



**Focused on
Growth and Innovation**

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

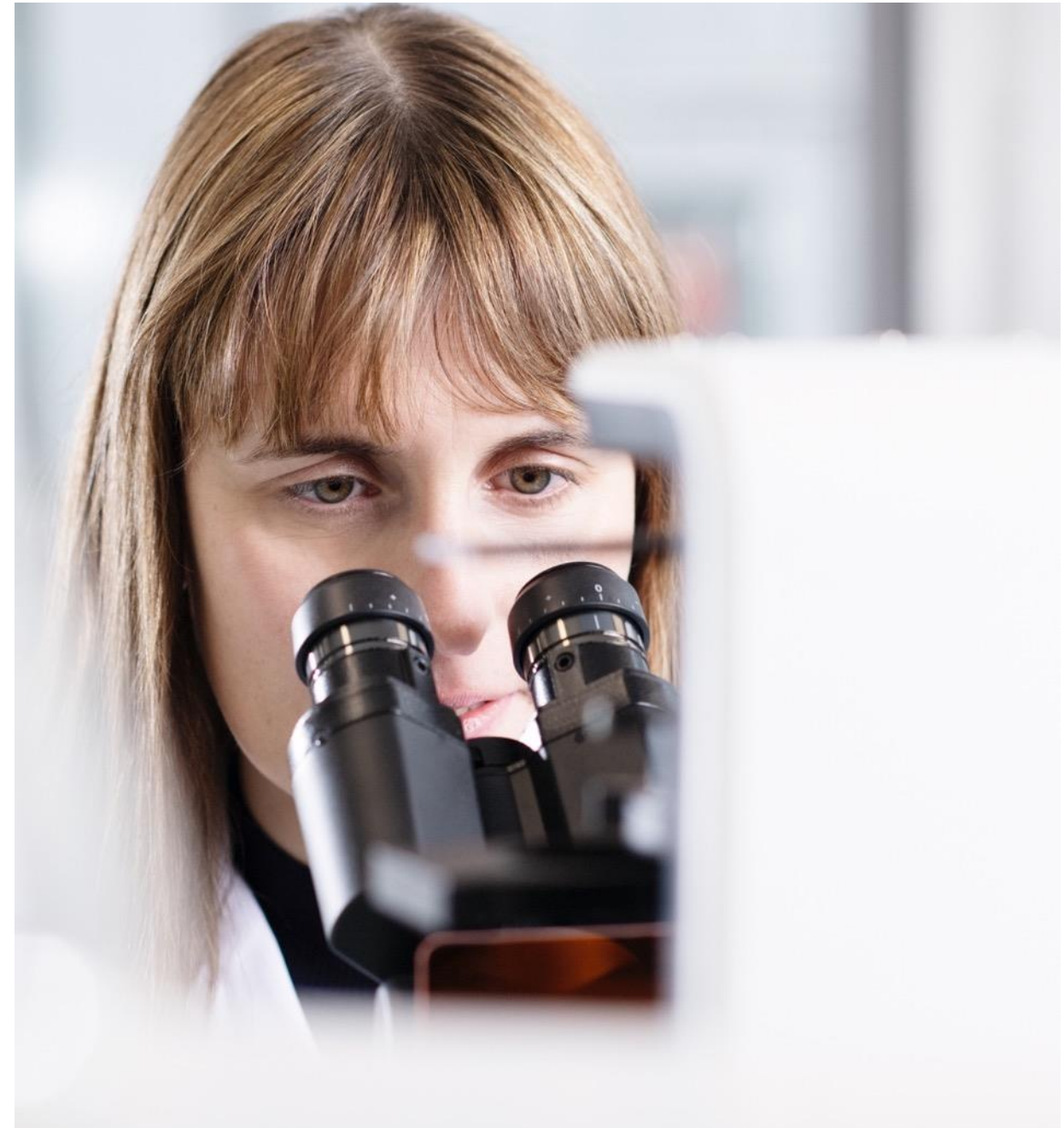
Investor presentation

January 24, 2022



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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

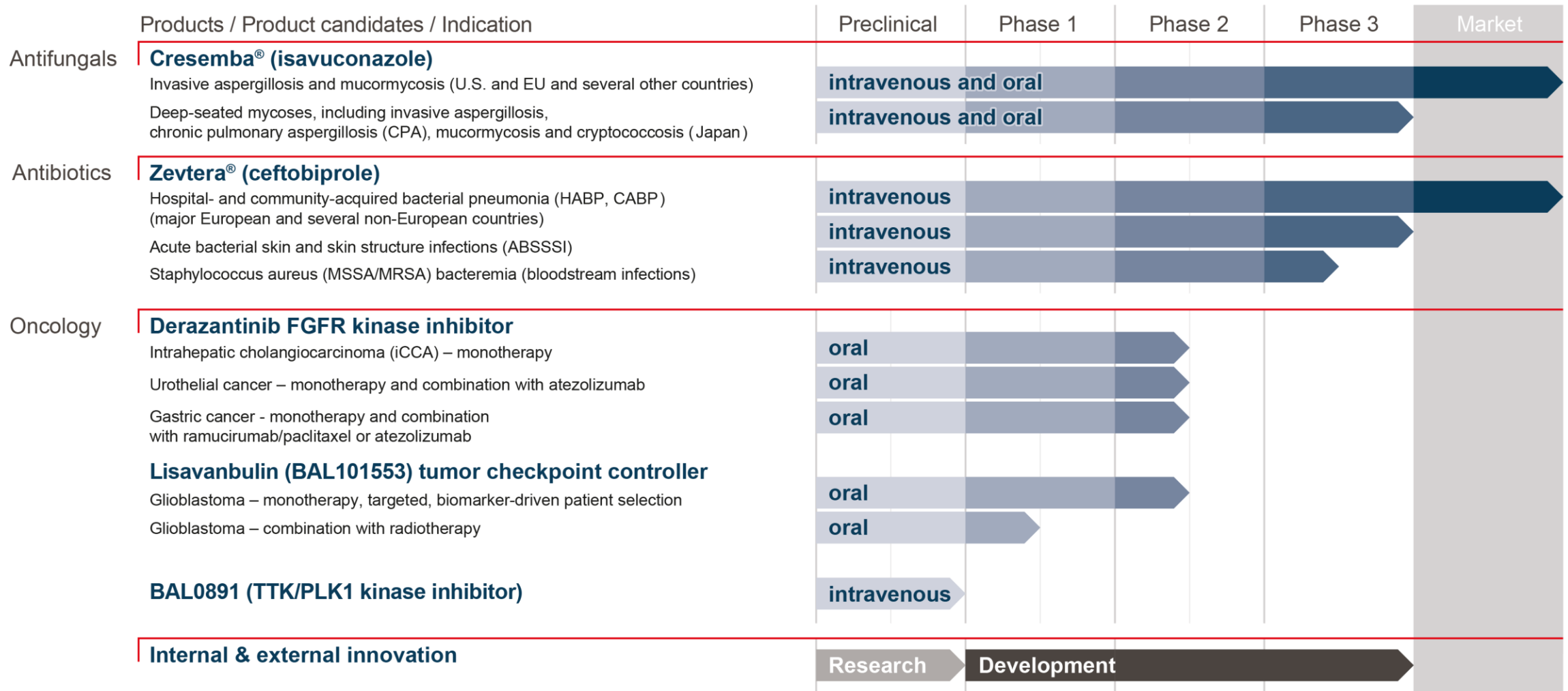


At a glance

- 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 three 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

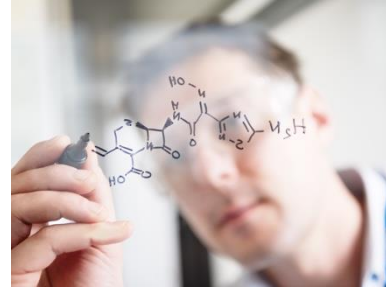


Our strategy



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[®] and Zevtera[®]



Leverage

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



Invest

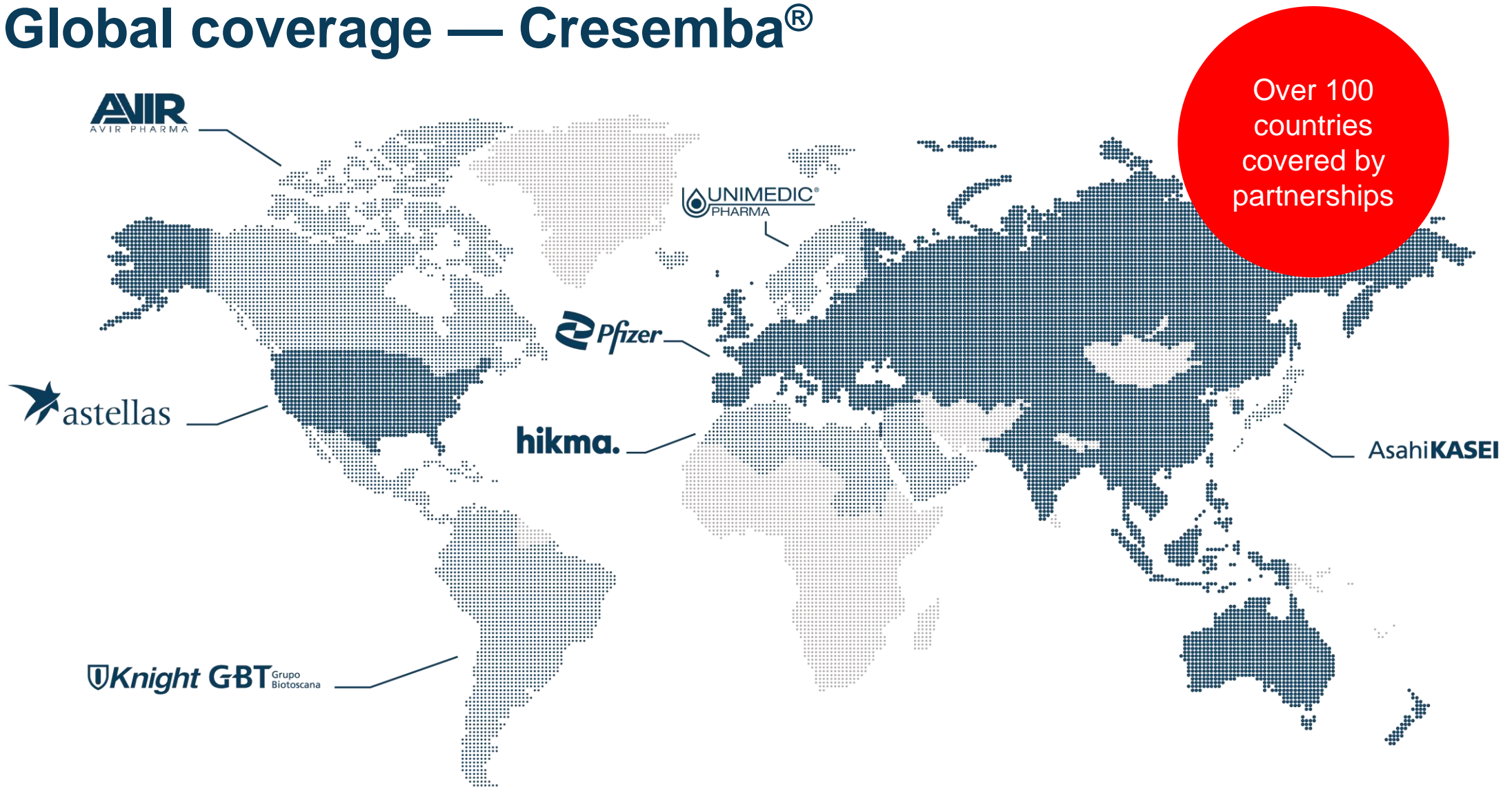
Invest in our clinical portfolio of targeted, small molecule, oncology drug candidates and the phase 3 ceftobiprole program



Innovate

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

Global coverage — Cresemba®



Over 100 countries covered by partnerships

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 295 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)

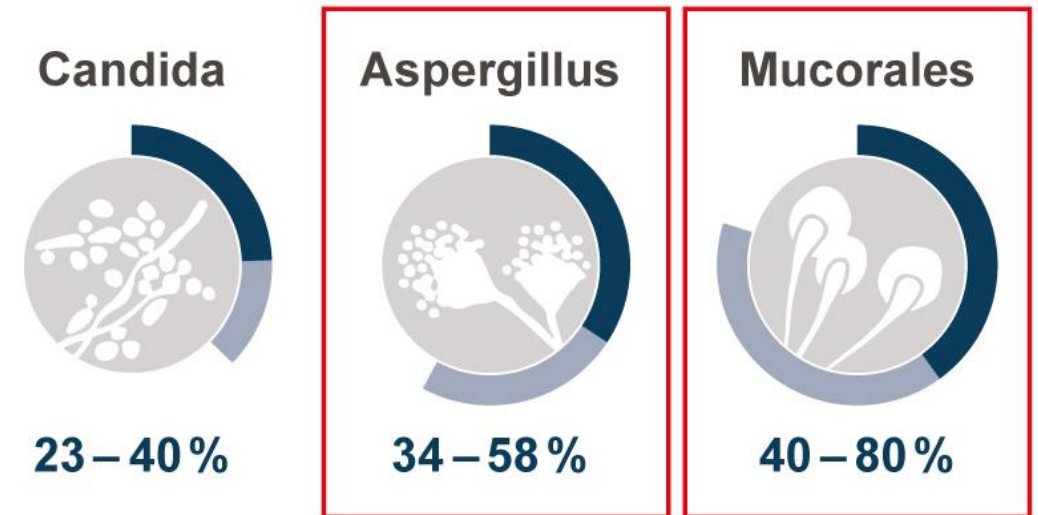
Invasive mold infections



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

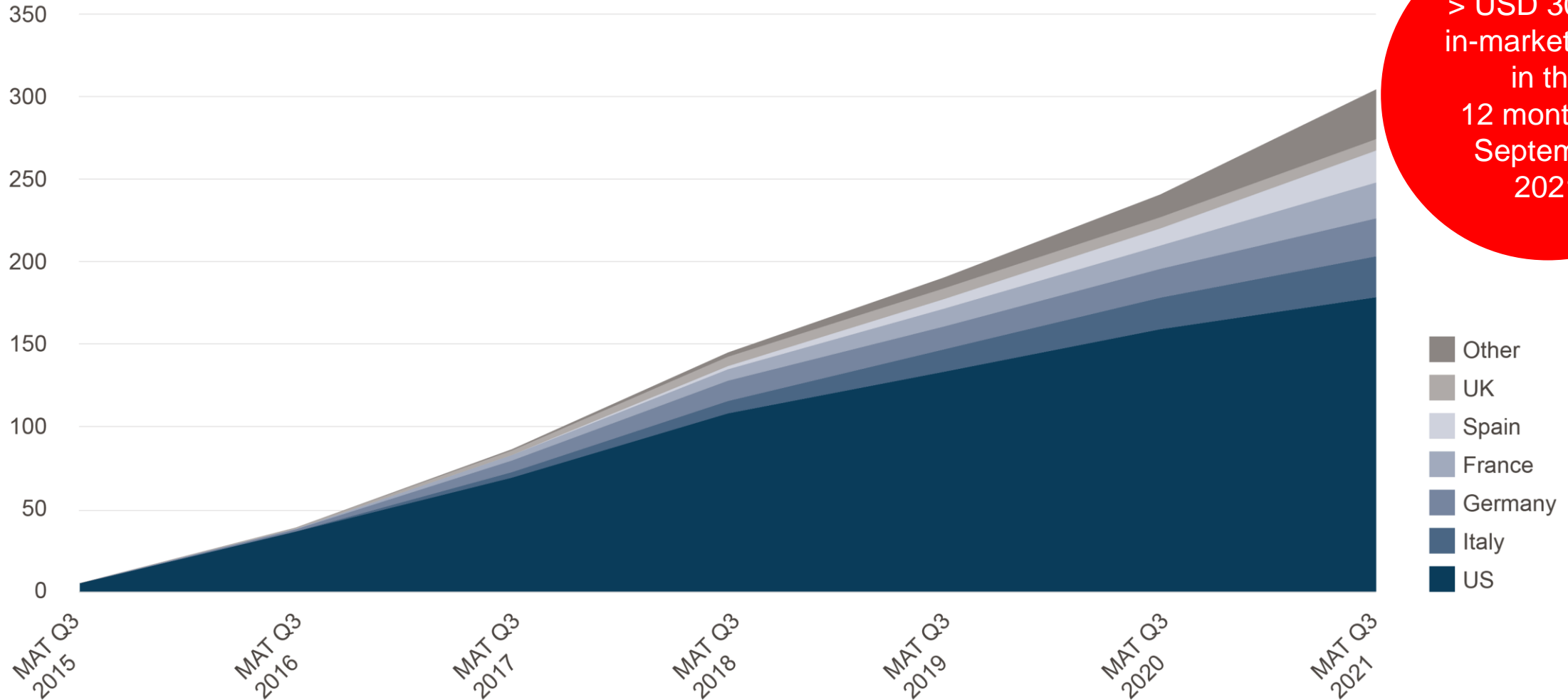
Mortality rates for invasive fungal infections**



**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

Sales in USD mn



> USD 300 mn
in-market sales
in the
12 months to
September
2021

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

Sales of best-in-class antifungals* by product

USD 3.2 bn sales (MAT Q3 2021)

- Potential to increase Cresemba[®] (isavuconazole) market share
 - Anticipated to be launched in ~70 countries by end-2022
 - 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, September 2021, rounding consistently applied

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[®]
(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

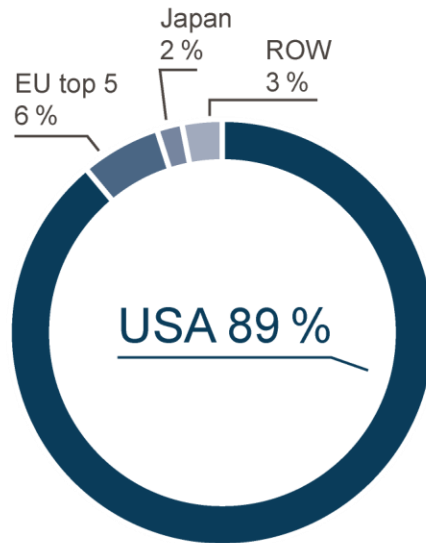
Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP), and community-acquired pneumonia (CAP). Not approved in the U.S.

MENA: Middle East and North Africa

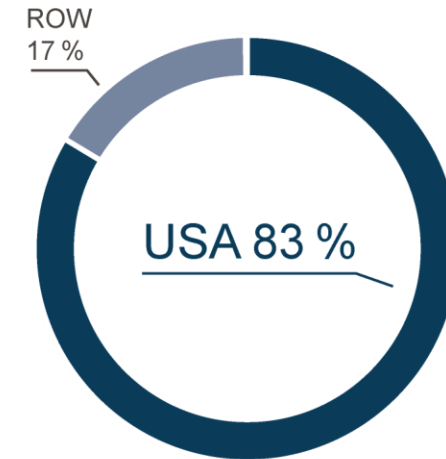


The hospital anti-MRSA antibiotic market — A USD 2.8 bn market* with the U.S. being the most important region

Daptomycin sales by region (2015, before LOE)



Ceftaroline sales by region (MAT Q3 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, September 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)¹, successfully completed



2. *Staphylococcus aureus* bacteremia (SAB)², patient enrolment completed in January 2022, topline results expected around mid-year 2022



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

¹ Overcash JS et al. ECCMID 2020, abstract 1594. (NCT03137173)

² Hamed K et al. Future Microbiol. 2020;15:35-48. (NCT03138733)

SAB – an area with high medical need

- Nearly 120,000 *S. aureus* bloodstream infections in the US (in 2017)¹
- ERADICATE targets complicated SAB, characterized by concomitant or metastatic infections such as bone, joint or heart valve infections; persistent bacteremia; or bacteremia in patients on dialysis
- Substantial morbidity and approximately 20% 30-day mortality²
- Limited antibiotic treatment options with only two approved treatments for SAB in the U.S. that cover both MSSA and MRSA, i.e. vancomycin and daptomycin

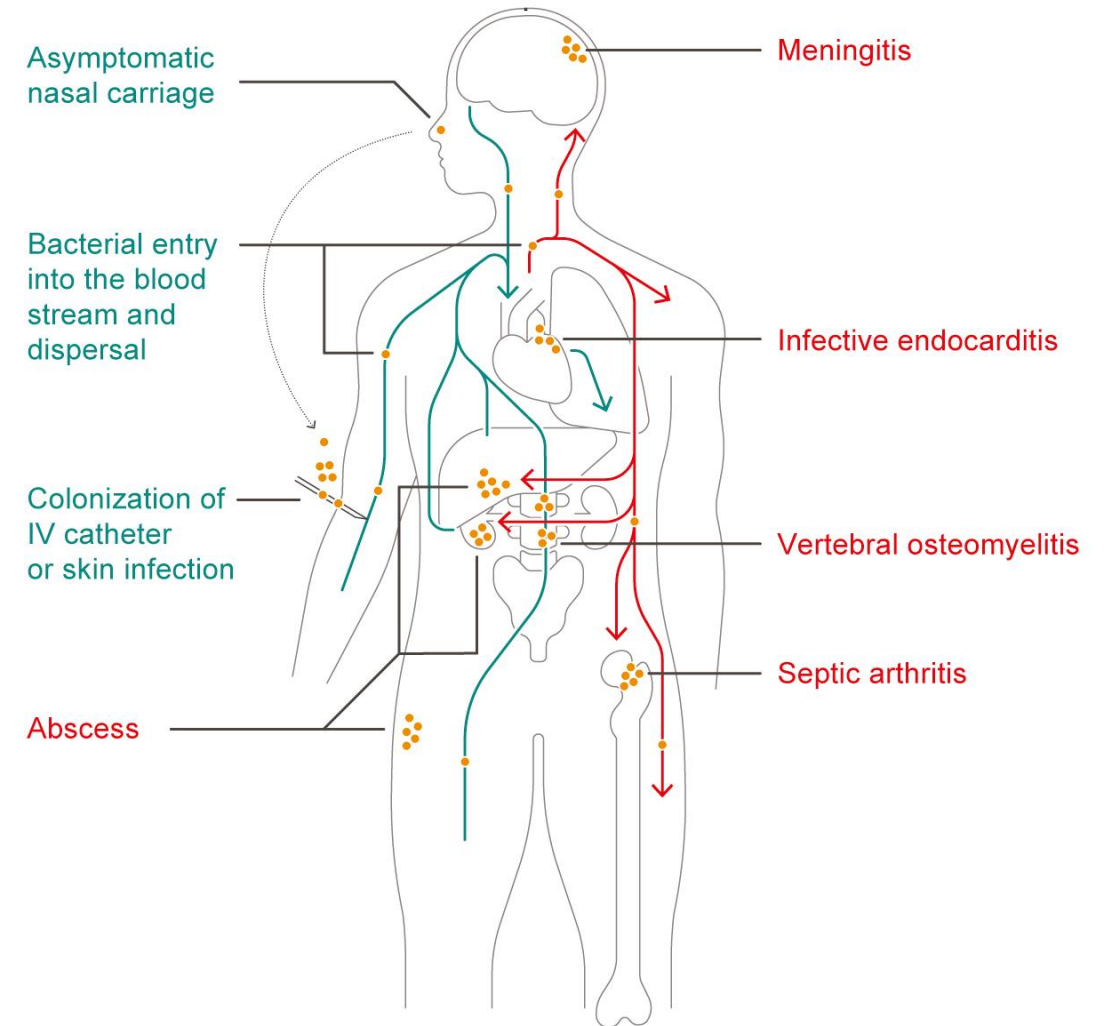
¹ MMWR, 2019;68:214–219.

² Hamed K et al. Future Microbiol. 2020;15:35-48.


MRSA: methicillin-resistant *Staphylococcus aureus*

MSSA: methicillin-susceptible *Staphylococcus aureus*

Causes and consequences of SAB



Adapted from Edwards AM et al. Trends Microbiol. 2011;19:184-190.

A microscopic 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.

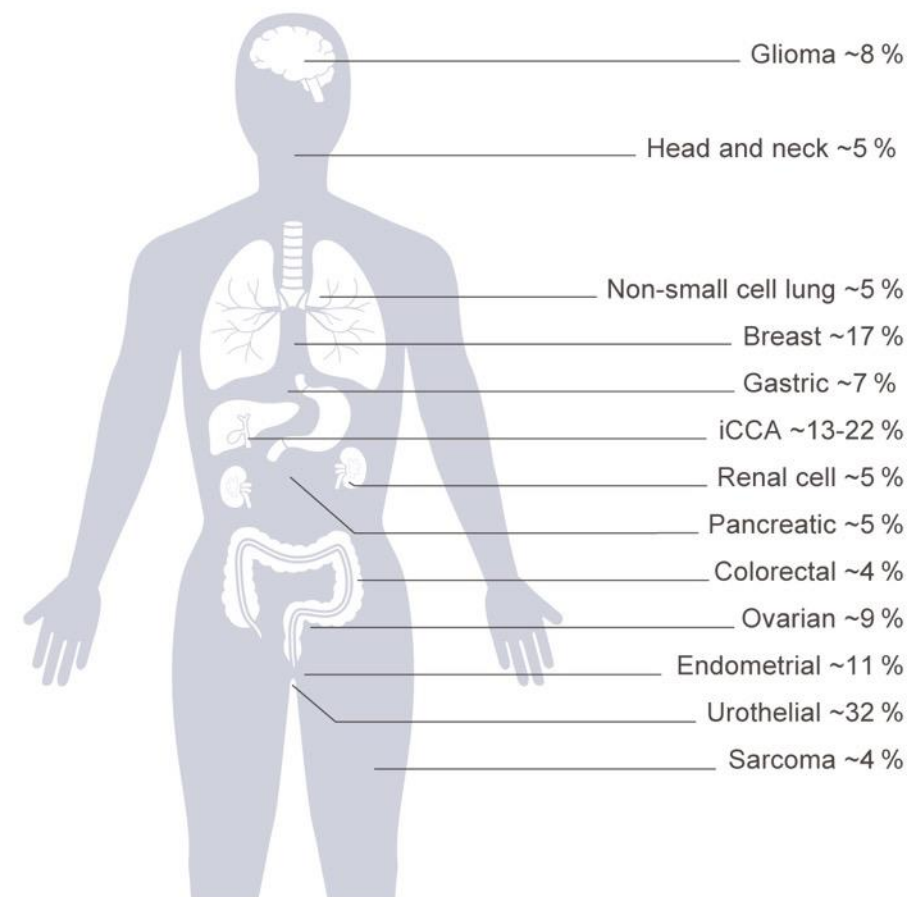
Oncology

Derazantinib

FGFR-driven tumors

Targeting FGFR-driven tumors as single agent and in combinations

- Small molecule, oral inhibitor of FGFR family of kinases
- Development strategy focused on achieving differentiation by leveraging unique properties of derazantinib
 - Kinase inhibition profile: exploring therapeutic potential of additional targets of derazantinib such as CSF1R and VEGFR2 kinase
 - Safety profile: exploring relevance for potential combination therapies
- Three clinical studies ongoing
 - FIDES-01 (Ph 2) in intrahepatic cholangiocarcinoma (iCCA)
 - FIDES-02 (Ph 1/2) in urothelial cancer
 - FIDES-03 (Ph 1/2) in gastric cancer



Sources: Helsten et al., Clin Cancer Res 2016 (22), 257-267; FGFR2 fusions in iCCA: Graham et al. Hum Pathol 2014 (45), 1630-1638; Jain et al. JCO Precis Oncol 2018 (2) 1-12

Phase 2 studies with FGFR-inhibitors in iCCA

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

Derazantinib ⁵ FIDES-01 Cohort 2*	Pemigatinib ⁶ (Incyte) FIGHT-202
23	20
9%	0%
74%	40%
7.3 months	2.1 months

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

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

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. Javle et al., J Clin Oncol 40, no. 4_suppl (February 01, 2022) 427-427. 6. Abou-Alfa et al. Lancet Oncol 2020;21(5):671-684.

*Interim analysis, based on investigator assessments.

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 H1 2022*
- Exploring an intensified dose regimen of derazantinib in two cohorts of the study:
 - Focus on maximizing efficacy by using an intensified dose regimen of 400 mg per day
 - as monotherapy in a second-or post second-line setting in FGFR-inhibitor naïve patients
 - as monotherapy or in combination with atezolizumab in first-line cisplatin-ineligible patients
 - Supported by the observed safety and tolerability profile of derazantinib and by pharmacology data
- Initial results from cohorts utilizing 400 mg per day dose regimen expected H1 2022

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

FIDES-02: NCT04045613; Chaudhry A et al. Journal of Clinical Oncology 2020; 38, no. 6_suppl. TPS590

Clinical program in gastric cancer – FIDES-03

Multi-cohort Phase 1b/2 study of derazantinib as monotherapy or in combination therapy with standard of care (ramucirumab/paclitaxel) or atezolizumab in patients with advanced HER2-negative gastric adenocarcinoma harboring FGFR genetic aberrations

- Substudies using derazantinib monotherapy or combination treatment, including:
 - Derazantinib monotherapy in various molecular subtypes
 - Combination of derazantinib with ramucirumab/paclitaxel
 - Combination of derazantinib with atezolizumab
- Exploring an intensified dose regimen of derazantinib 400 mg per day in monotherapy and in combination therapy
- Interim results in derazantinib monotherapy and recommended phase 2 dose of derazantinib in combination with ramucirumab/paclitaxel expected H1 2022
- Clinical supply agreement with Roche for atezolizumab
- Clinical trial collaboration and supply agreement with Lilly for ramucirumab

FGFR-inhibitors show differences in safety profiles

	Cholangiocarcinoma				Urothelial cancer
	DZB ¹ (N=103)	INF ² (N=108)	FUT ³ (N=67)	PEM ⁴ (N=146)	ERD ⁵ (N=99)
Dosing regimen	300 mg QD	125 mg Q4W QD for 3w	20 mg QD	13.5 mg Q3W QD for 2w	8 mg QD (titration to 9 mg)
Most frequent treatment-related adverse events	Phosphorus↑ Nausea AST↑	Phosphorus↑ Stomatitis Alopecia/PPES	Phosphorus↑ Diarrhea Dry mouth	Phosphorus↑ Alopecia Dysgeusia	Phosphorus↑ Stomatitis Dry mouth
Hyperphosphatemia	37%	74%	81%	55%	73%
Alanine aminotransferase (ALT) ↑	23%	8%	NR	2%	12%
Alopecia	14%	32%	NR	46%	27%
Diarrhea	20%	18%	37%	36%	37%
Dry eye	22%	31%	NR	21%	19%
Dry mouth	23%	21%	33%	29%	43%
Fatigue	20%	29%	NR	32%	21%
Hand-foot syndrome/PPES	2%	32%	18%*	15%	22%
Nail toxicities	7%	57%*	42%*	43%*	52%
Retinopathy [†]	1%	17%*	9%*	3%	21%
Stomatitis	2%	51%	NR	32%	55%

Abbreviations: DZB: derazantinib, INF: infigratinib, FUT: futibatinib, PEM: pemigatinib, ERD: erdafitinib; PPES: Palmar-plantar erythrodysesthesia syndrome; NR: not reported; QD: daily; Q3W/Q4W: every 3/4 weeks; w: weeks

Percentages refer to treatment-related adverse events except for annotated (*) adverse events regardless of causality.

[†]Refers to Retinal Pigment Epithelial Detachment (RPED) or Central Serous Retinopathy (CSR).

References:

¹ Droz Dit Busset et al. Annals of Oncology (2021) 32 (suppl_5): S376-S381 and Basilea data on file; ² Javle et al. Lancet Gastroenterol Hepatol. 2021 Oct;6(10):803-815 and Trusetiq U.S. Prescribing information (05/2021);

³ Goyal et al. J Clin Onc 38, no. 15_suppl (May 20, 2020) 108-108; ⁴ Abou-Alfa et al. Lancet Oncol. 2020 May;21(5):671-684 and Pemazyre™ U.S. Prescribing Information (06/2021);

⁵ Llorca et al. N Engl J Med. 2019 Jul 25;381(4):338-348 and Balversa™ U.S. prescribing information (07/2020).

Oncology

Lisavanbulin (BAL101553)

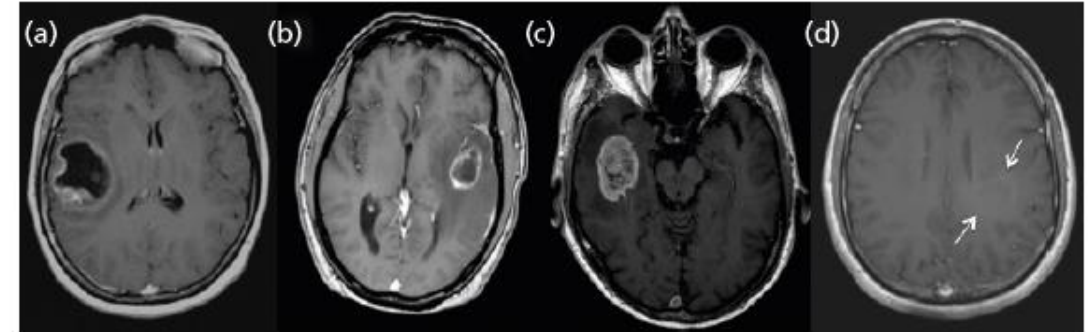
Glioblastoma
and other solid tumors



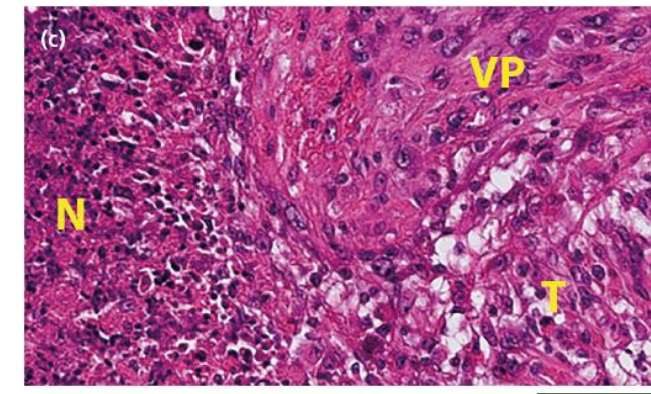
Unmet medical need in glioblastoma

- The most common primary brain cancer in adults with an incidence of 3-4 per 100,000 people, (though geographic variation exists) and a median age at onset of > 60 years
- Associated with poor prognosis, high morbidity and healthcare burden
- 5-year survival is below 5% with current standard of care (multimodality treatment including surgery, radiotherapy, chemotherapy)¹
- *MGMT*-promoter methylation status has been demonstrated as a predictor for the response to (radio)chemotherapy (temozolomide)²
- Established molecular markers used for classification include IDH mutations and/or 1p/19q codeletion³
- No molecular targeted therapy currently approved

Radiological and tissue presentations of glioblastoma



Variable glioblastoma appearances on post-gadolinium T1-weighted MRI: central necrotic mass with nodular rim enhancement (a,b), predominantly solid enhancement (c), lack of contrast uptake (d)



Histological glioblastoma; H&E stain.

100 μm

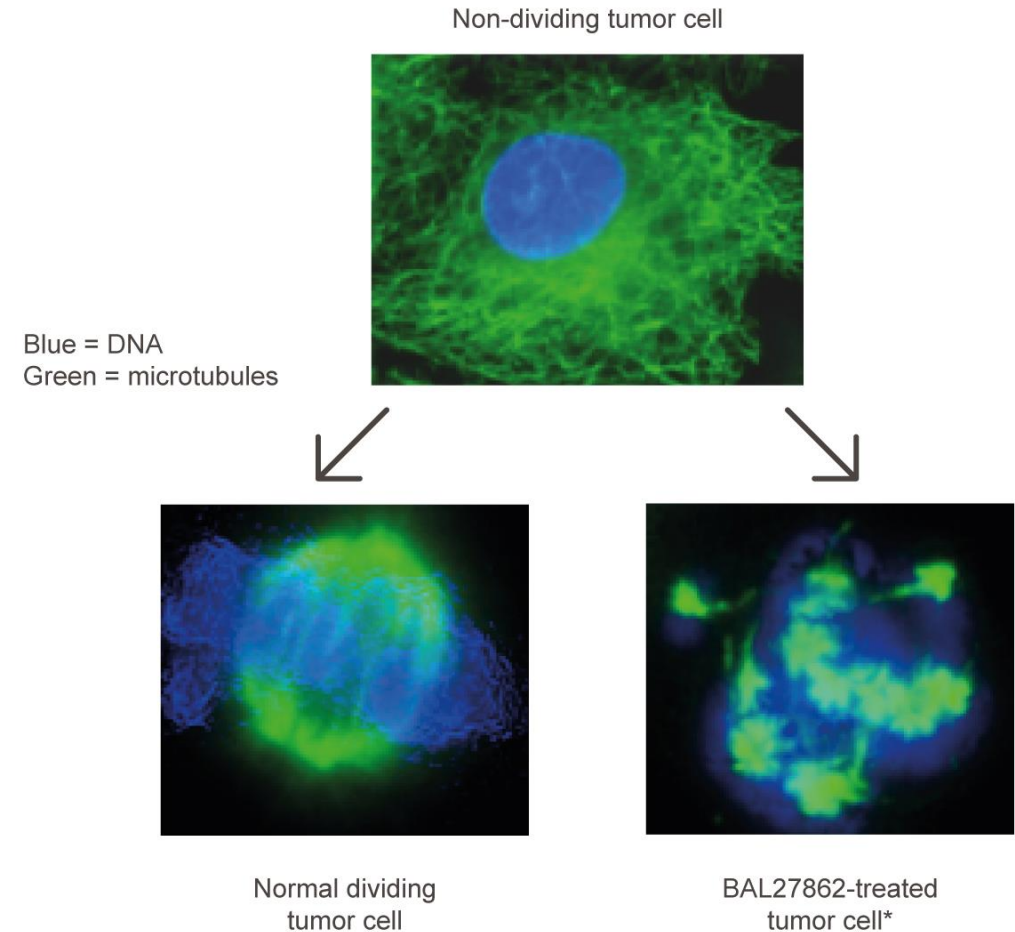
Histological features of glioblastoma include marked hypercellularity, nuclear atypia, microvascular proliferation, and necrosis (N: necrosis, VP: vascular proliferations, T: tumor)

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

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

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

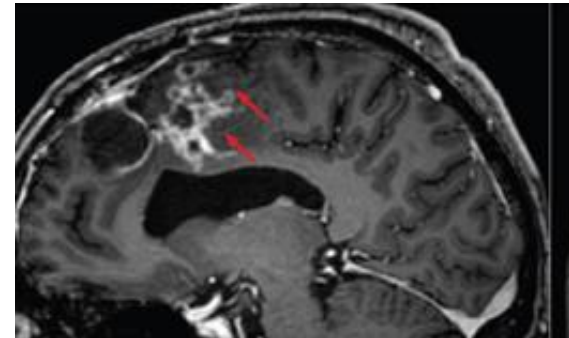


* Lisavanbulin (BAL101553) is a prodrug of BAL27862

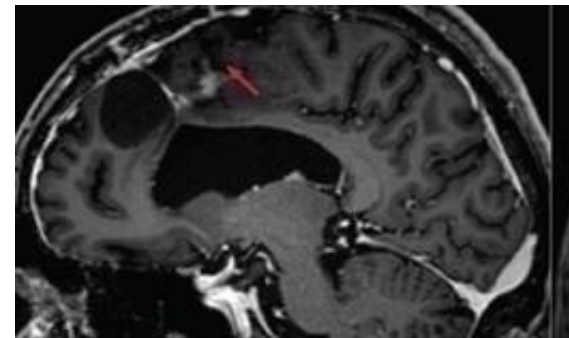
Biomarker-driven phase 2 study ongoing in recurrent glioblastoma

- 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):^{1, 2}
 - Three patients with EB1-positive glioblastoma
 - Two of the EB1-positive patients with long-lasting clinical benefit, ongoing for more than 2 years
 - One exceptional response with >80% reduction in glioblastoma tumor size
 - No clear clinical benefit for EB1-negative patients
- Phase 2 interim results expected H1 2022

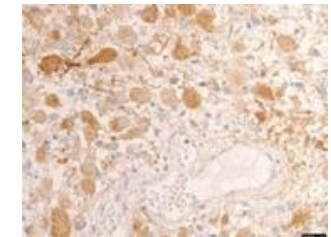
Glioblastoma tumor size reduction in an exceptional responder and EB1 staining of glioblastoma tissue compared to non-responding patients



Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



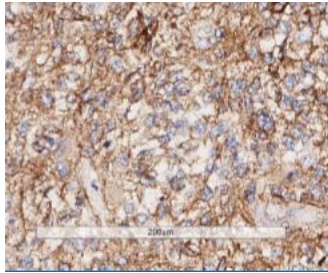
Non-responder

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

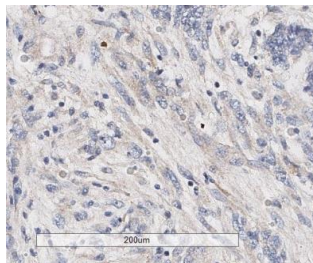
² Tiu et al. JCO 2021;39,15 suppl, TPS2068 (NCT02490800)

EB1-prevalence in glioblastoma and other cancer types

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

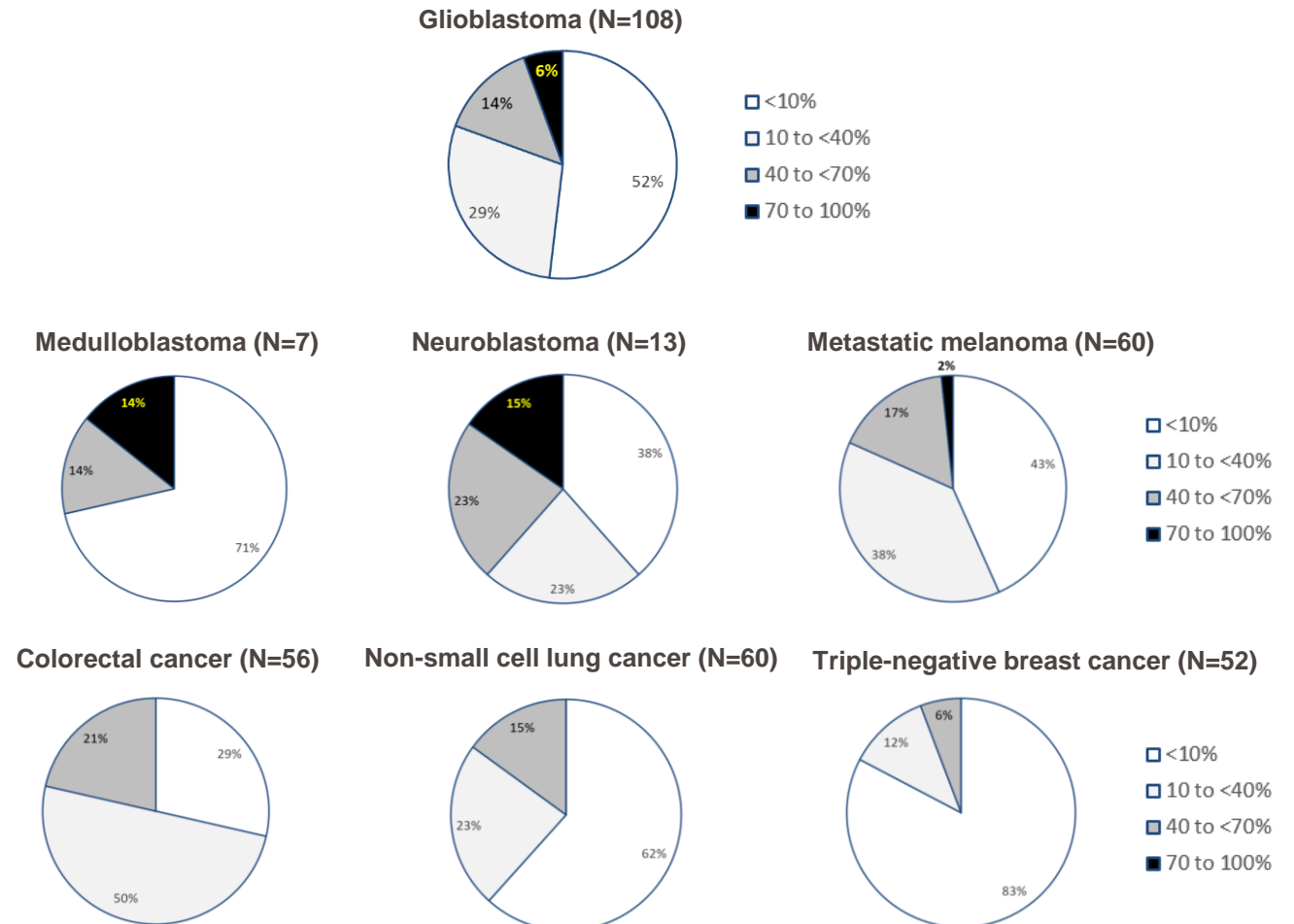


EB1-positive:
Tumor cells show moderate to strong EB1 staining



EB1-negative:
Absence of moderate to strong EB1 staining

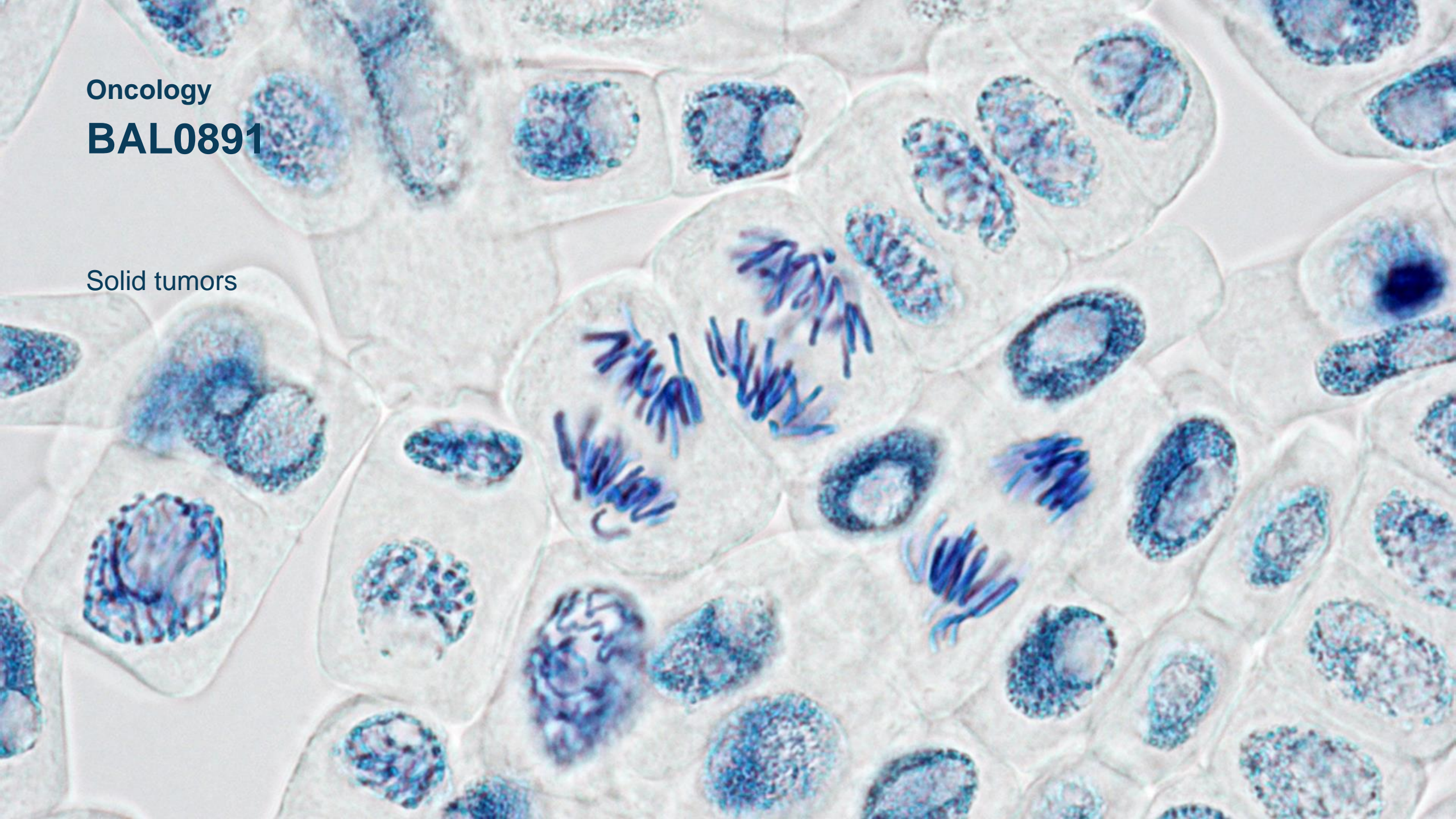
Prevalence of moderate/strong EB1 staining in various tumor types¹



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

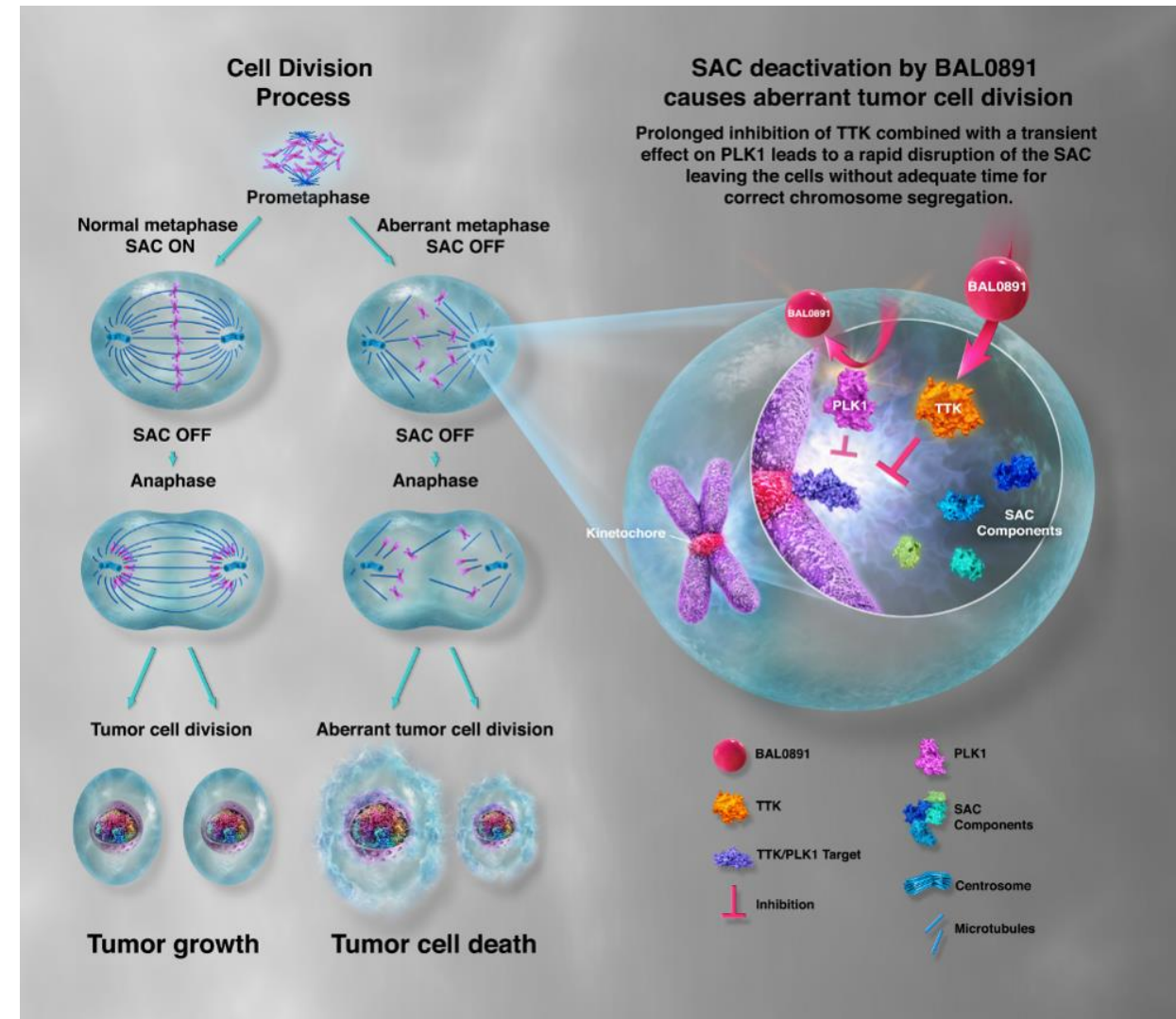
Oncology
BAL0891

Solid tumors



A first-in-class mitotic checkpoint inhibitor

- Unique dual inhibitor of threonine tyrosine kinase (TTK) and polo-like kinase 1 (PLK1)
 - Dual action of BAL0891, with prolonged TTK and transient PLK1 inhibition, leads to a rapid disruption of the spindle assembly checkpoint (SAC)
 - Cells are pushed through mitosis without adequate time for correct chromosome alignment and segregation
 - Activity results in aberrant tumor cell division leading to tumor cell death
 - Potent single-agent anti-cancer activity in preclinical models of human cancer
- FDA approved IND in December 2021
- Initiation of phase 1 study in patients with solid tumors planned for Q1 2022





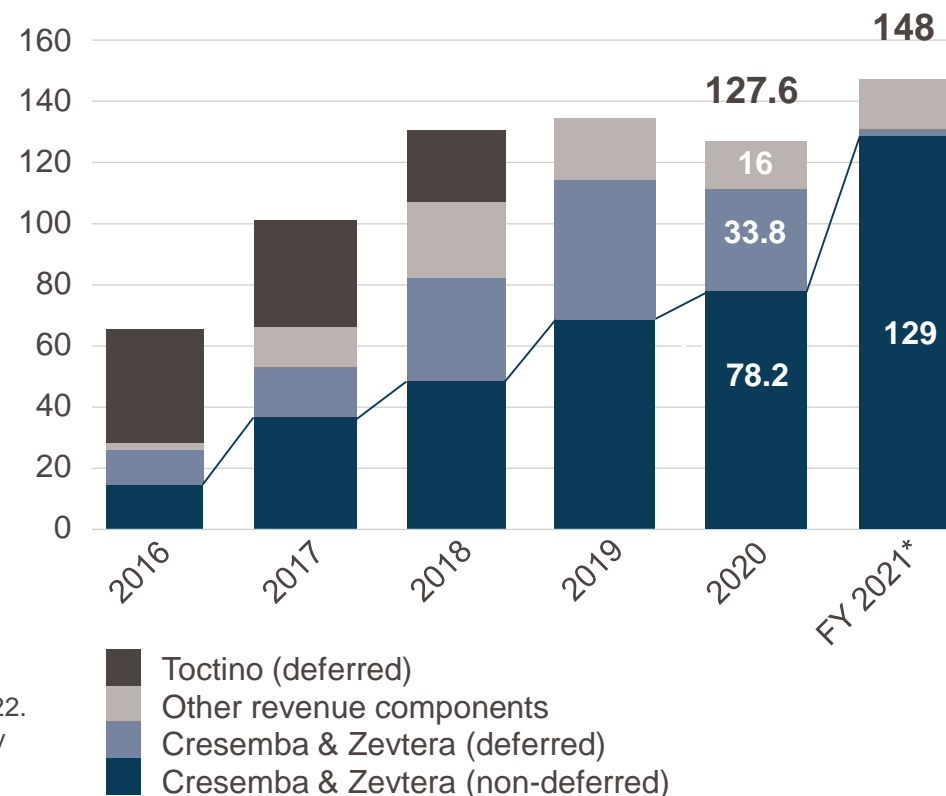
Financials & Outlook



2021 preliminary revenue and year-end cash-position exceed financial guidance

In CHF mn	FY 2021*	FY 2021e (guidance)	FY 2020 (actual)
Total revenue	148	134 – 144	127.6
thereof: Contributions Cresemba® & Zevtera®			
non-deferred	129	115 – 125	78.2
deferred		2.5	33.8
Operating loss	N/A	7 – 17	8.2
Cash and investments#	150 (173##)	142 - 147 (165 – 170##)	167.3

Continued strong double-digit growth in Cresemba & Zevtera non-deferred revenue contributions Y-o-Y, CHF mn



* The audited full financial statements as well as the annual report 2021 will be published on February 15, 2022. The final audited revenue for 2021 and the cash position as of year-end 2021 may differ from the preliminary reported numbers

Cash, cash equivalents, restricted cash and investments

Excluding impact from reduction of the outstanding convertible bonds in 2021

Outlook 2021 / 2022

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

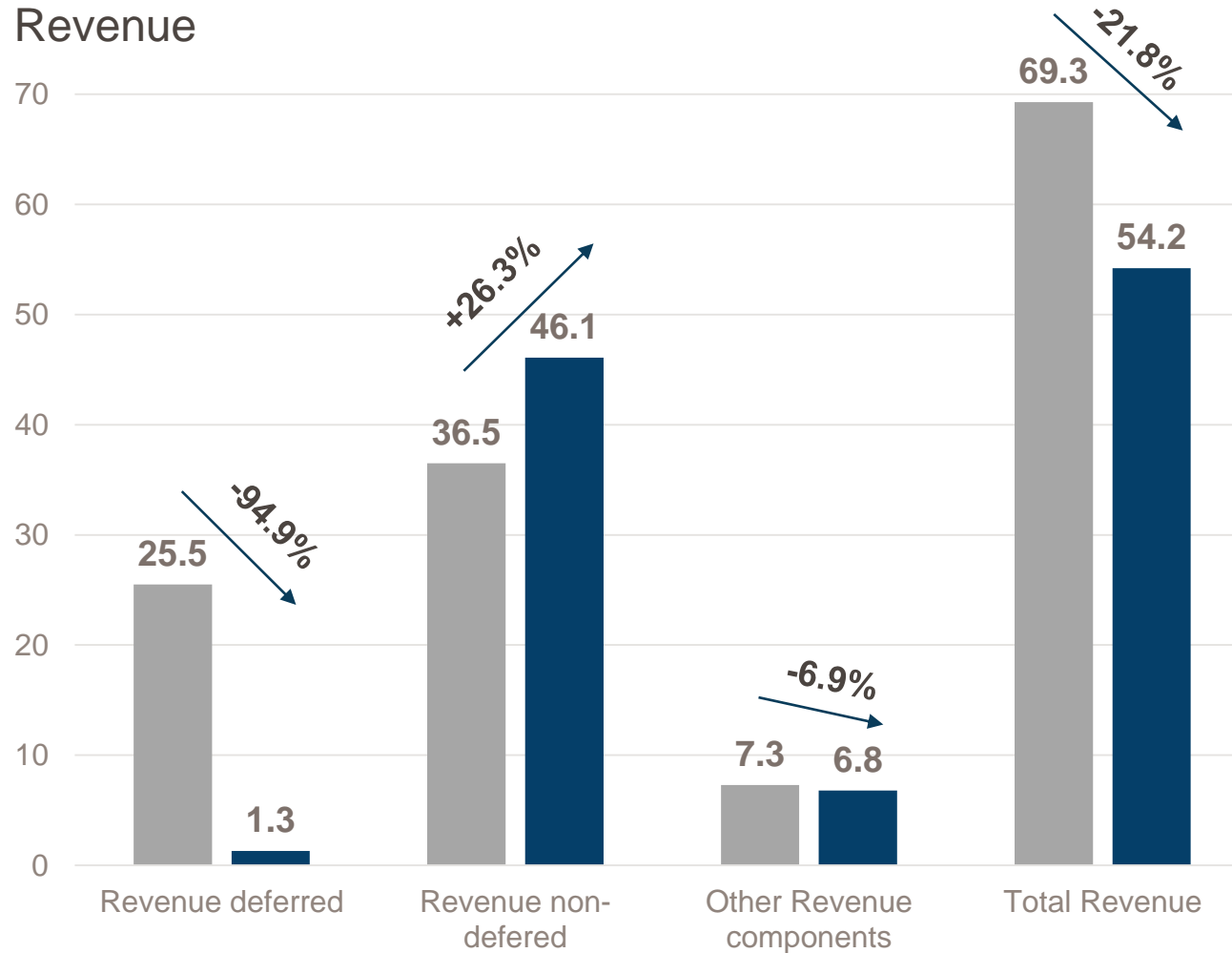
		H1 2021	H2 2021	H1 2022	H2 2022
Isavuconazole		✓ Complete patient enrolment in phase 3 study in Japan	✓ File NDA in Japan		Marketing authorization decision in Japan
Ceftobiprole			✓ Complete patient enrolment in SAB phase 3 study*	Topline results from SAB phase 3 study	
Derazantinib	FIDES-01 (iCCA)	✓ Topline results (FGFR2 gene fusions)			
		✓ Interim results (other FGFR2 genetic aberrations)		Topline results (other FGFR2 genetic aberrations)	
	FIDES-02 (urothelial cancer)			Interim results in monotherapy and combination therapy with atezolizumab in patients refractory to prior FGFR inhibitors Interim results in monotherapy (400 mg/day) in 2nd-line FGFR-inhibitor naïve patients and atezolizumab combination in 1st-line cisplatin-ineligible patients	
	FIDES-03 (gastric cancer)			Interim results in monotherapy (400 mg/day) and recommended phase 2 dose with ramucirumab/paclitaxel	Interim efficacy results in combination with ramucirumab/paclitaxel
Lisavanbulin				Interim results from phase 2 biomarker-driven glioblastoma study	Topline results from phase 2 biomarker-driven glioblastoma study
				Recommended phase 2 dose in phase 1 study in newly-diagnosed glioblastoma in combination with radiotherapy	
BAL0891			✓ IND approved by FDA	Initiate phase 1 study	

* Completed early January 2022

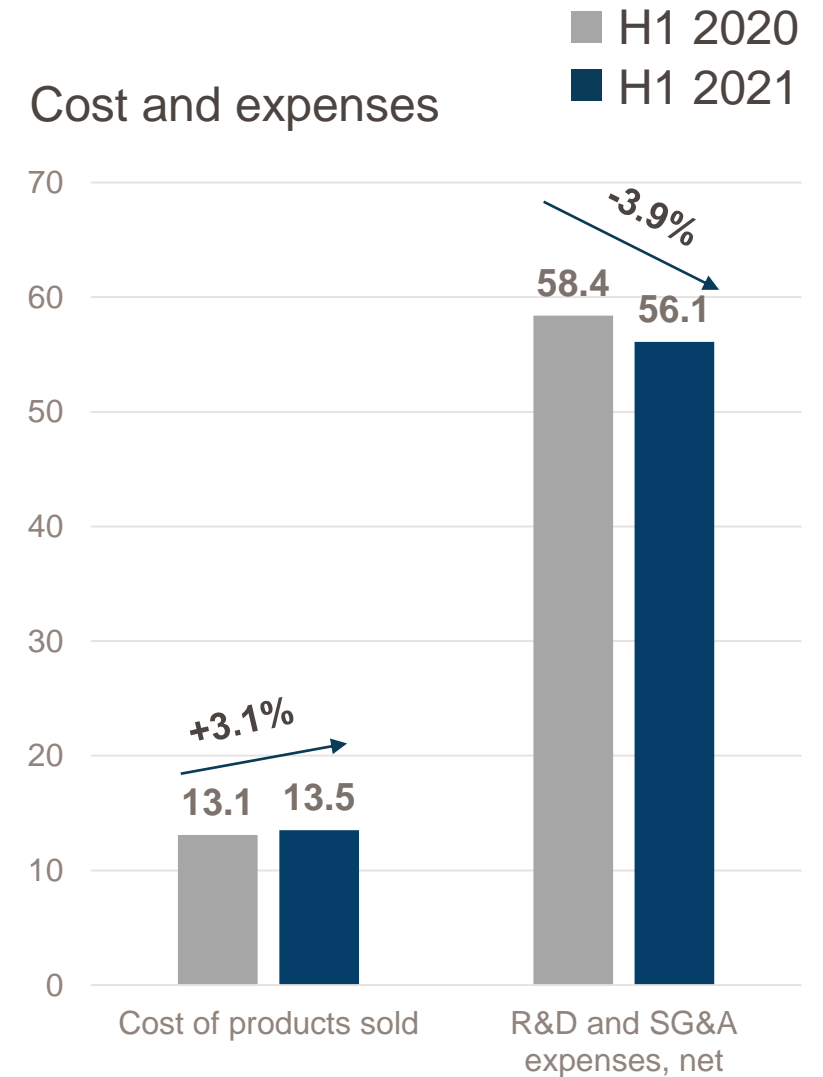
Appendix

Financial summary, in CHF mn (1/2)

Revenue



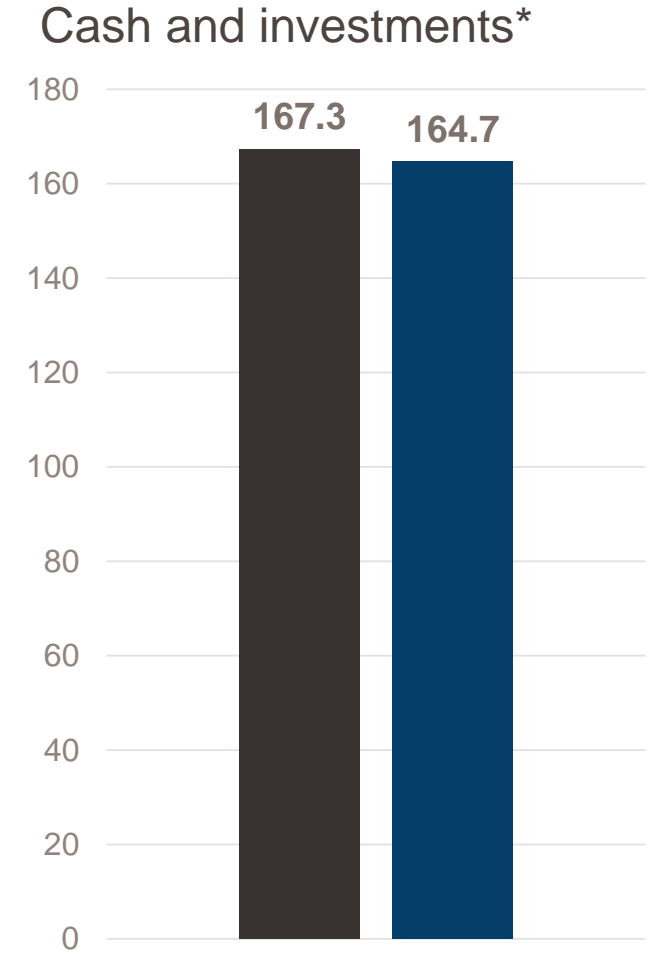
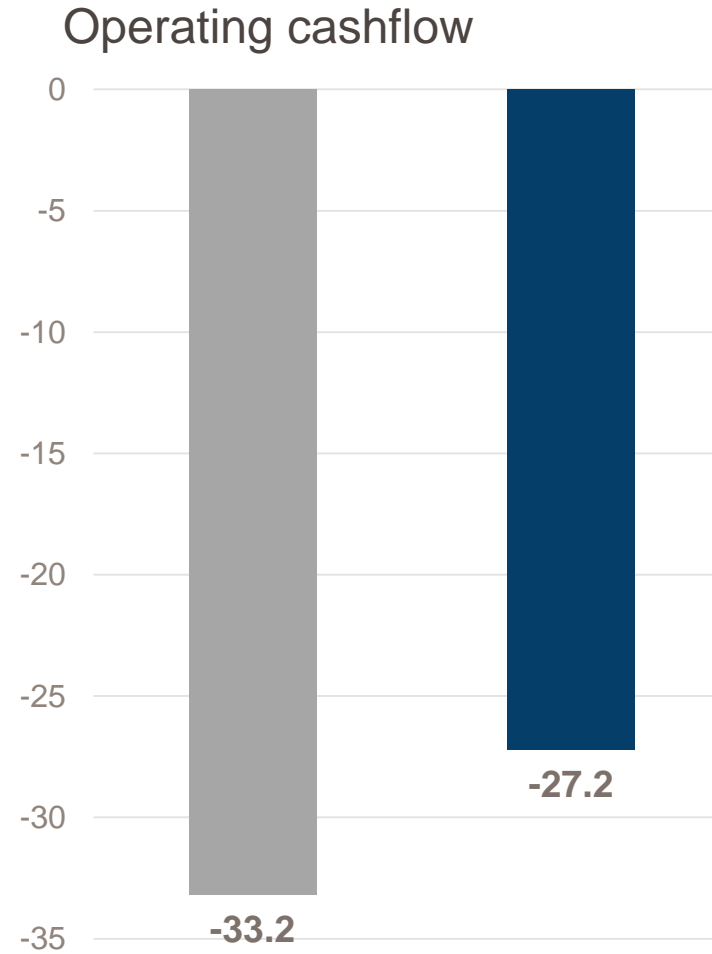
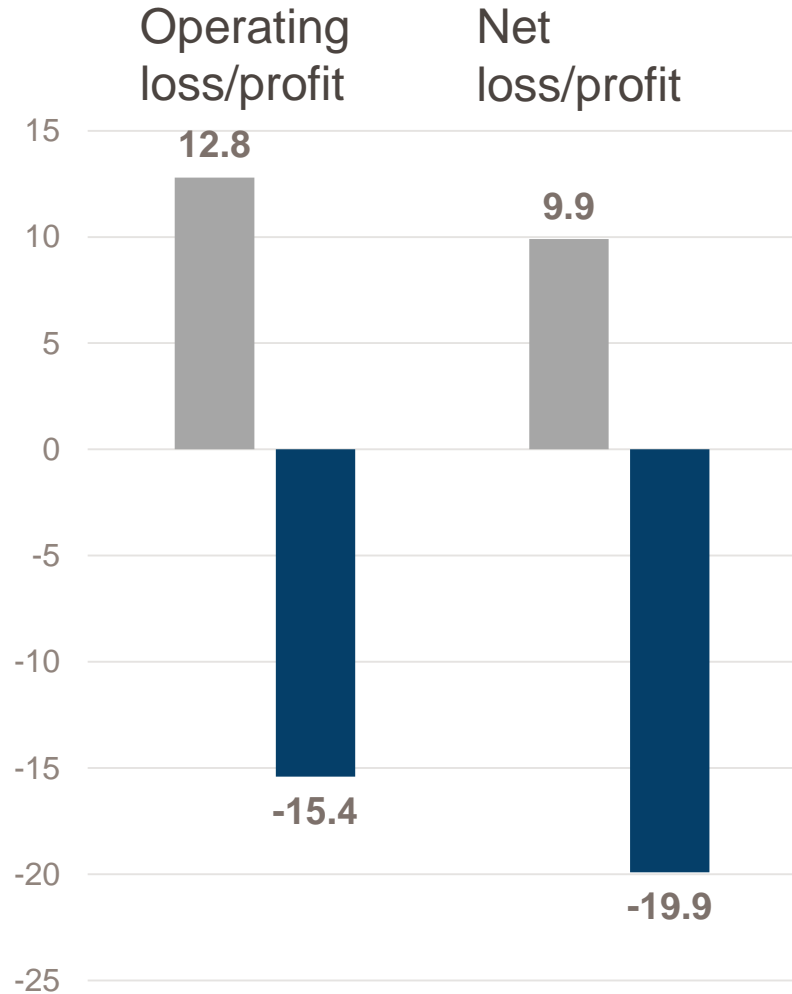
Cost and expenses



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

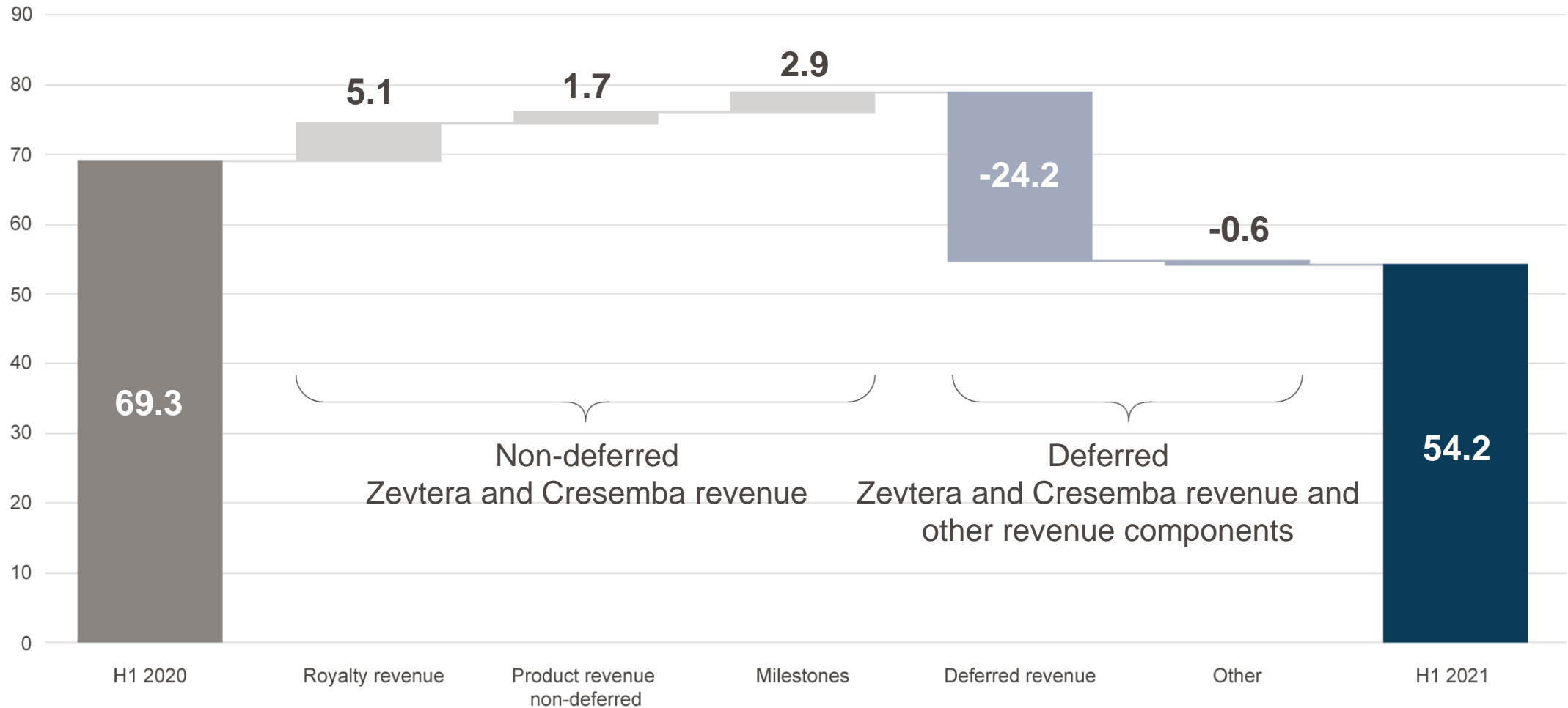
Financial summary, in CHF mn (2/2)

- H1 2020
- H1 2021
- YE 2020



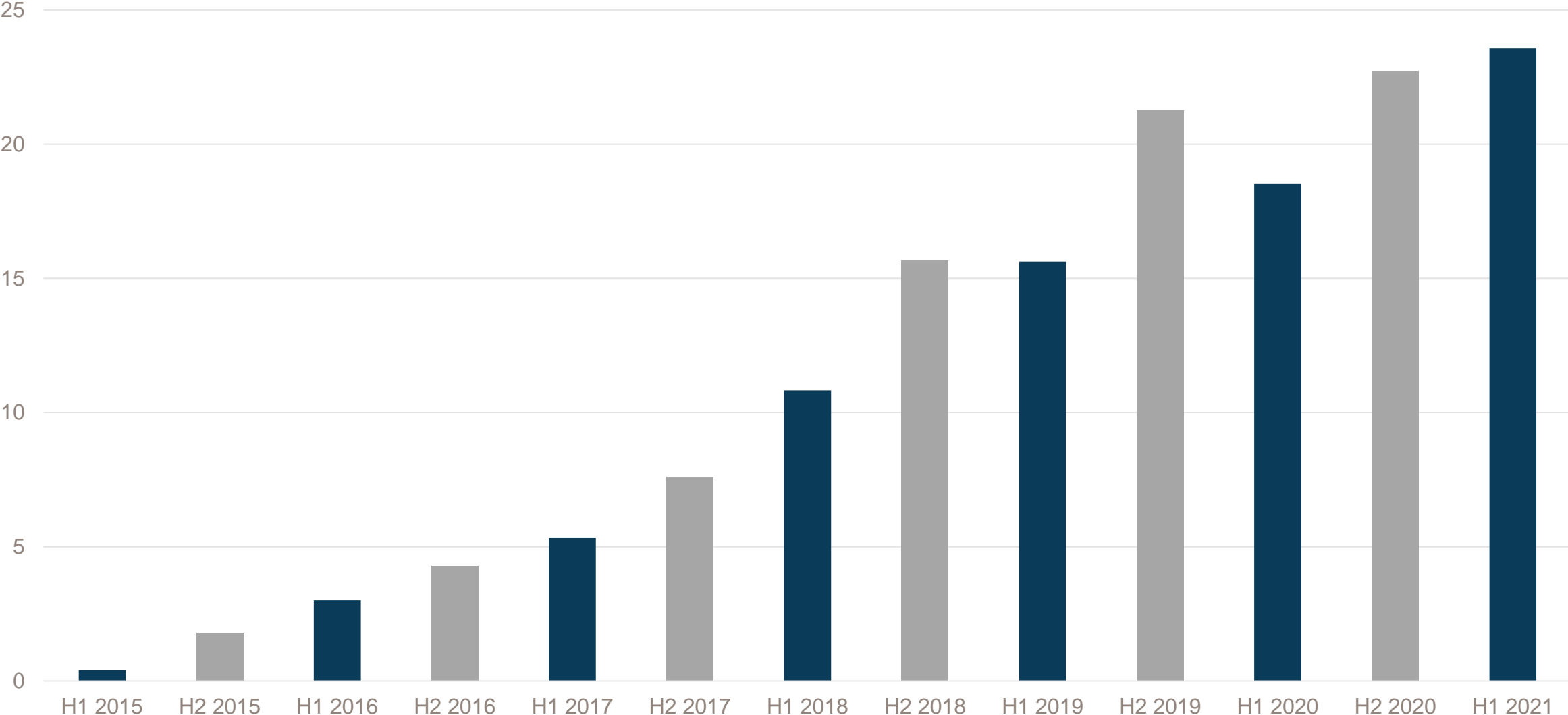
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently, *Cash, cash equivalents, restricted cash and investments

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



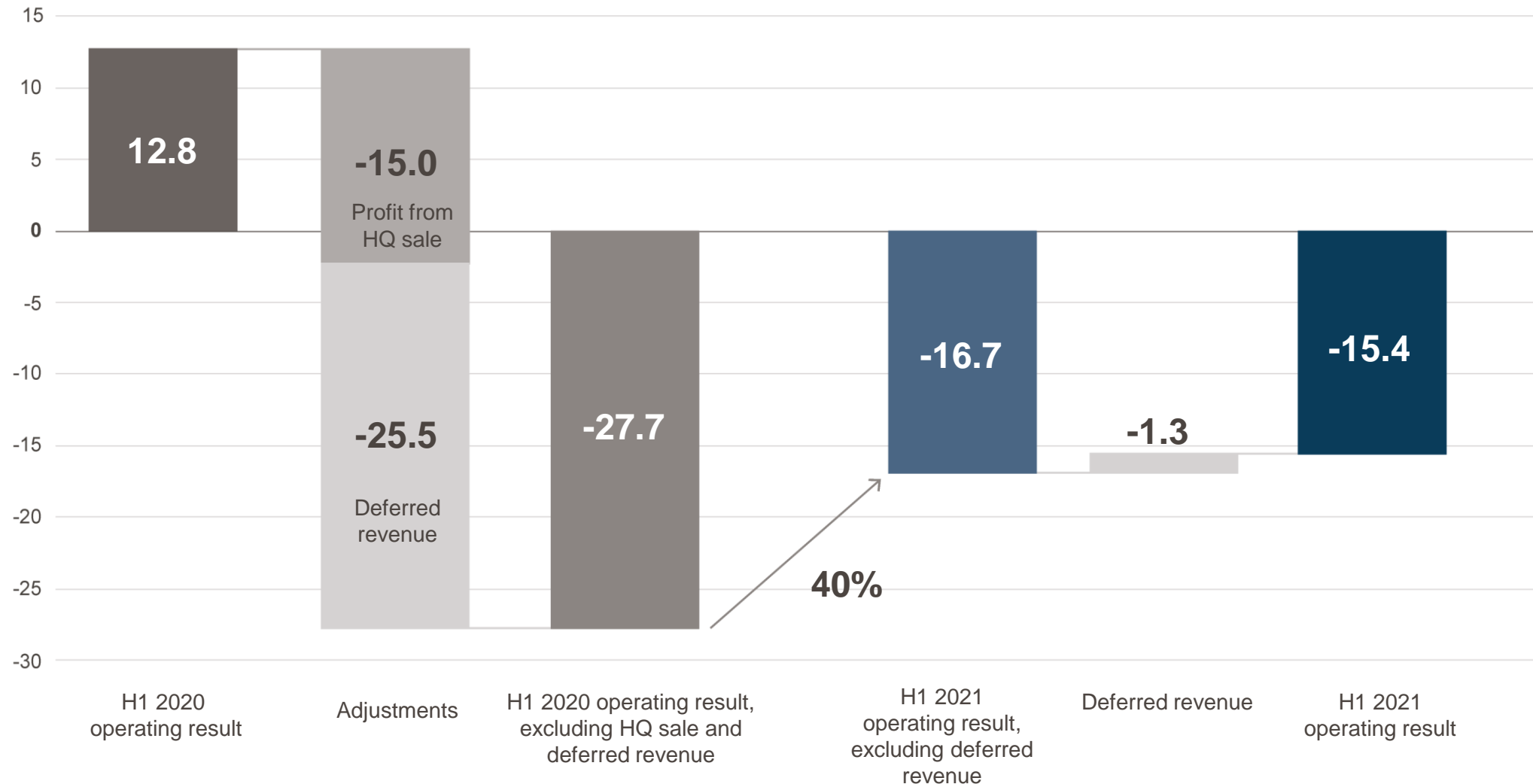
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

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



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

Significant improvement in underlying operating performance (CHF mn)

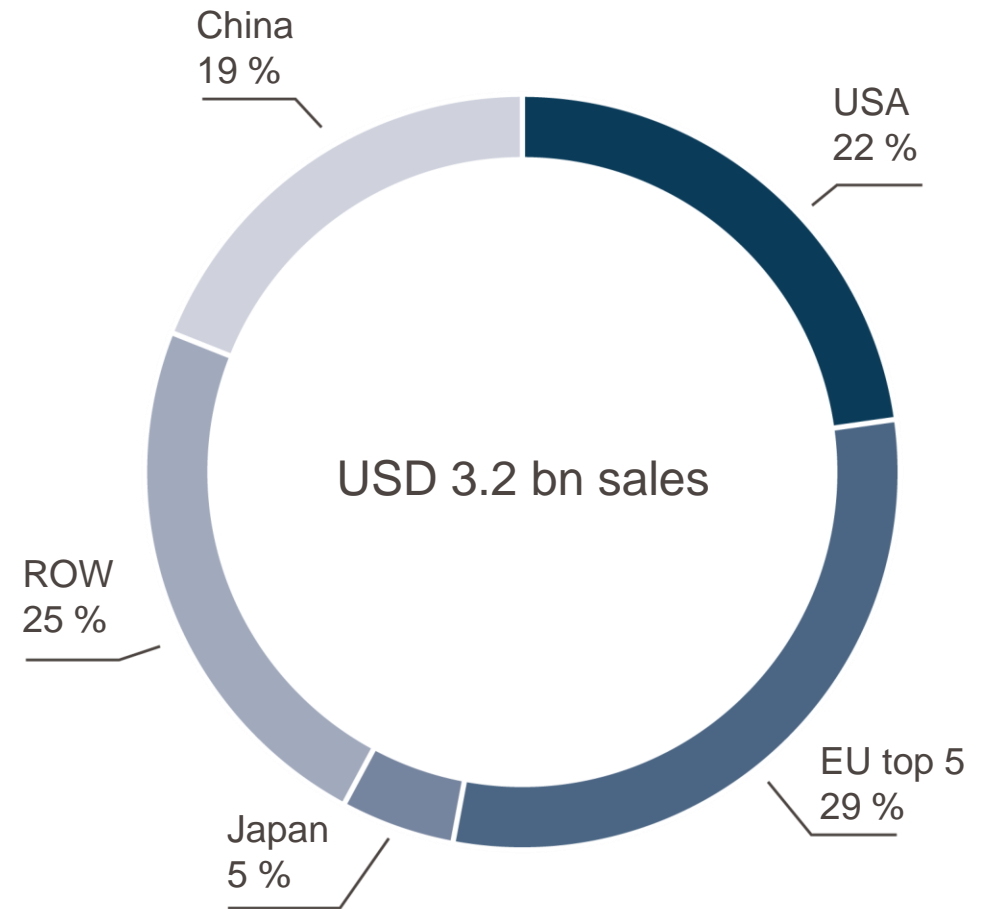


Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

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

USD 3.2 bn sales of best-in-class antifungals* (MAT Q3 2021)

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



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

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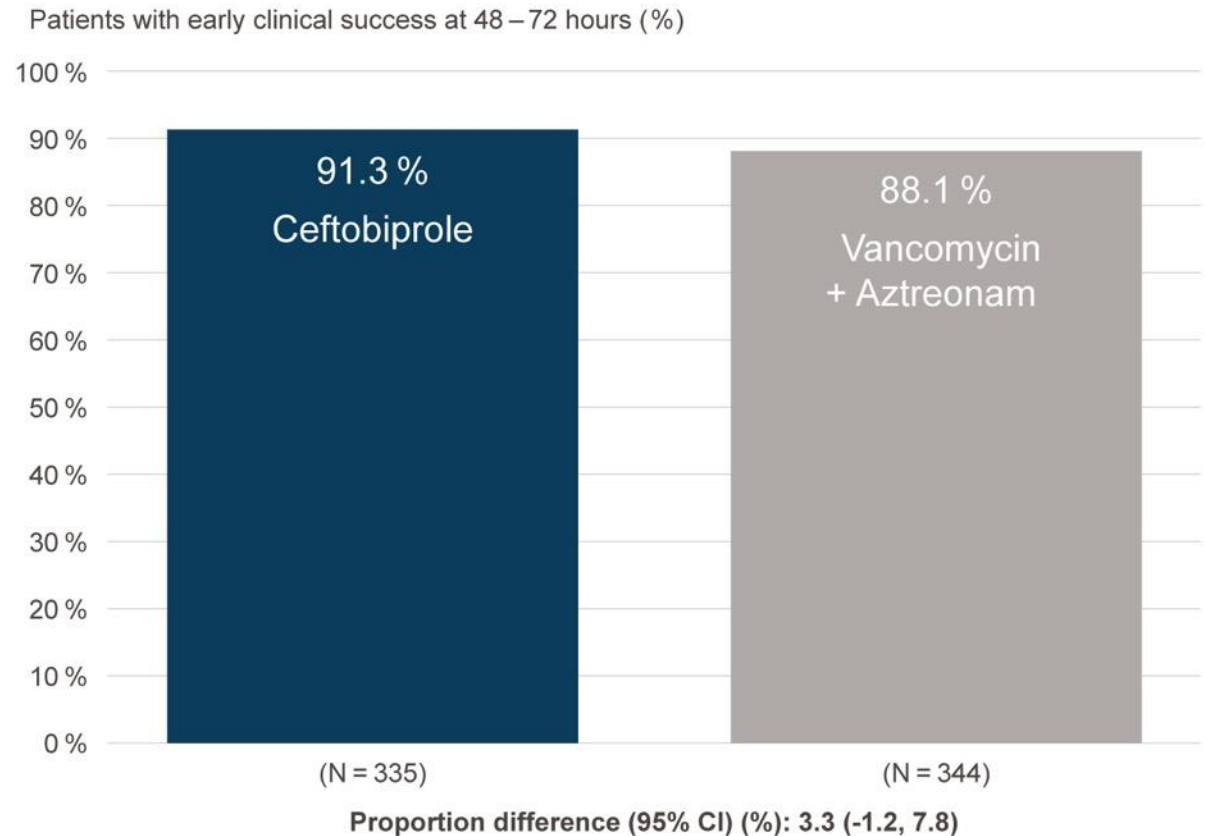
Ceftobiprole — Positive topline phase 3 results reported in ABSSSI

Key topline study¹ results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints



¹ 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 study¹ results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints

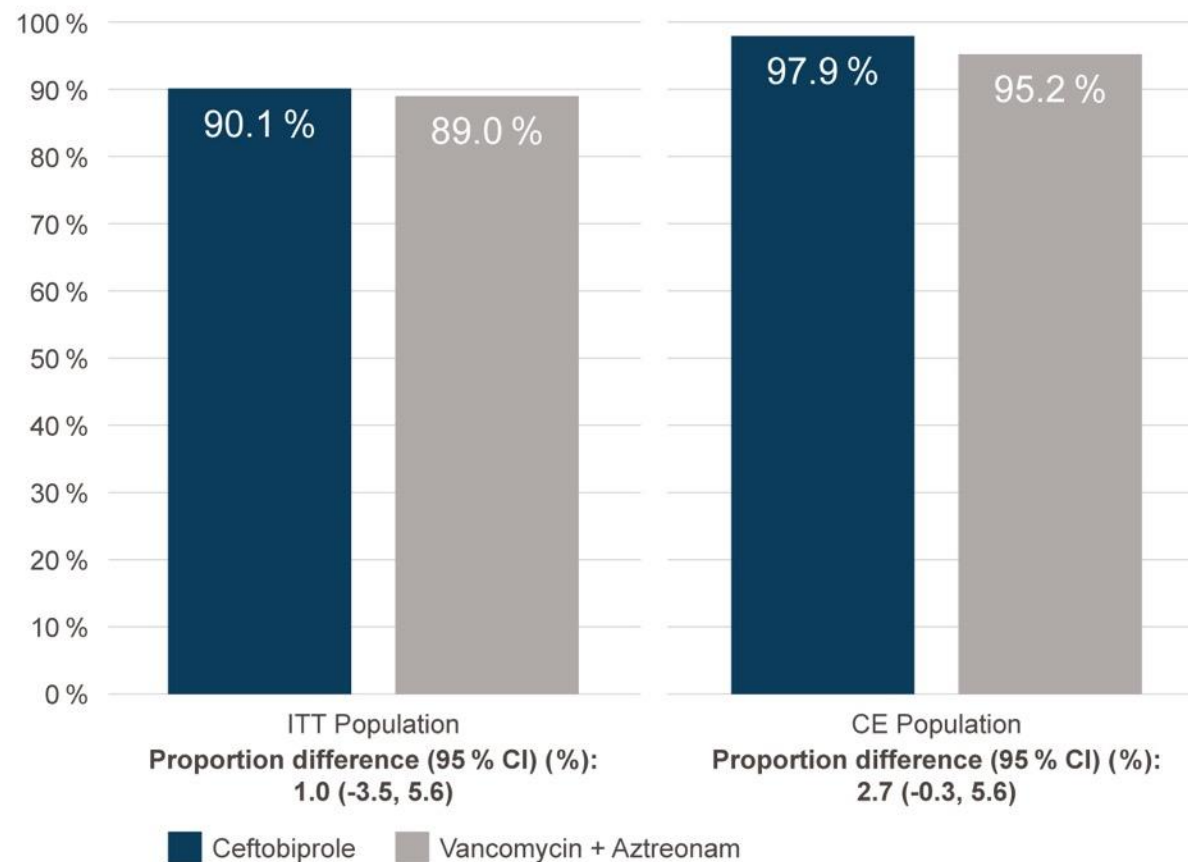


¹ NCT03137173

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 MRSA¹
- Superior activity profile in preclinical models of endocarditis compared to vancomycin and daptomycin²
- Low propensity for resistance development¹
- Gram-negative coverage¹ in cases with polymicrobial infections
- Efficacy demonstrated in Phase 3 clinical trials in pneumonia and complicated skin and soft tissue infections^{1,3,4}
- Established safety profile consistent with the cephalosporin class^{1,3}

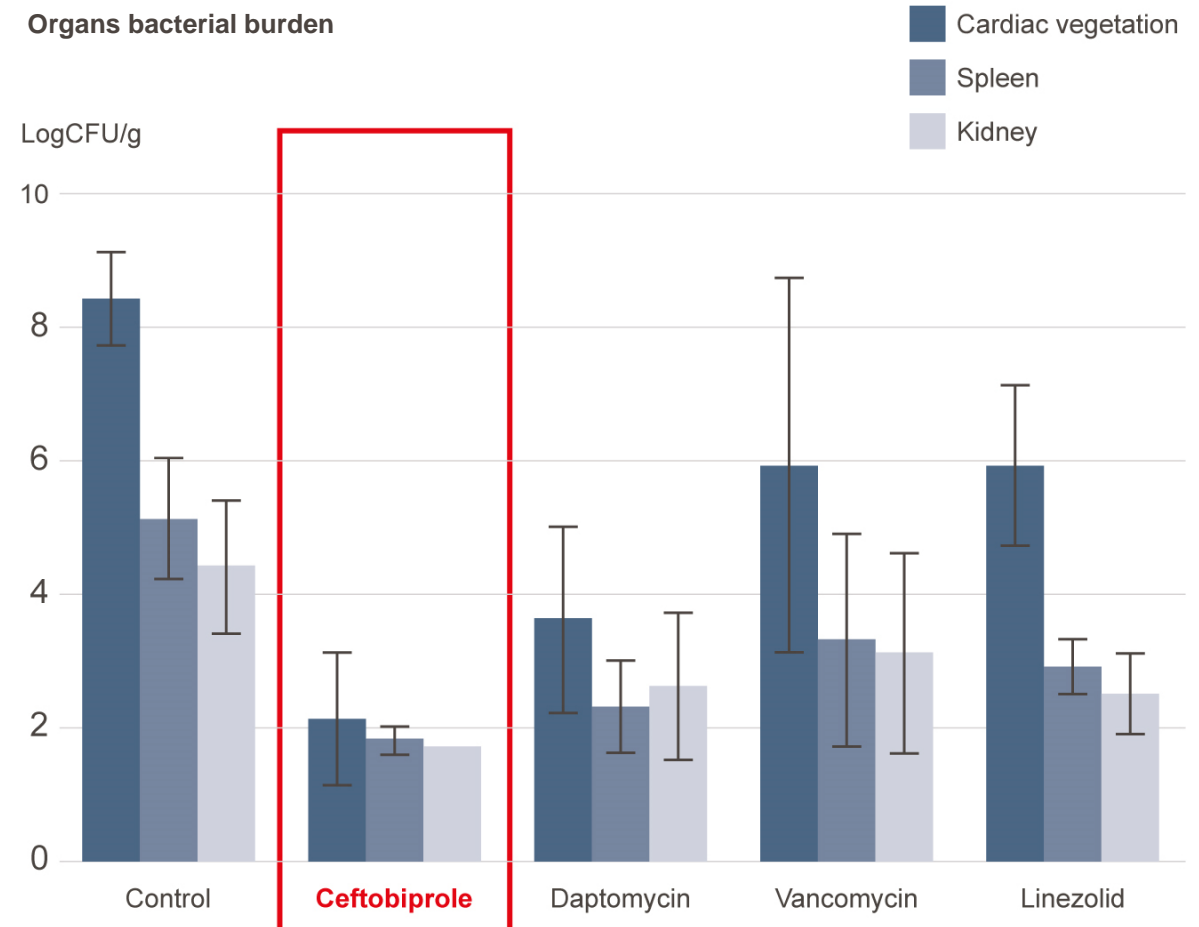
¹Syed YY. *Drugs*. 2014;74:1523-1542.

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

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

⁴Overcash 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 MRSA²

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

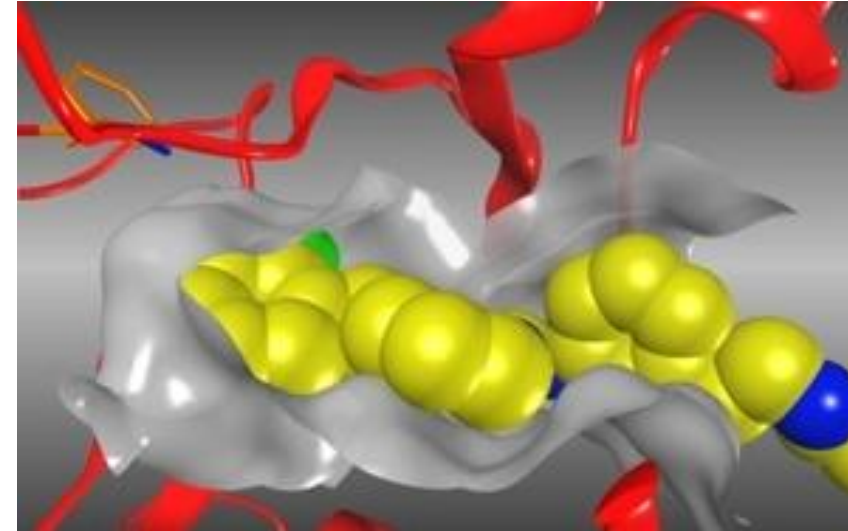
FGFR-inhibitors show differences in kinase-inhibition profiles¹

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

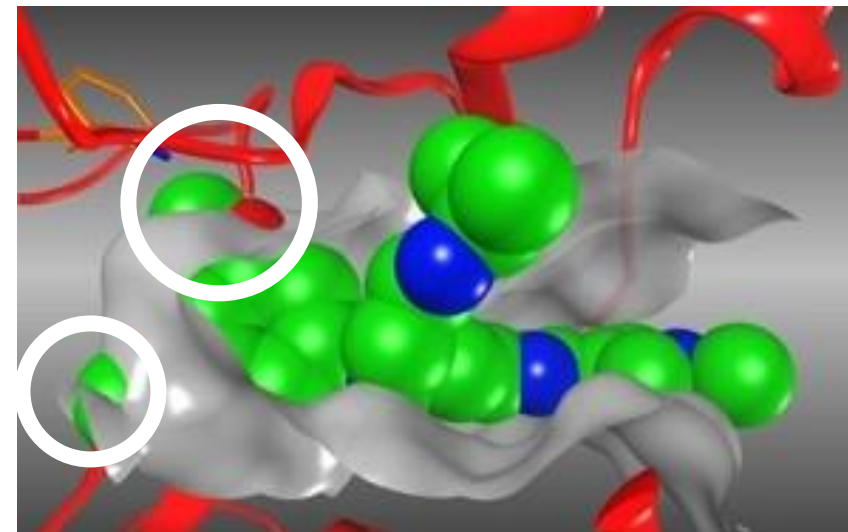
¹ McSheehy et al. Derazantinib (DZB): A dual FGFR/CSF1R-inhibitor active in PDX-models of urothelial cancer. Mol Cancer Ther. 2019 (18) (12 Supplement) LB-C12

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 derazantinib¹



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

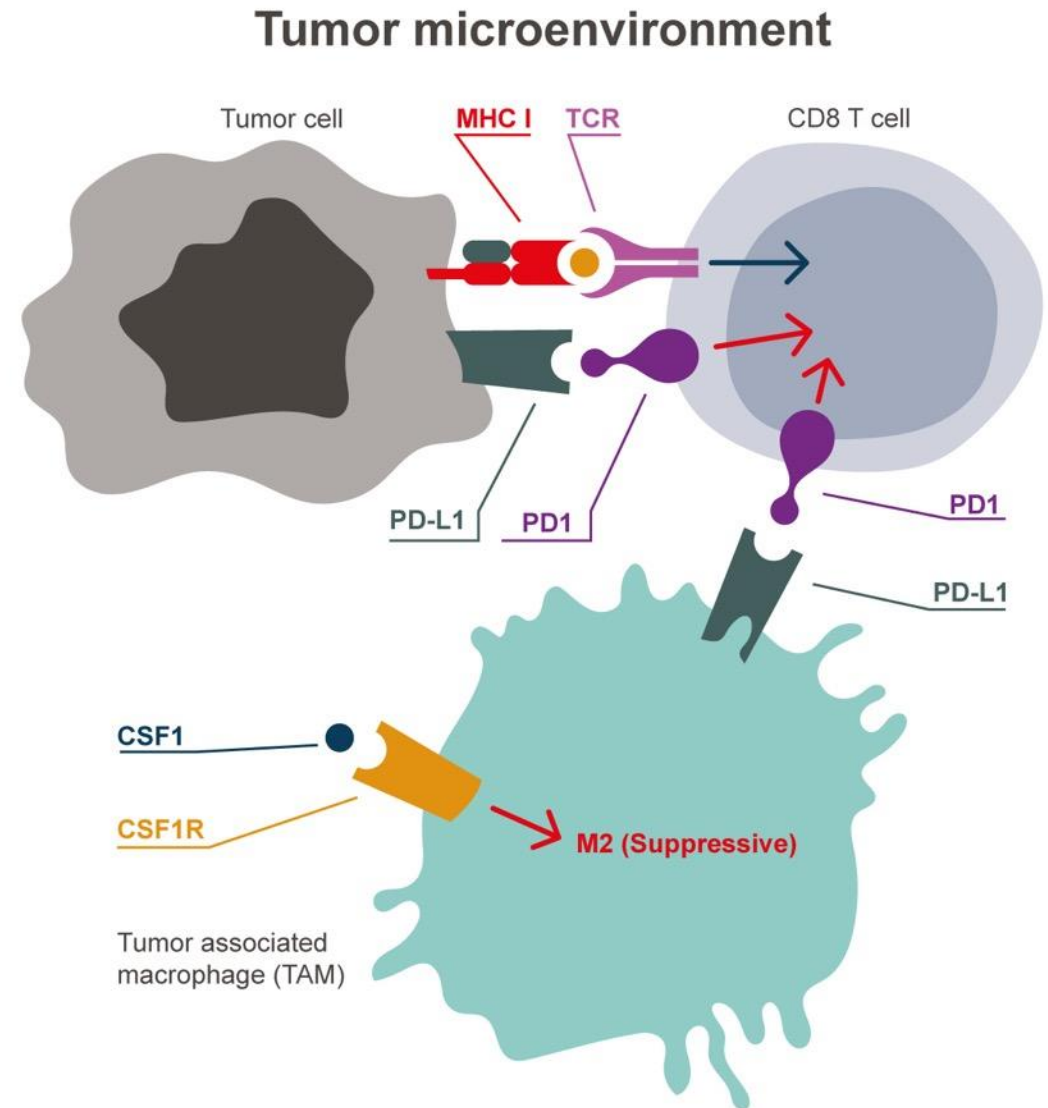


Erdafitinib (green) is too large (white circles) for the active site pocket of CSF1R (grey/red)

¹ 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 inhibitors¹
- 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



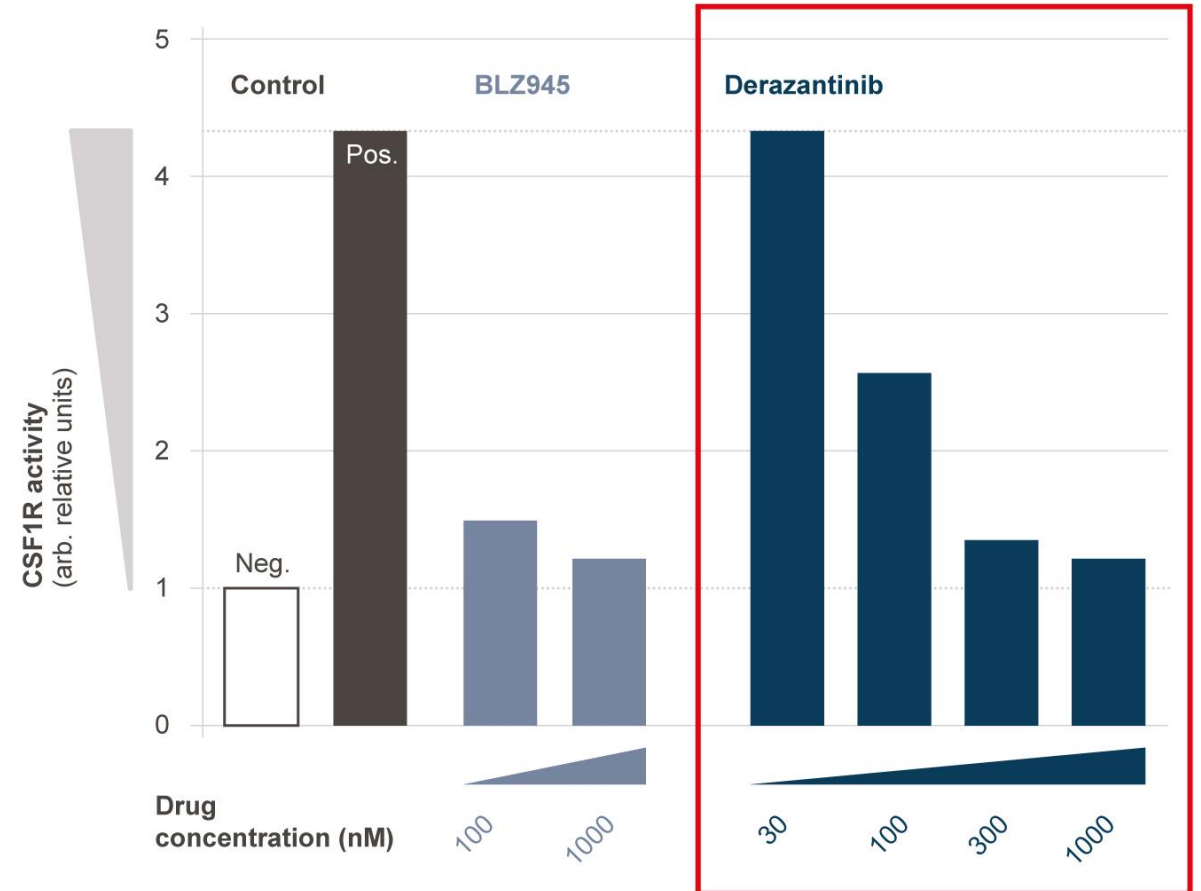
¹ X. Zheng et al. Redirecting tumor-associated macrophages to become tumoricidal effectors as a novel strategy for cancer therapy. *Oncotarget*. 2017;8(29):48436-48452

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

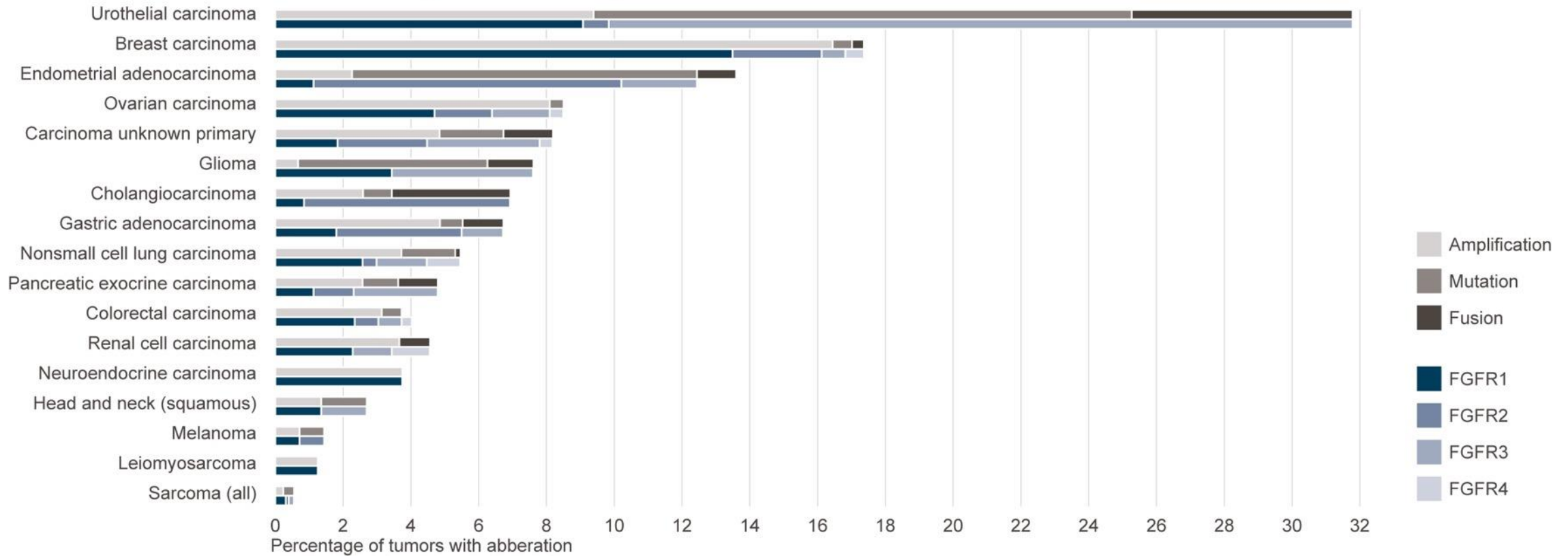


Method: bone-marrow-derived mouse macrophages were starved overnight, treated with CSF1 for 3 min, with or without pre-incubation with BLZ945 or DZB, and then extracted for subsequent immunoblot. The graph shows the quantification of the experiment based on densitometric analysis of the immunoblots

¹ 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

Frequency of currently known FGFR aberrations across tumor types



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

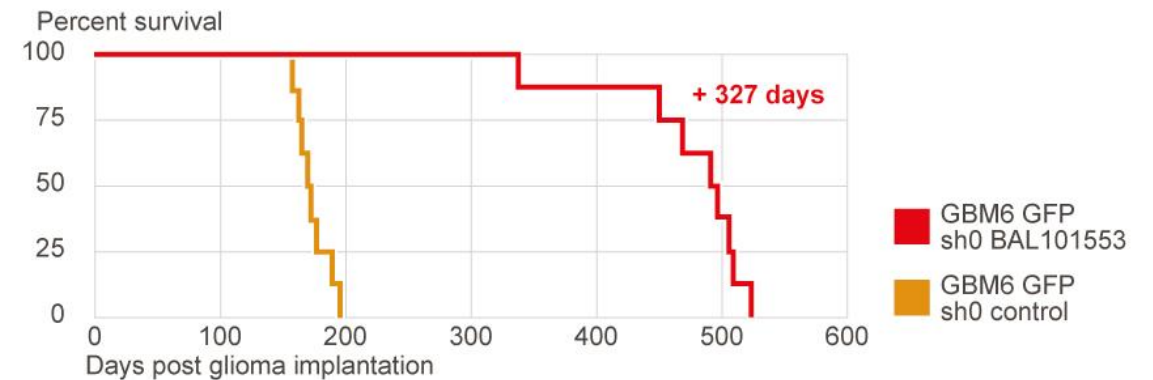
EB1 — A potential response-predictive clinical biomarker for lisavanbulin

- EB1 (plus-end binding protein)¹ is located on the microtubules and involved in microtubule dynamics
- Predictive of response to lisavanbulin in mouse models¹

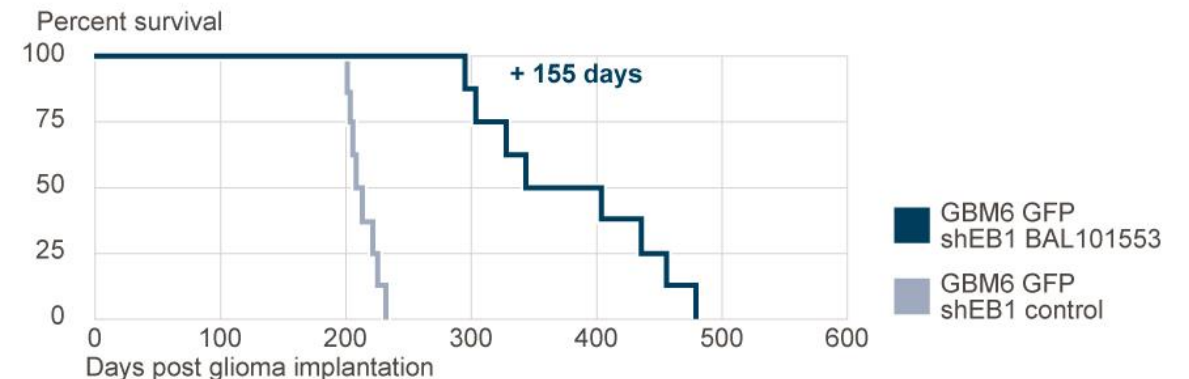
¹ Berges et al. EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after oral treatment with the novel tubulin-binding checkpoint activator BAL101553. Eur. J. Cancer 2018, 103, E61-62, A166

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**ta**p**hylococcus **a**ureus*
- MRSA: **M**ethicillin-**r**esistant ***S**ta**p**hylococcus **a**ureus*
- NDA: **N**ew **d**rug application
- ORR: **O**bjective **r**esponse **r**ate
- PFS: **P**rogression-**f**ree **s**urvival
- SAB: ***S**ta**p**hylococcus **a**ureus* **b**acteremia
- VEGFR2: **V**ascular **e**ndothelial **g**rowth **f**actor receptor **2**

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