



**Focused on  
Growth and Innovation**

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

H.C. Wainwright 22nd Annual Global Investment Conference

David Veitch, CEO presentation  
September 2020

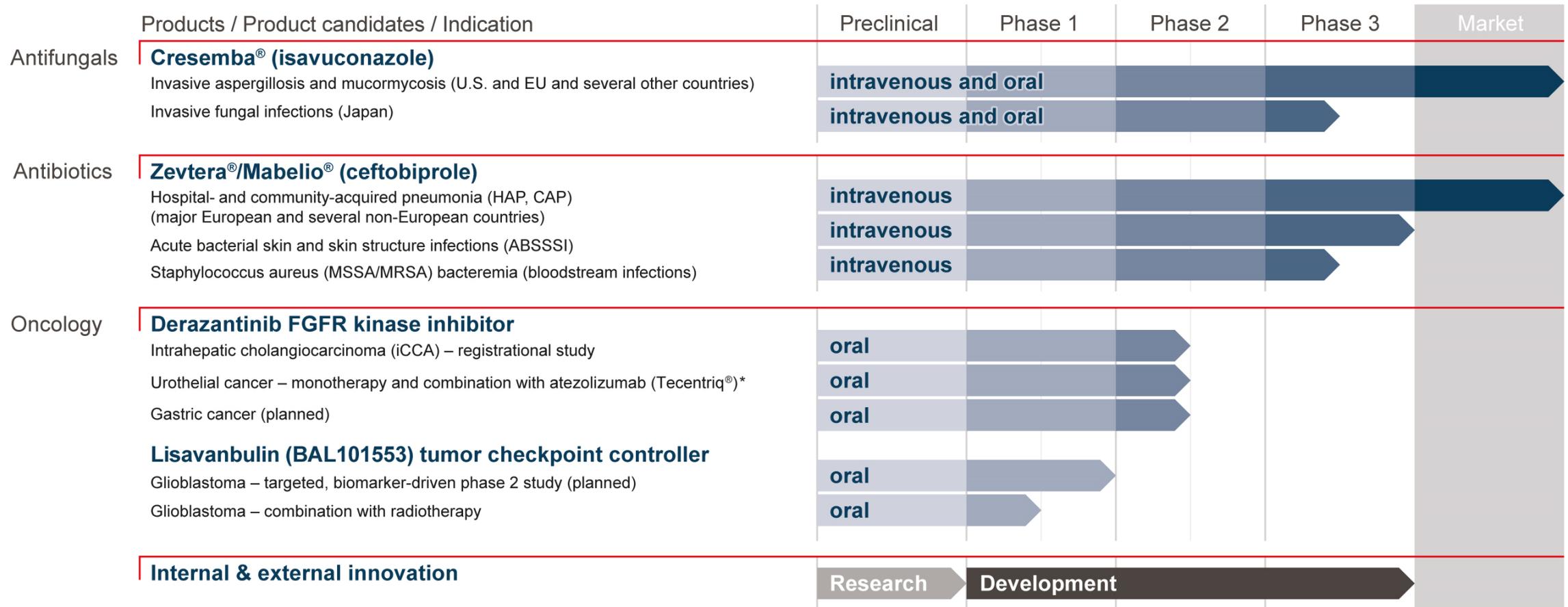


# At a glance

- Well funded, commercial-stage biotech 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

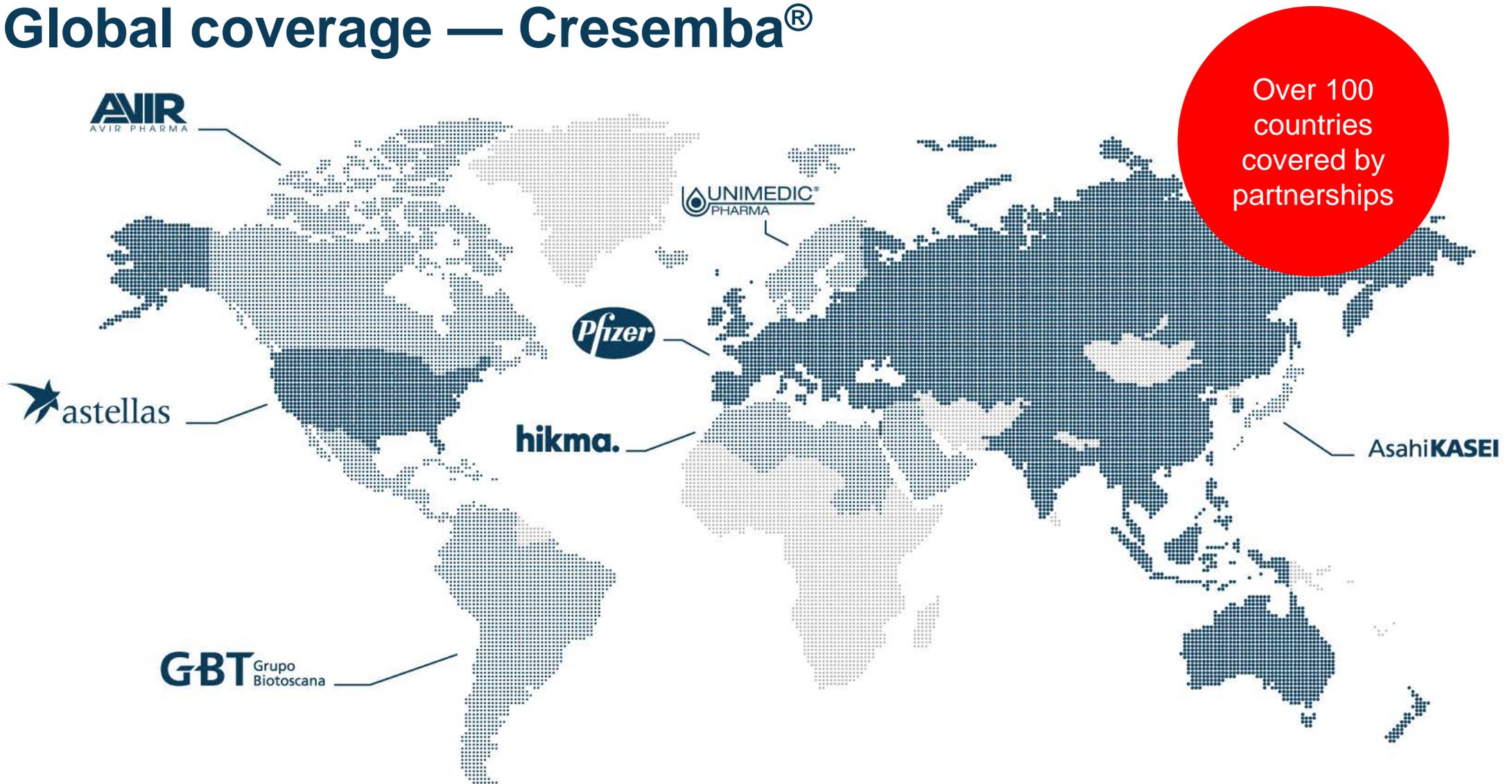


# Potential for sustainable growth and value creation based on commercialized brands and innovative pipeline



\* Tecentriq® is a registered trademark of Hoffmann-La Roche Ltd.

# 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®)

Double digit  
percentage  
royalties on  
sales by  
license  
partners

USD 1.1 bn  
in potential  
milestones  
remaining

Participation  
in sales of  
distribution  
partners  
through  
transfer price

USD ~255 mn  
upfront and  
milestone  
payments  
received

Antifungal

**Cresemba<sup>®</sup>**  
**(isavuconazole)**

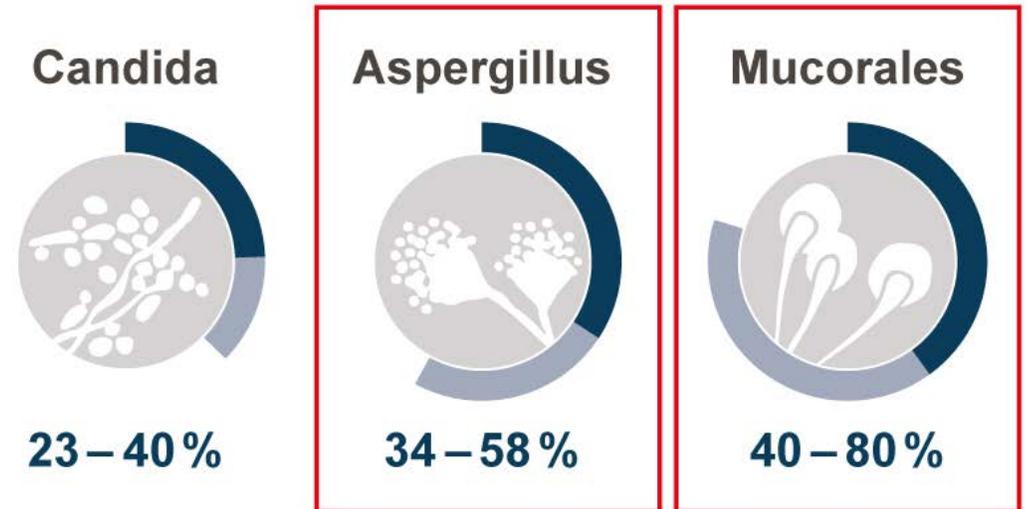
Invasive mold infections



# The market — Invasive fungal infections

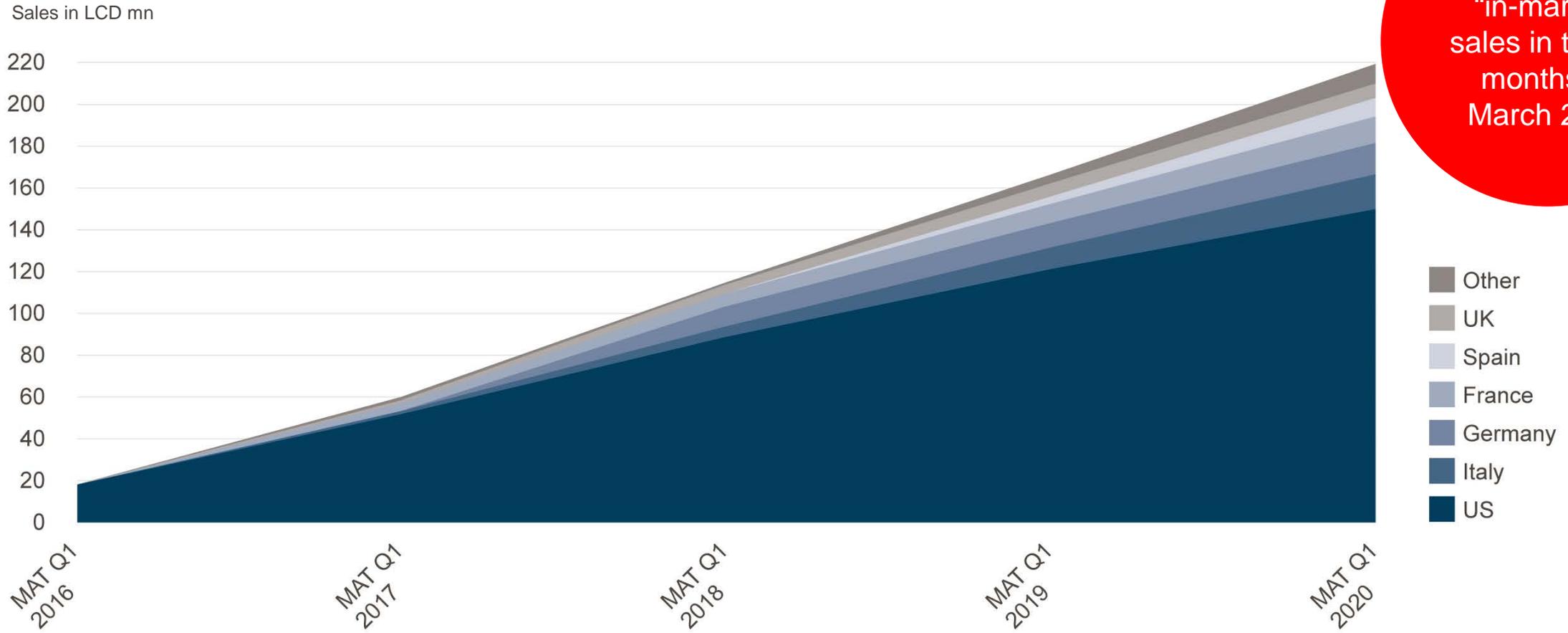
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand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

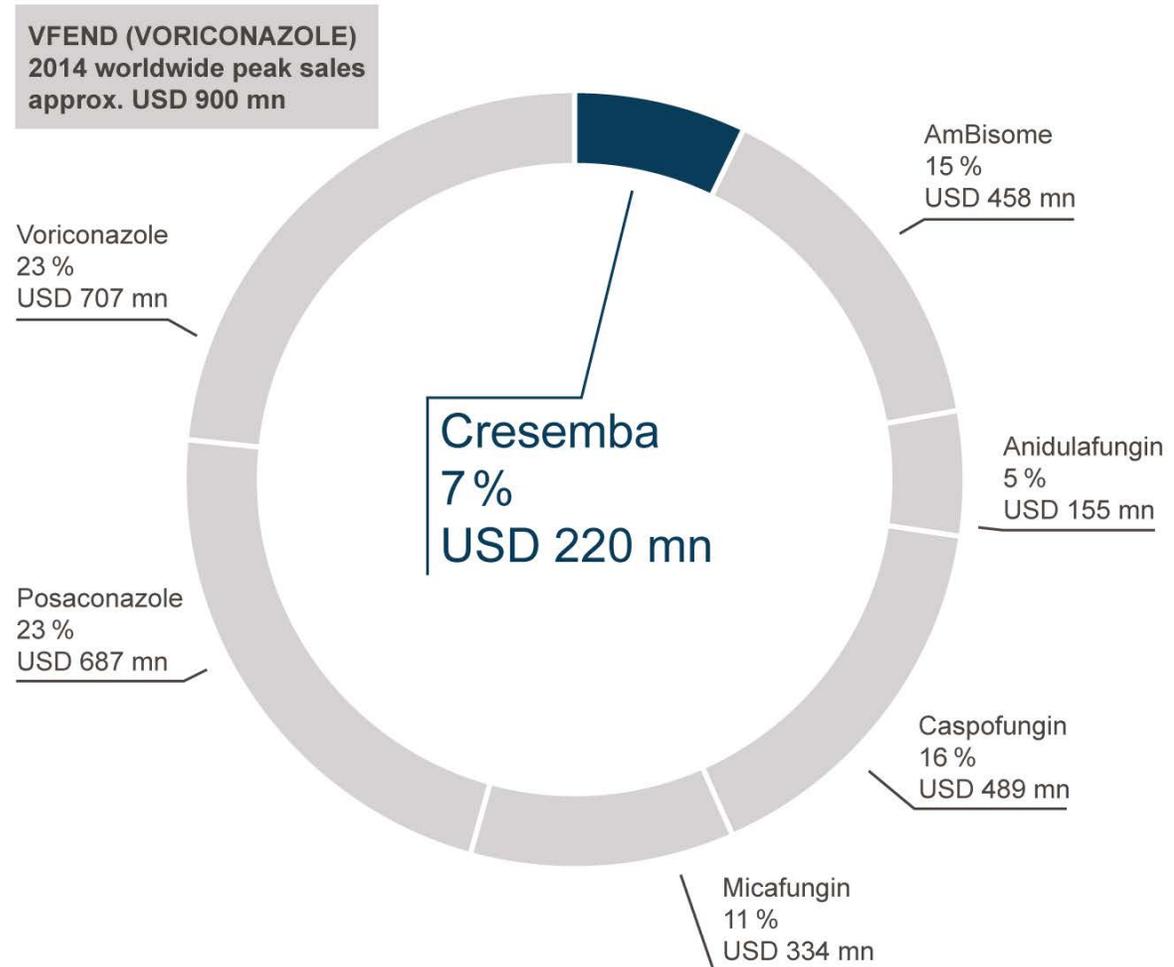
LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, March 2020

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

USD 3.1 bn sales (MAT Q1 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, March 2020

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Antibacterial

**Zevtera<sup>®</sup> / Mabelio<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

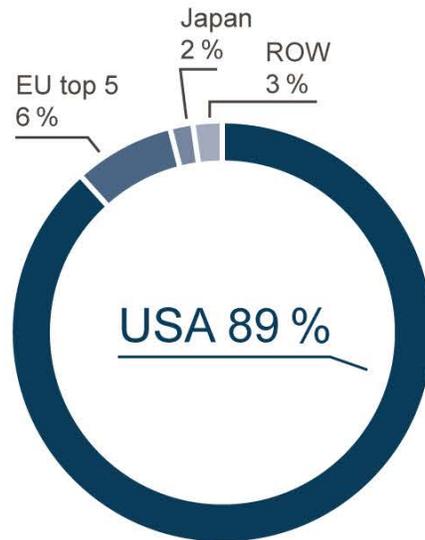
Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP), and community-acquired pneumonia (CAP). Not approved in the U.S.

MENA: Middle East and North Africa

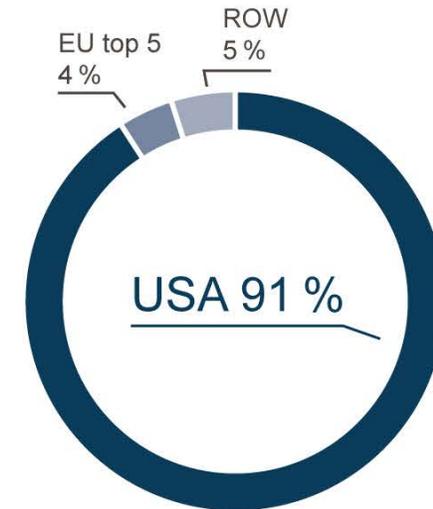


# The hospital anti-MRSA antibiotic market — A USD 3 bn market\* with the U.S. being the most important region

### Daptomycin sales by region (2015, before LOE)



### Ceftaroline sales by region (MAT Q1 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, March 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 128 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 Q1 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 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.

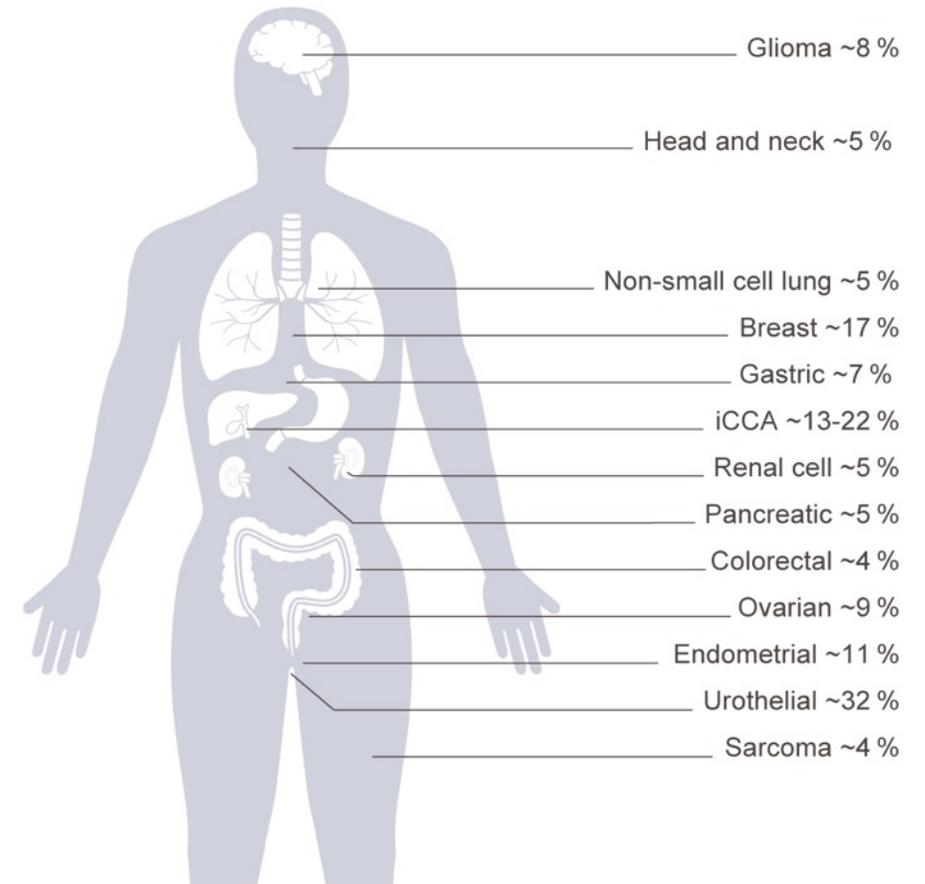
Oncology

# Derazantinib

FGFR-driven tumors

# Targeting FGFR-driven tumors as single agent and in 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
- Two clinical studies ongoing (FIDES-01 in iCCA & FIDES-02 in urothelial cancer)
- Plan to start a multi-cohort phase 1/2 study (FIDES-03) in patients with advanced gastric cancer in Q3 2020



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

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# Registrational phase 2 study in iCCA (FIDES-01)<sup>1</sup>

## **Cohort 1:** Patients with FGFR2 gene-fusion expressing iCCA (2nd line)

- Encouraging interim results, consistent with earlier phase 1/2 data<sup>2</sup>
- 21% ORR with six confirmed partial responses from 29 evaluable patients, 83% disease control rate
- Completed patient enrolment in July 2020
- Topline results expected H2 2020

## **Cohort 2:** Patients with FGFR2 gene mutations or amplifications

- Assessing the activity of derazantinib in a broader range of FGFR2-driven tumors
- Clinical benefit observed in a subset of iCCA patients in the phase 1/2 study<sup>2</sup>
- Aim to confirm phase 1/2 study results in a larger cohort of iCCA patients<sup>1</sup>
- Define the full therapeutic potential of derazantinib in iCCA with potential for differentiation
- Interim results expected H2 2020

<sup>1</sup> NCT03230318

<sup>2</sup> Droz Dit Busset et al. Annals of Oncology (2019) 30 (suppl\_5): abstract 3879 (NCT01752920)

# Clinical program in urothelial and gastric cancer

## FIDES-02<sup>1</sup> | Urothelial Cancer

*Multi-cohort Phase 1b/2 study of derazantinib monotherapy or in combination with atezolizumab (Tecentriq<sup>®</sup>) in patients with urothelial cancer expressing activating molecular FGFR 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
- First interim results expected in H2 2020

## FIDES-03 | Gastric Cancer

*Multi-cohort Phase 1b/2 study of derazantinib as monotherapy or in combination therapy with standard of care 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 and standard of care
  - Combination of derazantinib with atezolizumab (Tecentriq<sup>®</sup>)
- Expected start of enrolment in Q3 2020

<sup>1</sup> NCT04045613

# 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%#
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%

<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

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

Oncology

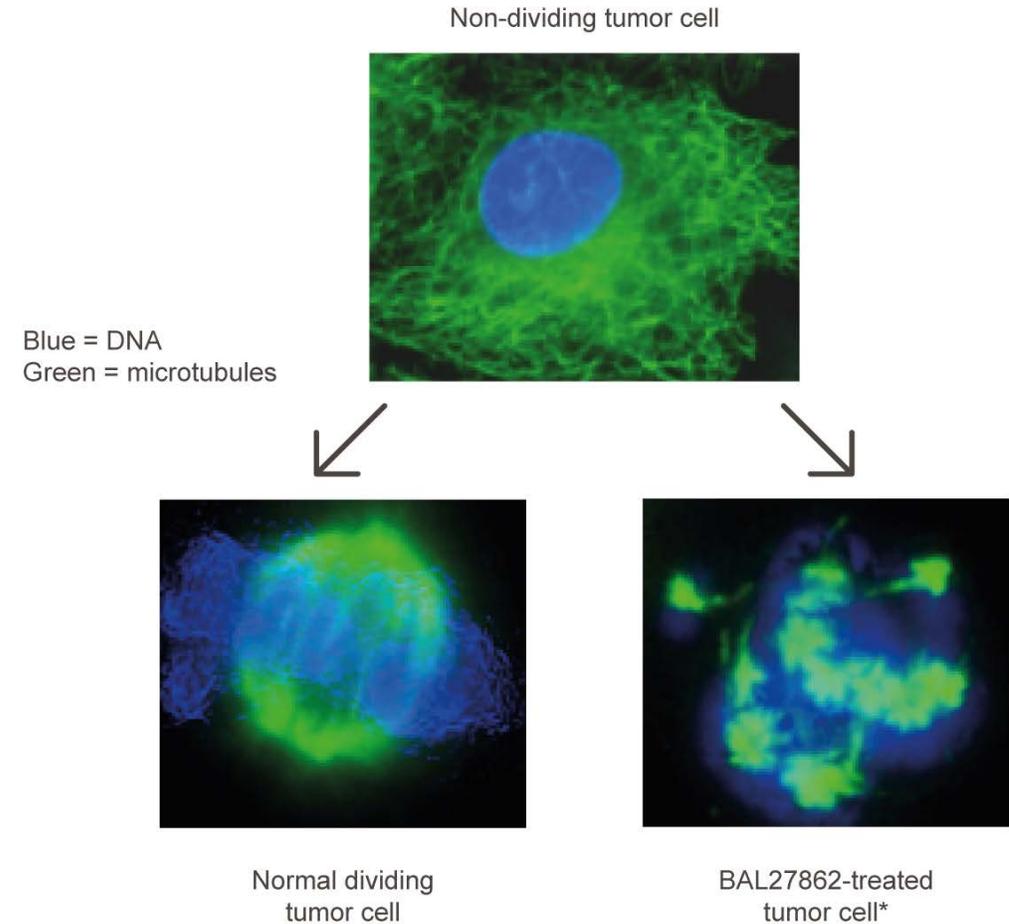
# Lisavanbulin (BAL101553)

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
- Clinical program focused on glioblastoma (GBM) using a biomarker-driven approach



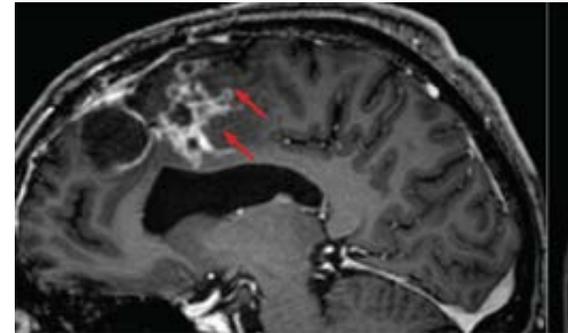
\* Lisavanbulin (BAL101553) is a prodrug of BAL27862

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

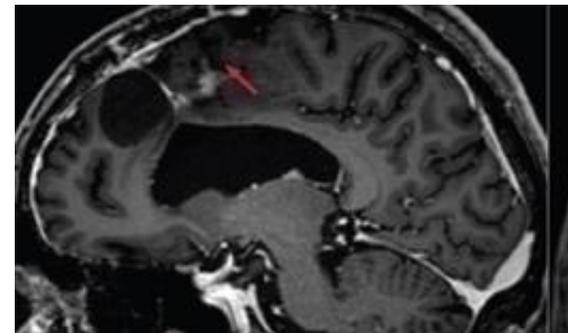
- EB1 (plus-end binding protein) is located on the microtubules and involved in microtubule dynamics and has been shown to be a response predictive marker for lisavanbulin in preclinical studies
- Strong EB1 staining was observed in a patient with an exceptional response to daily oral lisavanbulin in the phase 1 dose-escalation study in recurrent GBM<sup>1</sup>
  - Patient ongoing for more than two years
  - >80% reduction in GBM tumor size
- Biomarker-driven phase 2 study in patients with recurrent GBM using EB1-positivity as patient selection criterion, anticipated to start in the next few months

<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)

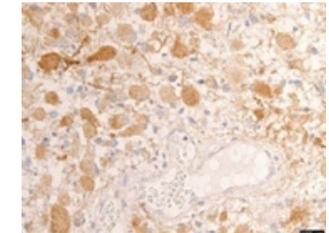
## GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



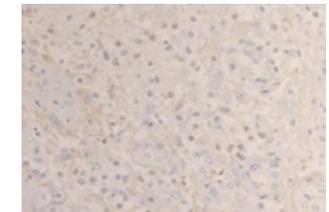
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



Non-responder



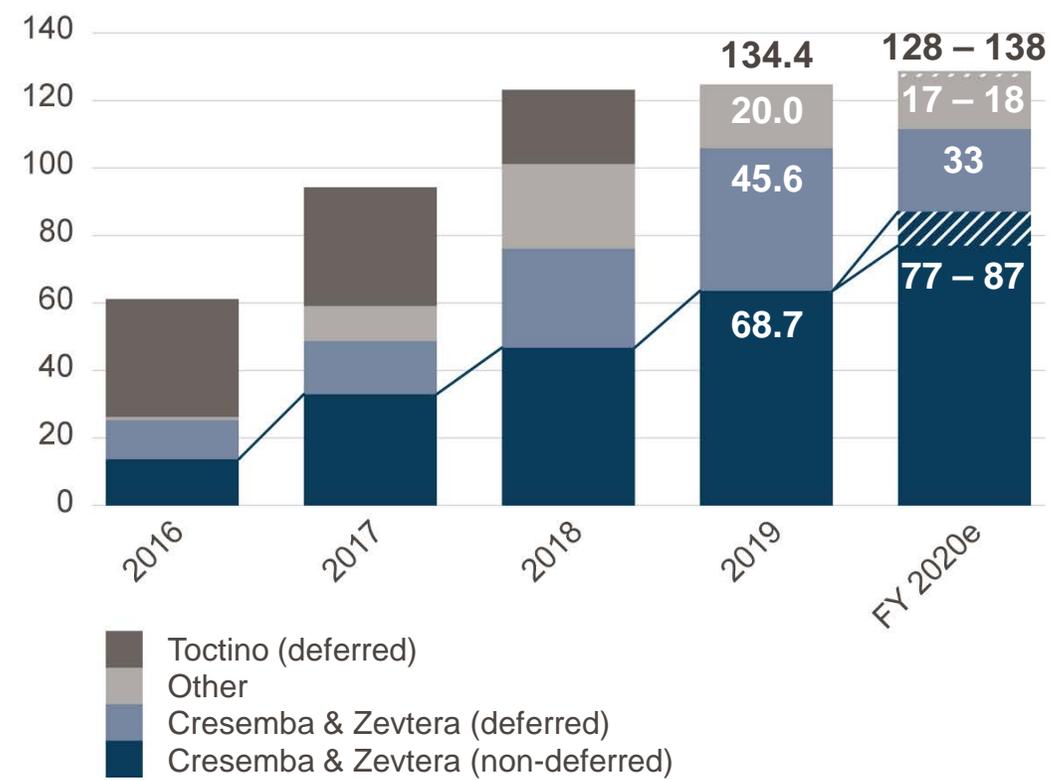
# Financials



# Financial guidance

In CHF mn	FY 2020e	FY 2019
Total revenue	128 – 138	134.4
thereof: Contributions Cresemba® & Zevtera®		
non-deferred	77–87	68.7
deferred	33	45.6
Operating loss	5-15	17.2
Cash and investments	150	161.0

Strong increase in non-deferred revenue contributions  
Y-o-Y, CHF mn



# Outlook 2020 / 2021

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

	H1 2020	H2 2020	H1 2021	H2 2021
<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
<b>Derazantinib</b>	<b>FIDES-01 (iCCA)</b>	✓ Complete patient enrolment in phase 2 registrational study (FGFR2 fusions) Topline results (FGFR2 fusions)		
		Interim results (other FGFR2 gene aberrations)		Topline results (other FGFR2 gene aberrations)
	<b>FIDES-02 (urothelial cancer)</b>	Safety data and recommended phase 2 dose (RP2D) for derazantinib/Tecentriq combination and expansion into phase 2	Interim results in derazantinib monotherapy	Interim results in combination therapy with Tecentriq
	<b>FIDES-03 (gastric cancer)</b>	✓ Clinical supply agreement with Roche in gastric cancer Start of phase 1/2 study		Interim results
<b>Lisavanbulin (Oral)</b>	✓ Full results of phase 1 study in glioblastoma* Start phase 2 biomarker-driven glioblastoma study		Interim results from phase 2 biomarker-driven glioblastoma study	Topline results from phase 2 biomarker-driven glioblastoma study
			Complete patient enrolment in phase 1 study in newly diagnosed glioblastoma	

\* Accepted for ESMO poster presentation (Sept. 2020)

# Disclaimer and forward-looking statements

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