



## UBS Global Healthcare Conference

David Veitch, CEO  
New York, May 21, 2019

# Disclaimer and forward-looking statements

## Forward looking statements

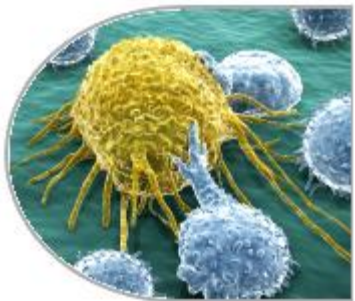
This communication including the accompanying oral presentation contains certain forward-looking statements, including, without limitation, statements containing the words “believes”, “anticipates”, “expects”, “supposes”, “considers” and words of similar import or which can be identified as discussions of strategy, plans or intentions. Such forward-looking statements are based on current expectations and belief of company management, which 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 Company’s products by the market in case they obtained regulatory approval, competition from other biotechnology, chemical and pharmaceutical companies, attraction and retention of skilled employees and dependence on key personnel, early stage of sales and marketing structure and dependence on partners for commercialization of products, limited manufacturing resources, management’s discretion as to 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. The company disclaims any obligation to update any such forward-looking statements to reflect future events or developments, except as required by applicable law.



# Basilea — At a glance



- Revenue-generating, commercial-stage Swiss biotech company with solid cash position (YE2018 ~CHF 223mn)
- Focused in the areas of oncology, hospital antibiotics and hospital antifungals



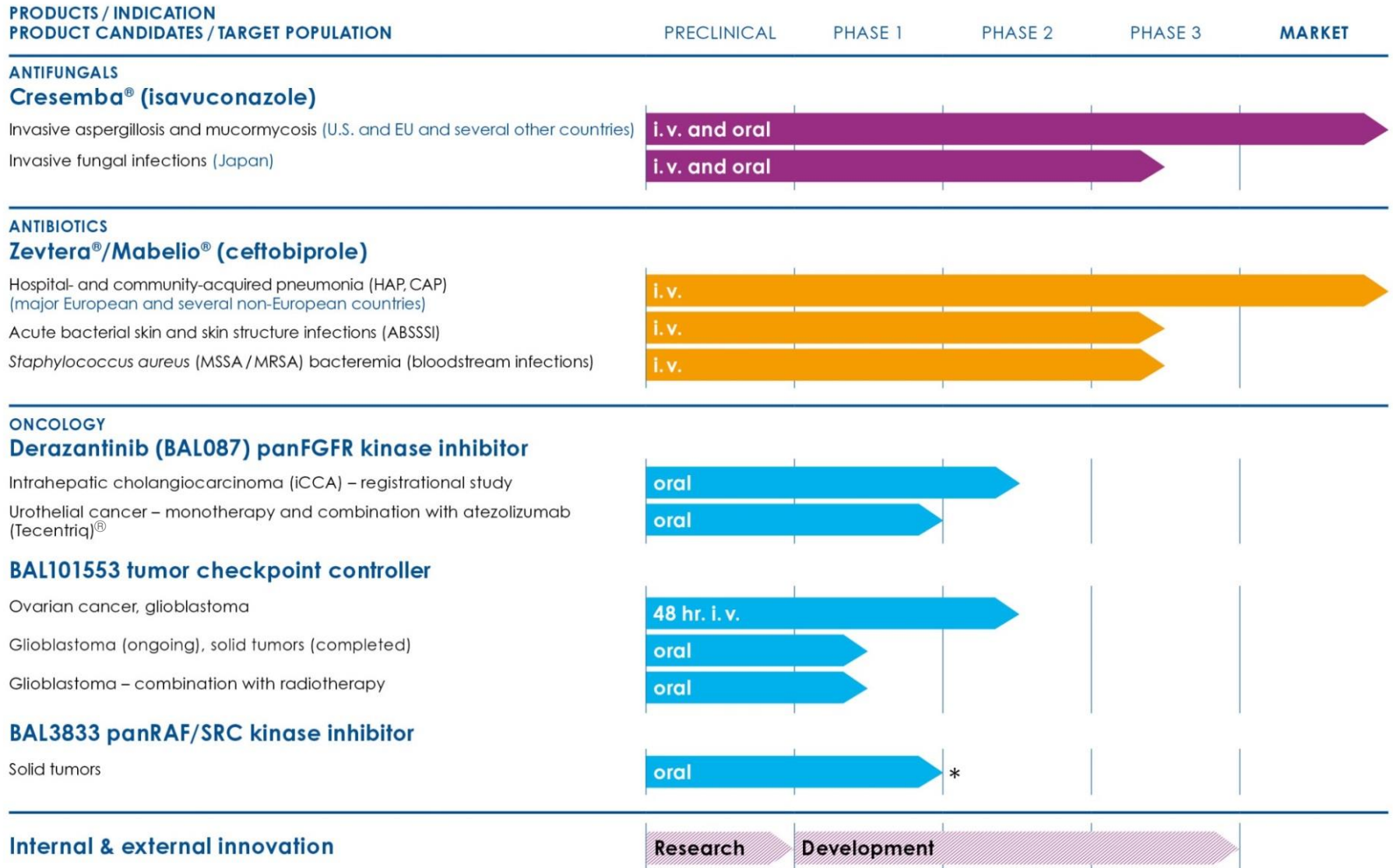
- Two marketed anti-infective brands (Cresemba and Zevtera) and three oncology drug candidates in development
- Potential for sustainable growth and value generation based on increasing revenues and selective investments into internal and external innovation



- Founded in 2000 as spin-off from Roche
- Listed on the SIX Swiss Stock Exchange since 2004 (SIX: BSLN)
- Based in life sciences hub Basel (Switzerland)



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



\* pre-clinical reformulation activities initiated

# Established strong partnerships to fully exploit commercial potential of Cresemba® and Zevtera®

## License partners

- **Pfizer**, for Europe (ex. Nordics), China, Asia-Pacific, Russia, Turkey and Israel (*Cresemba*)
- **Astellas**, for the U.S. (*Cresemba*)
- **Asahi Kasei Pharma**, for Japan (*Cresemba*)
- **CR Gosun**, for China (*Zevtera*)



CR Gosun

## Distribution partners

- **Correvio**, for Europe (ex. Nordics), Israel (*Zevtera*)
- **Hikma**, for MENA region (*Cresemba and Zevtera*)
- **Grupo Biotoscana**, for LatAm (*Cresemba and Zevtera*)
- **Unimedica**, for Nordics (*Cresemba and Zevtera*)
- **Avir**, for Canada (*Cresemba and Zevtera*)

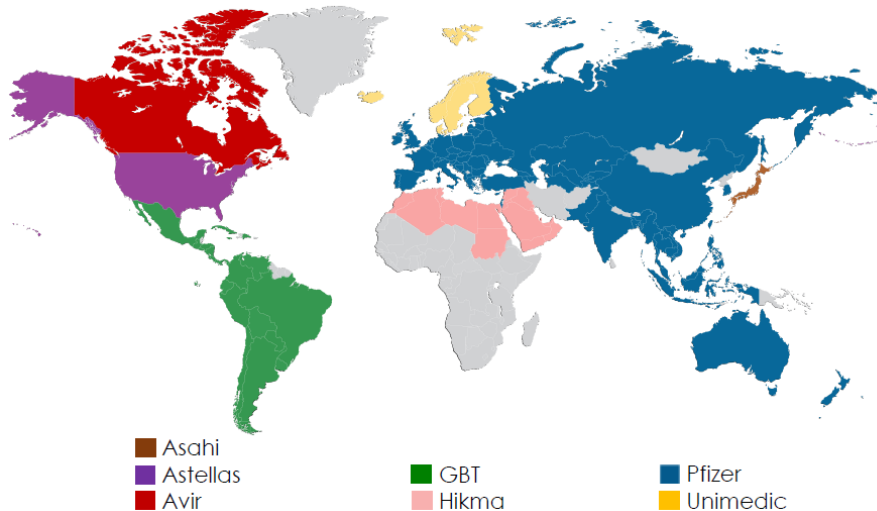


# >100 countries covered by partnerships — USD 1.1bn in total potential milestones

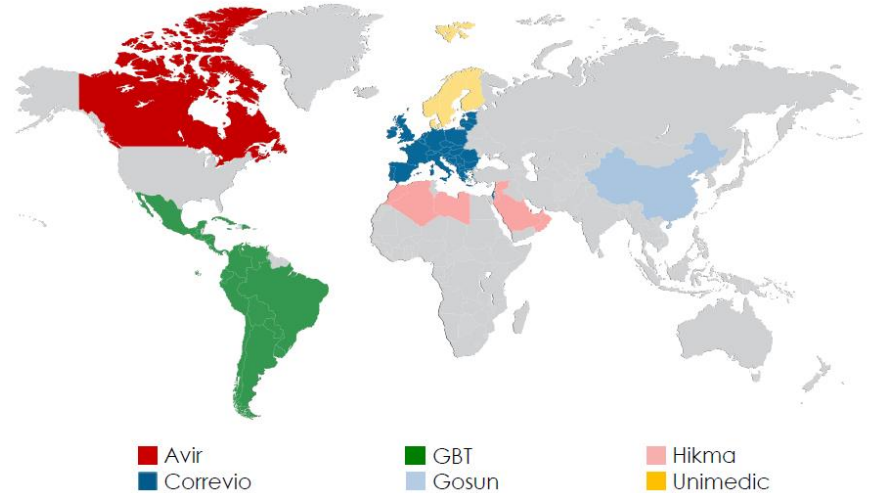
## Ongoing participation

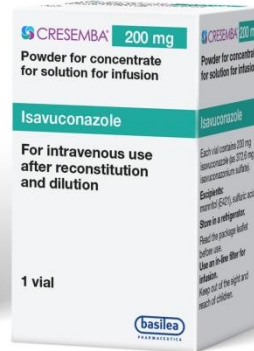
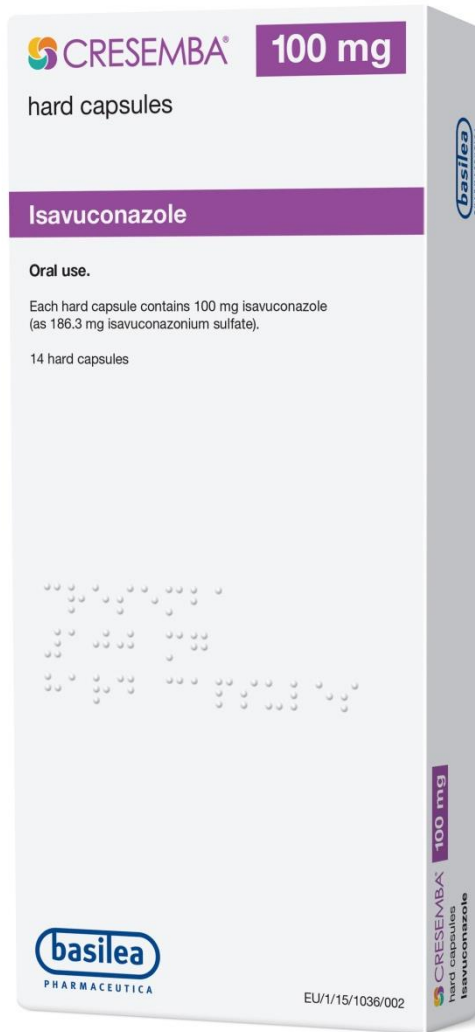
- Double-digit royalties on sales by license partners
- Participation through transfer price structure in sales by distribution partners
- Approximately USD 245mn upfront and milestone payments received; USD 1.1bn in potential milestones remaining

### Our Global Partnerships: Cresemba



### Our Global Partnerships: Zevtera





# Antifungal

## Cresemba® (isavuconazole)

- Invasive mold infections
- Marketed in North America, Europe and Latin America

# Invasive fungal infections — An area of continued high unmet medical need

- 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\*\*

CANDIDA  
23 – 40%



ASPERGILLUS  
34 – 58%



MUCORALES  
40 – 80%

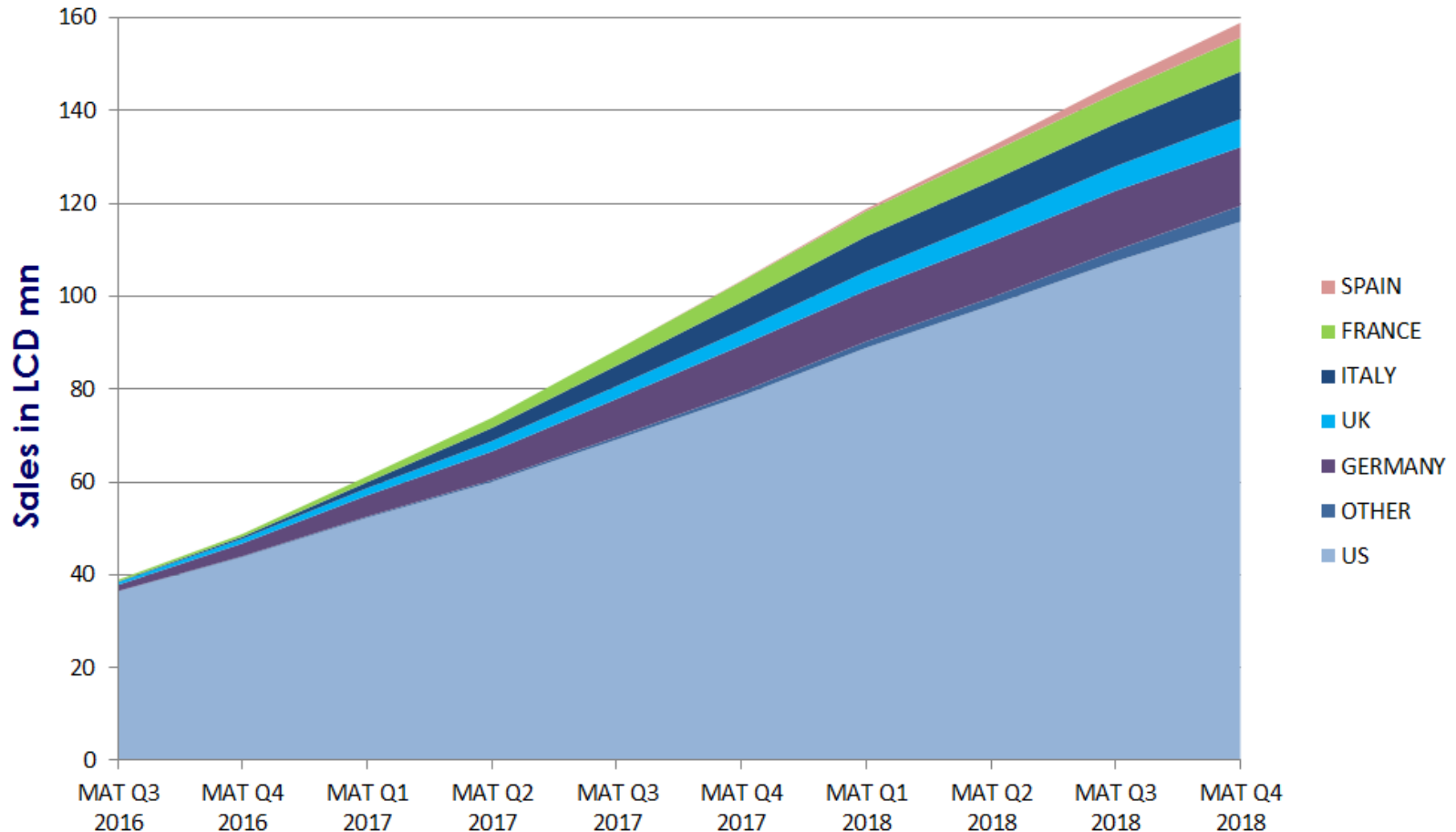


\*\*Kullberg/Arendrup *N Engl J Med* 2015, Baddley *Clin Infect Dis* 2010, Roden *Clin Infect Dis* 2005, Greenberg *Curr Opin Infect Dis* 2004



# Cresemba continues strong sales uptake in established and new markets

Approx. USD 160mn in-market sales in 2018

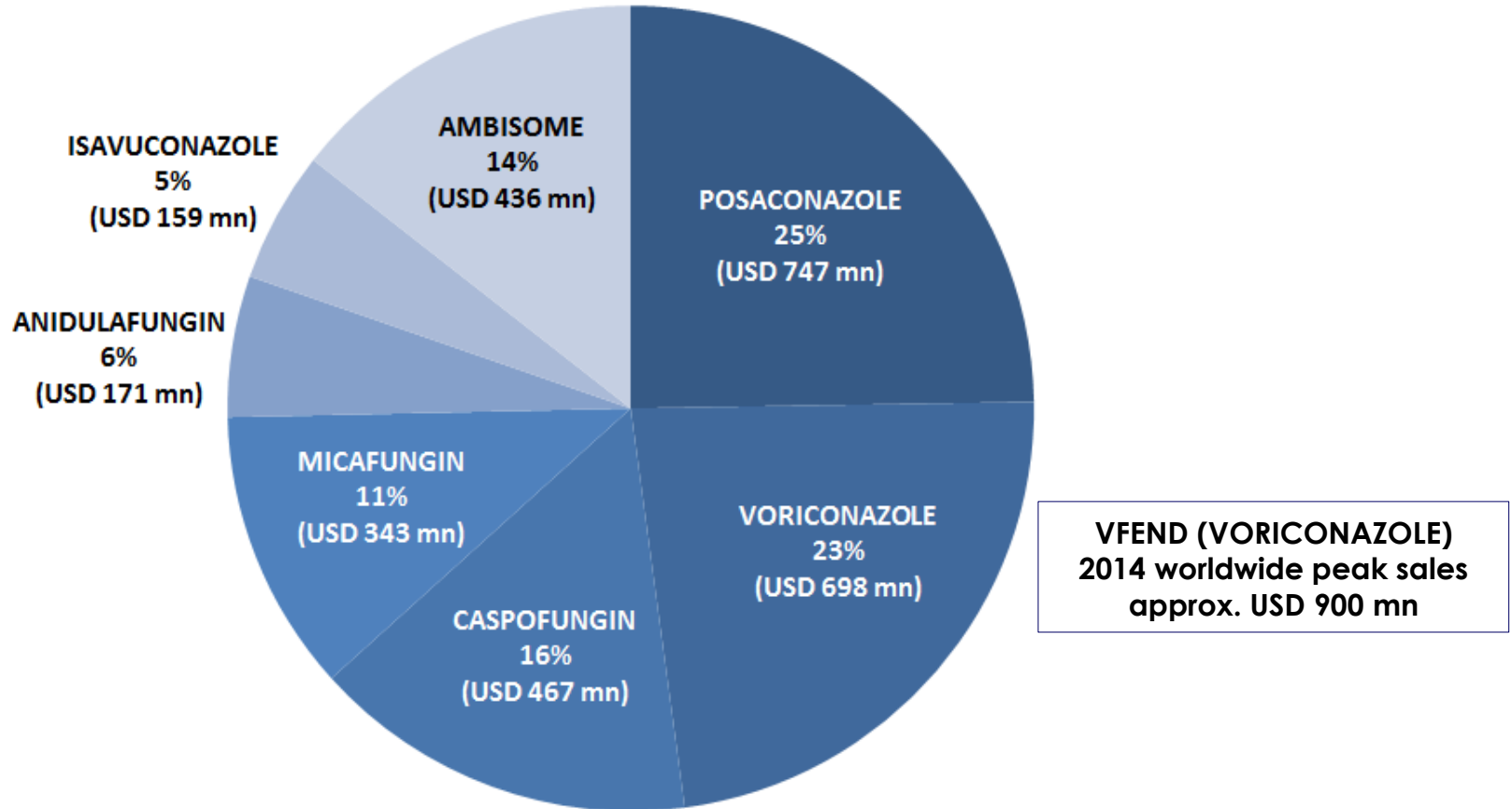


LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, December 2018



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

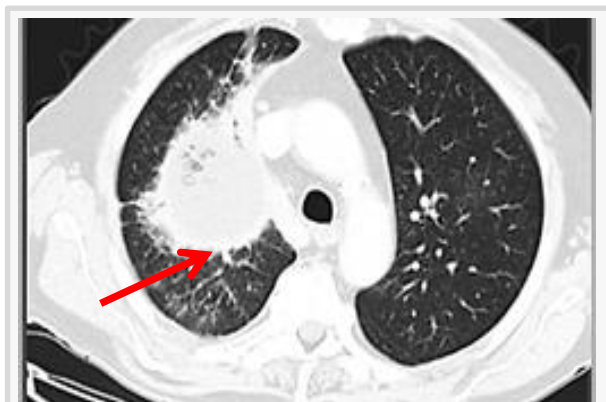
USD 3bn sales (MAT Q4 2018)



\* 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 2018



# Cresemba — Differentiated by spectrum, safety and tolerability



CT scan of patient with fungal pneumonia

- 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 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.

ECIL: The European Conference on Infections in Leukaemia



# Cresemba — Marketed in North America, Europe, Latin America and further country launches planned



For full U.S. prescribing information see:  
[www.cresemba.com](http://www.cresemba.com)

**Approved in Europe for the treatment of adults with:**  
invasive aspergillosis and mucormycosis for whom  
amphotericin B is inappropriate

**Approved in the U.S. for the treatment of adults with:**  
invasive aspergillosis and invasive mucormycosis

- Marketed in major European countries by Pfizer
  - USD 5mn sales milestone triggered in Q1 2019
- Marketed in the U.S. by Astellas
  - Astellas reported FY18 (ended 03/19) sales of USD 119mn (+37% Y-o-Y)
  - CHF 10mn sales milestone triggered in Q4 2018
- Anticipated to double the number of launched countries by end-2019
- Exclusivity through 2027 in the U.S. and potential pediatric exclusivity extension to 2027 (from 2025) in the EU





## Antibacterial

### Zevtera®/Mabelio® (ceftobiprole)

European box/vials  
Ceftobiprole is not approved in the U.S.

- Hospital\* and community-acquired pneumonia
- Marketed in major European countries, Argentina, Canada , Peru and Saudi-Arabia

\* HAP (excluding VAP)

# Zevtera/Mabelio — A fast-acting hospital antibiotic with activity against a broad range of bacteria



Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-acquired pneumonia (VAP), and community-acquired pneumonia (CAP)

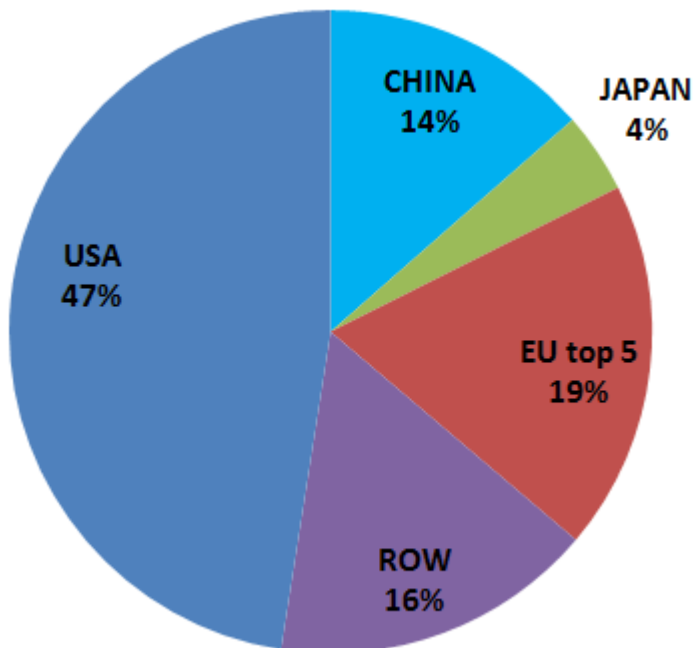
Not approved in the U.S.

- 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 major European markets, Argentina, Canada, Peru and Saudi Arabia

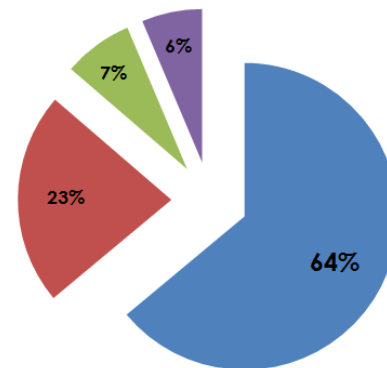


# Anti-MRSA hospital antibiotics market — A USD 3.1bn market with the U.S. being the most important region

## Global anti-MRSA hospital antibiotics sales\* USD 3.1bn (MAT Q4 2018)

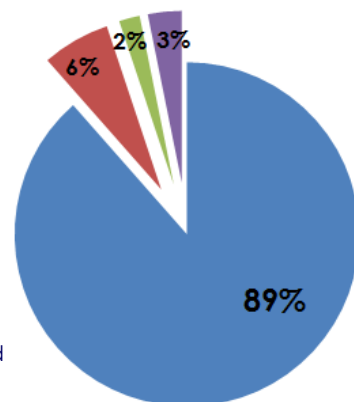


## Linezolid sales by region 2014 (before LOE)



■ USA ■ EU 5 ■ Japan ■ ROW

## Daptomycin sales by region 2015 (before LOE)



\* 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 2018



# Ceftobiprole — Strategy for accessing the important U.S. market providing attractive risk-return profile



\*The project is funded in part with Federal funds from the Department of Health and Human Services; Office of the Assistant Secretary for Preparedness and Response; Biomedical Advanced Research and Development Authority (BARDA) under Contract No. HSO100201600002C



**SAB:** *Staphylococcus aureus* bacteremia; **ABSSSI:** acute bacterial skin and skin structure infection; **CABP:** community-acquired bacterial pneumonia

- U.S. registration requires two cross-supportive phase 3 studies
  - FDA has approved Special Protocol Assessments for ABSSSI and SAB phase 3 studies
  - ABSSSI and SAB studies ongoing
- Few approved SAB agents available, with limitations, mainly related to resistance or tolerability
- For SAB, ceftobiprole has potential to be positioned as a rapidly cidal agent against both MSSA and MRSA with the favourable safety profile of a cephalosporin
- BARDA funding of up to USD 128mn (~70% of the total estimated program costs) to support U.S. phase 3 program\*
- QIDP designation (SAB, ABSSSI, CABP): exclusivity extended to 10 years upon approval





# Oncology

## Derazantinib (BAL087)

panFGFR kinase inhibitor  
for various solid tumors

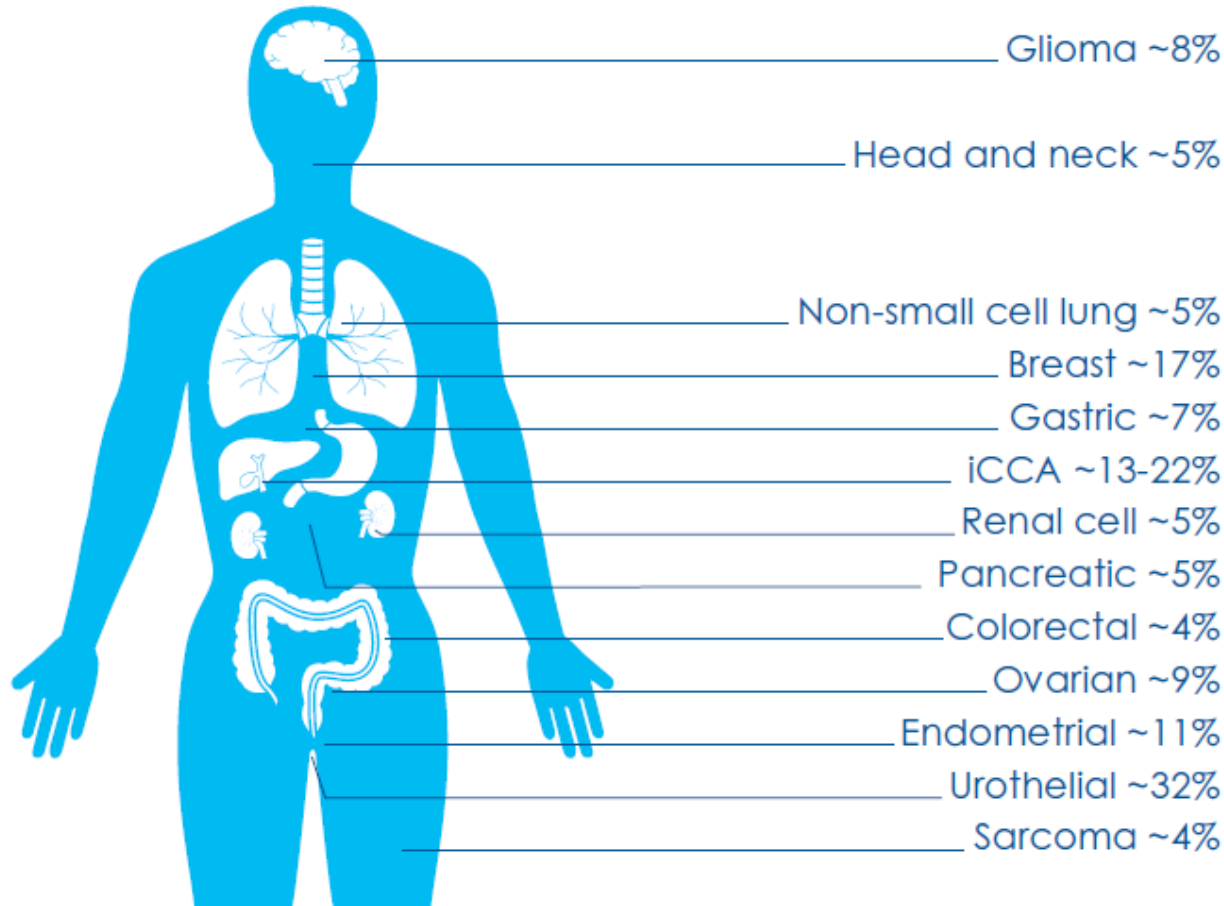
# Derazantinib — targeting FGFR-driven tumors as single agent and in combination with immunotherapy

- Small molecule, oral inhibitor of Fibroblast Growth Factor Receptor (FGFR) family of kinases in-licensed from ArQule Inc.
  - panFGFR kinase inhibitor with strongest activity against FGFR1, 2 and 3
  - Exploring therapeutic potential of additional targets of derazantinib, including targets not addressed by other selective FGFR inhibitors, such as CSF1R (Colony-stimulating Factor 1 Receptor) kinase
- Strong data foundation generated to support potential accelerated FDA approval in intrahepatic cholangiocarcinoma (iCCA), an indication with high unmet need and globally increasing incidence
- Orphan drug designation in iCCA granted by FDA and EMA
- Collaboration with Roche to study derazantinib and immune-checkpoint inhibitor atezolizumab (Tecentriq®) in a clinical study in urothelial cancer



# Derazantinib — Significant potential beyond iCCA and urothelial cancer

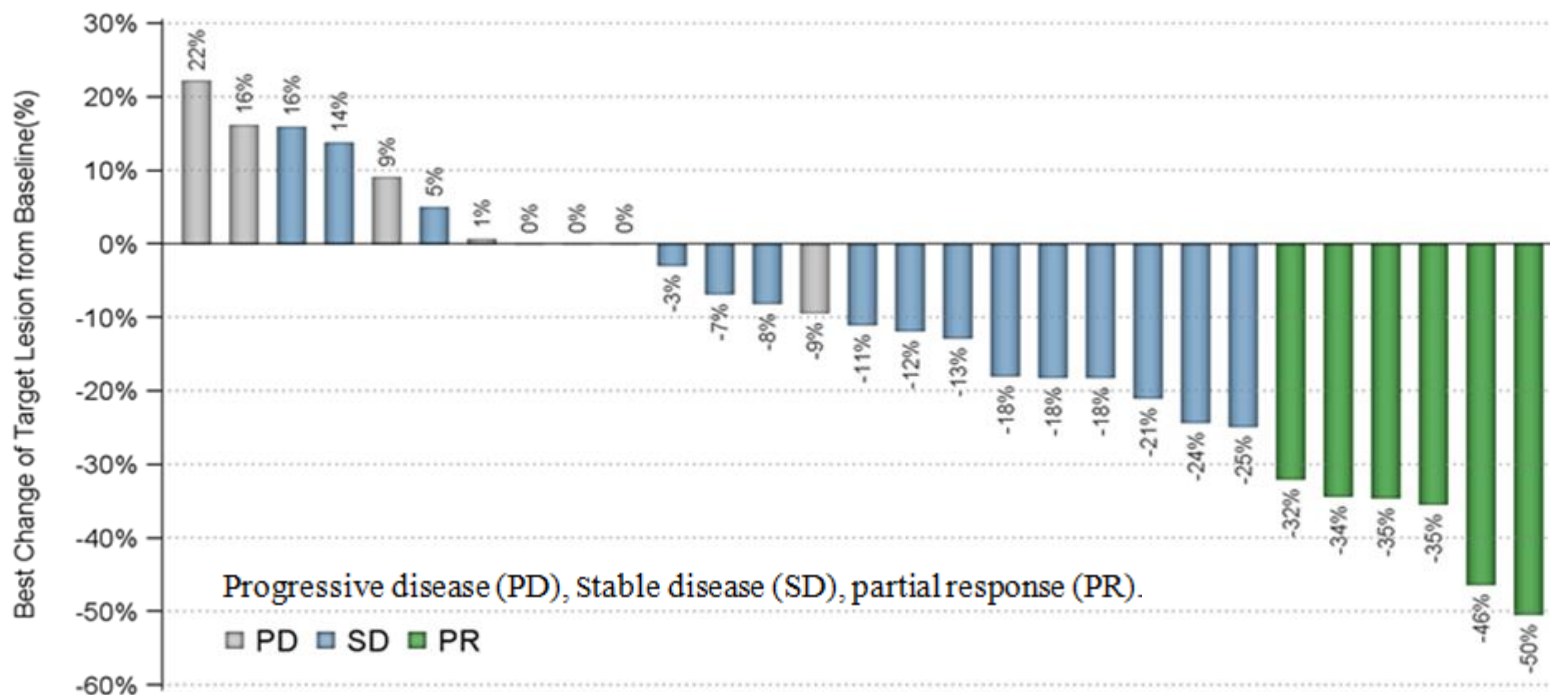
## Frequency of FGFR aberrations across different tumor types



Source:  
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



# Derazantinib — Established proof-of-concept in iCCA in phase 1/2a study



- Subgroup analysis of 29 patients with FGFR2-fusion positive iCCA:
  - Objective response rate of 21%
  - In 72% of patients, tumor response or disease stabilization for  $\geq 16$  weeks was achieved\*
  - Manageable safety profile

Sources: Mazzaferro et al. British Journal of Cancer 2018; \*Mazzaferro et al. J Clin Oncol 2017;35 suppl: abstract 4017



# Derazantinib — potential for accelerated approval with solid clinical data in iCCA

## Favorable clinical data from completed phase 1/2 study

- Promising anti-tumor efficacy and clinical safety shown in biomarker-driven clinical study in patients with FGFR2-gene-fusion expressing iCCA
- Derazantinib efficacy compares favorably to standard-of-care (SoC) chemotherapy (cross-trial comparison)
  - Objective Response Rate (ORR) 21% for derazantinib<sup>1</sup> versus <10% for SoC<sup>2,3</sup>
  - Progression-Free Survival (PFS) approx. 6 months<sup>1</sup> versus 3 months for SoC<sup>2,3</sup>
- Manageable safety profile and low discontinuation rate<sup>1,4</sup>

## Registrational phase 2 study, ongoing

- Patients with FGFR2-gene-fusion expressing iCCA (2nd-line)
- Encouraging interim results reported early 2019, consistent with the earlier phase 1/2 data
- 21% ORR with six confirmed partial responses from 29 evaluable patients, 83% disease control rate
- Safety profile and tolerability of continuous dosing schedule confirmed
- Final data to be presented mid-2020

### Sources:

- 1 V. Mazzaferro et al. Derazantinib (ARQ 087) in advanced or inoperable FGFR2 gene fusion-positive intrahepatic cholangiocarcinoma. *British Journal of Cancer* 2018
- 2 A. Lamarca et al. Second-line chemotherapy in advanced biliary cancer: a systematic review. *Annals of Oncology* 2014 (25), 2328-2338 ;
- 3 L. Fornaro et al. Second-line chemotherapy in advanced biliary cancer progressed to first-line platinum-gemcitabine combination: a multicenter survey and pooled analysis with published data. *Journal of Experimental & Clinical Cancer Research* 2015 (34), 156
- 4 K. P. Papadopoulos et al. A phase 1 study of ARQ 087, an oral pan-FGFR inhibitor in patients with advanced solid tumours. *British Journal of Cancer* 2017 , 1-8



# FGFR-inhibitors show differences in safety profiles

	Cholangiocarcinoma				Urothelial cancer	
	DZB <sup>1</sup> (N=29)	INF <sup>2</sup> (N=71)	FUT <sup>3</sup> (N=45)	PEM <sup>4</sup> (N=89)	PEM <sup>5</sup> (N=108)	ERD <sup>6*</sup> (N=99)
<b>Dosing regimen</b>	300mg QD	125mg Q4W QD for 3w	16 mg, 20 mg or 24 mg QD	13.5mg Q3W QD for 2w	13.5mg Q3W QD for 2w	8 mg QD (titr. to 9mg)
<b>Most frequent AEs</b>	Phosphorus↑ Dry mouth Nausea	Phosphorus↑ Fatigue Stomatitis	Phosphorus↑ Constipation AST↑	Phosphorus↑ Alopecia Diarrhoea	Diarrhoea Alopecia Constipation	Phosphorus↑ Stomatitis Dry mouth
<b>Blood phosphorus</b> ↑†	<b>76%</b>	<b>73%</b>	<b>80%</b>	<b>61%</b>	<b>31%</b>	<b>73%</b>
<b>Fatigue</b> † [G3]	<b>41%</b> [3%]	<b>49%</b> [4%]	NR	<b>36%</b> [4%]	<b>32%</b> [6%]	<b>≥21%</b> [≥2%]
<b>Alopecia</b> †	<b>28%</b>	<b>38%</b>	NR	<b>37%</b>	NR	<b>≥27%</b>
<b>Dry eye/xerophthalmia</b> †	<b>21%</b>	<b>32%</b>	NR	<b>20%</b>	NR	<b>≥19%</b>
<b>Central serous retinopathy</b>	<b>0%</b>	NR	NR	NR	NR	<b>21%</b>
<b>ALT</b> ↑	<b>31%</b>	NR	<b>31%</b>	NR	NR	<b>41%</b> <sup>7</sup>
<b>Hand-foot syndrome/PPE</b>	<b>0%</b>	<b>27%</b>	<b>22%</b>	NR	NR	<b>≥22%</b>
<b>Nail events (drug-related)</b>	<b>&lt;5%</b>	NR	NR	NR	NR	<b>52%</b>
<b>Stomatitis</b>	<b>7%</b>	<b>45%</b>	<b>22%</b>	<b>30%</b>	<b>34%</b>	<b>≥55%</b>

Sources: <sup>1</sup>Mazzaferro et al., Br J Cancer 2018 and Basilea data on file; <sup>2</sup>Javle et al., ESMO 2018; <sup>3</sup>Meric-Bernstam et al, ESMO WC GI Cancer, 2018;

<sup>4</sup>Hollebecque, et al., ESMO 2018; <sup>5</sup>Necchi, et al., ESMO 2018; <sup>6</sup>Siefker-Radtke et al., ASCO 2018; <sup>7</sup>Balversa™ US prescribing information (April 2019) based on reported laboratory abnormalities N=86 patients, regardless of causality.

Abbreviations: DZB: derazantinib, INF: infigratinib (BJG398), 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.

\*Drug-related events reported only; †assumed FGFR inhibitor class-effect



# FGFR-inhibitors show differences in kinase-inhibition profiles

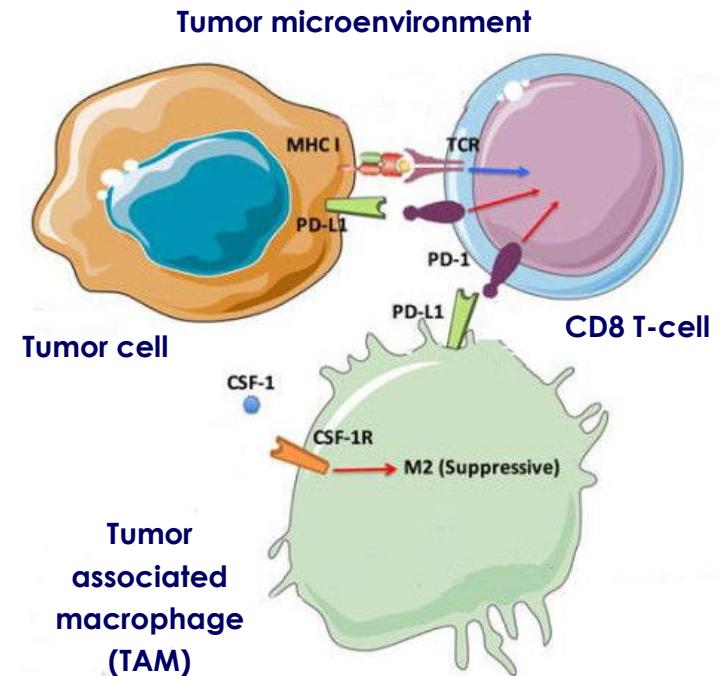
FGFR-inhibitor compound (Sponsor)	Parameter	FGFR1	FGFR2	FGFR3	FGFR4	CSF1R (FMS)
<b>Derazantinib</b> (Basilea)	Ratio to FGFR2 activity	4	1	4	77	3
<b>Pemigatinib</b> (Incyte)	Ratio to FGFR2 activity	3	1	4	39	231
<b>Erdafitinib</b> (Janssen)	Ratio to FGFR2 activity	2	1	2	13	95
<b>Rogaratinib</b> (Bayer)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

Source: Basilea data on file



# 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 inhibitors<sup>1</sup>
- 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 collaboration with Roche to study a combination of derazantinib and Roche's PD-L1-blocking immune-checkpoint inhibitor atezolizumab in patients with urothelial cancer



Blocking CSF1/CSF1R has the potential to reprogram tumor-promoting macrophages and enhance the response to immune checkpoint (PD1/PD-L1) inhibitors.<sup>2</sup>

Sources: 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  
2. 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/atezolizumab - a potential unique FGFR/IO combination in urothelial cancer

- Among FGFR-inhibitors, CSF1R inhibition seems unique to derazantinib
- In urothelial cancer, Keytruda® and Tecentriq® received label restrictions on the use for first-line treatment of patients with low PD-L1 expression
  - This subgroup of tumors shows frequent FGFR genomic abnormalities (mainly FGFR3 fusions)
  - Derazantinib combined with PD1/PD-L1 inhibitors may therefore provide benefits related to multiple mechanisms (FGFR inhibition, macrophage inhibition, enhanced response to immunotherapy) in this group of patients
- A phase 1/2 study exploring derazantinib as monotherapy and in combination with Tecentriq® anticipated to start mid-2019





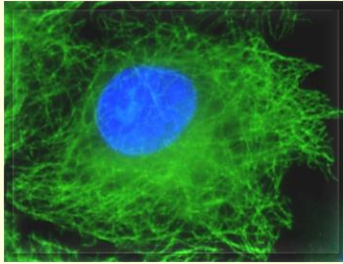
# Oncology

## BAL101553

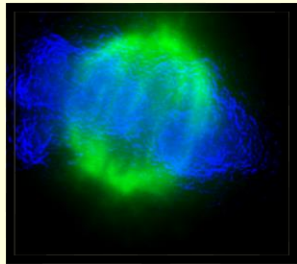
Treatment-refractory solid tumors,  
including glioblastoma

# BAL101553 — Novel tumor checkpoint controller crossing the blood-brain barrier

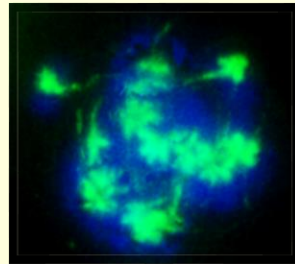
Bachmann AACR 2015



Non-dividing tumor cell



Normal dividing tumor cell



BAL27862-treated tumor cell\*

Blue = DNA  
Green = microtubules

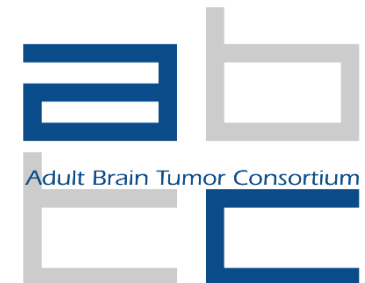
- Novel compound inducing tumor cell death through checkpoint activation
- Destabilizing the microtubule scaffold through a novel target-binding site
- Targeting diverse tumor types resistant to standard therapeutic approaches
- Crosses the blood-brain barrier with potent activity in brain tumor models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

\* BAL101553 is a prodrug of BAL27862

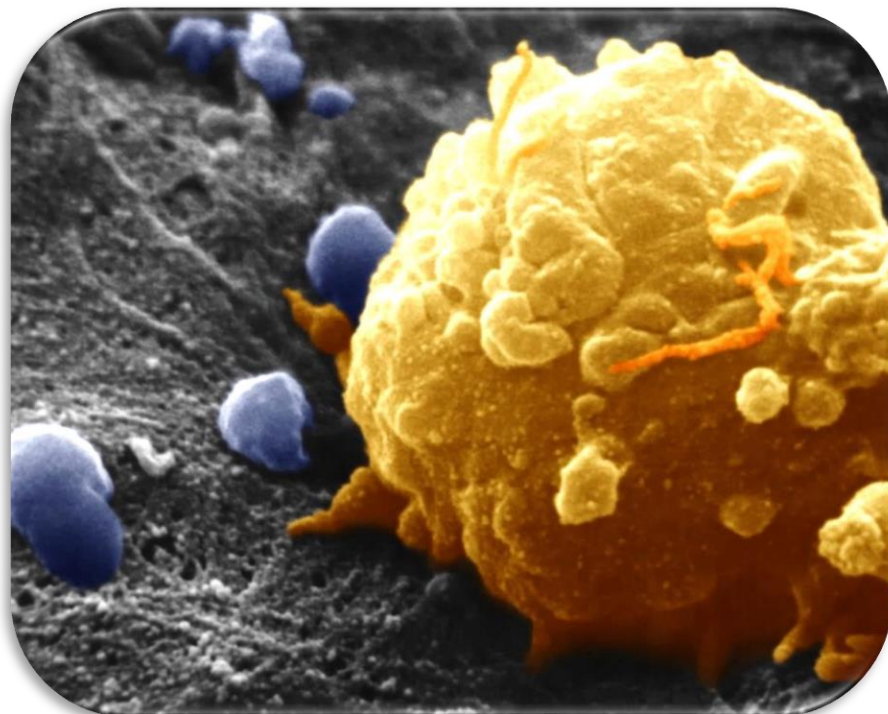


# BAL101553 — three ongoing clinical studies

- Phase 2a expansion (weekly 48-hour i.v.) in patients with recurrent glioblastoma or platinum-resistant ovarian cancer
  - Anticipated to complete around year-end 2019
- Phase 1 dose escalation (daily oral) in patients with recurrent glioblastoma
  - Anticipated to complete in mid-2019
- Phase 1 study (daily oral) in combination with radiotherapy in patients with newly diagnosed glioblastoma in collaboration with the Adult Brain Tumor Consortium (ABTC)<sup>1</sup>
  - Anticipated to complete patient enrolment mid-2020



<sup>1</sup>The ABTC is funded by the U.S. National Cancer Institute (NCI)

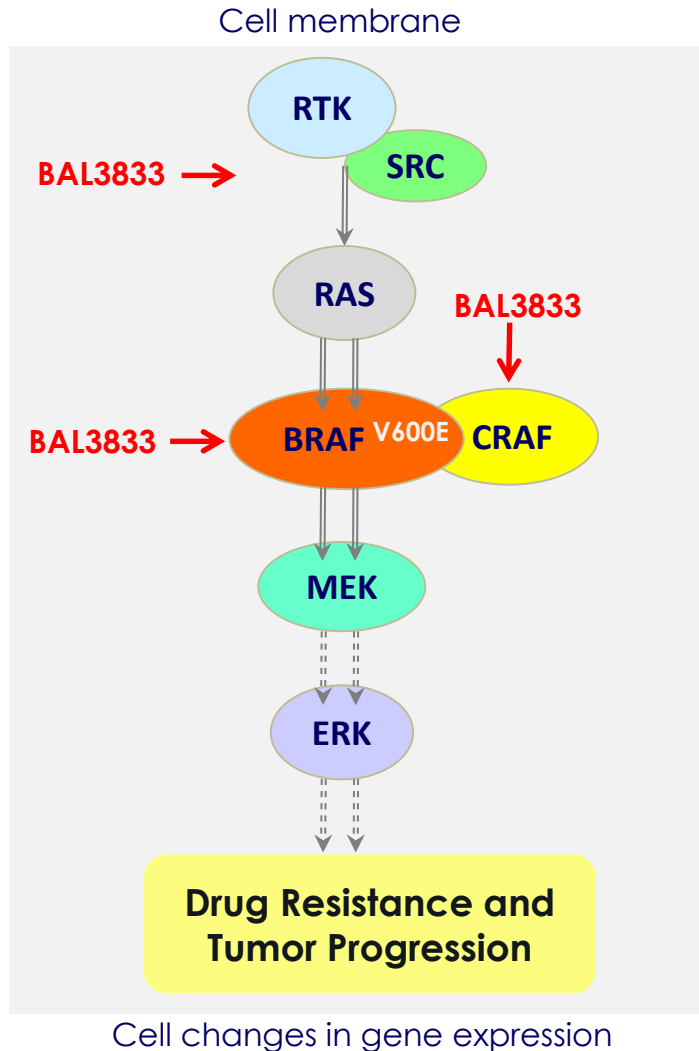


# Oncology

BAL3833

Treatment-refractory solid tumors,  
including metastatic melanoma  
and RAS-driven tumors

# BAL3833 — panRAF/SRC kinase inhibitor

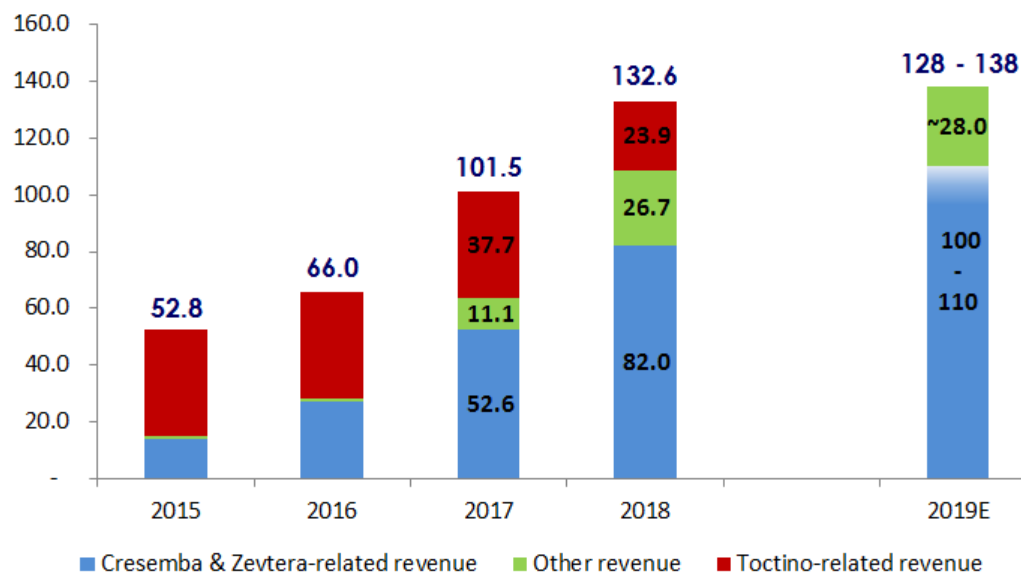


- In-licensed novel, oral, small molecule drug from consortium around Wellcome Trust & Institute of Cancer Research (ICR)
- Dual-targeting kinase inhibitor
- Targets resistance mechanisms associated with approved BRAF inhibitors (including vemurafenib and dabrafenib)
- Resistance-reversal activity in BRAF/MEK inhibitor- and immunotherapy-resistant melanoma models
- Potential in diverse non-melanoma tumor types:
  - e.g. RAS-driven tumors
  - Expanded biomarker program to aid tumor selection
- Phase 1 dose-escalation study completed
  - Broad dose range investigated, maximum tolerated dose (MTD) was not defined
  - Pre-clinical activities ongoing to explore alternative formulations

# Key financials 2018 and 2019 guidance

In CHF mn	FY 2019 guidance	FY 2018 actuals
Total revenue	128 - 138	132.6
<i>thereof: Contributions from Cresemba &amp; Zevtera</i>	<i>100 - 110</i>	<i>82.0</i>
Operating loss	20 - 30	24.1
Net operating cash consumption	55 - 65	79.2
Year-end cash and financial investments	n/a	223.0

## Strong increase in Cresemba & Zevtera revenue contributions Y-o-Y, CHF mn



# Focus 2019 and beyond

**Cresemba® & Zevtera®/Mabelio®** Increasing cash-generating revenues  
By the end of 2021, Cresemba to be on the market in >60 countries

**H1 2019**

**H2 2019**

**H1 2020**

**H2 2020**

**Ceftobiprole**

Top line results from phase 3 ABSSSI study

**Derazantinib**

✓ Interim analysis of phase 2 registrational study in iCCA FGFR2 fusions

✓ Collaboration with Roche in urothelial cancer

Expand ongoing iCCA study in other FGFR gene aberrations

Complete patient enrolment in phase 2 registrational study in iCCA

Start phase 2 study in urothelial cancer

Top line results from phase 2 registrational study in iCCA

Interim data from first cohort(s) in urothelial cancer

Interim data from iCCA in other FGFR gene aberrations

**BAL101553**

Complete patient enrolment in phase 1 study arm for recurrent glioblastoma (oral)

Top line results from phase 2a study in ovarian cancer and glioblastoma (48-hr. i.v.)

Complete patient enrolment in phase 1 study in newly diagnosed glioblastoma







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