



**Focused on  
Growth and Innovation**

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

Investor presentation

August 17, 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



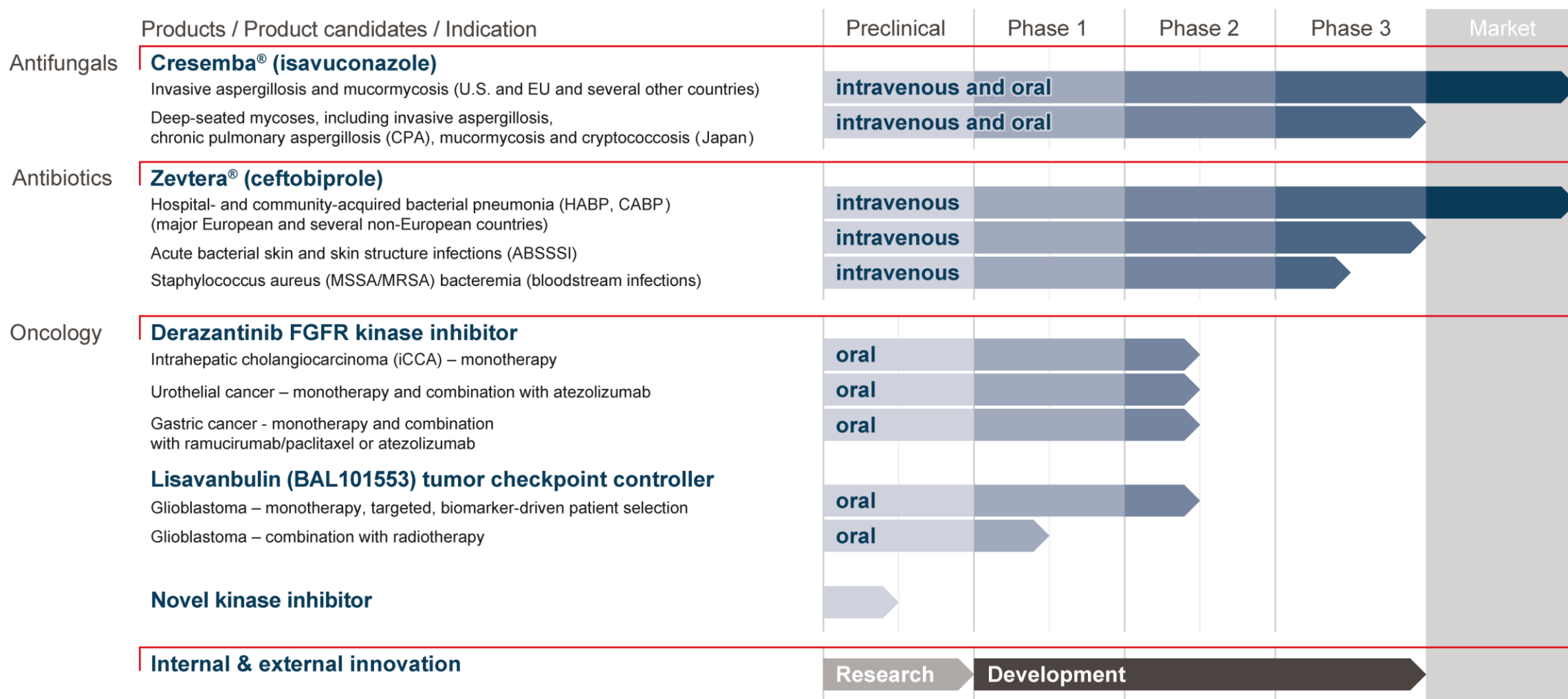
# At a glance

- 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

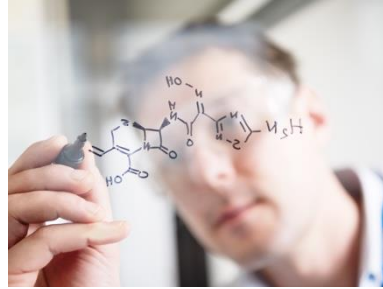


# Our strategy



## 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

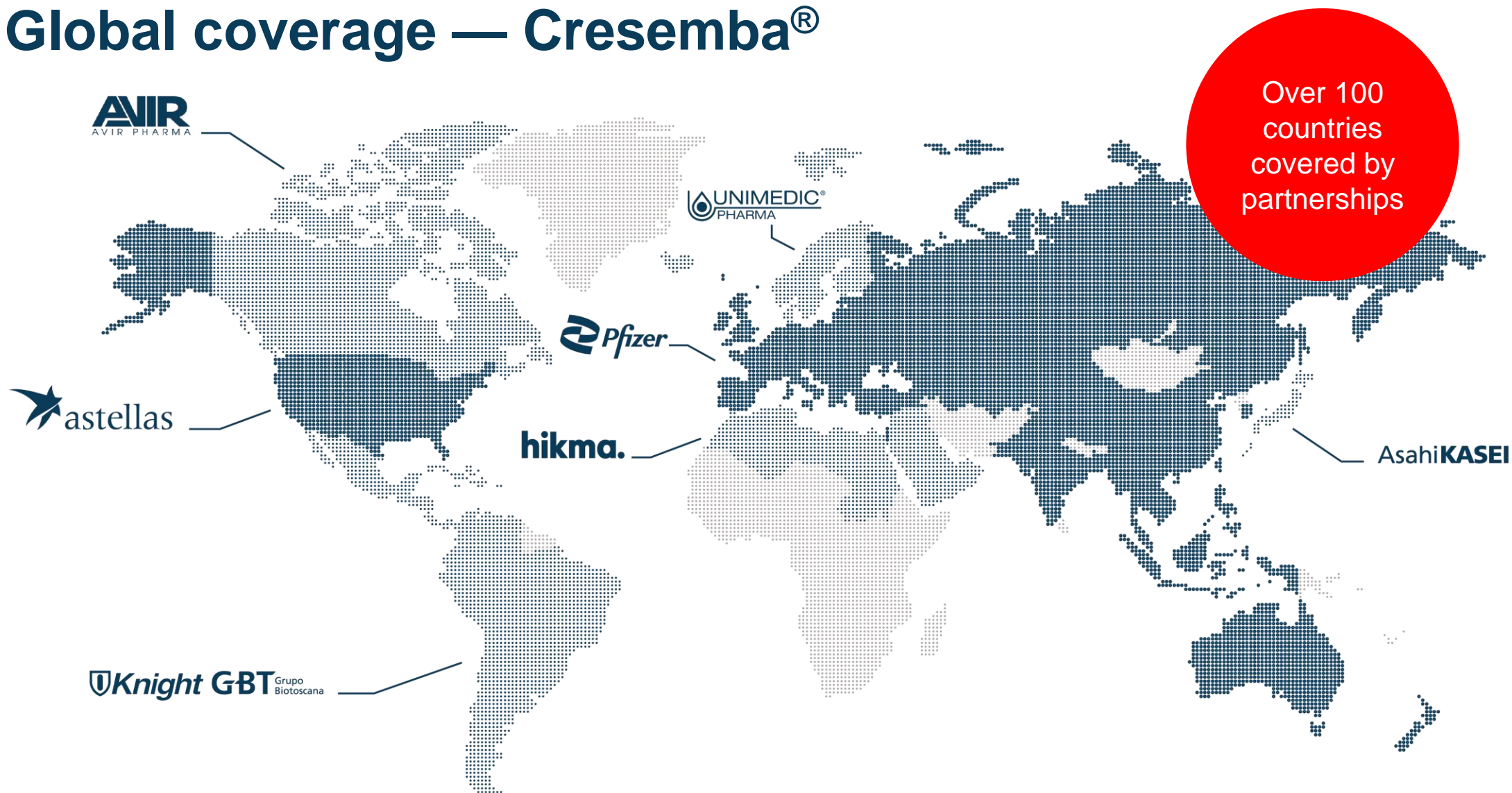
Invest in our clinical portfolio of targeted, small molecule, oncology drug candidates and the phase 3 ceftobiprole program



## 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)**

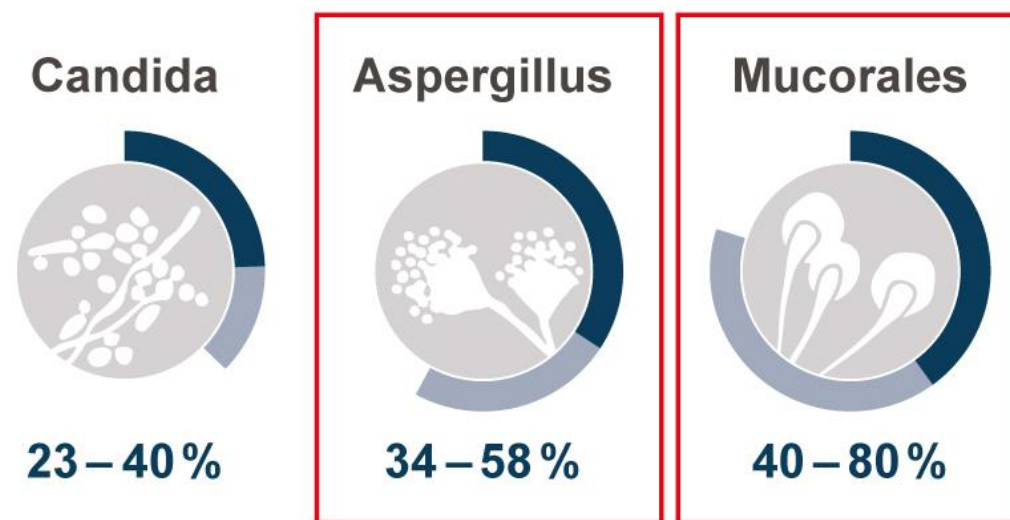
Invasive mold infections



# The market — Invasive fungal infections

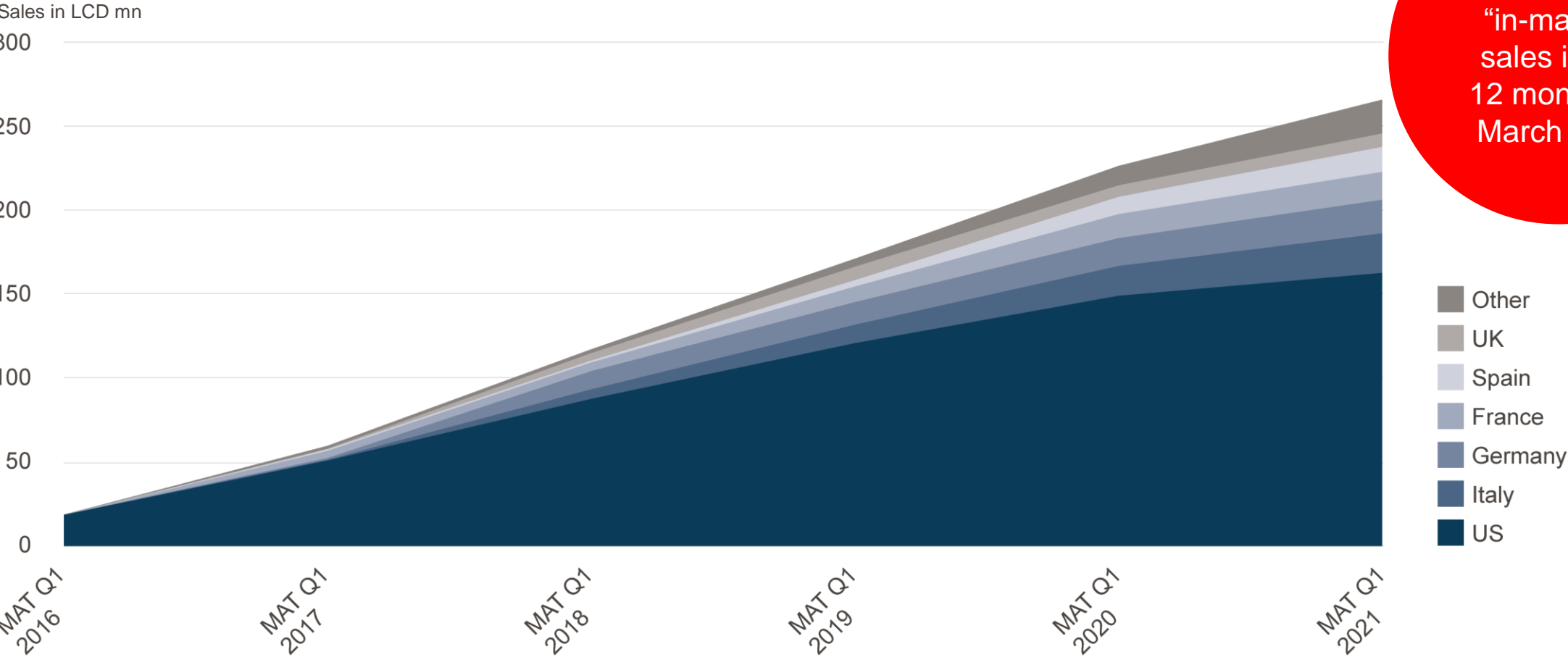
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand
- 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 266 mn  
“in-market”  
sales in the  
12 months to  
March 2021

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

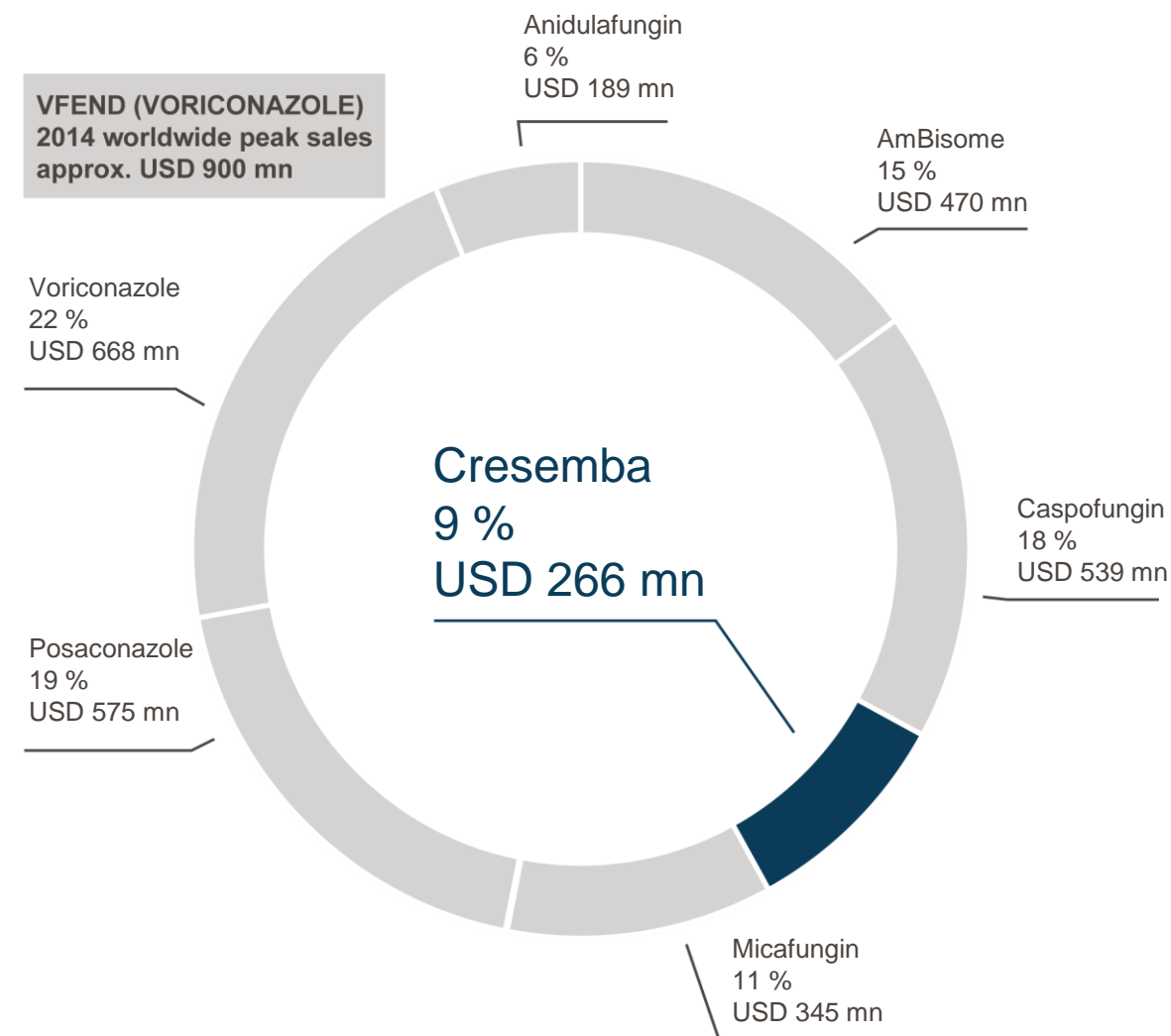


# Sales of best-in-class antifungals\* by product

USD 3.1 bn sales (MAT Q1 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; Sales figures in USD, corrected for currency fluctuations;  
Source: IQVIA, March 2021

# 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

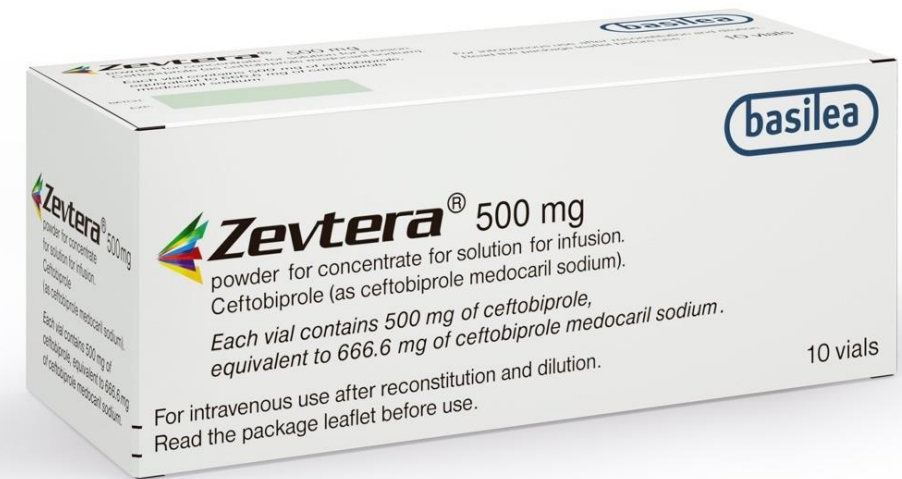
- Broad-spectrum anti-MRSA cephalosporin (including Gram-negative bacteria)
- Rapid bactericidal activity
- Potential to replace antibiotic combinations
- Early improvement in HAP, particularly in patients with MRSA, and CAP, including high-risk patients
- Cephalosporin class safety profile
- Marketed 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

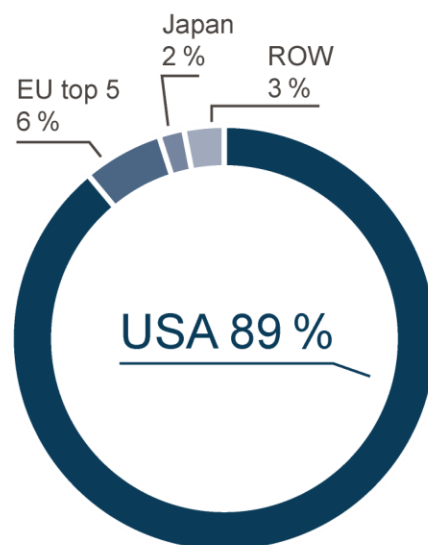


Focused on Growth and Innovation

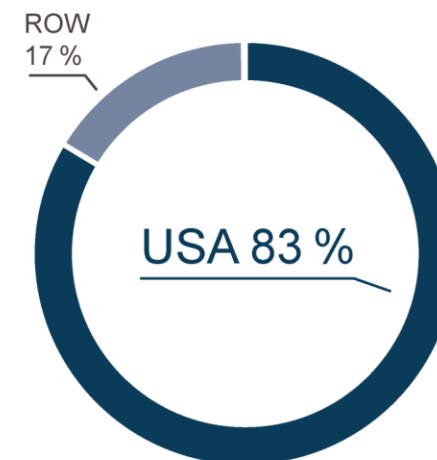


# The hospital anti-MRSA antibiotic market — A USD 2.8 bn market\* with the U.S. being the most important region

Daptomycin sales by region  
(2015, before LOE)



Ceftaroline sales by region  
(MAT Q1 2021)



\* 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 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

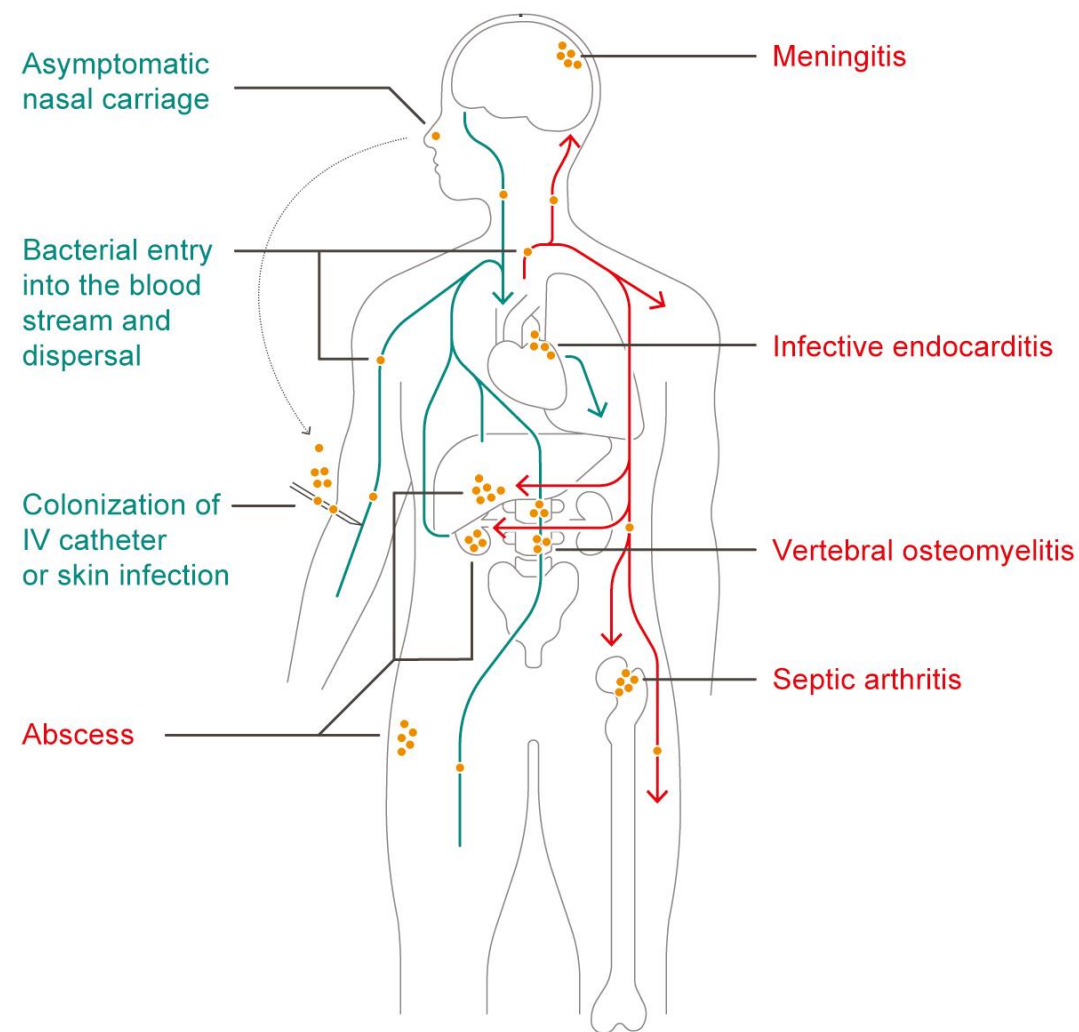
<sup>1</sup> MMWR, 2019;68:214–219.

<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.

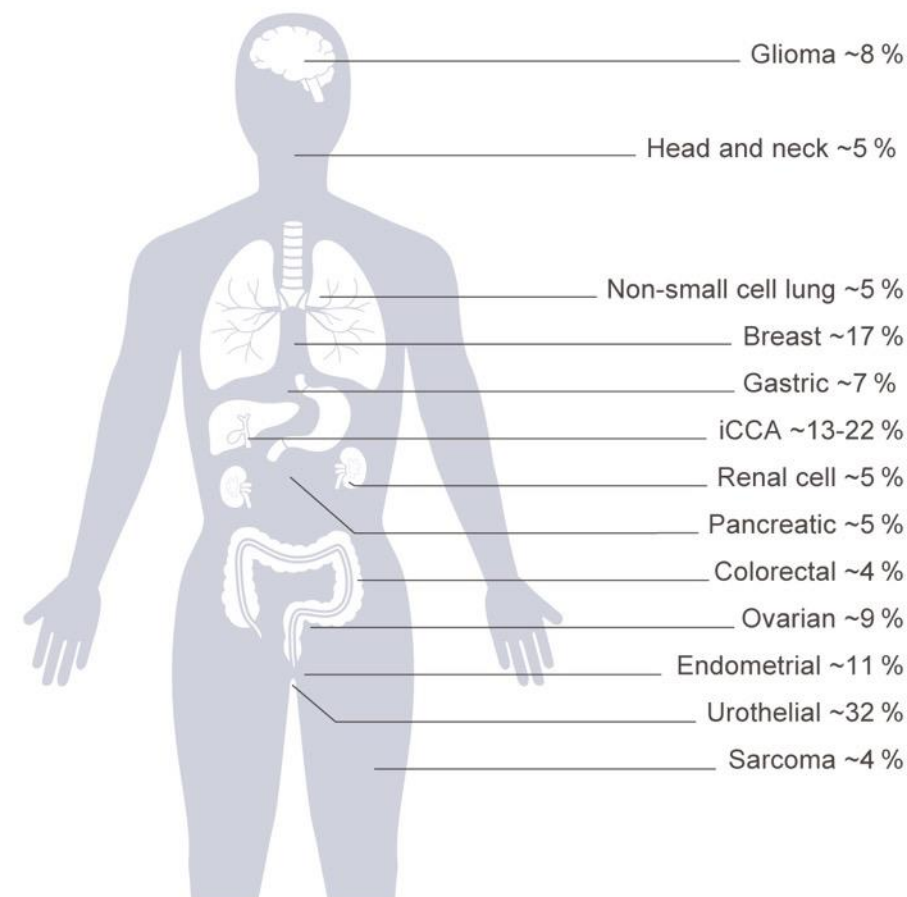
Oncology

# 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	75 %	84%	82%	83%
Median Progression-free survival	7.8 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. Basilea, data on file 2021. 2. Javle et al. J Clin Oncol 39, no. 3\_suppl (January 20, 2021) 265-265. 3. Abou-Alfa et al. J Clin Oncol 39, no. 15\_suppl (May 20, 2021) 4086-4086. 4. Goyal et al. Cancer Res 2021; 81, 13 Supplement, pp. CT010. 5. Droz Dit Busset et al., Annals of Oncology (2020) 31 (suppl\_5): S1217-S1239. (Pooled analysis of clinical trials and early access programs). 6 Abou-Alfa et al. Lancet Oncol 2020;21(5):671-684.

# Clinical program in urothelial cancer – FIDES-02

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

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

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

# 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



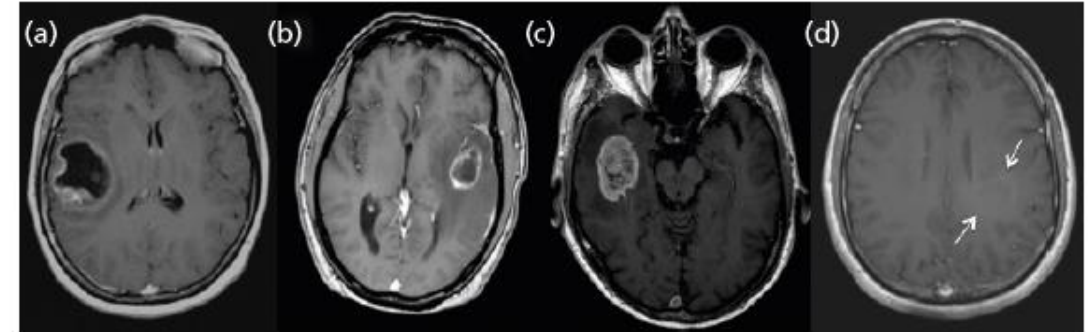
# Unmet medical need in glioblastoma

- 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

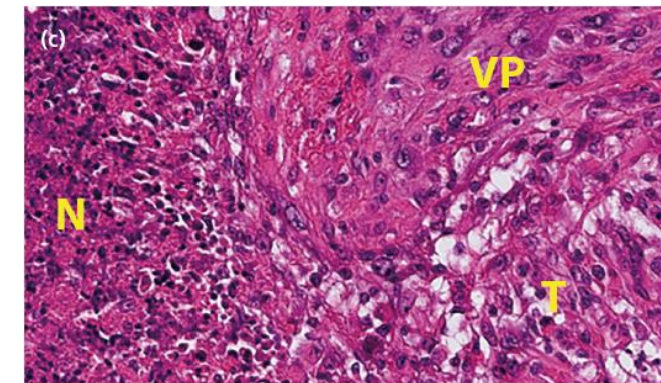
<sup>1</sup>Poon MTC et al. 2020; Sci Rep 10, 11622; <sup>2</sup>Hegi et al. NEJM 2005;352:997-1003

<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.

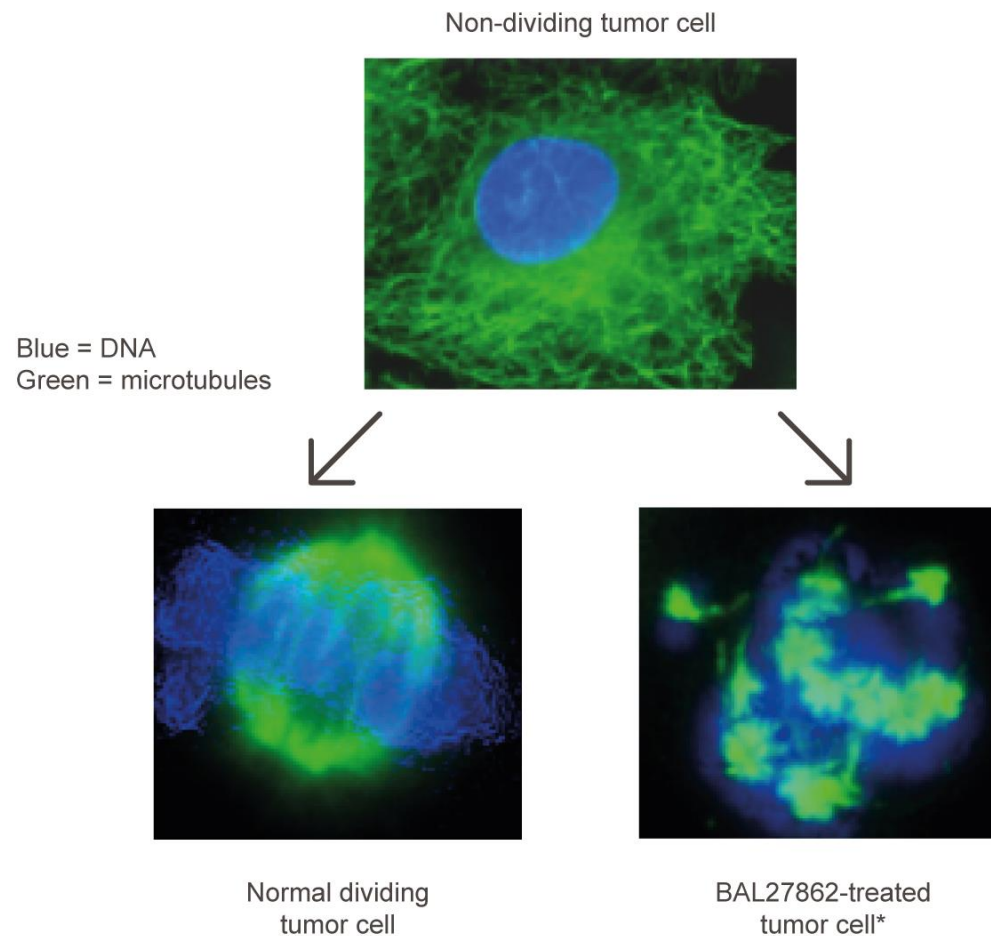
100 μm

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

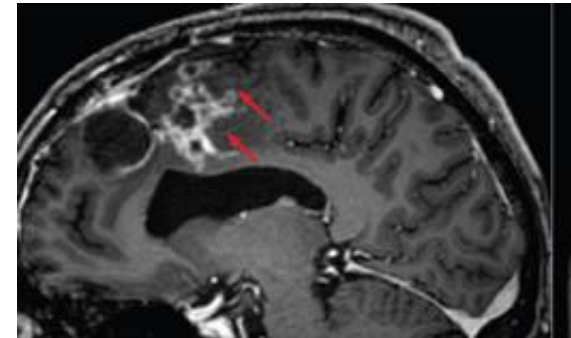


\* Lisavanbulin (BAL101553) is a prodrug of BAL27862

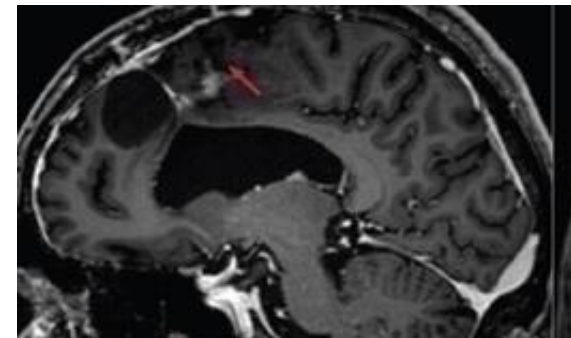
# Biomarker-driven phase 2 study ongoing in recurrent glioblastoma

- 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

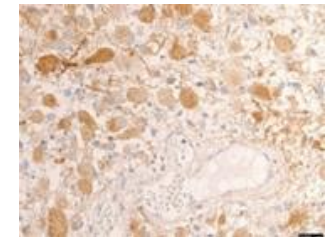
## Glioblastoma tumor size reduction in an exceptional responder and EB1 staining of glioblastoma tissue compared to non-responding patients



Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



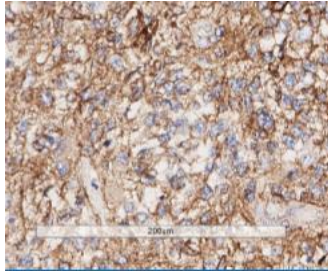
Non-responder

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

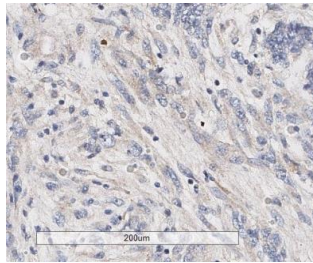
<sup>2</sup> Tiu et al. JCO 2021;39,15 suppl, TPS2068 (NCT02490800)

# EB1-prevalence in glioblastoma and other cancer types

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

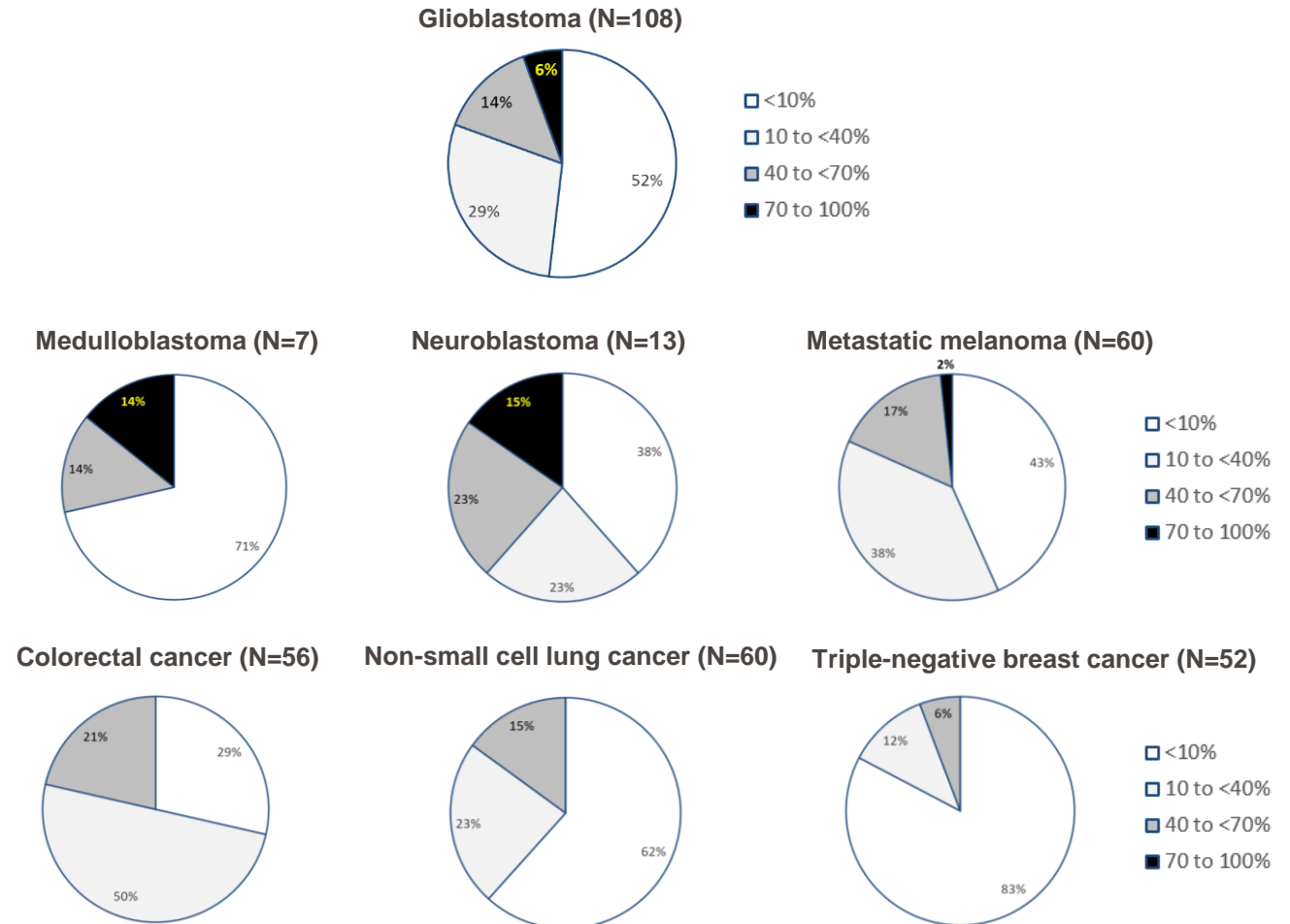


**EB1-positive:**  
Tumor cells show moderate to strong EB1 staining



**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

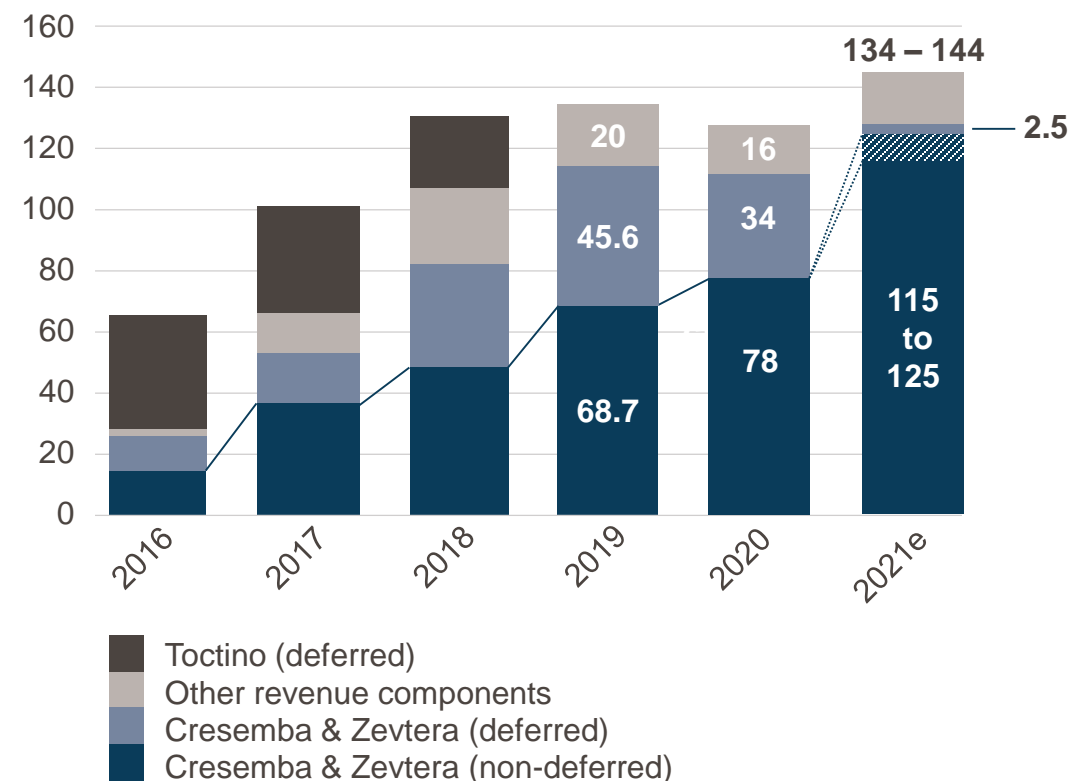




# 2021 financial guidance - increased revenue and improved operating result

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

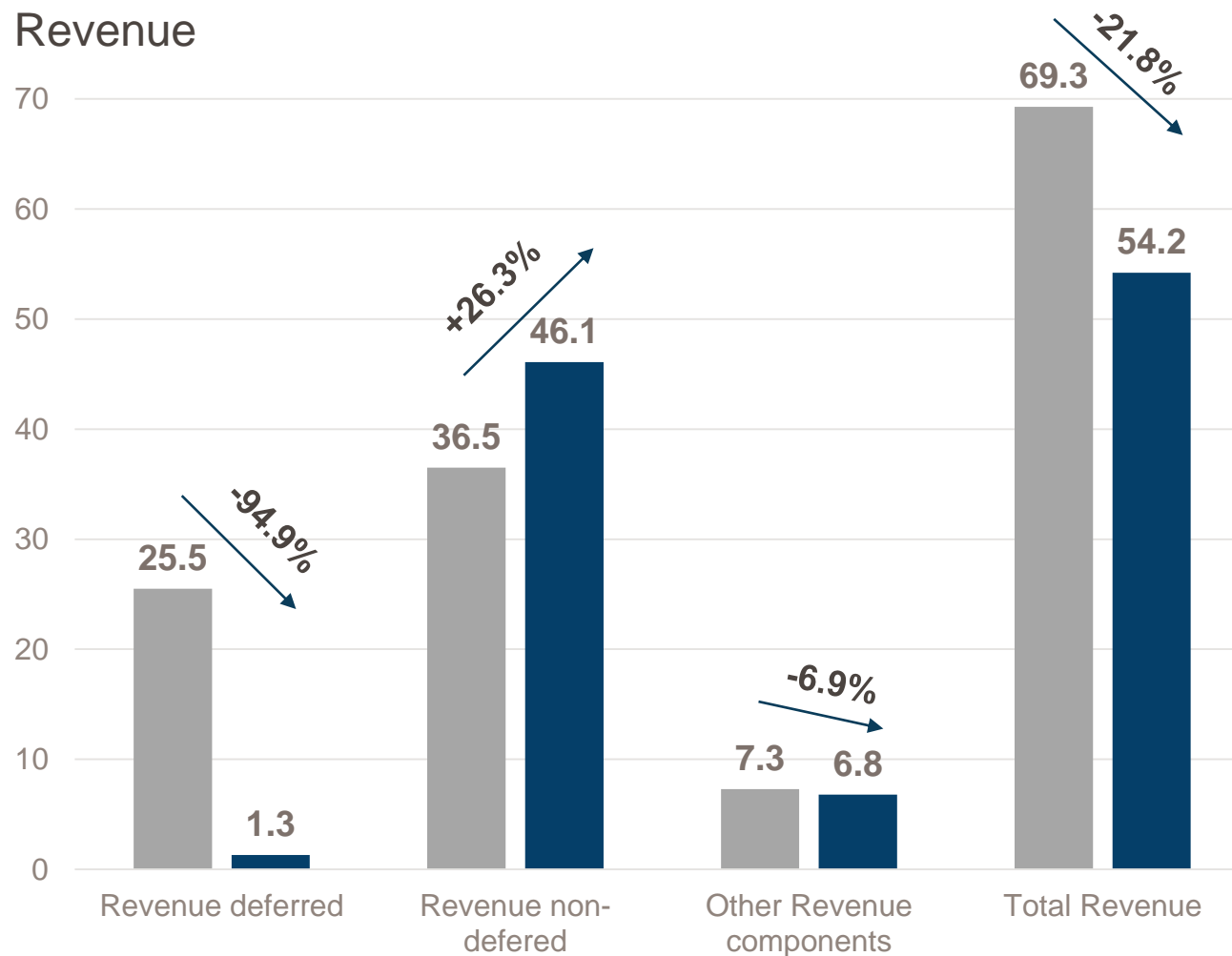
**Cresemba® & Zevtera® — Increasing cash flows**  
**By the end of 2022, Cresemba to be on the market in ~ 70 countries**

		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	

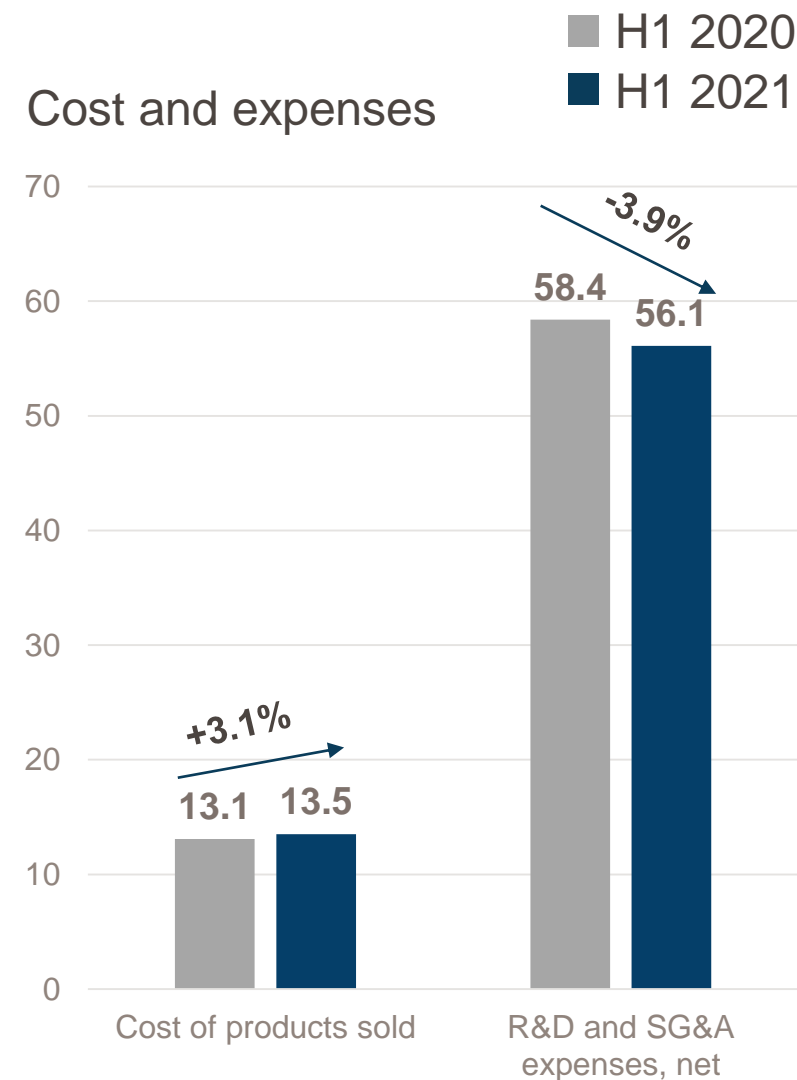
# Appendix

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

## Revenue



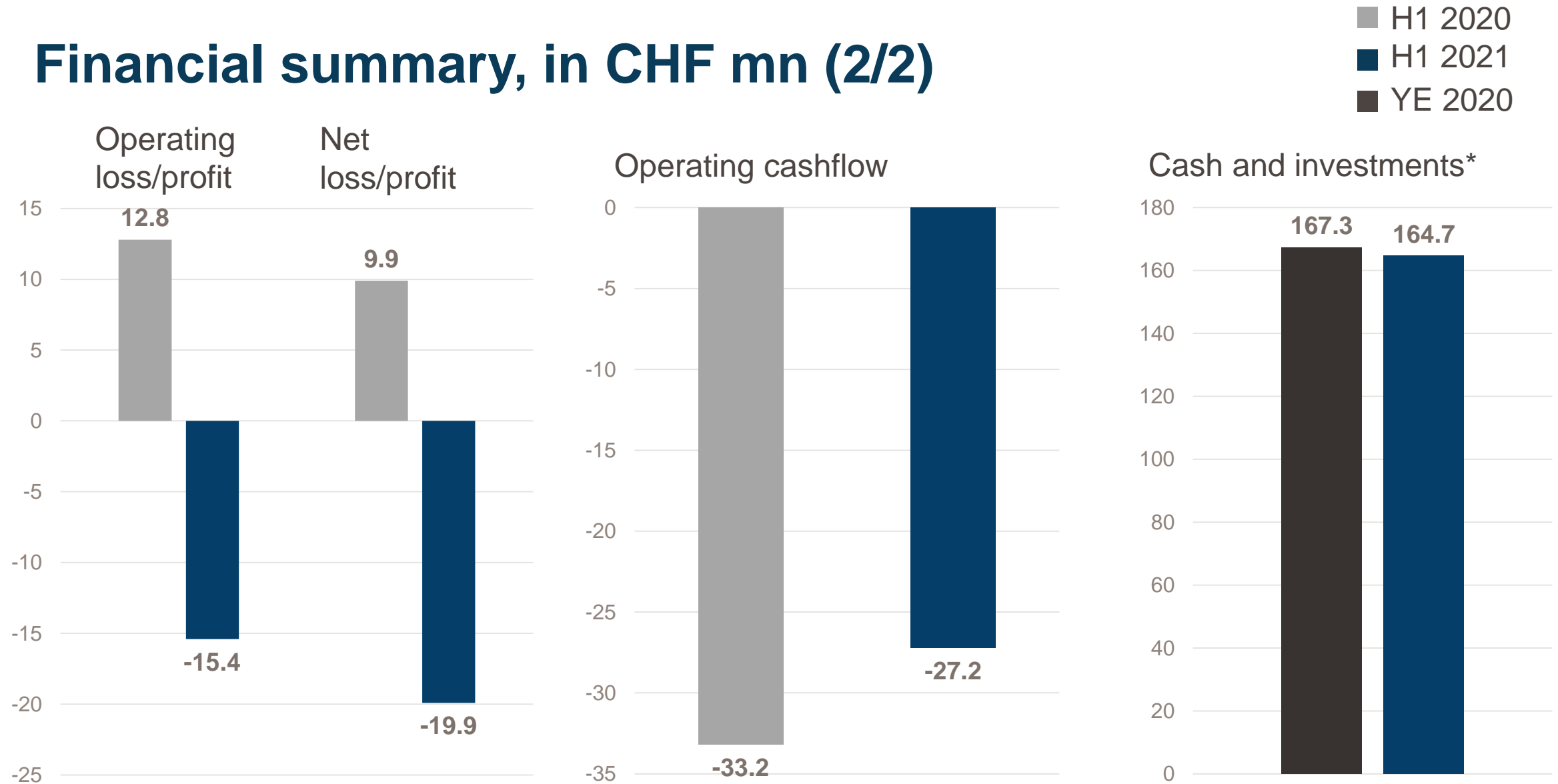
## Cost and expenses



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

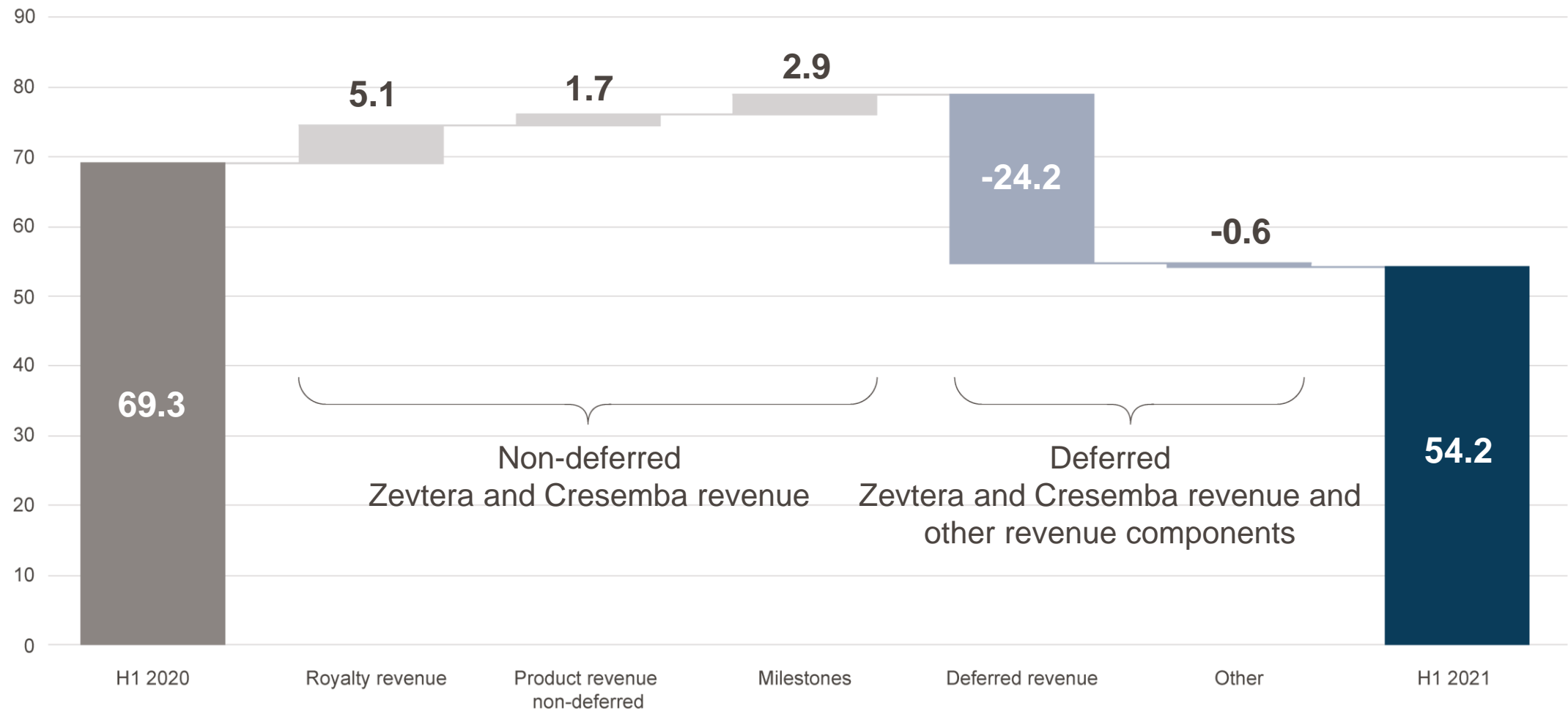


# Financial summary, in CHF mn (2/2)



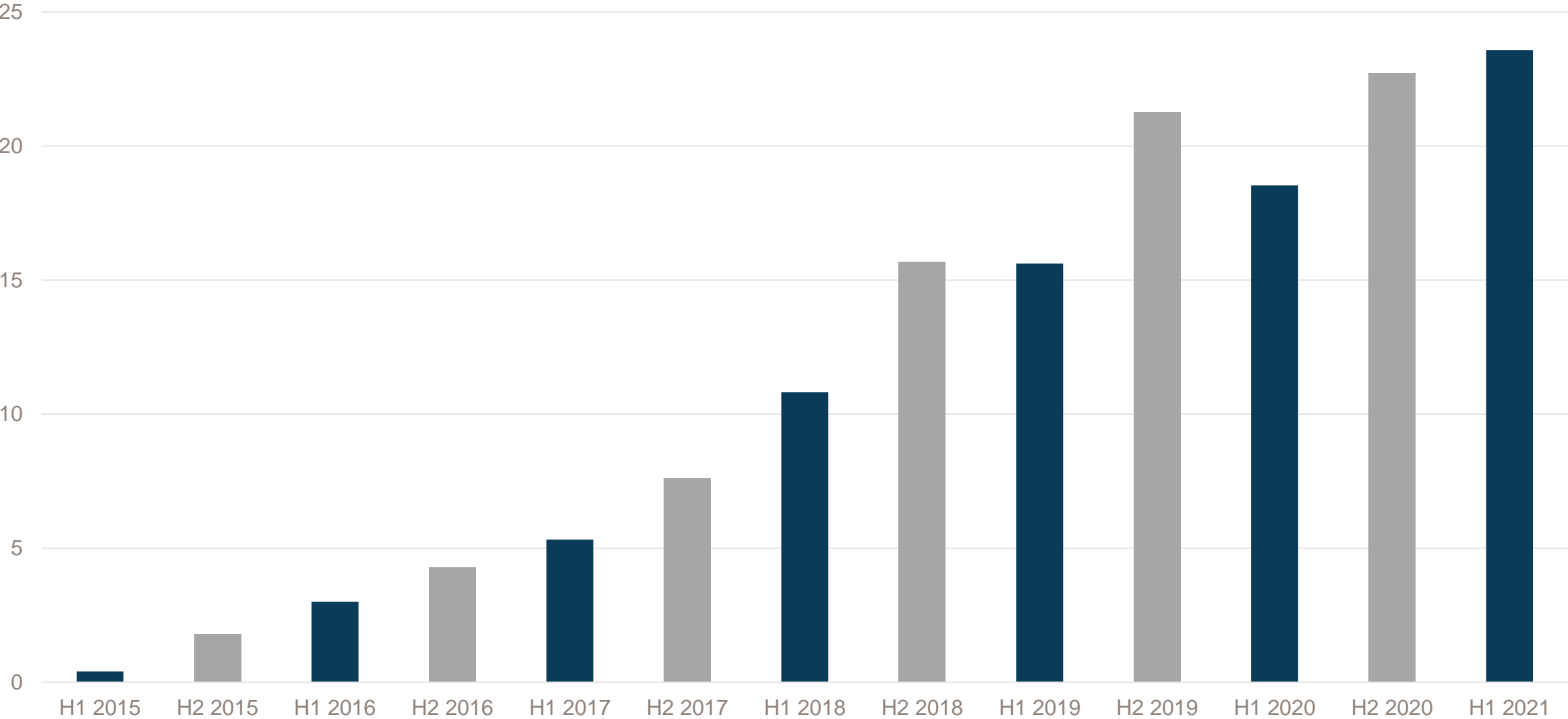
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently, \*Cash, cash equivalents, restricted cash and investments

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



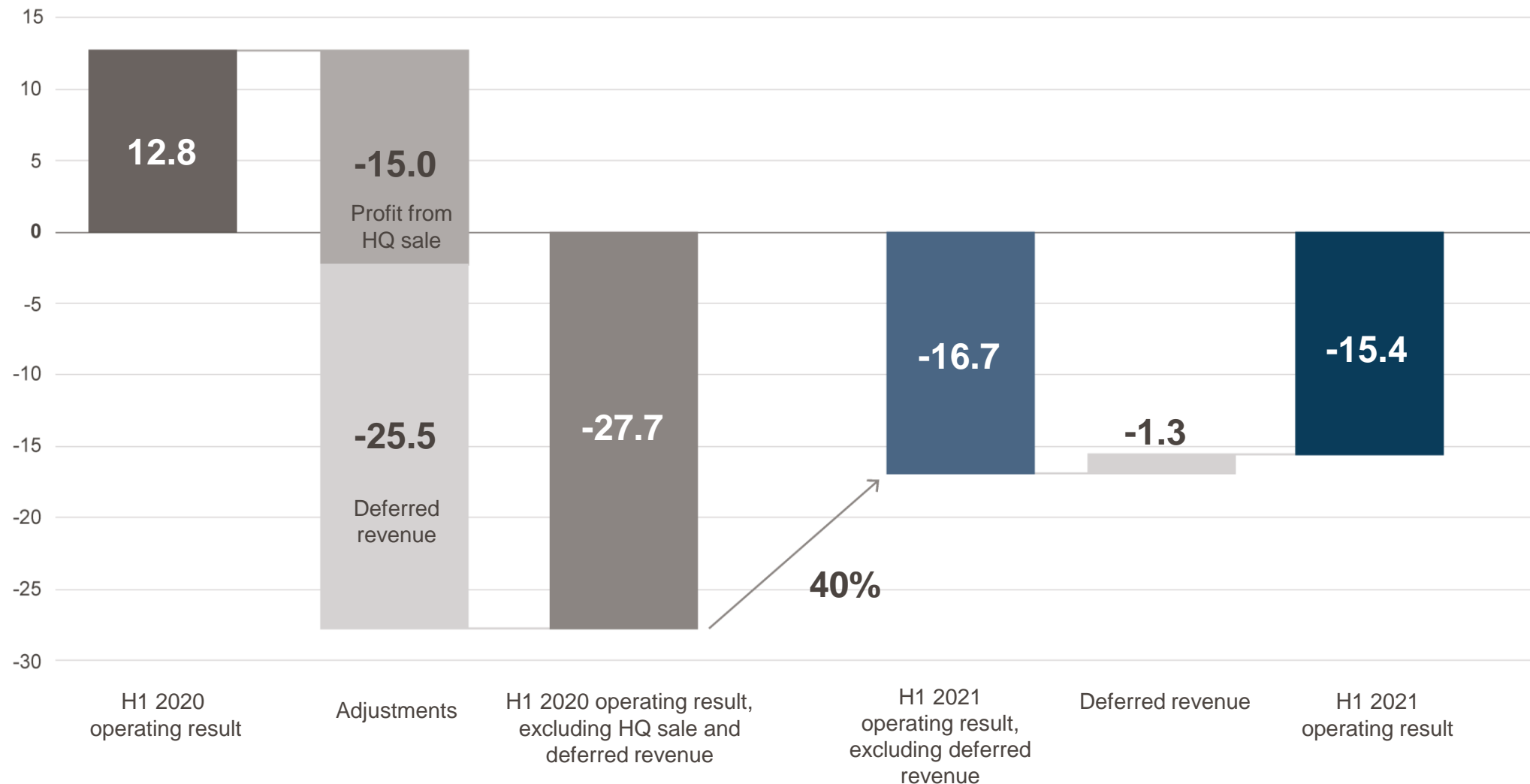
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

# 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

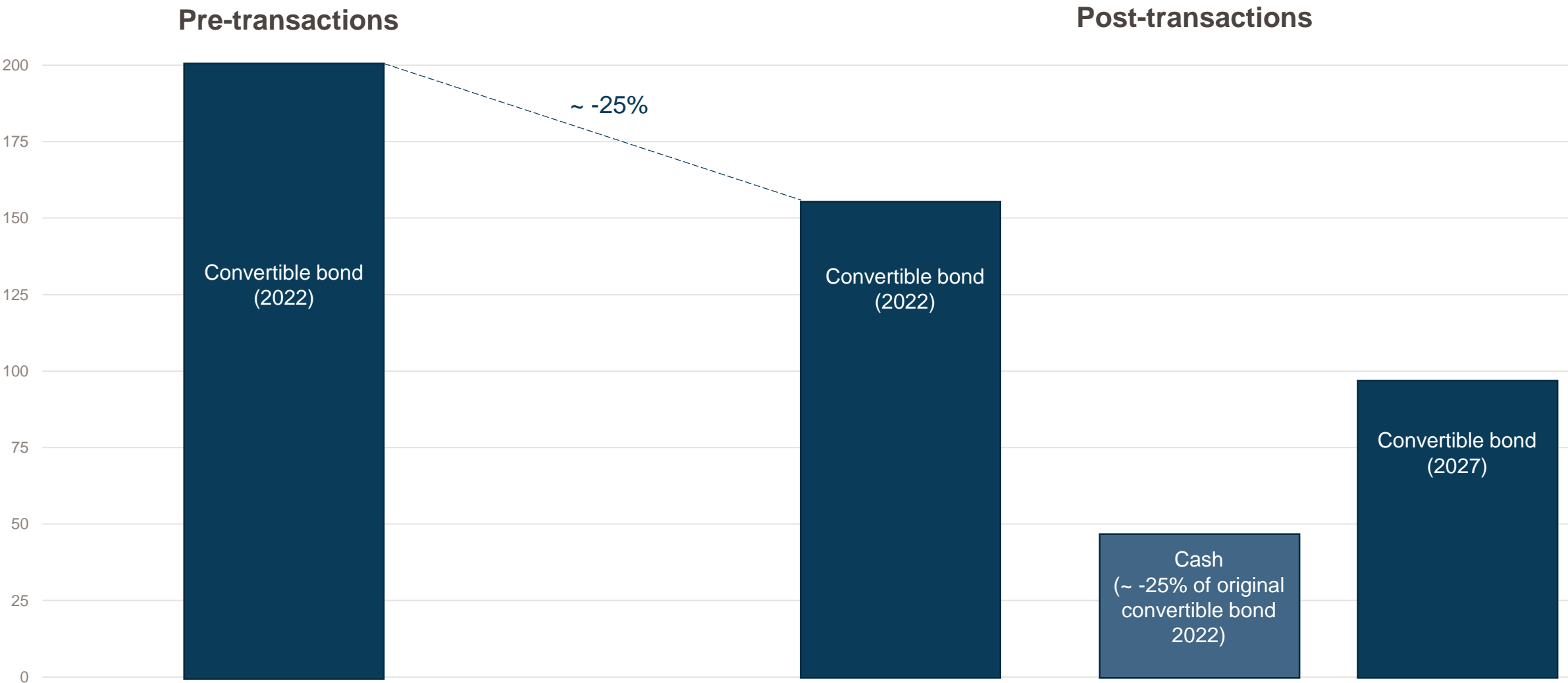
# Significant improvement in underlying operating performance (CHF mn)



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



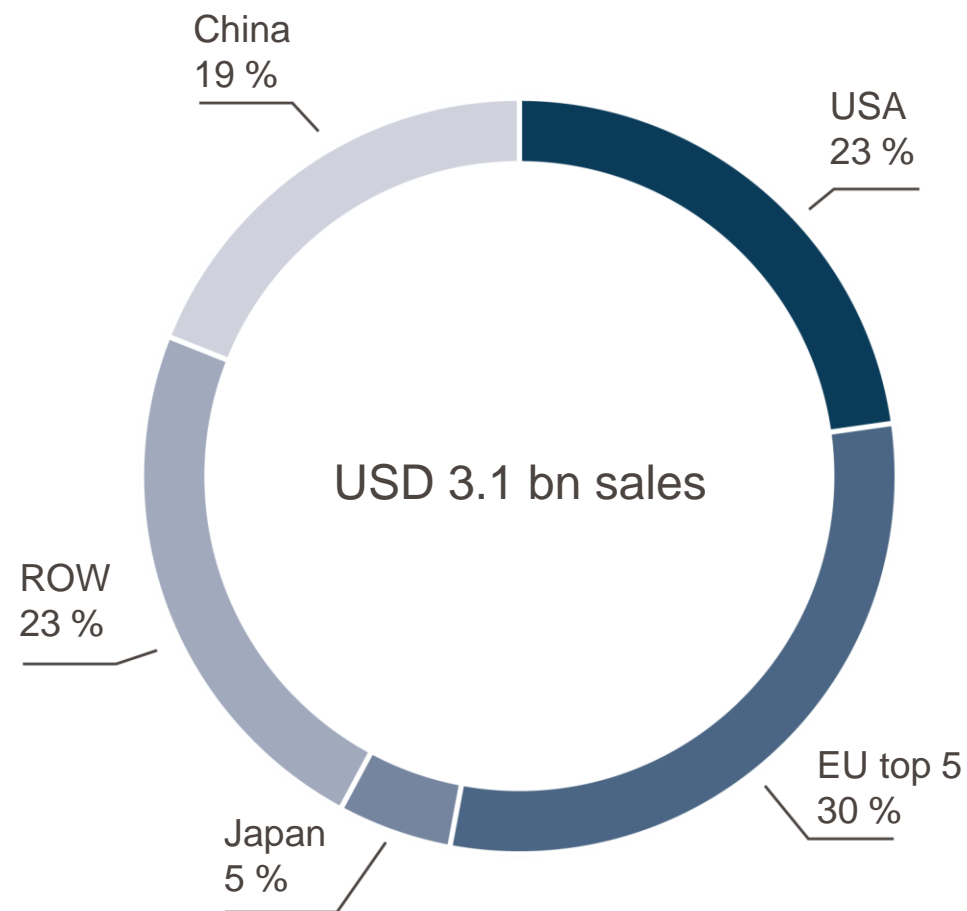
# Convertible bond transactions — successfully improved debt maturity profile (in CHF mn)



# 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 Q1 2021)

\* 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, March 2021

# Ceftobiprole — Positive topline phase 3 results reported in ABSSSI

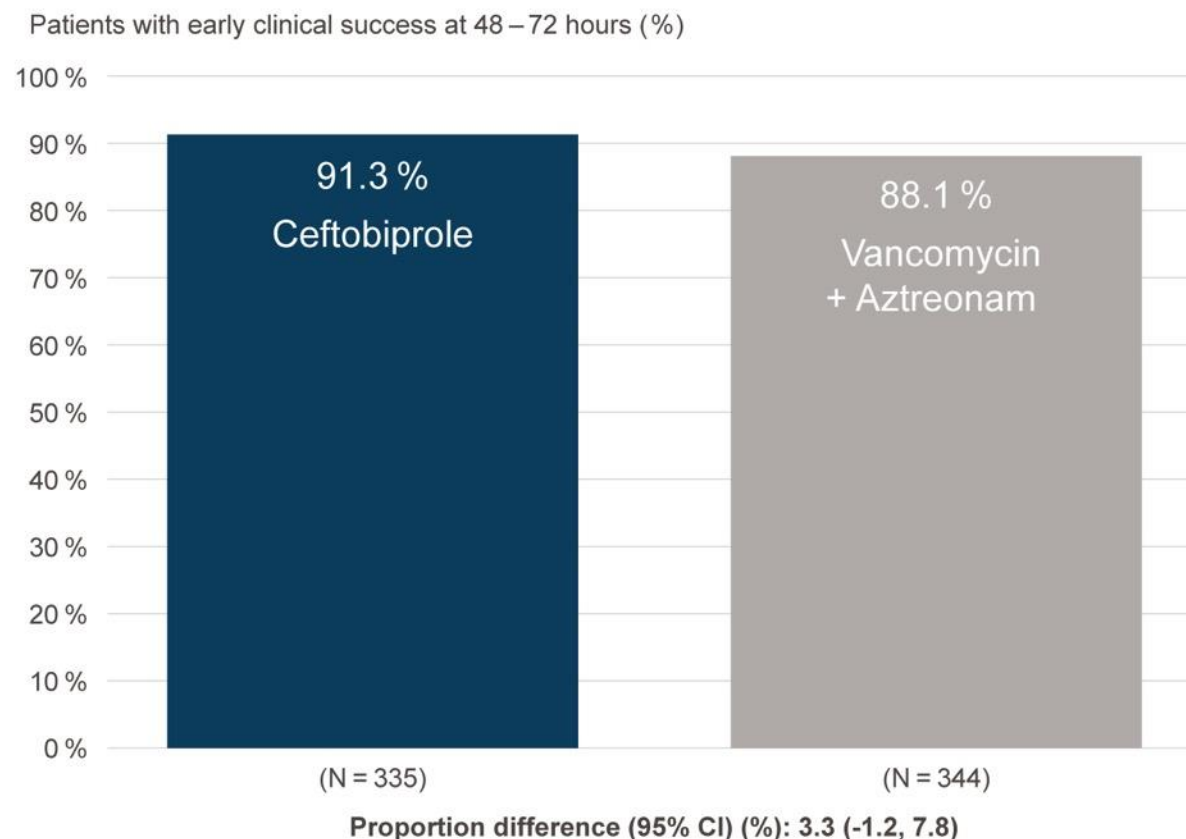
Key topline study<sup>1</sup> results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints



<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

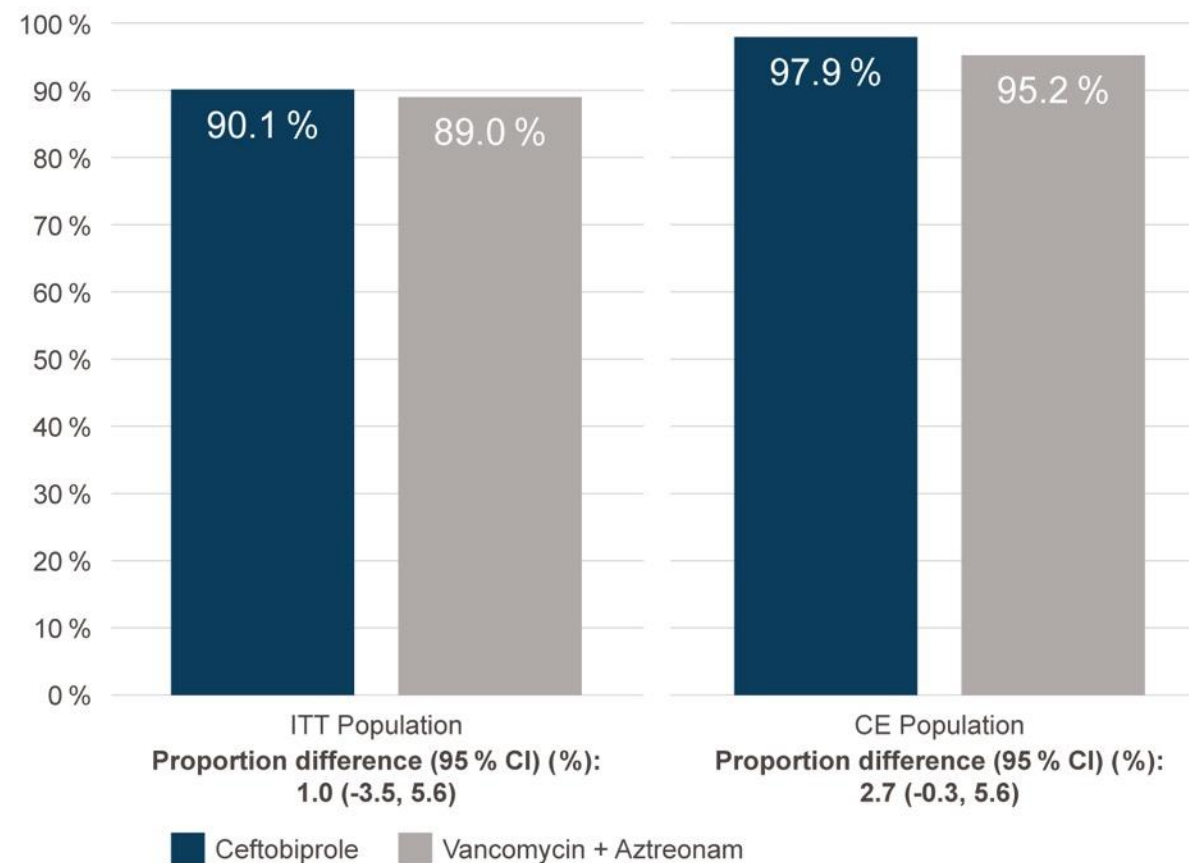


<sup>1</sup> NCT03137173

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>

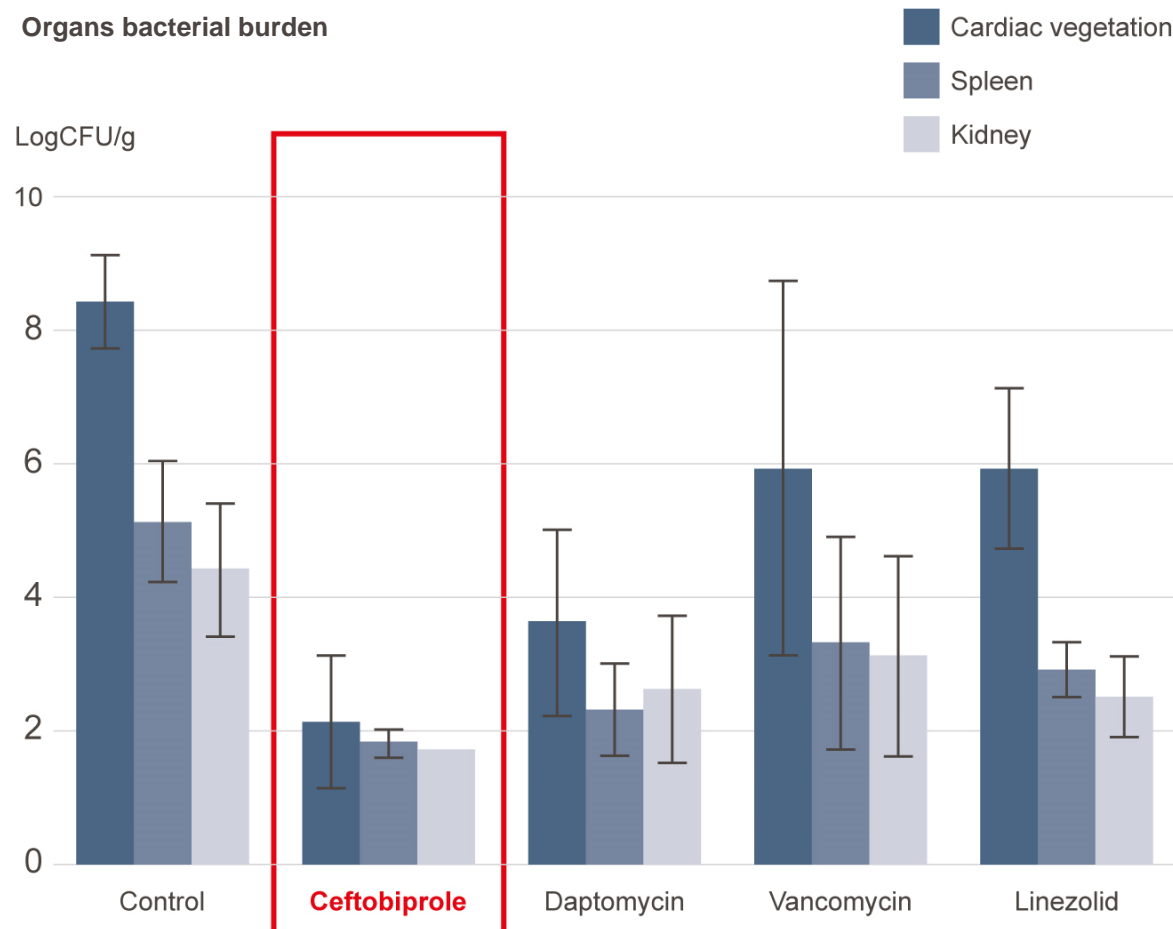
<sup>1</sup>Syed YY. Drugs. 2014;74:1523-1542.

<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 medocartil 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>

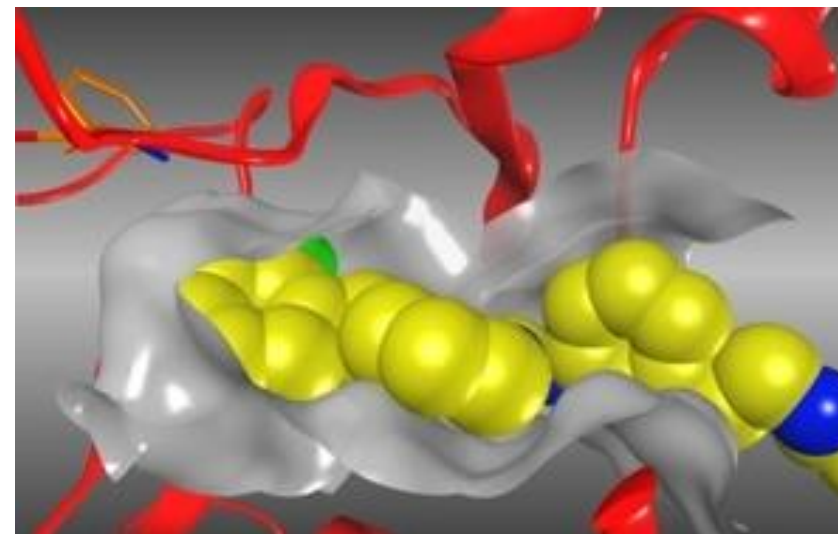
FGFR-inhibitor compound (Sponsor)	Parameter	FGFR1	FGFR2	FGFR3	FGFR4	CSF1R	VEGFR2
<b>Derazantinib</b> (Basilea)	Ratio to FGFR2 activity	4	1	4	77	3	6
<b>Pemigatinib</b> (Incyte)	Ratio to FGFR2 activity	3	1	4	39	231	62
<b>Erdafitinib</b> (Janssen)	Ratio to FGFR2 activity	2	1	2	13	95	6
<b>Rogaratinib</b>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

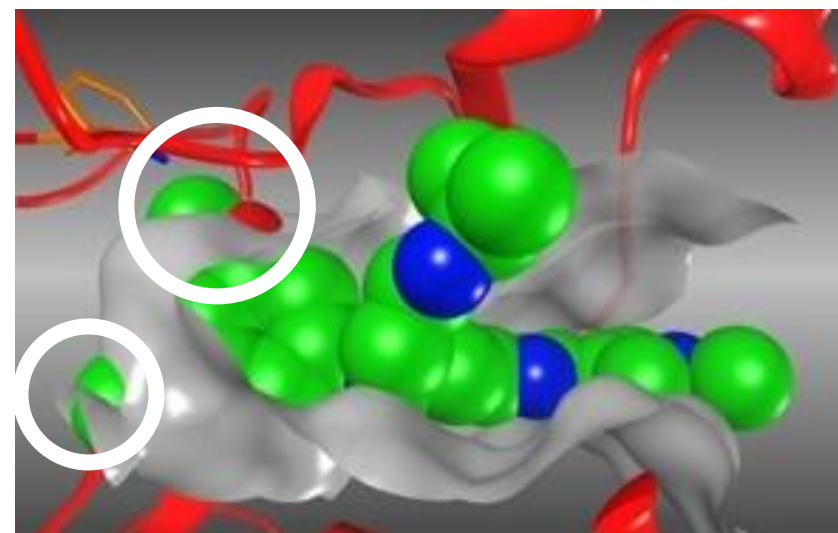


# In-silico analysis of derazantinib binding to CSF1R

- 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)



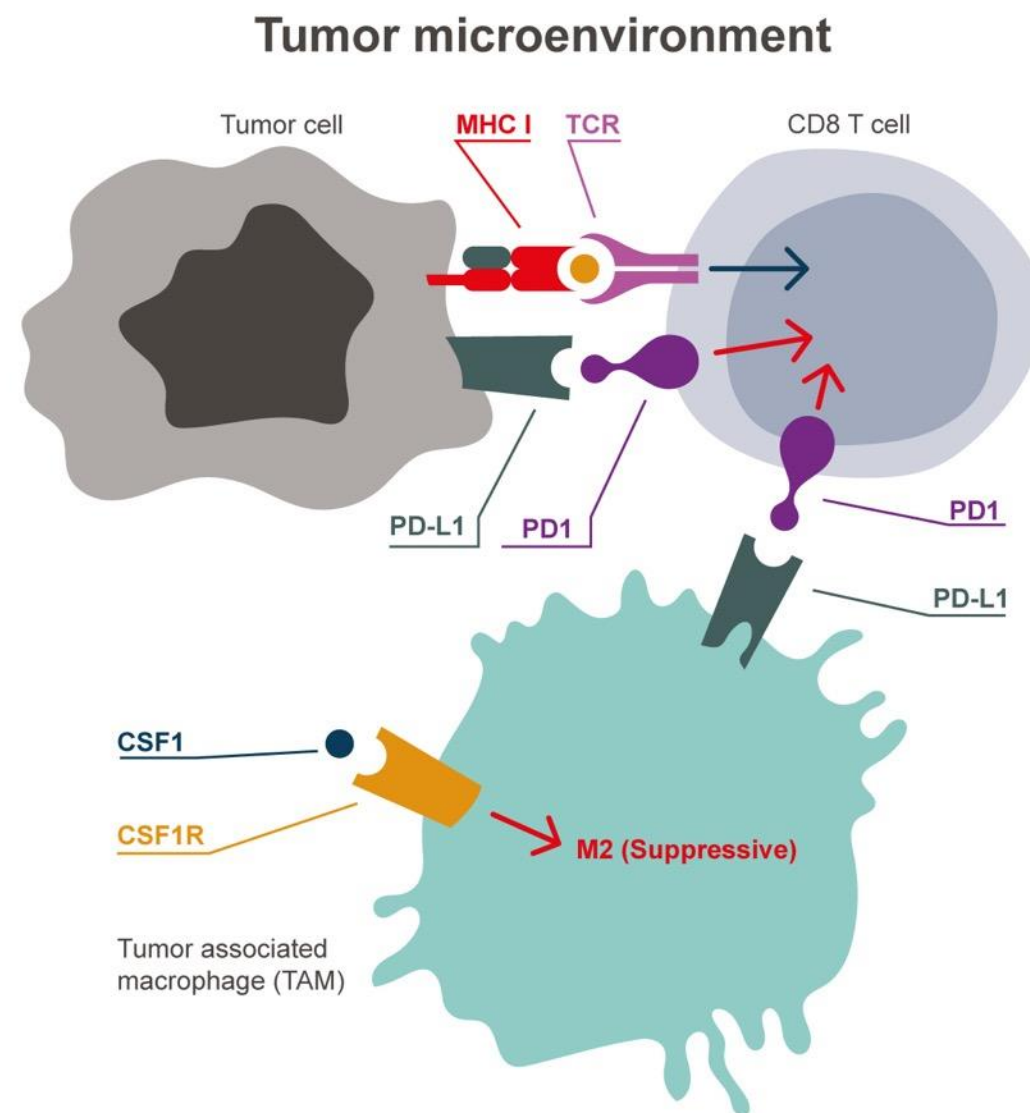
**Erdafitinib** (green) is too large (white circles) for the active site pocket of CSF1R (grey/red)

<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

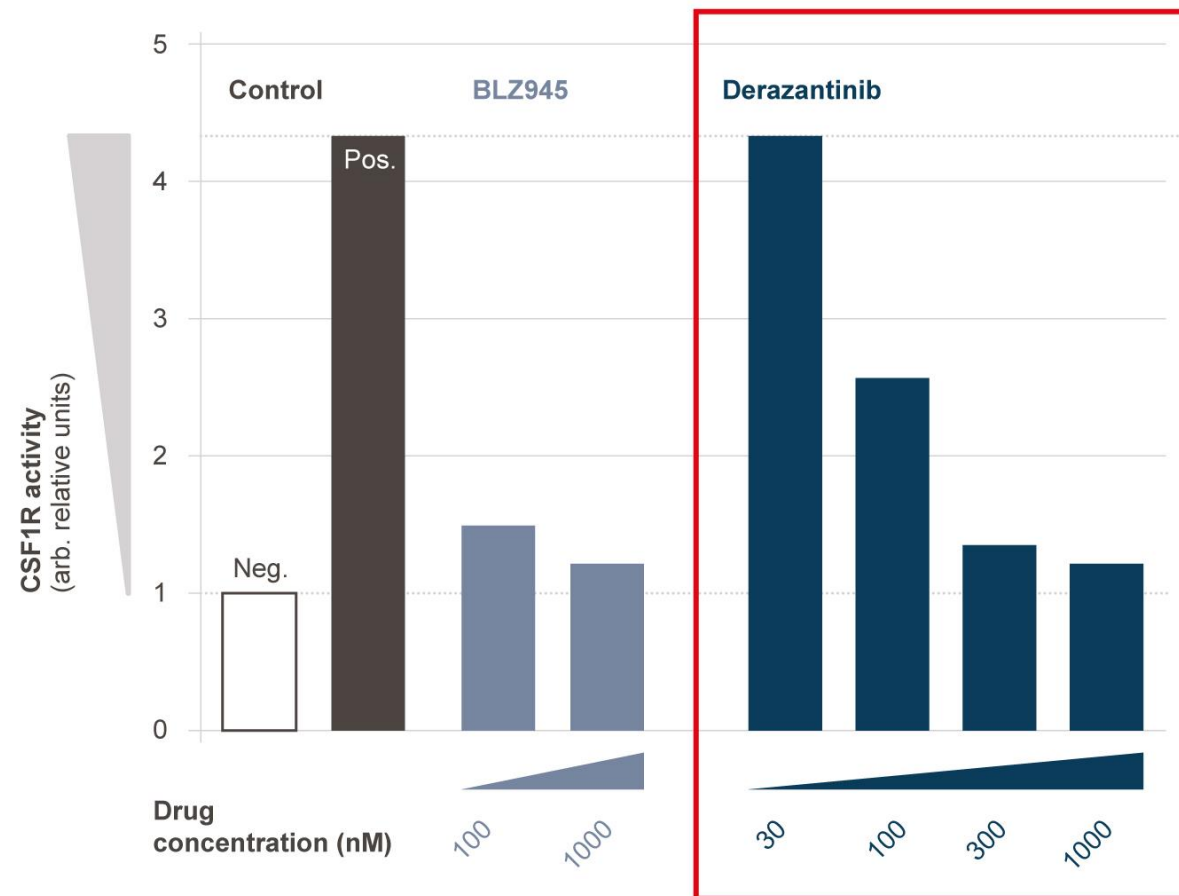


Graph adapted from: A. Ghasemzadeh et al. New Strategies in Bladder Cancer: A Second Coming for Immunotherapy. *Clin Cancer Res*. 2016;22(4):793-801

# 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

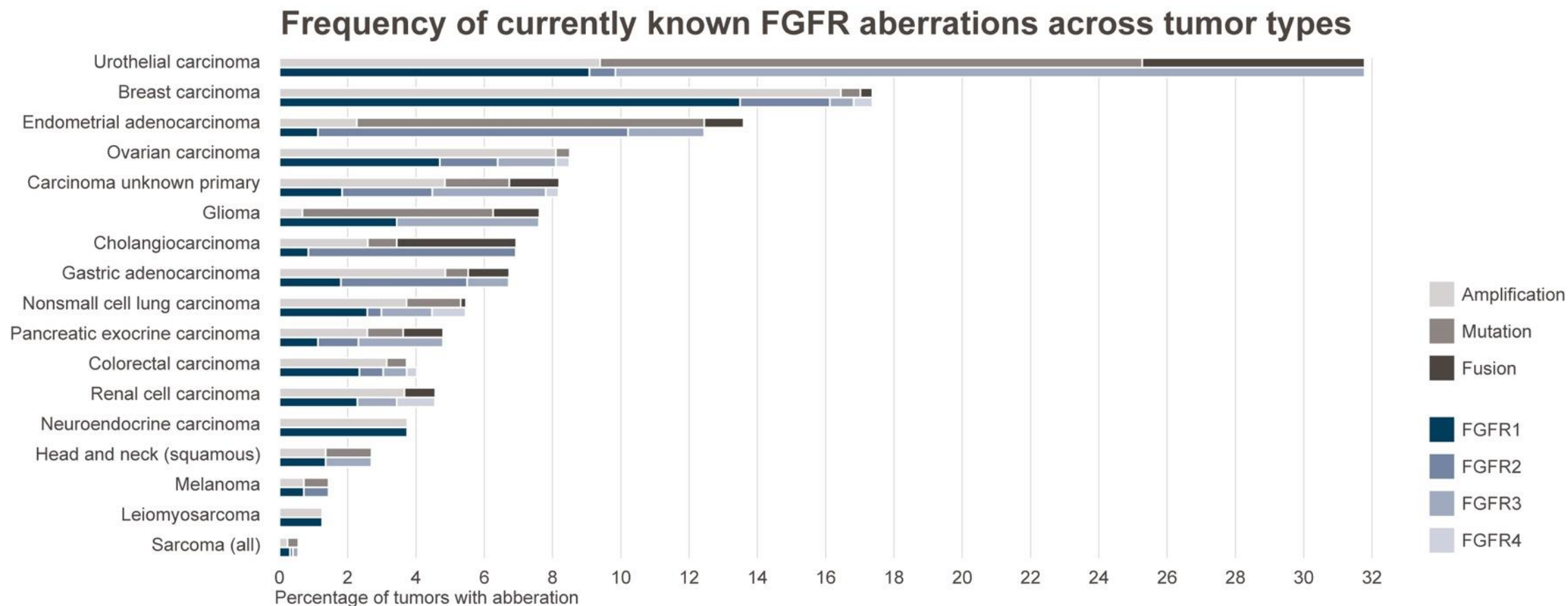
## Inhibition of CSF1R activity



*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



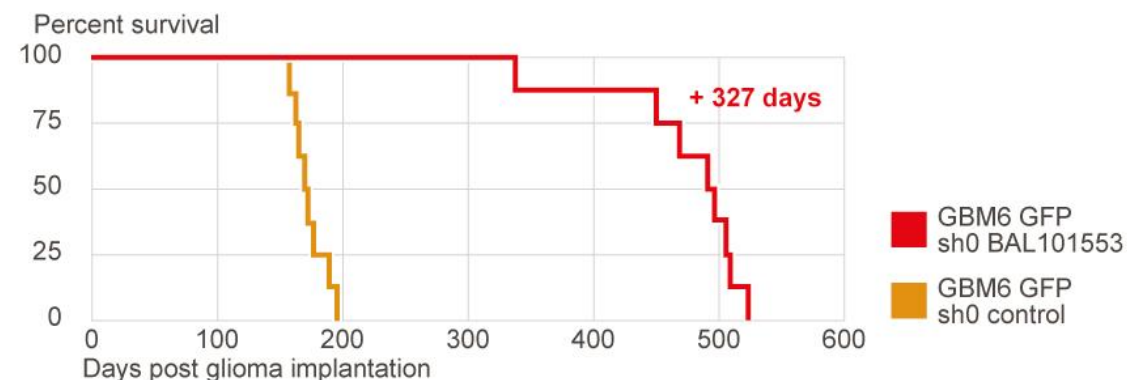
# EB1 — A potential response-predictive clinical biomarker for lisavanbulin

- 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>

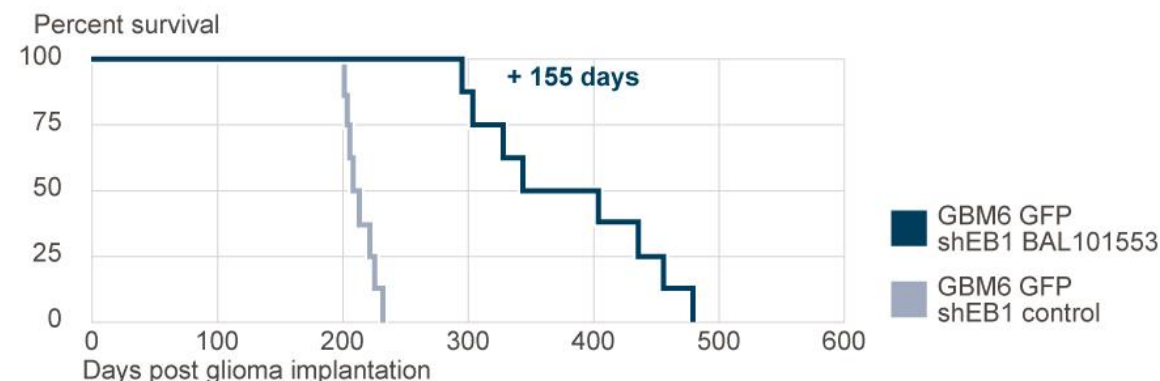
<sup>1</sup> Berges et al. EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after oral treatment with the novel tubulin-binding checkpoint activator BAL101553. Eur. J. Cancer 2018, 103, E61-62, A166

## 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**

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