



P H A R M A C E U T I C A

Disclaimer and forward-looking statements

Forward looking statements

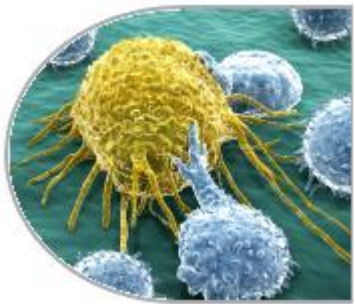
This communication including the accompanying oral presentation contains certain forward-looking statements, including, without limitation, statements containing the words “believes”, “anticipates”, “expects”, “supposes”, “considers” and words of similar import or which can be identified as discussions 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



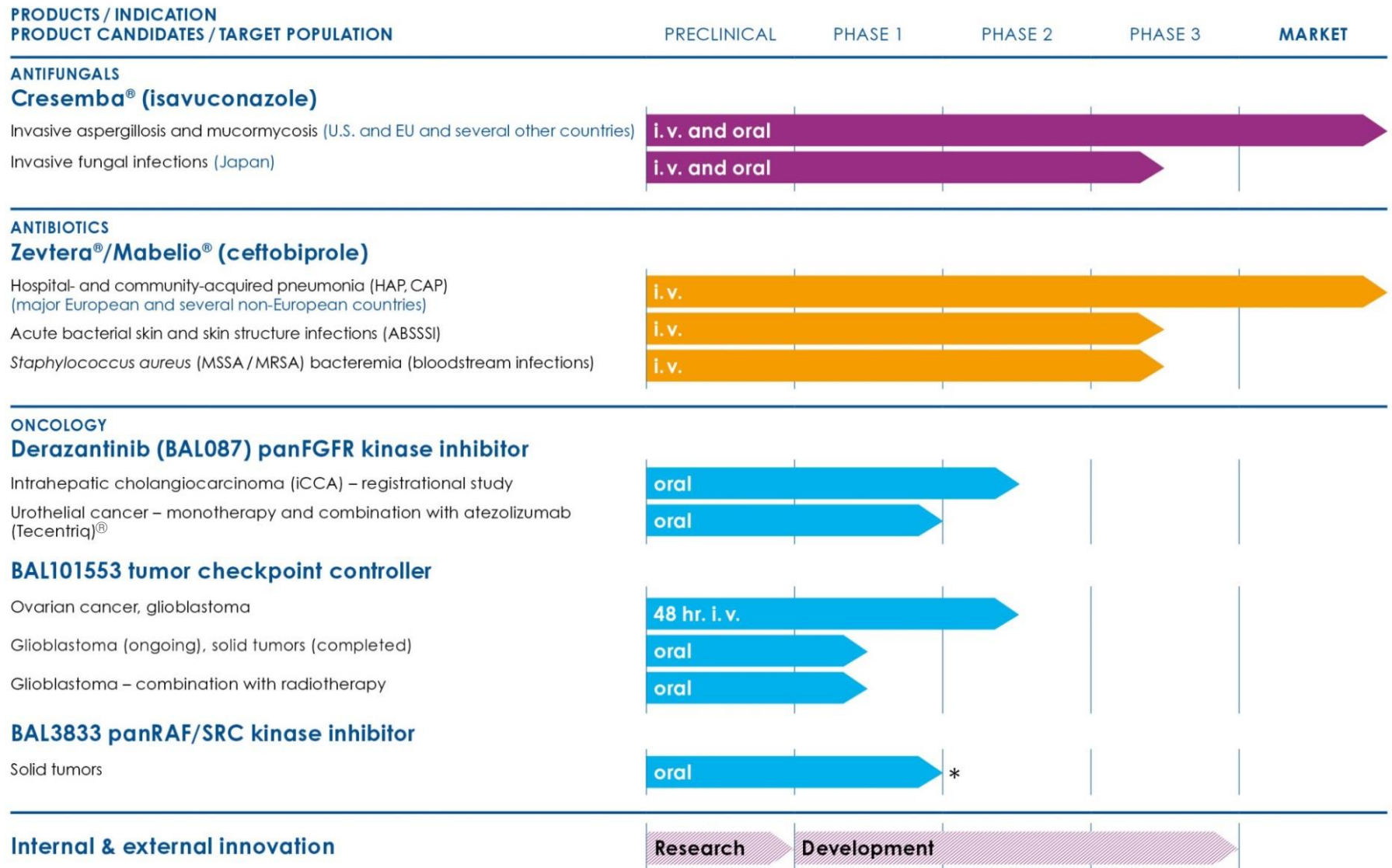
- Two marketed anti-infective brands (Cresemba and Zevtera) and three oncology drug candidates in development
- Potential for sustainable growth and value generation based on increasing revenues and selective investments into internal and external innovation



- 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); approx. 225 employees



Potential for sustainable growth and value creation based on commercialized products and differentiated 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*)



Distribution partners

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

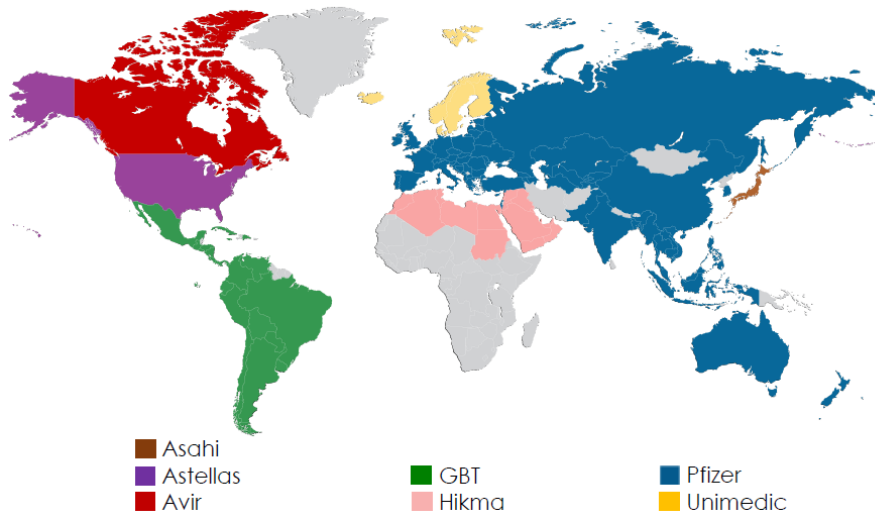


>100 countries covered by partnerships — USD 1.1bn in total potential milestones

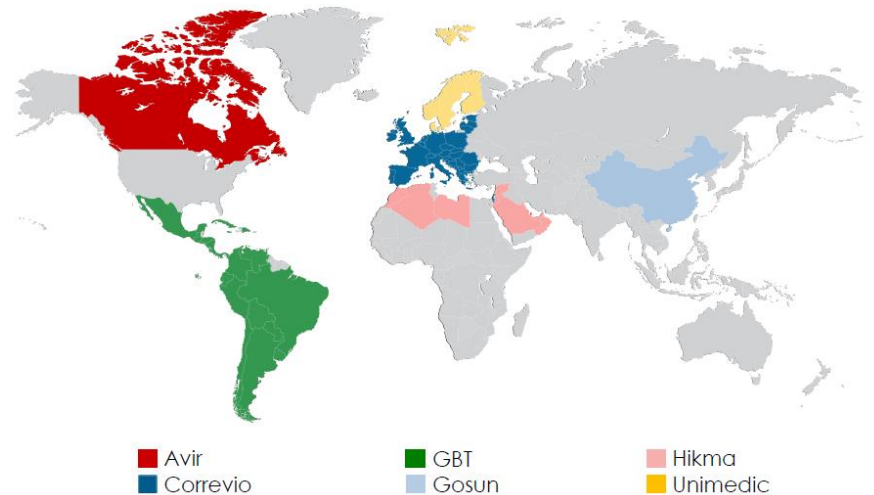
Ongoing participation

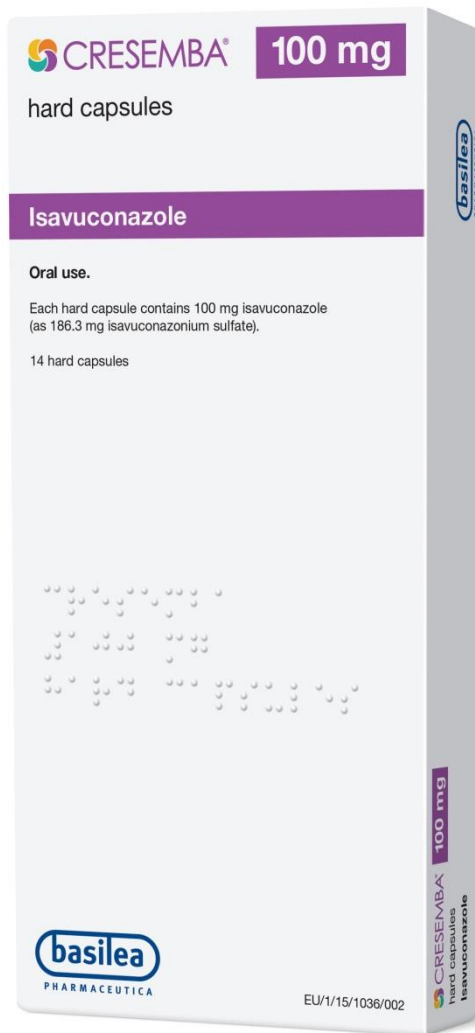
- Double-digit royalties on sales by license partners
- Participation through transfer price structure in sales by distribution partners
- Approximately USD 245mn upfront and milestone payments received; USD 1.1bn in potential milestones remaining

Our Global Partnerships: Cresemba



Our Global Partnerships: Zevtera





Antifungal

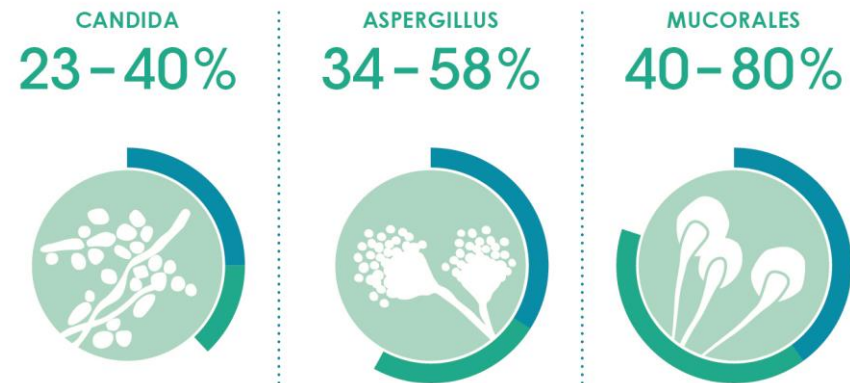
Cresemba® (isavuconazole)

- Invasive mold infections
- Marketed in the U.S. and Europe

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

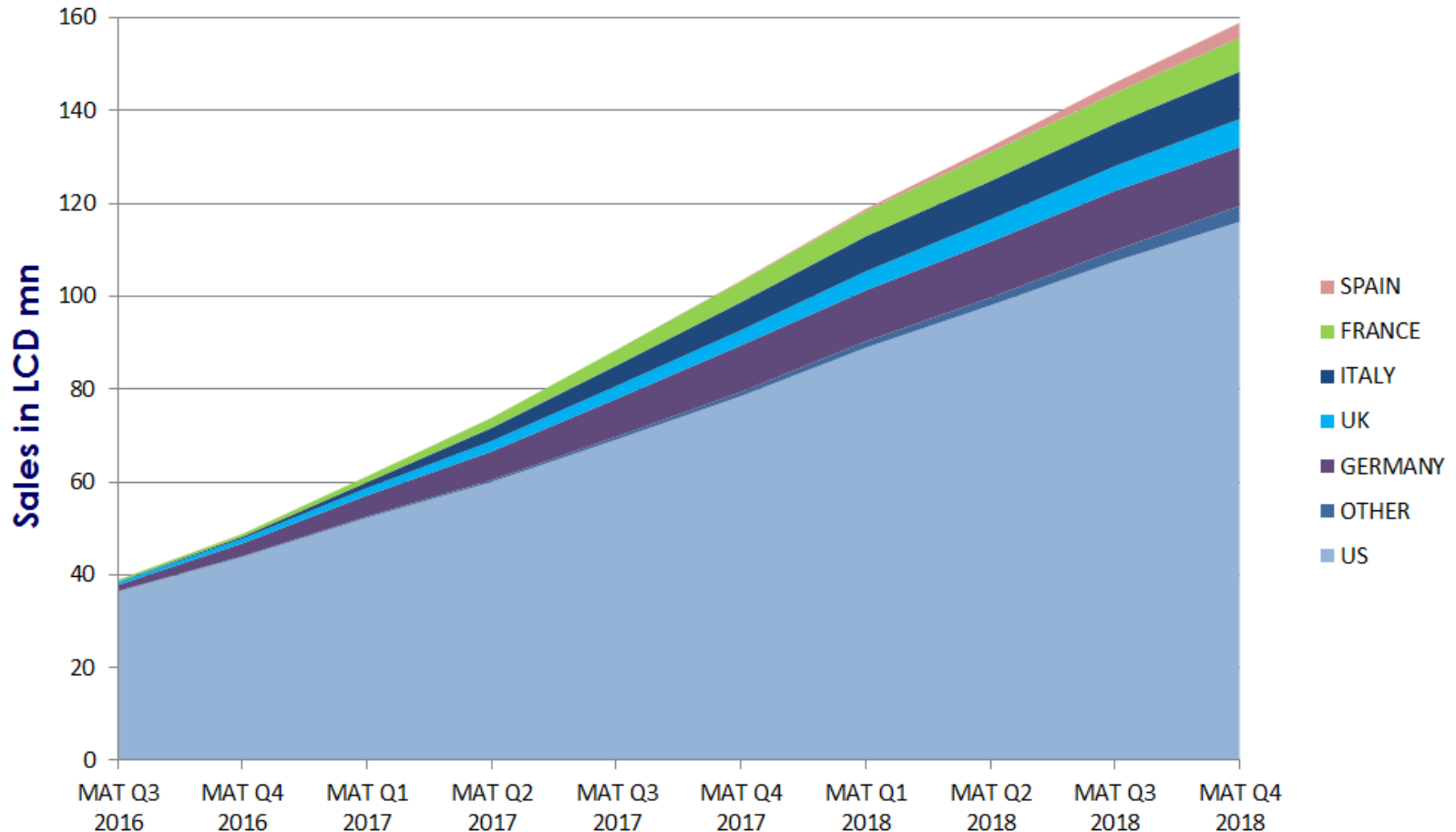
Mortality rates for invasive fungal infections**



**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

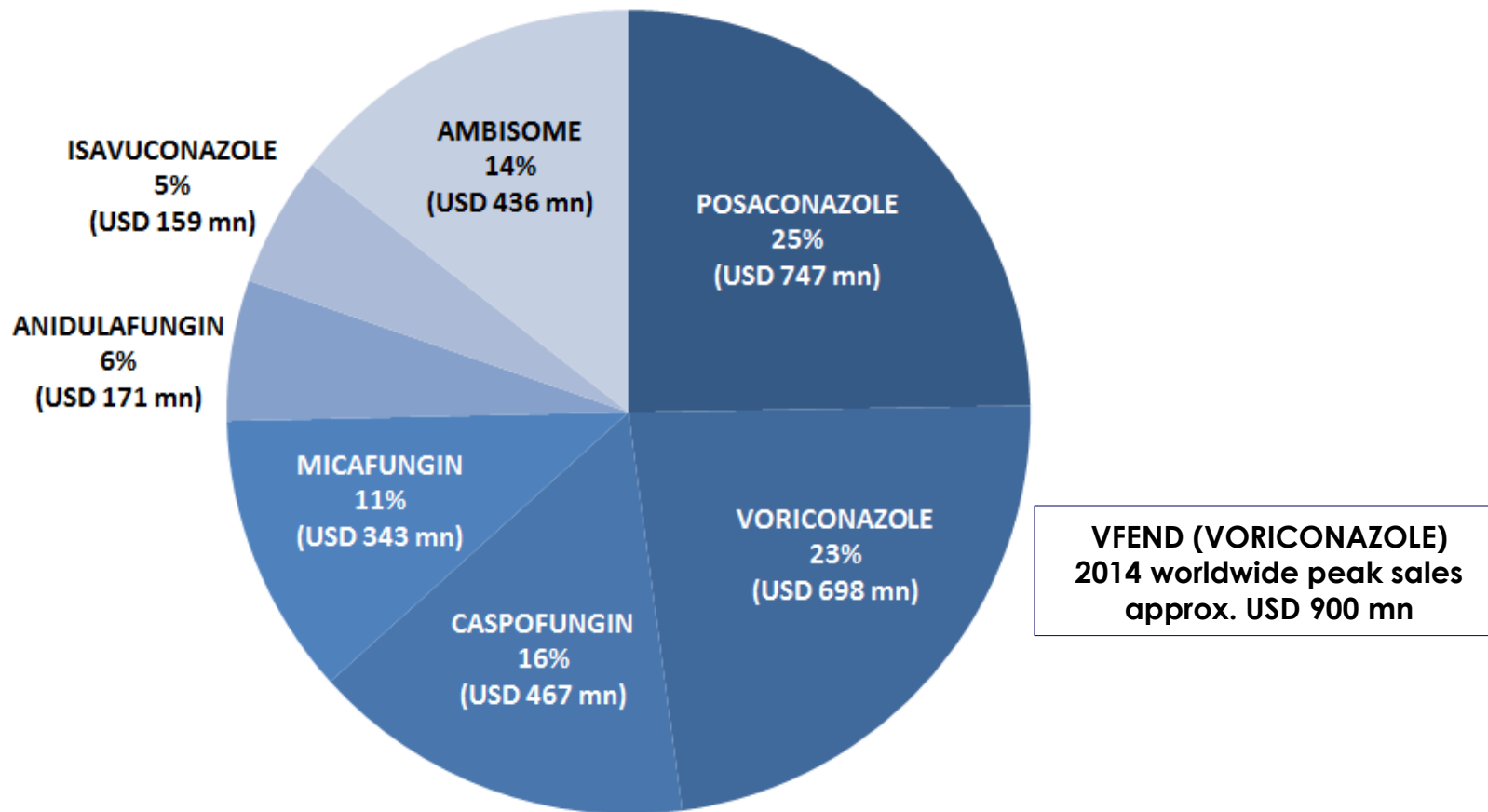


LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, December 2018



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 the EU and U.S. and further country launches planned



- Marketed in major European countries by Pfizer
 - USD 5mn sales milestone triggered in Q1 2019
- Marketed in the U.S. by Astellas
 - USD 113mn (+47% Y-o-Y) net sales 2018
 - 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

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





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



Antibacterial

Zevtera®/Mabelio® (ceftobiprole)

- 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)

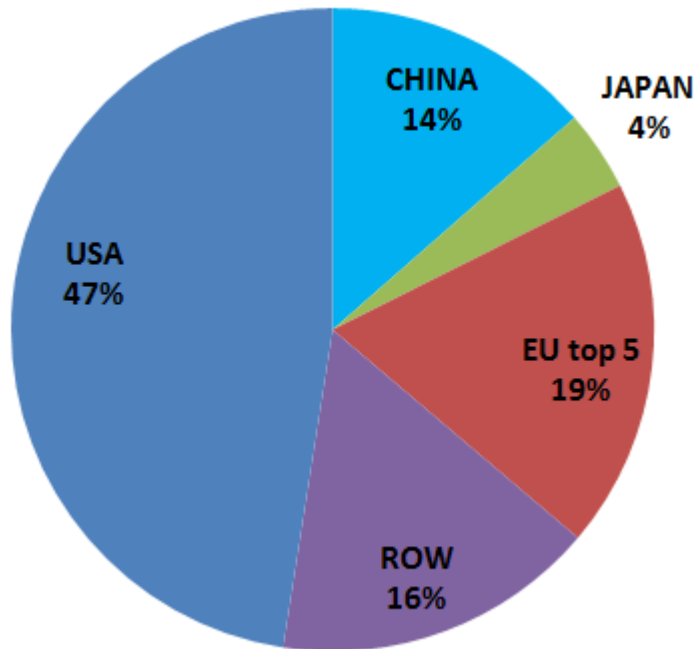
Not approved in the U.S.

- 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

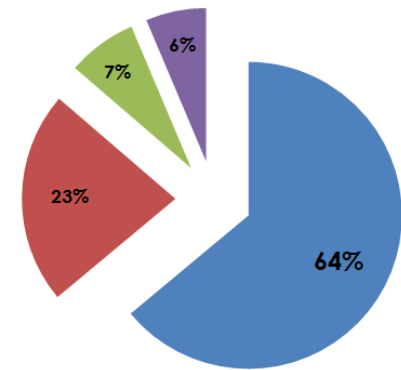


Anti-MRSA hospital antibiotics market — A USD 3.1bn market with the U.S. being the most important region

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

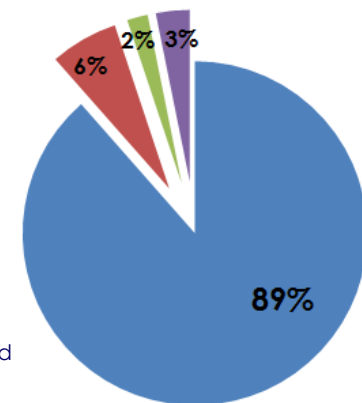


Linezolid sales by region 2014 (before LOE)



■ 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

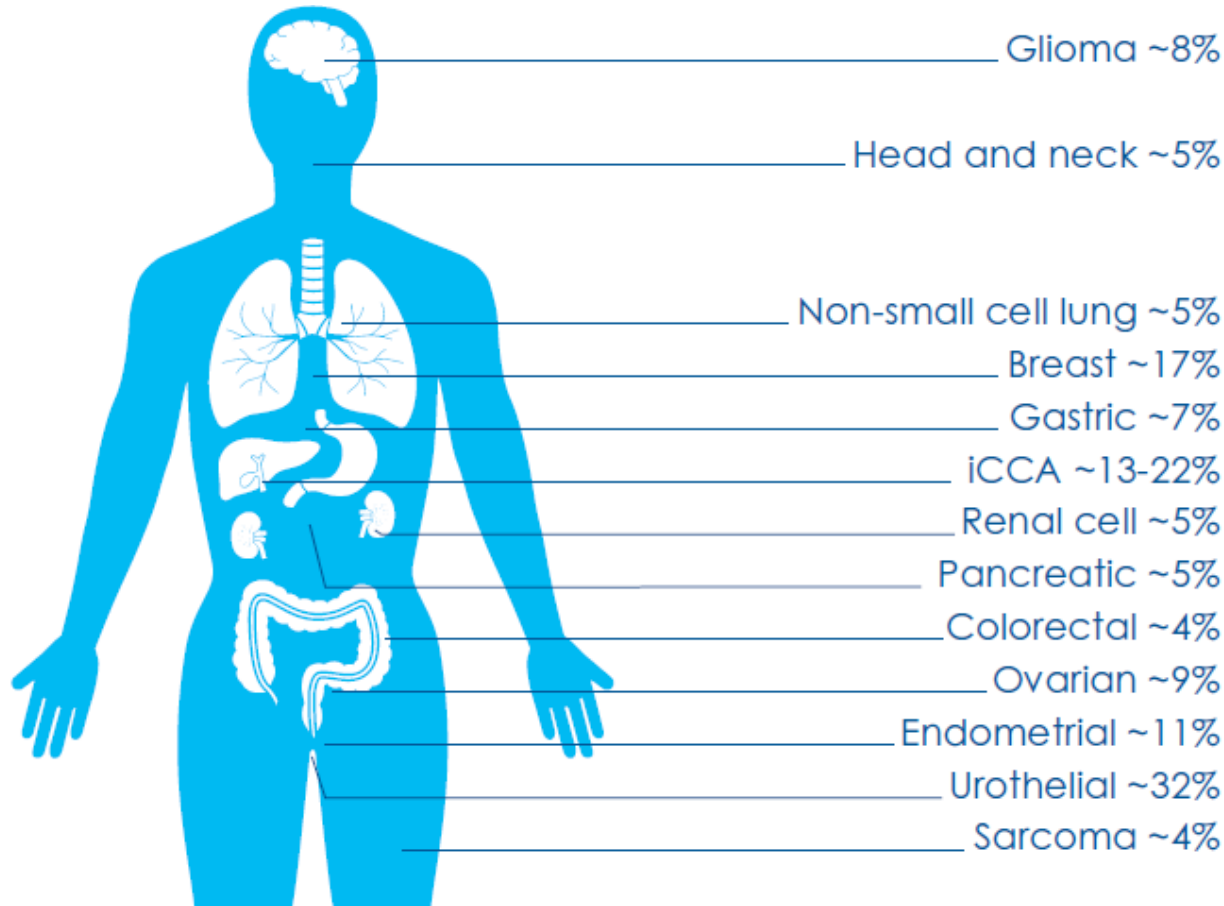
Derazantinib — targeting FGFR-driven tumors as single agent and in combination with immunotherapy

- 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

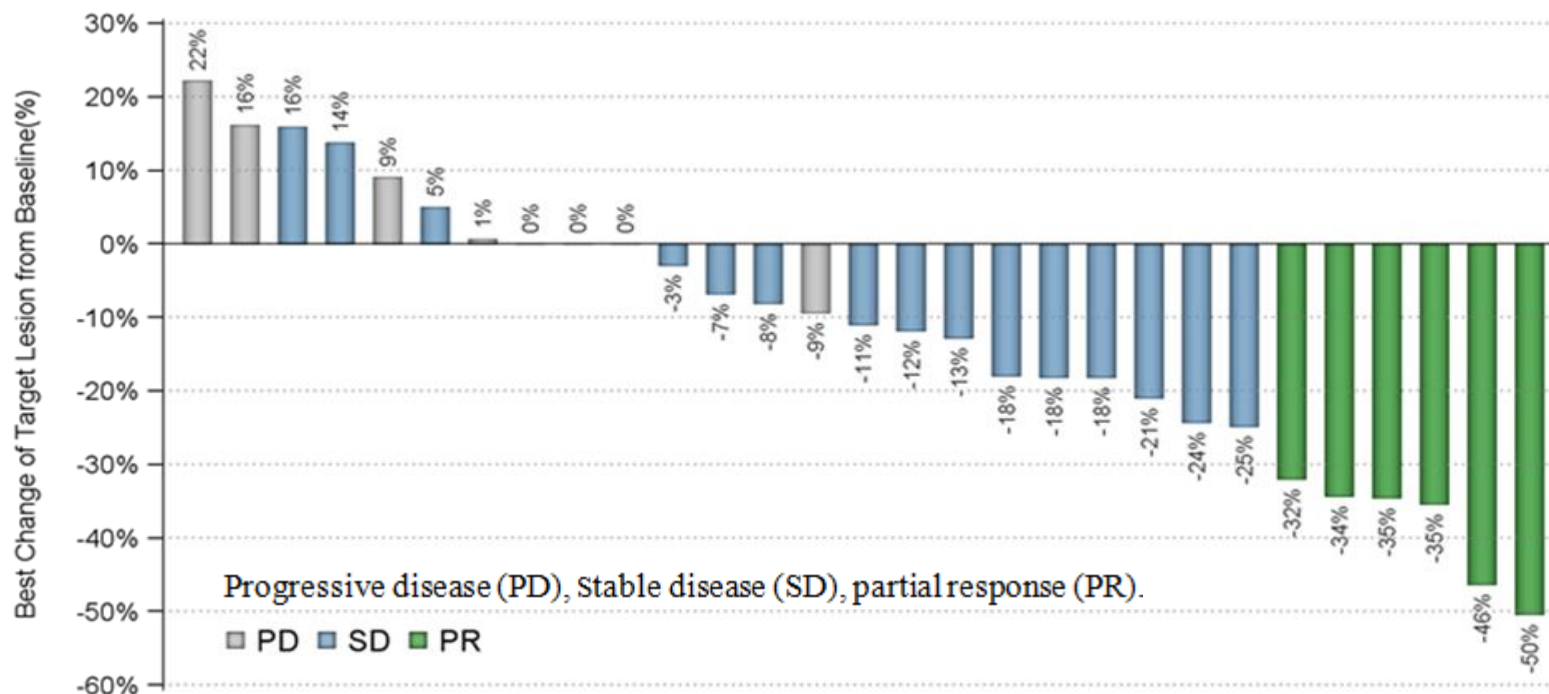


Source:

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

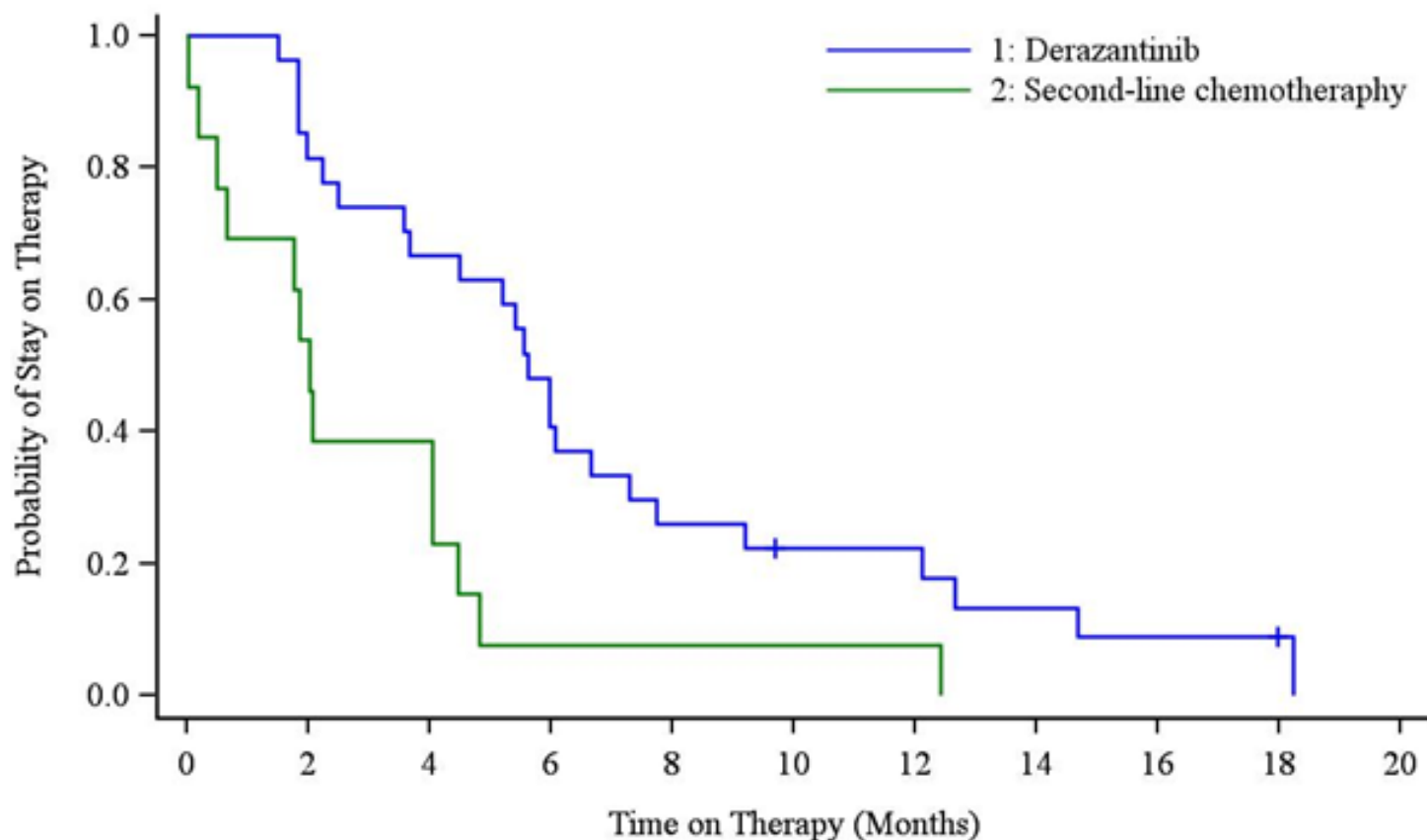


- Subgroup analysis of 29 patients with FGFR2-fusion positive iCCA:
 - Objective response rate of 21%
 - In 72% of patients, tumor response or disease stabilization for ≥ 16 weeks was achieved*
 - Manageable safety profile

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

Derazantinib — Time on treatment supports clinical benefit in FGFR2-fusion positive iCCA

Inpatient comparison of time on study with derazantinib compared to pre-study second-line chemotherapy



Source: Mazzaferro et al. British Journal of Cancer 2018

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 derazantinib¹ versus <10% for SoC^{2,3}
 - Progression-Free Survival (PFS) approx. 6 months¹ versus 3 months for SoC^{2,3}
- Manageable safety profile and low discontinuation rate^{1,4}

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 ¹ (N=29)	INF ² (N=71)	FUT ³ (N=45)	PEM ⁴ (N=89)	PEM ⁵ (N=108)	ERD ^{6*} (N=99)
Dosing regimen	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)
Most frequent AEs	Phosphorus↑ Dry mouth Nausea	Phosphorus↑ Fatigue Stomatitis	Phosphorus↑ Constipation AST↑	Phosphorus↑ Alopecia Diarrhoea	Diarrhoea Alopecia Constipation	Phosphorus↑ Stomatitis Dry mouth
Blood phosphorus↑†	76%	73%	80%	61%	31%	73%
Fatigue† [G3]	41% [3%]	49% [4%]	NR	36% [4%]	32% [6%]	≥21% [≥2%]
Alopecia†	28%	38%	NR	37%	NR	≥27%
Dry eye/xerophthalmia†	21%	32%	NR	20%	NR	≥19%
Central serous retinopathy	0%	NR	NR	NR	NR	21%
ALT ↑	31%	NR	31%	NR	NR	NR
Hand-foot syndrome/PPE	0%	27%	22%	NR	NR	≥22%
Nail events (drug-related)	<5%	NR	NR	NR	NR	52%
Stomatitis	7%	45%	22%	30%	34%	≥55%

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

⁴Hollebecque, et al., ESMO 2018; ⁵Necchi, et al., ESMO 2018; ⁶Siefker-Radtke et al., ASCO 2018

Abbreviations: DZB: derazantinib, INF: infigratinib (BJG398), FUT: futibatinib (TAS-120), PEM: pemigatinib (INC854828), ERD: erdafitinib;

PPE: 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

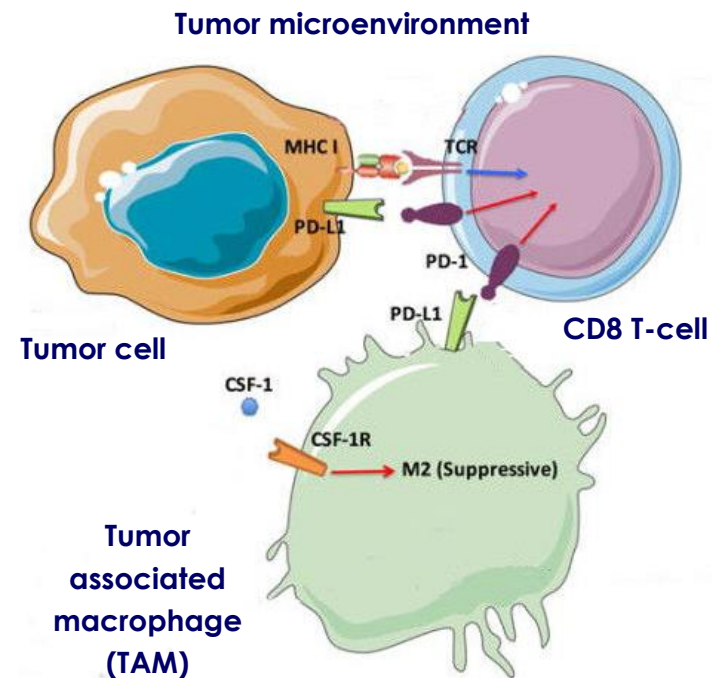
FGFR-inhibitor compound (Sponsor)	Parameter	FGFR1	FGFR2	FGFR3	FGFR4	CSF1R (FMS)
Derazantinib (Basilea)	Ratio to FGFR2 activity	4	1	4	77	3
Pemigatinib (Incyte)	Ratio to FGFR2 activity	3	1	4	39	231
Erdafitinib (Janssen)	Ratio to FGFR2 activity	2	1	2	13	95
Rogaratinib (Bayer)	Ratio to FGFR2 activity	5	1	6	18	116
Infigratinib (QED)	Ratio to FGFR2 activity	2	1	2	47	86
Futibatinib (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 inhibitors¹
- 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.²

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
- CSF1R inhibition may restore T-cell activity, downregulate immunosuppressive macrophage activity and improve susceptibility to PD1/PD-L1 inhibitors (immunotherapy)
- 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





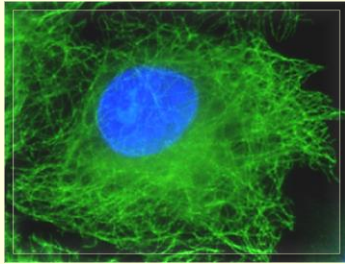
Oncology

BAL101553

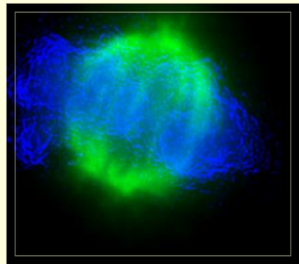
Treatment-refractory solid tumors,
including glioblastoma

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

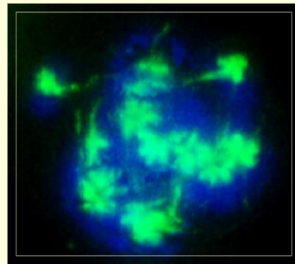
Bachmann AACR 2015



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

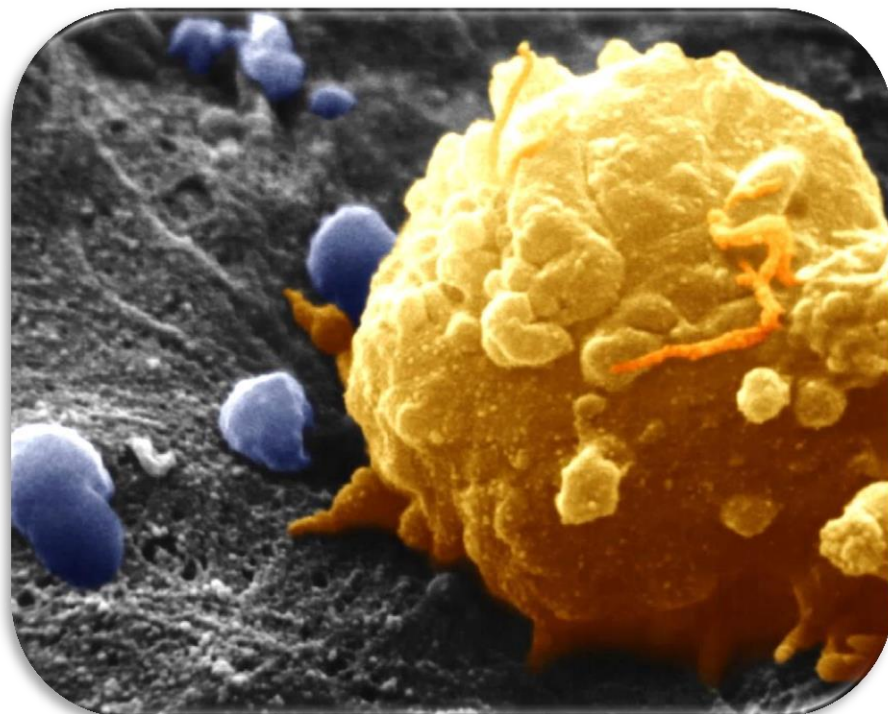
* BAL101553 is a prodrug of BAL27862

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 H1 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)¹
 - Anticipated to complete patient enrolment mid-2020



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

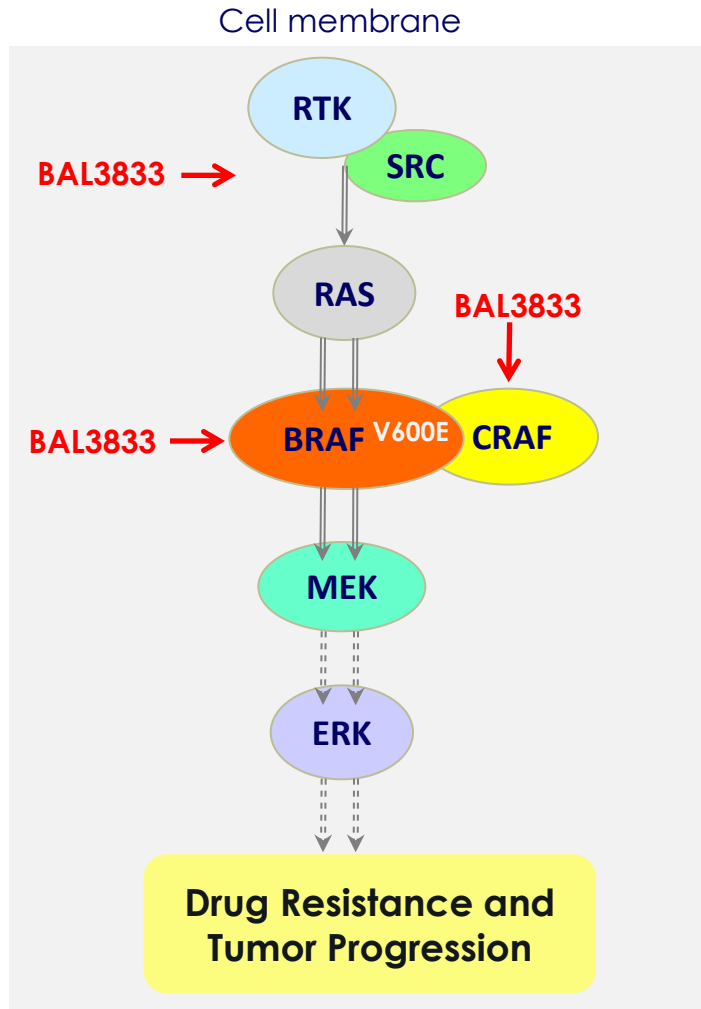


Oncology

BAL3833

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

BAL3833 — panRAF/SRC kinase inhibitor



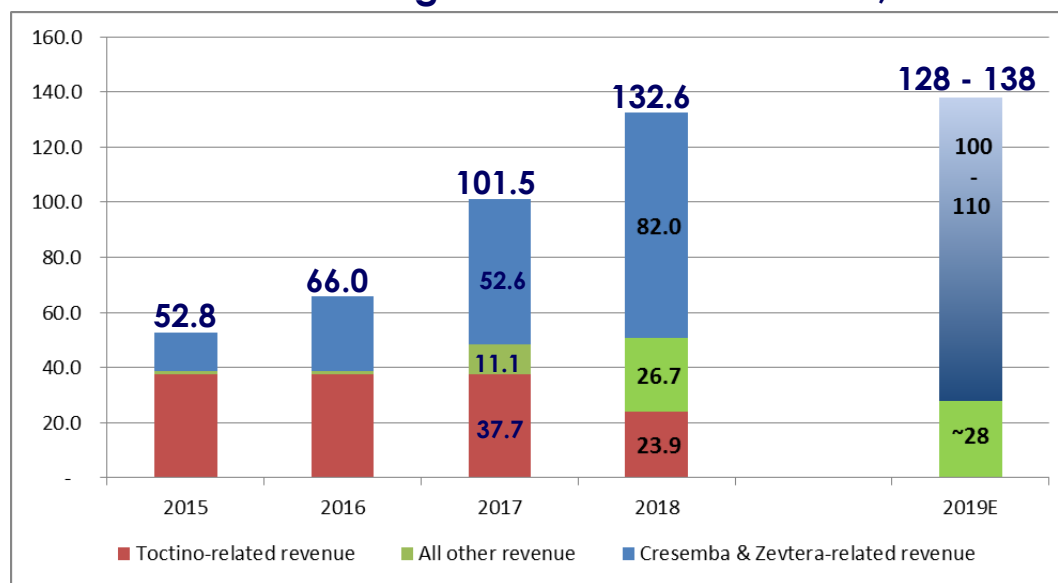
Cell changes in gene expression

- 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 to explore alternative formulations initiated

Key financials 2018 and 2019 guidance

In CHF mn	FY 2019 guidance	FY 2018 actuals	FY 2017 actuals
Total revenue	128 - 138	132.6	101.5
<i>thereof: Contributions from Cresemba & Zevtera</i>	100 - 110	82.0	52.6
Operating loss	20 - 30	24.1	17.1
Net operating cash consumption	55 - 65	79.2	+19.0
Year-end cash and financial investments	n/a	223.0	310.7

2015 – 2019E – Strong revenue increase 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

Start phase 2 study in
urothelial cancer

Complete patient
enrolment in phase 2
registrational study in
iCCA

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



Appendix

Basilea leadership

Management Committee



David Veitch – CEO



Bristol-Myers Squibb



2014



Donato Spota – CFO



2002



Marc Engelhardt, MD, Ph.D. – CMO



NOVARTIS



2010



Dr. Gerrit Hauck - Ph.D. – CTO



2018



Adesh Kaul – CCDO



POLYPHOR



2009



Laurenz Kellenberger, Ph.D. – CSO

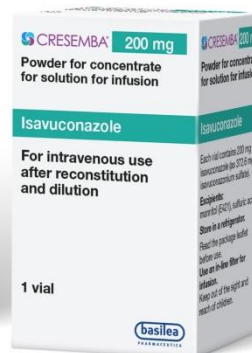
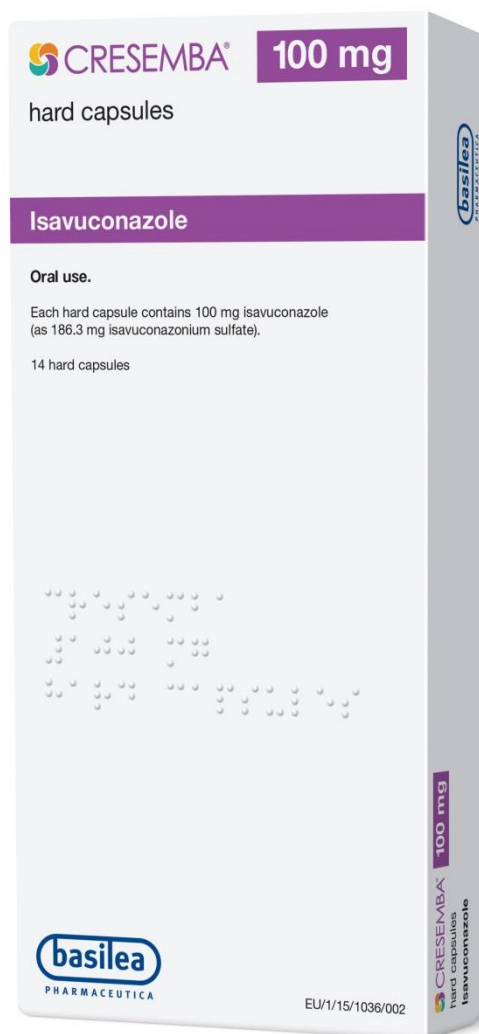


UNIVERSITY OF
CAMBRIDGE



2000





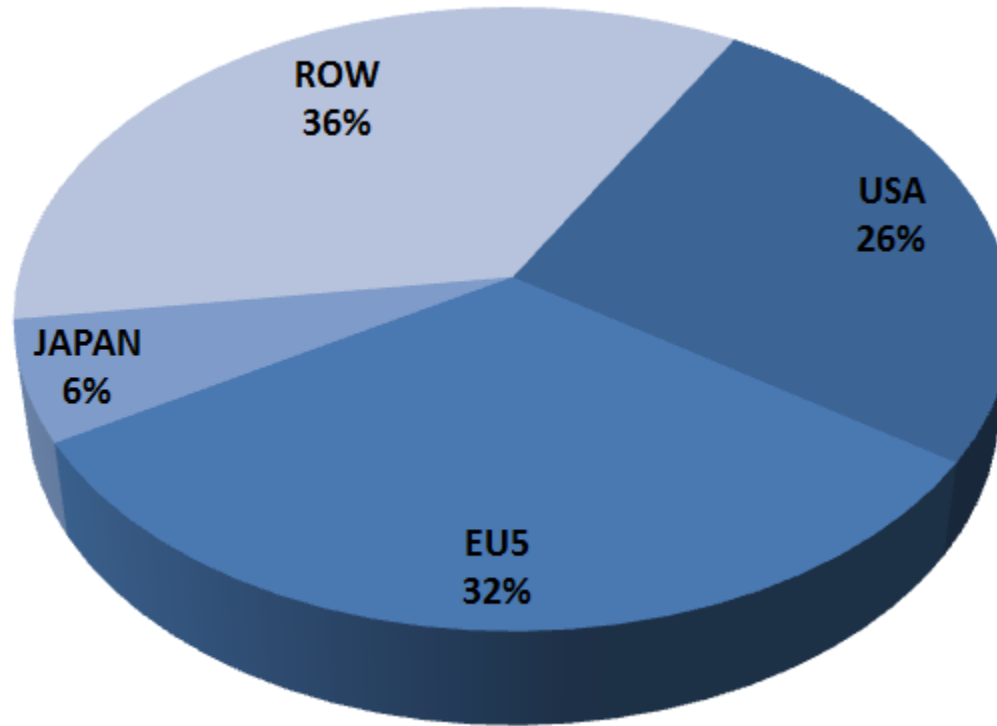
Antifungal

Cresemba® (isavuconazole)

- Invasive mold infections
- Marketed in the U.S. and Europe

Significant sales of best-in-class antifungals in all major regions — Covered by our partnerships

USD 3bn sales of best-in-class antifungals* (MAT Q4 2018)

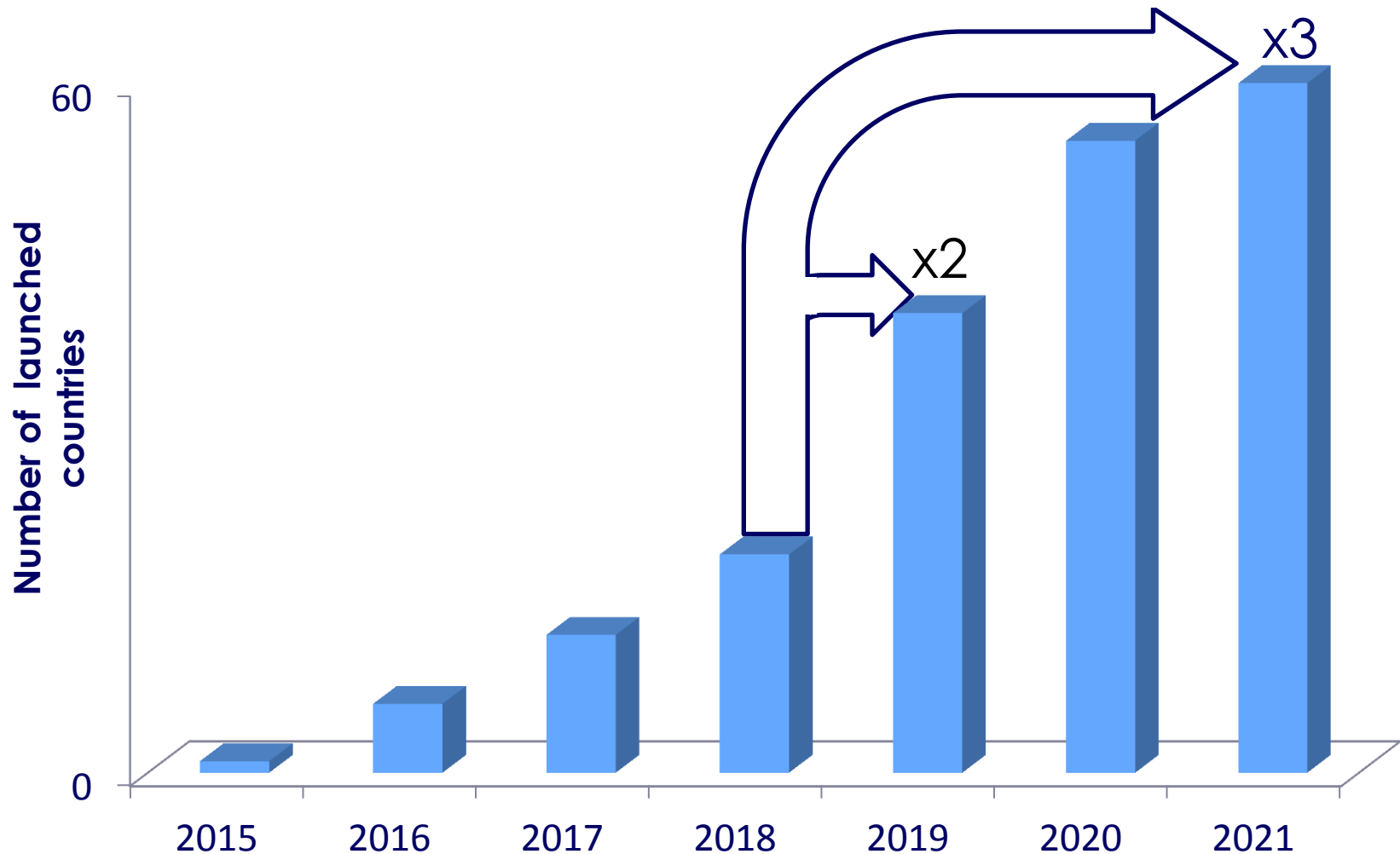


* Best-in-class antifungals: isavuconazole, posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin

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



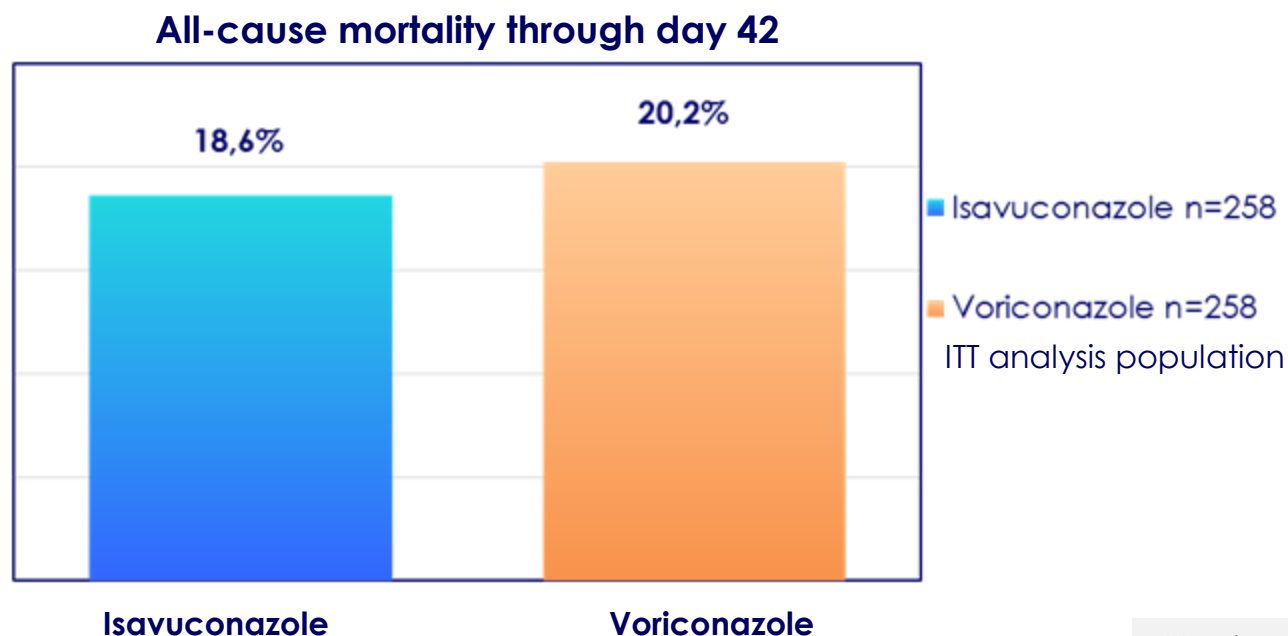
Cresemba – strong global roll out



Isavuconazole — SECURE phase 3 data (efficacy)

SECURE: Primary treatment of invasive fungal disease caused by *Aspergillus* spp. and other filamentous fungi

- Met primary objective of non-inferiority vs. voriconazole



Maertens *The Lancet* 2016



Isavuconazole — Positive VITAL phase 3 data

Treatment of invasive fungal disease caused by emerging fungi such as *Mucorales* spp., including patients with pre-existing renal impairment (open-label study, n=149)

- All-cause-mortality through day 42 in renally impaired patients with invasive aspergillosis (n=20) **for which i.v. voriconazole can only be used with caution:**
 - 15% (vs. 18.6% benchmark in SECURE study, excluding patients with moderate to severe renal impairment)*
- All-cause-mortality through day 42 in patients with confirmed mucormycosis (n=37), **including patients refractory or intolerant to other antifungal therapies:**
 - 38% (similar to data reported in the literature for amphotericin B)**

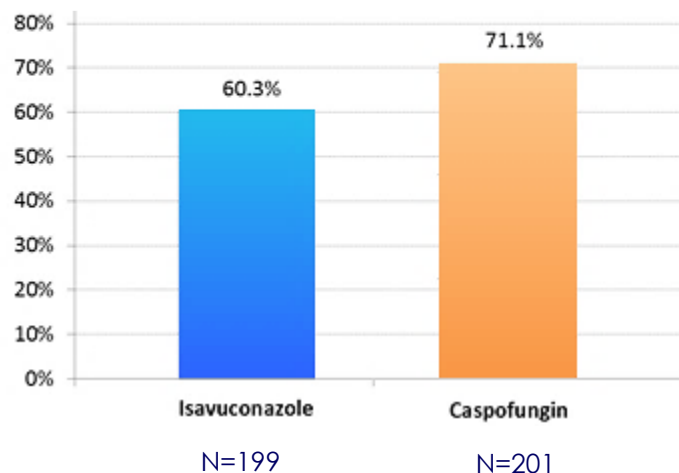
* Basilea Pharmaceutica data on file; ** Marty *The Lancet Infectious Diseases* 2016



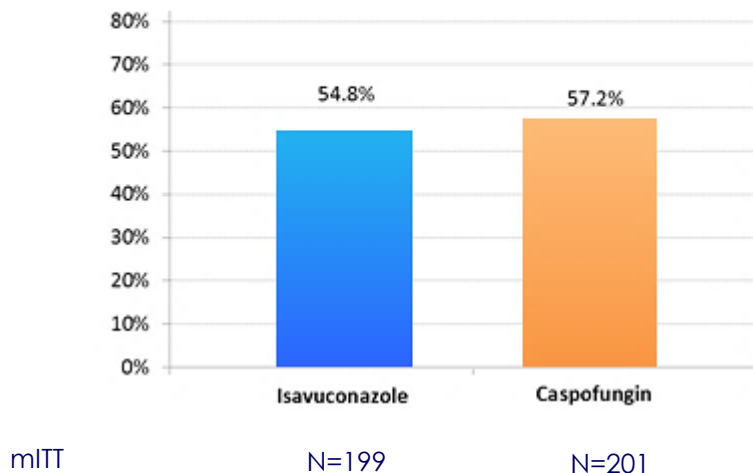
Isavuconazole — ACTIVE phase 3 topline data

ACTIVE: Primary treatment of candidemia/invasive candidiasis¹

Primary endpoint was not achieved:
Overall response at end of
i.v. treatment



Key secondary endpoint comparable
between treatment groups:
Overall response at 2 wks after end-of-therapy



- Overall response at end of i.v. treatment similar to other azoles and amphotericin B²
- Overall response 2 weeks after end-of-therapy comparable to caspofungin/voriconazole¹
- All-cause mortality at day 14 and day 56 comparable to caspofungin/voriconazole¹

¹ Kullberg *Clin Infect Dis* 2018

² Rex *N Engl J Med* 1994; Kullberg *Lancet* 2005; Mora-Duarte *N Engl J Med* 2002; Kuse *Lancet* 2007; Pappas *Clin Inf Dis* 2007; Reboli *N Engl J Med* 2007



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



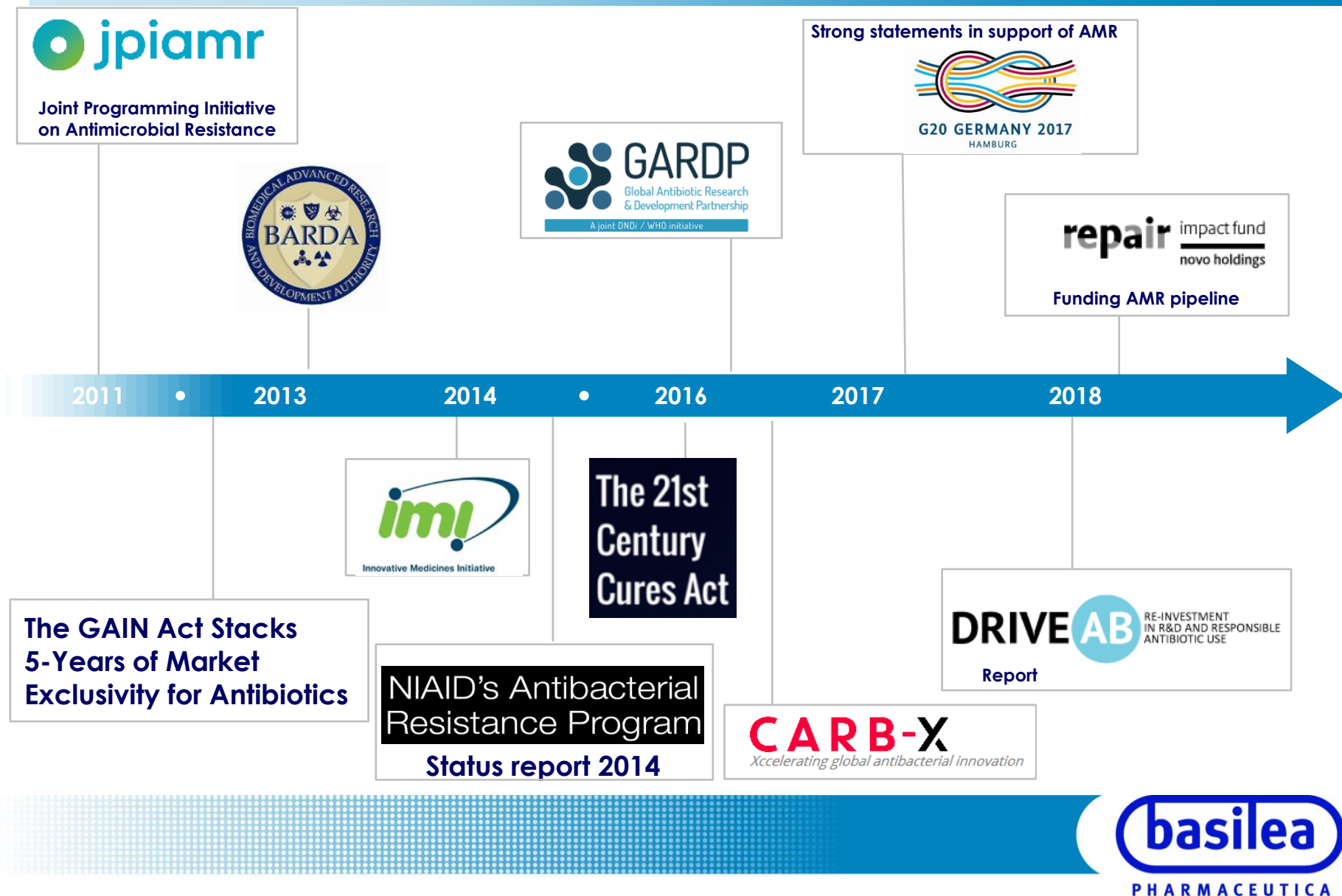
Antibacterial

Zevtera®/Mabelio® (ceftobiprole)

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

* HAP (excluding VAP)

Improving political and regulatory environment for novel antibiotics and antifungals



Phase 3 study with ceftobiprole in the treatment of patients with ABSSSI

- **Design:** randomized, double-blind, multi-center
- **Enrolment:** approximately 674 adult patients (male and female)
- **Indication:** acute bacterial skin and skin structure infection (ABSSSI)
- **Main inclusion criteria:** diagnosis of ABSSSI, requirement of i.v. treatment
- **Intervention:** ceftobiprole medocartil i.v.; comparator vancomycin i.v. (plus aztreonam to cover Gram-negative bacteria)
- **Primary endpoint:** non-inferiority of ceftobiprole to vancomycin (plus aztreonam) for early clinical response based on percentage reduction in lesion size at 48–72 hours after first treatment
- **Secondary endpoint** (primary for EMA): investigator-assessed clinical success at the test-of-cure (TOC) visit 15–22 days after randomization



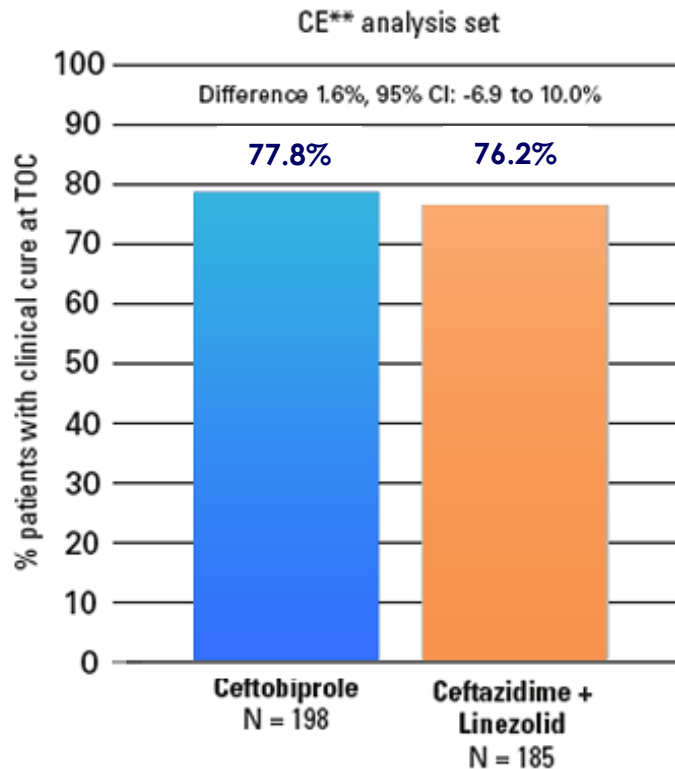
Phase 3 study with ceftobiprole in the treatment of patients with SAB

- **Design:** randomized, double-blind, multi-center
- **Enrolment:** approximately 390 adult patients (male and female)
- **Indications:** *Staphylococcus aureus* bacteremia (SAB), including endocarditis (IE) and other forms of complicated SAB
- **Main inclusion criteria:** Positive *S. aureus* blood culture and signs & symptoms for SAB
- **Intervention:** ceftobiprole medocartil i.v.; comparator daptomycin i.v. or daptomycin plus aztreonam to cover Gram-negative bacteria
- **Primary endpoint:** non-inferiority of ceftobiprole to daptomycin for overall success as assessed by an independent Data Review Committee (DRC) in the treatment of SAB, including IE, at the post-treatment evaluation (PTE) visit (70 days after randomization) in the modified intent-to-treat (mITT) population.
- **Secondary endpoints:** include all-cause mortality at Day 28 and Day 70 (PTE visit) in the intent-to-treat (ITT) and mITT populations; and time to *S. aureus* bloodstream clearance

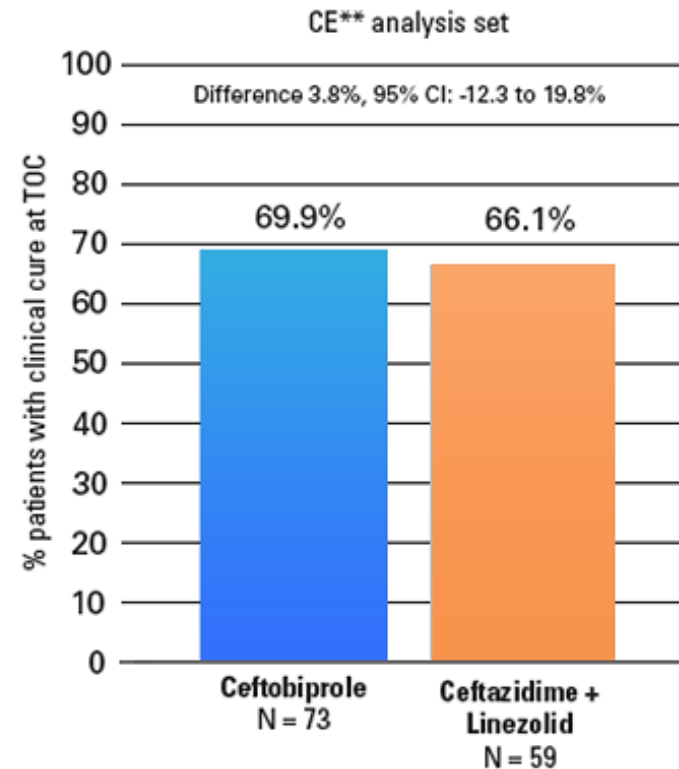


Ceftobiprole — Clinical study results indicate comparable efficacy to combination therapy in HAP*

Clinical Cure Rate – all patients



Clinical Cure Rate – ICU patients

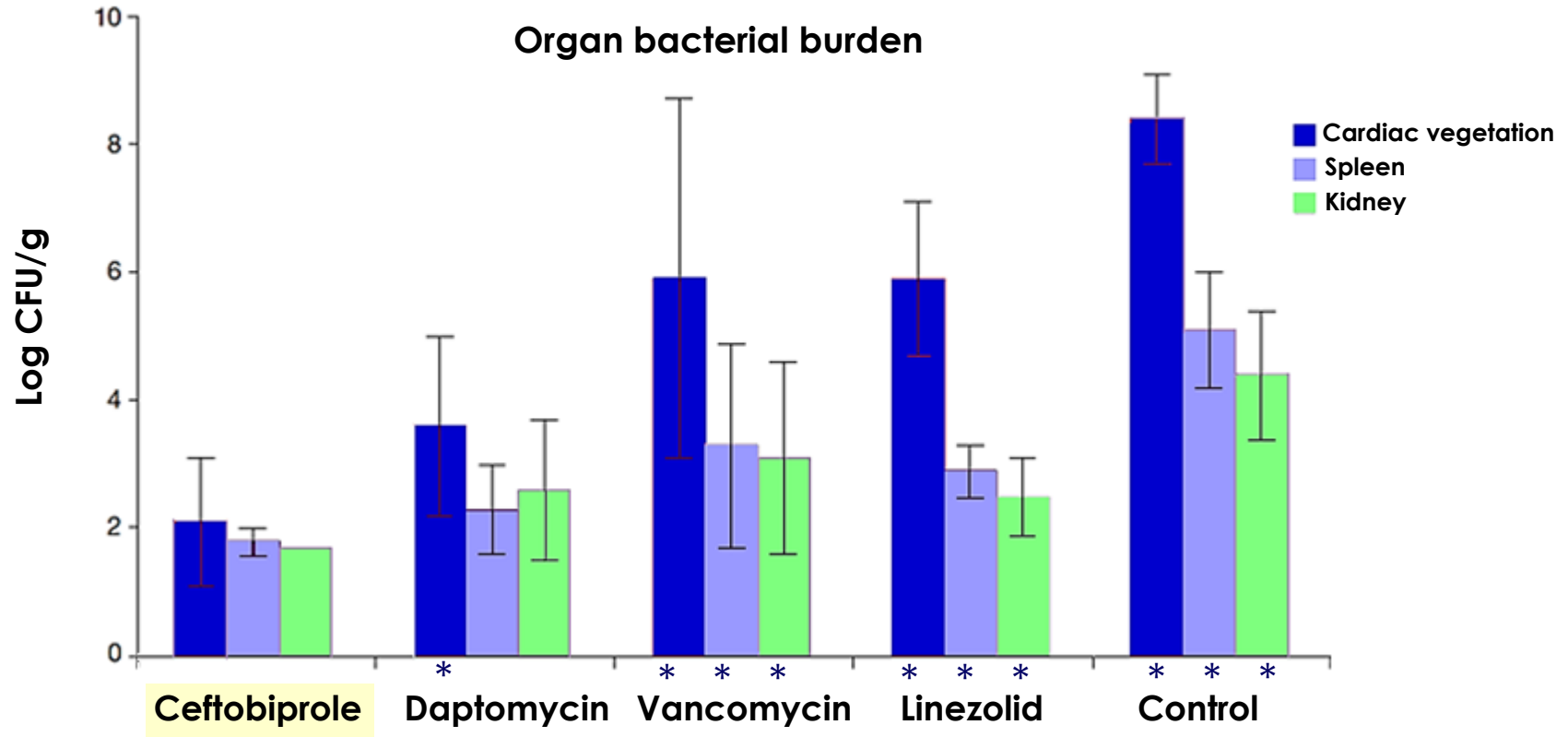


* Excluding VAP; ** Clinically evaluable

Awad *Clin Infect Dis* 2014



Ceftobiprole — Statistically significant lower bacterial burden in an endocarditis rabbit model



MRSA titers in cardiac vegetations (bacterial masses), spleens and kidneys of untreated and antibiotic treated rabbits infected with MRSA strain COL (highly methicillin-resistant)

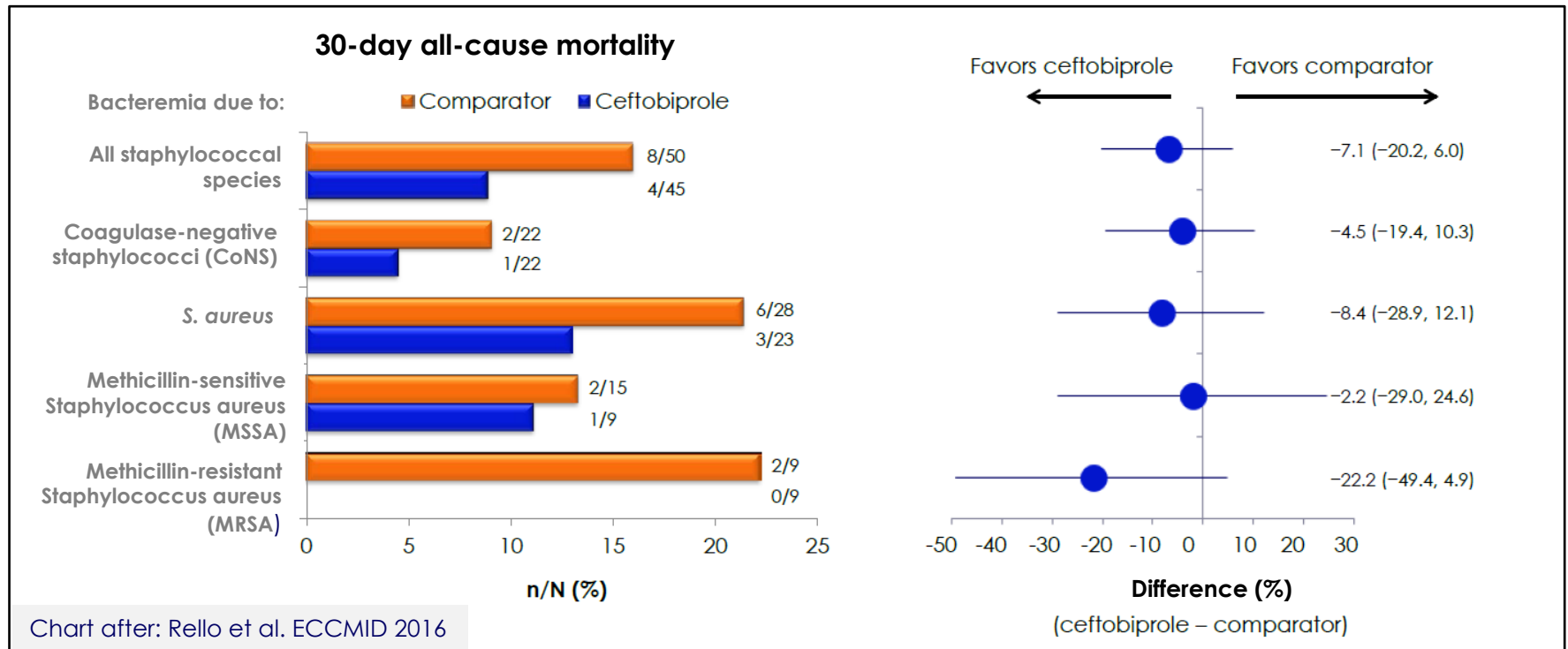
* Differences in favor of ceftobiprole statistically significant

Tattevin Antimicrob Agents Chemother 2010



Ceftobiprole — Trend towards lower 30-day all-cause-mortality for SAB* patients treated in phase 3 studies

- Pooled analysis from four double-blind, randomized phase 3 studies (2x ABSSSI, HABP, CABP)



Comparators: ABSSSI: vancomycin, vancomycin + ceftazidime / CABP: ceftriaxone ± linezolid / HABP: linezolid + ceftazidime

* *Staphylococcus aureus* bacteremia

Oncology

Derazantinib (BAL087)

panFGFR kinase inhibitor
for various solid tumors

Derazantinib — iCCA registrational phase 2 study ongoing

- **Design:** Multi-national, open-label, non-comparative study
- **Enrolment:** 100 adult patients
- **Indications:** Intrahepatic cholangiocarcinoma (iCCA) with FGFR2 fusions (2nd-line)
- **Main inclusion criteria:**
 - Adult subjects with locally advanced (inoperable) or metastatic iCCA whose tumors harbor FGFR2 gene fusions and who received at least one prior regimen of systemic therapy
 - Measurable disease by RECIST 1.1
- **Intervention:** 300 mg oral ARQ 087 once daily
- **Primary endpoint:** Objective Response Rate (ORR)
- **Secondary endpoints:** Progression-free survival (PFS), Overall Survival (OS), Duration of response (DoR), Safety



Algorithm for the management of patients with biliary tract cancer, including iCCA

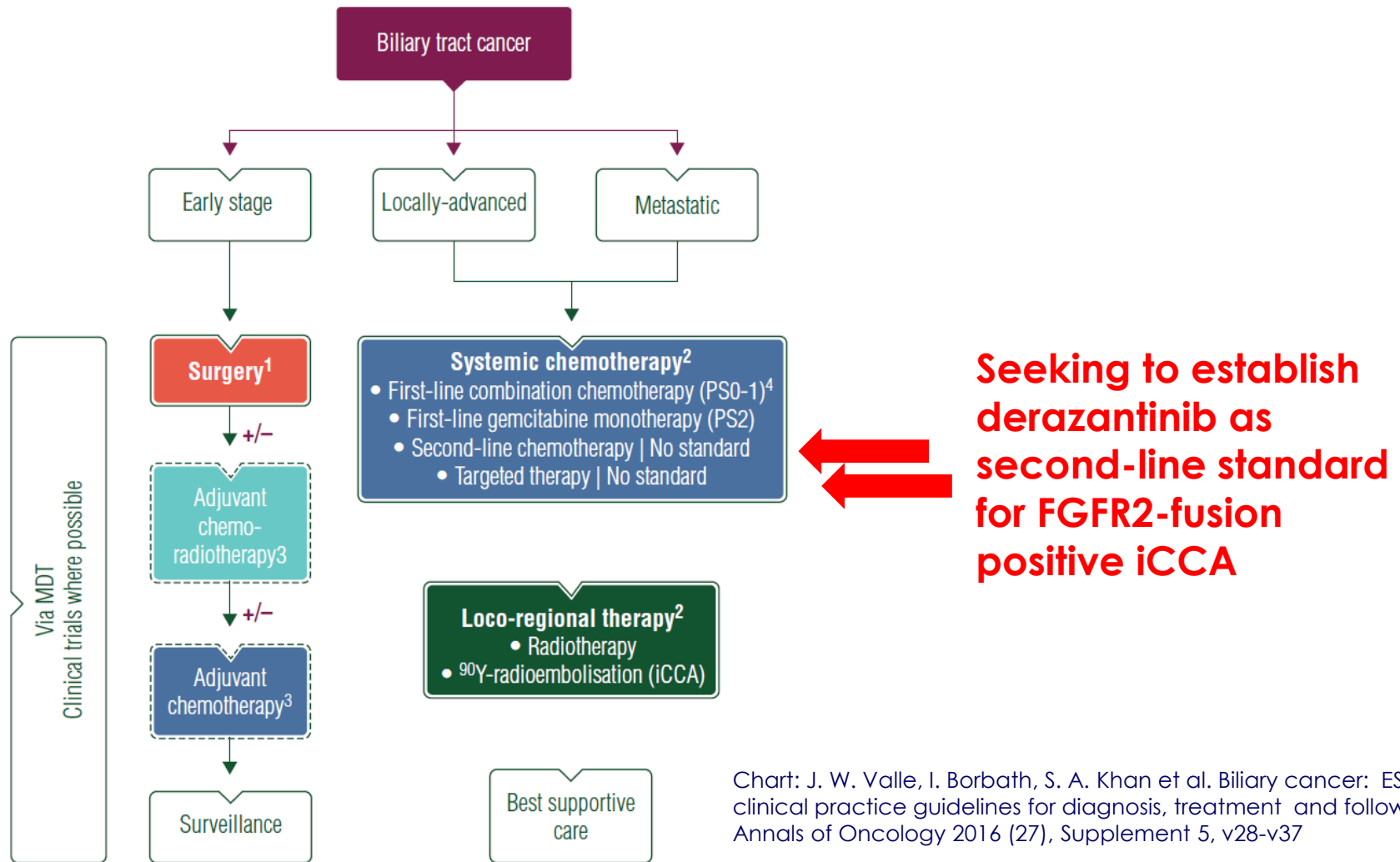
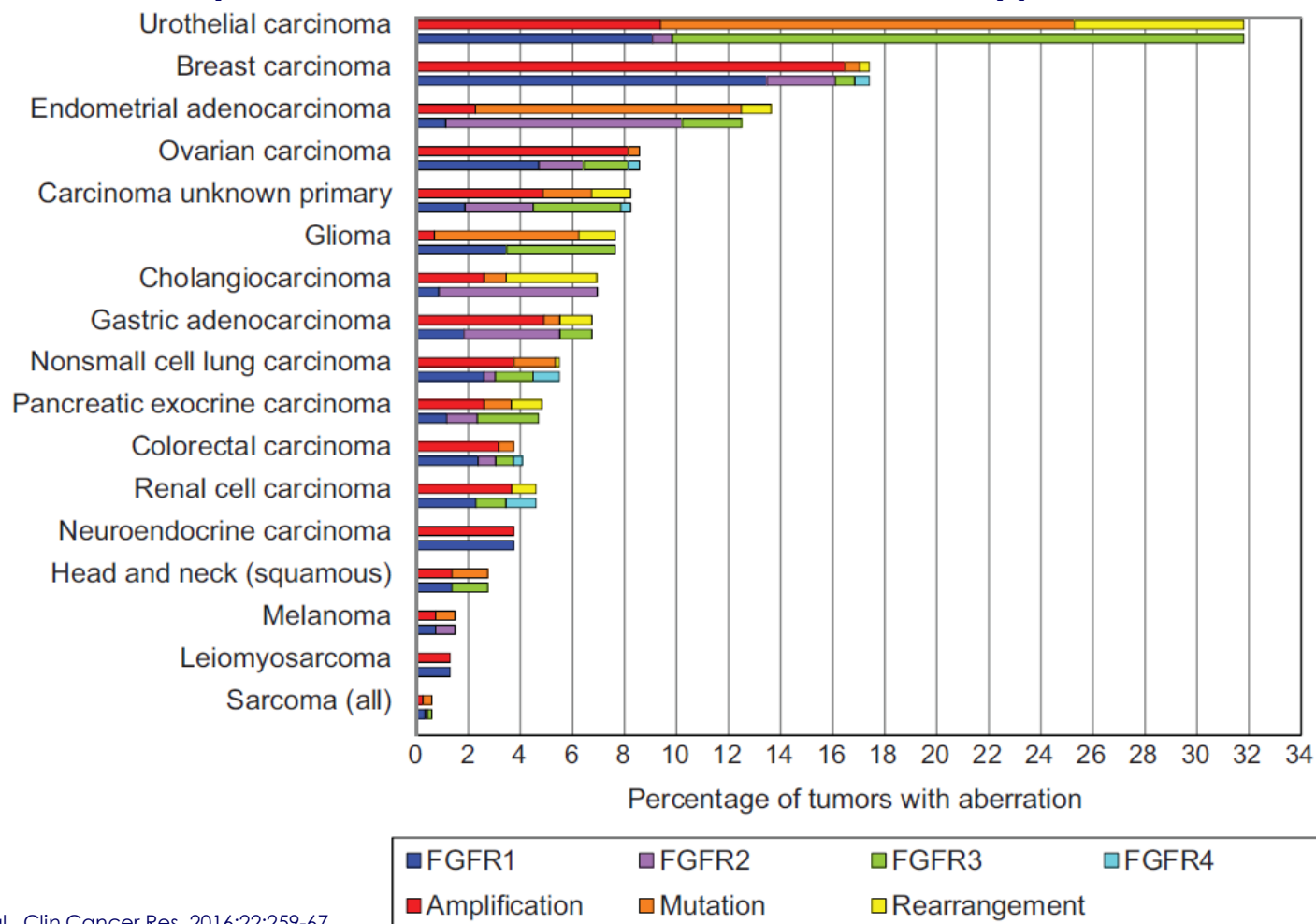


Chart: J. W. Valle, I. Borbath, S. A. Khan et al. Biliary cancer: ESMO clinical practice guidelines for diagnosis, treatment and follow-up. Annals of Oncology 2016 (27), Supplement 5, v28-v37

Derazantinib — Significant potential beyond iCCA

Frequency of currently known FGFR aberrations across tumor types



Source: Helsten et al., Clin Cancer Res. 2016;22:259-67



