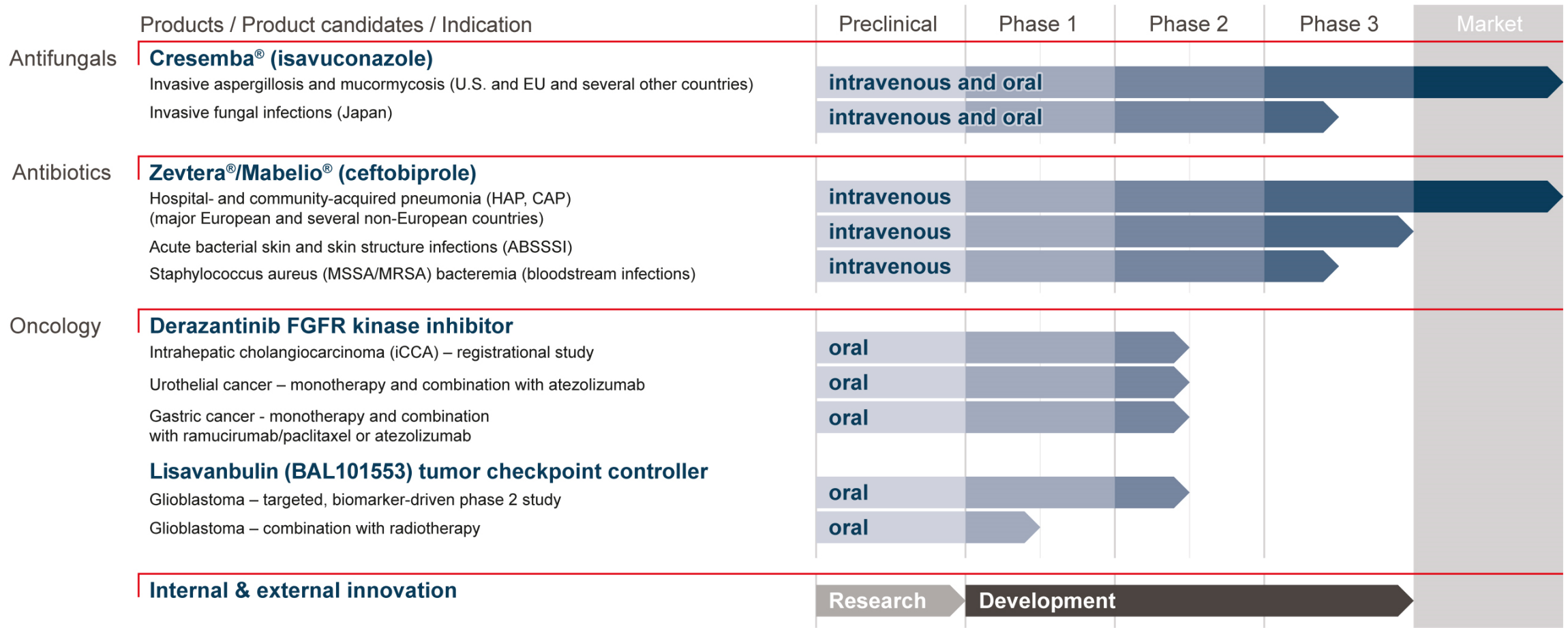


# At a glance

- Well funded, commercial-stage biopharmaceutical company with significantly growing cash flows from commercialized products
- Focused in the areas of oncology and infectious diseases
- Potential for sustainable growth and value creation based on commercialized brands and an innovative pipeline
- Experienced people with the proven expertise to take compounds from research to market
- Two revenue generating hospital anti-infective brands, Cresemba® and Zevtera® and two clinical oncology drug candidates
- Recognized ability to establish and manage partnerships in both the development and commercial phase, providing access to international markets
- Listed on SIX Swiss Stock Exchange, SIX: BSLN
- Based in life sciences hub, Basel, Switzerland



# Potential for sustainable growth and value creation based on commercialized brands and innovative pipeline

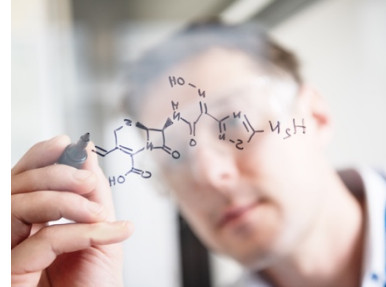


# Our strategy



## Foster

Foster an agile organisation based on a dynamic and open culture



## Focus

Focus on continuously increasing cash flow from our two commercial-stage hospital anti-infective brands, Cresemba<sup>®</sup> and Zevtera<sup>®</sup>



## Leverage

Leverage our expertise in bringing drugs from research to market by utilising appropriate partnerships with established organisations



## Invest

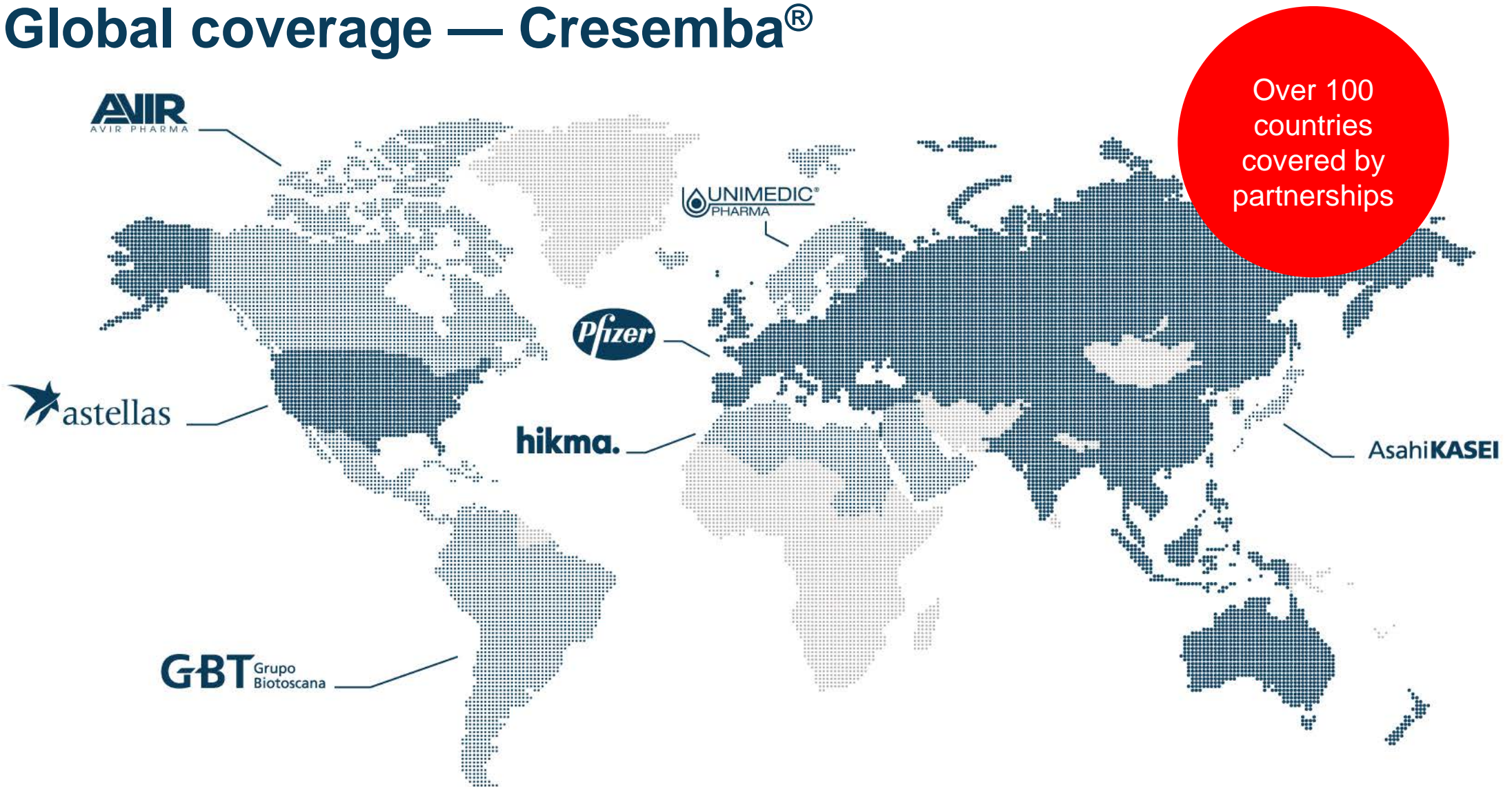
Invest in our clinical portfolio of targeted, small molecule, oncology drug candidates and the phase 3 ceftobiprole program



## Innovate

Continue to broaden our R&D pipeline through both internal and external innovation

# Global coverage — Cresemba®





# 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 250 mn  
upfront and  
milestone  
payments  
received



**Five reasons  
to invest**



# Five reasons to invest



## Growth

Well funded with increasing and sustainable cash flow through commercialized brands



## Prospects

Opportunity to share in pipeline value creation and proven approach to the successful commercialization of products around the world



## Leadership

Experienced team working in an agile culture able to turn pipeline projects into revenue generating brands



## Partnerships

Proven ability to build successful partnerships in research, development and commercialization with leading academic, governmental and industrial organisations



## Focus

One of the few biopharmaceutical companies in the world focused on the development and commercialization of targeted oncology small molecules and new antibiotics and antifungals

Antifungal

**Cresemba<sup>®</sup>**  
**(isavuconazole)**

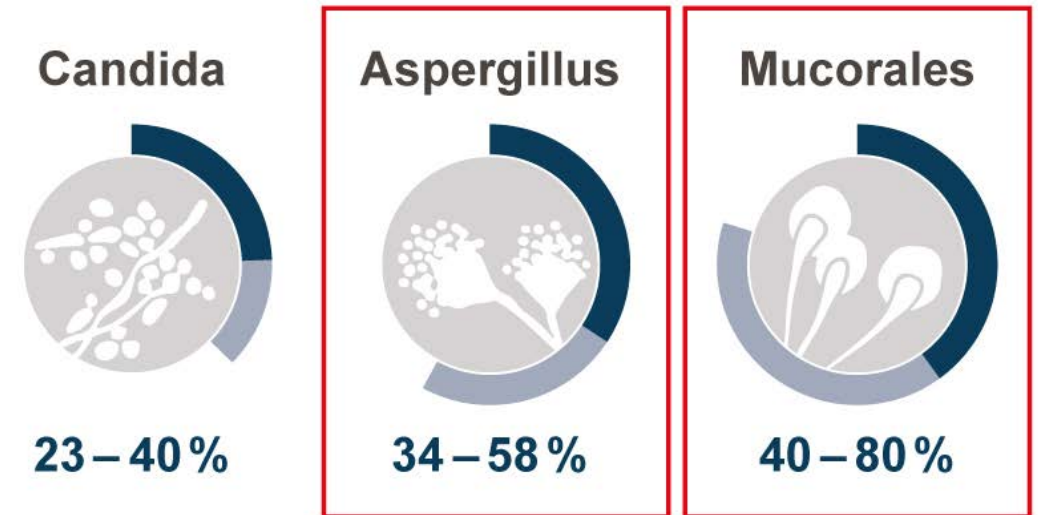
Invasive mold infections



# The market — Invasive fungal infections

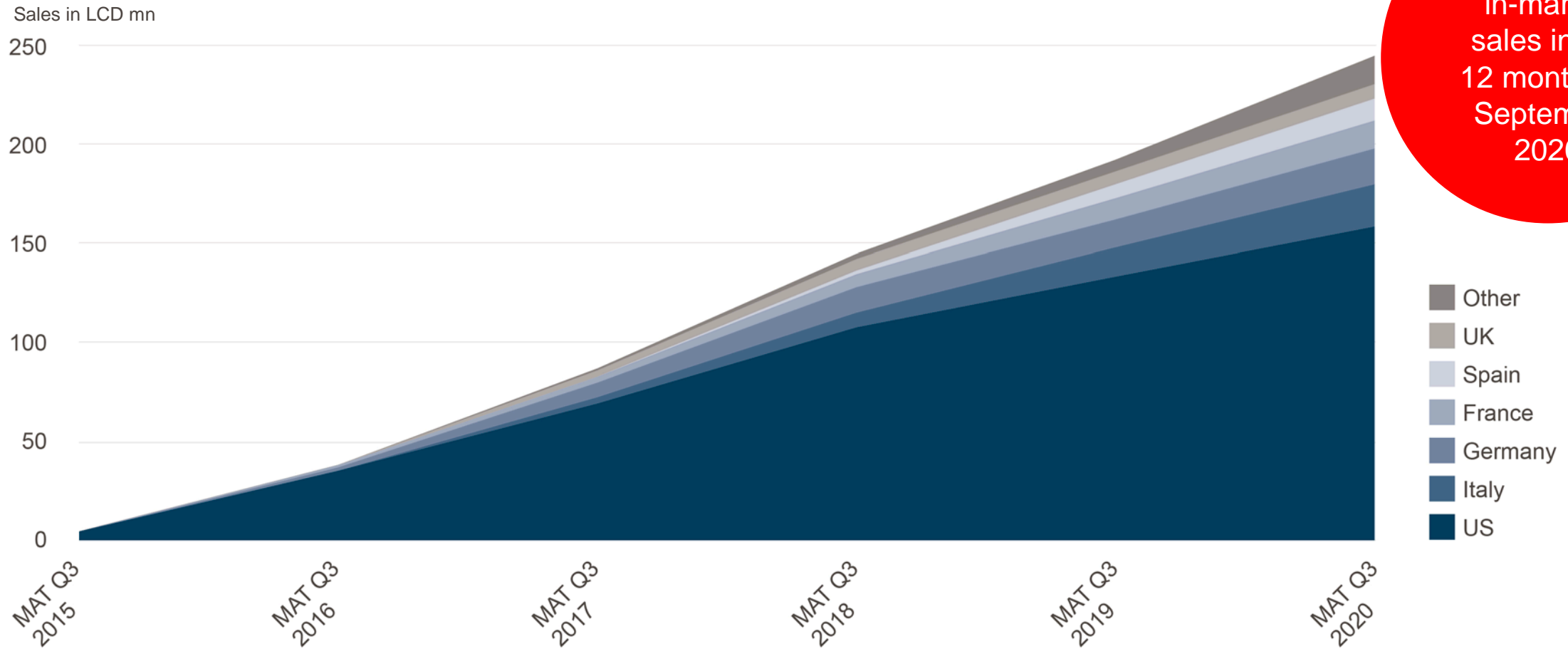
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand
- Mucorales infections on the rise – doubled from 2000 to 2013
- Limitations of current therapies (spectrum of activity, toxicity, effective plasma levels) drive the need for new agents

## Mortality rates for invasive fungal infections\*\*



\*\*Kullberg/Arendrup *N Engl J Med* 2015, Baddley *Clin Infect Dis* 2010, Roden *Clin Infect Dis* 2005, Greenberg *Curr Opin Infect Dis* 2004

# Cresemba continues strong in-market sales uptake



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

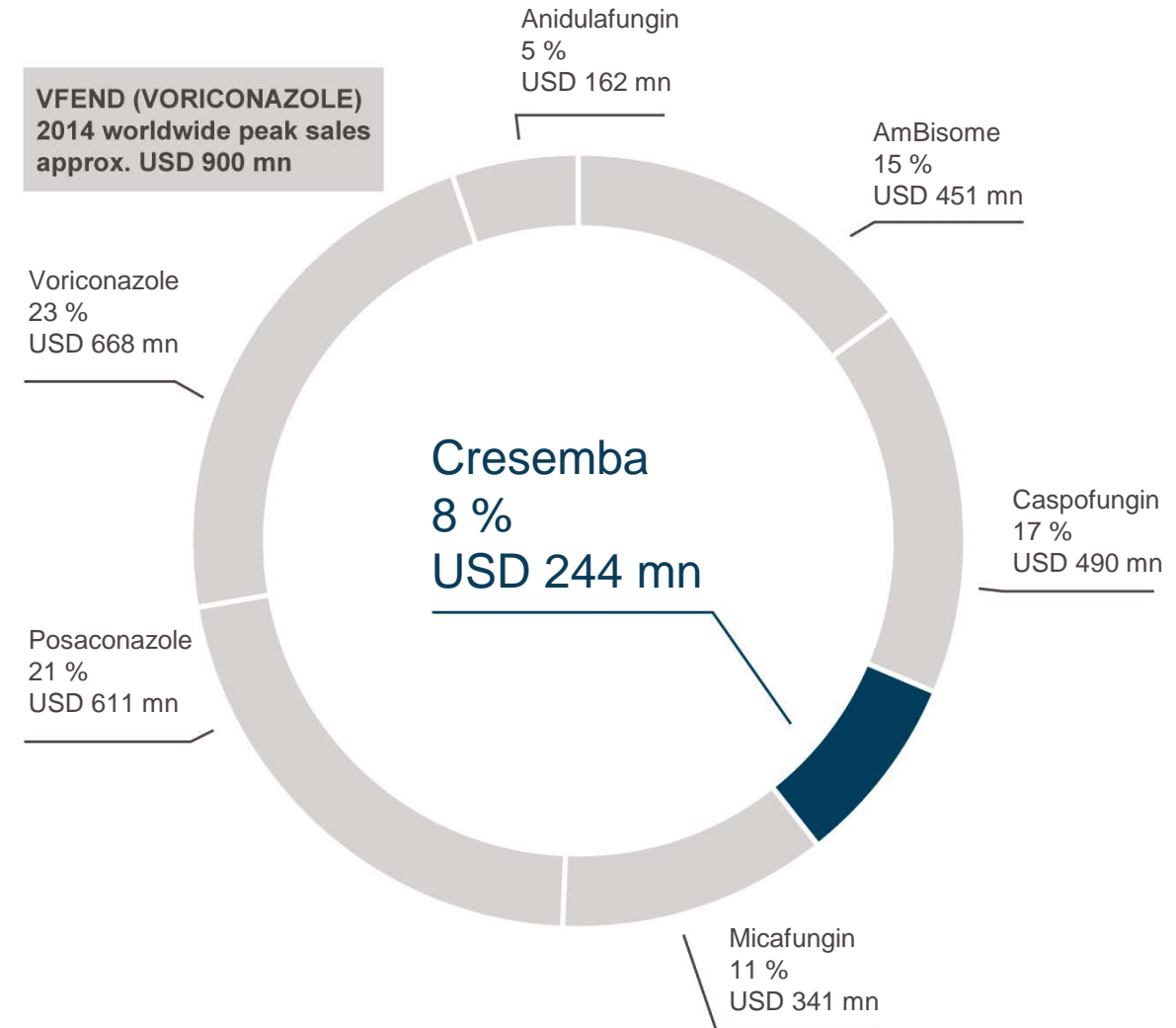
LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, September 2020

# Sales of best-in-class antifungals\* by product

USD 3.0 bn sales (MAT Q3 2020)

- Potential to increase Cresemba® (isavuconazole) market share
  - Anticipate to be launched in 60 countries by end-2021
  - Exclusivity through 2027 in the U.S. and potential pediatric exclusivity extension to 2027 (from 2025) in the EU

\* Best-in-class antifungals: isavuconazole, posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin



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

# Cresemba<sup>®</sup> — Differentiated by spectrum, safety and tolerability

- Broad spectrum of activity against molds, including emerging molds (mucorales)
- Consistent plasma levels
- Statistically fewer drug-related adverse events and treatment-emergent adverse events (liver, skin, eye) in invasive aspergillosis patients vs. voriconazole in SECURE phase 3 study
- Can be administered without restriction in patients with renal impairment
- Manageable drug-drug interaction profile
- Once daily maintenance dose, i.v./oral treatment
- ECIL-6 guideline: Cresemba<sup>®</sup> recommended for the first-line treatment of invasive aspergillosis in leukemia and hematopoietic stem cell transplant patients. ECIL states that isavuconazole is as effective as voriconazole with a better safety profile.



Antibacterial

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

Severe bacterial infections



# Zevtera<sup>®</sup> — An introduction

- Broad-spectrum anti-MRSA cephalosporin (including Gram-negative bacteria)
- Rapid bactericidal activity
- Potential to replace antibiotic combinations
- Early improvement in HAP, particularly in patients with MRSA, and CAP, including high-risk patients
- Cephalosporin class safety profile
- Marketed in selected countries in Europe, Latin America and the MENA-region as well as in Canada

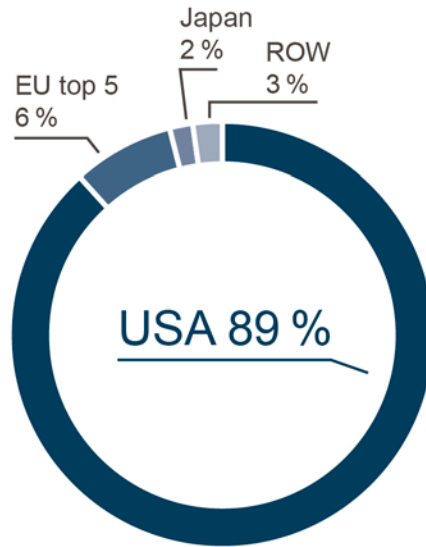
Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP), and community-acquired pneumonia (CAP). Not approved in the U.S.

MENA: Middle East and North Africa

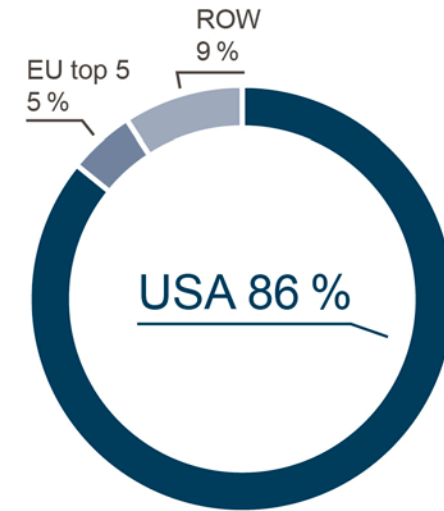


# The hospital anti-MRSA antibiotic market — A USD 2.8 bn market\* with the U.S. being the most important region

Daptomycin sales by region (2015, before LOE)



Ceftaroline sales by region (MAT Q3 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, September 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 ~130 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 H1 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

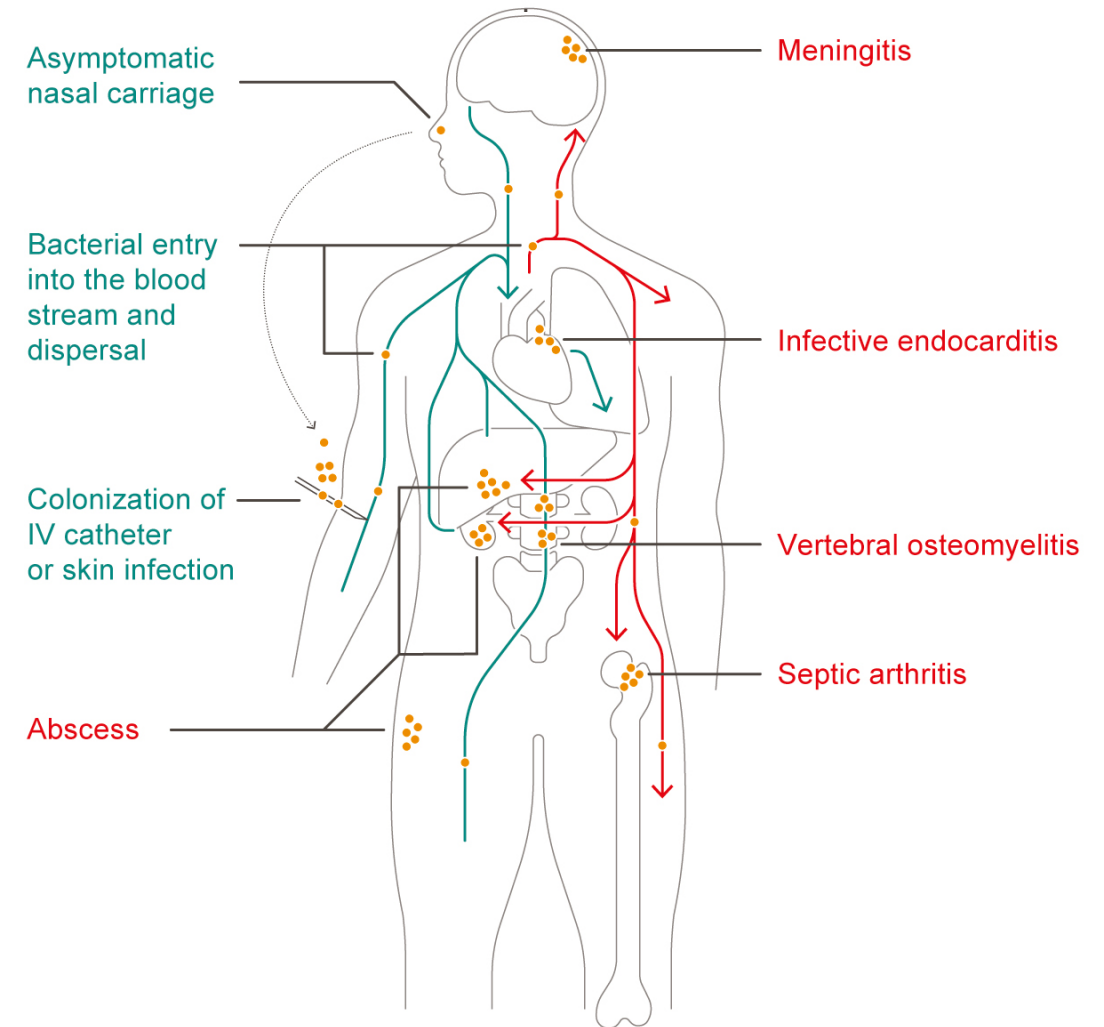
<sup>1</sup> MMWR, 2019;68:214–219.

<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.

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 scheme is dominated by shades of orange and yellow.

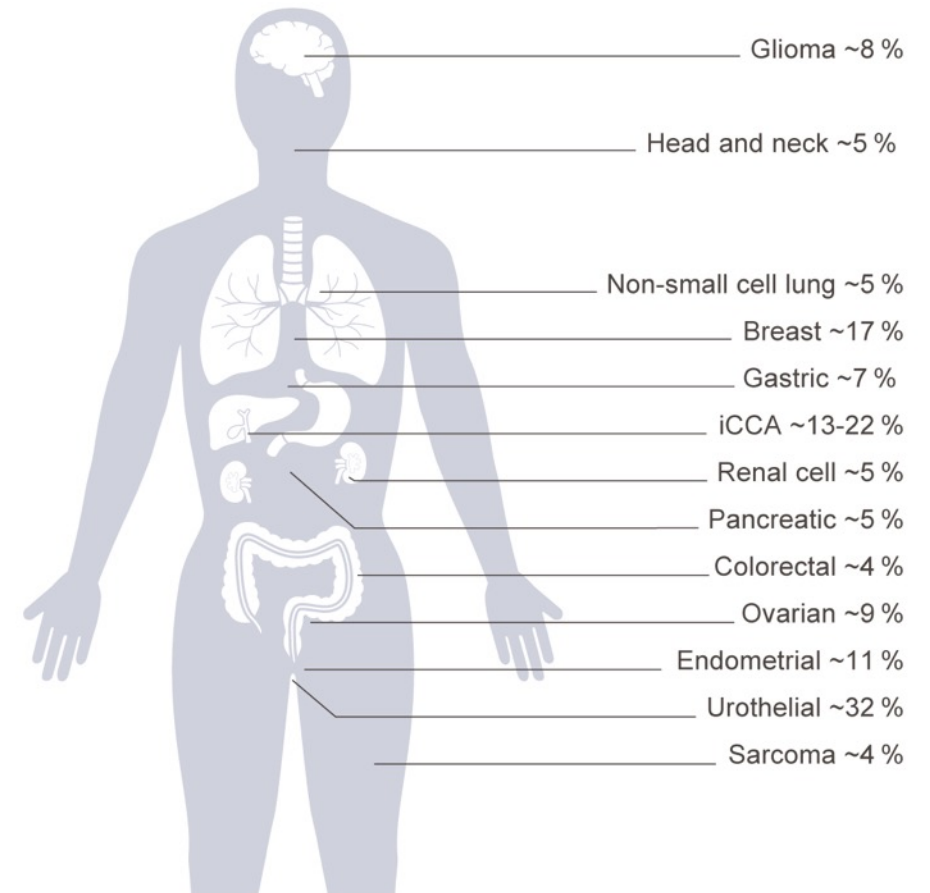
Oncology

# 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
- 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

# 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
  - Manageable safety profile with low incidence of nail toxicity, retinal events, hand-foot syndrome and stomatitis
- Topline results expected Q1 2021

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

- Define the full therapeutic potential in iCCA with potential for differentiation
- Encouraging interim results - progression-free survival consistent with outcome in patients with FGFR2 gene-fusions<sup>3</sup>
  - Pooled data from 23 patients treated in clinical studies and from the early access and compassionate use programs
  - 7.2 months median progression free survival and 8.2 months median duration of treatment
- Interim results expected H1 2021

<sup>1</sup> NCT03230318

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

<sup>3</sup> Droz Dit Busset et al. Annals of Oncology (2020) 31 (suppl\_5): abstract 45P (NCT01752920, NCT03230318)



# 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 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
- Successful completion of phase 1b cohort
  - Recommended phase 2 dose for the combination at full standard doses of derazantinib and atezolizumab
  - No dose-limiting toxicities observed
- Clinical supply agreement with Roche for atezolizumab

## FIDES-03<sup>2</sup> | 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 with ramucirumab/paclitaxel
  - Combination of derazantinib with atezolizumab
- Clinical supply agreement with Roche for atezolizumab and clinical trial collaboration and supply agreement with Lilly for ramucirumab

<sup>1</sup> NCT04045613; Chaudhry A et al. Journal of Clinical Oncology 2020; 38, no. 6\_suppl. TPS590. (NCT04045613)

<sup>2</sup> NCT04604132

# FGFR-inhibitors show differences in safety profiles

	Cholangiocarcinoma				Urothelial cancer	
	DZB <sup>1</sup> (N=44)	INF <sup>2</sup> (N=71)	FUT <sup>3</sup> (N=67)	PEM <sup>4</sup> (N=146)	PEM <sup>5</sup> (N=108)	ERD <sup>6</sup> (N=87)
Dosing regimen	300mg QD	125mg Q4W QD for 3w	20 mg QD	13.5mg Q3W QD for 2w	13.5mg Q3W QD for 2w	8 mg QD (titration to 9mg)
Most frequent safety events	Phosphorus↑ Nausea Vomiting	Phosphorus↑ Fatigue Stomatitis	Phosphorus*↑ Diarrhea* Dry mouth*	Phosphorus↑ Alopecia Diarrhoea	Diarrhoea Alopecia Constipation	Phosphorus↑ Stomatitis Fatigue
Blood phosphorus↑†	59%	73%	88%	60%	31%	76%
Fatigue†	43%	49%	NR	42%	32%	54%#
Alopecia†	20%	38%	NR	49%	40%	26%
Dry eye/xerophthalmia†	16%	32%	NR	35%#	NR	28%#
Retinopathy†	0%	NR	9%	6%‡	NR	25%
Alanine aminotransferase (ALT) ↑	30%**	NR	NR	43%**	NR	41%**
Hand-foot syndrome/PPE	0%	27%	18%	15%	NR	26%
Nail toxicities	<5%	NR	42%	43%#	NR	41%#
Stomatitis	11%	45%	NR	35%	34%	56%

<sup>1</sup> Droz Dit Busset et al., ESMO 2019 and Basilea data on file, <sup>2</sup> Javle et al., ESMO 2018, <sup>3</sup> Goyal et al., ASCO 2020, <sup>4</sup> Pemazyre™ U.S. Prescribing Information (April 2020), <sup>5</sup> Necchi, et al., ESMO 2018,

<sup>6</sup> Balversa™ U.S. prescribing information (April 2019)

† assumed FGFR inhibitor class-effect; \*futibatinib treatment-related adverse events

# includes various and different adverse reactions; for details see Pemazyre™ U.S. Prescribing Information (April 2020) and Balversa™ U.S. prescribing information (April 2019);

† Refers to reported adverse events of Retinal Pigment Epithelial Detachment (RPED) for pemigatinib, Central Serous Retinopathy (CSR)/RPED for erdafitinib and CSR for futibatinib

‡ reported incidence is from 466 patients who received Pemazyre™ across clinical trials;

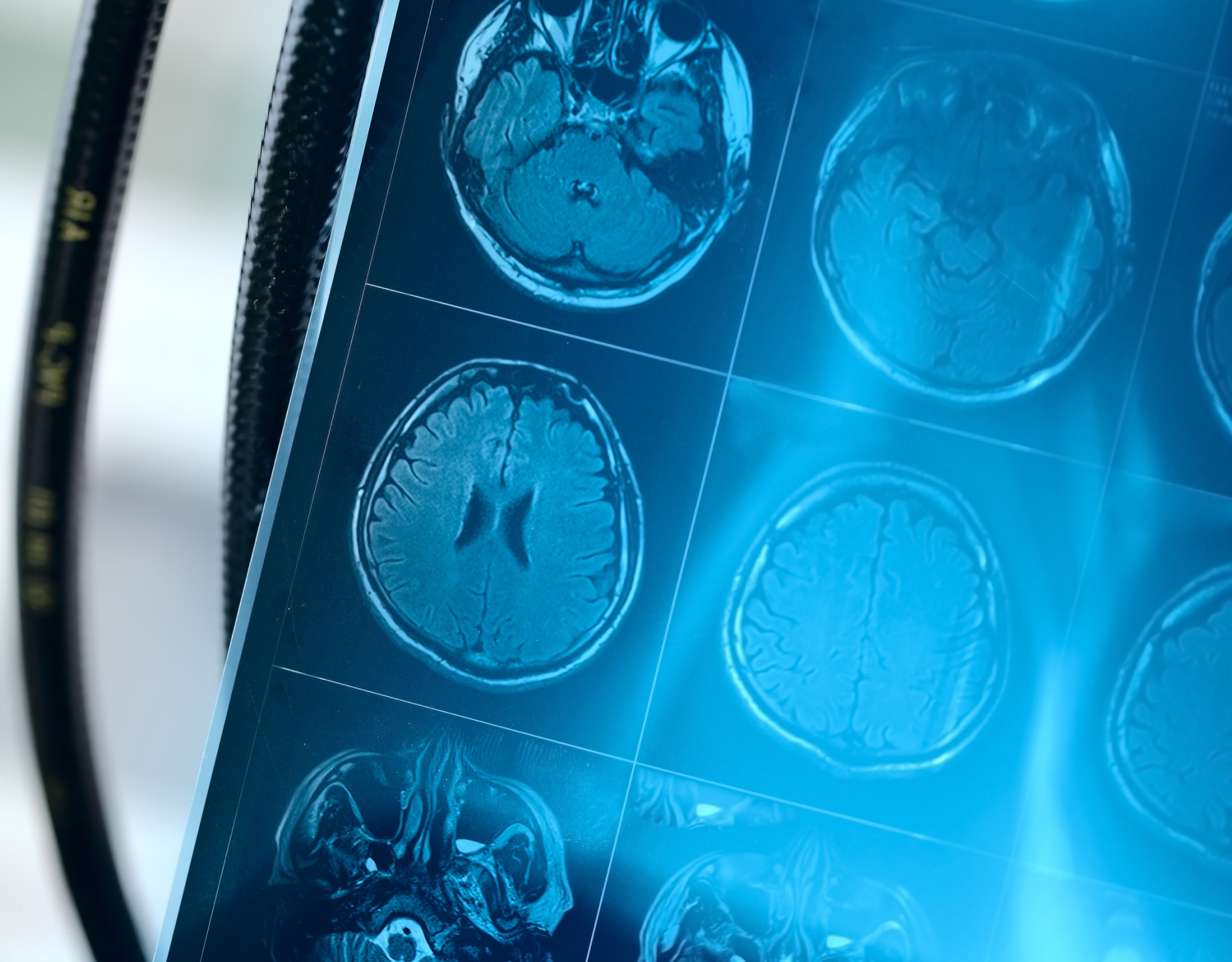
\*\* based on reported adverse events for DZB; based on reported laboratory abnormalities, regardless of causality for PEM and ERD.

Abbreviations: DZB: derazantinib, INF: infigratinib (BGJ398), FUT: futibatinib (TAS-120), PEM: pemigatinib (INCB54828), ERD: erdafitinib; PPE: Palmar-plantar erythrodysesthesia; NR: not reported; QD: daily; Q3W/Q4W: every 3/4 weeks; w: weeks

Oncology

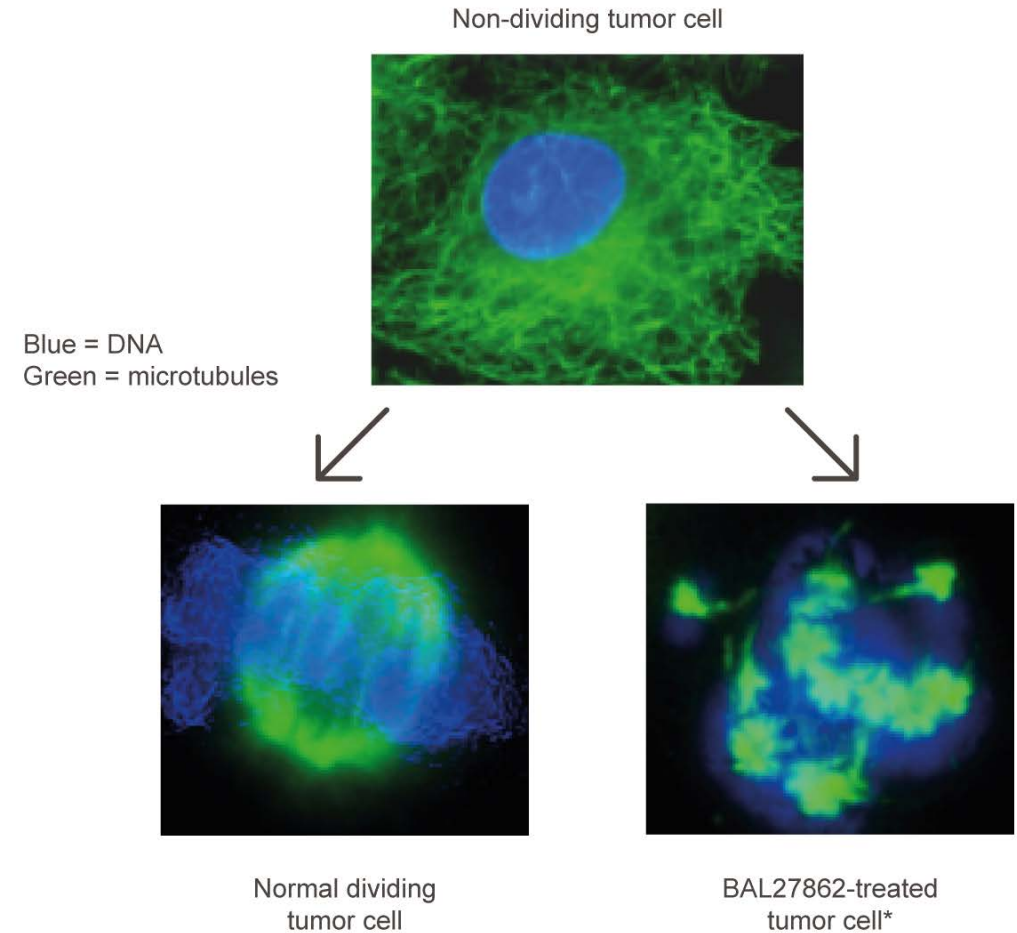
# Lisavanbulin (BAL101553)

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
- Biomarker-driven phase 2 study in patients with recurrent glioblastoma (GBM) using EB1-positivity as patient selection criterion ongoing

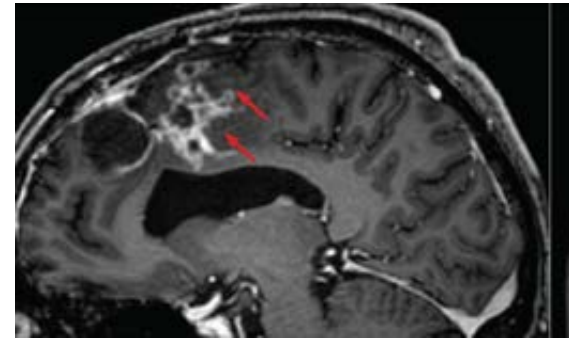


\* Lisavanbulin (BAL101553) is a prodrug of BAL27862

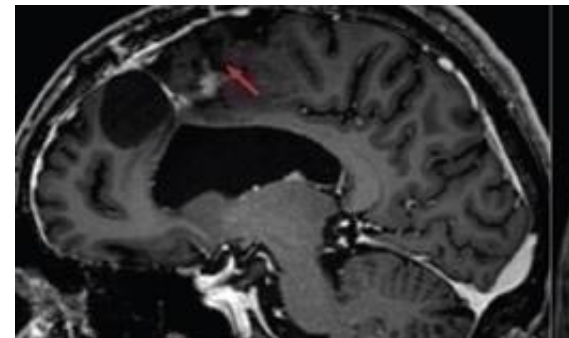
# EB1 — A potential response-predictive clinical biomarker for lisavanbulin

- 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

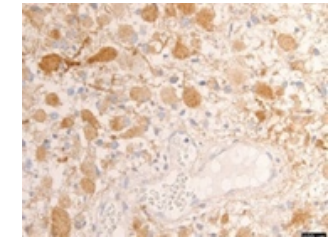
## GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



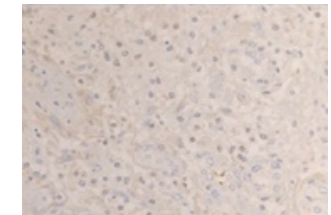
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



Non-responder

<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)



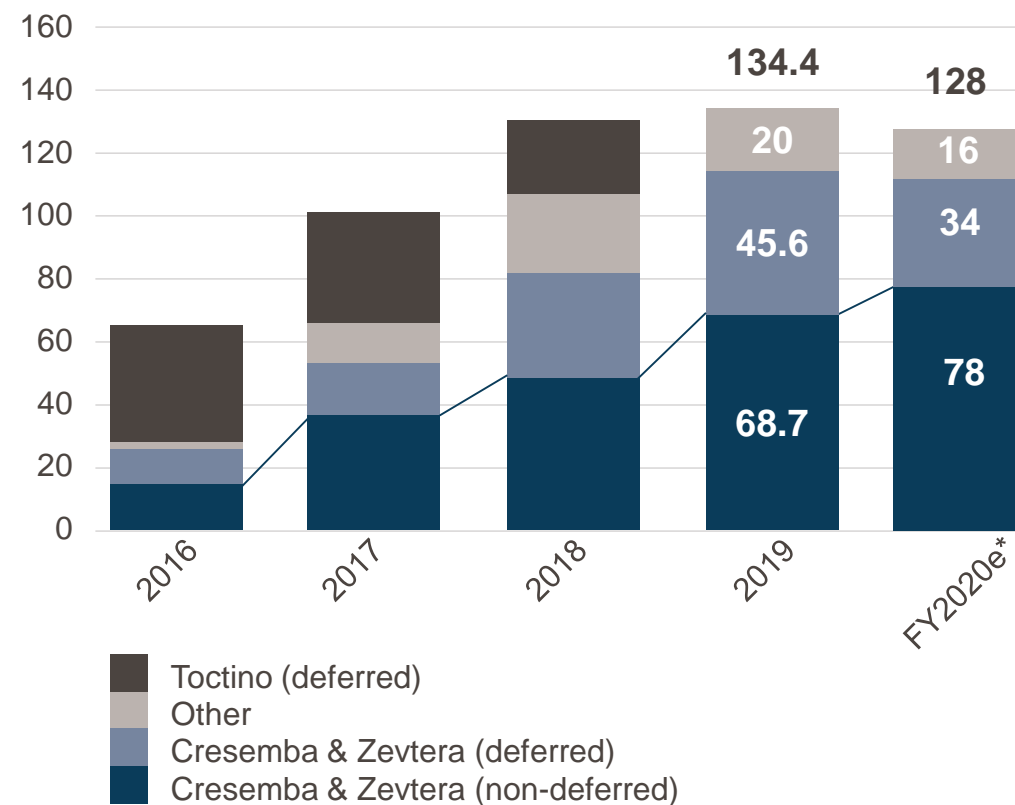
# Financials



# Financial guidance

In CHF mn	FY 2019 actuals	FY 2020 guidance	FY 2020e*
Total revenue	134.4	128–138	128
thereof: Contributions Cresemba® & Zevtera®			
non-deferred	68.7	77–87	78
deferred	45.6	33	34
Operating loss	17.2	5–15	N/A
Cash and investments	161.0	150	167

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



\* 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.

# Milestones & 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>			✓ Approval in China		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)	Interim results (other FGFR2 gene aberrations)	
	<b>FIDES-02 (urothelial cancer)</b>		✓ Safety data and recommended phase 2 dose (RP2D) for derazantinib/ atezolizumab combination and expansion into phase 2	Interim results in derazantinib monotherapy	Interim results in combination therapy with atezolizumab
	<b>FIDES-03 (gastric cancer)</b>	✓ Clinical supply agreement with Roche	✓ Start of phase 1/2 study		Interim results in monotherapy and recommended phase 2 dose with ramucirumab and paclitaxel
		✓ Clinical trial collaboration and supply agreement with Lilly			
<b>Lisavanbulin (Oral)</b>			✓ Full results of phase 1 study in glioblastoma (GBM) ✓ Start phase 2 biomarker-driven GBM study		Interim results from phase 2 biomarker-driven GBM study
					Recommended phase 2 dose in phase 1 study in newly-diagnosed GBM in combination with radiotherapy



# Milestones & Outlook 2021 / 2022

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

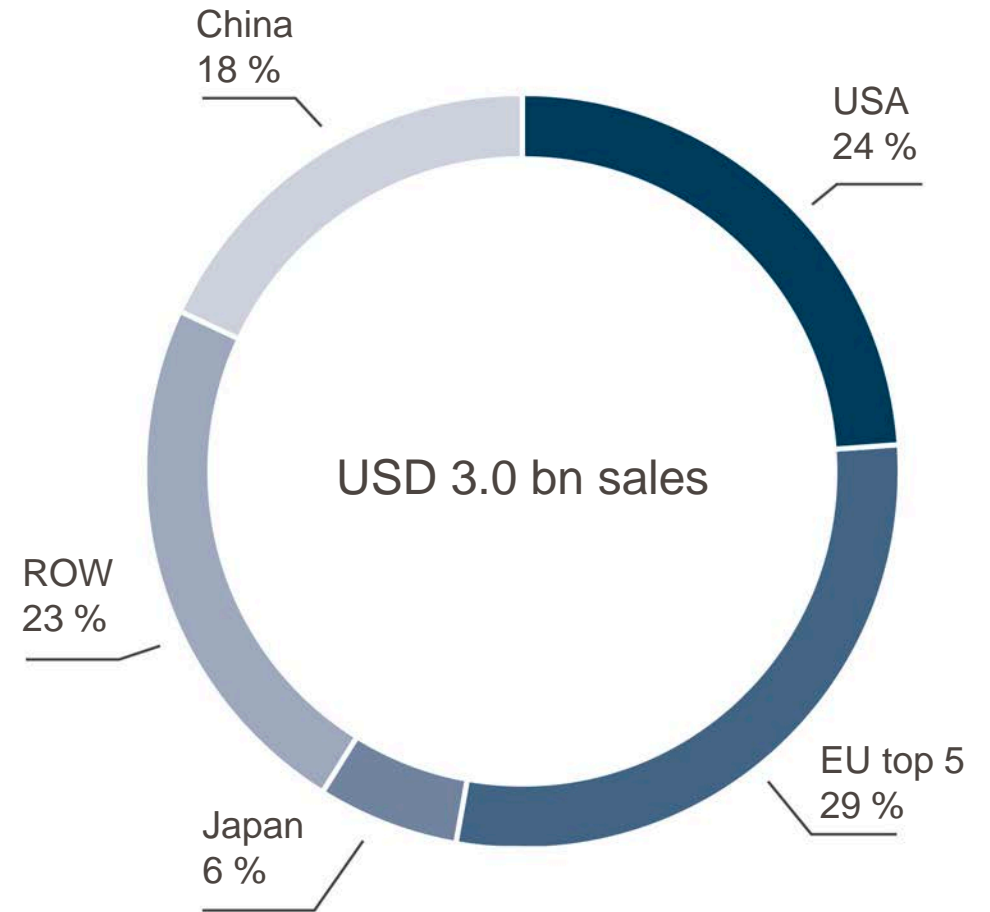
		H1 2021	H2 2021	H1 2022	H2 2022
<b>Isavuconazole</b>		✓ Complete patient enrolment in phase 3 study in Japan	Topline results from phase 3 study in Japan		
<b>Ceftobiprole</b>			Complete patient enrolment in SAB phase 3 study	Topline results from SAB phase 3 study	
<b>Derazantinib</b>	<b>FIDES-01 (iCCA)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

# Appendix

# Significant sales of best-in-class antifungals in all major regions — Covered by our partnerships

USD 3.0 bn sales of best-in-class antifungals\* (MAT Q3 2020)

\* Best-in-class antifungals: isavuconazole, posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin



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

Confidential/proprietary information of Basilea Pharmaceutica International Ltd. – not for distribution

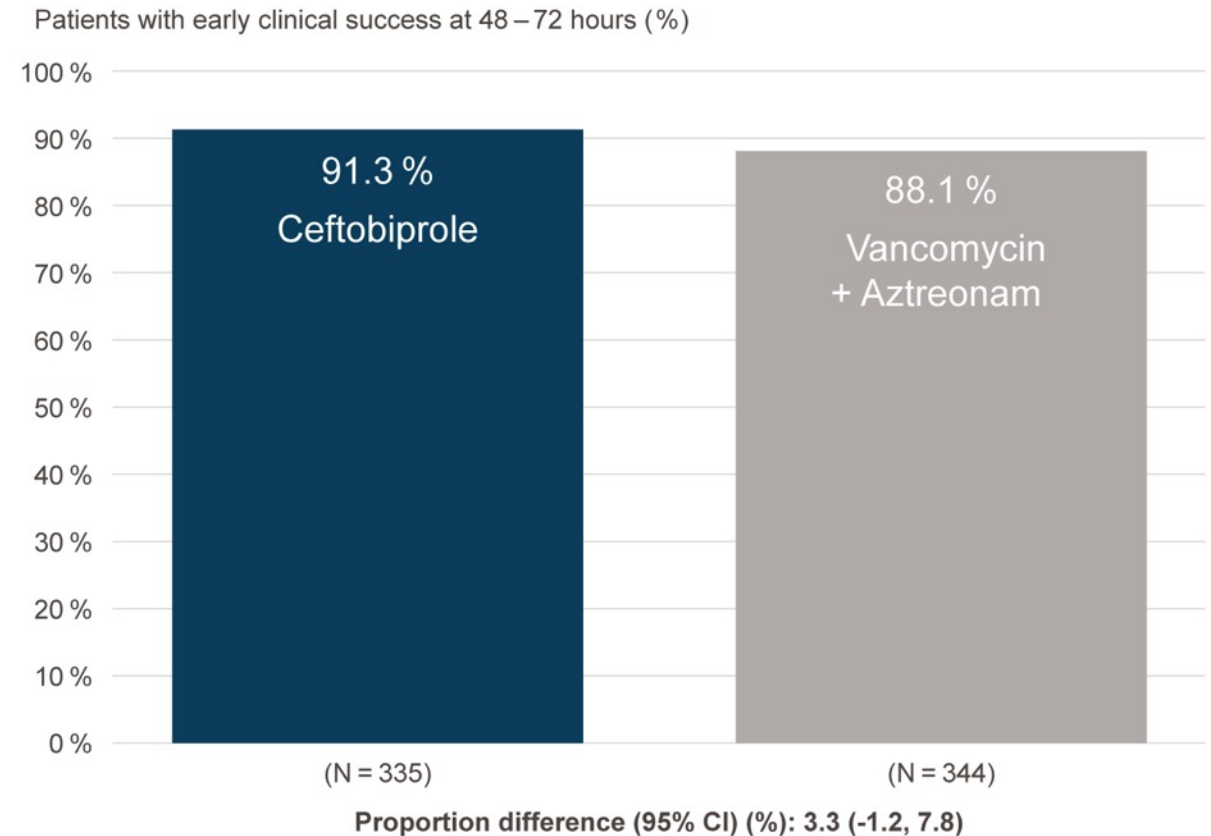
# Ceftobiprole — Positive topline phase 3 results reported in ABSSSI

Key topline study<sup>1</sup> results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints



<sup>1</sup> NCT03137173  
ABSSSI: Acute bacterial skin and skin structure infections

## Early clinical response at 48–72h after start of treatment (ITT population)



ITT: intent-to-treat  
Pre-defined limit of non-inferiority = lower limit of 95 % CI for difference > -10 %

# Ceftobiprole — Positive topline phase 3 results reported in ABSSSI

Key topline study<sup>1</sup> results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints

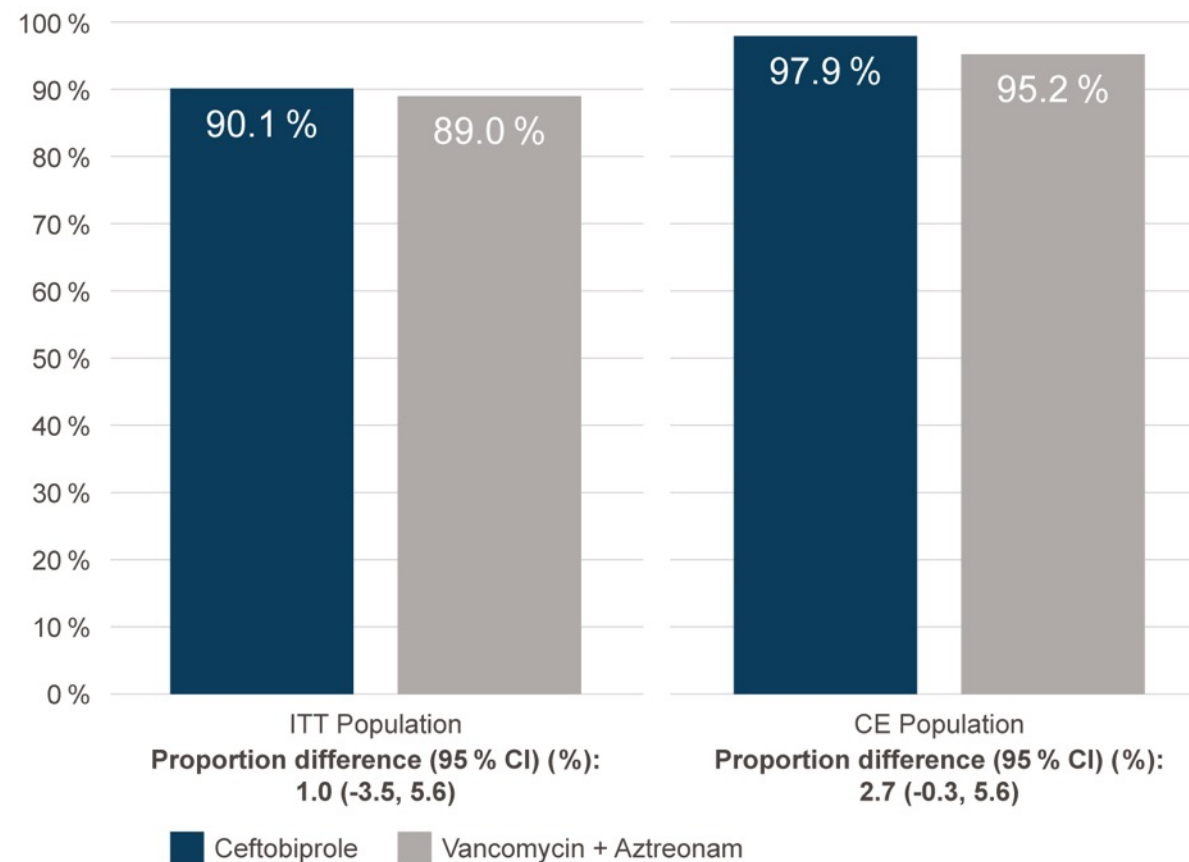


<sup>1</sup> NCT03137173

ABSSSI: Acute bacterial skin and skin structure infections

## Investigator-assessed clinical success at test-of-cure (TOC) 15-22 days after randomization (ITT, CE populations)

Patients with clinical success at the TOC visit (%)



CE: clinically evaluable; ITT: intent-to-treat

# 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>

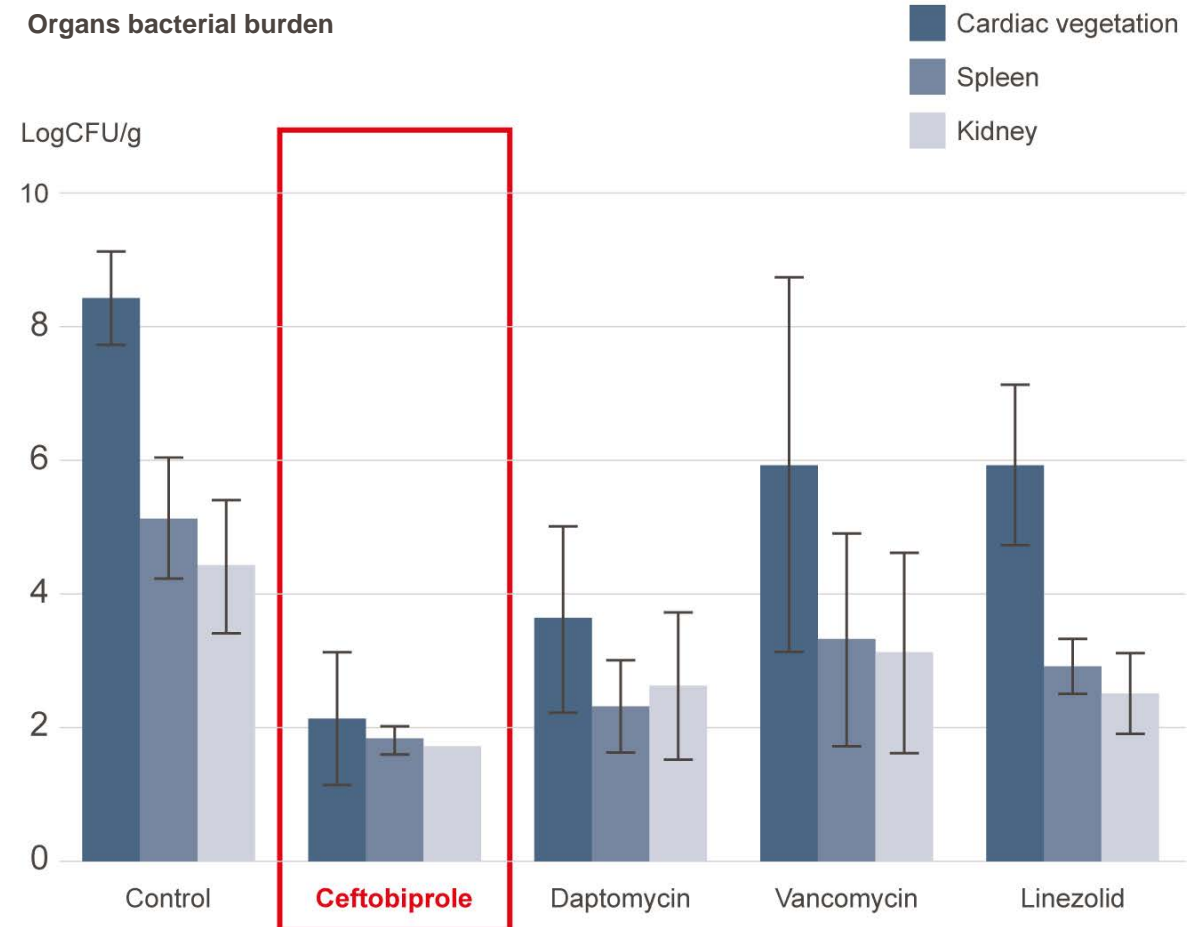
<sup>1</sup>Syed YY. *Drugs*. 2014;74:1523-1542.

<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>

# Phase 3 study with ceftobiprole in the treatment of patients with SAB



- **Design:** randomized, double-blind, multi-center
- **Enrolment:** approximately 390 adult patients (male and female)
- **Indications:** *Staphylococcus aureus* bacteremia (SAB), including endocarditis (IE) and other forms of complicated SAB
- **Main inclusion criteria:** Positive *S. aureus* blood culture and signs & symptoms for SAB
- **Intervention:** ceftobiprole medocaril i.v.; comparator daptomycin i.v. or daptomycin plus aztreonam to cover Gram-negative bacteria
- **Primary endpoint:** overall success as assessed by an independent Data Review Committee (DRC) in the treatment of SAB, including IE, at the post-treatment evaluation (PTE) visit (70 days after randomization) in the modified intent-to-treat (mITT) population.
- **Secondary endpoints:** includes all-cause mortality at Day 28 and Day 70 (PTE visit) in the intent-to-treat (ITT) and mITT populations; and time to *S. aureus* bloodstream clearance

# FGFR-inhibitors show differences in kinase-inhibition profiles<sup>1</sup>

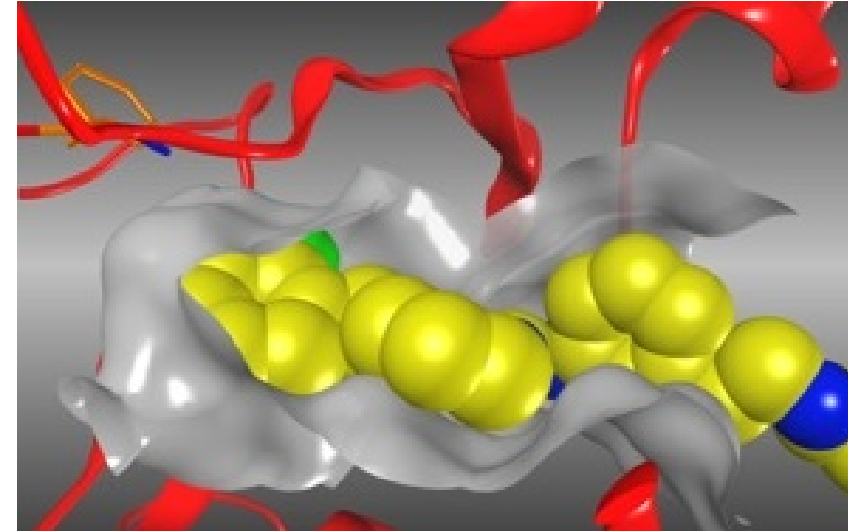
FGFR-inhibitor compound (Sponsor)	Parameter	FGFR1	FGFR2	FGFR3	FGFR4	CSF1R	VEGFR2
<b>Derazantinib</b> (Basilea)	Ratio to FGFR2 activity	4	1	4	77	3	6
<b>Pemigatinib</b> (Incyte)	Ratio to FGFR2 activity	3	1	4	39	231	62
<b>Erdafitinib</b> (Janssen)	Ratio to FGFR2 activity	2	1	2	13	95	6
<b>Rogaratinib</b> (Bayer)	Ratio to FGFR2 activity	5	1	6	18	116	48
<b>Infigratinib</b> (QED)	Ratio to FGFR2 activity	2	1	2	47	86	55
<b>Futibatinib</b> (Taiho)	Ratio to FGFR2 activity	2	1	2	18	NA	NA

<sup>1</sup> McSheehy et al. Derazantinib (DZB): A dual FGFR/CSF1R-inhibitor active in PDX-models of urothelial cancer. Mol Cancer Ther. 2019 (18) (12 Supplement) LB-C12

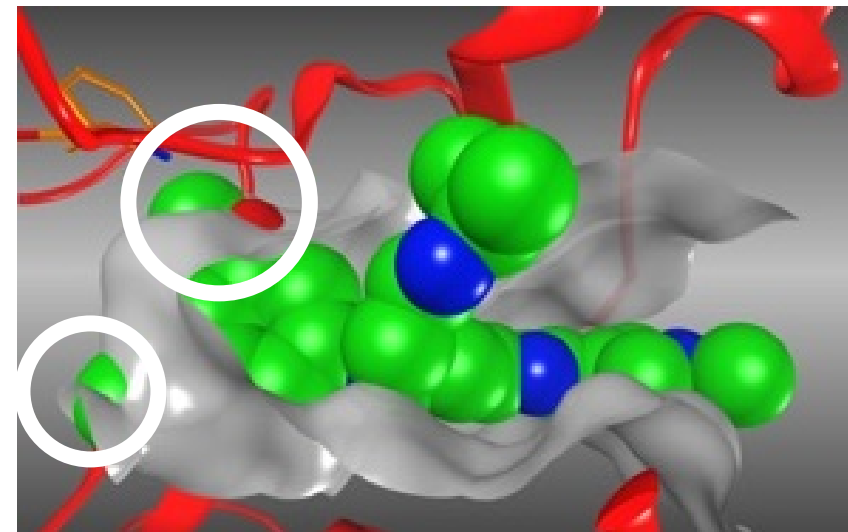


# In-silico analysis of derazantinib binding to CSF1R

- Crystal structures indicate differences in inhibitor binding sites of FGFR and CSF1R kinases
- Improved kinase inhibition activity of derazantinib against CSF1R versus other FGFR-inhibitors can be explained by the unique chemical structure of derazantinib<sup>1</sup>



**Derazantinib** (yellow) fits to smaller active site pocket of CSF1R (grey/red)

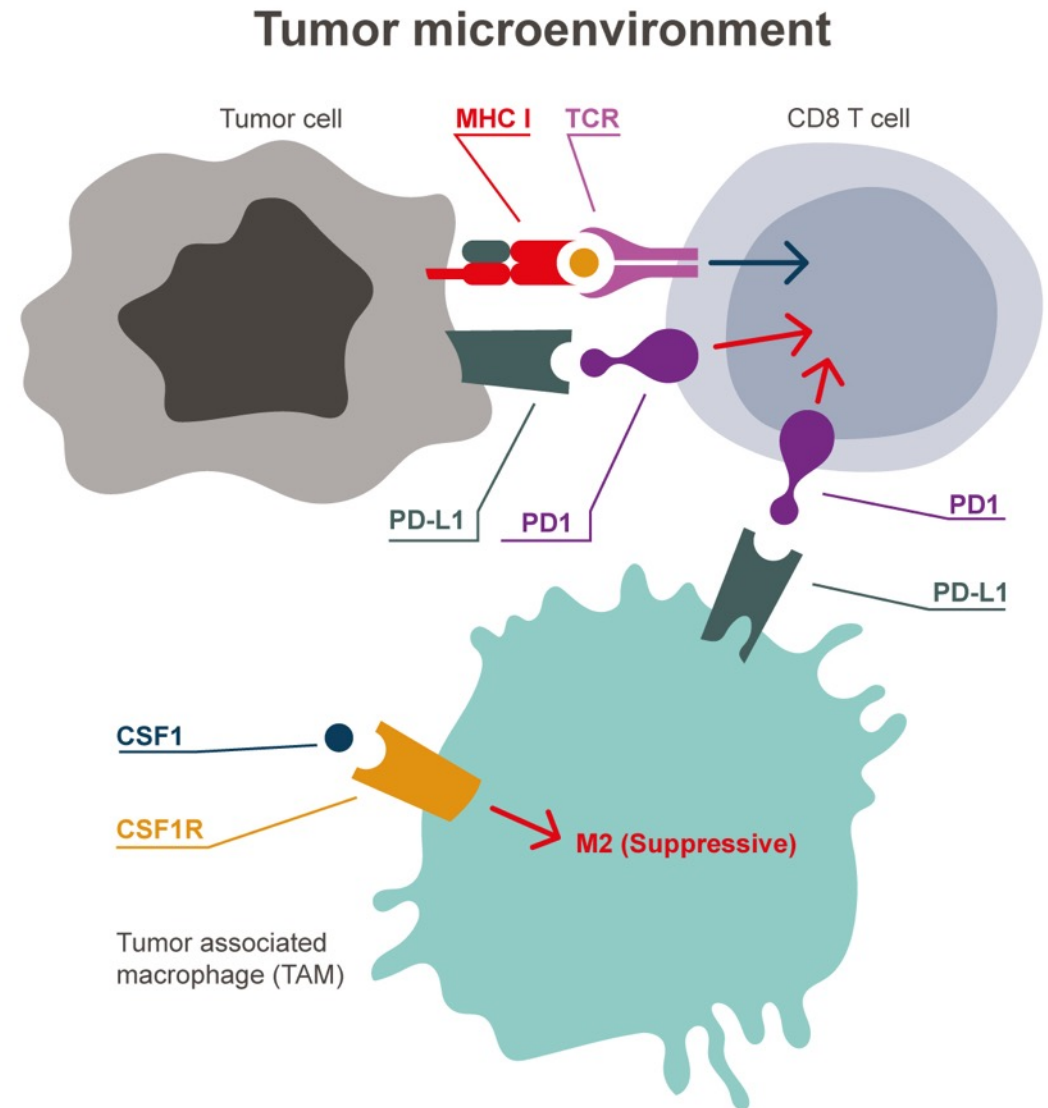


**Erdafitinib** (green) is too large (white circles) for the active site pocket of CSF1R (grey/red)

<sup>1</sup> McSheehy et al. Derazantinib (DZB): A dual FGFR/CSF1R-inhibitor active in PDX-models of urothelial cancer. Mol Cancer Ther. 2019 (18) (12 Supplement) LB-C12

# Potential therapeutic relevance of CSF1R-inhibition

- Derazantinib is active in inhibiting FGFR kinases and CSF1R (Colony-stimulating factor-1 receptor)
- CSF1R-inhibition may reprogram immunosuppressive tumor-infiltrating macrophages, restore T-cell activity and thereby improve the susceptibility to PD1/PD-L1 inhibitors<sup>1</sup>
- Derazantinib may address several oncogenic mechanisms at the same time, i.e. inhibiting FGFR and making the tumor more susceptible to immunotherapy
- Basilea entered into a clinical supply agreement with Roche to study a combination of derazantinib and Roche's PD-L1-blocking immune-checkpoint inhibitor atezolizumab in patients with urothelial and gastric cancer



<sup>1</sup> X. Zheng et al. Redirecting tumor-associated macrophages to become tumoricidal effectors as a novel strategy for cancer therapy. *Oncotarget*. 2017;8(29):48436-48452

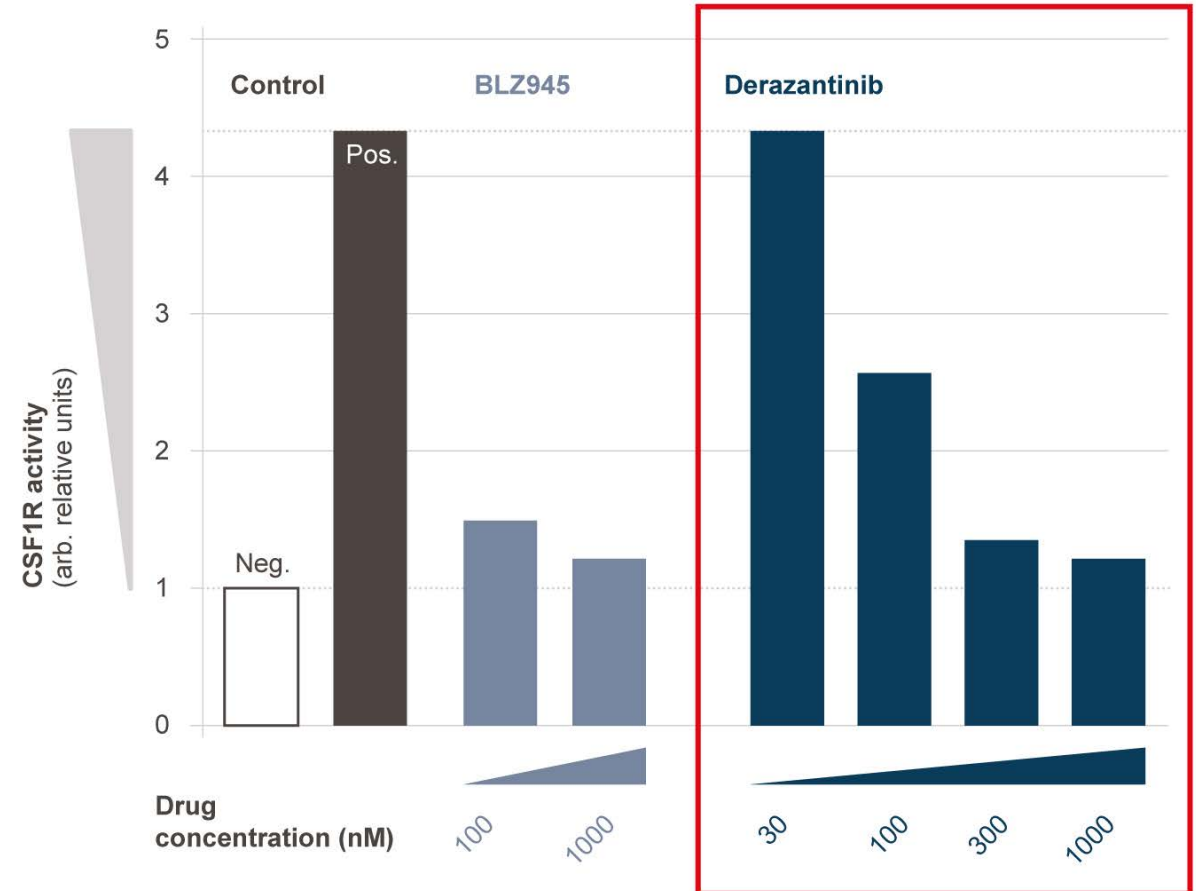
Graph adapted from: A. Ghasemzadeh et al. New Strategies in Bladder Cancer: A Second Coming for Immunotherapy. *Clin Cancer Res*. 2016;22(4):793-801

# Derazantinib inhibits mouse macrophage CSF1R activity

- Derazantinib treatment reduced CSF1-stimulated CSF1R activation (pCSF1R) in a concentration-dependent manner
- The maximum effect is similar to the specific CSF1R inhibitor BLZ945
- Derazantinib active-concentration is achievable in patients

<sup>1</sup> McSheehy et al. Derazantinib (DZB): A dual FGFR/CSF1R-inhibitor active in PDX-models of urothelial cancer. Mol Cancer Ther. 2019 (18) (12 Supplement) LB-C12

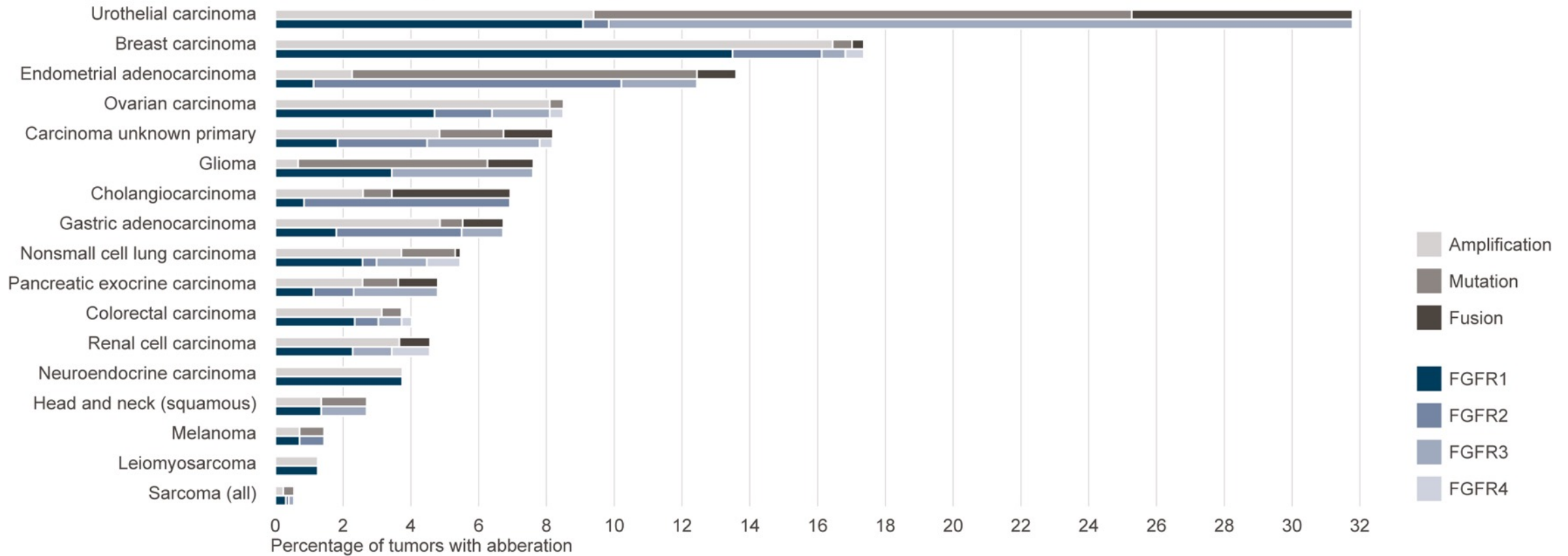
## Inhibition of CSF1R activity



*Method: bone-marrow-derived mouse macrophages were starved overnight, treated with CSF1 for 3 min, with or without pre-incubation with BLZ945 or DZB, and then extracted for subsequent immunoblot. The graph shows the quantification of the experiment based on densitometric analysis of the immunoblots*

# Derazantinib — Significant potential beyond iCCA

Frequency of currently known FGFR aberrations across tumor types



Source: Helsten et al., Clin Cancer Res. 2016;22:259-67

# Derazantinib — Multi-cohort phase 1/2 study in advanced urothelial cancer (FIDES-02)<sup>1</sup>

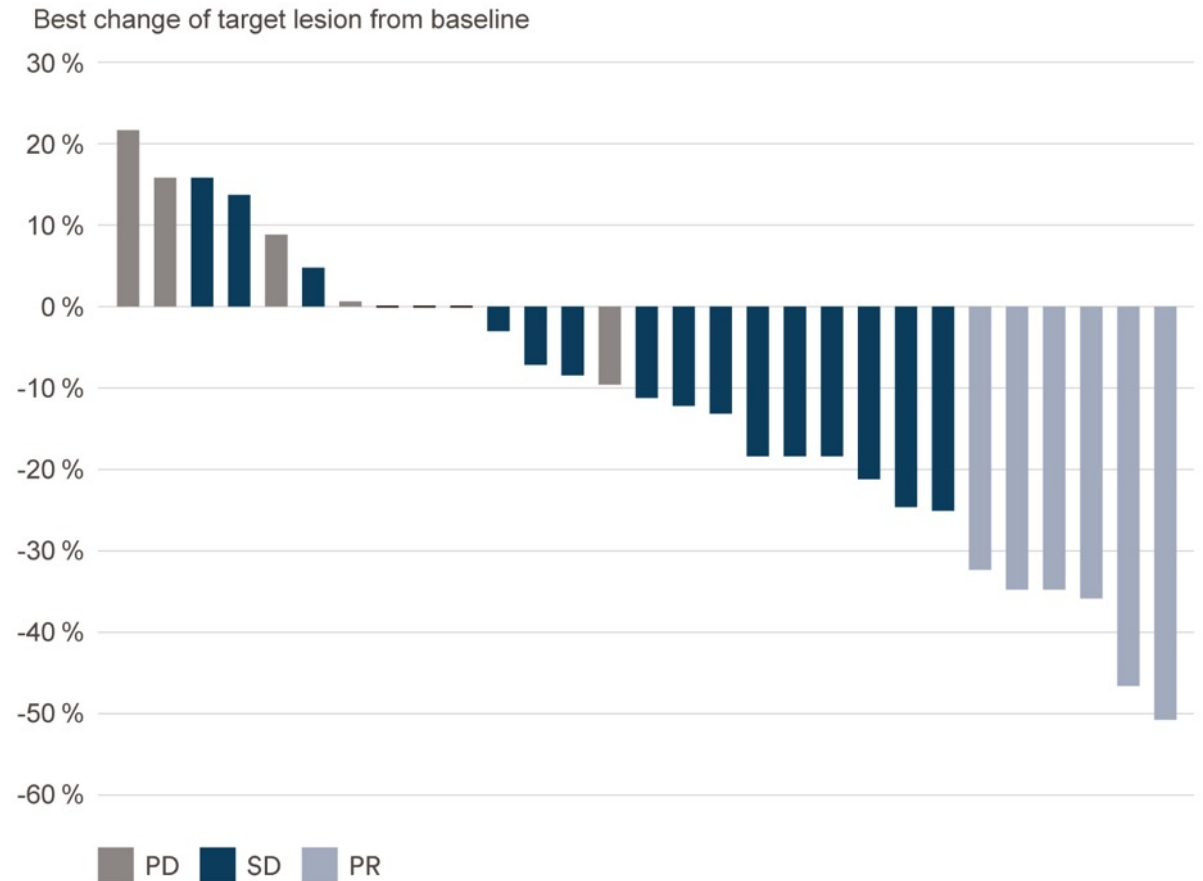
- Derazantinib as single agent and in combination with atezolizumab in patients with advanced urothelial cancer testing positive for mutations or fusions of FGFR1, FGFR2 or FGFR3 genes
- The subgroup of patients with low PD-L1 expression have limited clinical benefit from the treatment with PD1/PD-L1 inhibitors. This subgroup, however, shows frequent FGFR genomic abnormalities (mainly FGFR3 fusions)
- Derazantinib combined with PD1/PD-L1 inhibitors may provide benefits related to multiple mechanisms (FGFR-inhibition, macrophage modulation, enhanced response to immunotherapy), in particular in the low PD-L1 expression subgroup
- Across a total of four sub-studies, FIDES-02 potentially can enroll up to approximately 300 patients
- Patient cohorts in various treatment settings, including:
  - Post-chemotherapy/immunotherapy recurrence (second-and post second-line)
  - First-line platinum-ineligible
  - Resistance to prior FGFR-inhibitor treatment
- Study conducted in multiple centers in Asia-Pacific, Europe and North America
- Clinical supply agreement with Roche for the immune-checkpoint inhibitor atezolizumab

<sup>1</sup> NCT04045613

# Derazantinib — Established proof-of-concept in iCCA in phase 1/2a study

- Subgroup analysis of 29 patients with FGFR2-fusion positive iCCA:
  - Objective response rate of 21%
  - In 72% of patients, tumor response or disease stabilization for ≥16 weeks was achieved\*
- Compares favorably to Standard-of-Care (SoC) chemotherapy (cross-trial comparison)
  - Objective Response Rate (ORR) 21% for derazantinib<sup>1</sup> versus <10% for SoC<sup>2, 3</sup>
  - Progression-Free Survival (PFS) approx. 6 months<sup>1</sup> versus 3 months for SoC<sup>2, 3</sup>
- Manageable safety profile<sup>1, 4</sup>

<sup>1</sup> V. Mazzaferro et al. Derazantinib (ARQ 087) in advanced or inoperable FGFR2 gene fusion-positive intrahepatic cholangiocarcinoma. *British Journal of Cancer* 2018 <sup>2</sup> A. Lamarca et al. Second-line chemotherapy in advanced biliary cancer: a systematic review. *Annals of Oncology* 2014 (25), 2328-2338; <sup>3</sup> L. Fornaro et al. Second-line chemotherapy in advanced biliary cancer progressed to first-line platinum-gemcitabine combination: a multicenter survey and pooled analysis with published data. *Journal of Experimental & Clinical Cancer Research* 2015 (34), 156 <sup>4</sup> K. P. Papadopoulos et al. A phase 1 study of ARQ 087, an oral pan-FGFR inhibitor in patients with advanced solid tumors. *British Journal of Cancer* 2017, 1-8



Sources: Mazzaferro et al. *British Journal of Cancer* 2018;  
\* Mazzaferro et al. *J Clin Oncol* 2017;35 suppl: abstract 4017

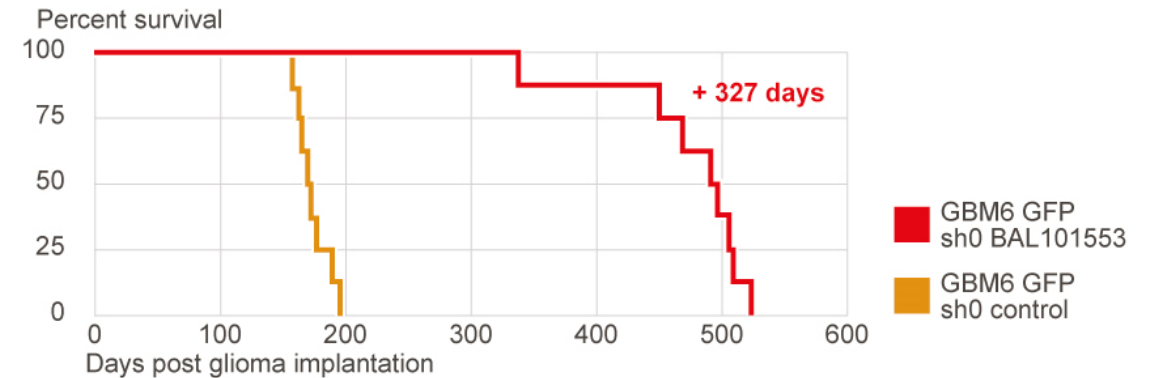
# EB1 — A potential response-predictive clinical biomarker for lisavanbulin

- EB1 (plus-end binding protein)<sup>1</sup> is located on the microtubules and involved in microtubule dynamics
- Predictive of response to lisavanbulin in mouse models<sup>1</sup>

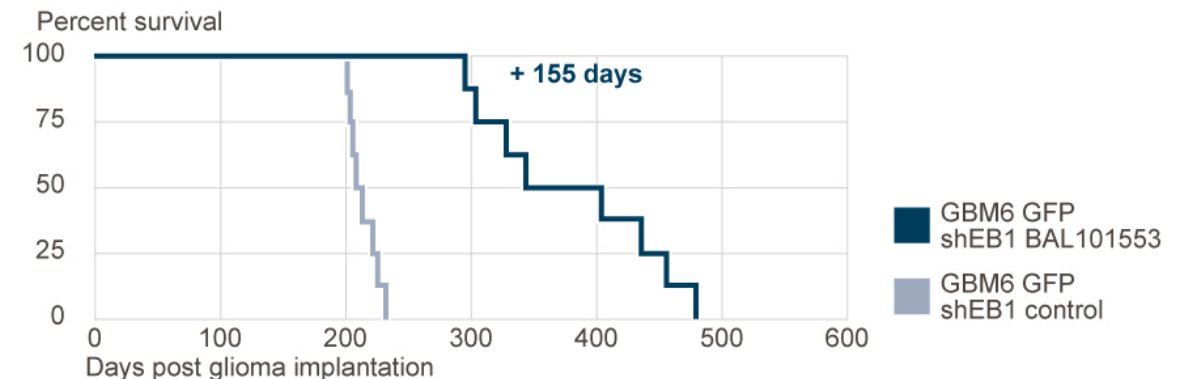
<sup>1</sup> Berges et al. EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after oral treatment with the novel tubulin-binding checkpoint activator BAL101553. Eur. J. Cancer 2018, 103, E61-62, A166

## Effect of lisavanbulin (BAL101553) on survival in mice with EB1-expressing or EB1 downregulated GBM

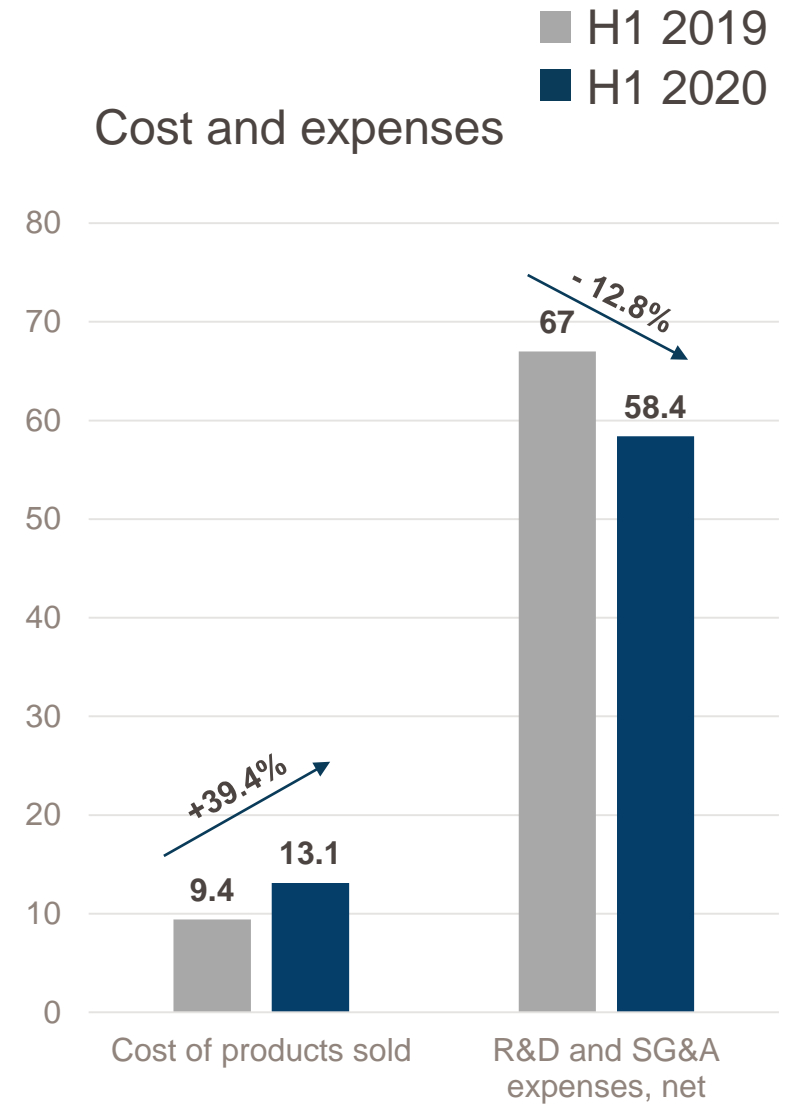
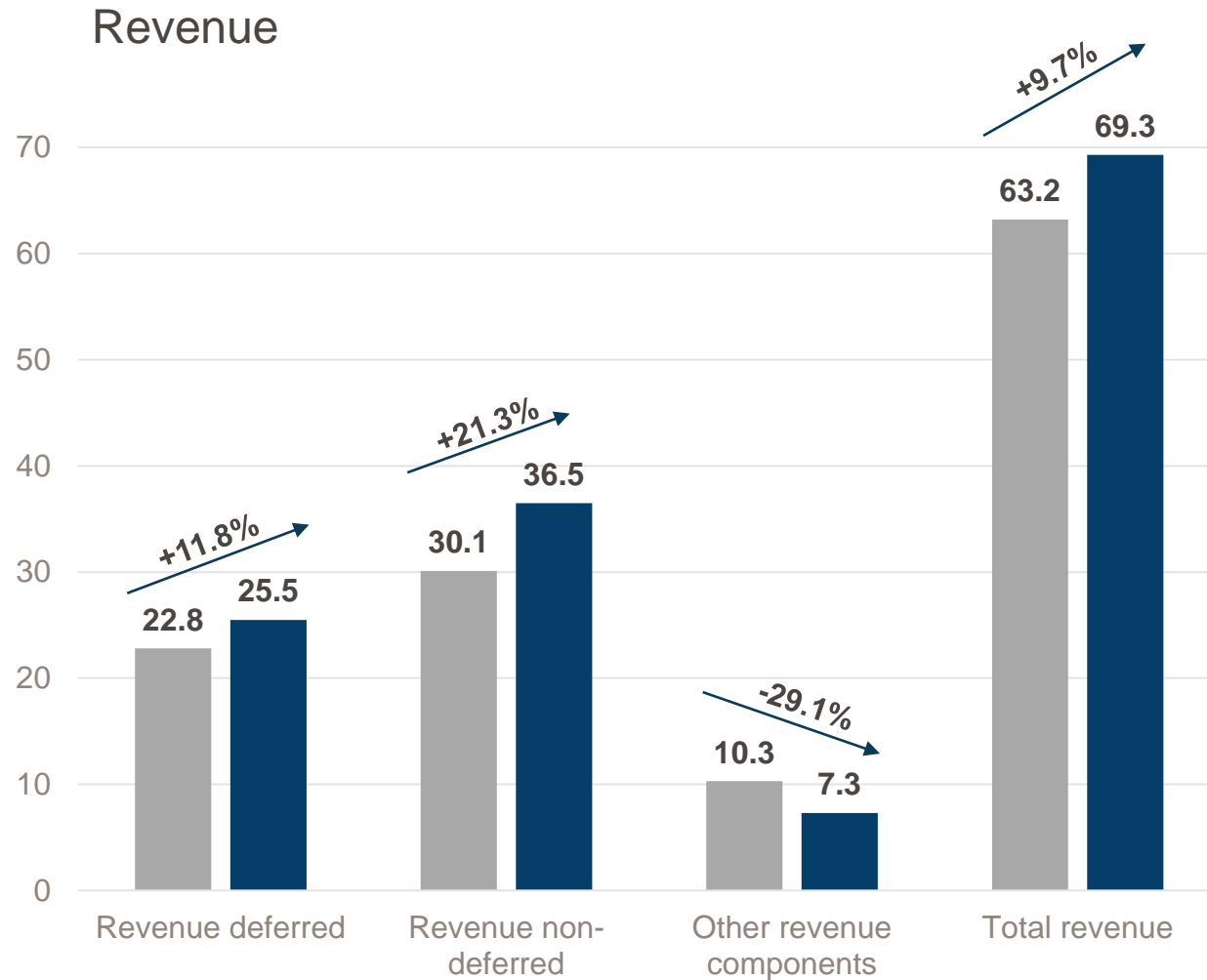
### EB1-expressing GBM



### EB1-downregulated GBM



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

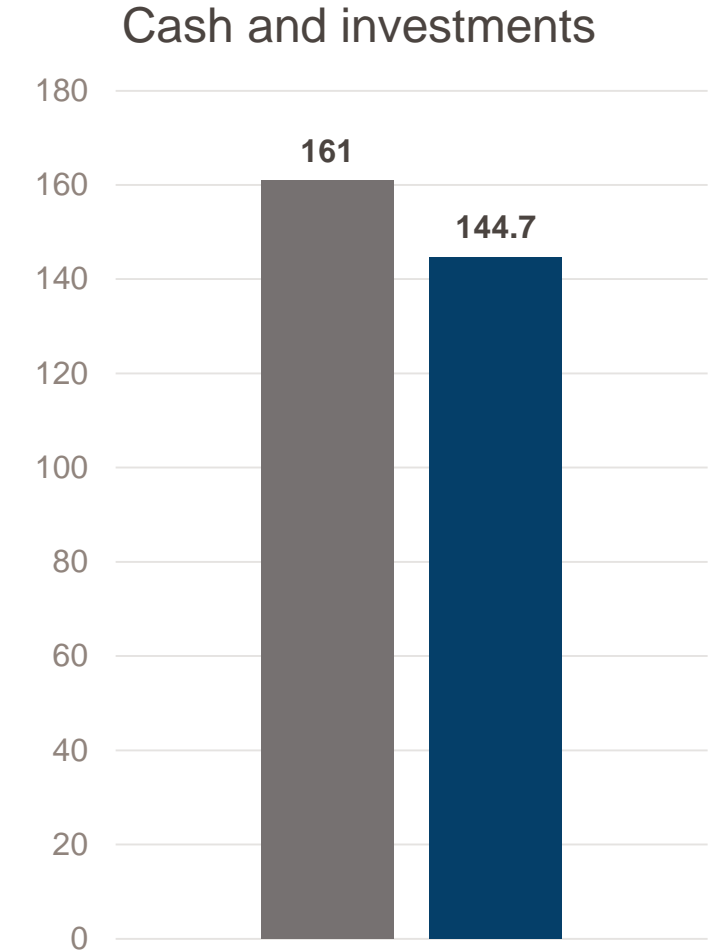
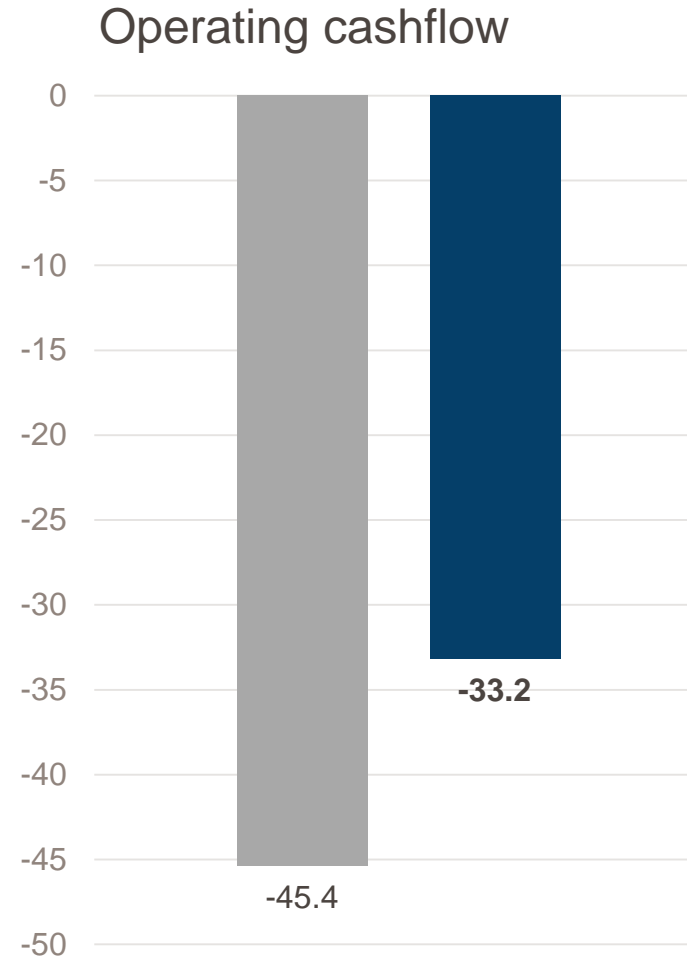
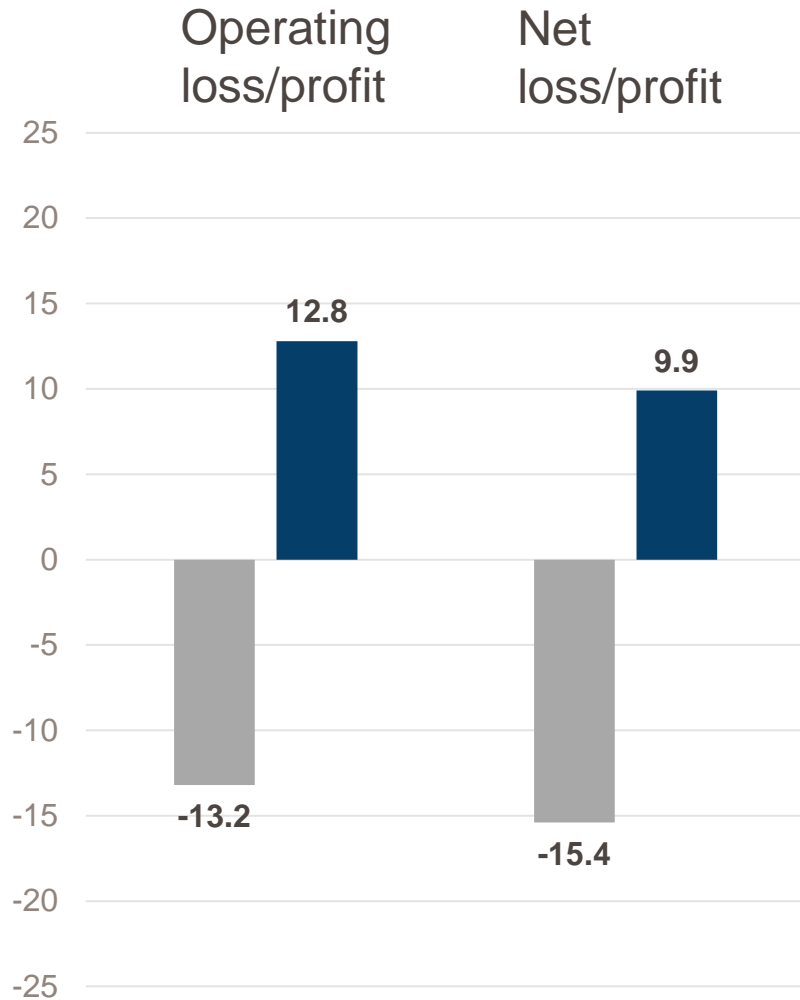


Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently



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

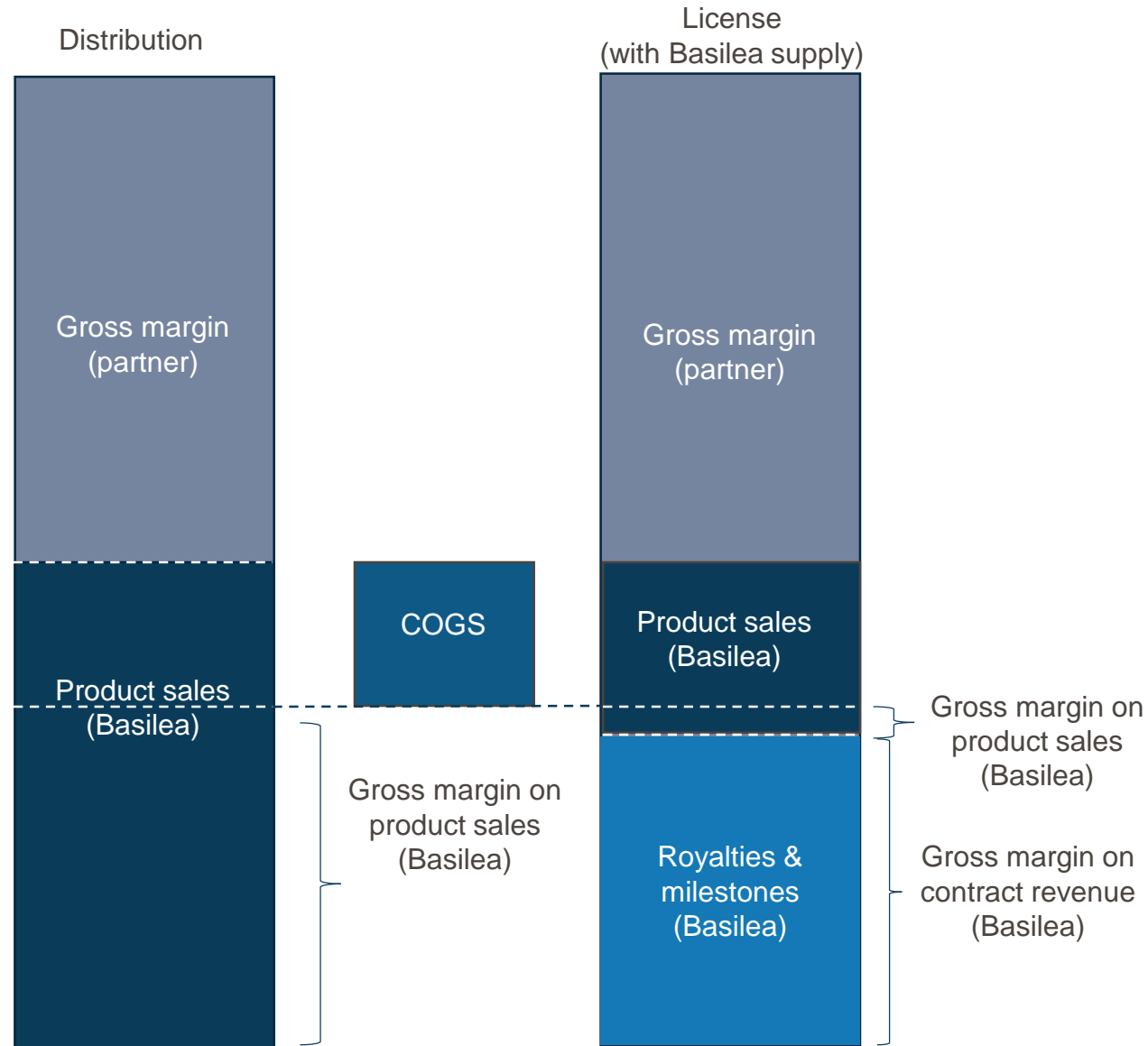
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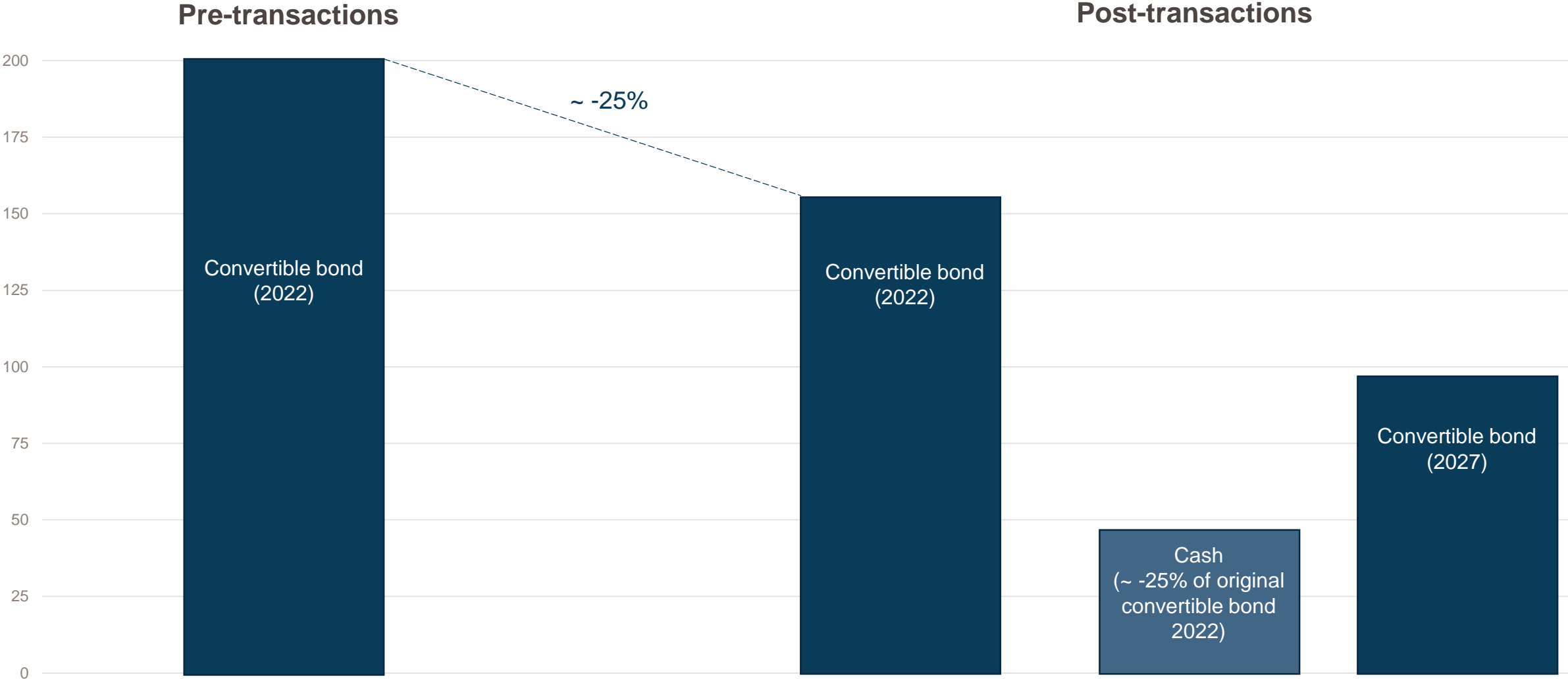
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

# 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)



# 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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