



**Focused on  
Growth and Innovation**

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

Investor presentation

September 30, 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



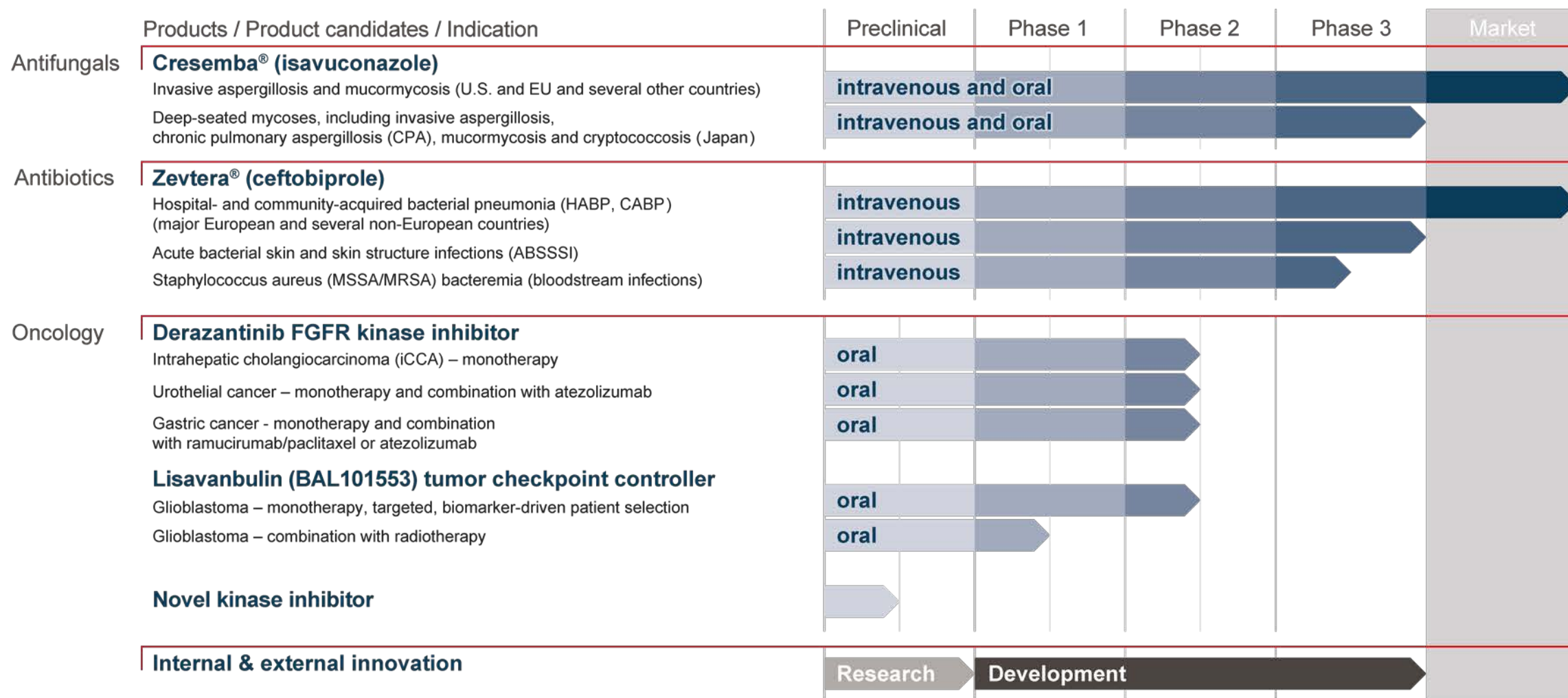


# At a glance

- 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



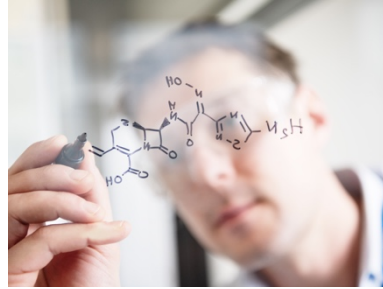


# Our strategy



## 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

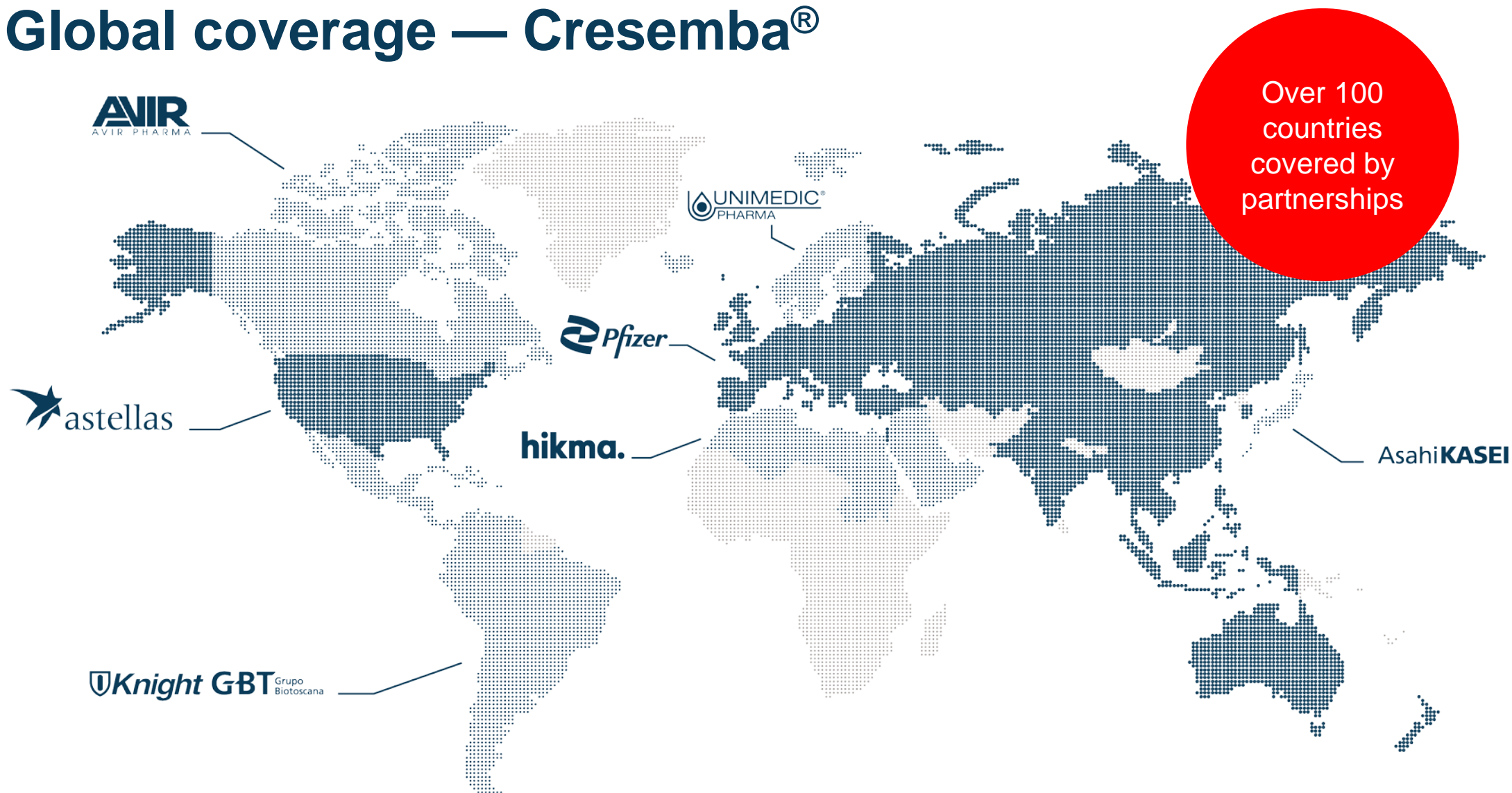


## Innovate

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®)



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





## 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® (isavuconazole)**

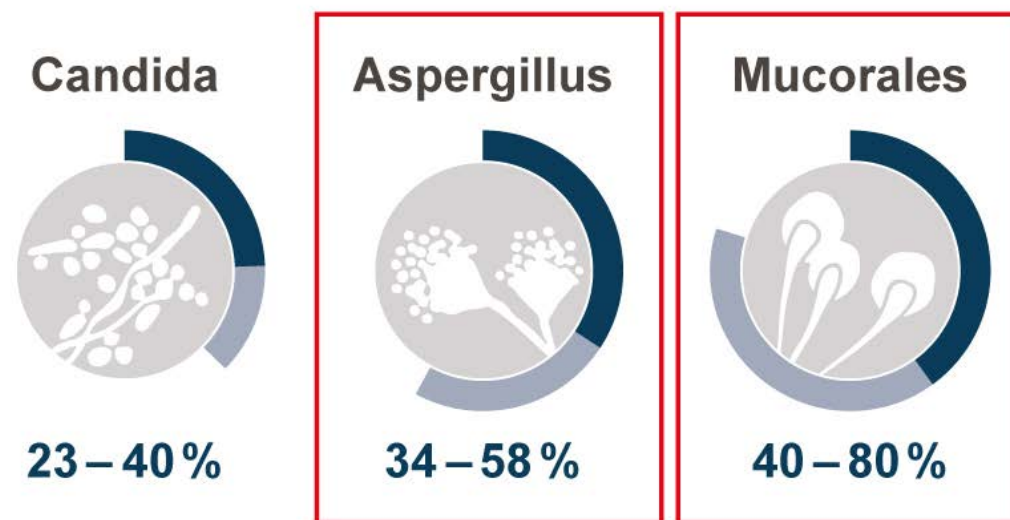
Invasive mold infections



# 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

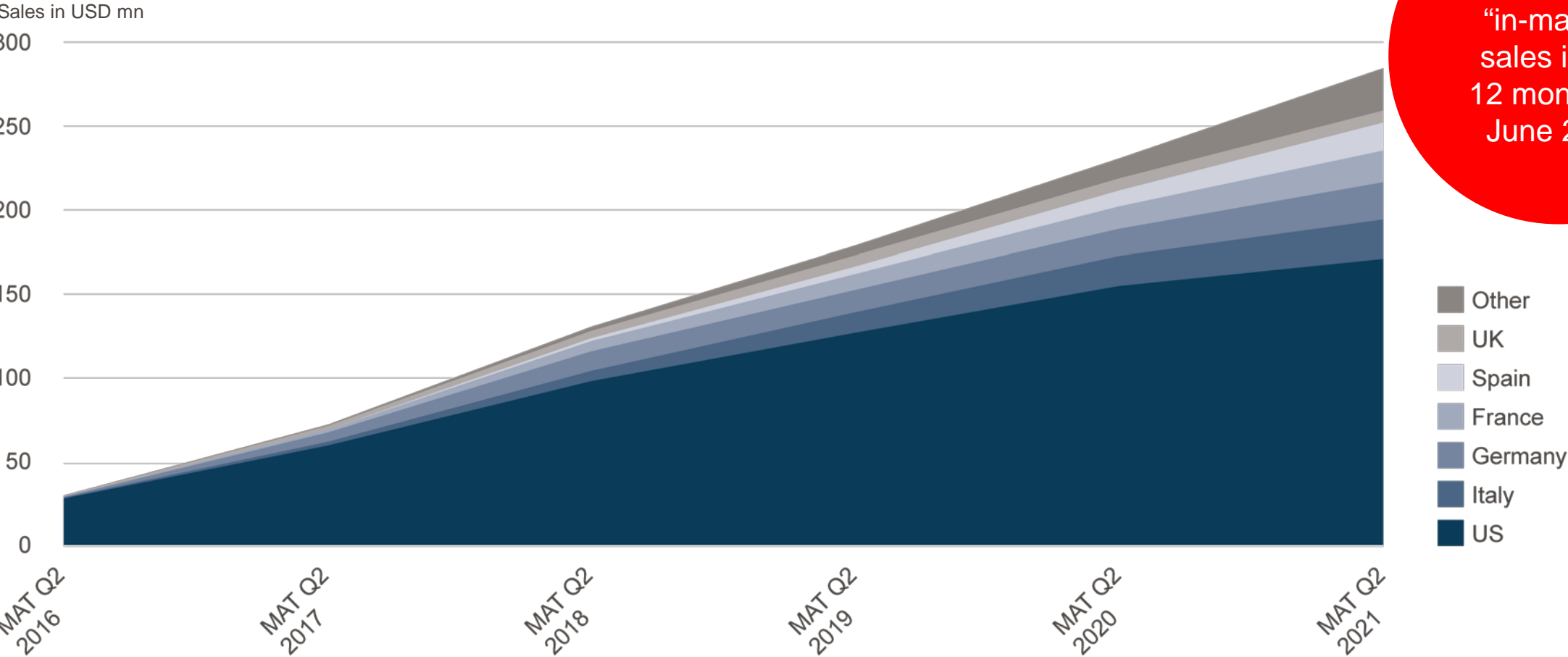
## Mortality rates for invasive fungal infections\*\*



\*\*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 285 mn  
“in-market”  
sales in the  
12 months to  
June 2021

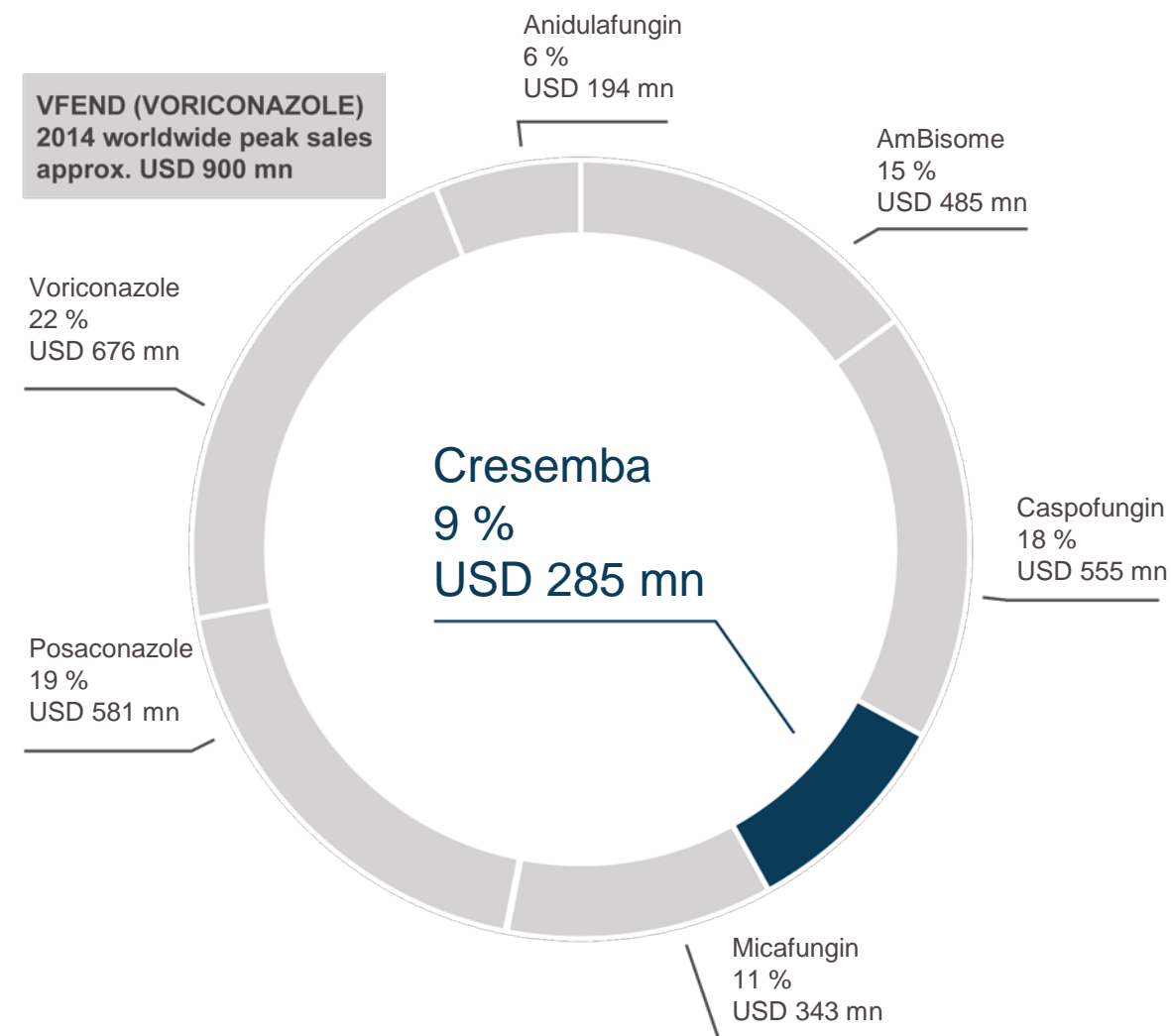
MAT: Moving annual total; Source: IQVIA, June 2021

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

USD 3.1 bn sales (MAT Q2 2021)

- 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; Source: IQVIA, June 2021

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

# Cresemba® — 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® 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>**  
**(ceftobiprole)**

Severe bacterial infections



# Zevtera® — 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

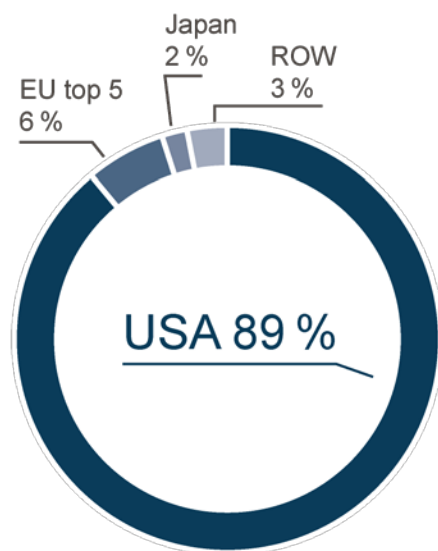


Focused on Growth and Innovation

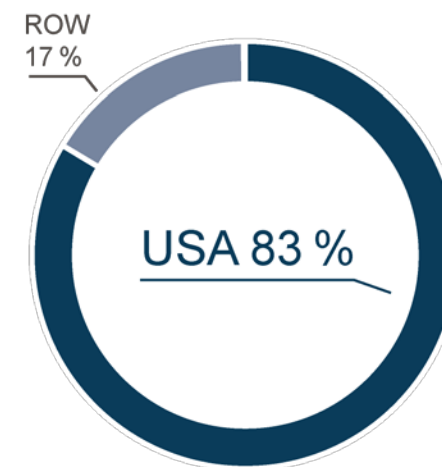


# The hospital anti-MRSA antibiotic market — A USD 2.6 bn market\* with the U.S. being the most important region

Daptomycin sales by region  
(2015, before LOE)



Ceftaroline sales by region  
(MAT Q2 2021)



\* Vancomycin, linezolid, teicoplanin, daptomycin, tigecycline, telavancin, ceftaroline, dalbavancin, ceftobiprole, oritavancin and tedizolid (daptomycin and tigecycline are partial sales in the USA in IQVIA data)

MRSA: Methicillin-resistant *Staphylococcus aureus*; LOE: Loss of exclusivity; ROW: Rest of world; MAT: Moving annual total; Source: IQVIA, June 2021



# 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)<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

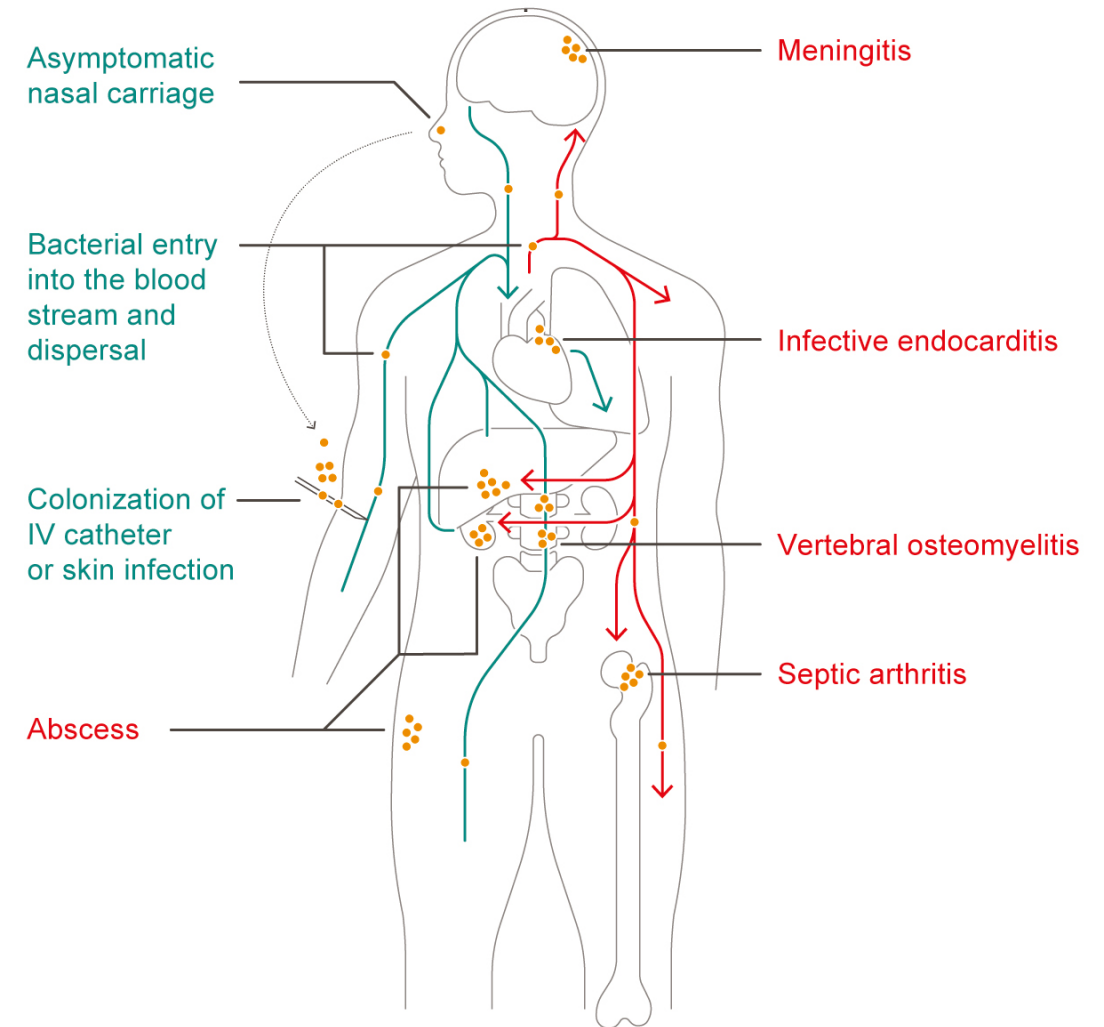
<sup>1</sup> MMWR, 2019;68:214–219.

<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 have long, thin, hair-like projections extending from them. The background is a solid orange color.

Oncology

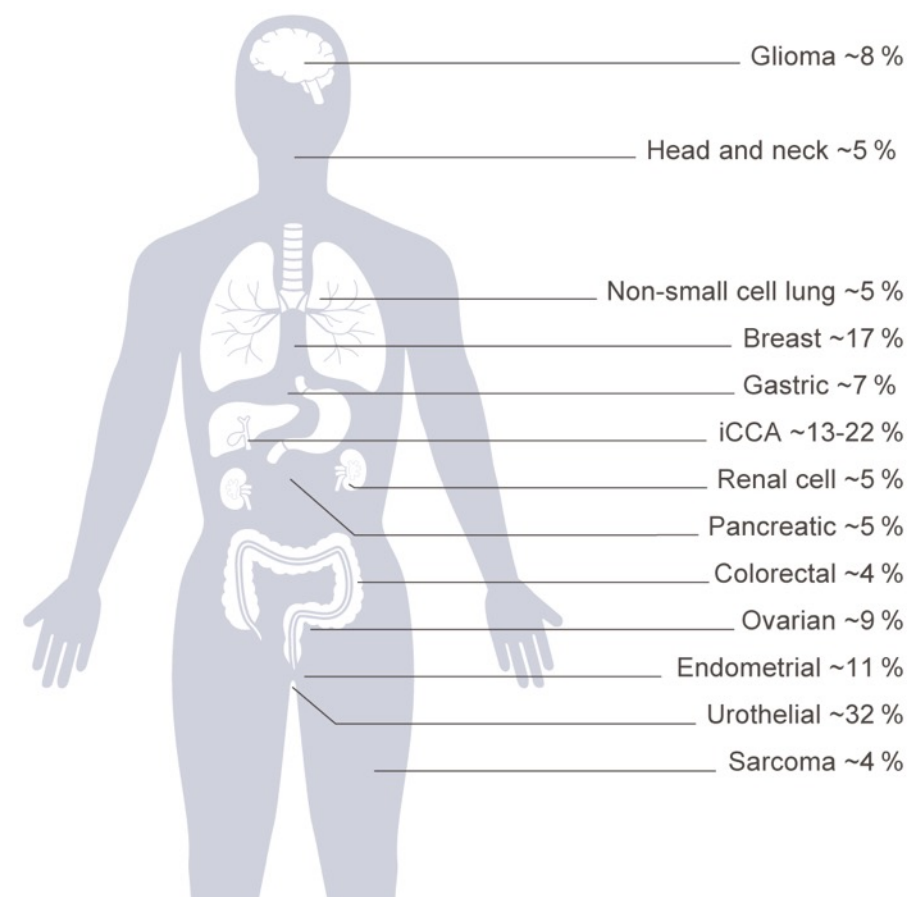
# Derazantinib

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 <sup>1</sup> FIDES-01 Cohort 1	Infigratinib <sup>2</sup> (QED)	Pemigatinib <sup>3</sup> (Incyte) FIGHT-202	Futibatinib <sup>4</sup> (Taiho) FOENIX-CCA2
N	103	108	108	103
Objective response rate	21%	23%	37%	42%
Disease control rate	76 %	84%	82%	83%
Median Progression-free survival	8.0 months	7.3 months	7.0 months	9.0 months

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

- 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

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

\*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. Droz Dit Busset et al., ESMO 2021 and Basilea data on file. 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



# 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

# 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% <sup>#</sup>
Alopecia†	20%	38%	NR	49%	40%	26%
Dry eye/xerophthalmia†	16%	32%	NR	35% <sup>#</sup>	NR	28% <sup>#</sup>
Retinopathy†	0%	NR	9%	6% <sup>‡</sup>	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% <sup>#</sup>	NR	41% <sup>#</sup>
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

<sup>#</sup> 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

<sup>‡</sup> 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; PPES: Palmar-plantar erythrodysesthesia; NR: not reported; QD: daily; Q3W/Q4W: every 3/4 weeks; w: weeks

Oncology

# Lisavanbulin (BAL101553)

Glioblastoma  
and other solid tumors





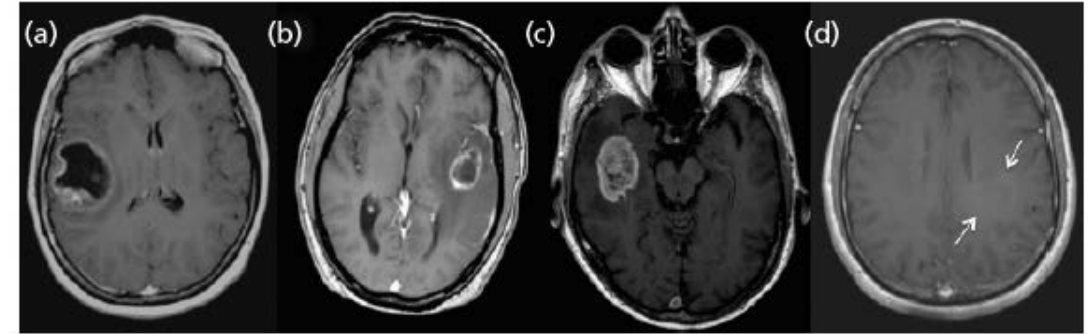
# Unmet medical need in glioblastoma

- 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)<sup>1</sup>
- *MGMT*-promoter methylation status has been demonstrated as a predictor for the response to (radio)chemotherapy (temozolomide)<sup>2</sup>
- Established molecular markers used for classification include IDH mutations and/or 1p/19q codeletion<sup>3</sup>
- No molecular targeted therapy currently approved

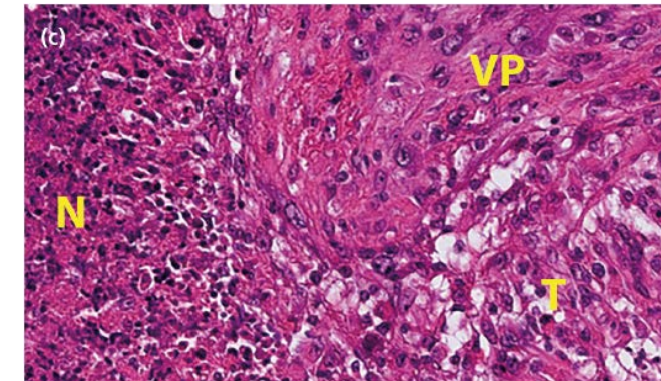
<sup>1</sup>Poon MTC et al. 2020; Sci Rep 10, 11622; <sup>2</sup>Hegi et al. NEJM 2005;352:997-1003

<sup>3</sup>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)



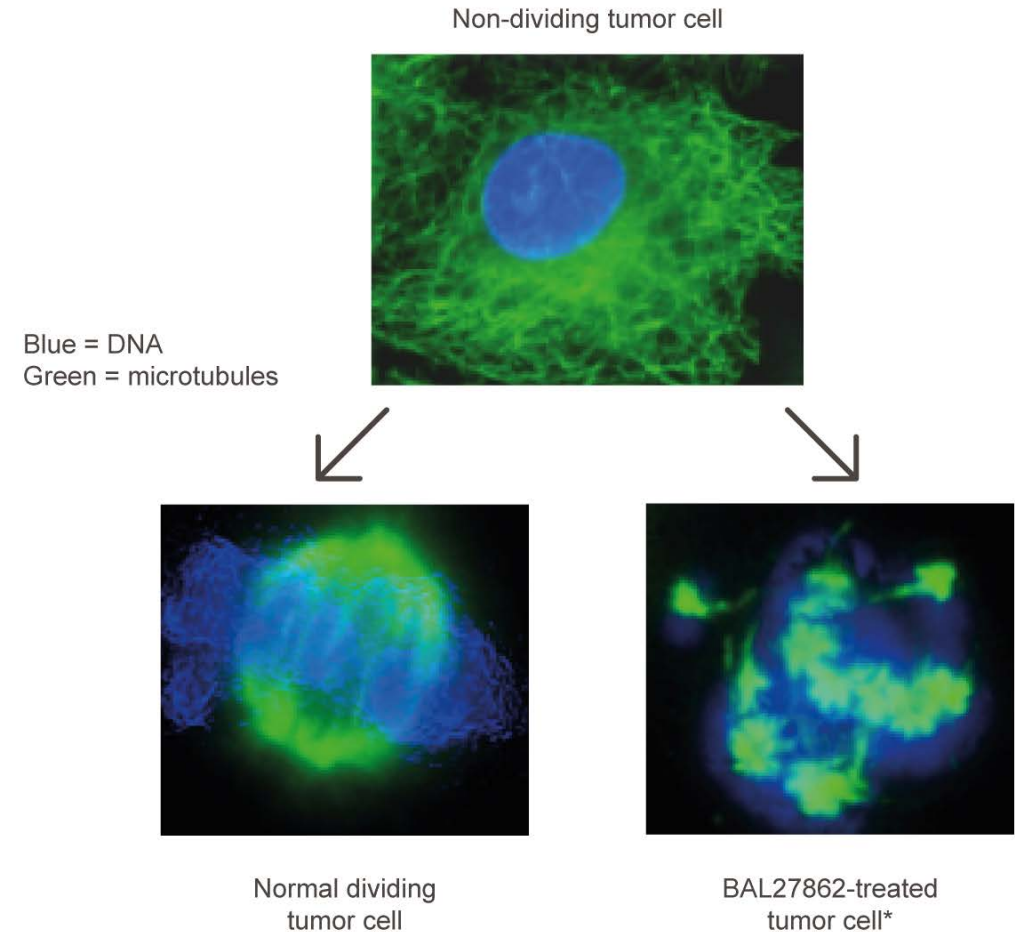
Histological glioblastoma; H&E stain.

100 μm

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

# 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
- Crosses the blood-brain barrier with potent activity in brain tumor models alone and in combination
- Comprehensive biomarker program to optimize patient selection, e.g. EB1 (end-binding protein 1)
- Orphan drug designation granted for the treatment of malignant glioma

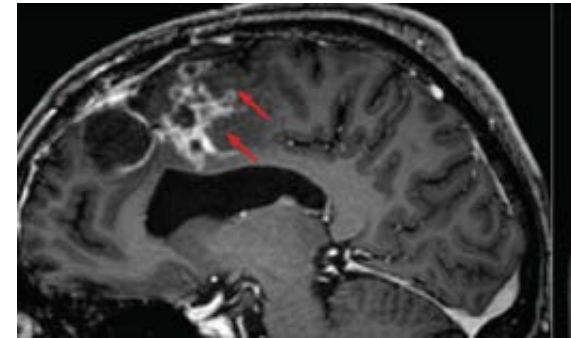


\* Lisavanbulin (BAL101553) is a prodrug of BAL27862

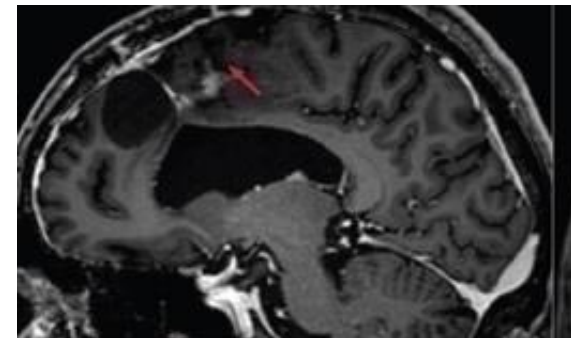
# Biomarker-driven phase 2 study ongoing in recurrent glioblastoma

- 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):<sup>1, 2</sup>
  - 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
- Phase 2 interim results expected H2 2021

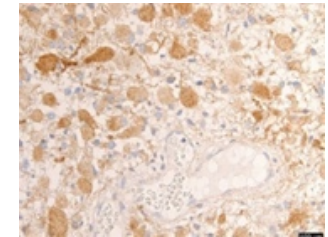
## Glioblastoma tumor size reduction in an exceptional responder and EB1 staining of glioblastoma tissue compared to non-responding patients



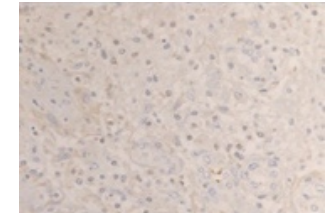
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



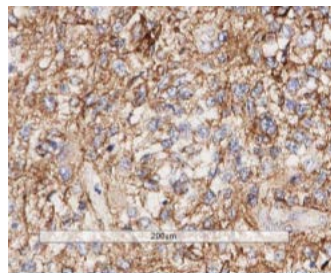
Non-responder

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

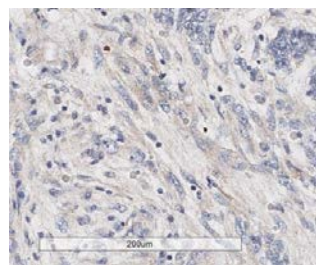
<sup>2</sup> Tiu et al. JCO 2021;39,15 suppl, TPS2068 (NCT02490800)

# EB1-prevalence in glioblastoma and other cancer types

Example of an EB1-positive and EB1-negative glioblastoma tissue sample<sup>1</sup>

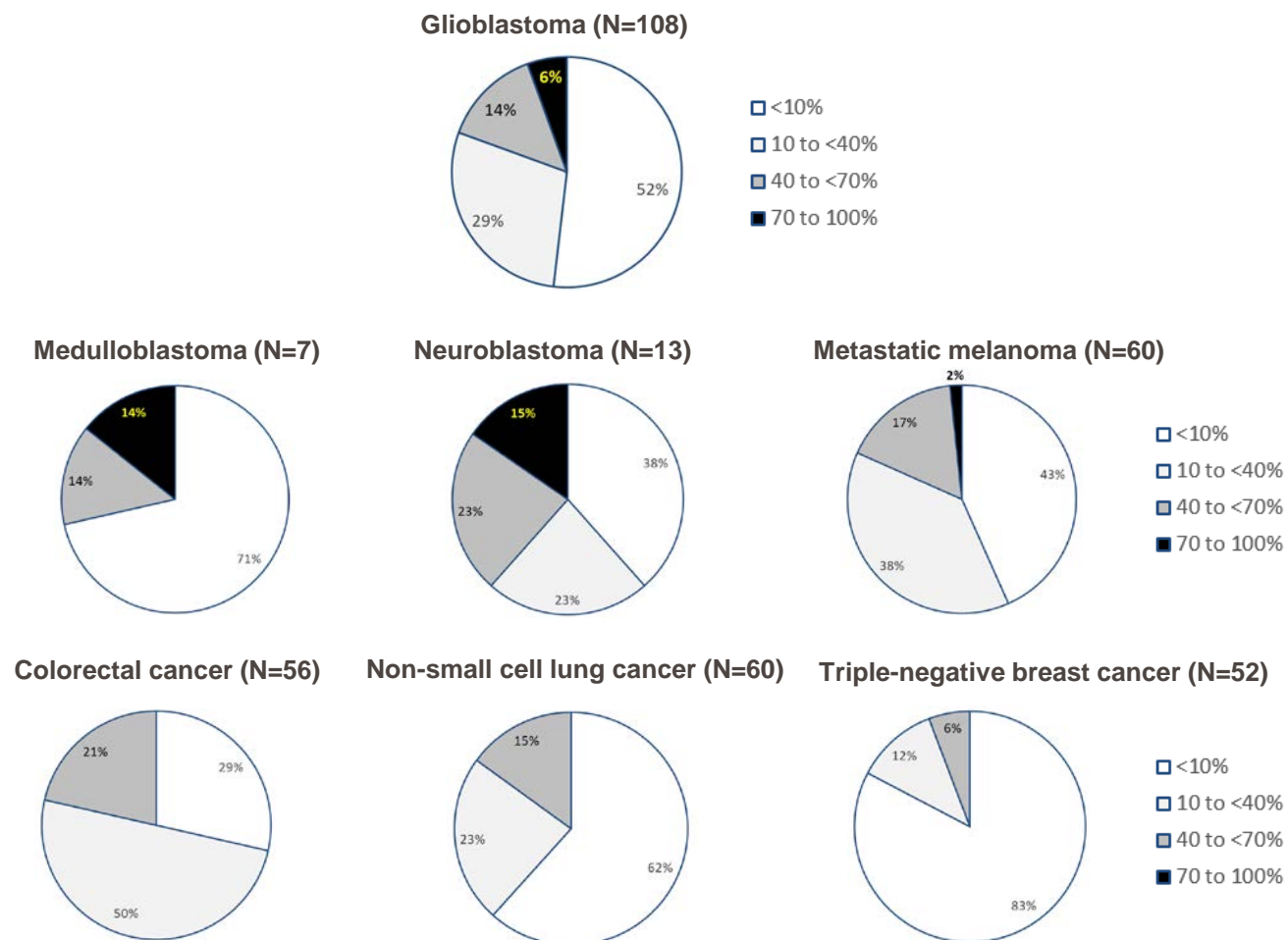


**EB1-positive:**  
Tumor cells show moderate to strong EB1 staining



**EB1-negative:**  
Absence of moderate to strong EB1 staining

Prevalence of moderate/strong EB1 staining in various tumor types<sup>1</sup>



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





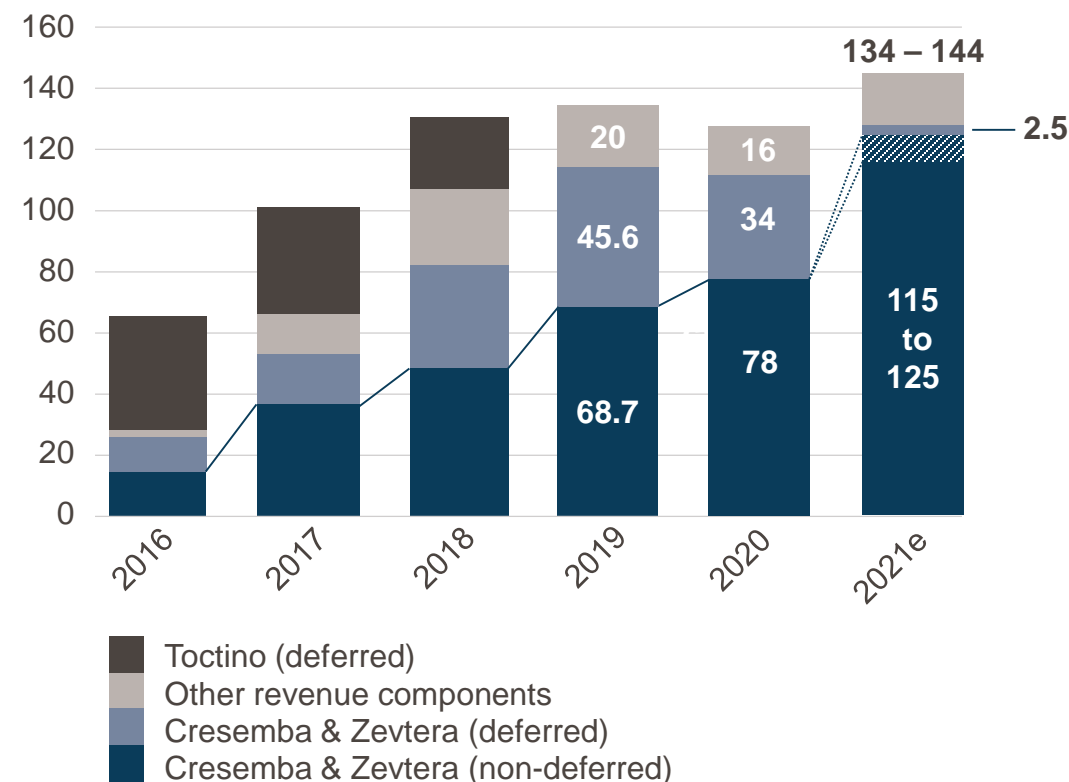
## Financials & Outlook



# 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 deferred	115 – 125 2.5	108–118 2.5	78.2 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

# Outlook 2021 / 2022

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

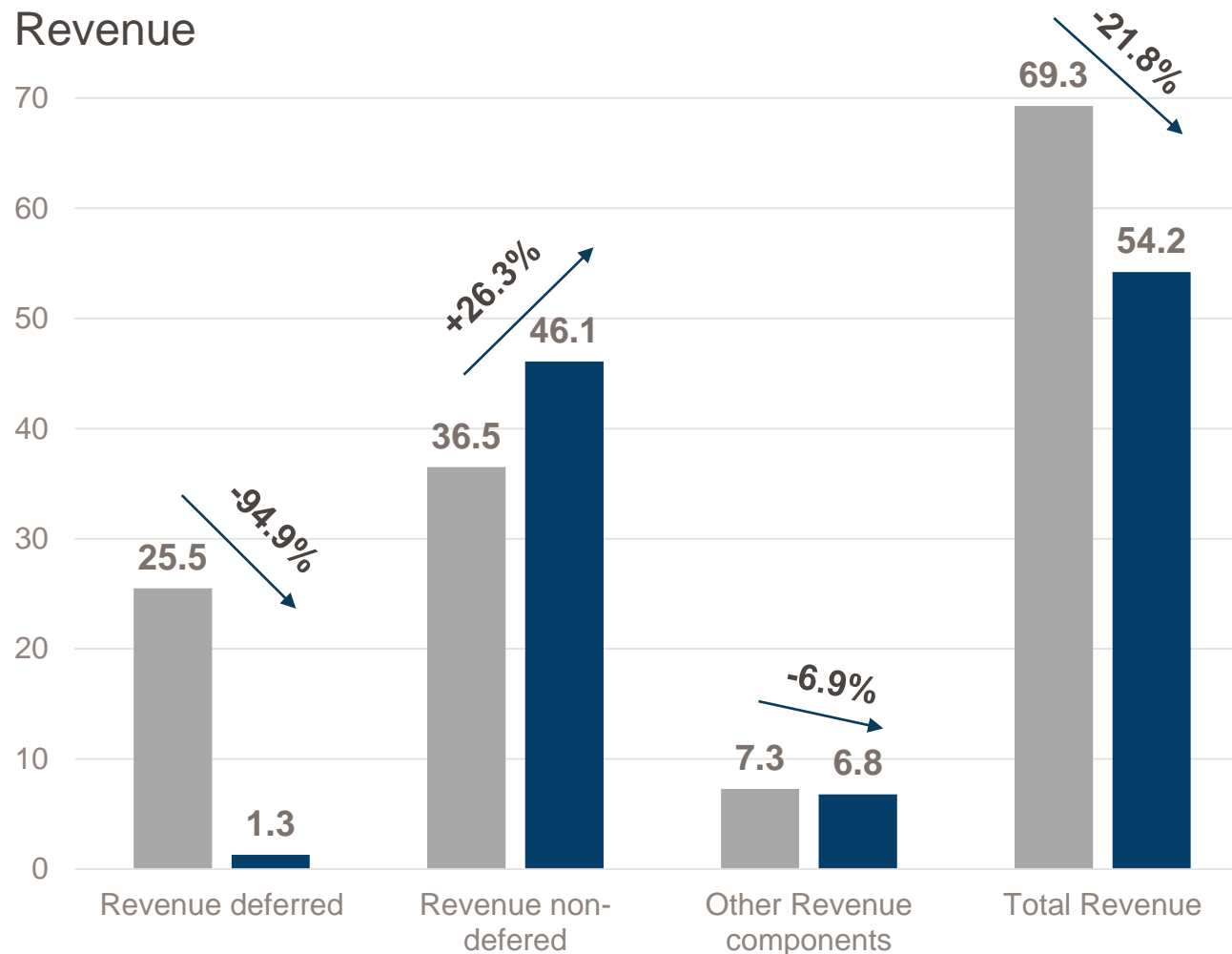
		H1 2021	H2 2021	H1 2022	H2 2022
<b>Isavuconazole</b>		✓ Complete patient enrolment in phase 3 study in Japan	✓ File NDA in Japan		
<b>Ceftobiprole</b>			Complete patient enrolment in SAB phase 3 study	Topline results from SAB phase 3 study	
<b>Derazantinib</b>	<b>FIDES-01 (iCCA)</b>	✓ Topline results (FGFR2 gene fusions)			
		✓ Interim results (other FGFR2 genetic aberrations)		Topline results (other FGFR2 genetic aberrations)	
	<b>FIDES-02 (urothelial cancer)</b>		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	
	<b>FIDES-03 (gastric cancer)</b>			Interim results in monotherapy (400 mg/day) and recommended phase 2 dose with ramucirumab/paclitaxel	Interim efficacy results in combination with ramucirumab/paclitaxel
<b>Lisavanbulin</b>			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		
<b>Novel kinase inhibitor (for cancer therapy)</b>			File IND application	Initiate phase 1 study	



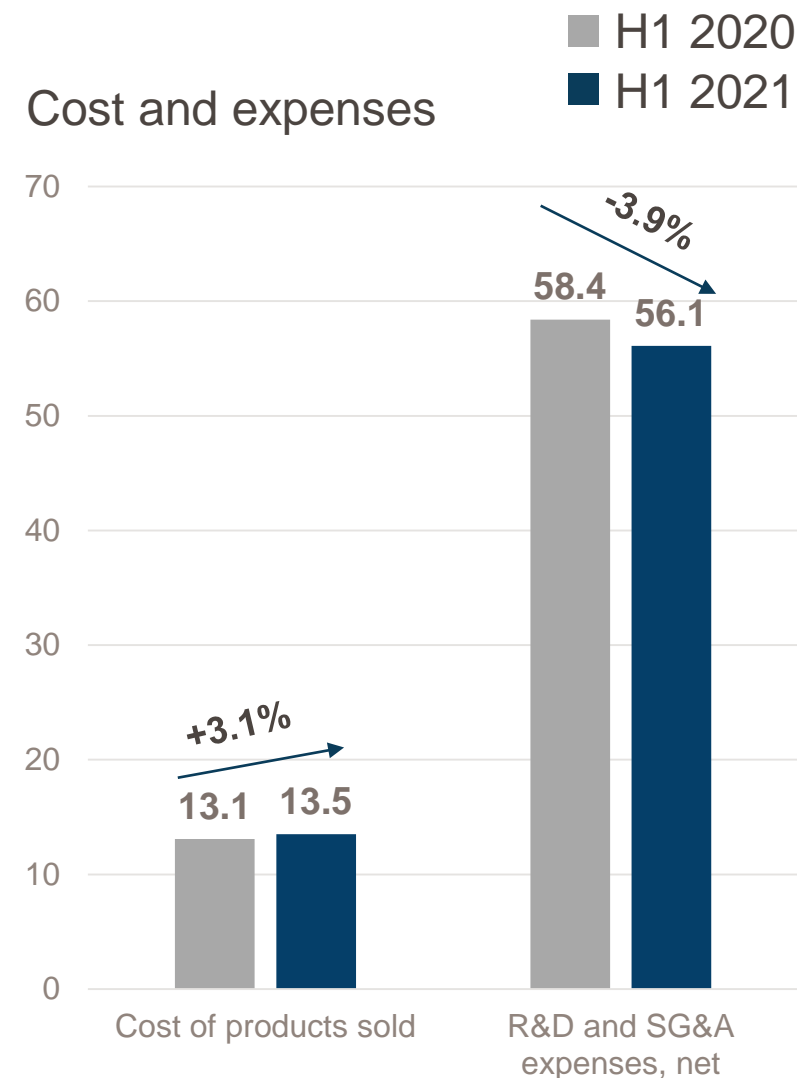
# Appendix

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

## Revenue

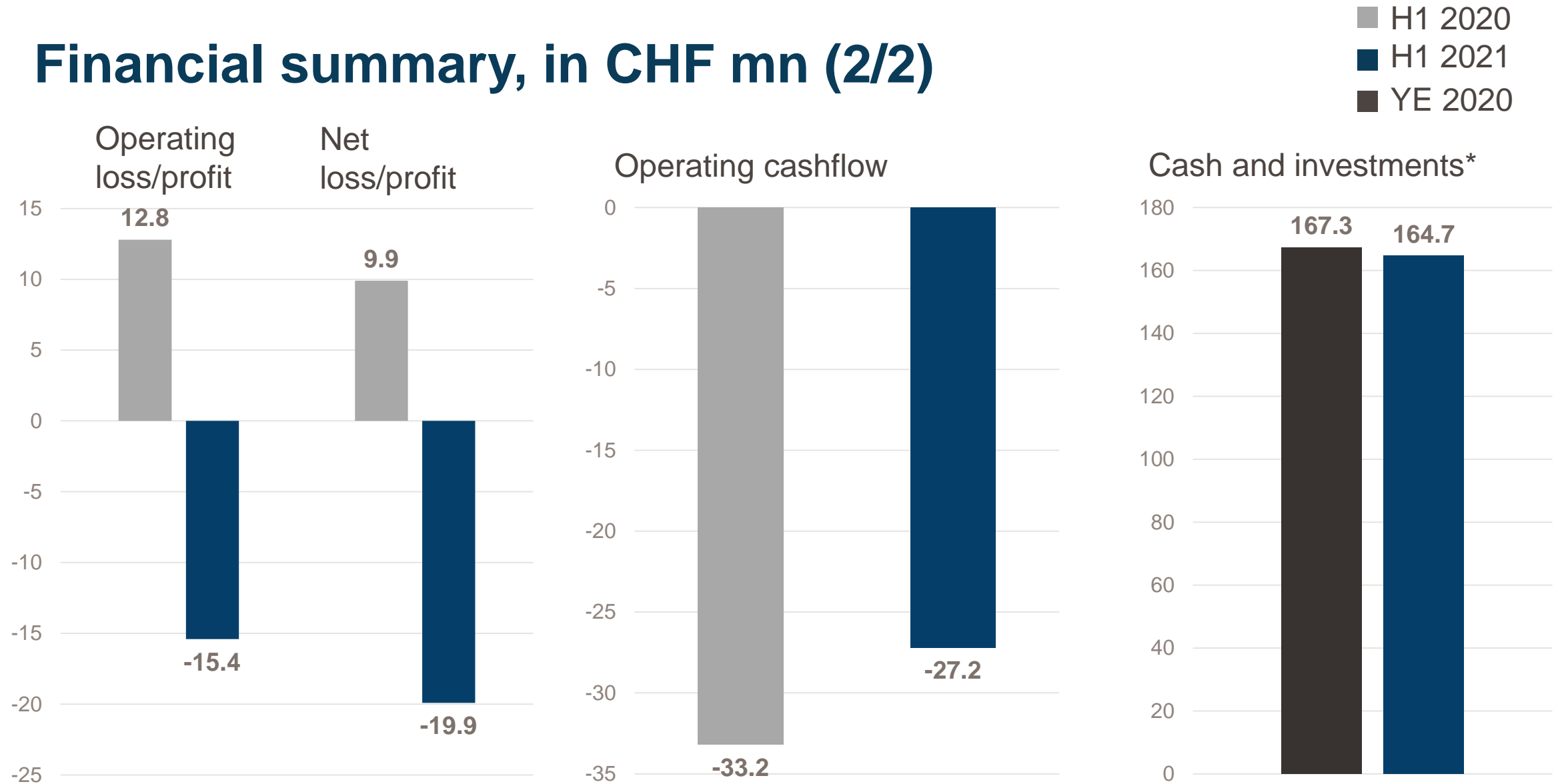


## Cost and expenses



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

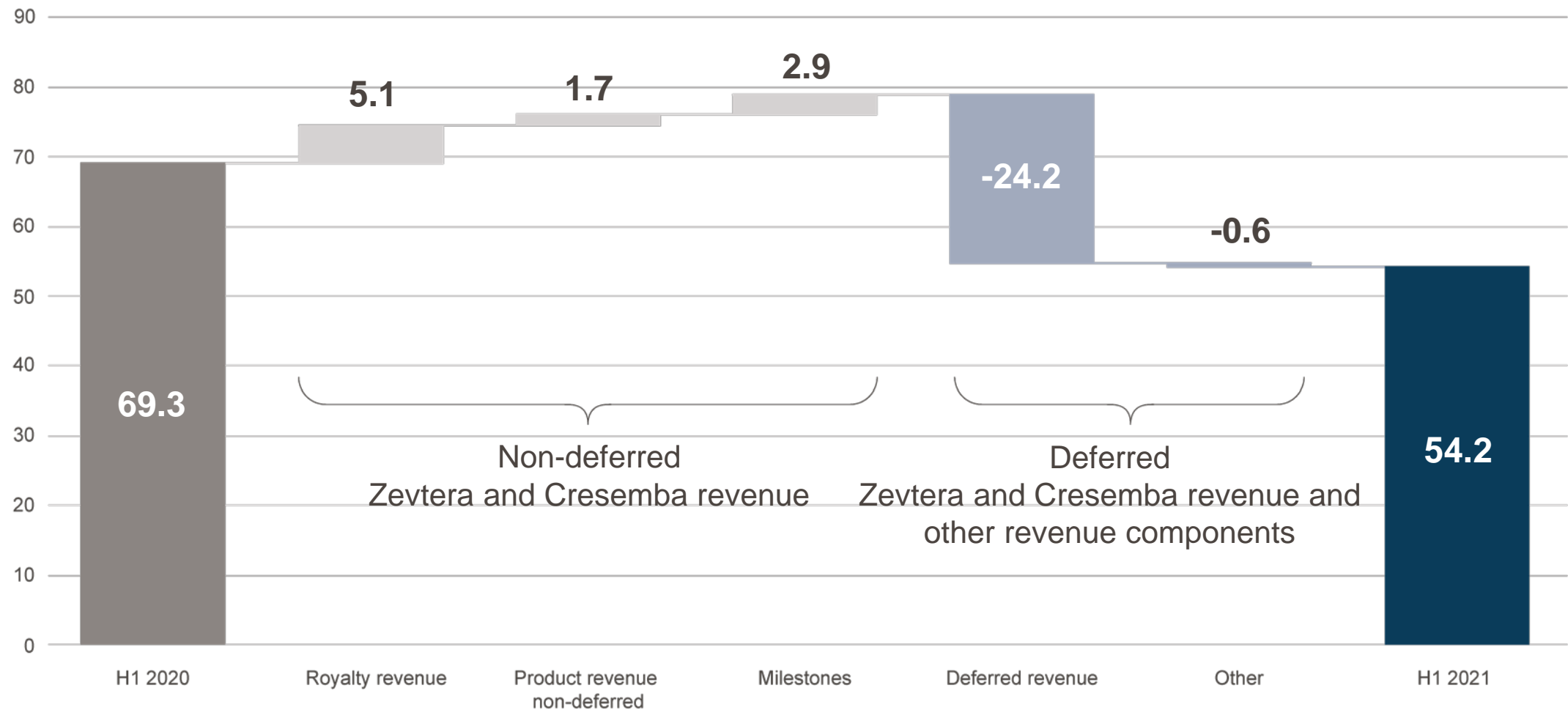
# Financial summary, in CHF mn (2/2)



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

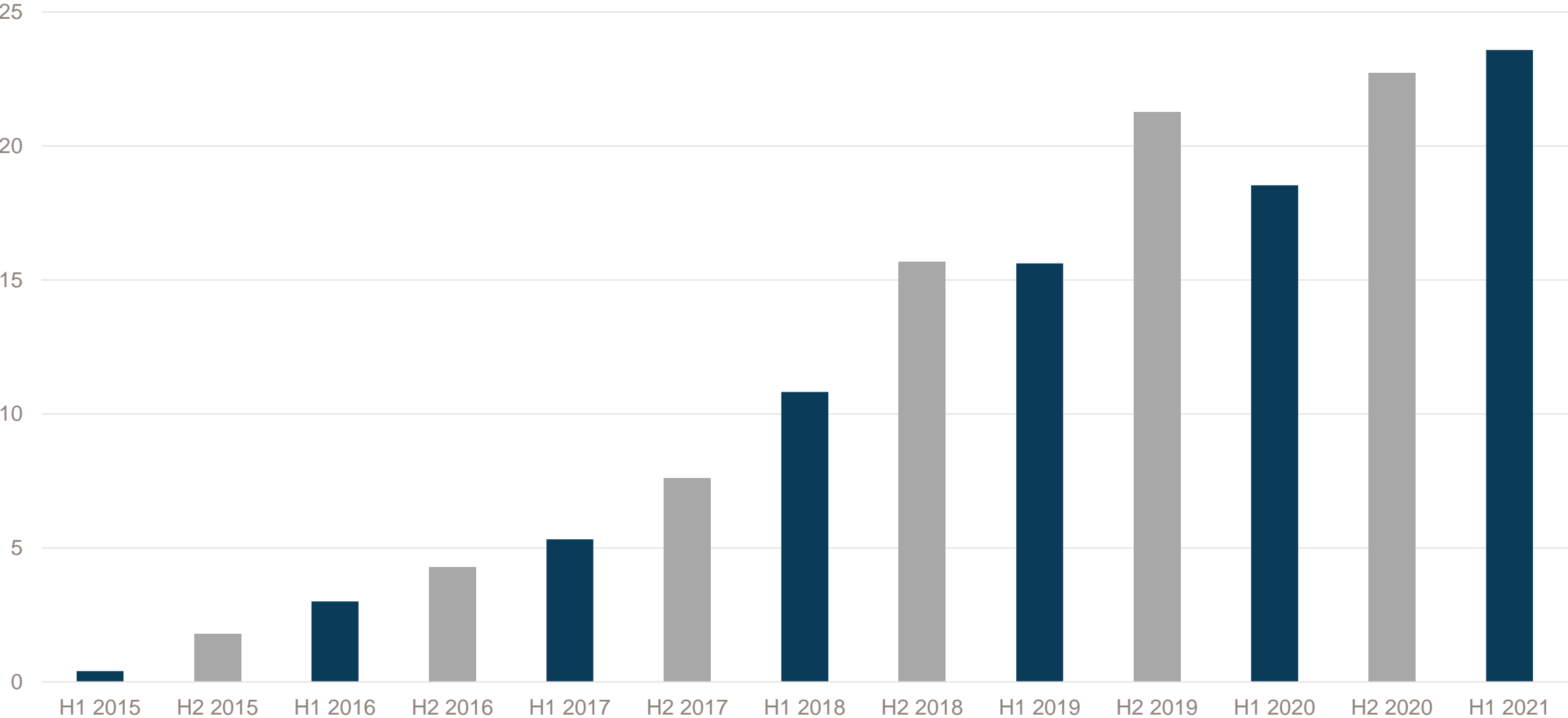


# Significant growth in non-deferred revenues based on higher royalties, product revenue and milestones (in CHF mn)



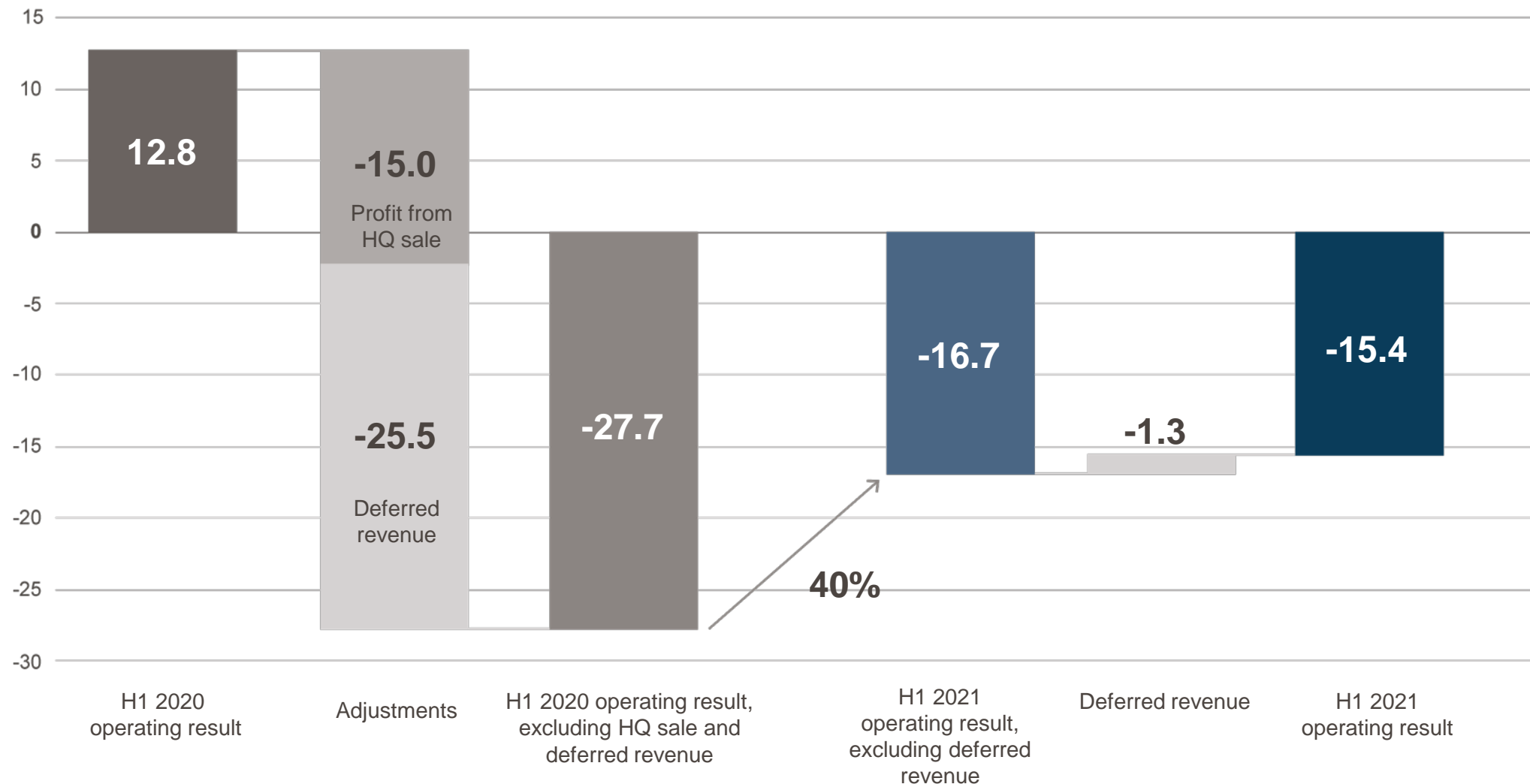
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

# 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

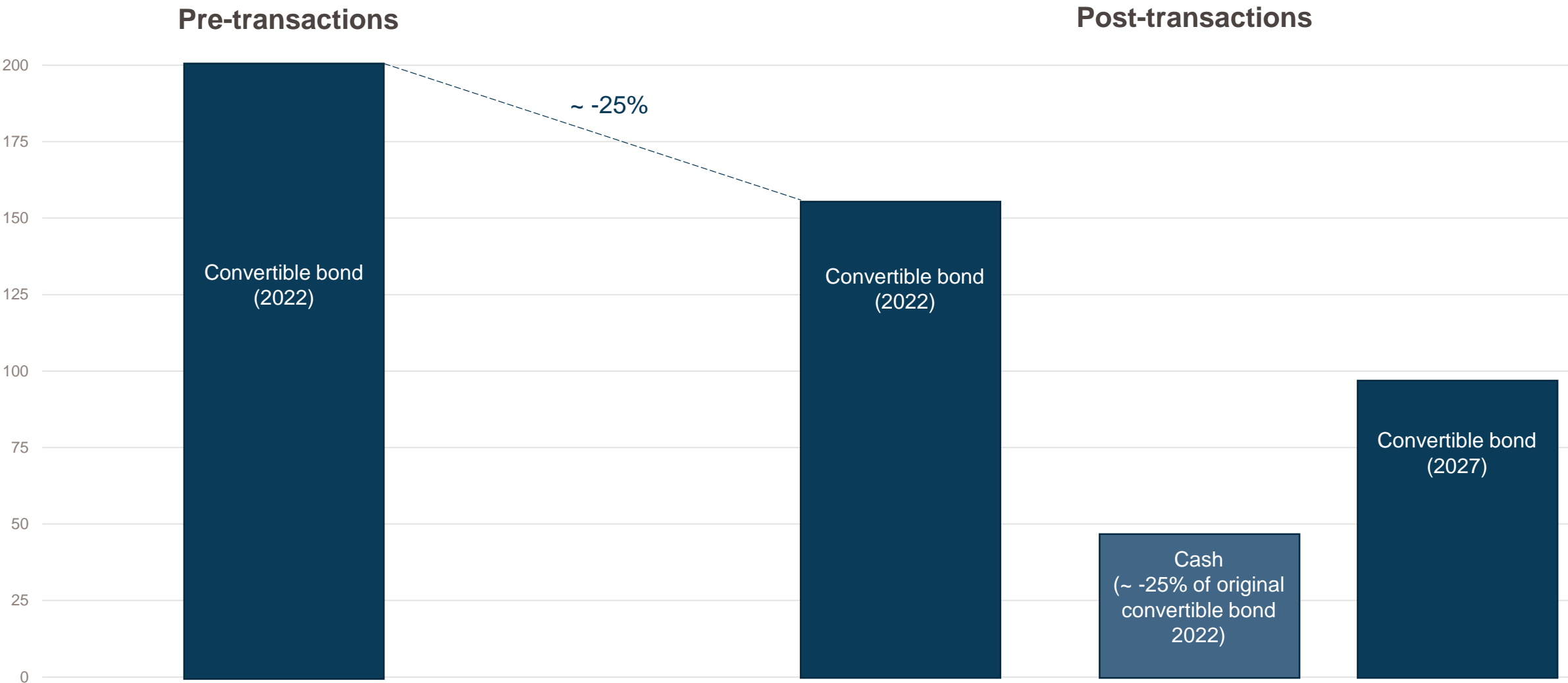
# Significant improvement in underlying operating performance (CHF mn)



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



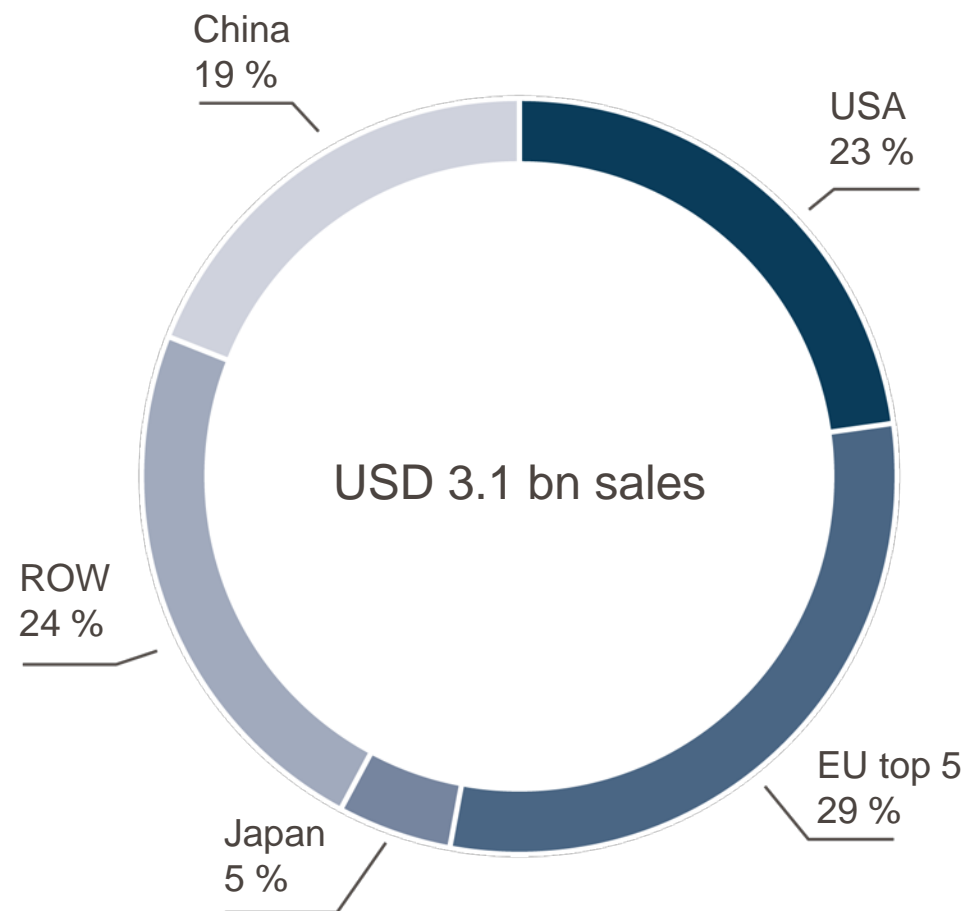
# Convertible bond transactions — successfully improved debt maturity profile (in CHF mn)



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

USD 3.1 bn sales of best-in-class antifungals\*  
(MAT Q2 2021)

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



MAT: Moving annual total; Source: IQVIA, June 2021

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# Ceftobiprole — Positive topline phase 3 results reported in ABSSSI

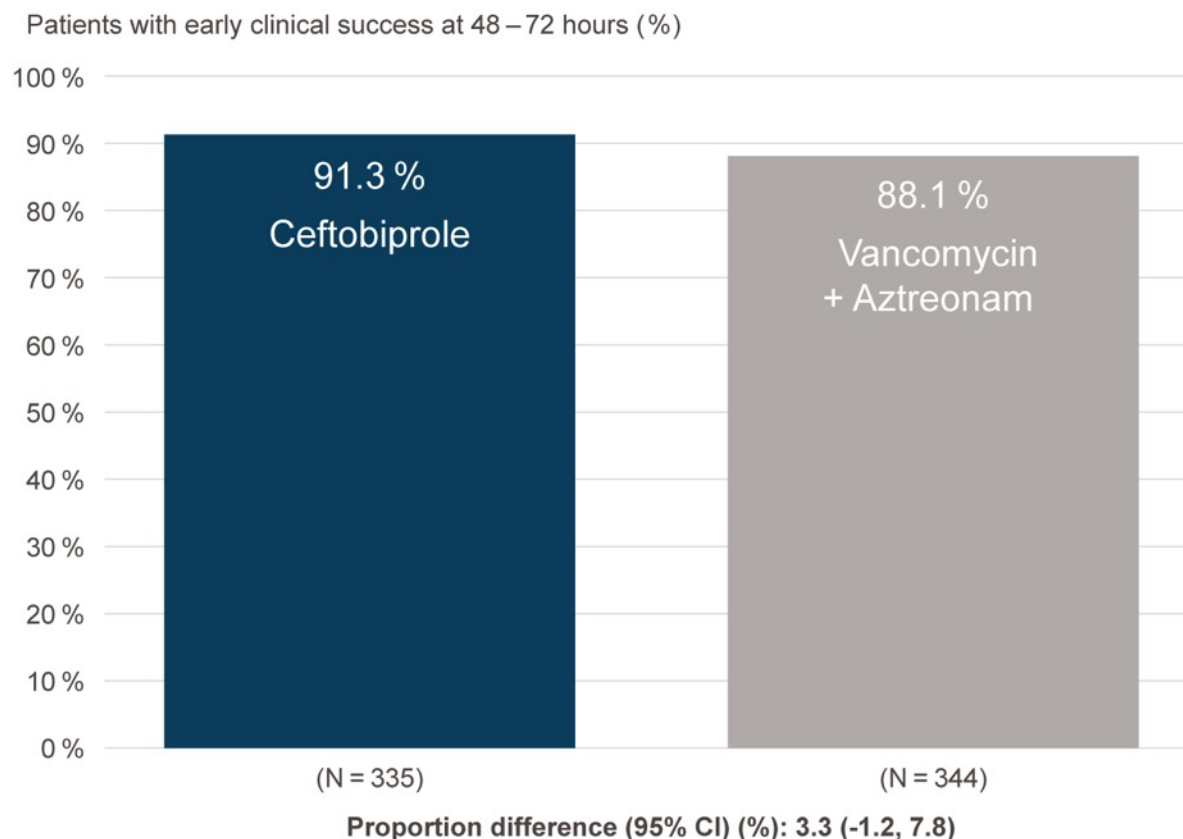
Key topline study<sup>1</sup> results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints



<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

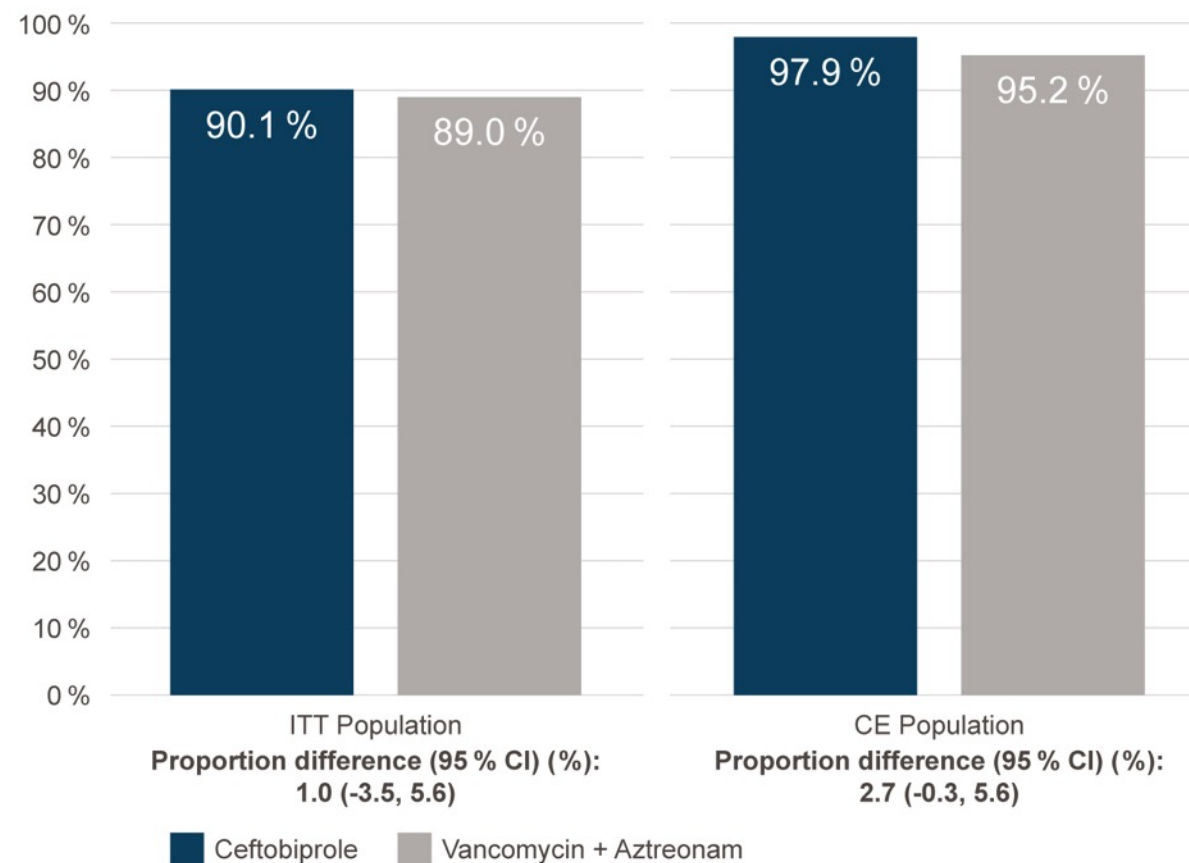


<sup>1</sup> NCT03137173

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>

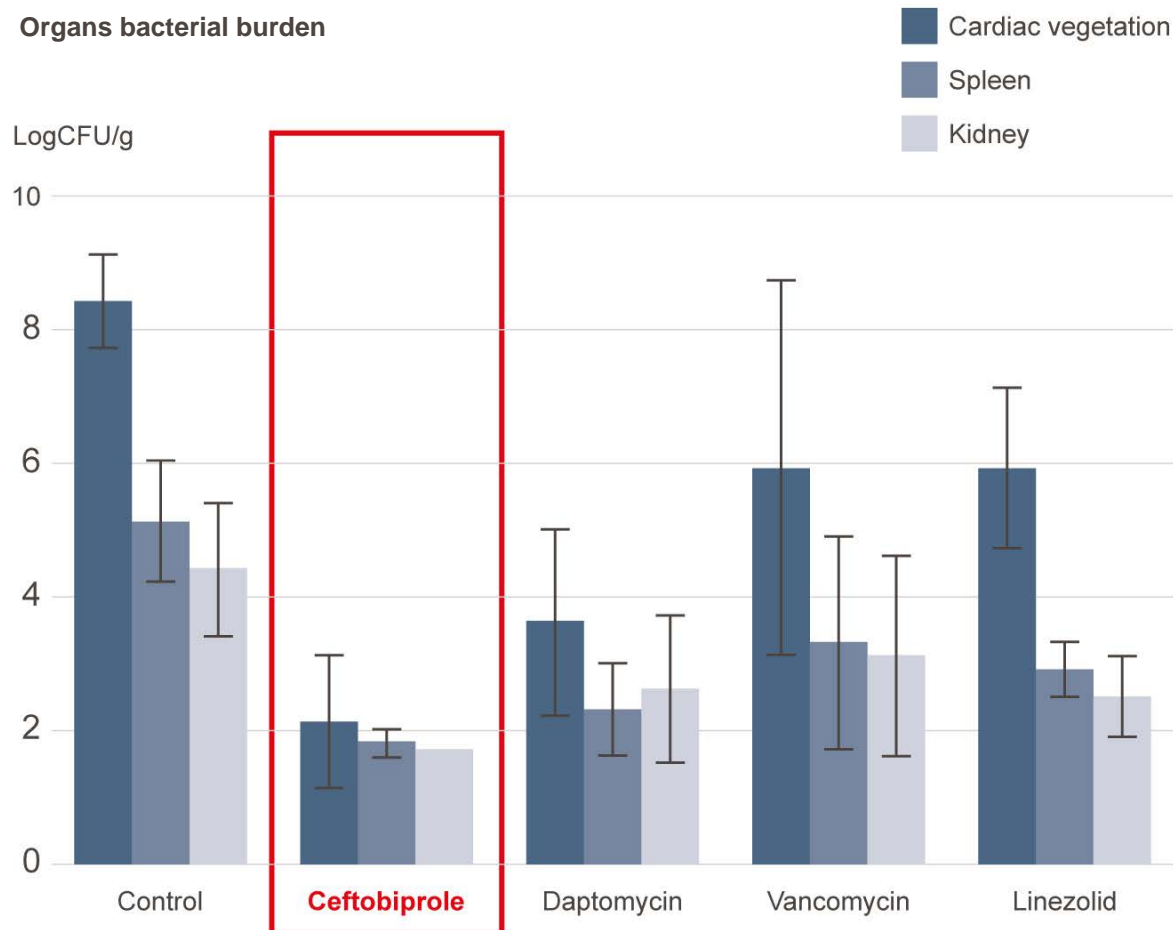
<sup>1</sup>Syed YY. Drugs. 2014;74:1523-1542.

<sup>2</sup>Tatteen 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

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

## FIDES-01 Cohort 1 (N=103)

### FGFR2 fusions (~15% of iCCA)

#### Topline results:

ORR: 21.4%

DCR: 74.8%

Median PFS: 7.8 months

- Consistent with earlier Phase1/2 data<sup>1</sup> and with interim analysis from FIDES-01
- Clinical proof of concept supporting anticancer efficacy and a favorable benefit to risk profile

## FIDES-01 Cohort 2 (N=43) - ongoing

### FGFR2 mutations/amplifications (~5% of iCCA)

#### Interim results (n=14):

DCR: 79% (1 confirmed CR, 1 unconfirmed PR, 9 SD)

#### Pooled data from 23 patients

(clinical studies/EAP)<sup>2</sup>

Median PFS 7.2 months

- Encouraging PFS in pooled analysis consistent with outcome in patients with FGFR2 gene fusions
- Interim analysis successfully completed based on at least 8 patients with PFS >3 months (PFS not yet mature)
- Topline results expected H1 2022

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

FIDES-01: NCT03230318

<sup>1</sup>Mazzaferro et al. Br J Cancer. 2019

<sup>2</sup>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

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

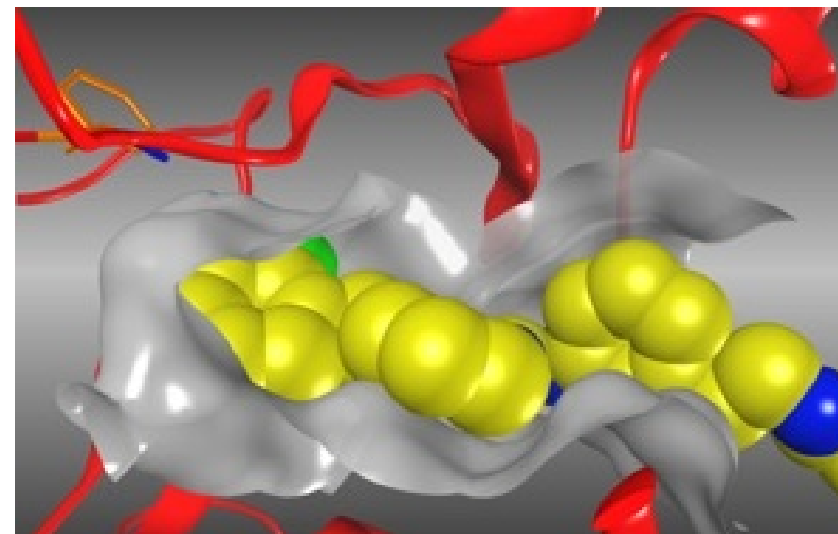
FGFR-inhibitor compound (Sponsor)	Parameter	FGFR1	FGFR2	FGFR3	FGFR4	CSF1R	VEGFR2
<b>Derazantinib</b> (Basilea)	Ratio to FGFR2 activity	4	1	4	77	3	6
<b>Pemigatinib</b> (Incyte)	Ratio to FGFR2 activity	3	1	4	39	231	62
<b>Erdafitinib</b> (Janssen)	Ratio to FGFR2 activity	2	1	2	13	95	6
<b>Rogaratinib</b> (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

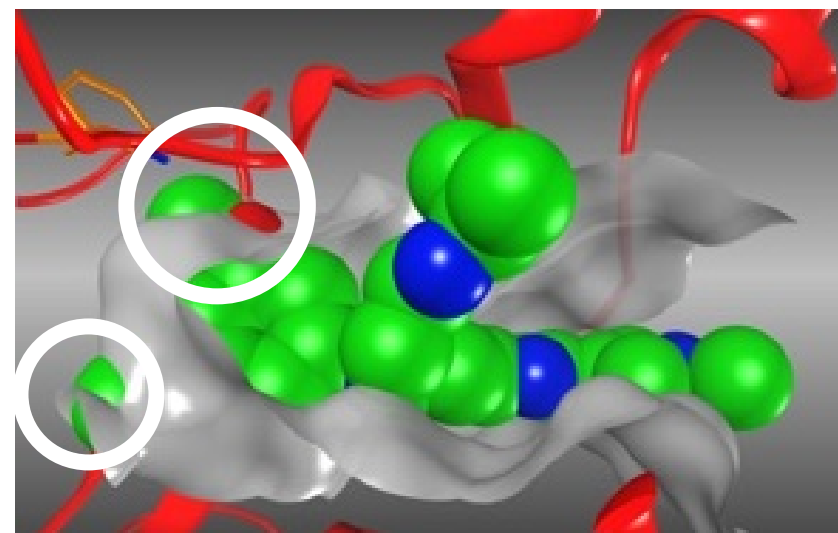


# In-silico analysis of derazantinib binding to CSF1R

- 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)



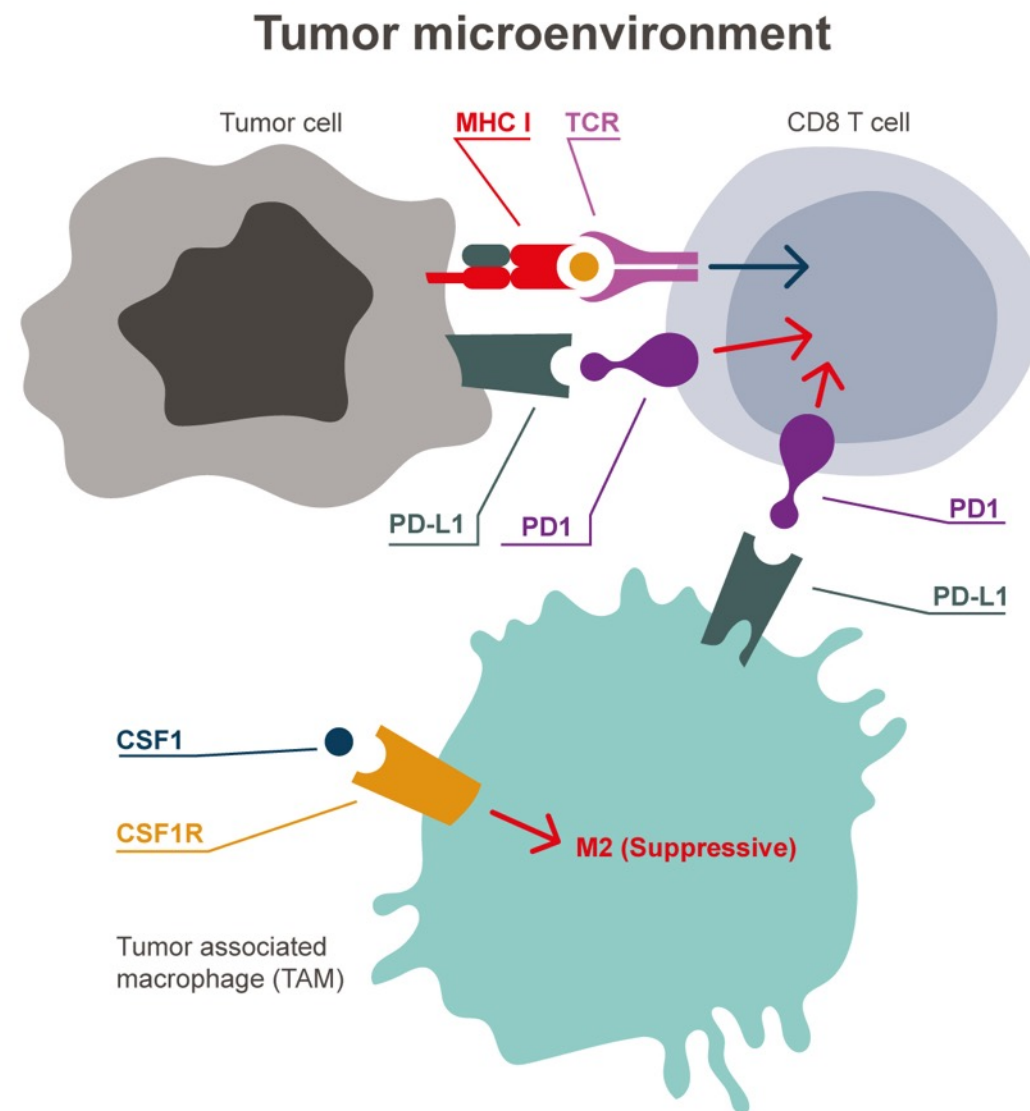
**Erdafitinib** (green) is too large (white circles) for the active site pocket of CSF1R (grey/red)

<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

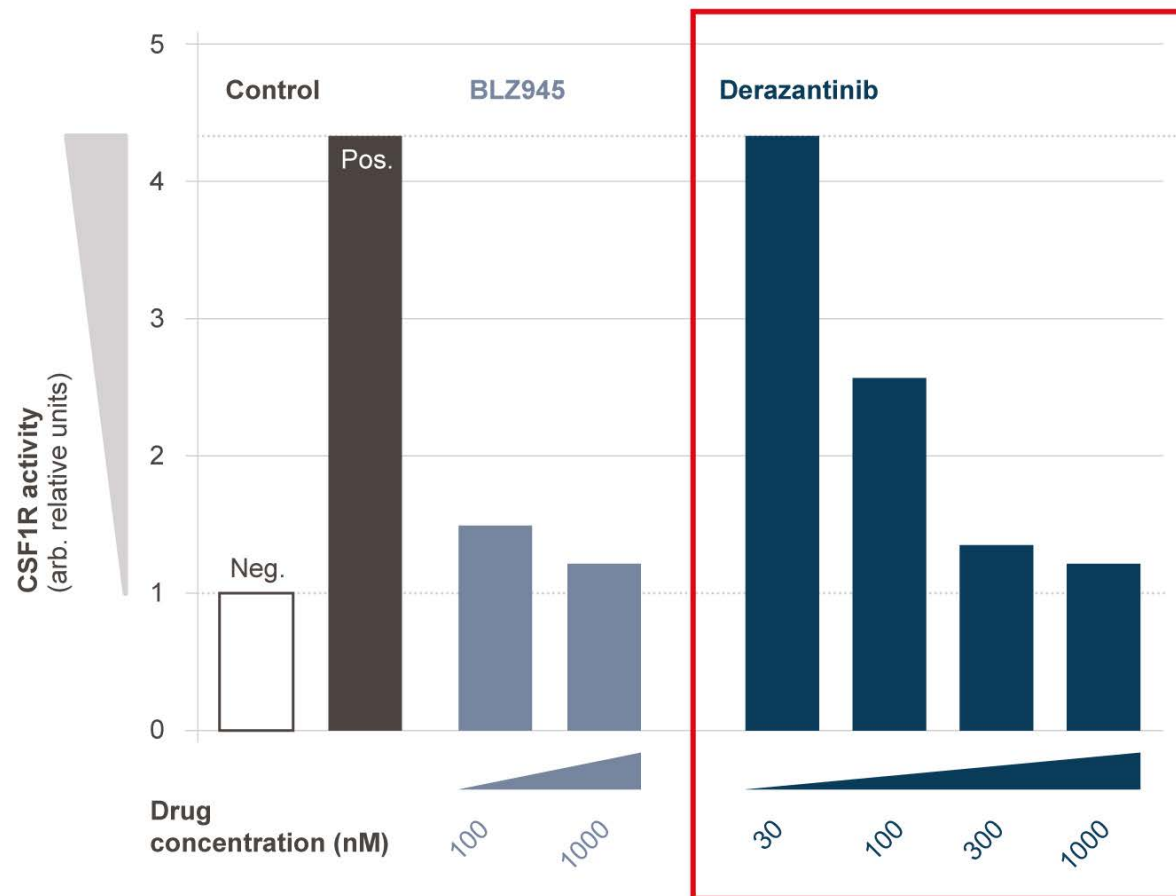


Graph adapted from: A. Ghasemzadeh et al. New Strategies in Bladder Cancer: A Second Coming for Immunotherapy. *Clin Cancer Res*. 2016;22(4):793-801

# 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

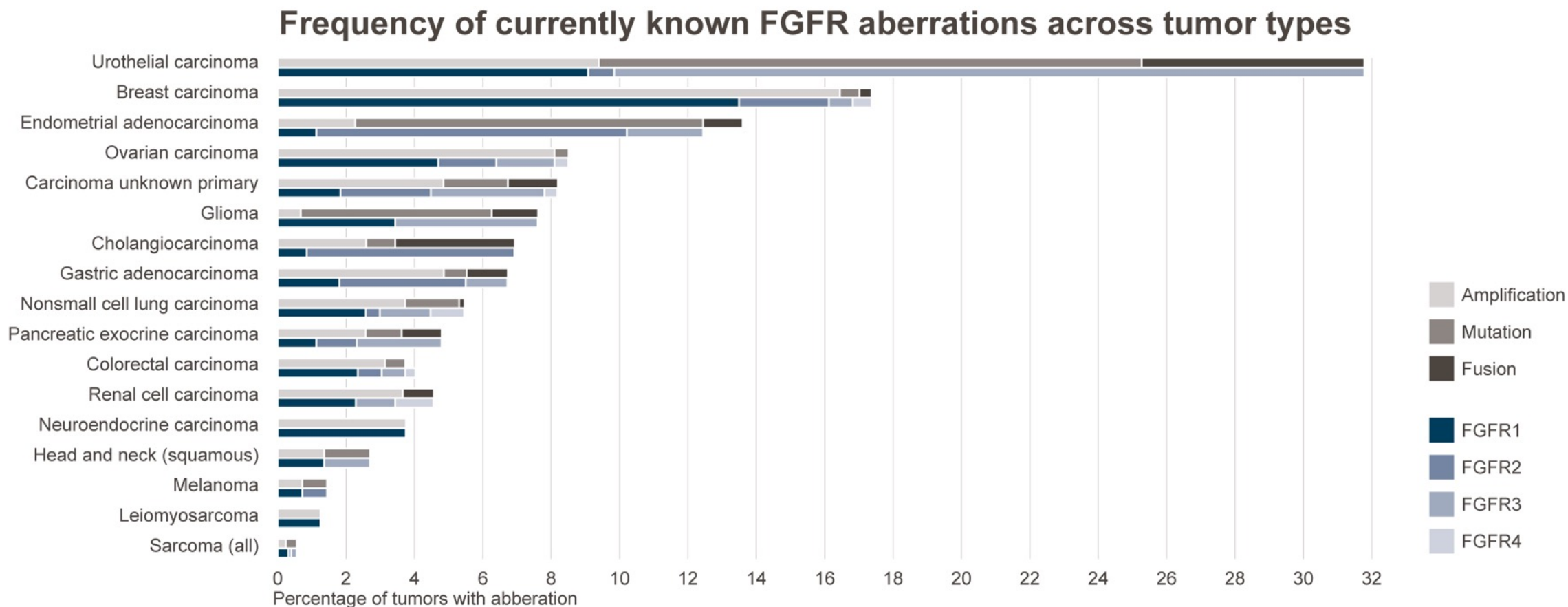
## Inhibition of CSF1R activity



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

<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

# Derazantinib — Significant potential beyond iCCA



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



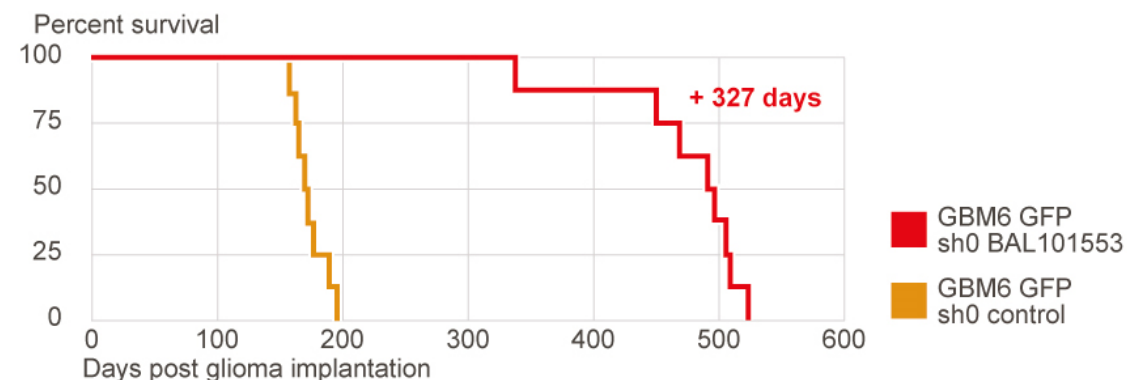
# EB1 — A potential response-predictive clinical biomarker for lisavanbulin

- 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>

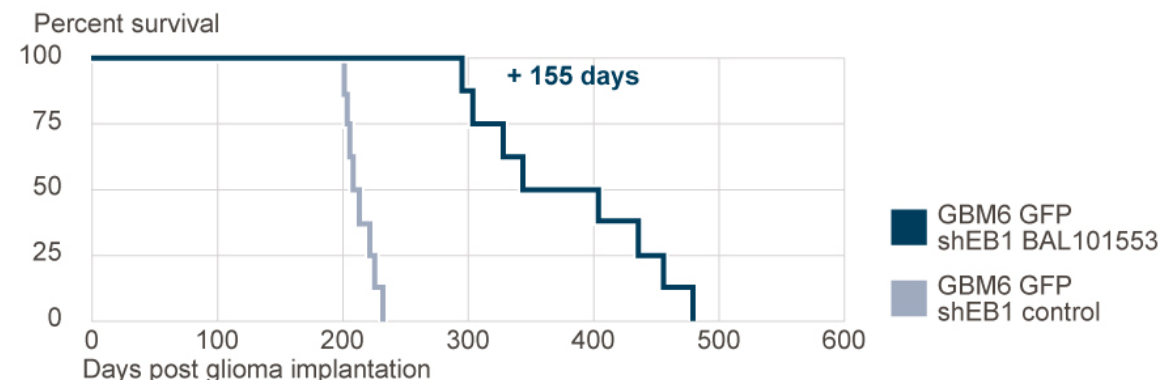
<sup>1</sup> Berges et al. EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after oral treatment with the novel tubulin-binding checkpoint activator BAL101553. Eur. J. Cancer 2018, 103, E61-62, A166

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

### EB1-expressing GBM



### EB1-downregulated GBM



# Glossary

- ABSSSI: **A**cute **b**acterial **s**kin and **s**kin **s**tructure **i**nfections
- CSF1R: **C**olony-**s**timulating **f**actor **1** receptor
- EAP: **E**xpanded **a**ccess **p**rogram
- FGFR: **F**ibroblast **g**rowth **f**actor receptor
- FIDES: **F**ibroblast growth factor **i**nhibition with **d**erazantinib in **s**olid tumors
- iCCA: **I**ntrahepatic **c**holangi**c**arcinoma
- IND: **I**nvestigational **n**ew **d**rug
- MSSA: **M**ethicillin-**s**usceptible **S**taphylococcus **a**ureus
- MRSA: **M**ethicillin-**r**esistant **S**taphylococcus **a**ureus
- NDA: **N**ew **d**rug application
- 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 receptor **2**

# 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 lisavanbulin and their 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.



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