# Focused on Growth and Innovation

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

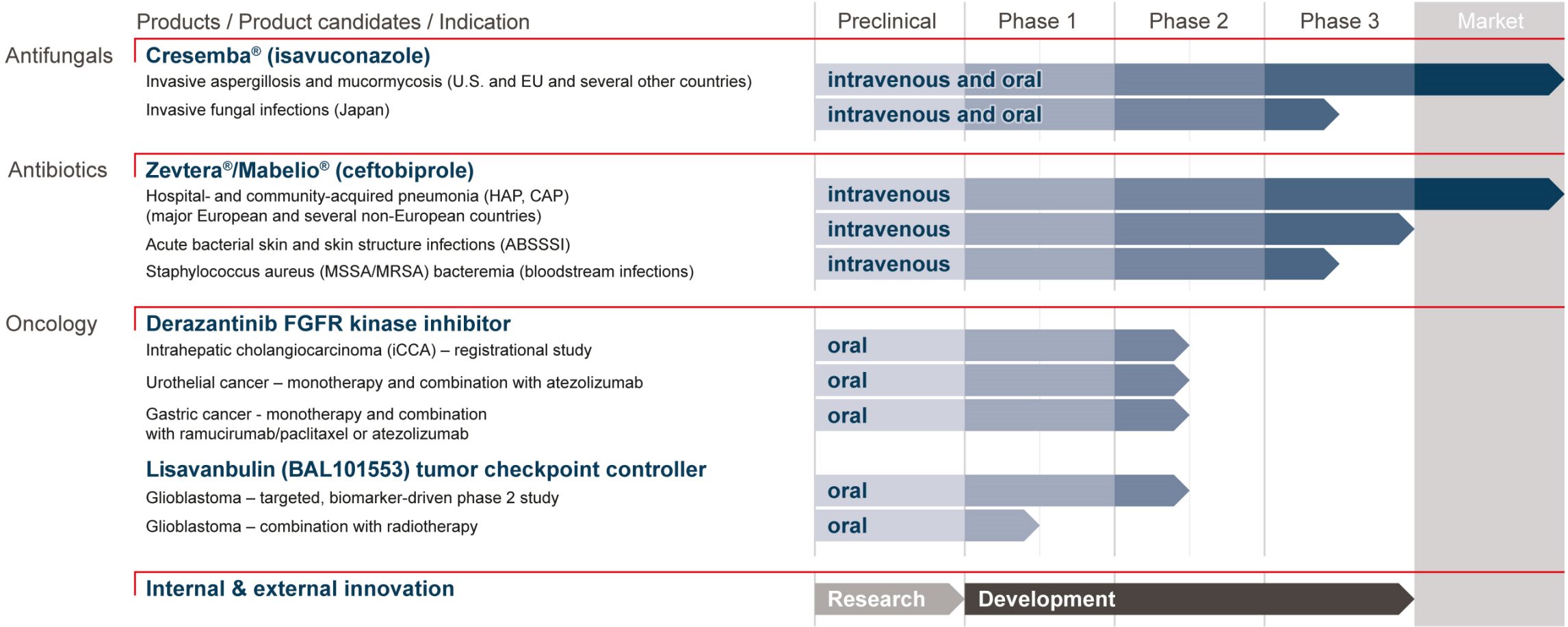
Bryan Garnier Virtual Healthcare Conference

David Veitch, CEO presentation
November 16, 2020

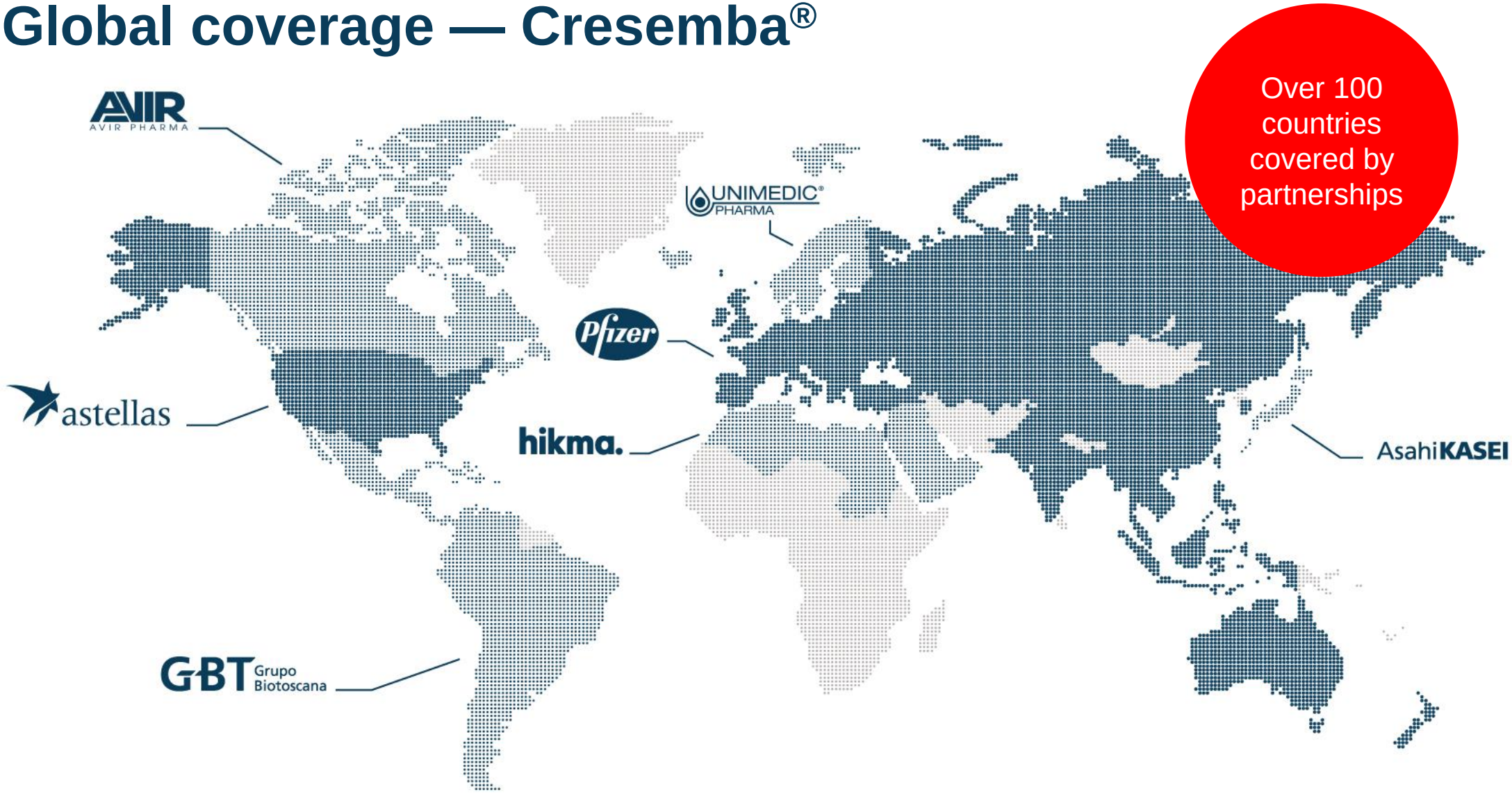
# At a glance

- 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

Pfizer
Europe (excl. Nordics), China Asia-Pacific, Russia, Turkey ans Israel (Cresemba®)

astellas
U.S. (Cresemba®)

AsahiKASEI
Japan (Cresemba®)

CR Gosun
China (Zevtera®)

## Distribution partners

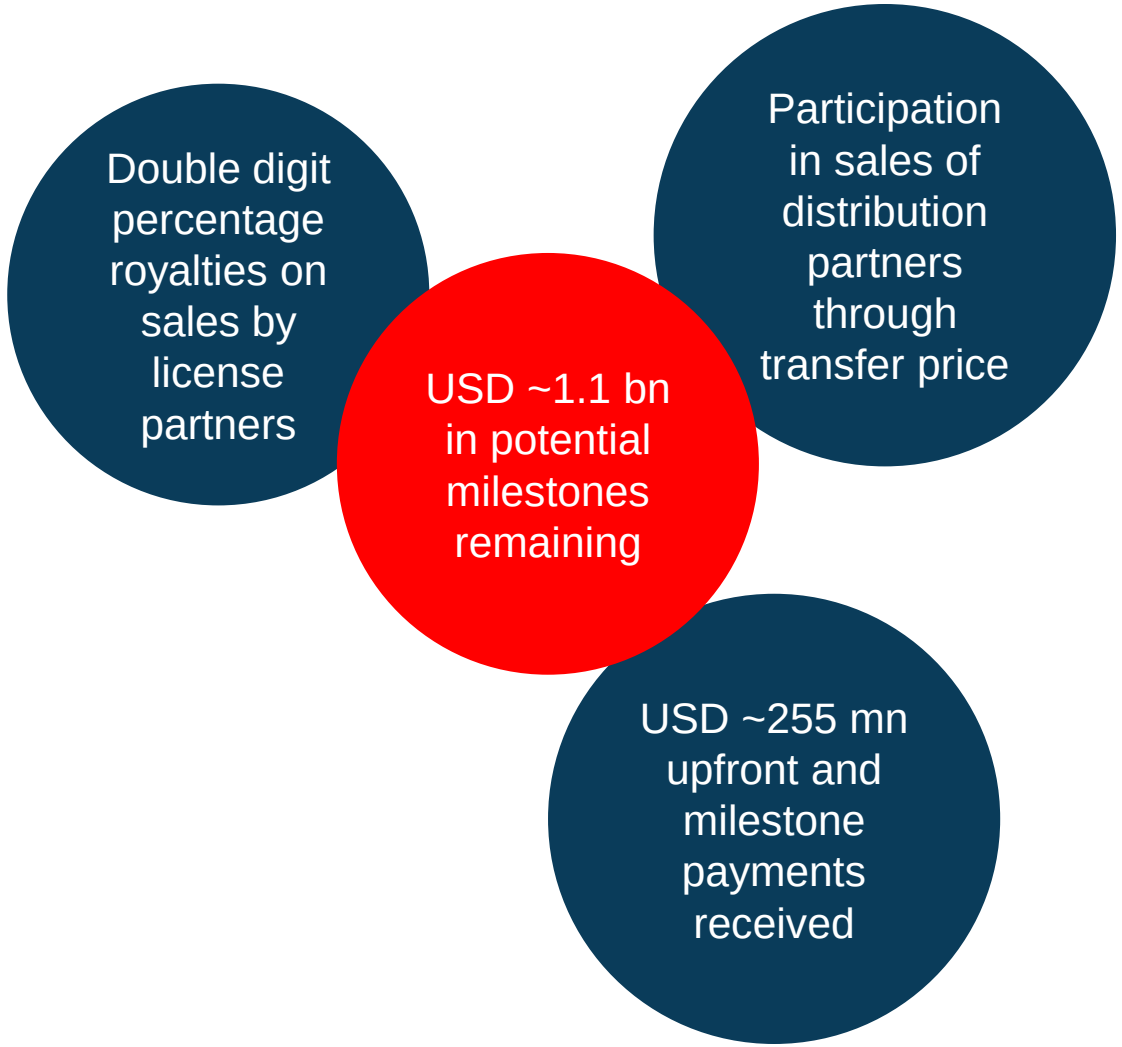
correvio
Europe (excl. Nordics), Israel (Zevtera®)

hikma.
MENA region (Cresemba® and Zevtera®)

GBT Grupo Biotoscana
LatAm (Cresemba® and Zevtera®)

UNIMEDIC® PHARMA
Nordics (Cresemba® and Zevtera®)

AVIR PHARMA
Canada (Cresemba® and Zevtera®)



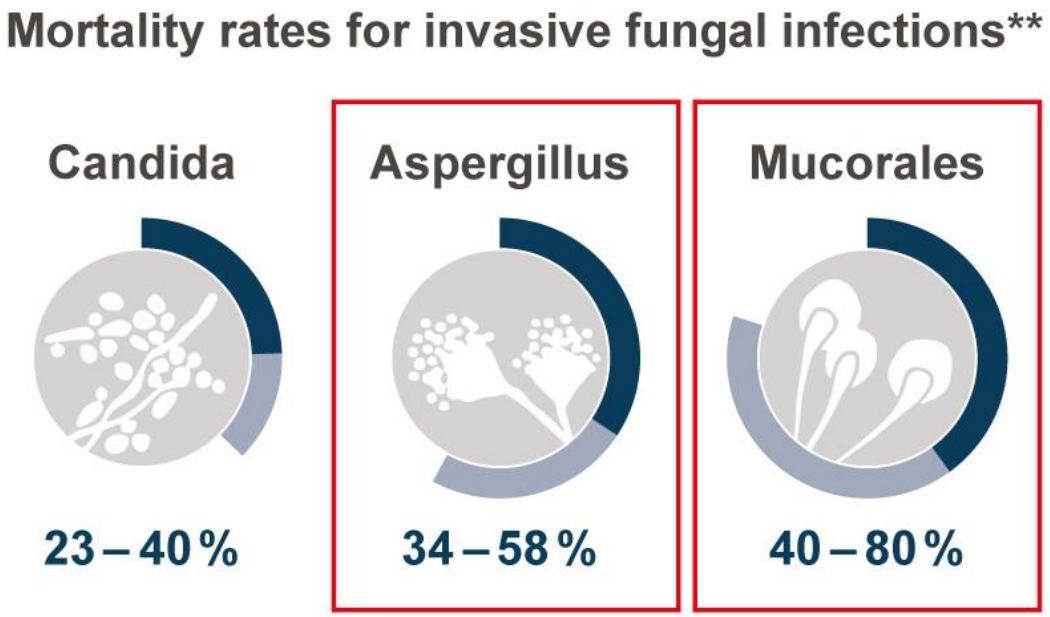
Antifungal

# Cresemba® (isavuconazole)

Invasive mold infections

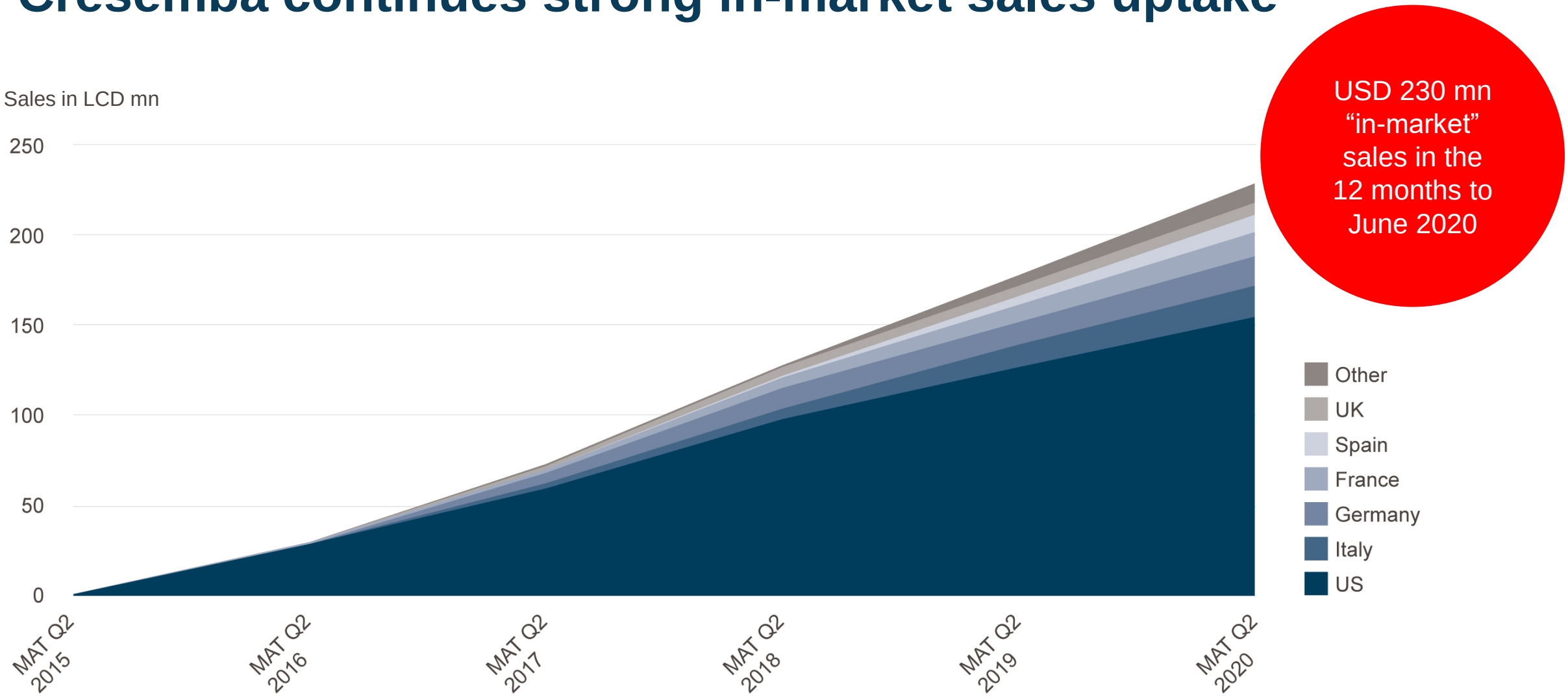
# The market — Invasive fungal infections

- 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




**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

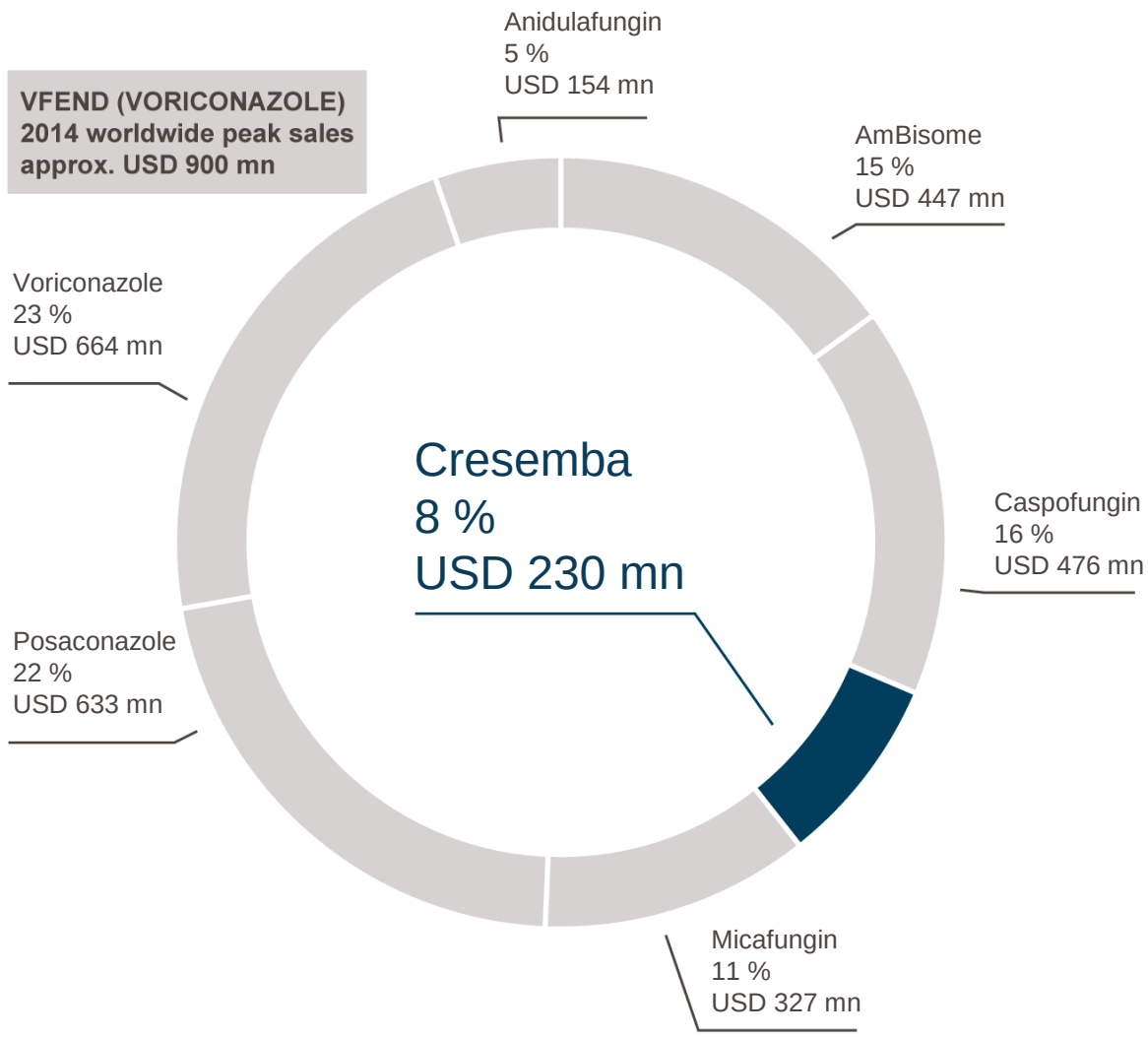



LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, June 2020

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

## USD 2.9 bn sales (MAT Q2 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, June 2020

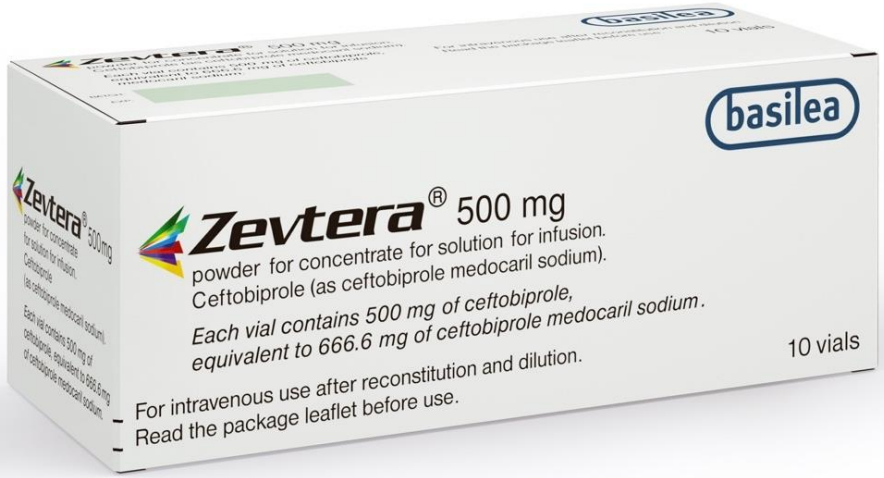
Antibacterial

# Zevtera® / Mabelio® (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

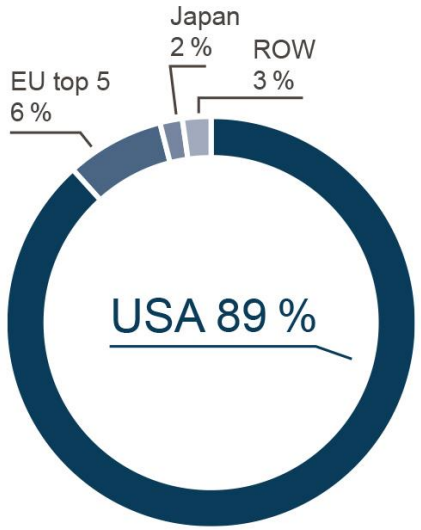



Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP), and community-acquired pneumonia (CAP). Not approved in the U.S.

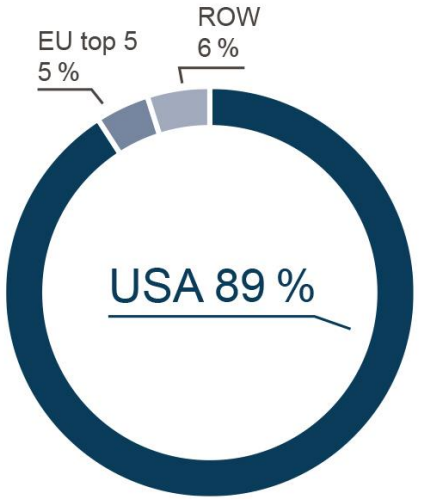
MENA: Middle East and North Africa

# 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 Q2 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, June 2020

# Strategy for accessing the U.S. market

- Two cross-supportive phase 3 studies under FDA Special Protocol Assessment (SPA)

1. Acute Bacterial Skin and Skin Structure Infections (ABSSSI)[1] successfully completed

TARGET
ABSSSI Study

2. *Staphylococcus aureus* bacteremia (SAB)[2] ongoing, topline results from phase 3 study expected in Q1 2022

ERADICATE
SAB Study

- Phase 3 program largely funded by BARDA (up to USD ~130 mn, ~70% of total program costs)

BIOMEDICAL ADVANCED RESEARCH AND DEVELOPMENT AUTHORITY
BARDA

- 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)

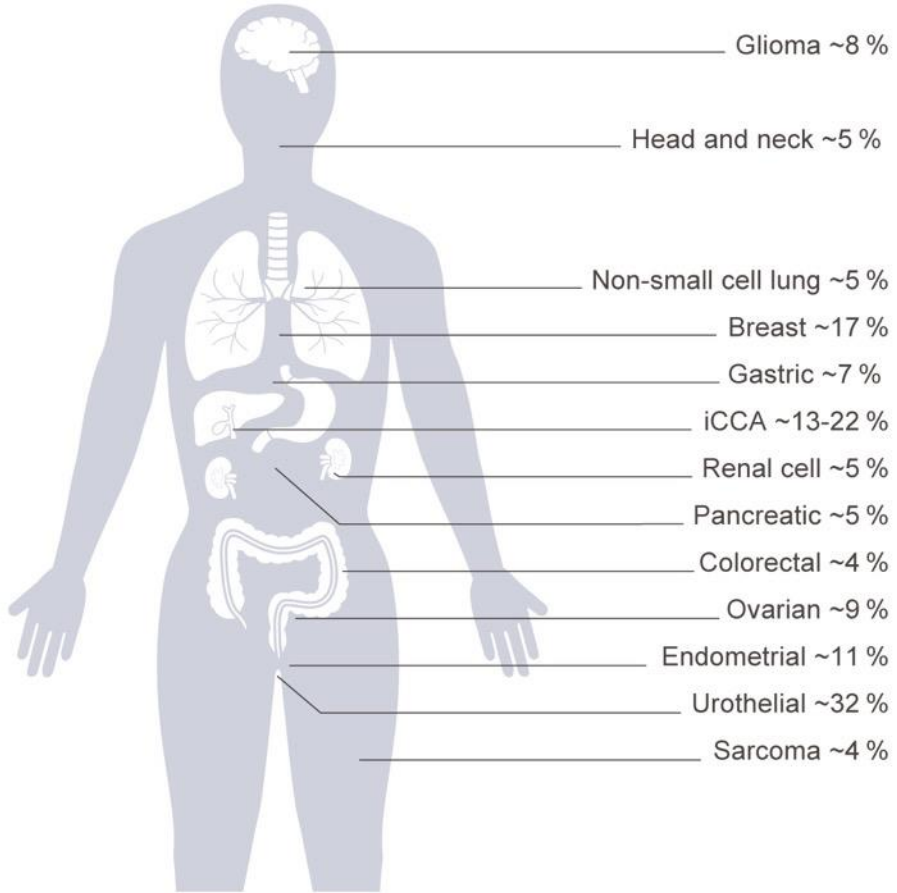
Oncology

# 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

# Registrational phase 2 study in iCCA (FIDES-01)[1]

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

- Encouraging interim results, consistent with earlier phase 1/2 data[2]
  - 21% ORR with six confirmed partial responses from 29 evaluable patients, 83% disease control rate
  - Manageable safety profile with low incidence of nail toxicity, retinal events, hand-foot syndrome and stomatitis
- Topline results expected H2 2020

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

- Define the full therapeutic potential in iCCA with potential for differentiation
- Encouraging interim results - progression-free survival consistent with outcome in patients with FGFR2 gene-fusions[3]
  - Pooled data from 23 patients treated in clinical studies and from the early access and compassionate use programs
  - 7.2 months median progression free survival and 8.2 months median duration of treatment

[1] NCT03230318
[2]Droz Dit Busset et al. Annals of Oncology (2019) 30 (suppl_5): abstract 3879 (NCT01752920)
[3]Droz Dit Busset et al. Annals of Oncology (2020) 31 (suppl_5): abstract 45P (NCT01752920, NCT03230318)

# Clinical program in urothelial and gastric cancer

## FIDES-02[1] | Urothelial Cancer

*Multi-cohort Phase 1b/2 study of derazantinib monotherapy or in combination with atezolizumab 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
- Successful completion of phase 1b cohort
  - Recommended phase 2 dose for the combination at full standard doses of derazantinib and atezolizumab
  - No dose-limiting toxicities observed
- Clinical supply agreement with Roche for atezolizumab

## 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 with ramucirumab/paclitaxel
  - Combination of derazantinib with atezolizumab
- Clinical supply agreement with Roche for atezolizumab and clinical trial collaboration and supply agreement with Lilly for ramucirumab

[1] NCT04045613; Chaudhry A et al. Journal of Clinical Oncology 2020; 38, no. 6_suppl. TPS590. (NCT04045613)

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

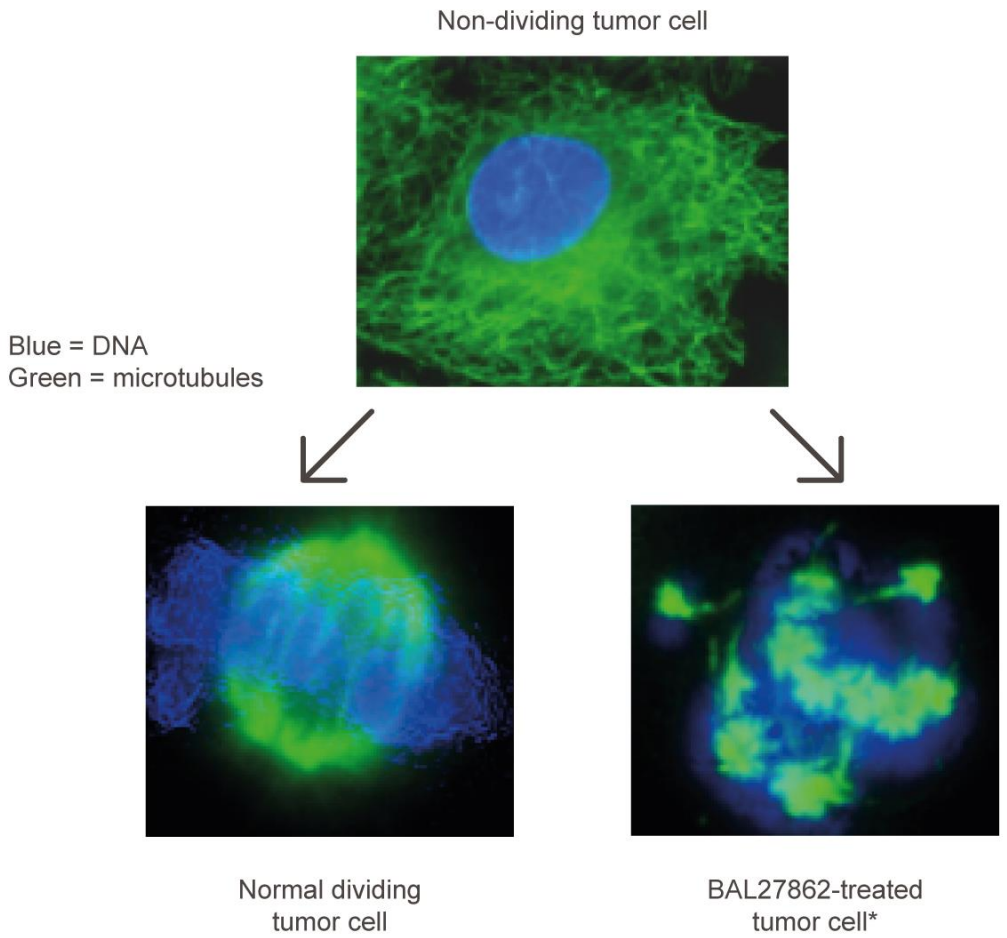
Oncology

# 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
- Biomarker-driven phase 2 study in patients with recurrent glioblastoma (GBM) using EB1-positivity as patient selection criterion ongoing

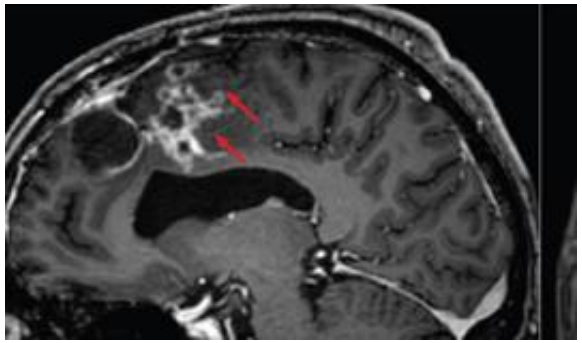



* Lisavanbulin (BAL101553) is a prodrug of BAL27862

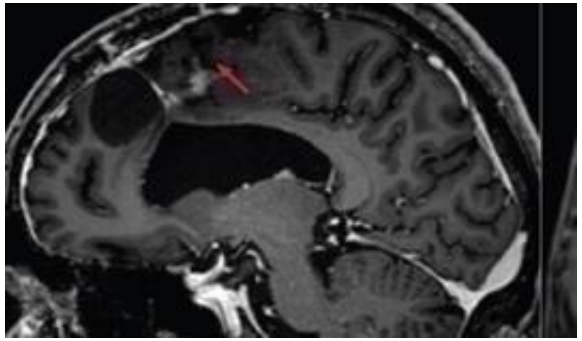
# EB1 — A potential response-predictive clinical biomarker for lisavanbulin

- 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[1]
  - Patient ongoing for more than two years
  - >80% reduction in GBM tumor size

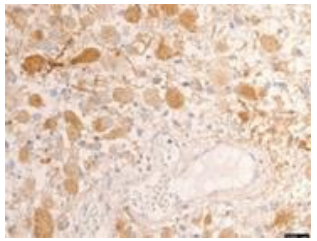
## GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



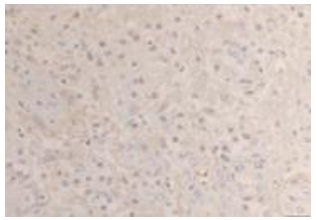
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



Non-responder

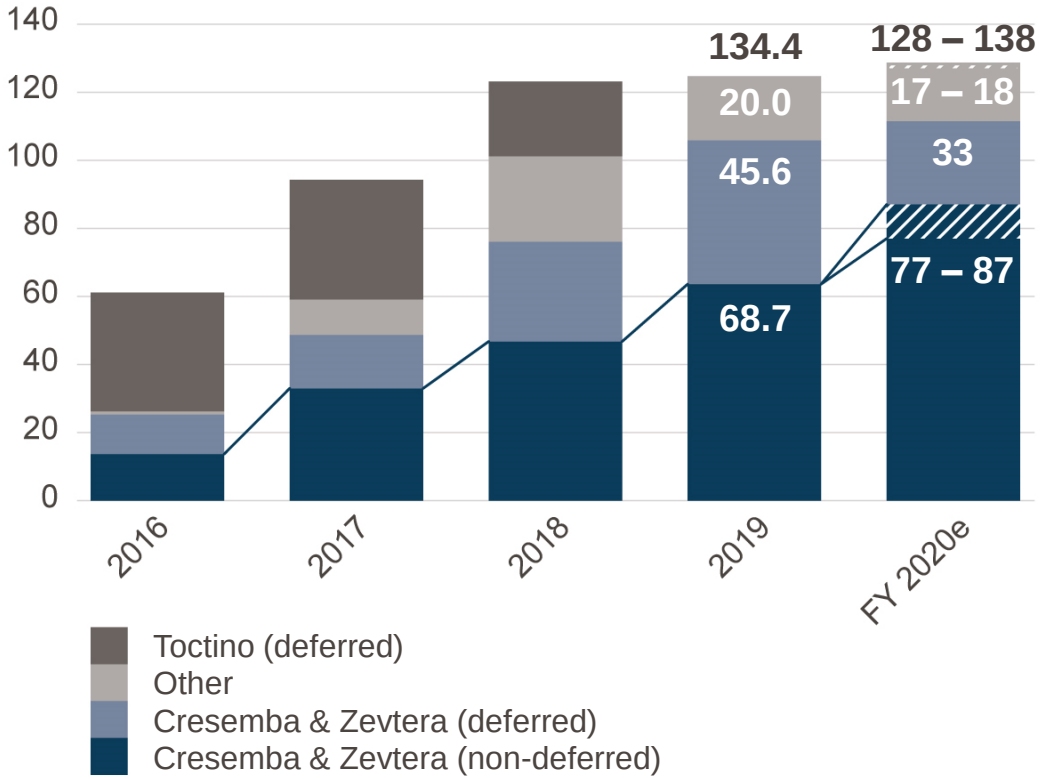
[1] Lopez et al. Phase 1/2a study of once daily oral BAL101553, a novel tumor checkpoint controller, in adult patients with progressive or recurrent glioblastoma or high-grade glioma. JCO 2019;37:15 suppl, 2025 (NCT02490800)

# Financials

# Financial guidance

| In CHF mn | FY 2020e | FY 2019 |
|---|---|---|
| Total revenue | 128 – 138 | 134.4 |
| thereof:<br>Contributions Cresemba® & Zevtera®<br>non-deferred<br>deferred | <br><br>77–87<br>33 | <br><br>68.7<br>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**



# Milestones & 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 |
|---|---|---|---|---|---|
| **Isavuconazole** | | | Complete patient enrolment in phase 3 study in Japan | | Topline results from phase 3 study in Japan |
| **Ceftobiprole** | | | ✓ Approval in China | | Complete patient enrolment in SAB phase 3 study |
| **Derazantinib** | **FIDES-01 (iCCA)** | ✓ 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) |
| | **FIDES-02 (urothelial cancer)** | | ✓ Safety data and recommended phase 2 dose (RP2D) for derazantinib/atezolizumab combination and expansion into phase 2 | Interim results in derazantinib monotherapy | Interim results in combination therapy with atezolizumab |
| | **FIDES-03 (gastric cancer)** | ✓ Clinical supply agreement with Roche | ✓ Start of phase 1/2 study | | Interim results |
| | | | ✓ Clinical trial collaboration and supply agreement with Lilly | | |
| **Lisavanbulin (Oral)** | | ✓ 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

This communication, including the accompanying oral presentation, contains certain forward-looking statements, including, without limitation, statements containing the words “believes”, “anticipates”, “expects”, “supposes”, “considers”, and words of similar import, or which can be identified as discussions of strategy, plans or intentions. Such forward-looking statements are based on the current expectations and belief of company management, and are subject to numerous risks and uncertainties, which may cause the actual results, financial condition, performance, or achievements of Basilea, or the industry, to be materially different from any future results, performance, or achievements expressed or implied by such forward-looking statements. Such factors include, among others, the following: the uncertainty of pre-clinical and clinical trials of potential products, limited supplies, future capital needs and the uncertainty of additional funding, compliance with ongoing regulatory obligations and the need for regulatory approval of the company’s operations and potential products, dependence on licenses, patents, and proprietary technology as well as key suppliers and other third parties, including in preclinical and clinical trials, acceptance of Basilea’s products by the market in the event that they obtain regulatory approval, competition from other biotechnology, chemical, and pharmaceutical companies, attraction and retention of skilled employees and dependence on key personnel, and dependence on partners for commercialization of products, limited manufacturing resources, management’s discretion as to the use of proceeds, risks of product liability and limitations on insurance, uncertainties relating to public health care policies, adverse changes in governmental rules and fiscal policies, changes in foreign currency and other factors referenced in this communication. Given these uncertainties, prospective investors are cautioned not to place undue reliance on such forward-looking statements. Basilea disclaims any obligation to update any such forward-looking statements to reflect future events or developments, except as required by applicable law. Derazantinib and its uses are investigational and have not been approved by a regulatory authority for any use. Efficacy and safety have not been established. The information presented should not be construed as a recommendation for use. The relevance of findings in nonclinical/preclinical studies to humans is currently being evaluated.

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