



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

**David Veitch  
CEO**

UBS Global Healthcare Virtual Conference  
May 26, 2021

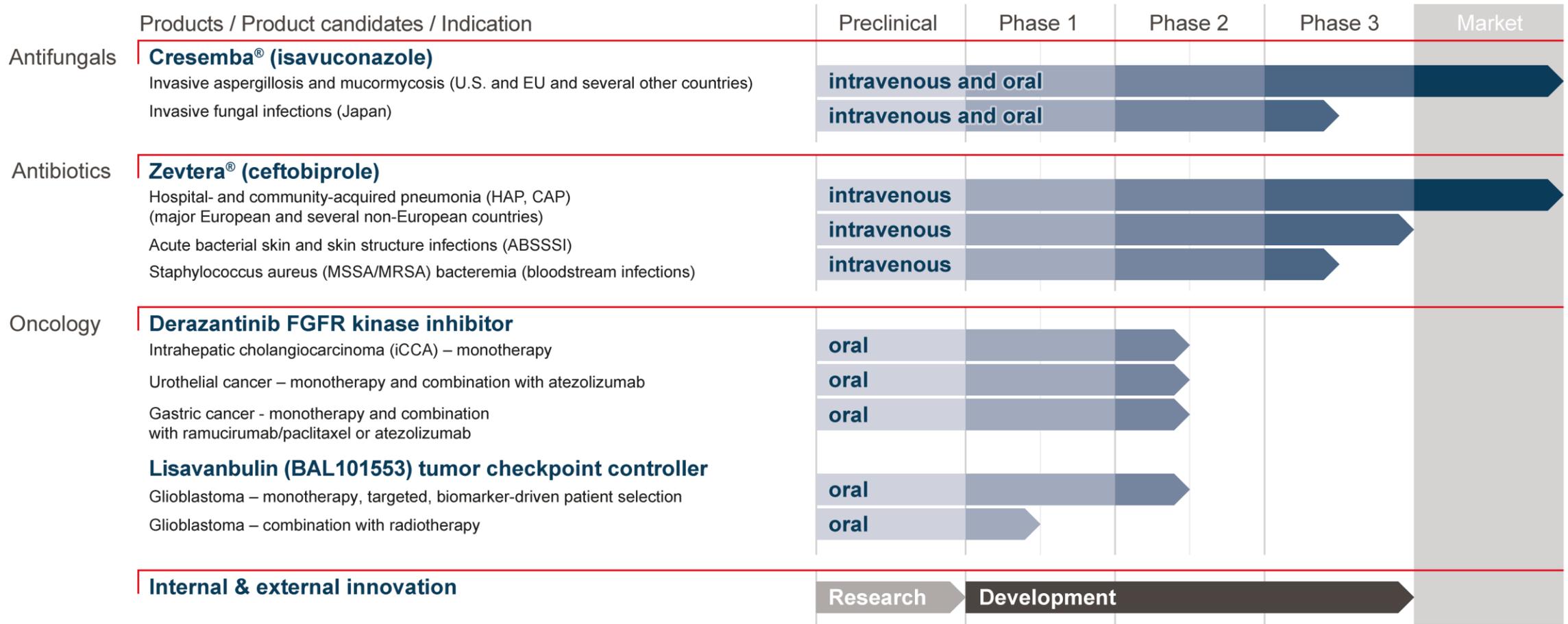


# At a glance

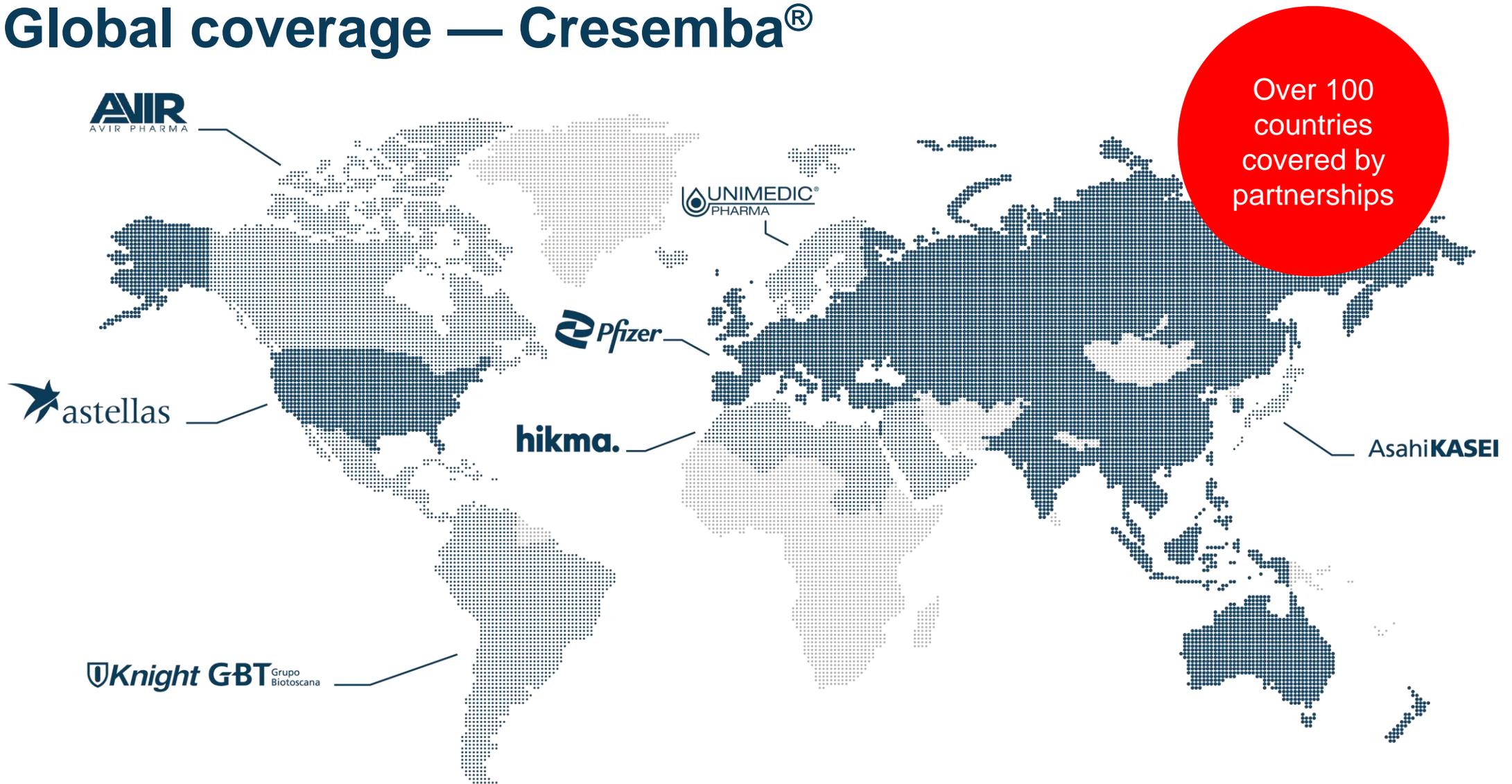
- Well funded, commercial-stage biopharmaceutical company with significantly growing cash flows from commercialized products
- Focused in the areas of oncology and infectious diseases
- Potential for sustainable growth and value creation based on commercialized brands and an innovative pipeline
- Experienced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



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®)

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)**

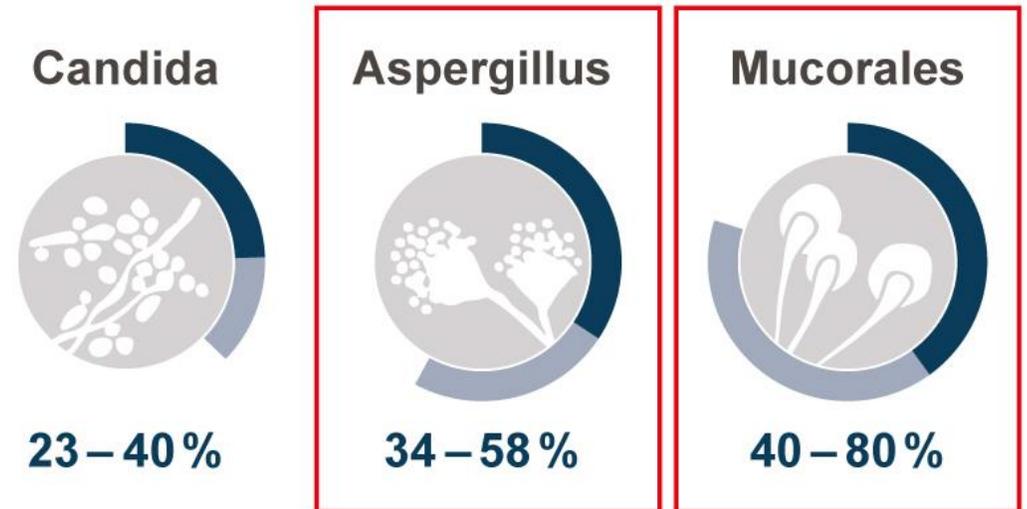
Invasive mold infections



# The market — Invasive fungal infections

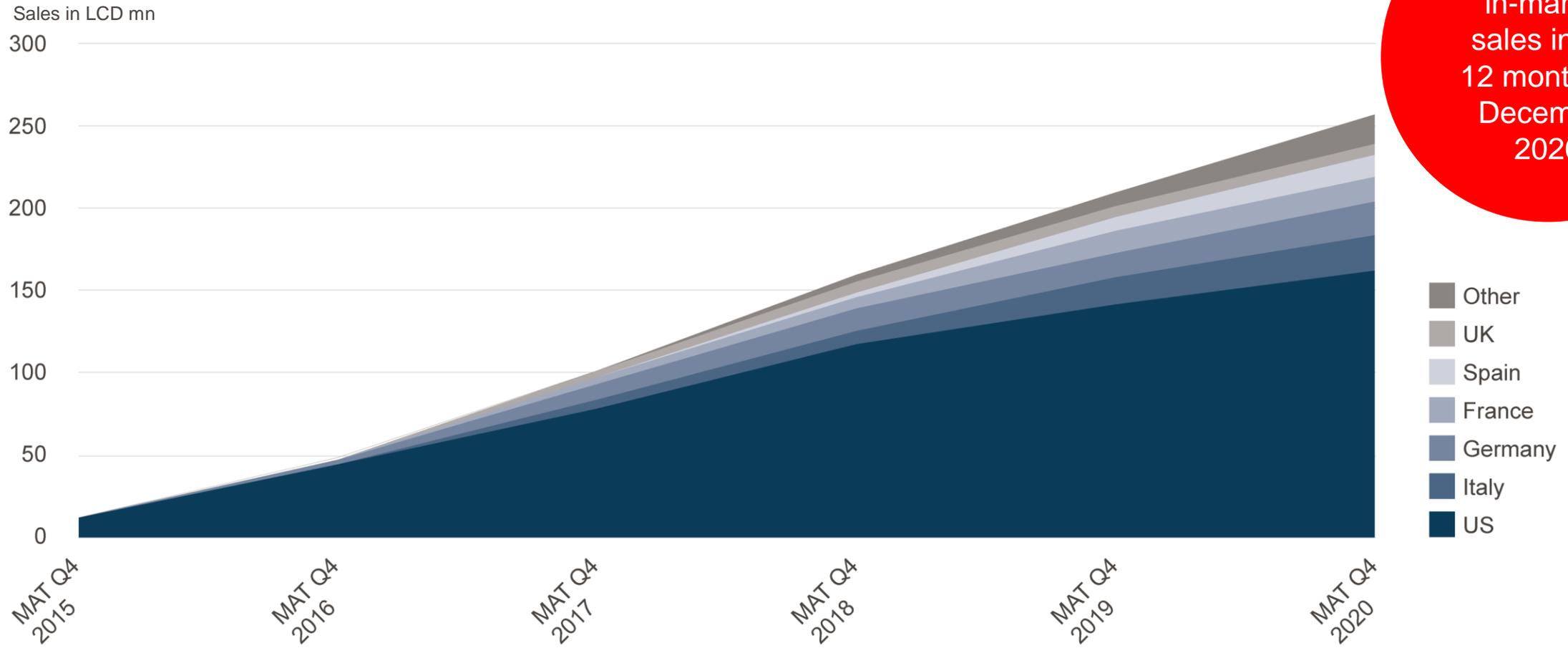
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand
- 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 256 mn  
 "in-market"  
 sales in the  
 12 months to  
 December  
 2020

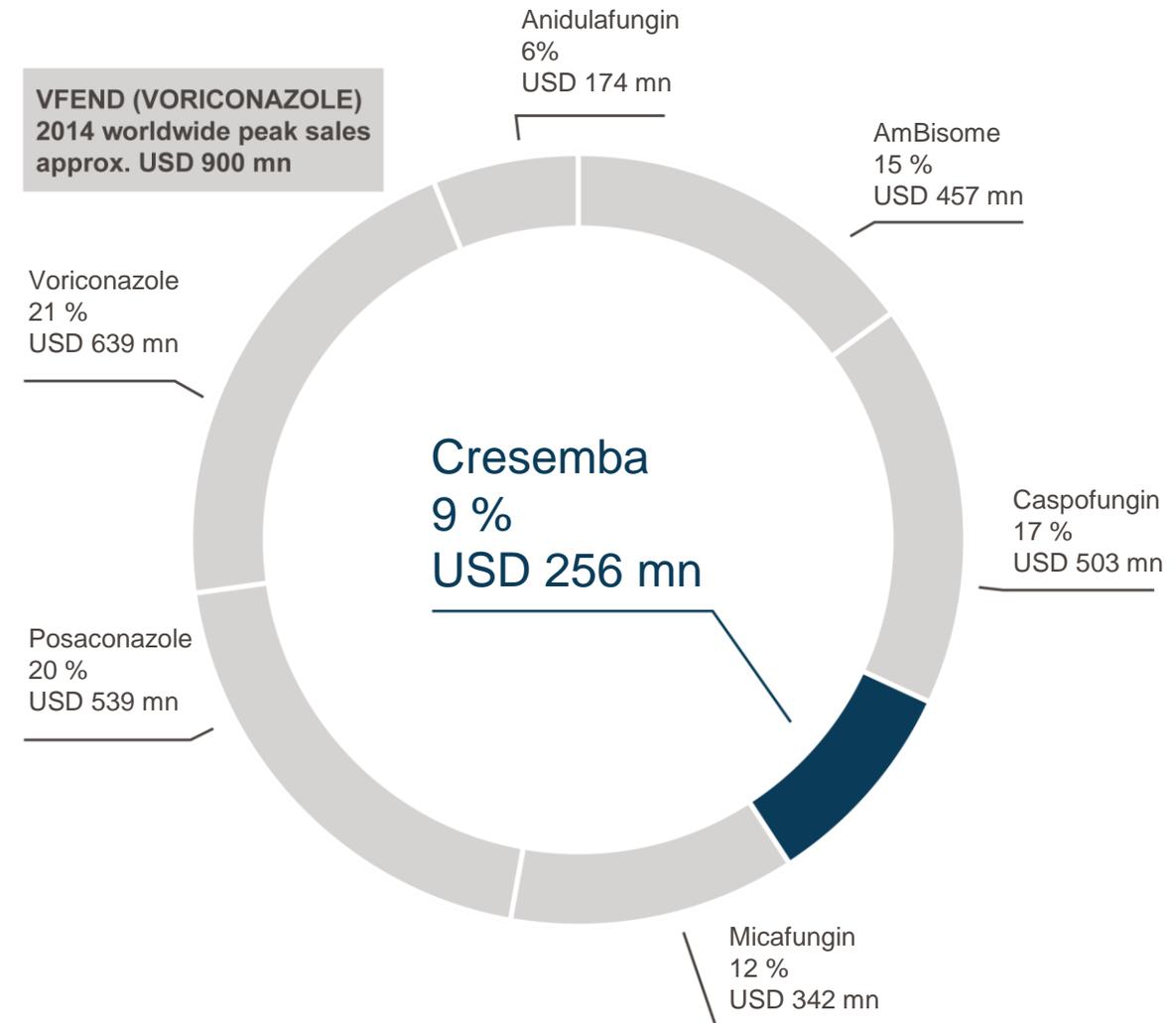
LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, December 2020

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

USD 3.0 bn sales (MAT Q4 2020)

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

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



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

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

Severe bacterial infections



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

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

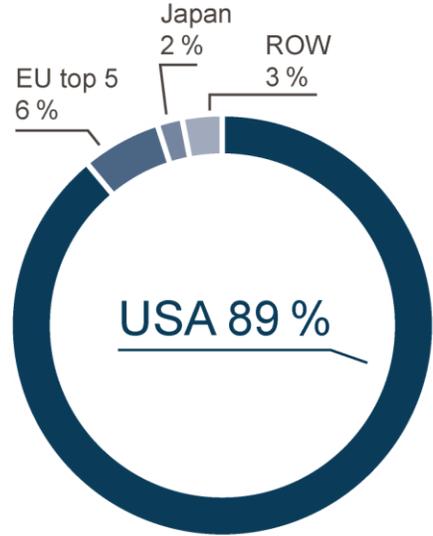
Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP), and community-acquired pneumonia (CAP). Not approved in the U.S.

MENA: Middle East and North Africa

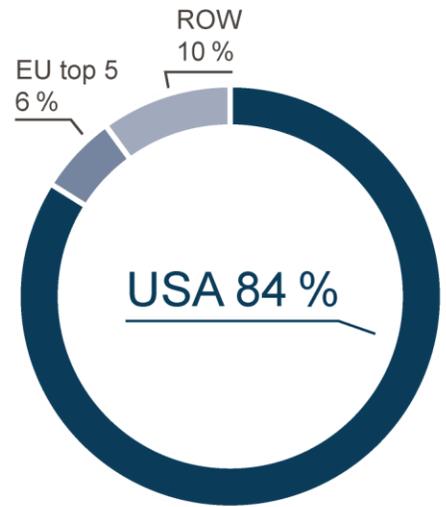


# The hospital anti-MRSA antibiotic market — A USD 2.6 bn market\* with the U.S. being the most important region

Daptomycin sales by region (2015, before LOE)



Ceftaroline sales by region (MAT Q4 2020)



\* Vancomycin, linezolid, teicoplanin, daptomycin, tigecycline, telavancin, ceftaroline, dalbavancin, oritavancin, and tedizolid

MRSA: Methicillin-resistant *Staphylococcus aureus*; LOE: Loss of exclusivity; ROW: Rest of world  
 MAT: Moving annual total; Sales figures in USD, corrected for currency fluctuations; Source: IQVIA, December 2020

# Strategy for accessing the U.S. market

- Two cross-supportive phase 3 studies under FDA Special Protocol Assessment (SPA)
- Phase 3 program largely funded by BARDA (up to USD ~130 mn, ~70% of total program costs)

1. Acute Bacterial Skin and Skin Structure Infections (ABSSSI)<sup>1</sup> successfully completed



2. *Staphylococcus aureus* bacteremia (SAB)<sup>2</sup> ongoing, topline results from phase 3 study expected in H1 2022



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

<sup>1</sup> Overcash JS et al. ECCMID 2020, abstract 1594. (NCT03137173)

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

A microscopic view of cells, likely cancer cells, with a strong orange tint. The cells are spherical and have numerous thin, hair-like projections extending from their surfaces. The background is a dense field of similar cells, creating a textured, cellular appearance.

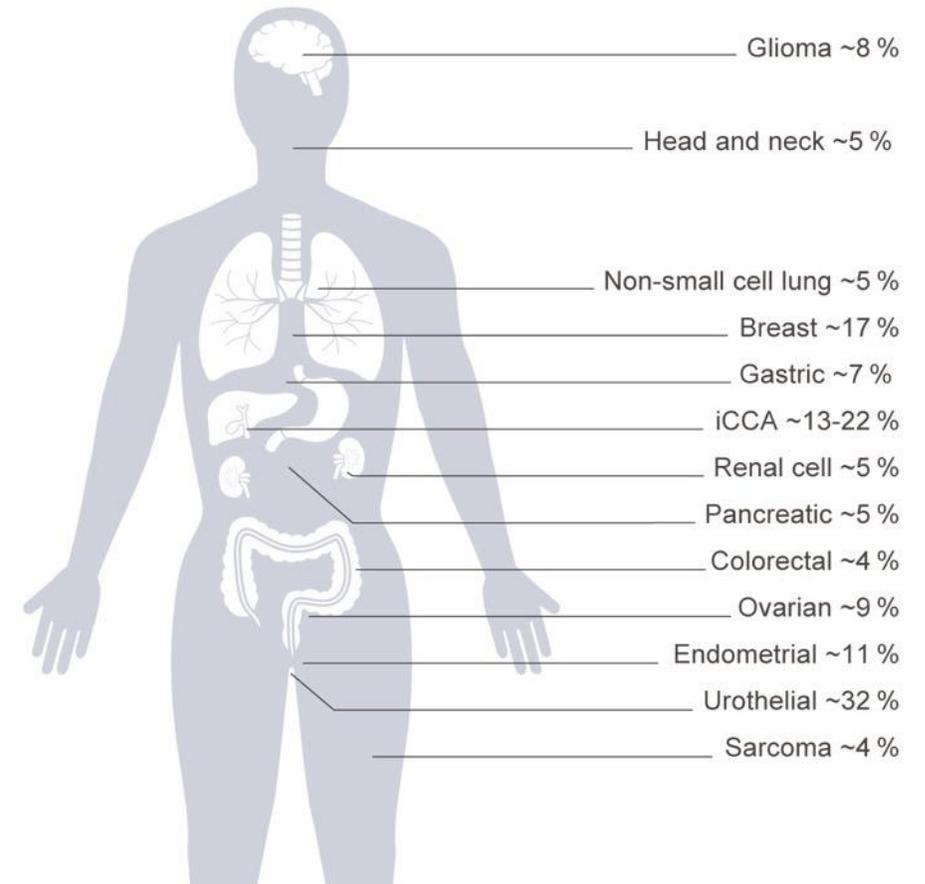
Oncology

# Derazantinib

FGFR-driven tumors

# Targeting FGFR-driven tumors as single agent and in combination with immunotherapy

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



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

# 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: 20.4%  
Median PFS: 6.6 months

- Consistent with earlier Phase1/2 data<sup>1</sup> and with interim analysis from FIDES-01
- Clinical proof of concept for derazantinib as monotherapy in its first indication established

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

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

Pooled data from 23 patients  
(clinical studies/EAP)<sup>2</sup>  
Median PFS 7.2 months

**Interim results (n=14):**  
DCR: 79% (1 confirmed CR, 1 unconfirmed PR, 9 SD)

- 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

# Clinical program in urothelial cancer – FIDES-02

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

- Substudies (N≈300) in various treatment settings, including:
  - Post-chemotherapy/immunotherapy recurrence (second-line and post second-line)
  - First-line platinum-ineligible, PD-L1-low
  - Resistance to prior FGFR-inhibitor treatment
- Successful completion of phase 1b cohort
  - Recommended phase 2 dose for the combination established
  - No dose-limiting toxicities observed
- Clinical supply agreement with Roche for atezolizumab
- Plan to amend the study protocol to explore a higher dose of derazantinib in two cohorts of this study
  - Supported by the observed safety and tolerability profile of derazantinib at the current dose of 300 mg per day
  - May provide additional benefits in monotherapy and combination to patients with FGFR-positive urothelial cancer
  - Considers the evolving treatment and competitive landscape in urothelial cancer in patients both with and without FGFR genetic aberrations
- Interim results in derazantinib monotherapy expected H1 2021
- Interim results in combination therapy with atezolizumab expected H2 2021

# 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
- Clinical supply agreement with Roche for atezolizumab
- Clinical trial collaboration and supply agreement with Lilly for ramucirumab
- Interim results in derazantinib monotherapy and recommended phase 2 dose of derazantinib in combination with ramucirumab/paclitaxel expected H2 2021

# 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 <sup>↑</sup> Nausea Vomiting	Phosphorus <sup>↑</sup> Fatigue Stomatitis	Phosphorus* <sup>↑</sup> Diarrhea* Dry mouth*	Phosphorus <sup>↑</sup> Alopecia Diarrhoea	Diarrhoea Alopecia Constipation	Phosphorus <sup>↑</sup> Stomatitis Fatigue
Blood phosphorus <sup>↑†</sup>	59%	73%	88%	60%	31%	76%
Fatigue <sup>†</sup>	43%	49%	NR	42%	32%	54% <sup>#</sup>
Alopecia <sup>†</sup>	20%	38%	NR	49%	40%	26%
Dry eye/xerophthalmia <sup>†</sup>	16%	32%	NR	35% <sup>#</sup>	NR	28% <sup>#</sup>
Retinopathy <sup>†</sup>	0%	NR	9%	6% <sup>‡</sup>	NR	25%
Alanine aminotransferase (ALT) <sup>↑</sup>	30% <sup>**</sup>	NR	NR	43% <sup>**</sup>	NR	41% <sup>**</sup>
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)

<sup>†</sup> 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);

<sup>†</sup> 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;

<sup>\*\*</sup> 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

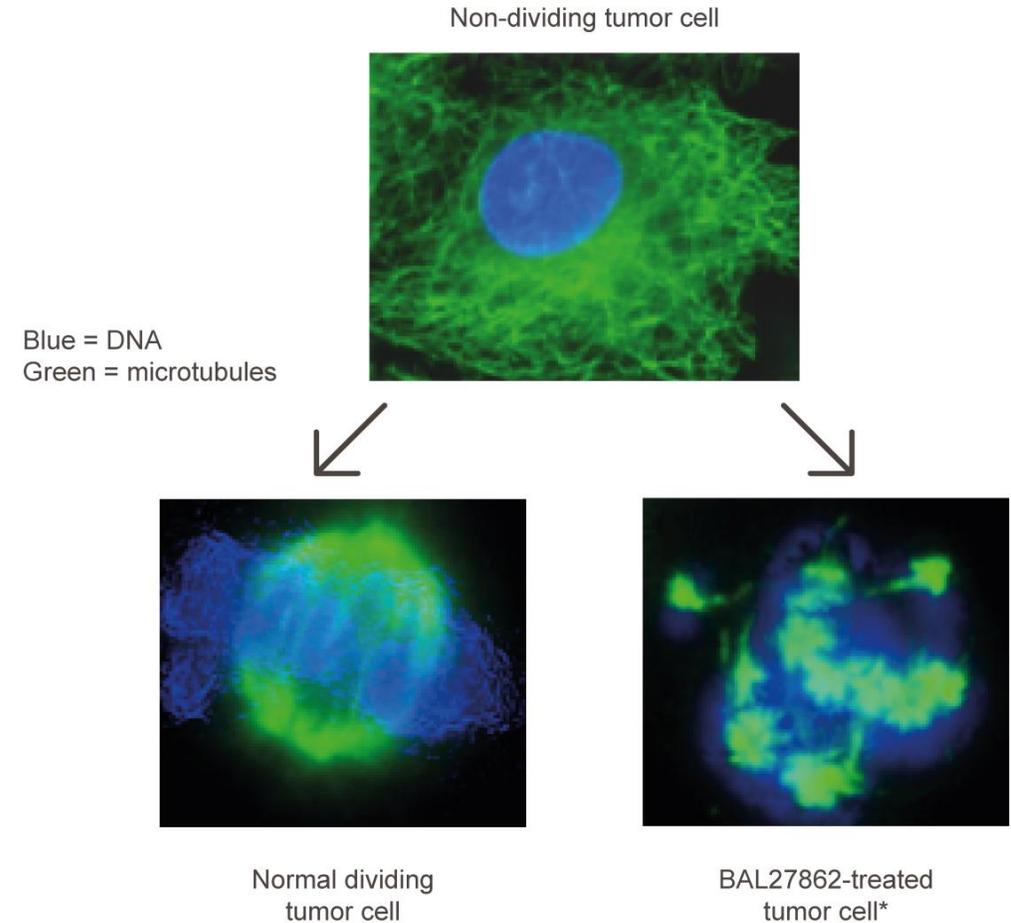
# Lisavanbulin (BAL101553)

Glioblastoma  
and other solid tumors



# Novel tumor checkpoint controller crossing the blood-brain barrier

- Novel compound inducing tumor cell death through spindle assembly checkpoint activation
- Targeting diverse tumor types resistant to standard therapeutic approaches
- Flexible dosing potential, including daily oral dosing
- Comprehensive biomarker program to optimize patient selection
- Crosses the blood-brain barrier with potent activity in brain tumor models alone and in combination



\* Lisavanbulin (BAL101553) is a prodrug of BAL27862

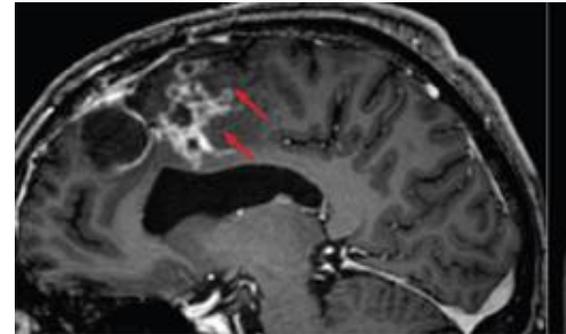
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# Biomarker-driven phase 2 study ongoing in recurrent glioblastoma

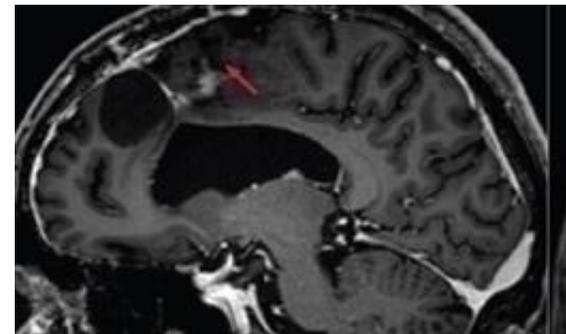
- EB1 (end-binding protein 1) is located on the microtubules and involved in microtubule dynamics and has been shown to be a response predictive marker for lisavanbulin in preclinical studies
- Strong EB1 staining was observed in a patient with an exceptional response to daily oral lisavanbulin in the phase 1 dose-escalation study in recurrent glioblastoma<sup>1</sup>
  - Patient ongoing for more than two years
  - >80% reduction in glioblastoma tumor size
- Interim results expected H2 2021

<sup>1</sup> Lopez et al. Phase 1/2a study of once daily oral BAL101553, a novel tumor checkpoint controller, in adult patients with progressive or recurrent glioblastoma or high-grade glioma. JCO 2019;37:15 suppl, 2025 (NCT02490800)

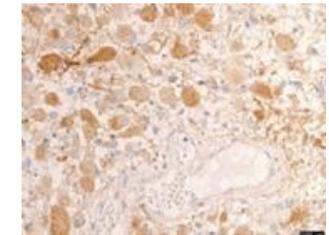
## Glioblastoma tumor size reduction in an exceptional responder and EB1 staining of glioblastoma tissue compared to non-responding patients



Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



Non-responder



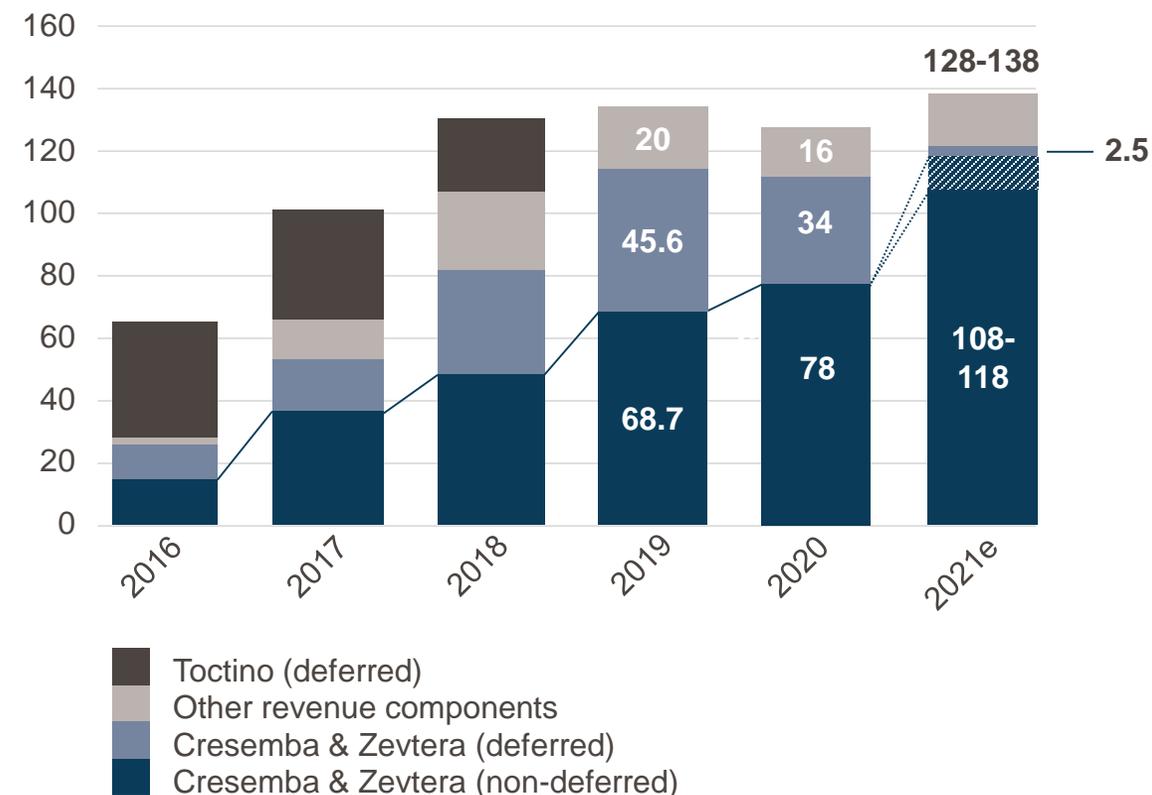
# Financials & Outlook



# Financial guidance

In CHF mn	FY 2020	FY 2021 guidance
Total revenue	127.6	128 - 138
thereof: Contributions Cresemba® & Zevtera® non-deferred	78.2	108 - 118
deferred	33.8	2.5
Operating loss	8.2	13 - 23
Cash and investments*	167.3	155 – 160**

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 potential impact from a reduction of the outstanding convertible bonds

# Milestones & Outlook 2021 / 2022

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

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

# Disclaimer and forward-looking statements

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**Focused on Growth and Innovation**

**Grenzacherstrasse 487  
PO Box  
4005 Basel  
Switzerland**

**[investor\\_relations@basilea.com](mailto:investor_relations@basilea.com)  
[www.basilea.com](http://www.basilea.com)**

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