



Investor presentation

September 2019



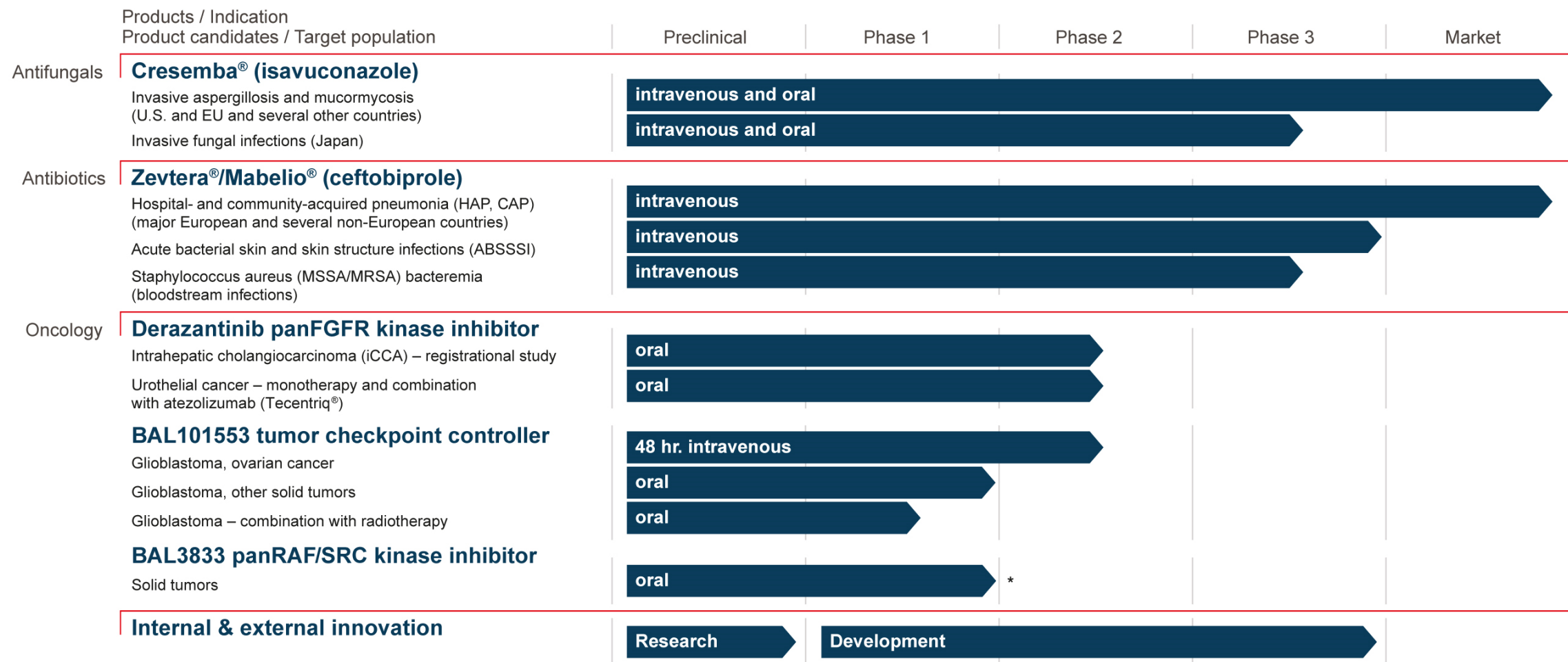
“Patients are at the heart
of what we do”

Basilea at a glance

- Revenue-generating, commercial-stage biotech company with solid cash position (HY2019 ~CHF 178mn)
- Focused in the areas of oncology, hospital antifungals and hospital antibiotics
- 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
- Listed on the SIX Swiss Stock Exchange (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 ongoing.

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

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

License partners

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

Distribution partners

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

>100 countries covered by partnerships

Ongoing participation

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



Global partnerships for Cresemba®



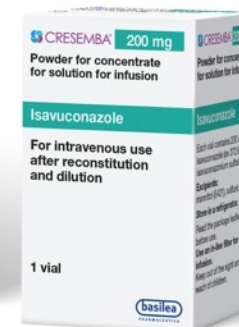
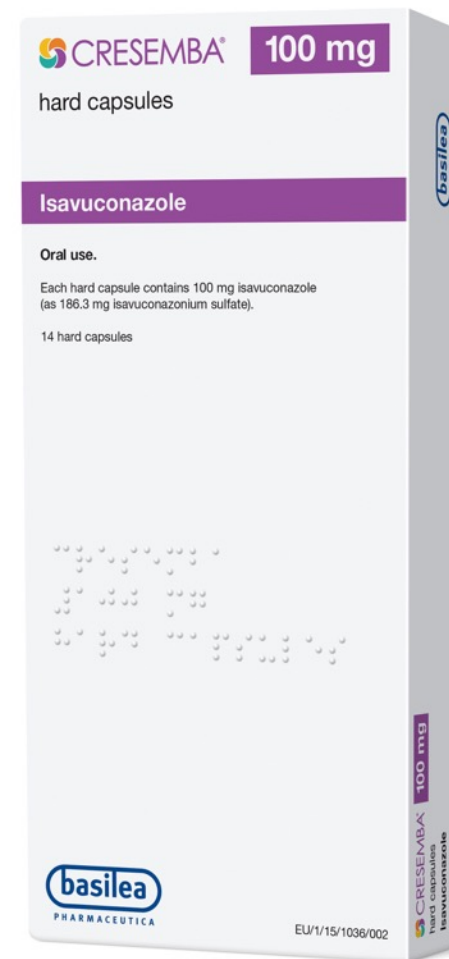
Global partnerships for Zevtera[®]/Mabelio[®]



Antifungal

Cresemba® (isavuconazole)

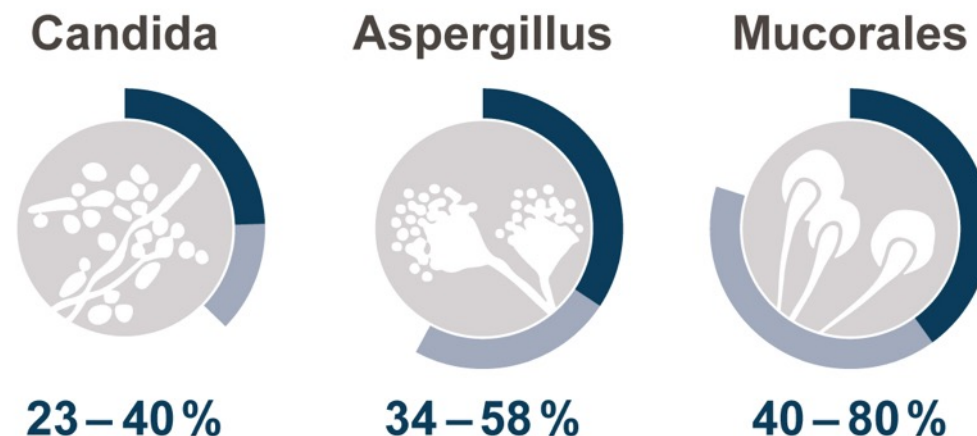
Invasive mold infections



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

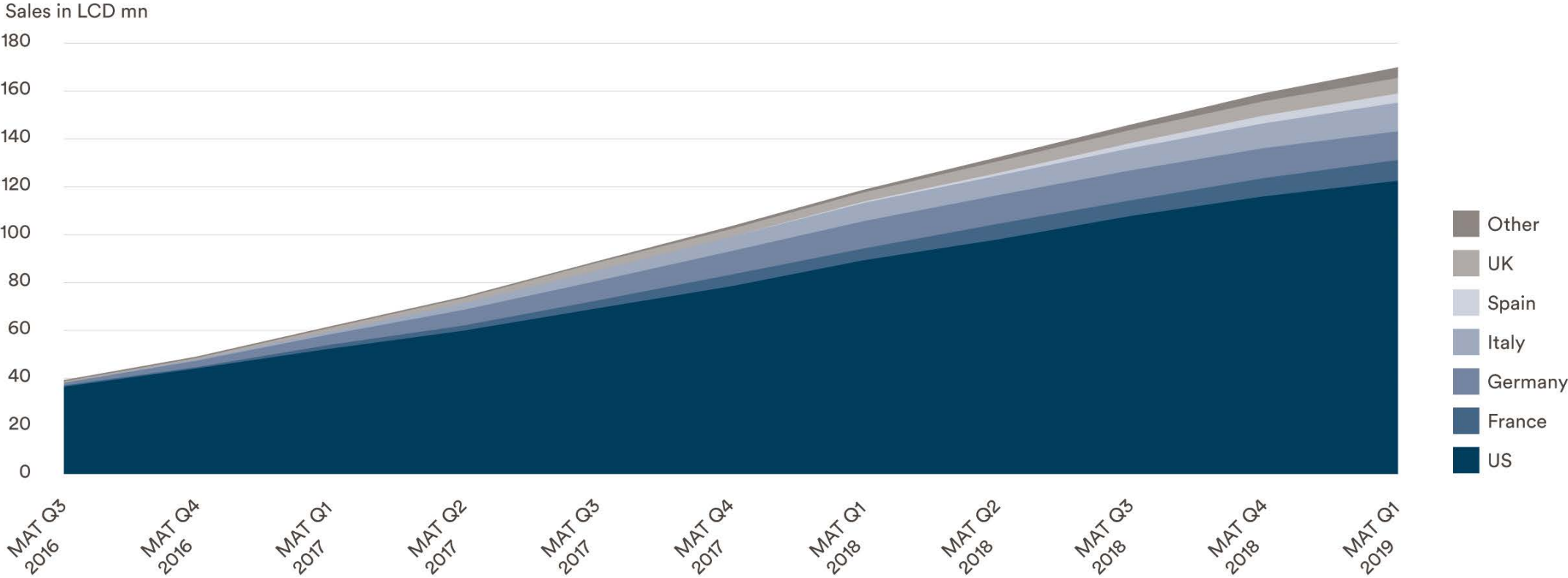
Mortality rates for invasive fungal infections**



**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

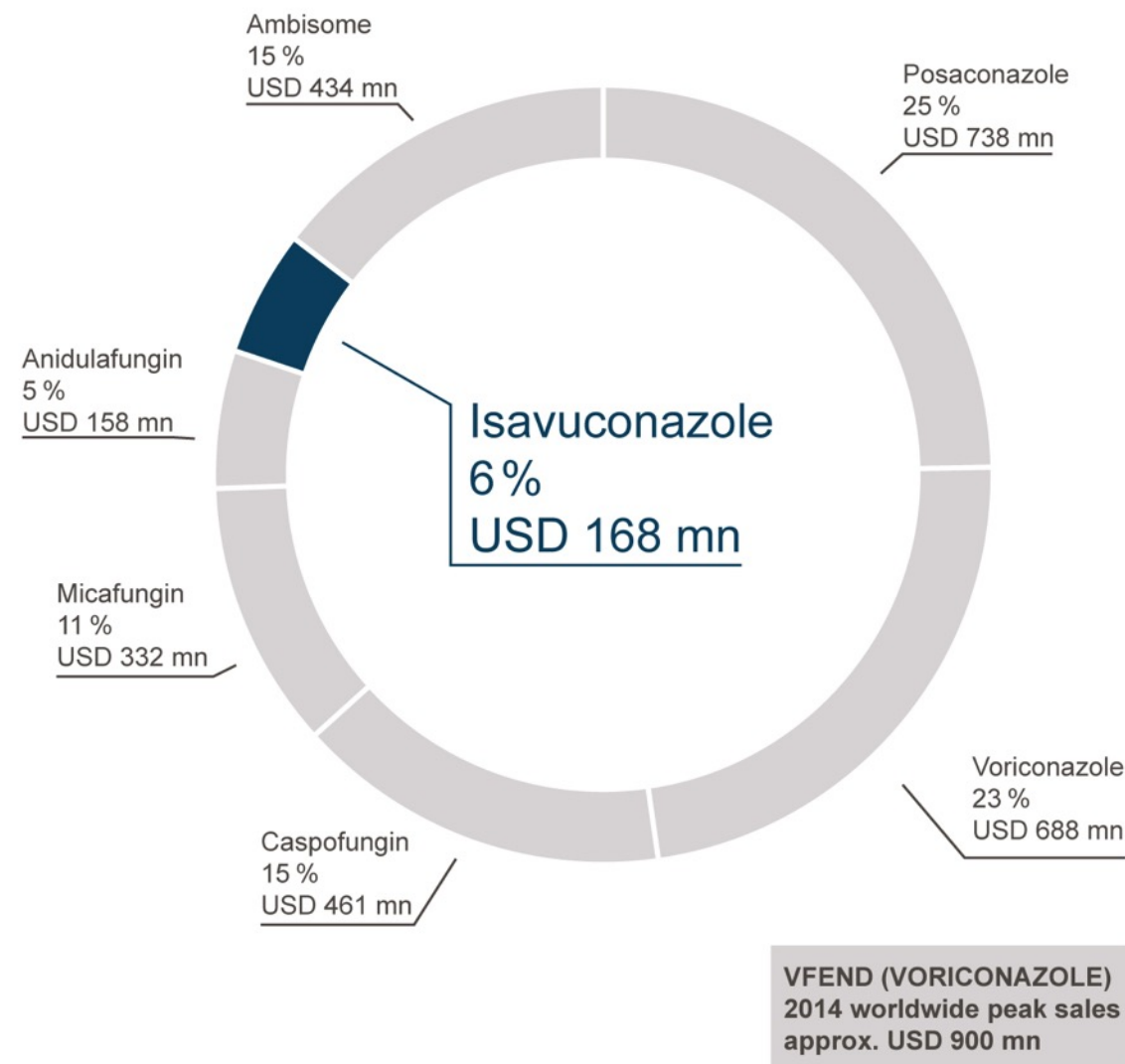
Approx. USD 170mn in-market sales in MAT Q1 2019



LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, March 2019

Sales of best-in-class antifungals* by product

USD 3bn sales (MAT Q1 2019)



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

MAT: Moving annual total; Sales figures in USD, corrected for currency fluctuations;
Source: IQVIA, March 2019

Cresemba® — Differentiated by spectrum, safety and tolerability

- 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.

Cresemba® — Marketed in North America, Europe, Israel and selected Latin American countries

- Marketed in major European countries by Pfizer
 - USD 5mn sales milestone triggered in Q1 2019
- Marketed in the U.S. by Astellas
 - Astellas reported H1 2019 sales of USD 67mn (+24% Y-o-Y)
 - CHF 10mn sales milestone triggered in Q4 2018
- Anticipated to double the number of launched countries in 2019
- Exclusivity through 2027 in the U.S. and potential pediatric exclusivity extension to 2027 (from 2025) in the EU



Antibacterial

Zevtera[®]/Mabelio[®] (ceftobiprole)

Hospital*-and community-acquired pneumonia



* HAP (excluding VAP)

basilea

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

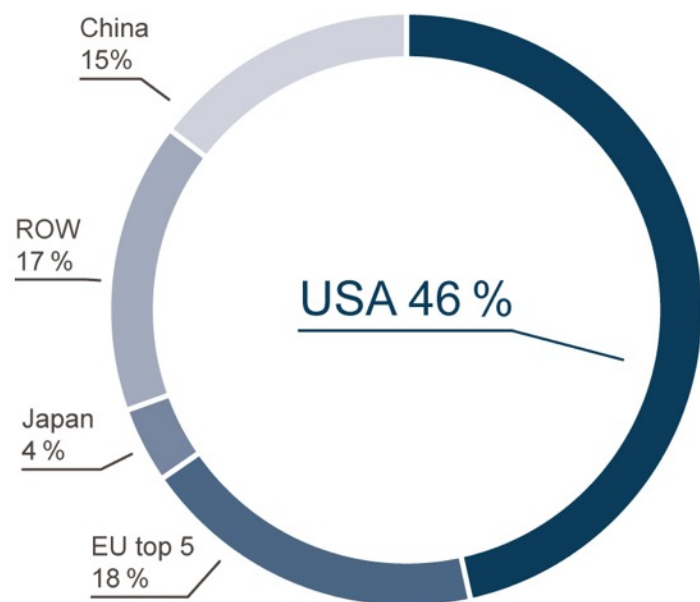
- Broad-spectrum anti-MRSA cephalosporin (including Gram-negative bacteria)
- Rapid bactericidal activity
- Potential to replace antibiotic combinations
- Early improvement in HAP, particularly in patients with MRSA, and CAP, including high-risk patients
- Cephalosporin class safety profile
- Marketed in selected countries in Europe, Latin America and the MENA-region as well in Canada

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

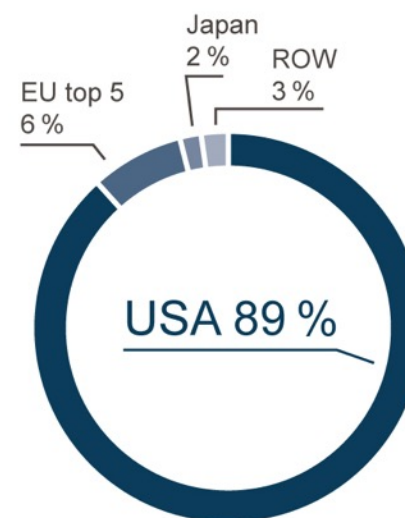


Anti-MRSA hospital antibiotics market — A USD 3.1bn market with the U.S. being the most important region

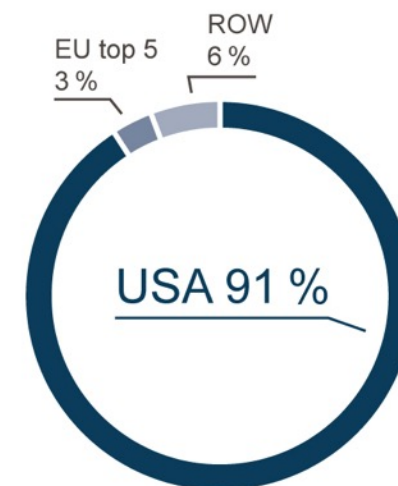
Global anti-MRSA hospital antibiotics sales*
USD 3.1bn (MAT Q1 2019)



Daptomycin sales
by region 2015
(before LOE)



Ceftaroline sales
by region
(MAT Q1 2019)



* Vancomycin, linezolid, teicoplanin, daptomycin, tigecycline, telavancin, ceftaroline, dalbavancin, oritavancin and tedizolid
MRSA: Methicillin-resistant *Staphylococcus aureus*; LOE: Loss of exclusivity; ROW: Rest of world
MAT: Moving annual total; Sales figures in USD, corrected for currency fluctuations; Source: IQVIA, March 2019

Two phase 3 studies are required to gain U.S. regulatory approval for ceftobiprole

- Two cross-supportive studies under FDA Special Protocol Assessment (SPA)
- Acute Bacterial Skin and Skin Structure Infections (successfully completed)¹
- Partial funding of phase 3 program by BARDA (up to USD 128mn, ~70% of total program costs)



- *Staphylococcus aureus* bacteremia (ongoing, anticipated to report topline results in H2 2021)²



- Qualified Infectious Disease Product (QIDP) designation extends U.S. market exclusivity to 10 years from approval

¹ NCT03137173

² NCT03138733

Ceftobiprole — positive topline phase 3 results reported in ABSSSI

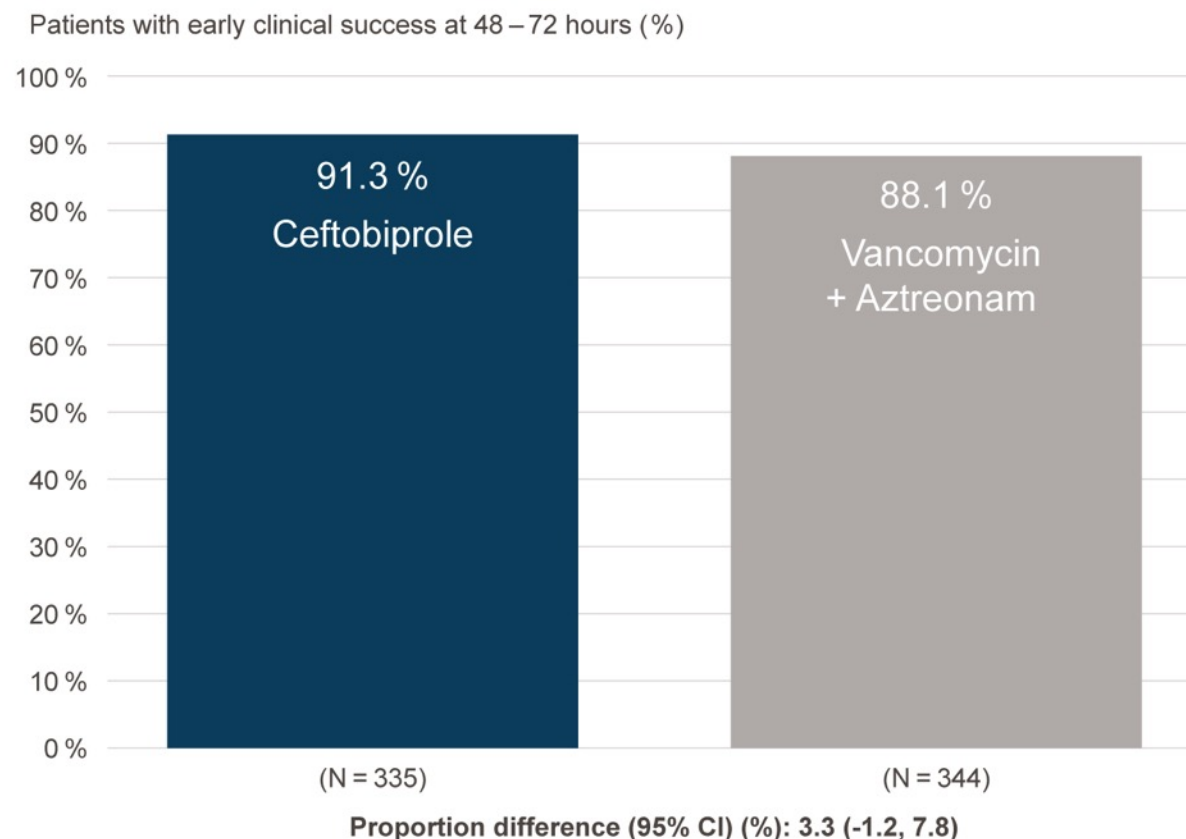
Key topline study¹ results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints



¹ NCT03137173

ABSSSI: Acute bacterial skin and skin structure infections

Early clinical response at 48–72h after start of treatment (ITT population)



ITT: intent-to-treat

Pre-defined limit of non-inferiority = lower limit of 95 % CI for difference > -10 %

Ceftobiprole — positive topline phase 3 results reported in ABSSSI

Key topline study¹ results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints

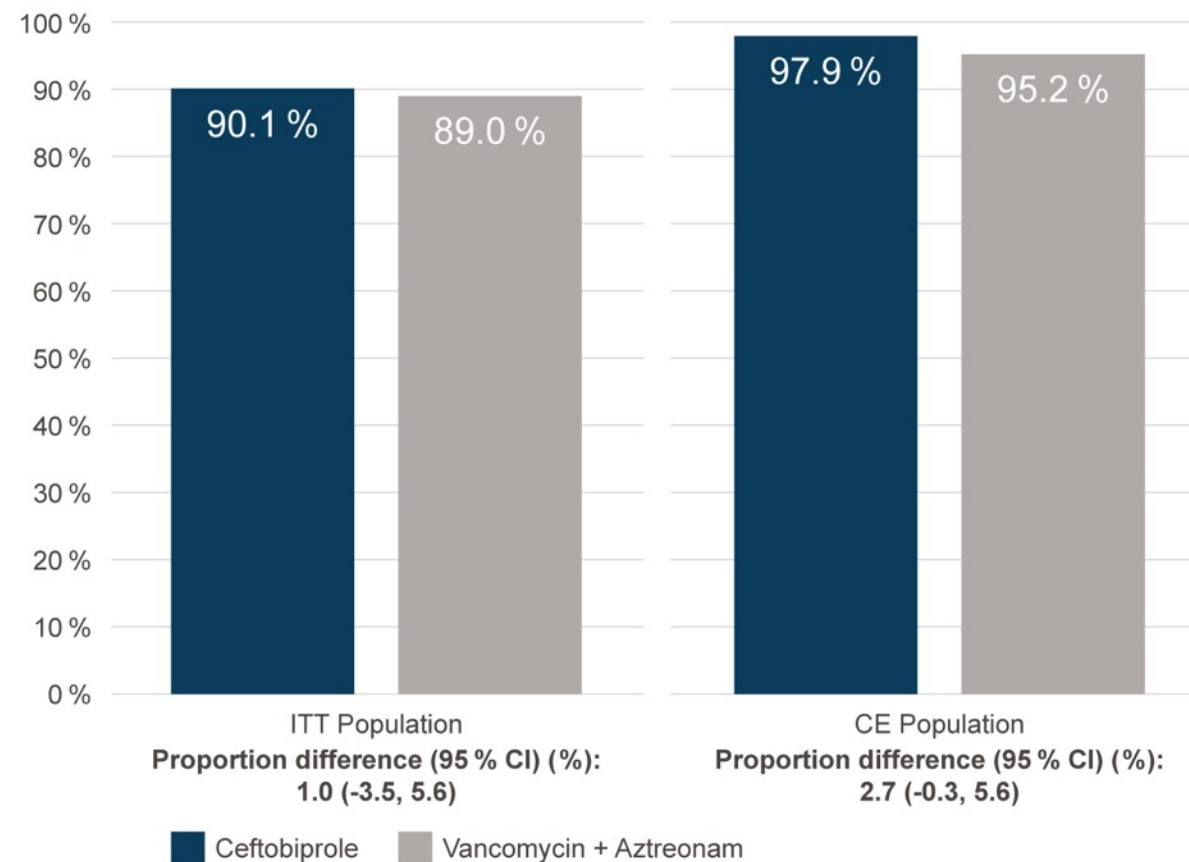


¹ NCT03137173

ABSSSI: Acute bacterial skin and skin structure infections

Investigator-assessed clinical success at test-of-cure (TOC) 15-22 days after randomization (ITT, CE populations)

Patients with clinical success at the TOC visit (%)



CE: clinically evaluable; ITT: intent-to-treat

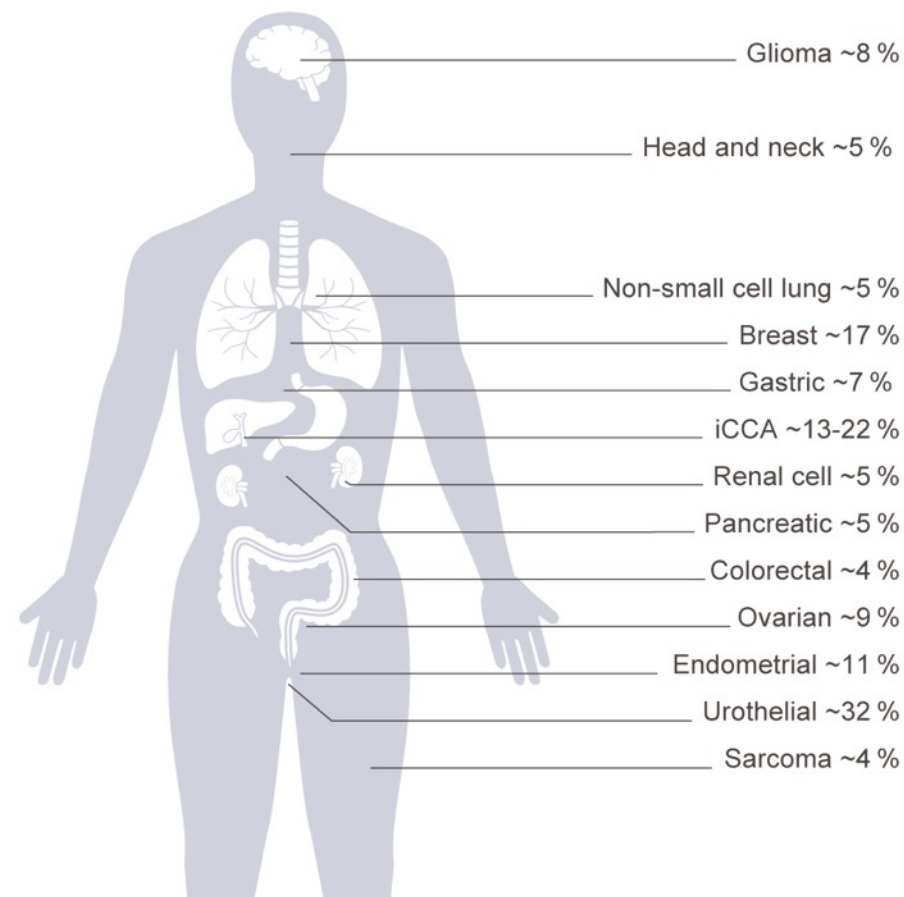
Oncology

Derazantinib

FGFR-driven 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
 - panFGFR kinase inhibitor with strongest activity against FGFR1, 2 and 3
- Development strategy focused on achieving differentiation by leveraging unique properties of derazantinib
 - Kinase inhibition profile: exploring therapeutic potential of additional targets of derazantinib such as CSF1R (Colony-stimulating Factor 1 Receptor) kinase
 - Safety profile: exploring relevance for potential combination therapies
- Two clinical studies ongoing
 - Urothelial cancer phase 1/2 study: Monotherapy and in combination with immune-checkpoint inhibitor atezolizumab (Tecentriq®)
 - Intrahepatic cholangiocarcinoma (iCCA) registrational phase 2 study: Monotherapy in FGFR2 gene fusions and other FGFR2 genetic aberrations



Sources: Helsten et al., Clin Cancer Res 2016 (22), 257-267; FGFR2 fusions in iCCA: Graham et al. Hum Pathol 2014 (45), 1630-1638; Jain et al. JCO Precis Oncol 2018 (2) 1-12

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

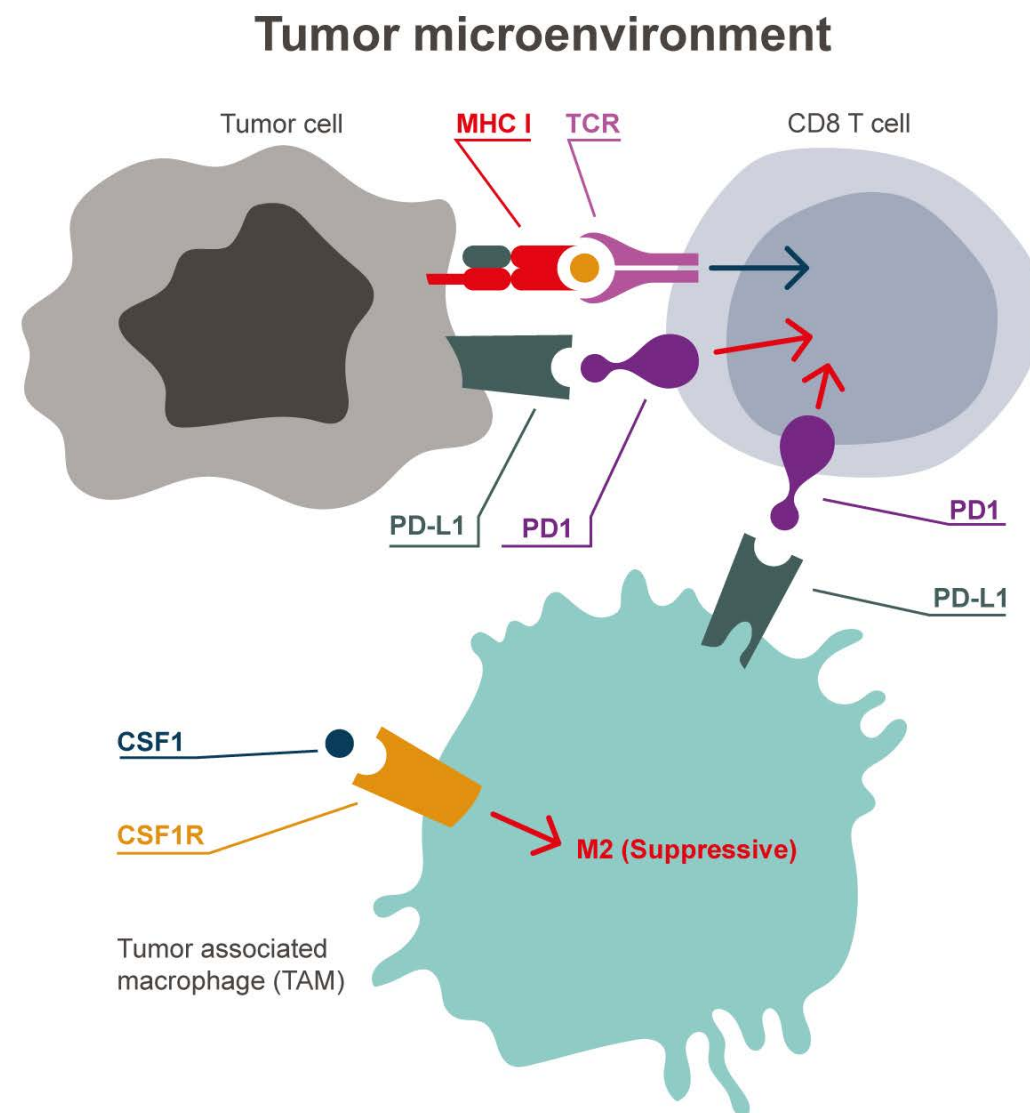
Source: Basilea data on file

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 clinical supply agreement with Roche to study a combination of derazantinib and Roche's PD-L1-blocking immune-checkpoint inhibitor atezolizumab (Tecentriq®) in patients with urothelial cancer

Sources:

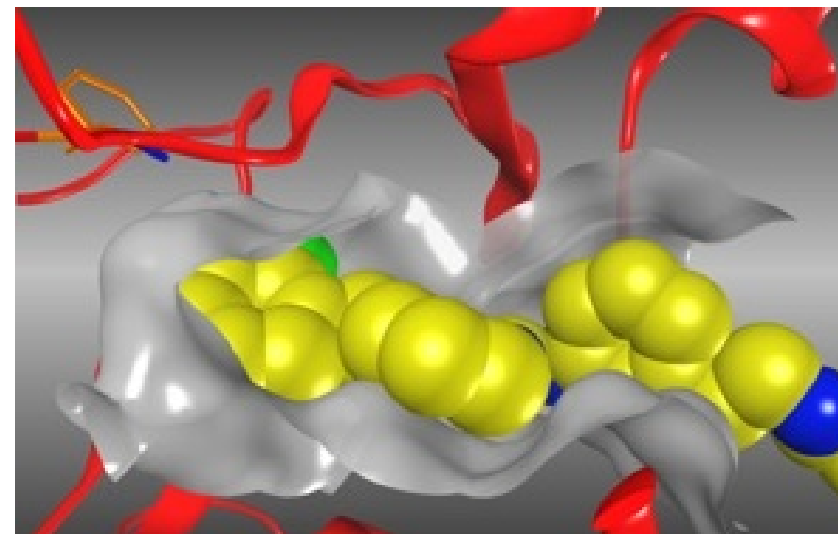
¹ X. Zheng et al. Redirecting tumor-associated macrophages to become tumoricidal effectors as a novel strategy for cancer therapy. *Oncotarget*. 2017;8(29):48436-48452



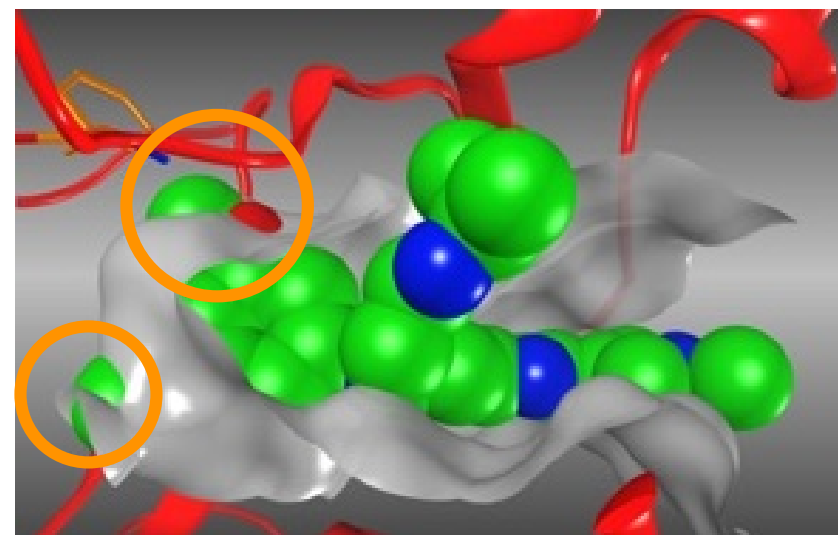
² Graph adapted from: A. Ghasemzadeh et al. New Strategies in Bladder Cancer: A Second Coming for Immunotherapy. *Clin Cancer Res*. 2016;22(4):793-801

In-silico analysis of derazantinib binding to CSF1R

- Crystal structures¹ indicate differences in inhibitor binding sites of FGFR and CSF1R kinases
- Improved kinase inhibition activity of derazantinib against CSF1R versus other FGFR-inhibitors can be explained by the unique chemical structure of derazantinib



Derazantinib (yellow) fits to smaller active site pocket of CSF1R (grey/red)



Erdafitinib (green) is too large (orange circles) for the active site pocket of CSF1R (grey/red)

¹ FGFR: 3RHX.pdb, J.Biol.Chem. 286: 20677-20687 (2011); CSF1R: 3LCD.pdb, Bioorg.Med.Chem.Lett. 20: 1543-1547(2010)

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%]	<i>NR</i>	36% [4%]	32% [6%]	≥21% [≥2%]
Alopecia[†]	28%	38%	<i>NR</i>	37%	<i>NR</i>	≥27%
Dry eye/xerophthalmia[†]	21%	32%	<i>NR</i>	20%	<i>NR</i>	≥19%
Central serous retinopathy	0%	<i>NR</i>	<i>NR</i>	<i>NR</i>	<i>NR</i>	21%
ALT ↑	31%	<i>NR</i>	31%	<i>NR</i>	<i>NR</i>	41% ⁷
Hand-foot syndrome/PPE	0%	27%	22%	<i>NR</i>	<i>NR</i>	≥22%
Nail events (drug-related)	<5%	<i>NR</i>	<i>NR</i>	<i>NR</i>	<i>NR</i>	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; ⁷ Balversa™ U.S. prescribing information (April 2019) based on reported laboratory abnormalities N=86 patients, regardless of causality.

Abbreviations: DZB: derazantinib, INF: infgratinib (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

Derazantinib — Multi-cohort phase 1/2 study in advanced urothelial cancer (FIDES-02)¹

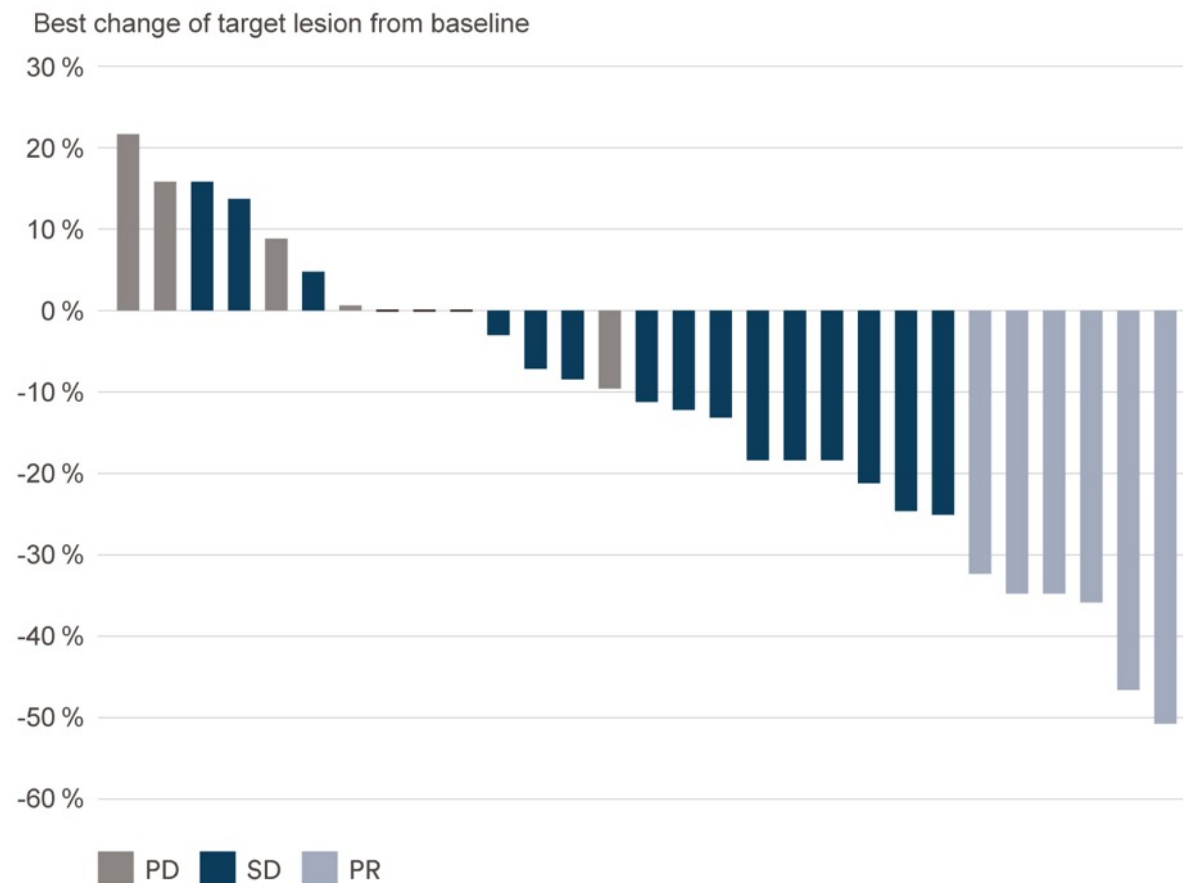
- Derazantinib as single agent and in combination with atezolizumab (Tecentriq®) in patients with advanced urothelial cancer testing positive for mutations or fusions of FGFR1, FGFR2 or FGFR3 genes
- The subgroup of patients with low PD-L1 expression have limited clinical benefit from the treatment with PD1/PD-L1 inhibitors. This subgroup, however, shows frequent FGFR genomic abnormalities (mainly FGFR3 fusions)
- Derazantinib combined with PD1/PD-L1 inhibitors may provide benefits related to multiple mechanisms (FGFR-inhibition, macrophage modulation, enhanced response to immunotherapy), in particular in the low PD-L1 expression subgroup
- Across a total of four sub-studies, FIDES-02 potentially can enroll up to approximately 300 patients
- Patient cohorts in various treatment settings, including:
 - Post-chemotherapy/immunotherapy recurrence (second-and post second-line)
 - First-line platinum-ineligible
 - Resistance to prior FGFR-inhibitor treatment
- Study conducted in multiple centers in Asia-Pacific, Europe and North America
- Clinical supply agreement with Roche for the immune-checkpoint inhibitor atezolizumab (Tecentriq®)

¹ NCT04045613

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*
- 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^{1, 4}

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 tumors. British Journal of Cancer 2017, 1-8



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

Derazantinib — Potential for accelerated U.S. approval based on registrational phase 2 study in iCCA (FIDES-01)¹

Cohort 1: 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
- Topline data expected mid-2020

Cohort 2: Patients with FGFR2 gene mutations or amplifications

- Started in June 2019
- Assessing the activity of derazantinib in a broader range of FGFR2-driven tumors
- Define the full therapeutic potential of derazantinib in iCCA with potential for differentiation
- Interim data expected H2 2020

¹ NCT03230318

Oncology

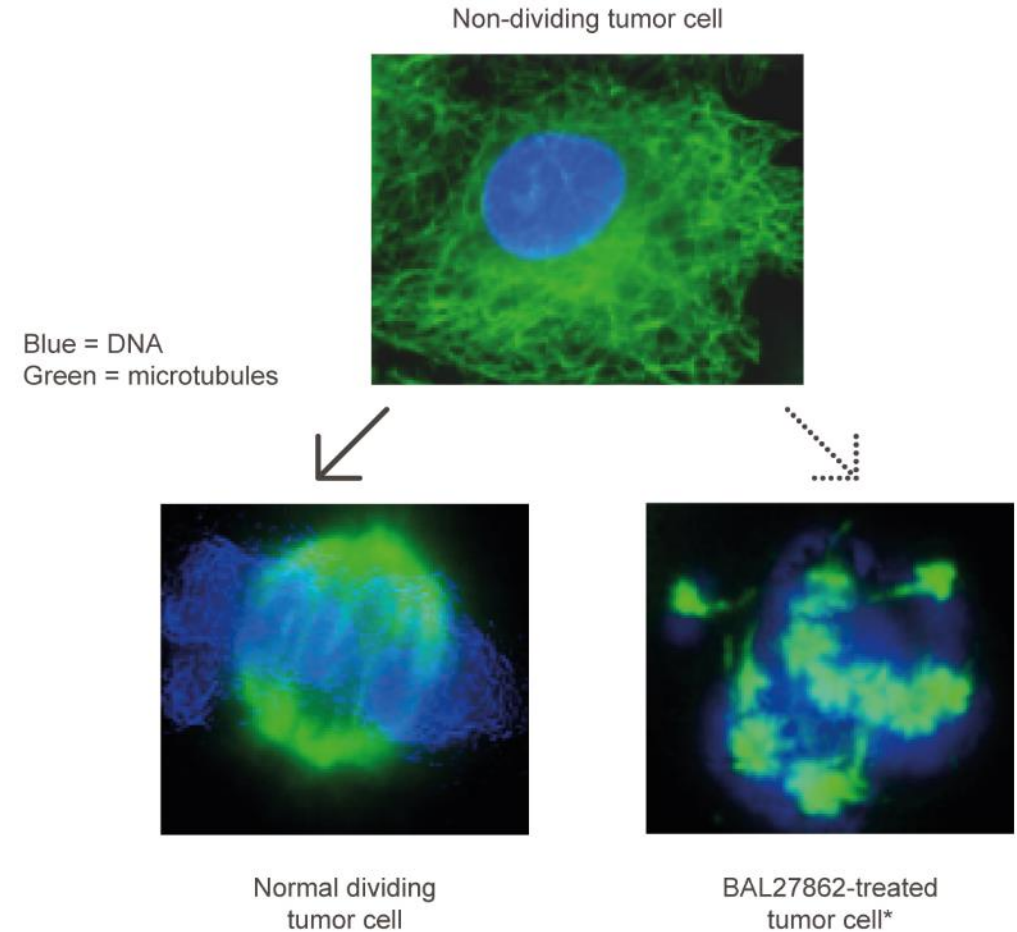
BAL101553

Glioblastoma and ovarian cancer



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

- Novel compound inducing tumor cell death through spindle assembly 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

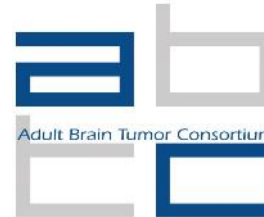


* BAL101553 is a prodrug of BAL27862

Proprietary information of Basilea Pharmaceutica International Ltd. – not for distribution

BAL101553 — Three ongoing clinical studies

- Phase 2a expansion (weekly 48-hour i.v.) in patients with recurrent glioblastoma (GBM) or platinum-resistant ovarian cancer¹
 - Anticipated to complete around year-end 2019
- Phase 1 dose escalation (daily oral) in patients with recurrent glioblastoma²
 - Completed patient enrolment in August 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)³
 - Anticipated to complete patient enrolment mid-2020



¹ NCT02895360

² NCT02490800

³ NCT03250299; the ABTC is funded by the U.S. National Cancer Institute (NCI)

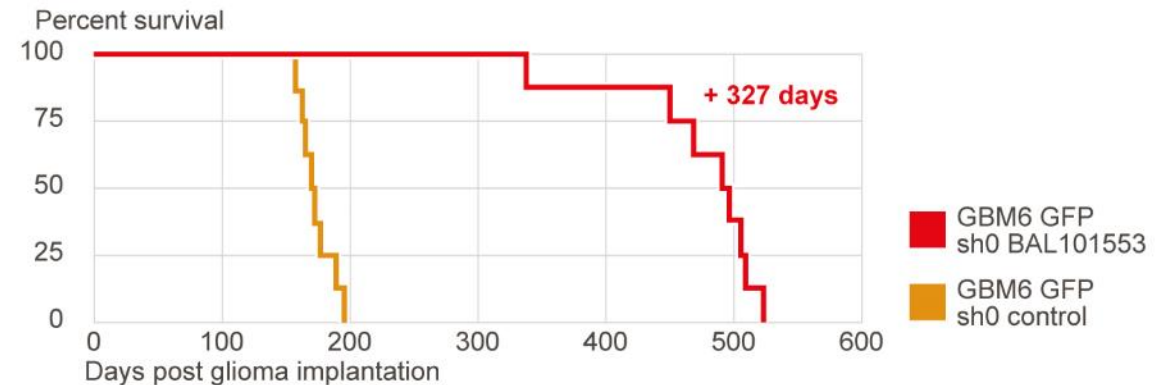
EB1 — A potential response-predictive clinical biomarker for BAL101553

- EB1 (plus-end binding protein)¹ is located on the microtubules and involved in microtubule dynamics
- Predictive of response to BAL101553 in mouse models¹

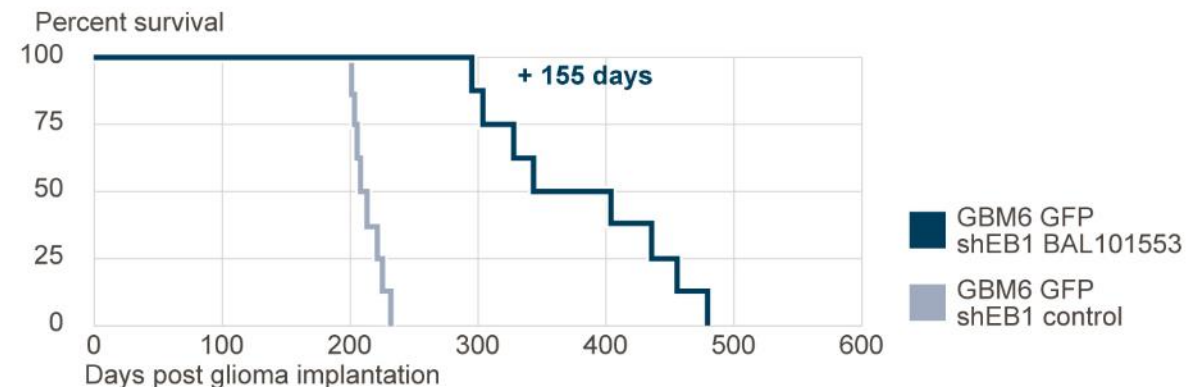
¹ Berges et al. Eur. J. Cancer 2018, 103,E61-62

Effect of BAL101553 on survival in mice with EB1-expressing or EB1 downregulated GBM

EB1-expressing GBM



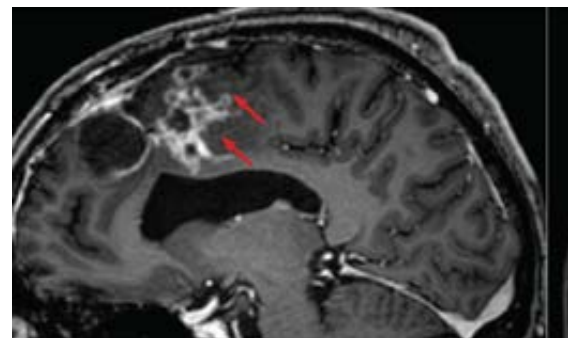
EB1-downregulated GBM



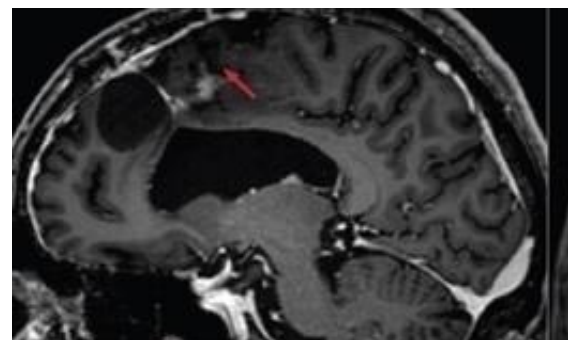
EB1 — A potential response-predictive clinical biomarker for BAL101553

- Strong EB1 staining was observed in a patient with an exceptional response to daily oral BAL101553 in the phase 1 dose-escalation study in recurrent GBM¹
 - Patient ongoing for > 15 months
 - ~70% reduction in GBM tumor size
- Potential utility of EB1 to support a biomarker-driven clinical program is currently being assessed

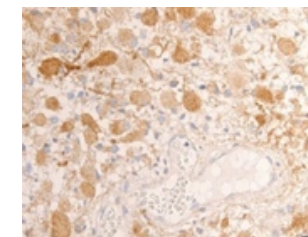
GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



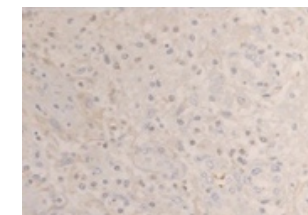
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



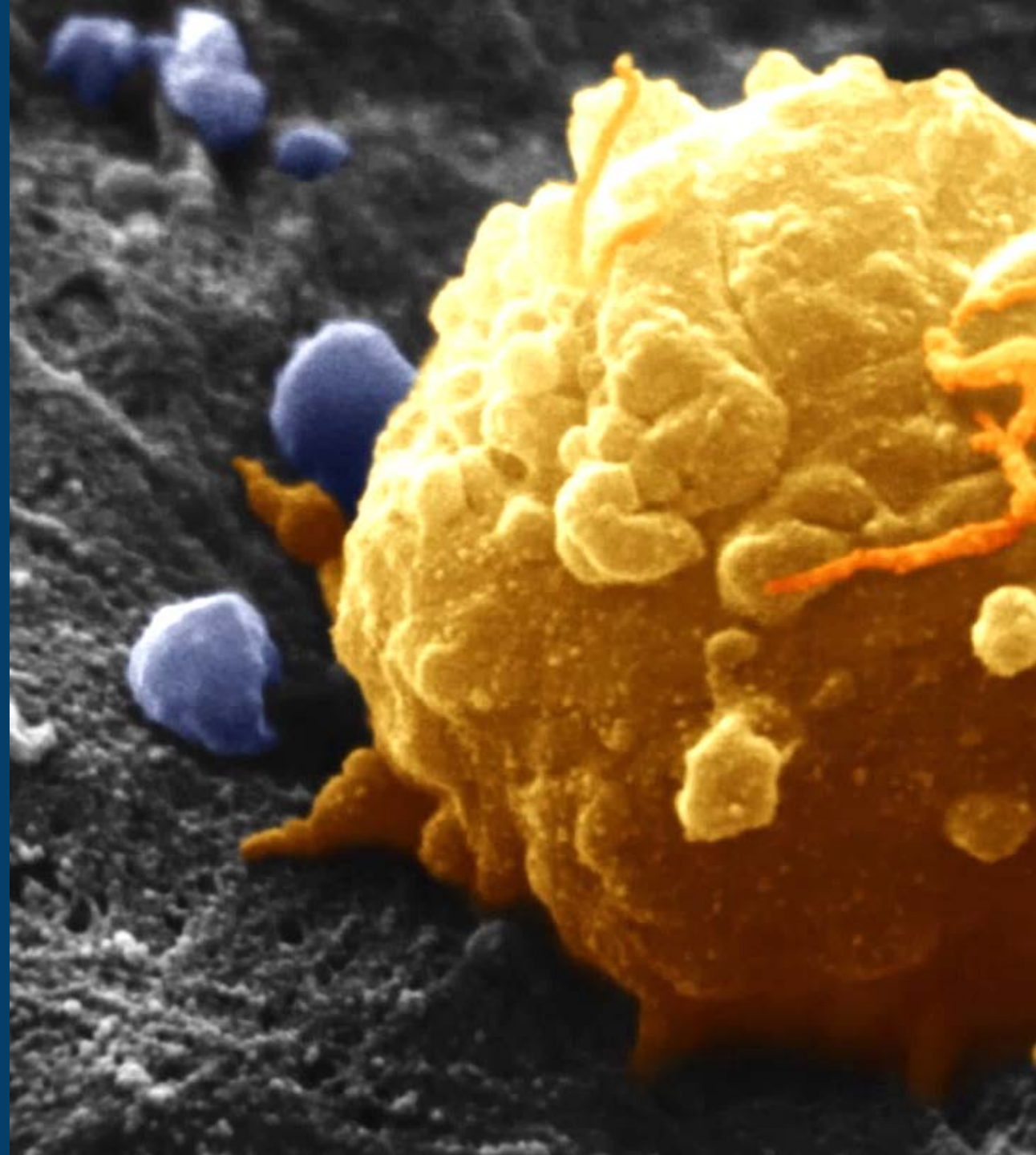
Non-responder

¹ Lopez et al, JCO 2019;37:15 suppl:2025

Oncology

BAL3833

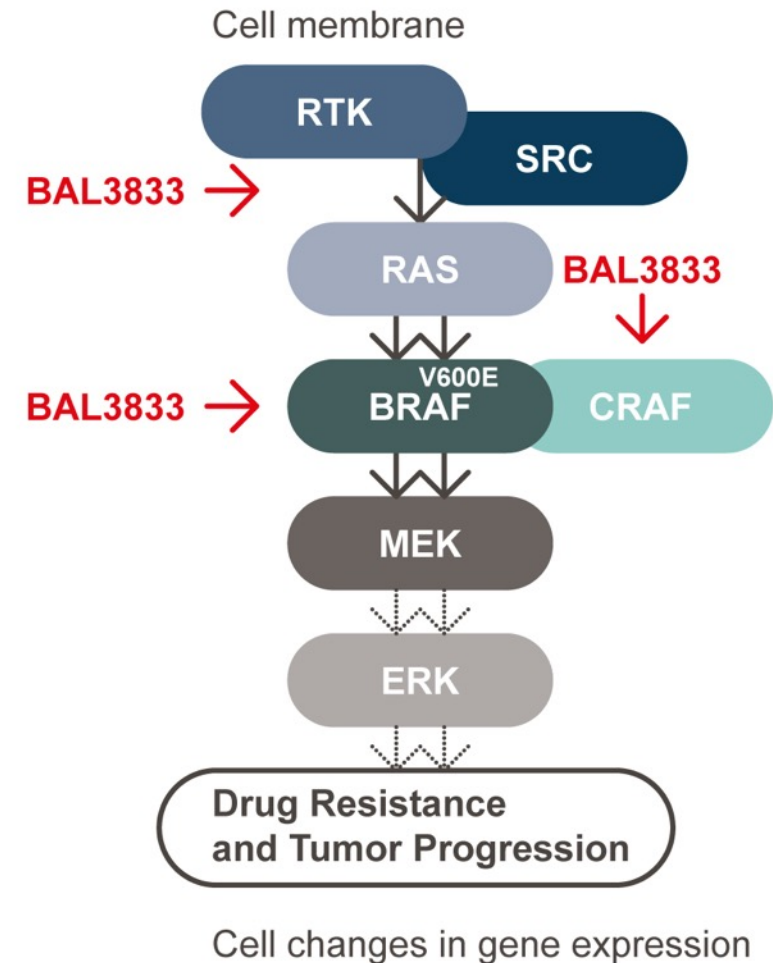
Melanoma and RAS-driven tumors



BAL3833 — panRAF/SRC kinase inhibitor

- In-licensed novel, oral, small molecule drug from consortium including the Wellcome Trust & Institute of Cancer Research (ICR)
- Resistance-reversal activity in BRAF/MEK inhibitor- and immunotherapy-resistant melanoma models
- Potential in diverse non-melanoma tumor types:
 - RAS-driven tumors
 - Expanded biomarker program to aid tumor selection
- Phase 1 dose-escalation study completed¹
 - Broad dose range investigated, maximum tolerated dose (MTD) was not defined
 - Current formulation not continued based on pharmacokinetic profile
 - Conducting pre-clinical activities to explore alternative formulations

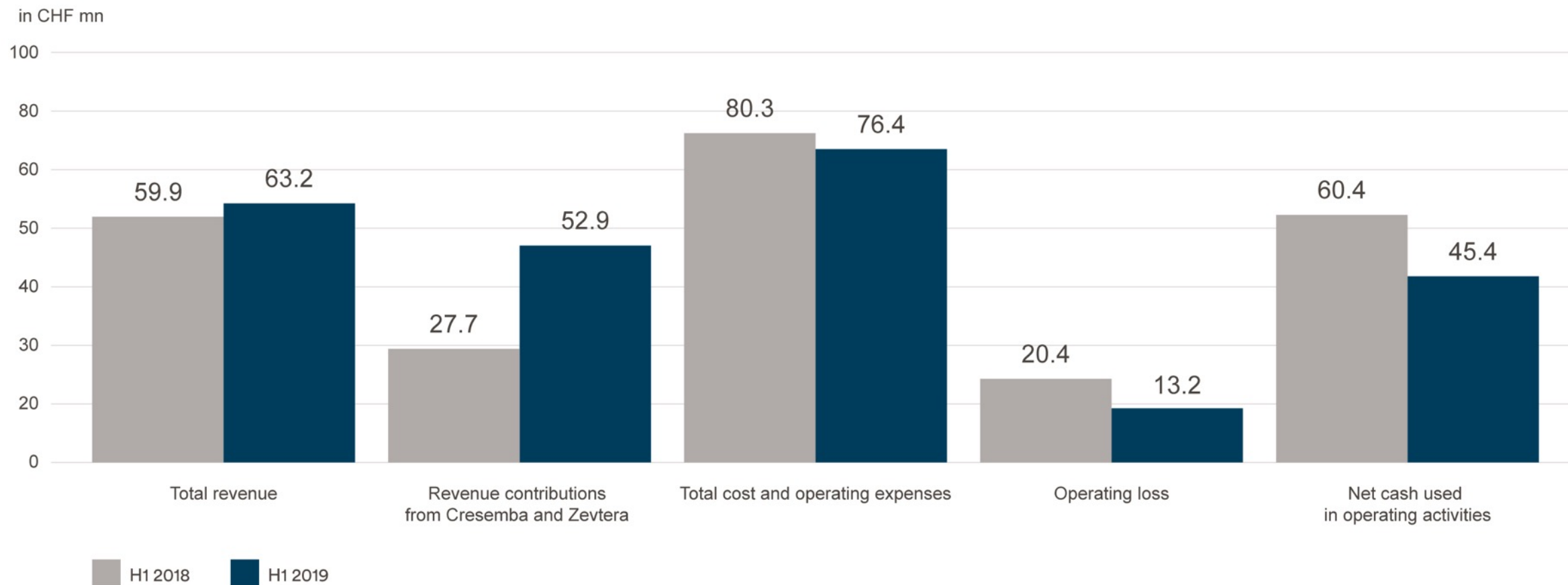
¹ NCT02437227





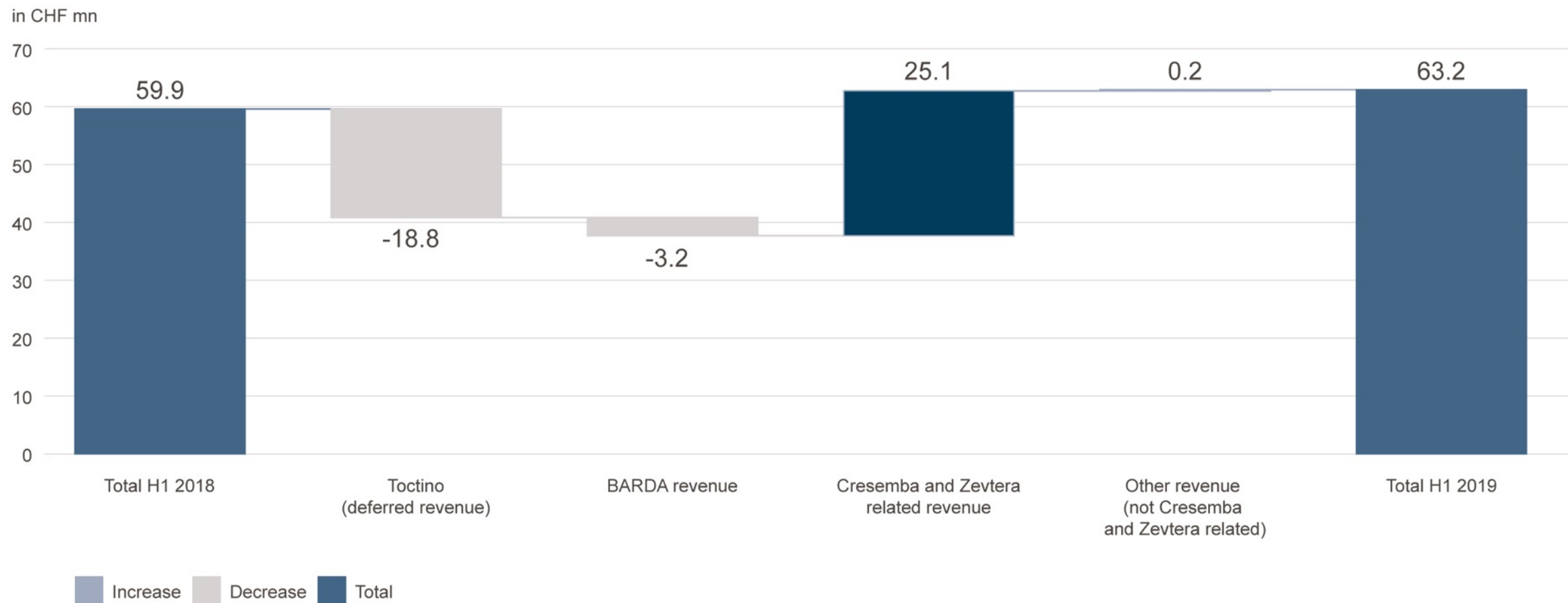
Financials

Financial summary H1 2019 and H1 2018

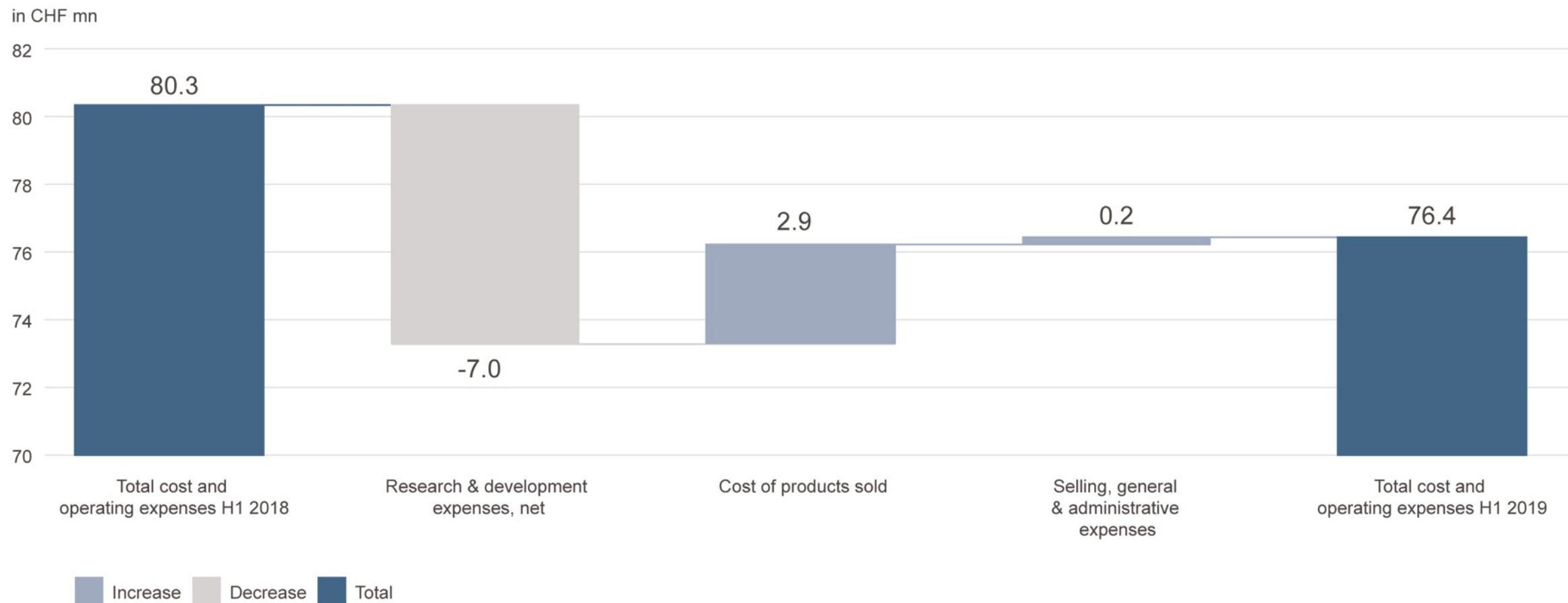


Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

Revenue H1 2019 versus H1 2018



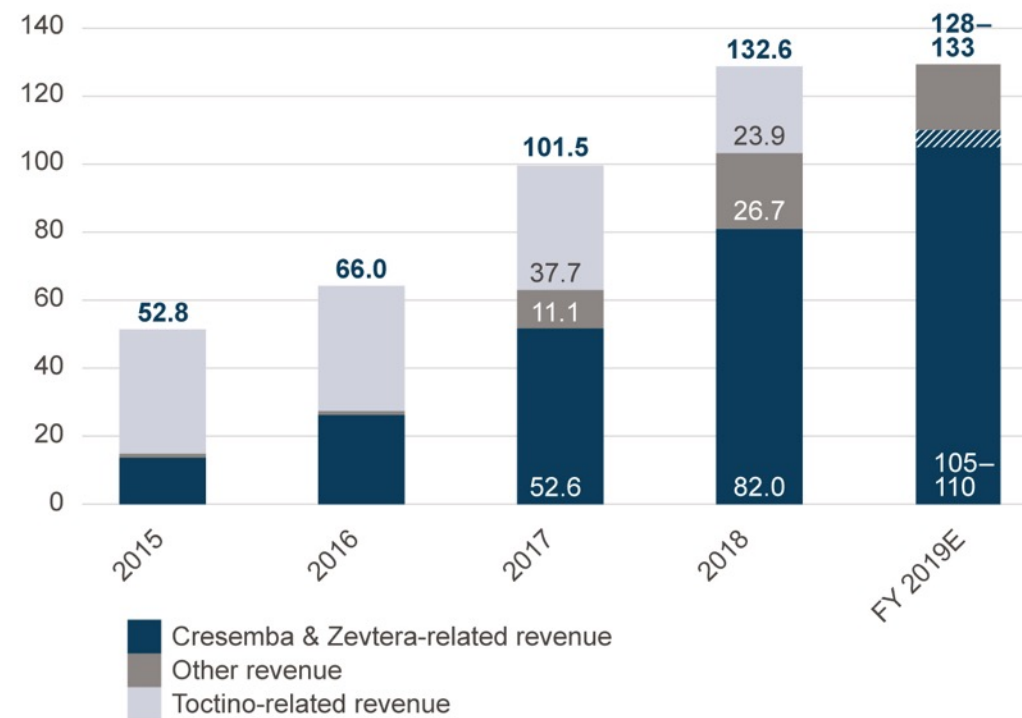
Cost and operating expenses H1 2019 versus H1 2018



Financial guidance 2019

In CHF mn	FY 2019 guidance	FY 2018 actuals	Y-o-Y change
Total revenue	128 – 133	132.6	-3% to +0%
thereof: Contributions Cresemba & Zevtera	105 – 110	82.0	+28% to +34%
Operating loss	22 – 27	24.1	-9% to +12%
Net operating cash consumption	60 – 65	79.2	-24% to -18%

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	✓ Positive topline results from phase 3 ABSSSI study			
Derazantinib	✓ Positive interim results of phase 2 registrational study in iCCA FGFR2 fusions		Complete patient enrolment in phase 2 registrational study in iCCA FGFR2 fusions	Topline results from phase 2 registrational study in iCCA FGFR2 fusions
	✓ Extend ongoing phase 2 iCCA study in other FGFR gene aberrations			Interim data from iCCA in other FGFR gene aberrations
	✓ Clinical supply agreement with Roche in urothelial cancer	✓ Start phase 1/2 study in urothelial cancer		Interim data from first cohort(s) in urothelial cancer
BAL101553		✓ Complete patient enrolment in phase 1 study arm for recurrent glioblastoma (oral)	Full results from phase 1 study arm for recurrent glioblastoma (oral)	
		Complete phase 2a study in ovarian cancer and glioblastoma (48-hr. i.v.)		
				Complete patient enrolment in phase 1 study in newly diagnosed glioblastoma (oral)

Appendix

Basilea leadership — Management Committee



**David
Veitch** CEO

2014



**Adesh
Kaul** CFO

2009



**Marc
Engelhardt**
MD, Ph.D. CMO

2010



**Gerrit
Hauck**
Ph.D. CTO

2018



**Laurenz
Kellenberger**
Ph.D. CSO

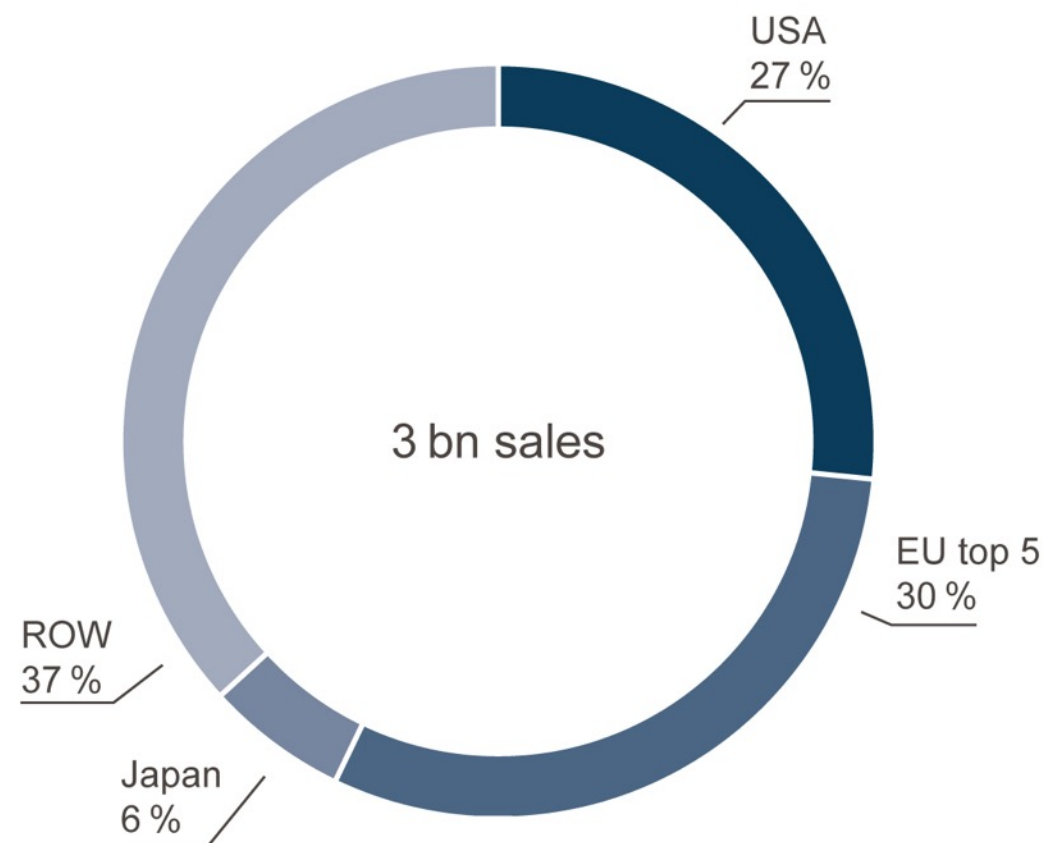
2000



Cresemba®
(isavuconazole)

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

USD 3bn sales of best-in-class antifungals*
(MAT Q1 2019)

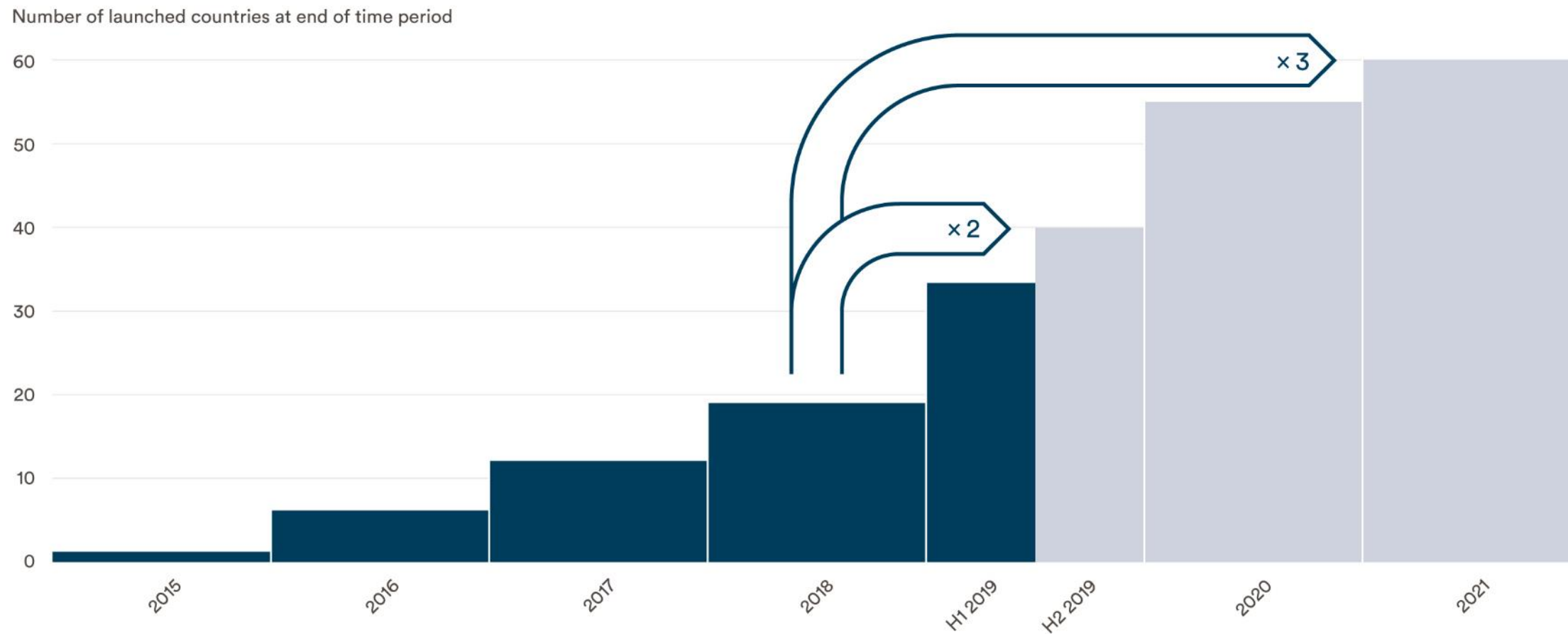


* Best-in-class antifungals: isavuconazole, posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin

MAT: Moving annual total; Sales figures in USD, corrected for currency fluctuations;
Source: IQVIA, March 2019

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Cresemba — strong global roll out

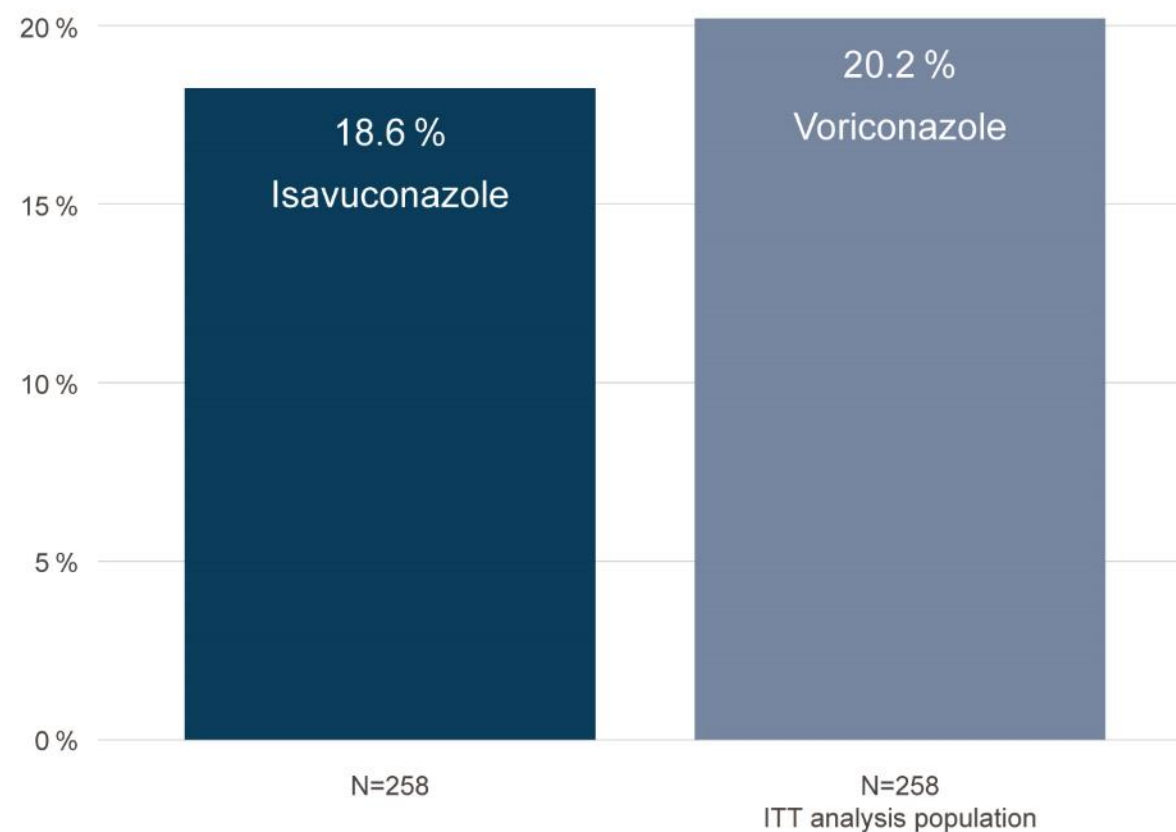


Isavuconazole — SECURE phase 3 data (efficacy)

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

- Met primary objective of non-inferiority vs. voriconazole

All-cause mortality through day 42



Source: Maertens et al, Lancet 2016;387:760-769
NCT00412893

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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=146)

- 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)**

* Perfect et al, Mycoses 2018;61:420-429; ** Marty et al, Lancet Infect Dis 2016;16:828-837
NCT00634049

Zevtera[®]/Mabelio[®] **(ceftobiprole)**

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** (primary for FDA): 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

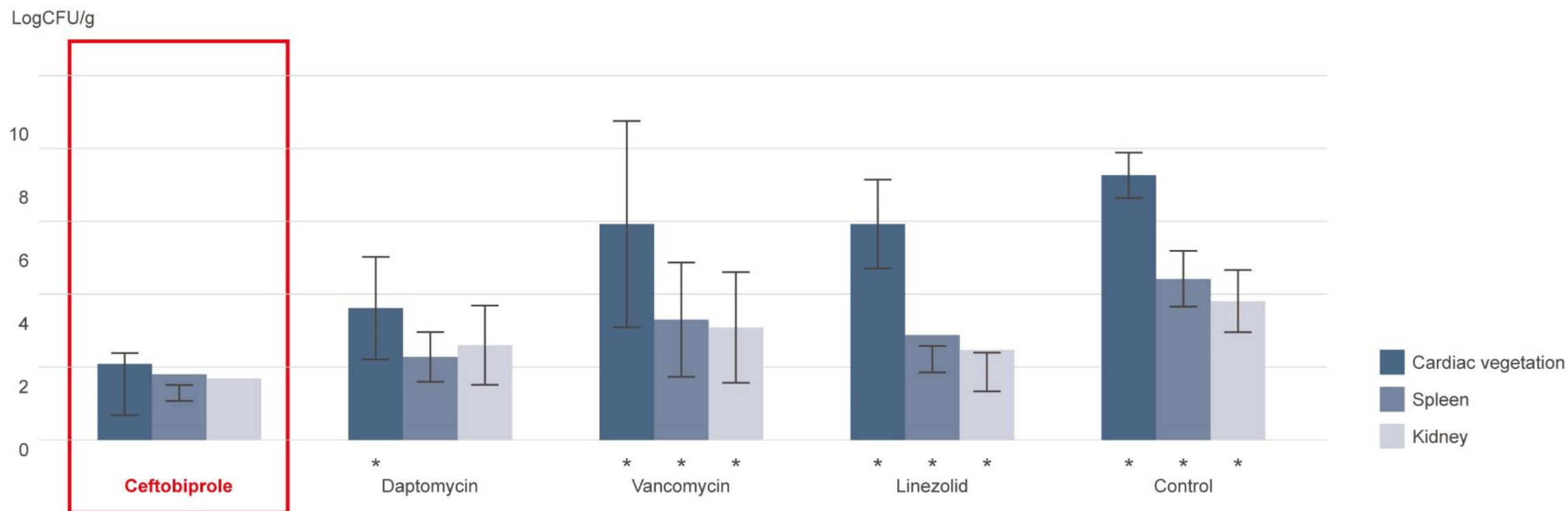
Phase 3 study with ceftobiprole in the treatment of patients with SAB



- **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:** 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:** includes 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 — 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)



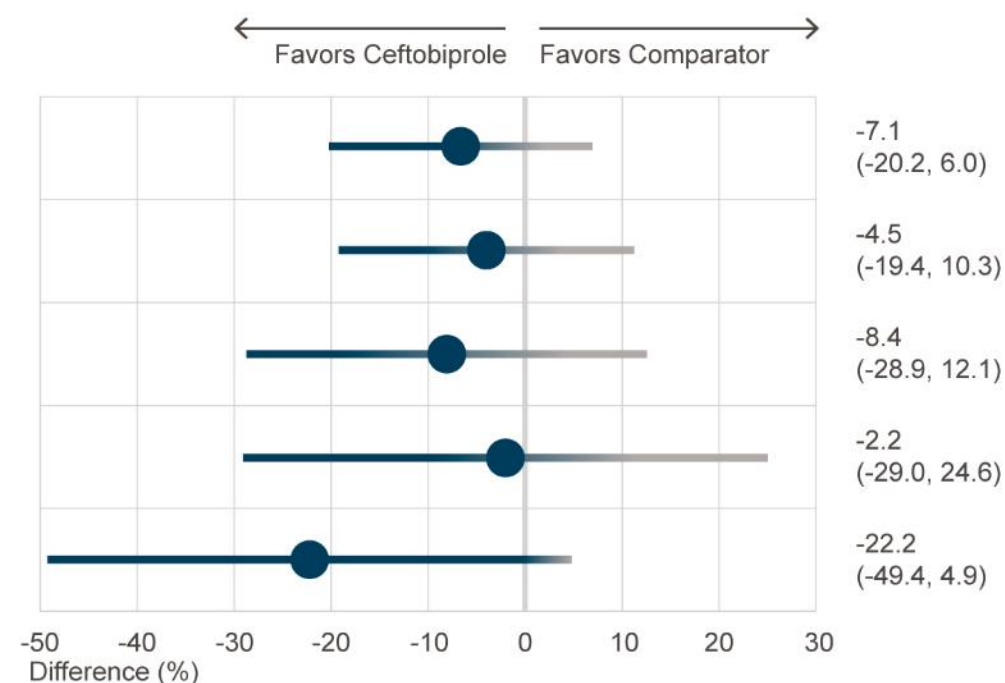
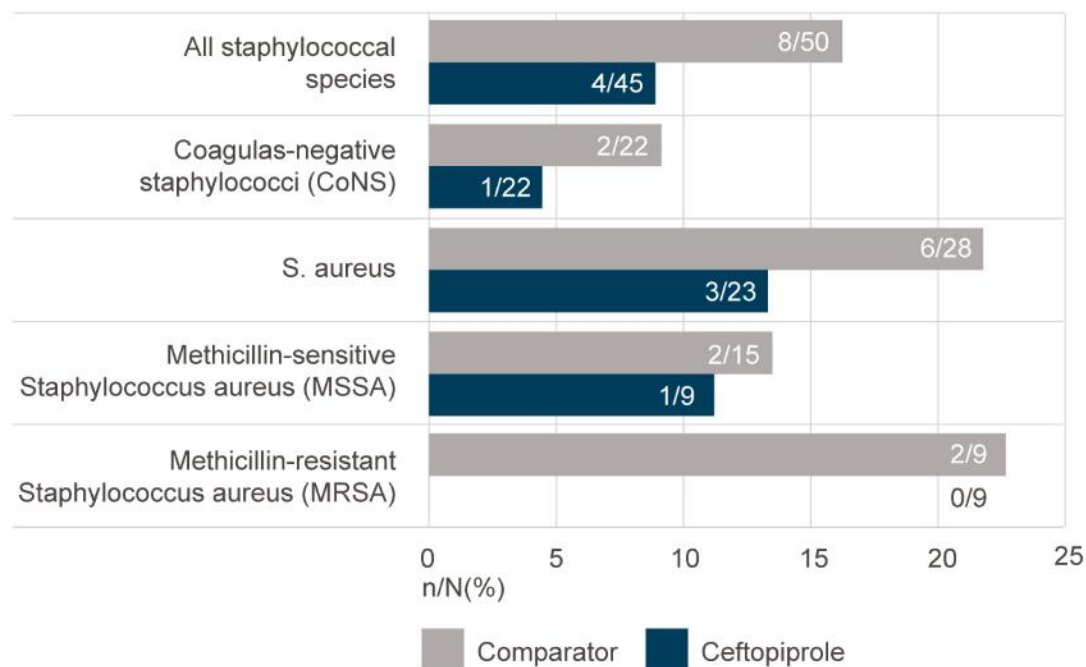
* Differences in favor of ceftobiprole statistically significant
Source: Tattevin et al, Antimicrob Agents Chemother 2010;54:610-613

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)

30-day all-cause mortality

Bacteremia due to:



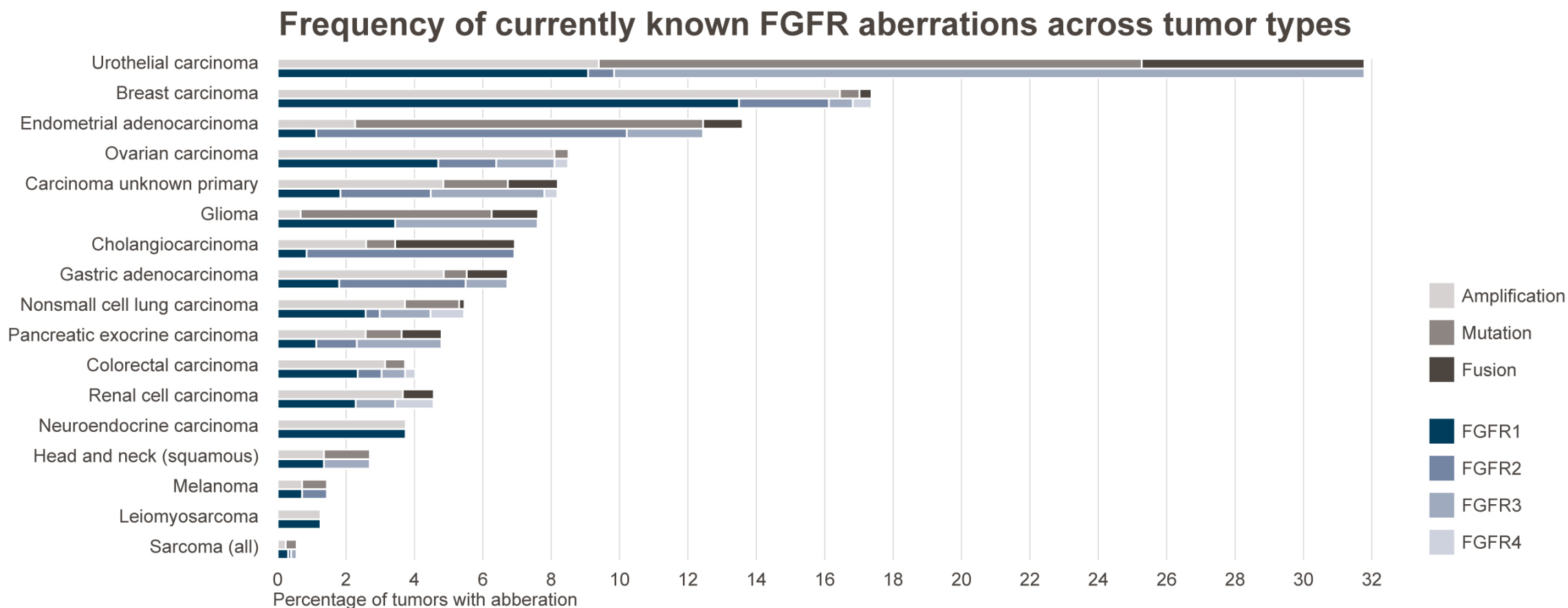
Comparators: ABSSSI: vancomycin, vancomycin + ceftazidime / CABP: ceftriaxone ± linezolid / HABP: linezolid + ceftazidime

* *Staphylococcus aureus* bacteremia

Chart based on: Rello et al. ECCMID 2016

Derazantinib

Derazantinib — Significant potential beyond iCCA



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

Disclaimer and forward-looking statements

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