



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

David Veitch
CEO

ZKB Swiss Equity Conference 2021
November 03, 2021

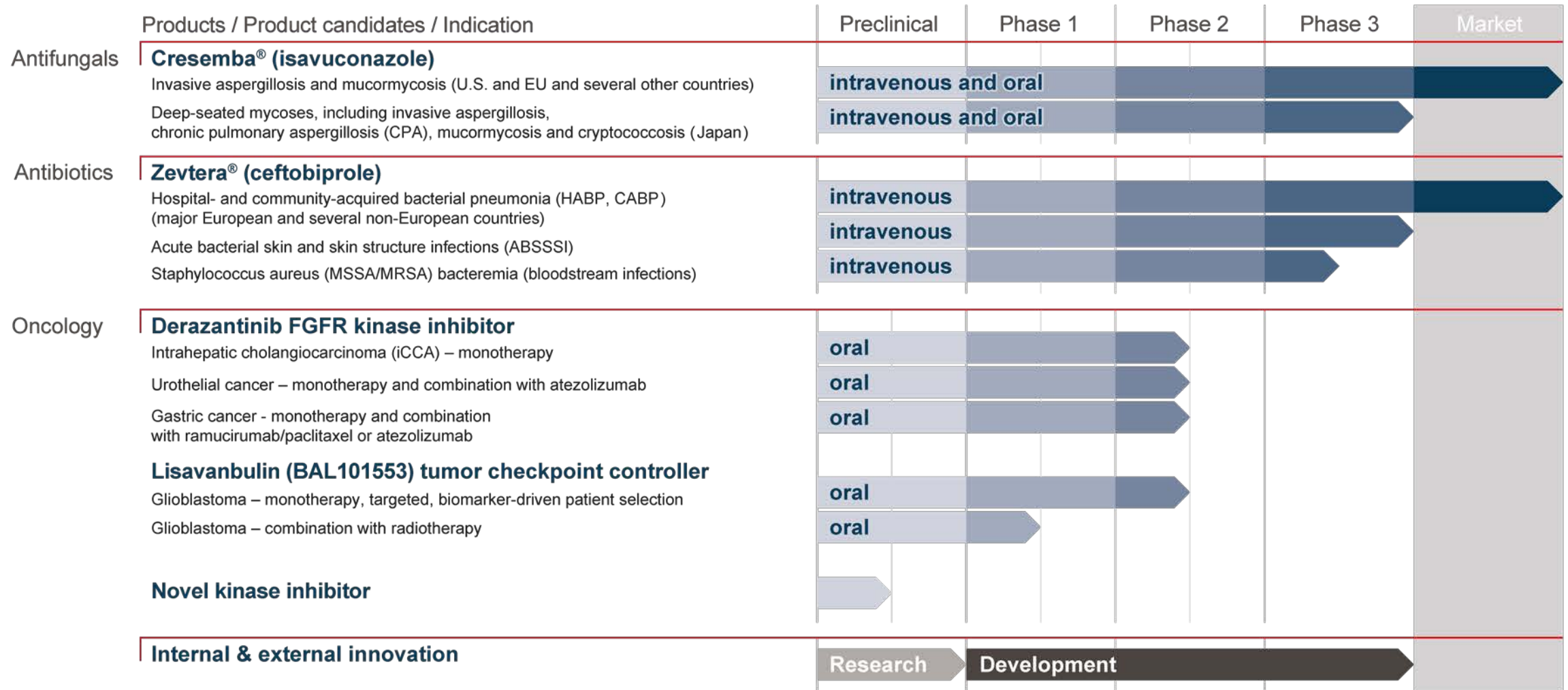


At a glance

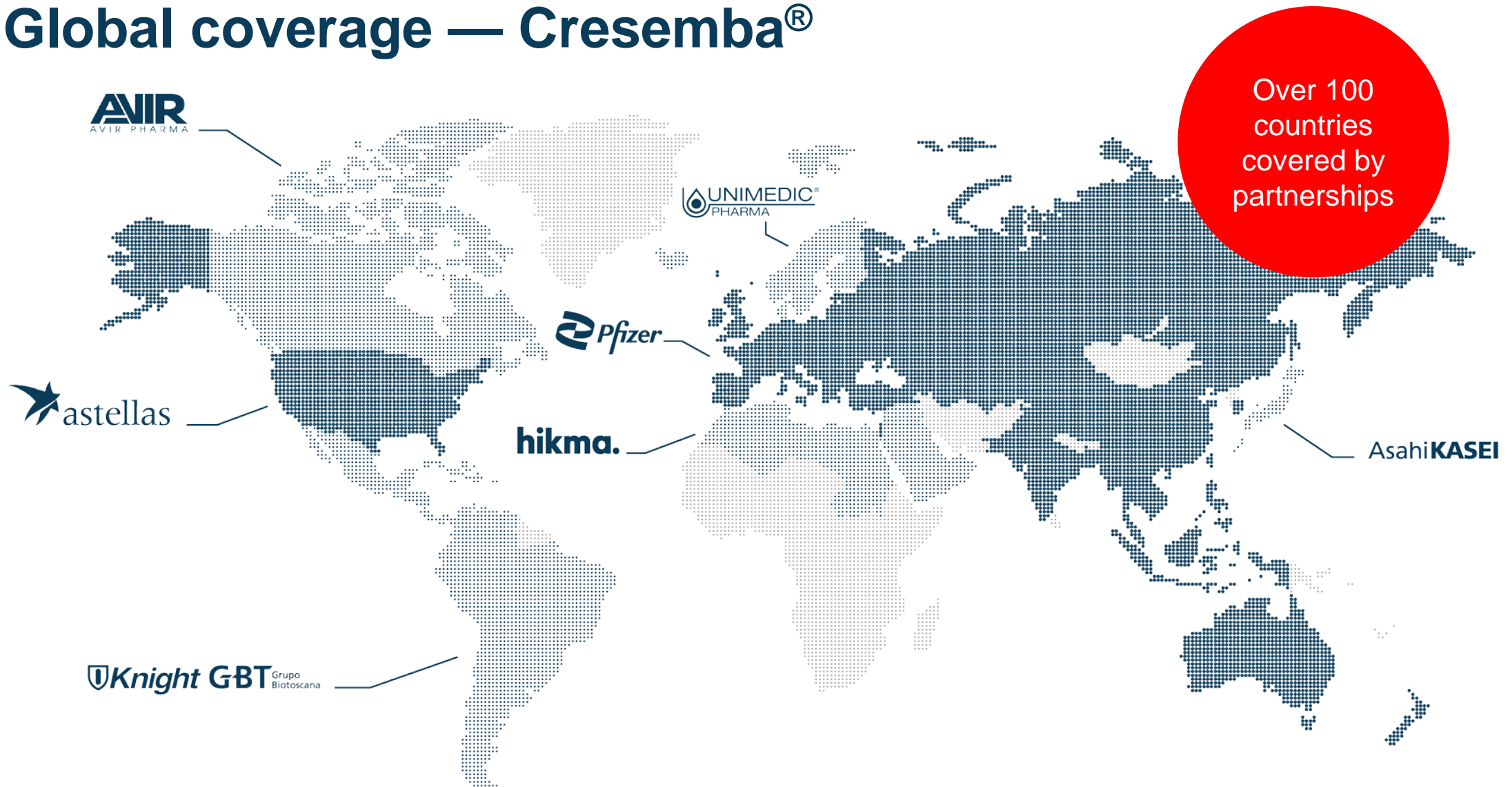
- Well funded, commercial-stage biopharmaceutical company with significantly growing cash flows from commercialized products
- Focused in the areas of oncology and infectious diseases
- Potential for sustainable growth and value creation based on commercialized brands and an innovative pipeline
- Experienced 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



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

Antifungal

**Cresemba®
(isavuconazole)**

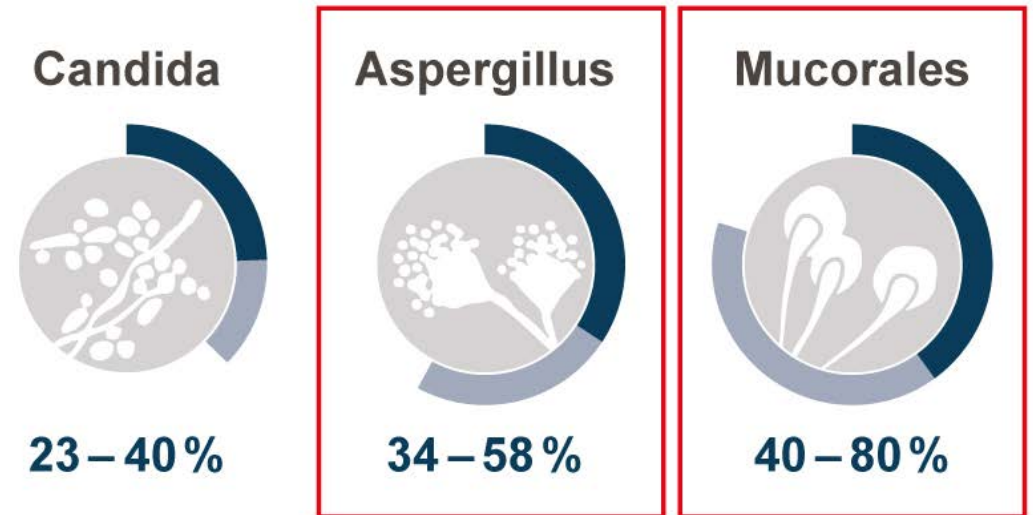
Invasive mold infections



The market — Invasive fungal infections

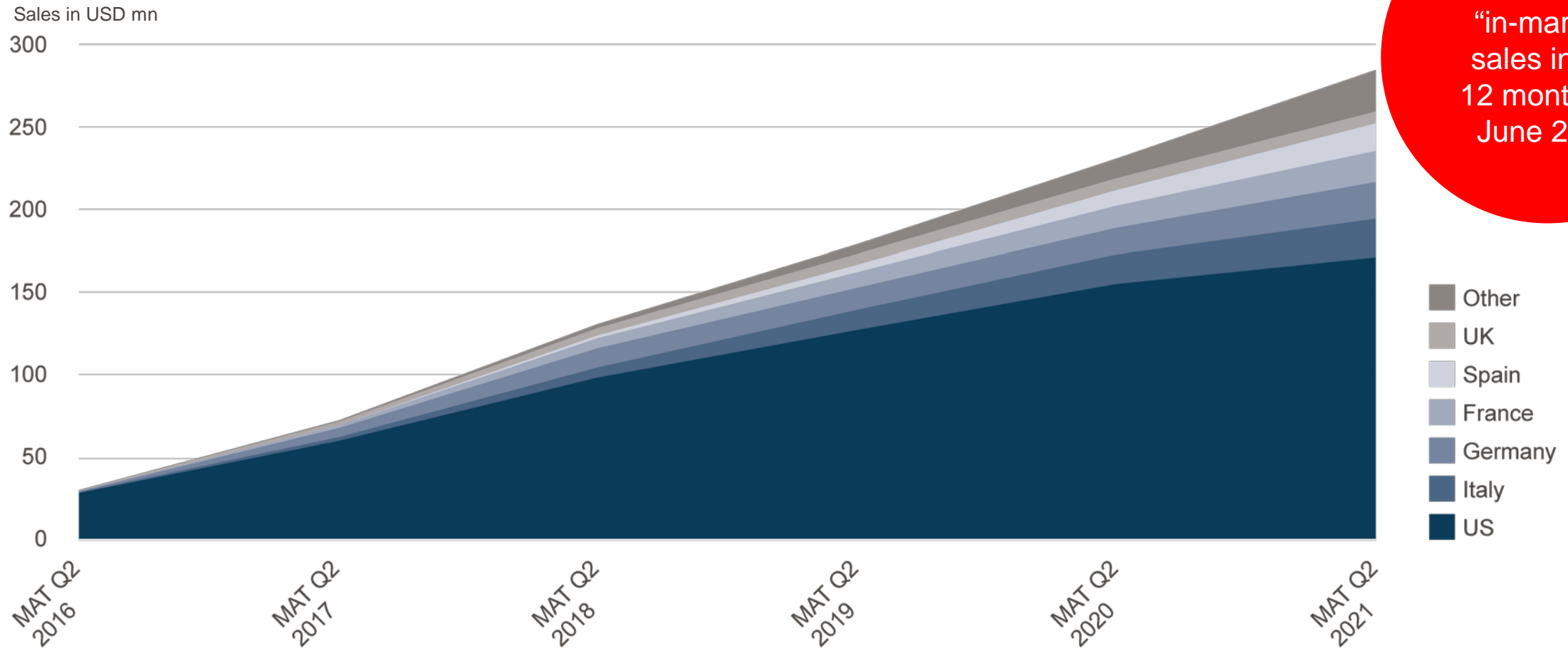
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand
- 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

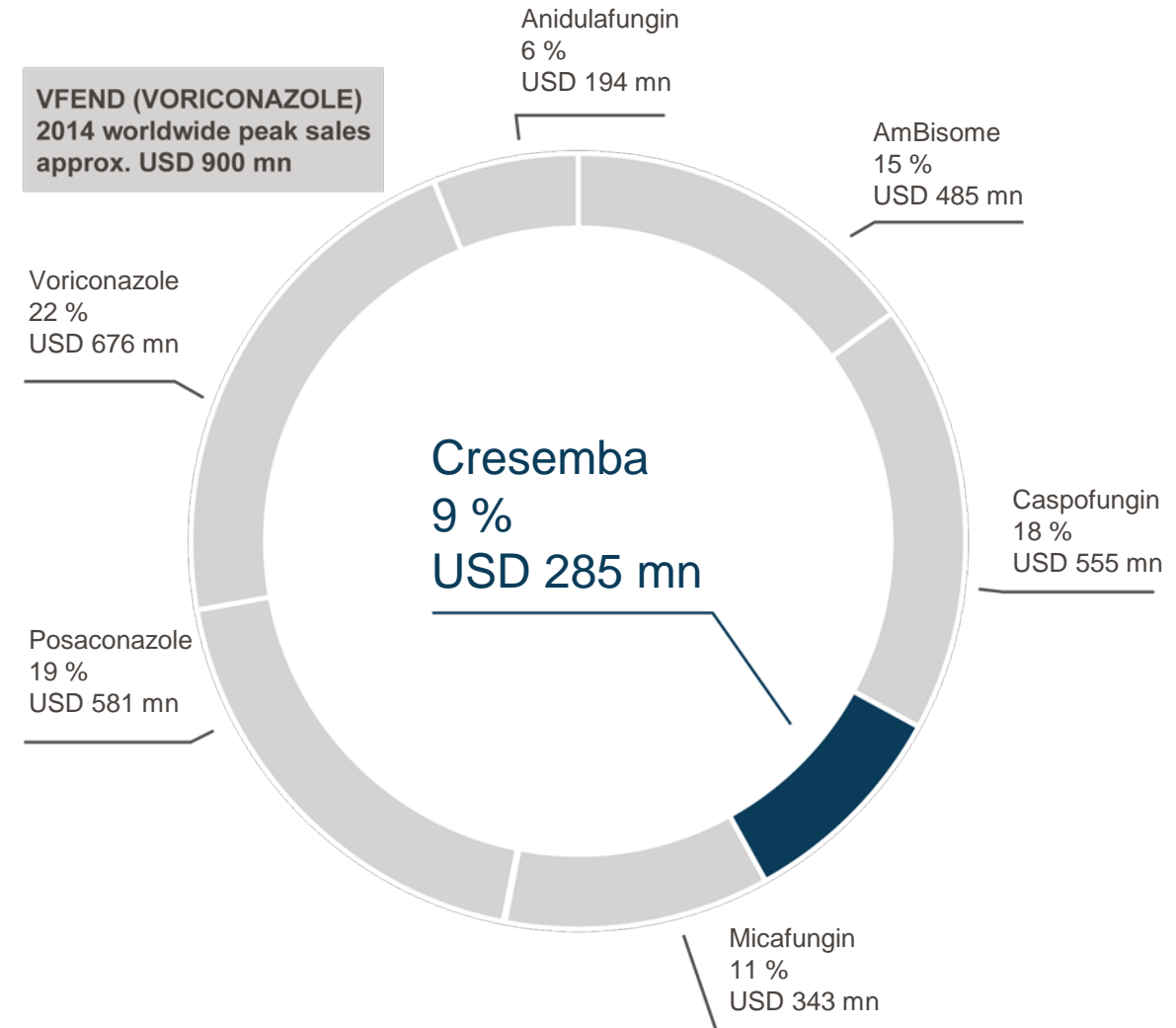
MAT: Moving annual total; Source: IQVIA, June 2021

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

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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

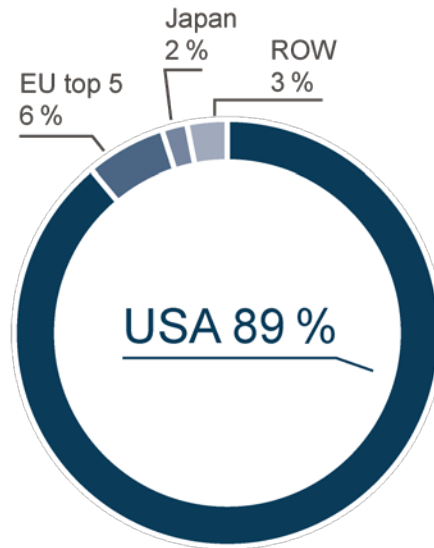
Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP), and community-acquired pneumonia (CAP). Not approved in the U.S.

MENA: Middle East and North Africa

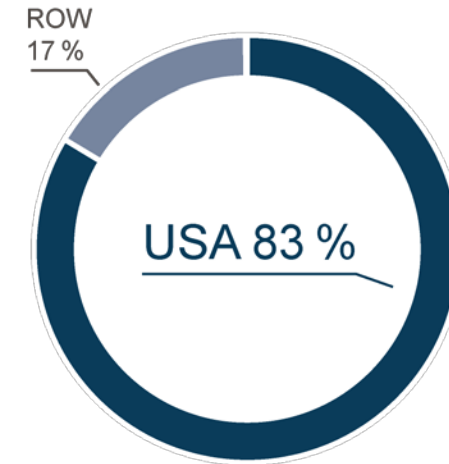


The hospital anti-MRSA antibiotic market — A USD 2.6 bn market* with the U.S. being the most important region

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)¹ successfully completed




2. *Staphylococcus aureus* bacteremia (SAB)² 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

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

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

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.

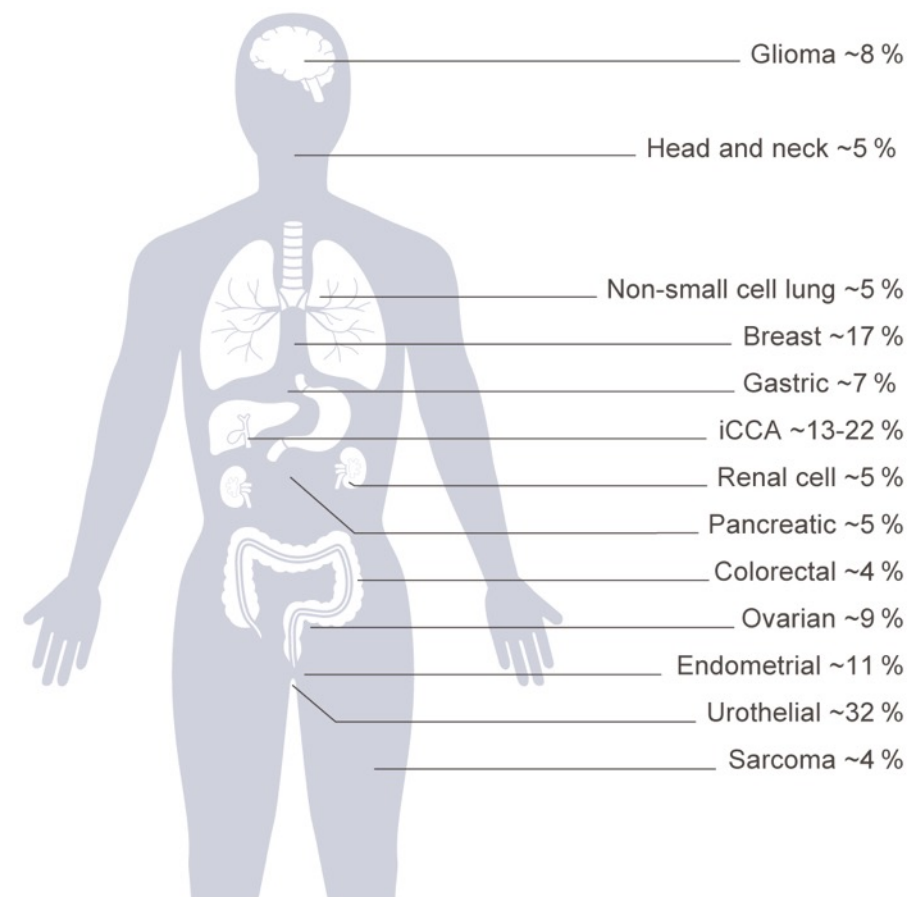
Oncology

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 Pooled ⁵	Pemigatinib ⁶ (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

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

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

⁶ Balversa™ U.S. prescribing information (April 2019)

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

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

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

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

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

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

Oncology

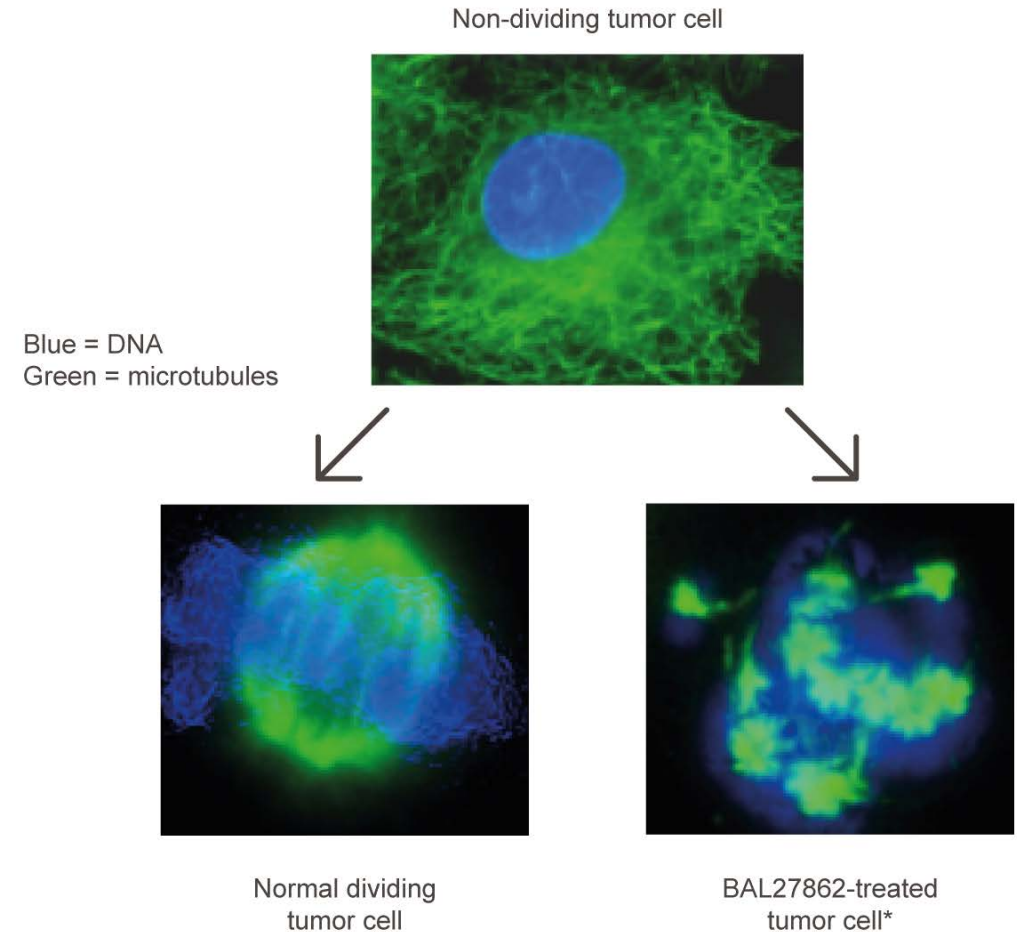
Lisavanbulin (BAL101553)

Glioblastoma
and other solid tumors



Novel tumor checkpoint controller crossing the blood-brain barrier

- Novel compound inducing tumor cell death through spindle assembly checkpoint activation
- Targeting diverse tumor types resistant to standard therapeutic approaches
- Flexible dosing potential, including daily oral dosing
- 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

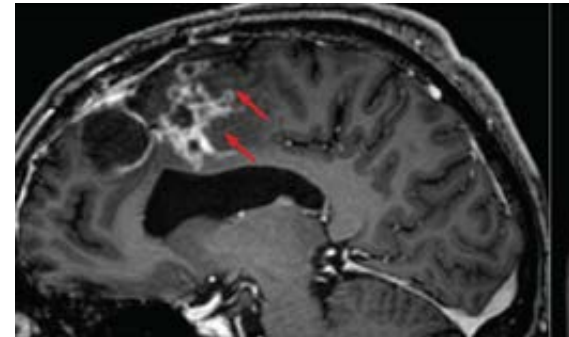


* Lisavanbulin (BAL101553) is a prodrug of BAL27862

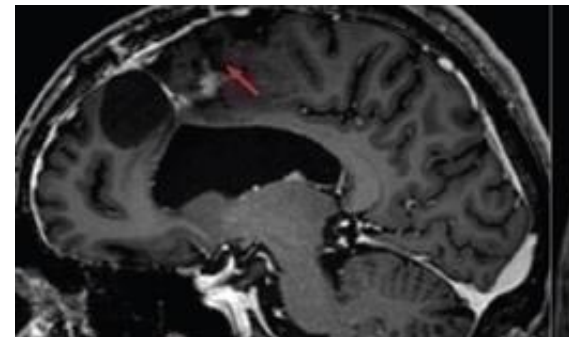
Biomarker-driven phase 2 study ongoing in recurrent glioblastoma

- 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 H2 2021

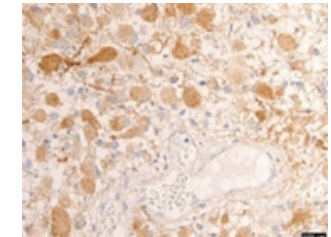
Glioblastoma tumor size reduction in an exceptional responder and EB1 staining of glioblastoma tissue compared to non-responding patients



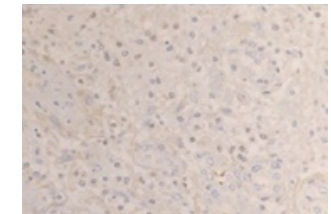
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



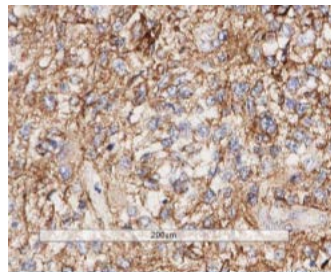
Non-responder

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

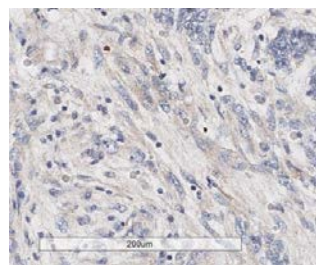
² Tiu et al. JCO 2021;39,15 suppl, TPS2068 (NCT02490800)

EB1-prevalence in glioblastoma and other cancer types

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

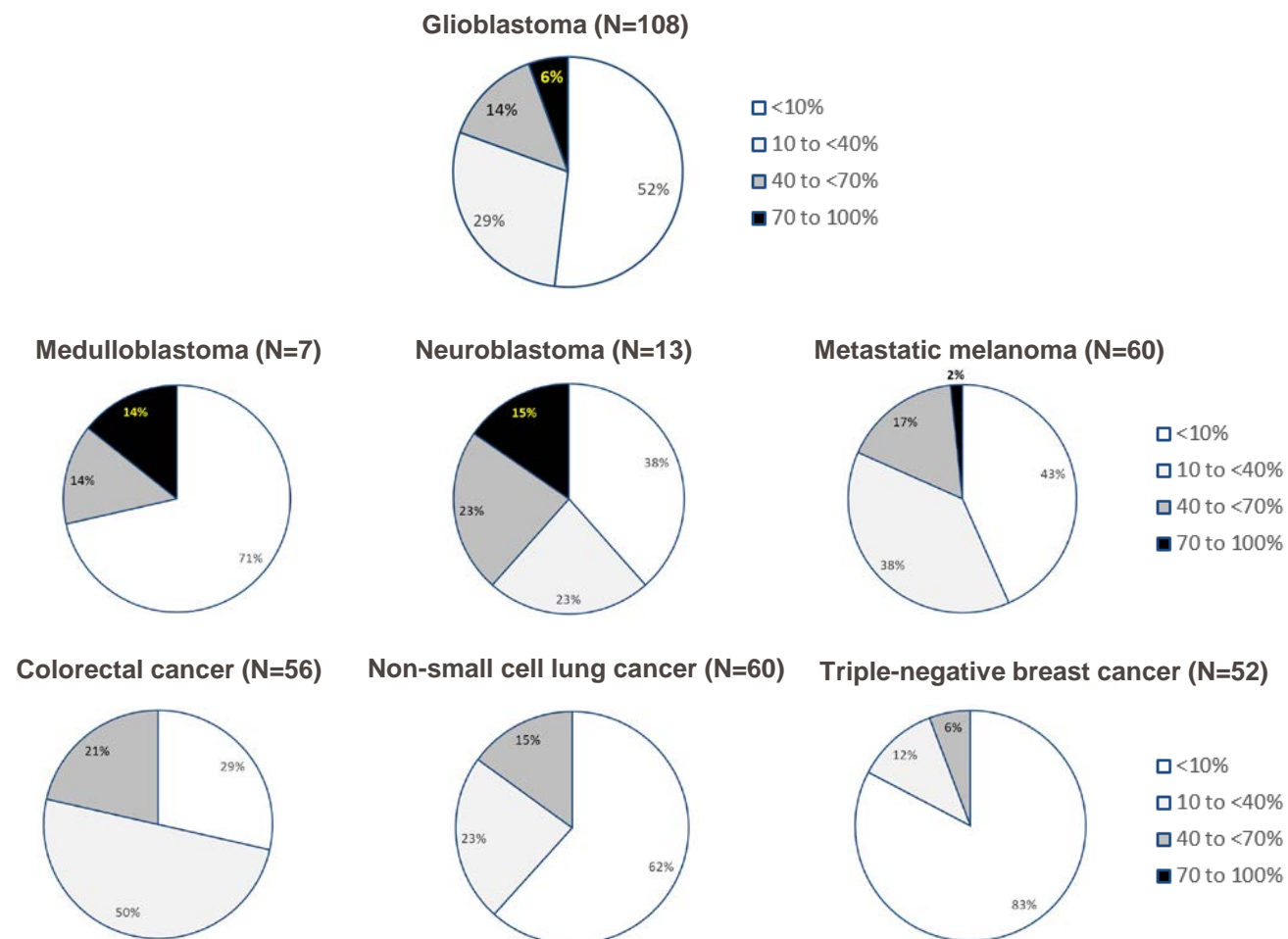


EB1-positive:
Tumor cells show moderate to strong EB1 staining



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.



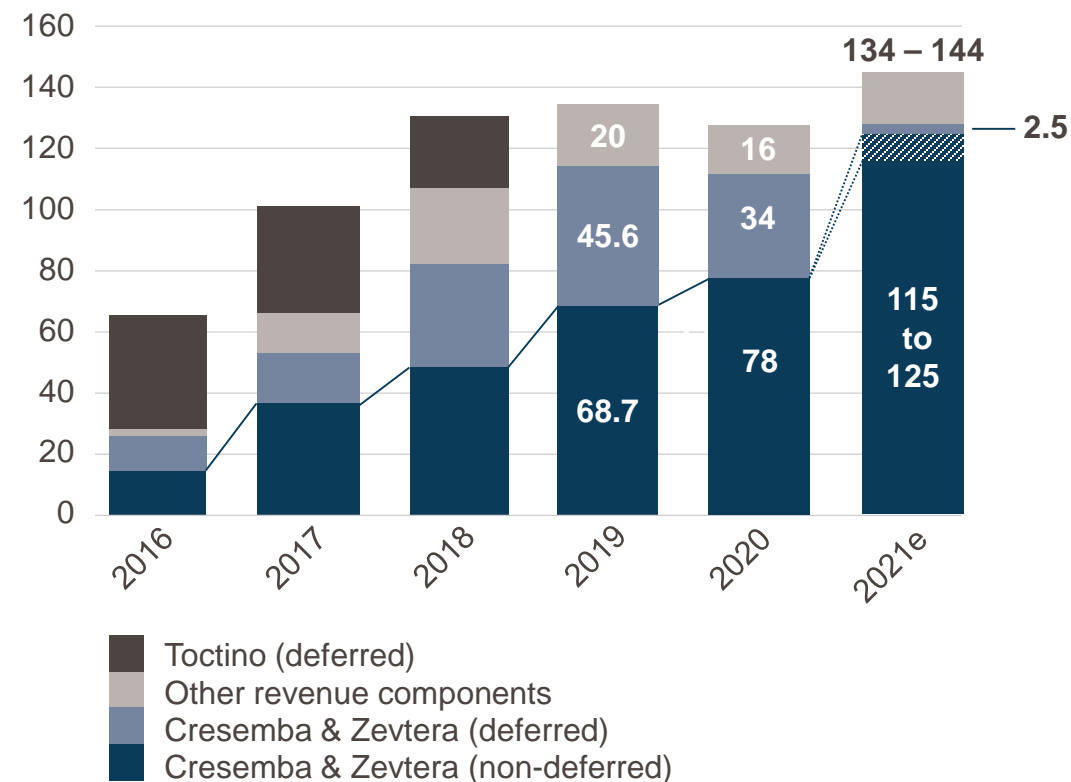
Financials & Outlook



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
Isavuconazole		✓ Complete patient enrolment in phase 3 study in Japan	✓ File NDA 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		
Novel kinase inhibitor (for cancer therapy)			File IND application	Initiate phase 1 study	

Disclaimer and forward-looking statements

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Focused on Growth and Innovation

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