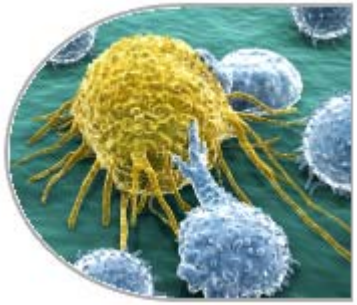

basilea
PHARMACEUTICA

Disclaimer and forward-looking statements

Forward looking statements

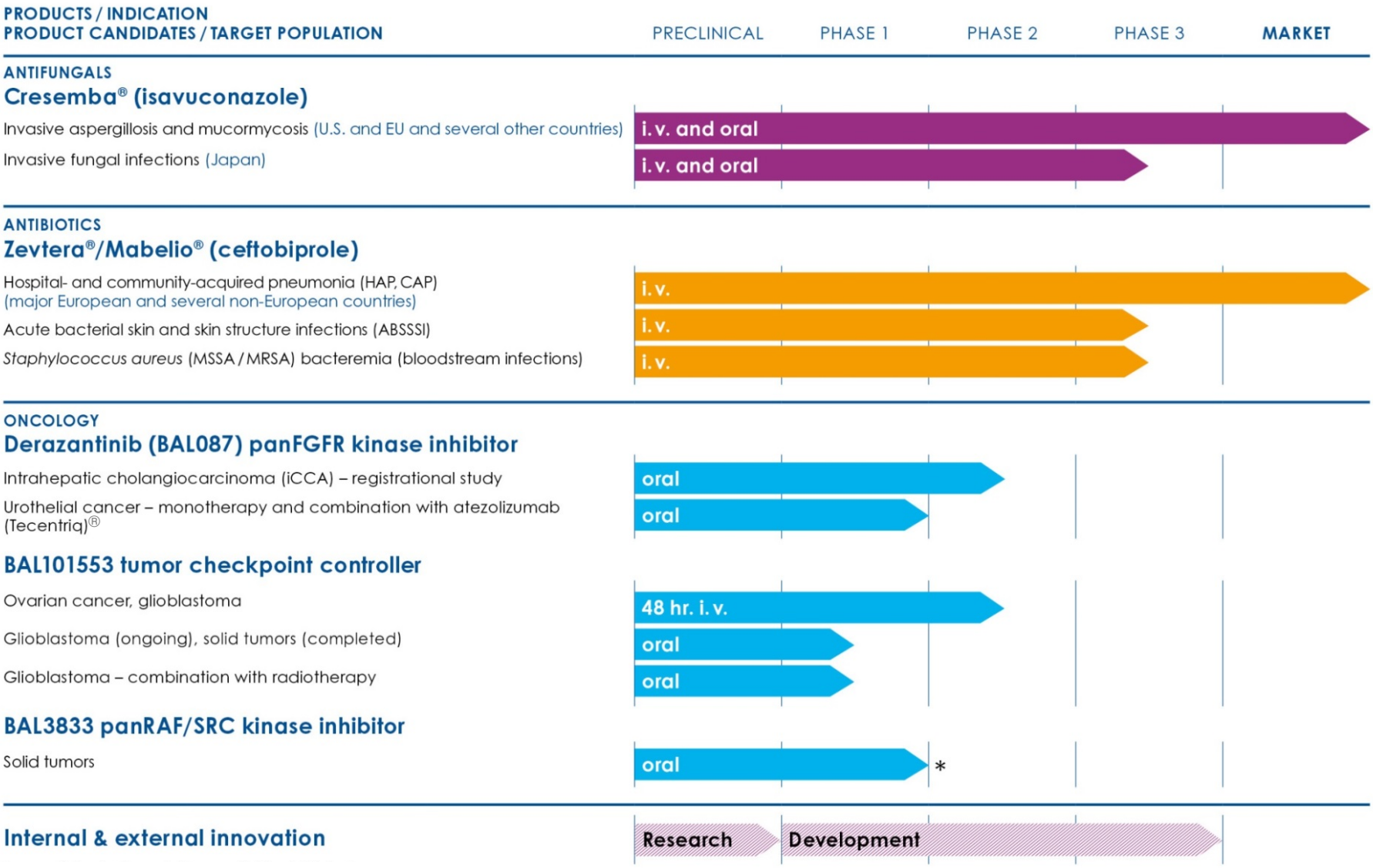
This communication including the accompanying oral presentation contains certain forward-looking statements, including, without limitation, statements containing the words "believes", "anticipates", "expects", supposes", "considers" and words of similar import or which can be identified as discussions 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. 220 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

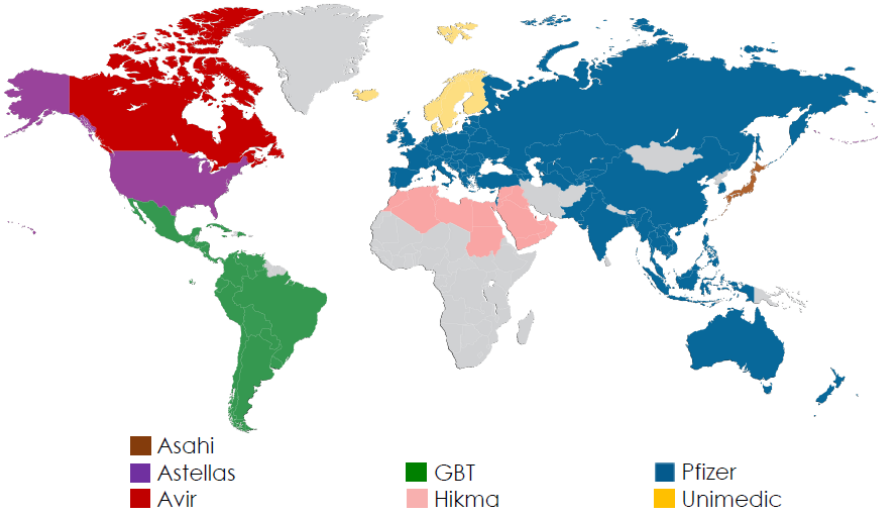
- **Correvio (formerly Cardiome),** for Europe (ex. Nordics), Israel *(Zevtera)*
- **Hikma,** for MENA region *(Cresemba and Zevtera)*
- **Grupo Biotoscana,** for LatAm *(Cresemba and Zevtera)*
- **Unimedic,** for Nordics *(Cresemba and Zevtera)*
- **Avir,** for Canada *(Cresemba and Zevtera)*

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

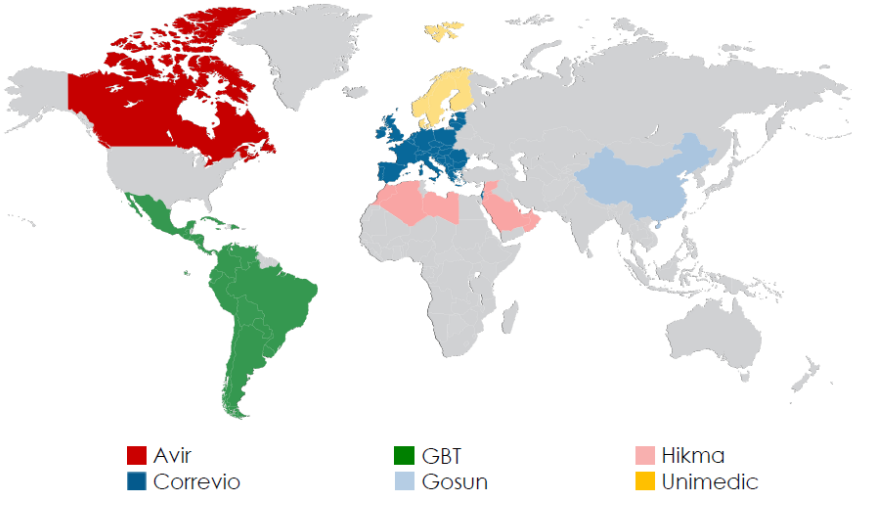
Ongoing participation

- Double-digit royalties on sales by license partners
- Participation through transfer price structure in sales by distribution partners
- Approximately USD 240mn upfront and milestone payments received; USD 1.1bn in potential milestones outstanding

Our Global Partnerships: Cresemba



Our Global Partnerships: Zevtera



Antifungal

Cresemba®
(isavuconazole)

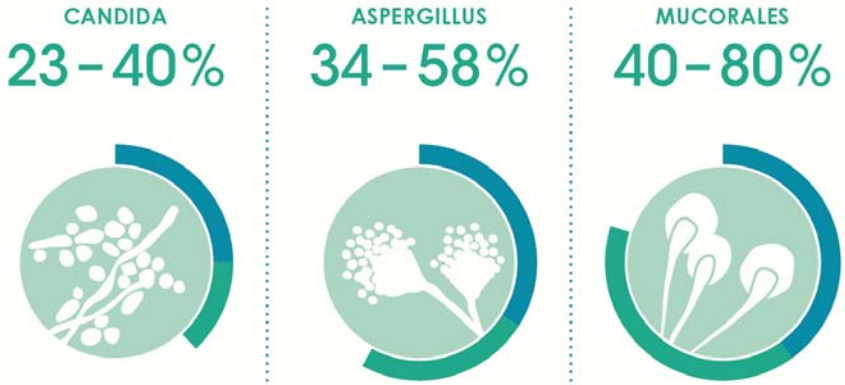
- Invasive mold infections
- Marketed in the U.S., Europe

basilea
PHARMACEUTICA

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

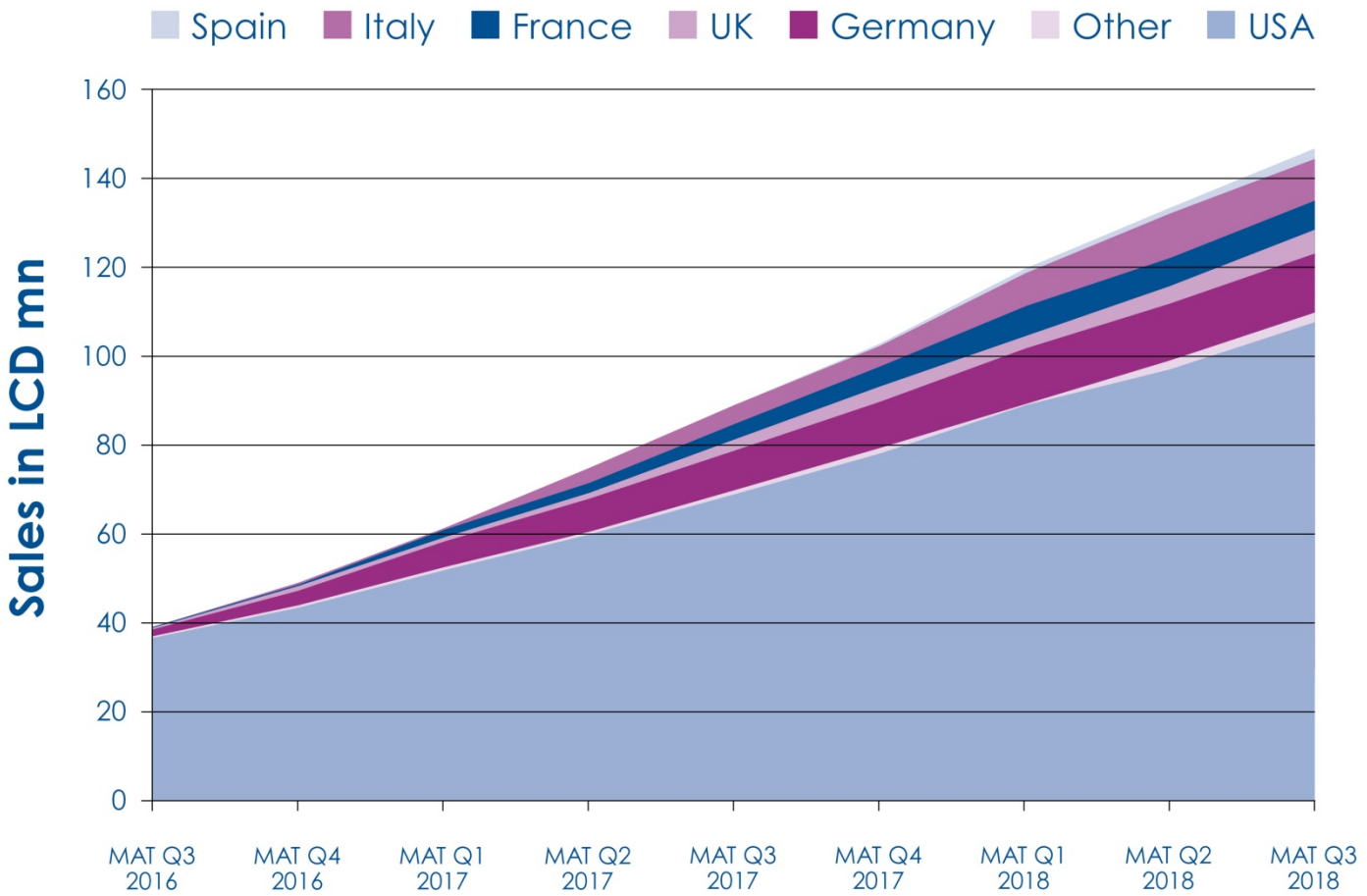
Mortality rates for invasive fungal infections**



**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

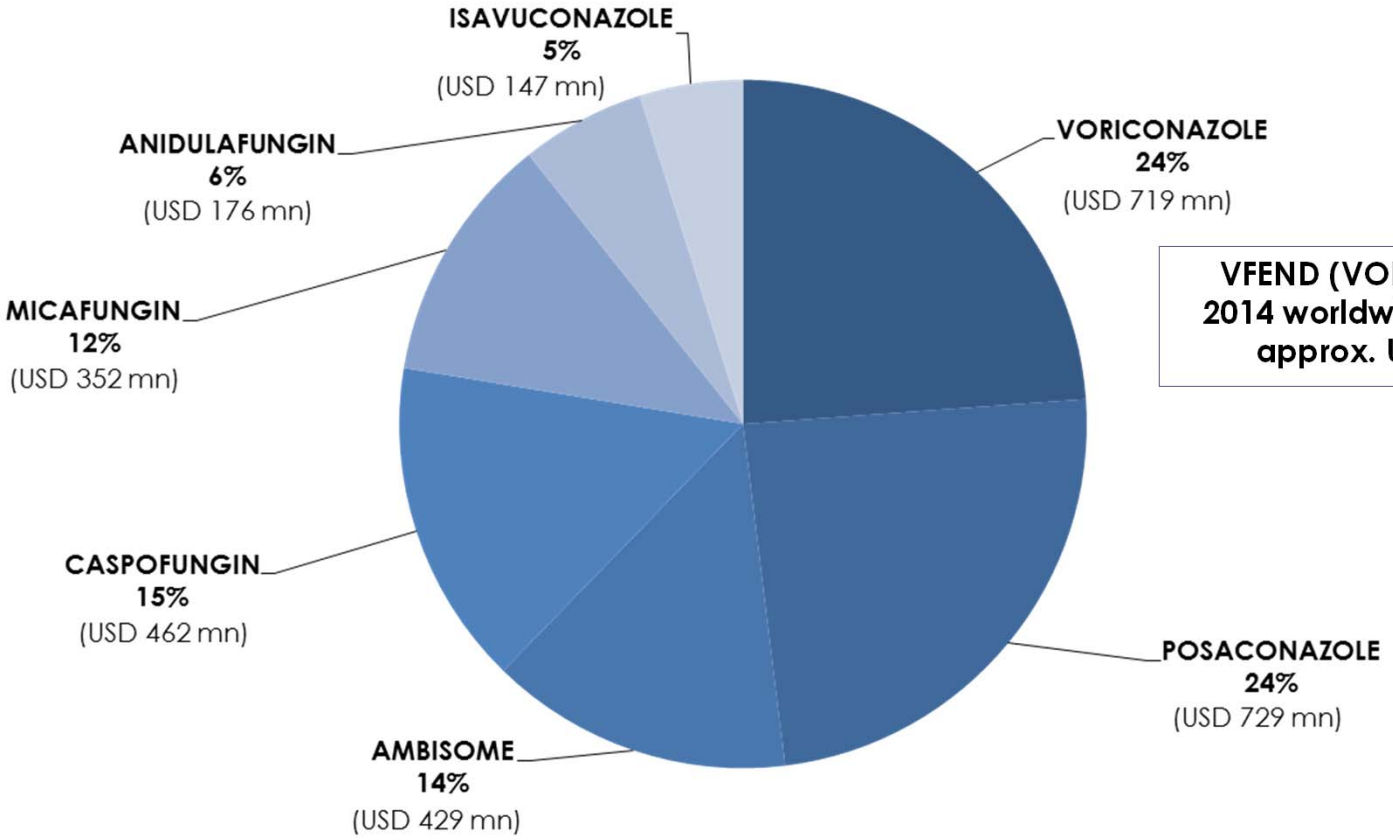
USD 147 mn annual in-market sales by Q3 2018



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

Sales of best-in-class antifungals* by product

USD 3.0 bn sales (MAT Q3 2018)

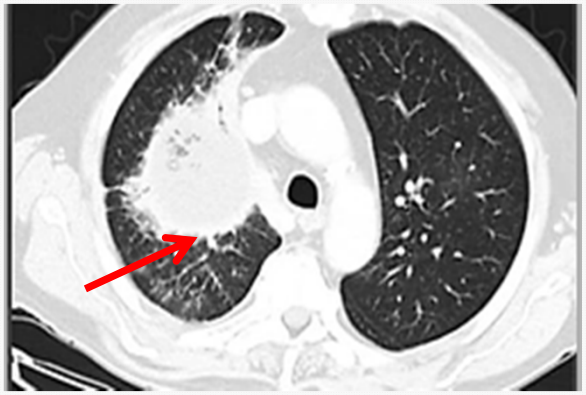


VFEND (VORICONAZOLE)
2014 worldwide peak sales approx. USD 900 mn

* 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, September 2018

Cresemba — Differentiated by spectrum, safety and tolerability

CRESEMBA® (ISAVUCONAZOLE)



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

basilea PHARMACEUTICA

Cresemba — Marketed in the EU and U.S. and further country launches planned

For full U.S. prescribing information see:
www.cresemba.com

- Marketed in major European countries by Pfizer
- 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

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)

basilea PHARMACEUTICA

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

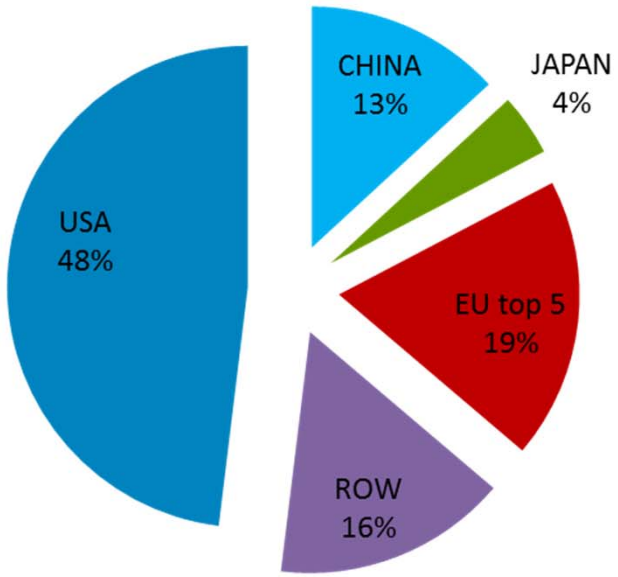
Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-acquired pneumonia (VAP), and community-acquired pneumonia (CAP)

Not approved in the U.S.

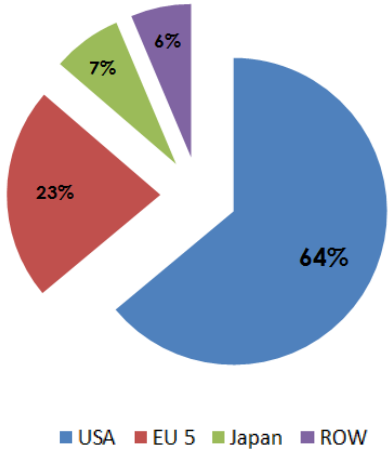
- Broad-spectrum anti-MRSA cephalosporin (including Gram-negative bacteria)
- Rapid bactericidal activity
- Potential to replace antibiotic combinations
- Early improvement in HAP, particularly in patients with MRSA, and CAP, including high-risk patients
- Cephalosporin class safety profile
- Marketed 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

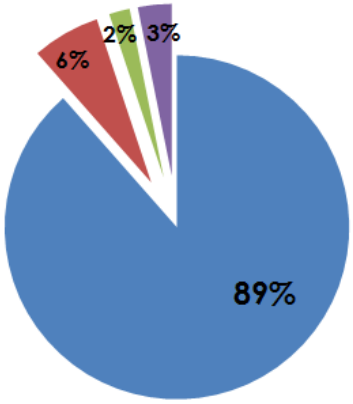
Global anti-MRSA hospital antibiotics sales*
USD 3.1bn (MAT Q3 2018)



Linezolid sales by region 2014 (before LOE)



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, September 2018

basilea PHARMACEUTICA

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

- 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 started in 2018
- 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

*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

Oncology

Derazantinib (BAL087)

panFGFR kinase inhibitor
for various solid tumors

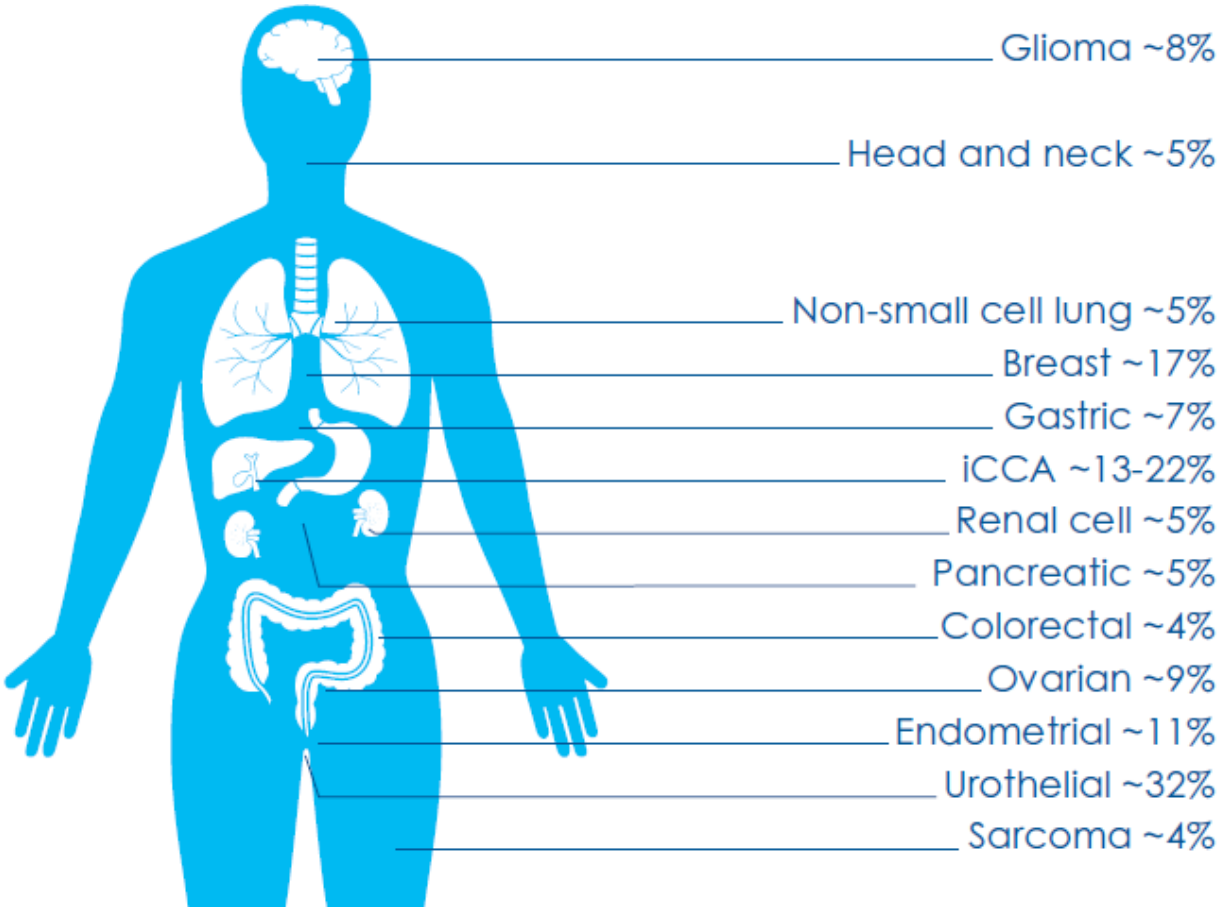
basilea
PHARMACEUTICA

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

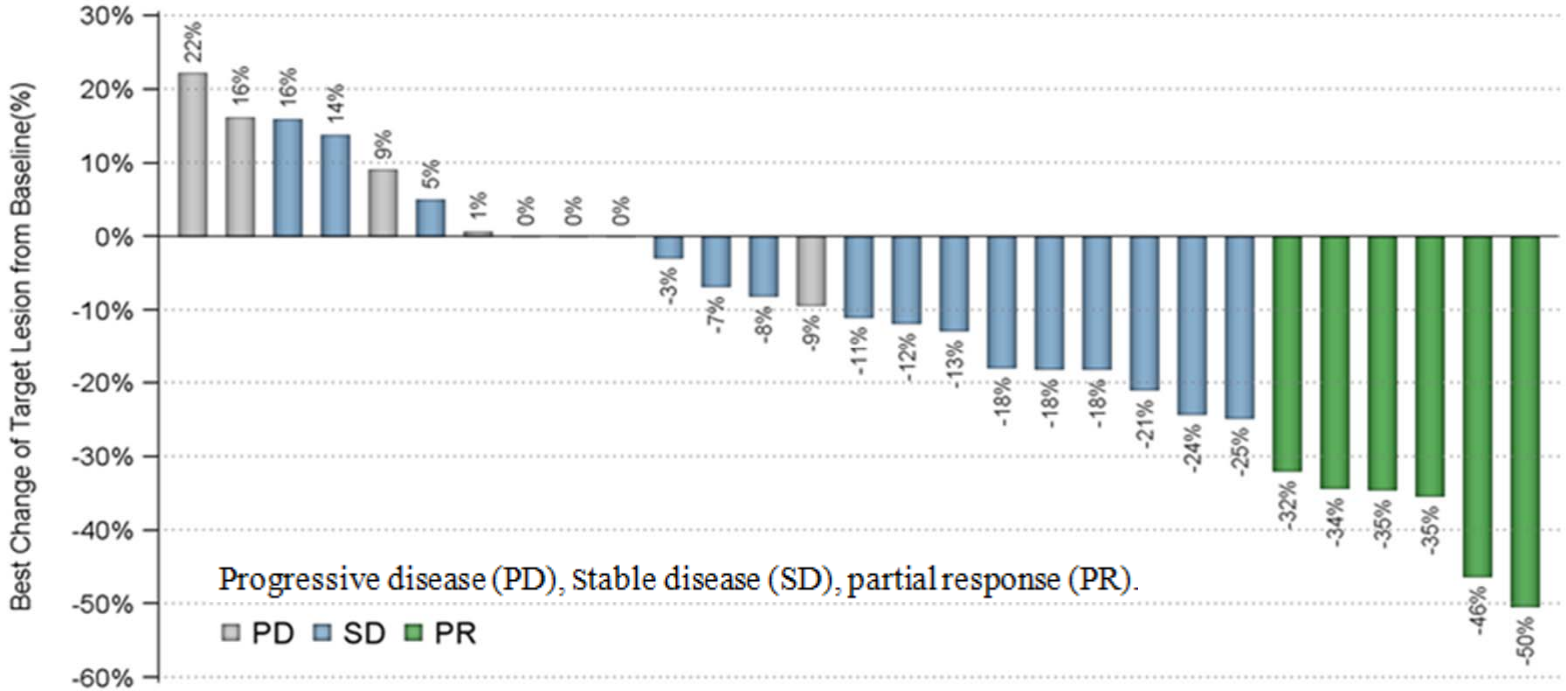
Frequency of FGFR aberrations across different tumor types



Source:
Helsten et al., Clin Cancer Res 2016 (22), 257-267; FGRF2 fusions in iCCA: Graham et al. Hum Pathol 2014 (45), 1630-1638; Jain et al. JCO Precis Oncol 2018 (2) 1-12

basilea PHARMACEUTICA

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

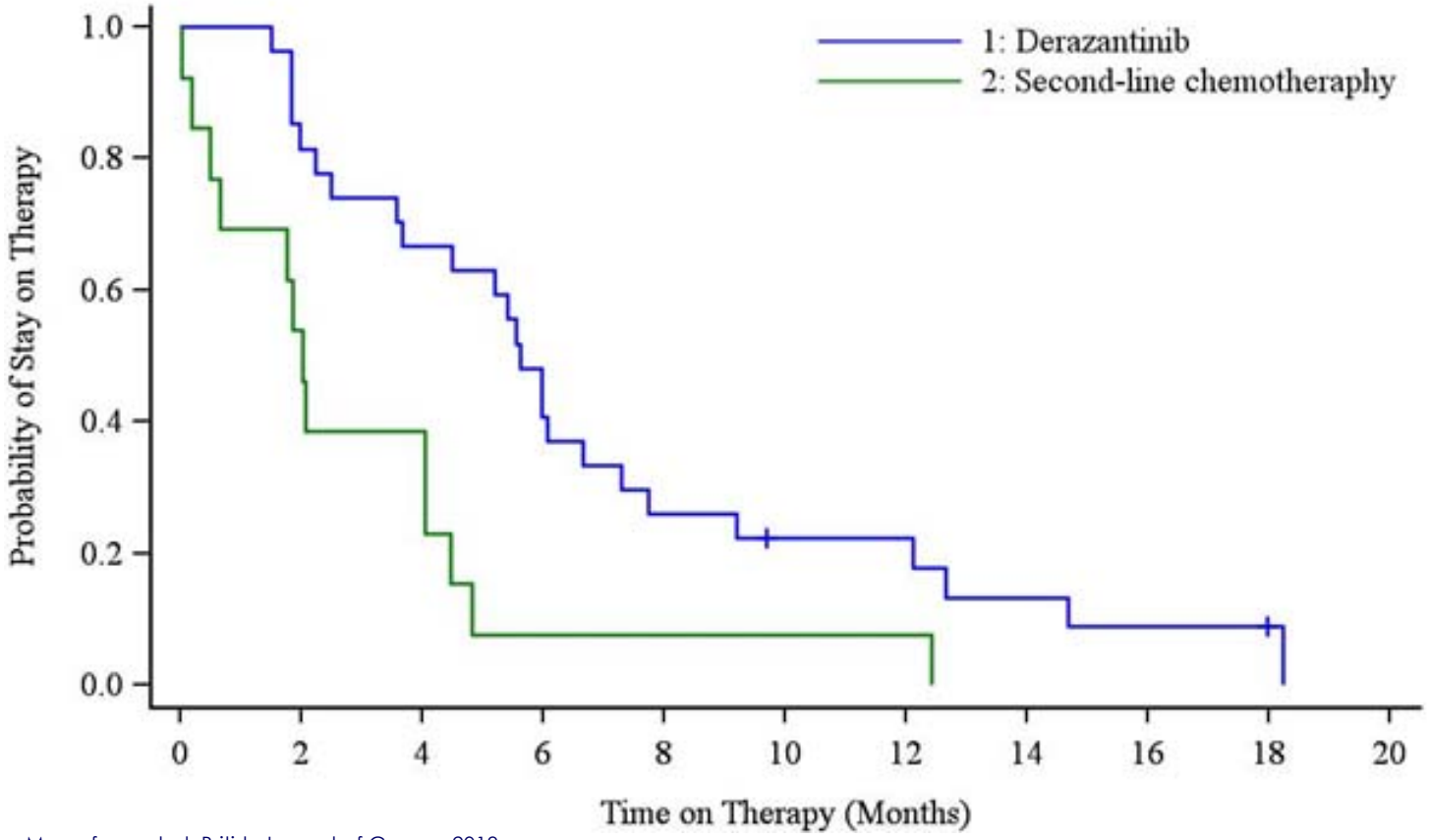


- Subgroup analysis of 29 patients with FGFR2-fusion positive iCCA:
  - Objective response rate of 21%
  - In 72% of patients, tumor response or disease stabilization for ≥16 weeks was achieved*
  - Manageable safety profile

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

Intrapatient 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 derazantinib[1] versus <10% for SoC[2,3]
  - Progression-Free Survival (PFS) approx. 6 months[1] 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/xeropthalmia†	**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: [1]Mazzaferro et al., Br J Cancer 2018 and Basilea data on file; [2]Javle et al., ESMO 2018; [3]Meric-Bernstam et al, ESMO WC GI Cancer, 2018; [4]Hollebecque, et al., ESMO 2018; [5]Necchi, et al., ESMO 2018; [6]Siefker-Radtke et al., ASCO 2018

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

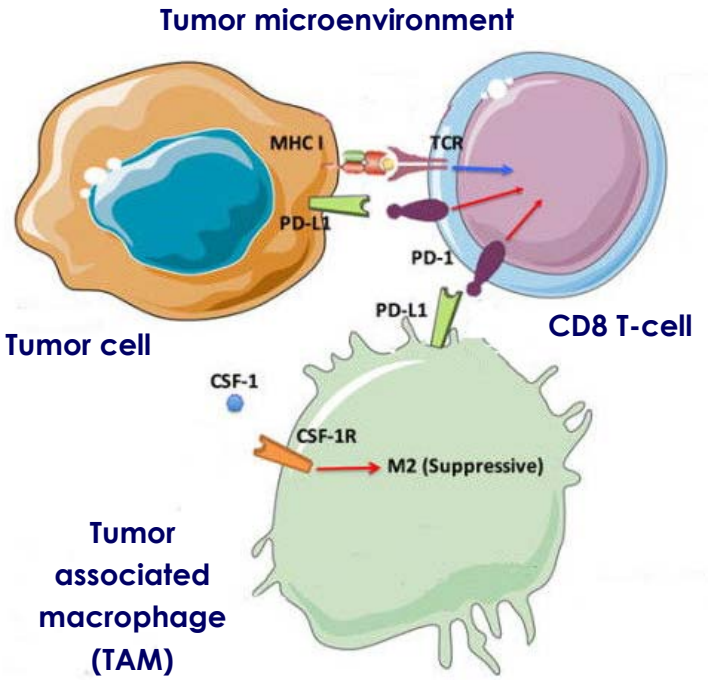
FGFR-inhibitors show differences in kinase-inhibition profiles

FGFR-inhibitor compound (Sponsor)	Parameter	FGFR1	FGFR2	FGFR3	FGFR4	CSF1R (FMS)
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 inhibitors[1]
- 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

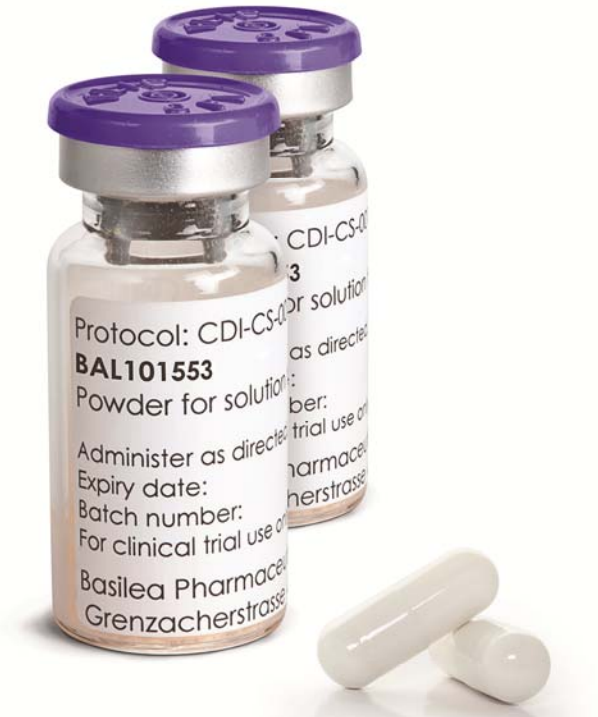


Blocking CSF1/CSF1R has the potential to reprogram tumor-promoting macrophages and enhance the response to immune checkpoint (PD1/PD-L1) inhibitors. [2]

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

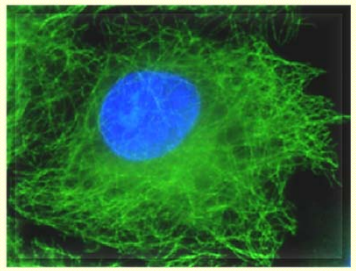
BAL101553

Treatment-refractory solid tumors,
including glioblastoma

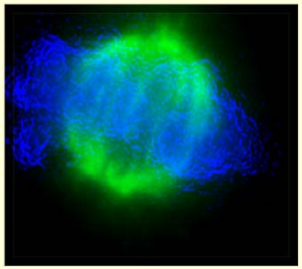
basilea
PHARMACEUTICA

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

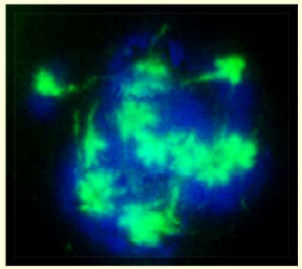
Bachmann AACR 2015



Non-dividing tumor cell



Normal dividing tumor cell



BAL27862-treated tumor cell*

Blue = DNA
Green = microtubules

* BAL101553 is a prodrug of BAL27862

- 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

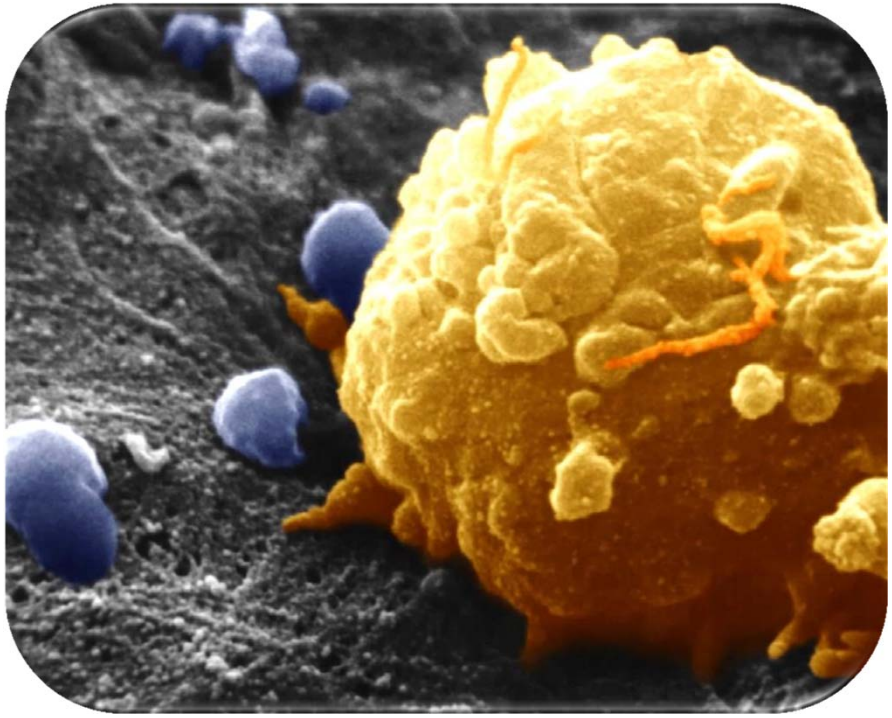
BAL101553 — three ongoing clinical studies

- 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

Adult Brain Tumor Consortium

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

basilea PHARMACEUTICA



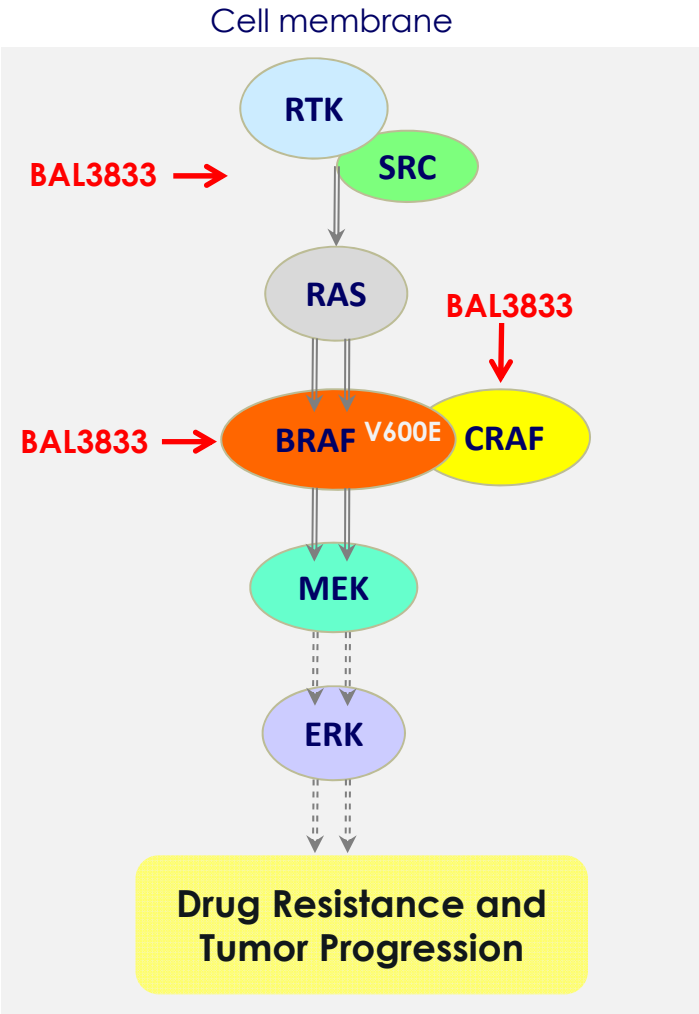
Oncology

BAL3833

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

basilea
PHARMACEUTICA

BAL3833 — panRAF/SRC kinase inhibitor

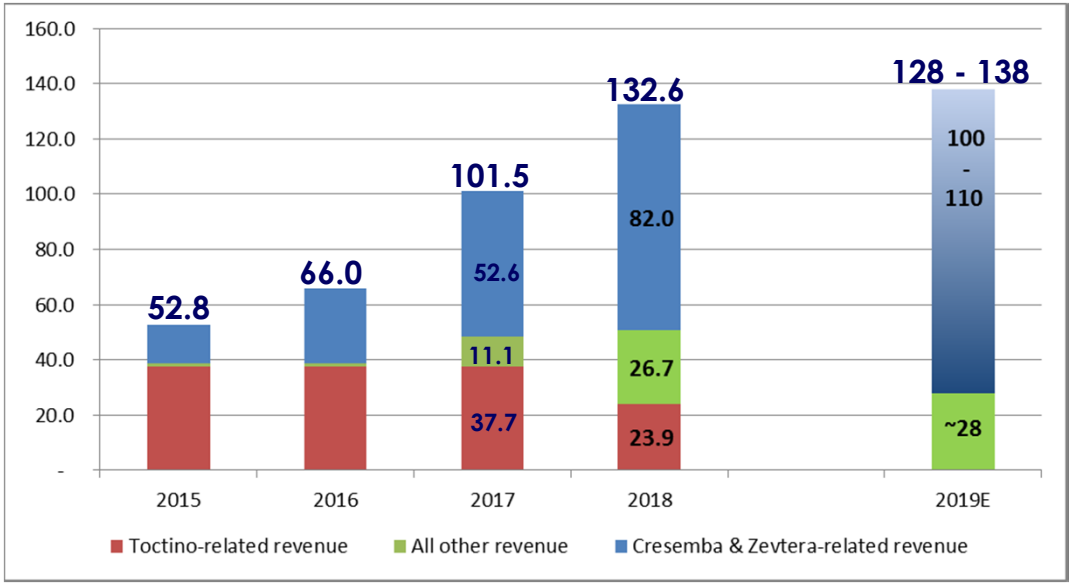


- In-licensed novel, oral, small molecule drug from consortium around Wellcome Trust & Institute of Cancer Research (ICR)
- Dual-targeting kinase inhibitor
- Targets resistance mechanisms associated with approved BRAF inhibitors (including vemurafenib and dabrafenib)
- Resistance-reversal activity in 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 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
thereof: Contributions from Cresemba & Zevtera	*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		Complete patient enrolment in phase 2 registrational study in iCCA	Top line results from phase 2 registrational study in iCCA
	✓ Collaboration with Roche in urothelial cancer	Start phase 2 study in urothelial cancer		Interim data from first cohort(s) in urothelial cancer
	Expand ongoing iCCA study in other FGFR gene aberrations			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 · SmithKline Beecham

2014

Donato Spota – CFO

Roche

2002

Marc Engelhardt, MD, Ph.D. – CMO

NOVARTIS · BRACCO

2010

Dr. Gerrit Hauck - Ph.D. – CTO

SANOFI

2018

Adesh Kaul – CCDO

POLYPHOR · Genedata solutions in silico

2009

Laurenz Kellenberger, Ph.D. – CSO

UNIVERSITY OF CAMBRIDGE · Roche

2000

Antifungal

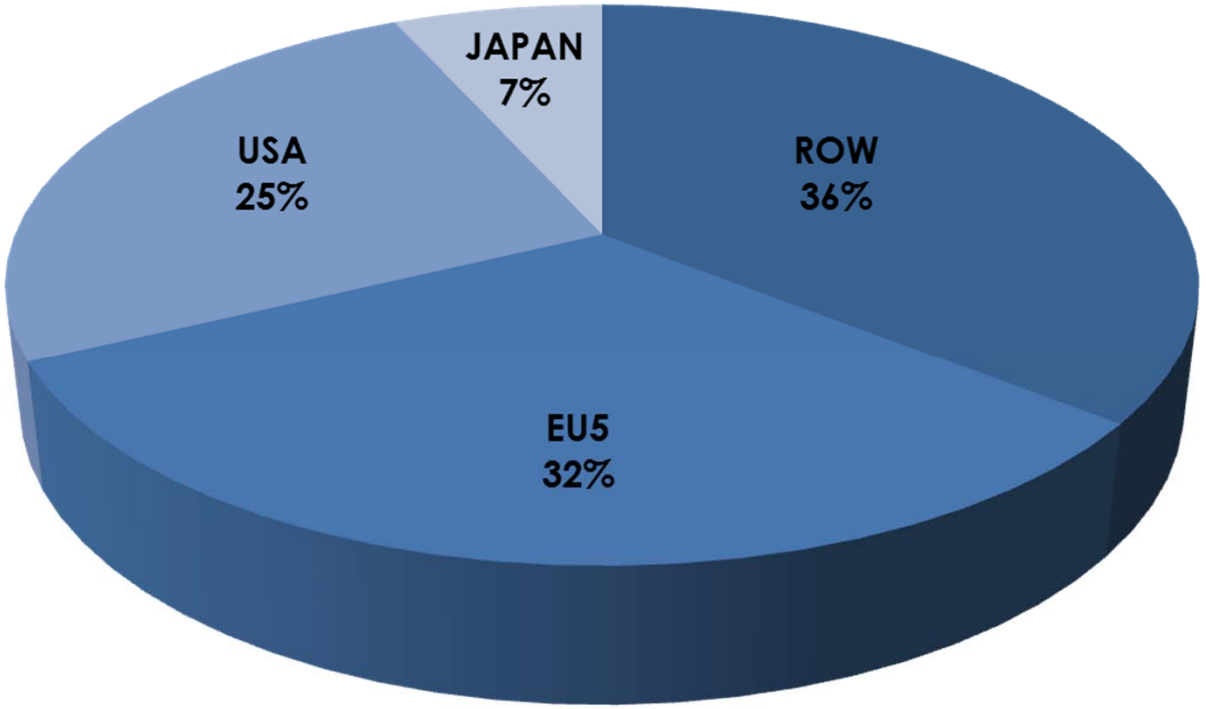
Cresemba®
(isavuconazole)

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

basilea
PHARMACEUTICA

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

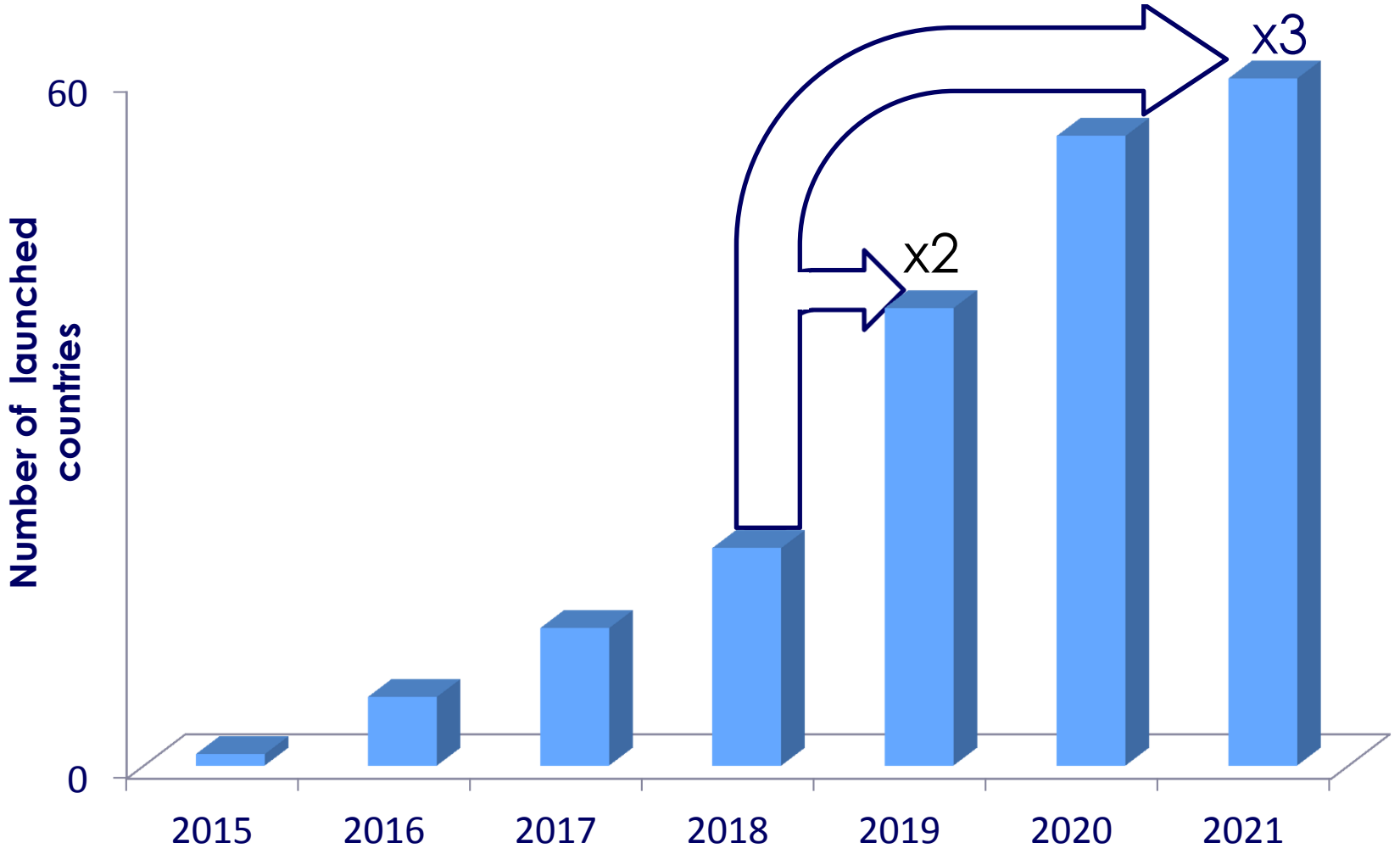
USD 3.0 bn sales of best-in-class antifungals* (MAT Q3 2018)



* 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, September 2018

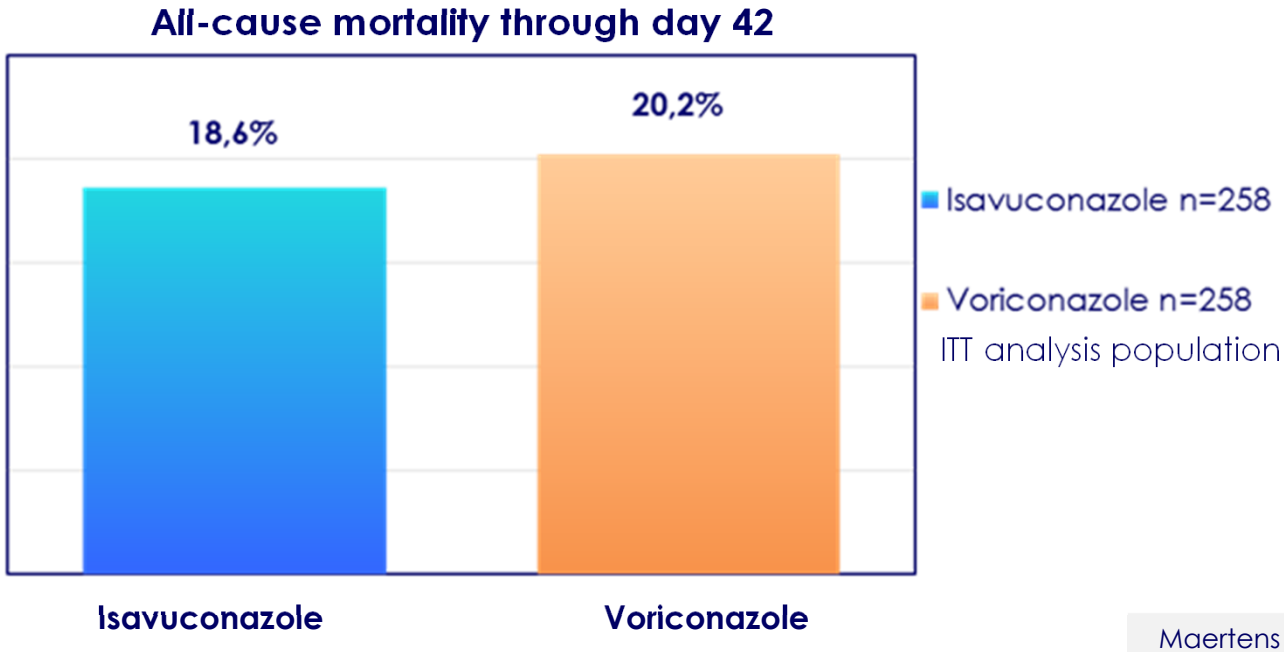
Cresemba – strong global roll out



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)

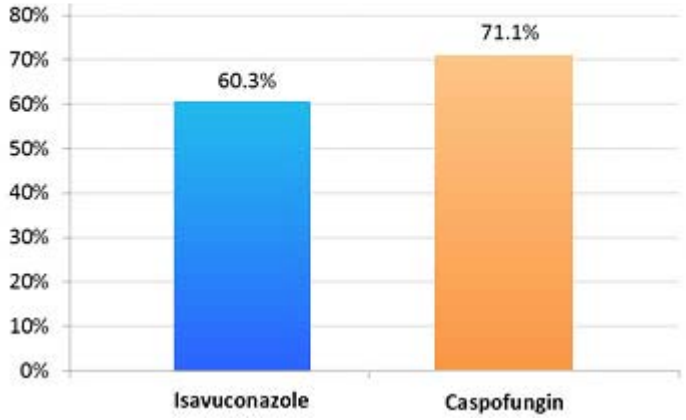
- All-cause-mortality through day 42 in renally impaired patients with invasive aspergillosis (n=20) **for which i.v. voriconazole can only be used with caution:**
  - 15% (vs. 18.6% benchmark in SECURE study, excluding patients with moderate to severe renal impairment)*
- All-cause-mortality through 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

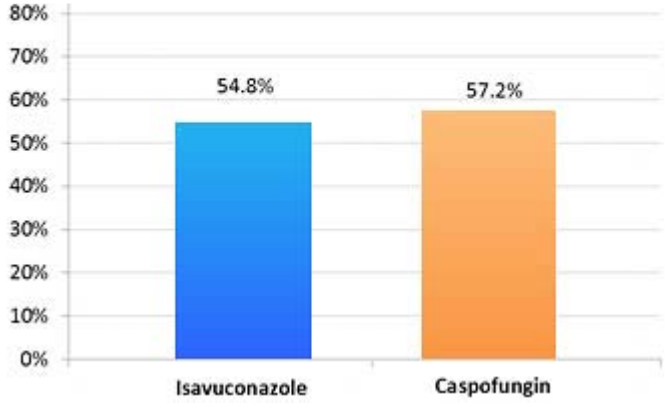
ACTIVE: Primary treatment of candidemia/invasive candidiasis[1]

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



N=199 N=201

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



mITT N=199 N=201

- Overall response at end of i.v. treatment similar to other azoles and amphotericin B[2]
- Overall response 2 weeks after end-of-therapy comparable to caspofungin/voriconazole[1]
- All-cause mortality at day 14 and day 56 comparable to caspofungin/voriconazole[1]

[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

Antibacterial

Zevtera®/Mabelio® (ceftobiprole)

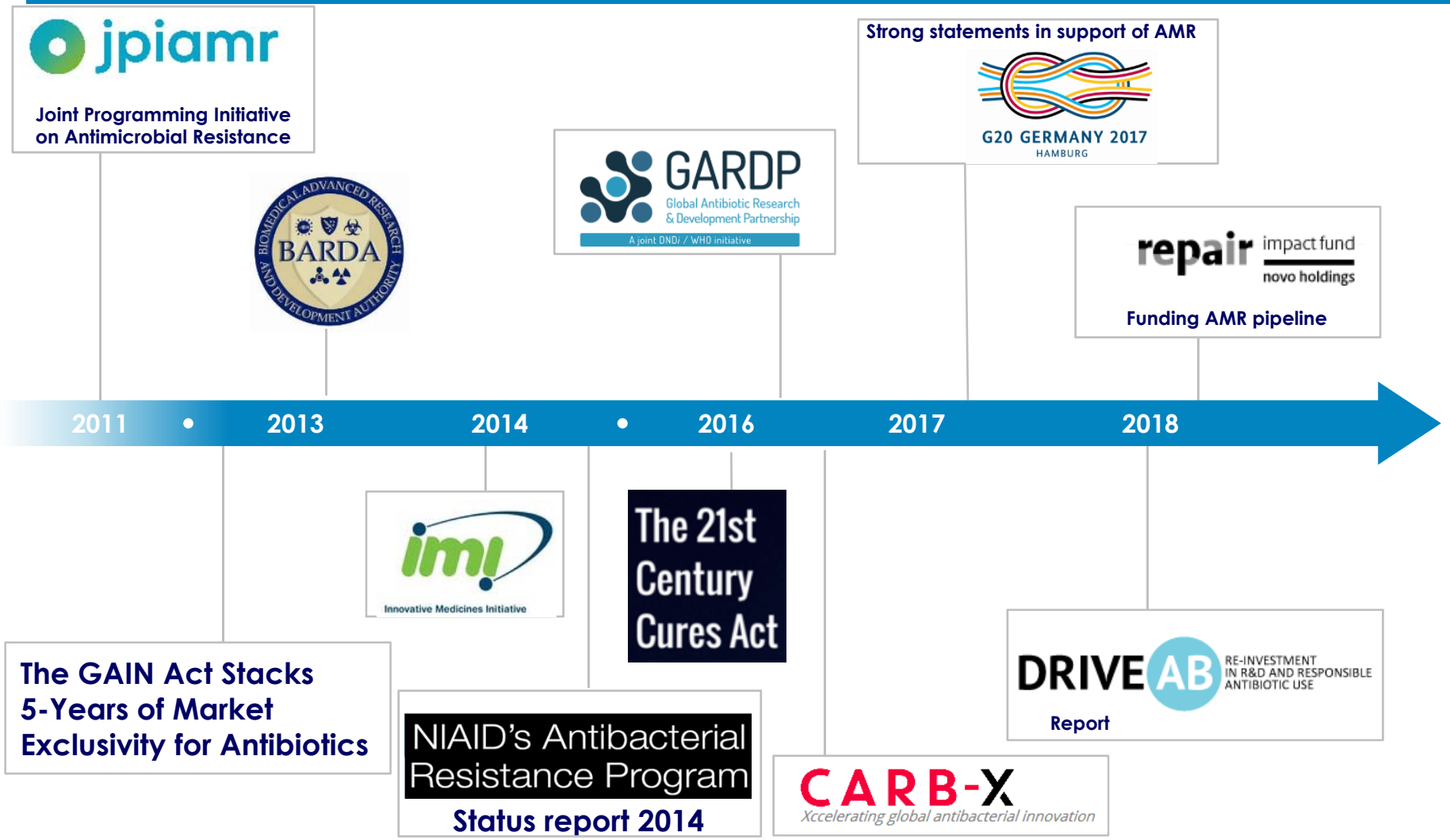
European box/vials
Ceftobiprole is not approved in the U.S.

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

* HAP (excluding VAP)

basilea PHARMACEUTICA

Improving political and regulatory environment for novel antibiotics and antifungals



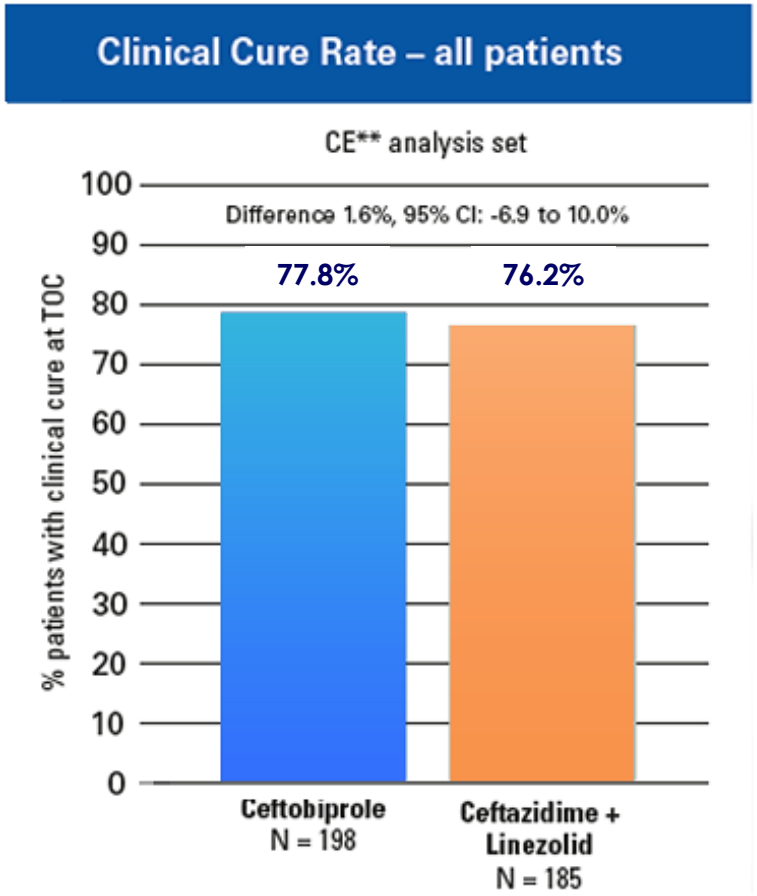
Phase 3 study with ceftobiprole in the treatment of patients with ABSSSI

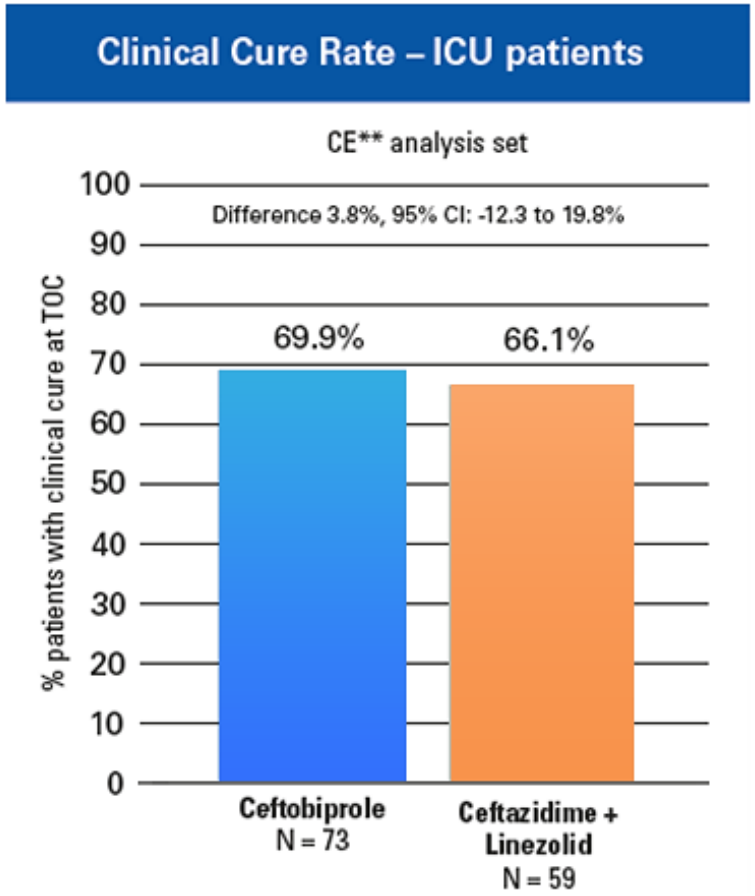
- **Design:** randomized, double-blind, multi-center
- **Enrolment:** approximately 674 adult patients (male and female)
- **Indication:** acute bacterial skin and skin structure infection (ABSSSI)
- **Main inclusion criteria:** diagnosis of ABSSSI, requirement of i.v. treatment
- **Intervention**: ceftobiprole medocaril 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 medocaril 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*

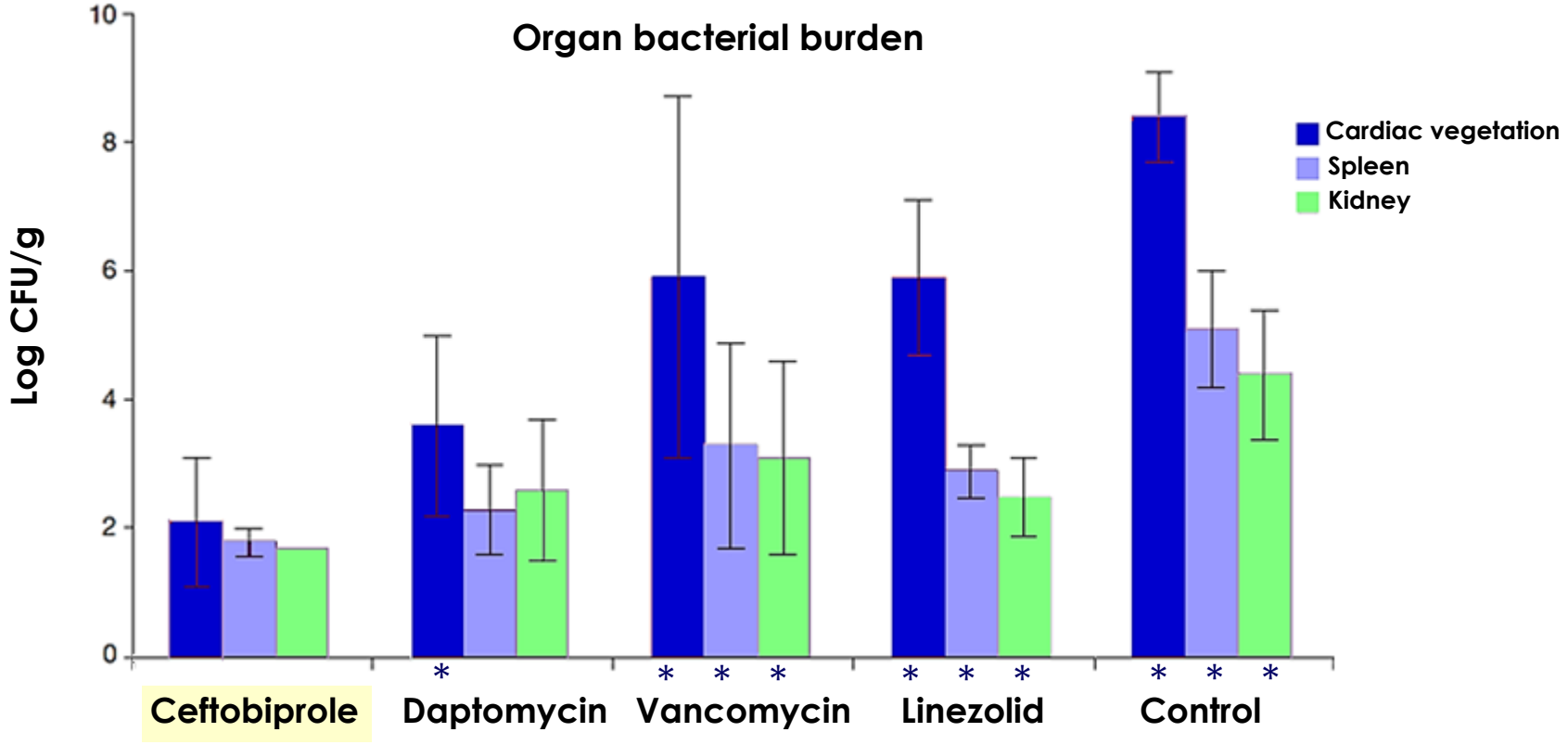




* Excluding VAP; ** Clinically evaluable

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)

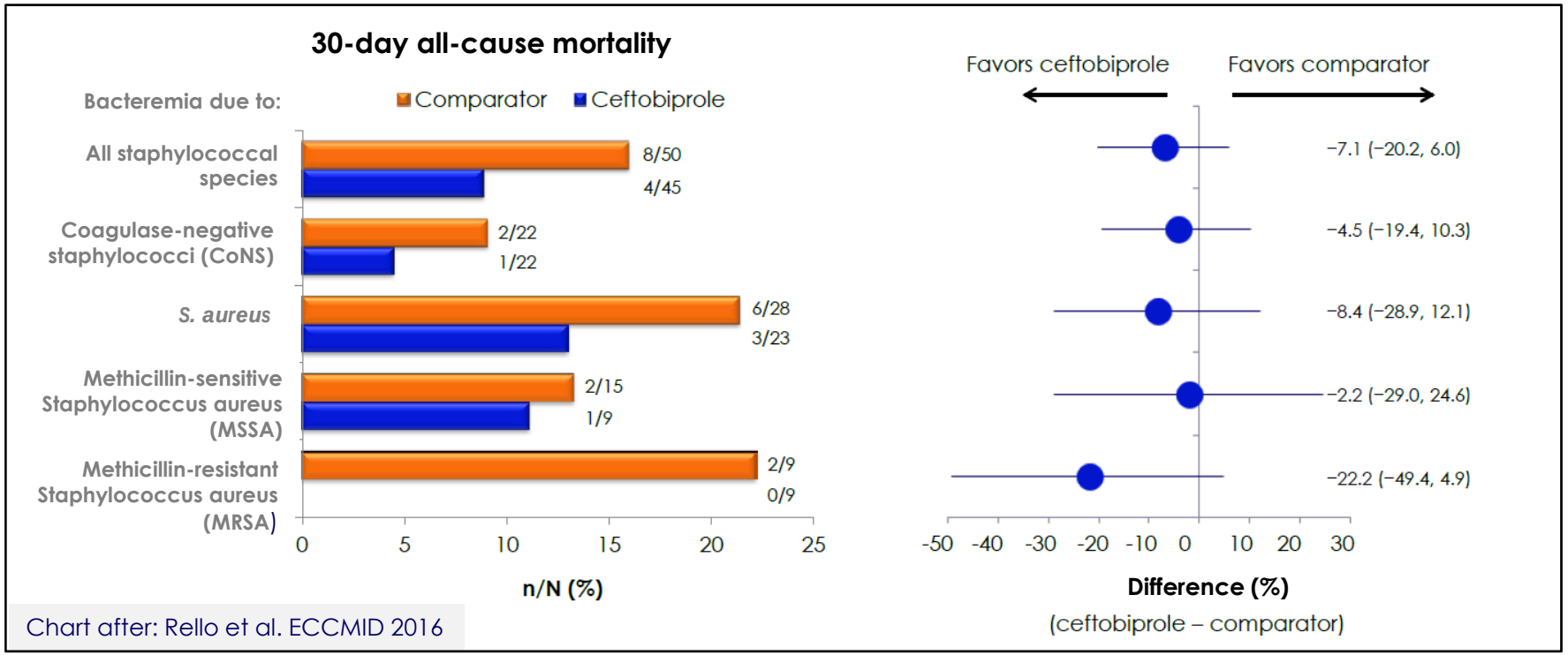


Chart after: Rello et al. ECCMID 2016

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

* *Staphylococcus aureus* bacteremia

Oncology

Derazantinib (BAL087)

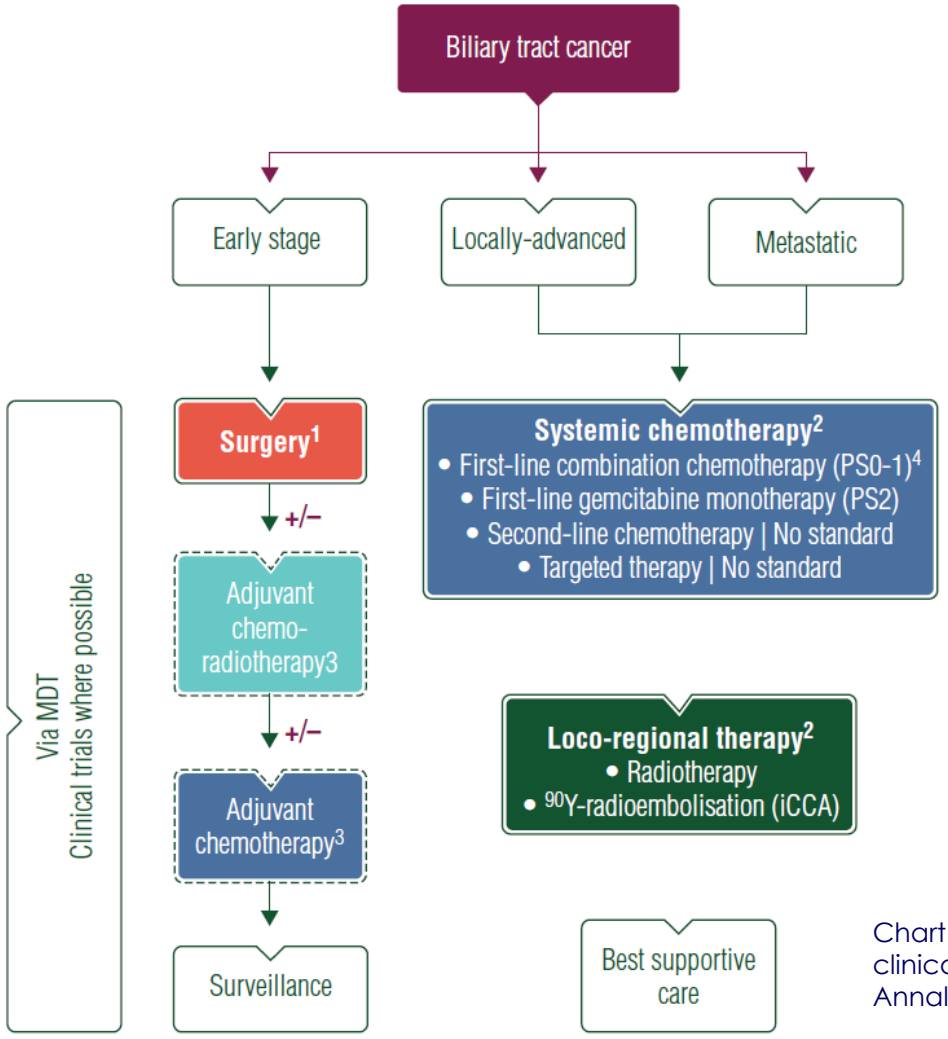
panFGFR kinase inhibitor
for various solid tumors

basilea
PHARMACEUTICA

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

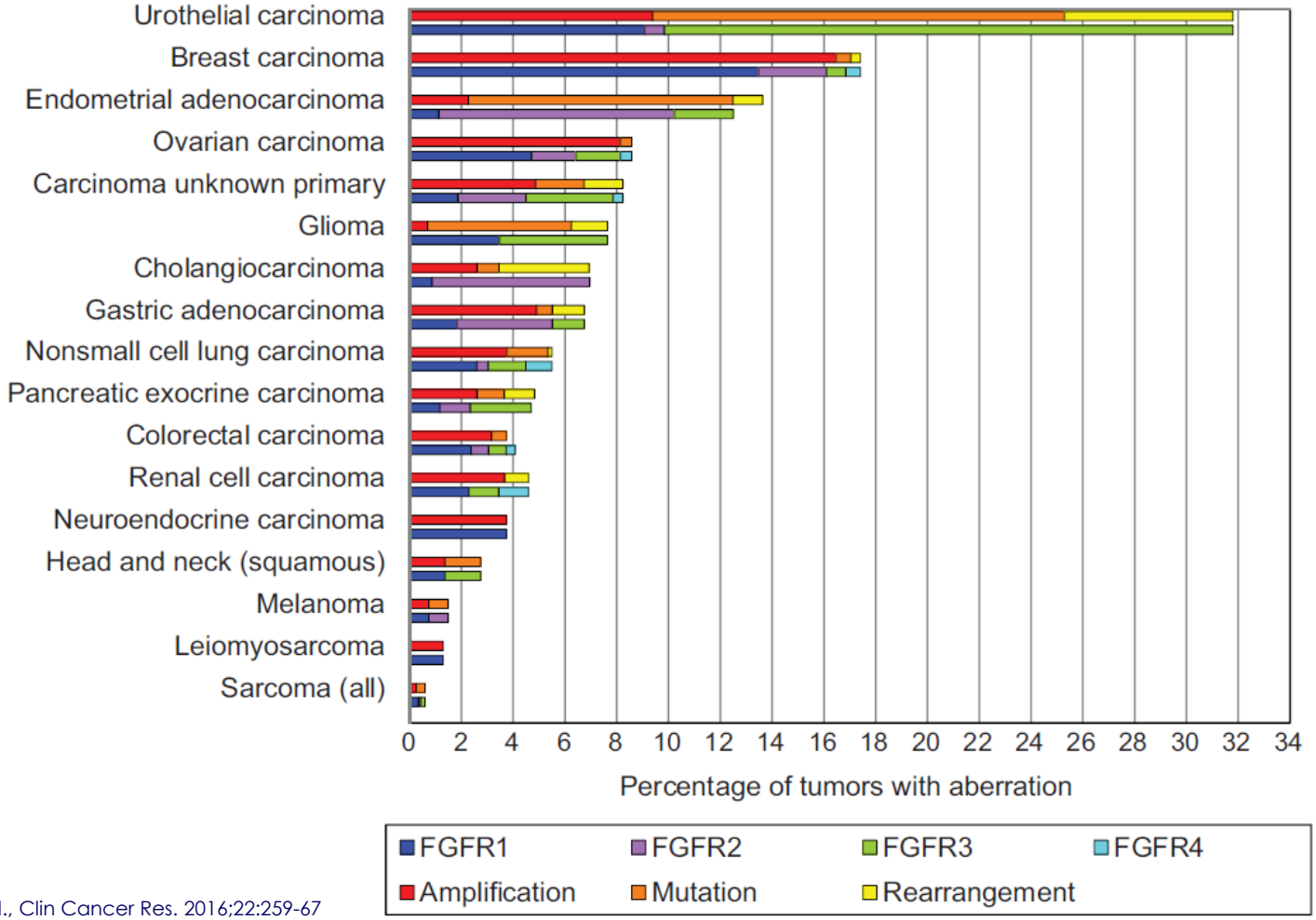


Seeking to establish derazantinib as second-line standard for FGFR2-fusion positive iCCA

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

Grenzacherstrasse 487 • PO Box • 4005 Basel • Switzerland
investor_relations@basilea.com • www.basilea.com