



basilea

David Veitch, CEO

Disclaimer and forward-looking statements

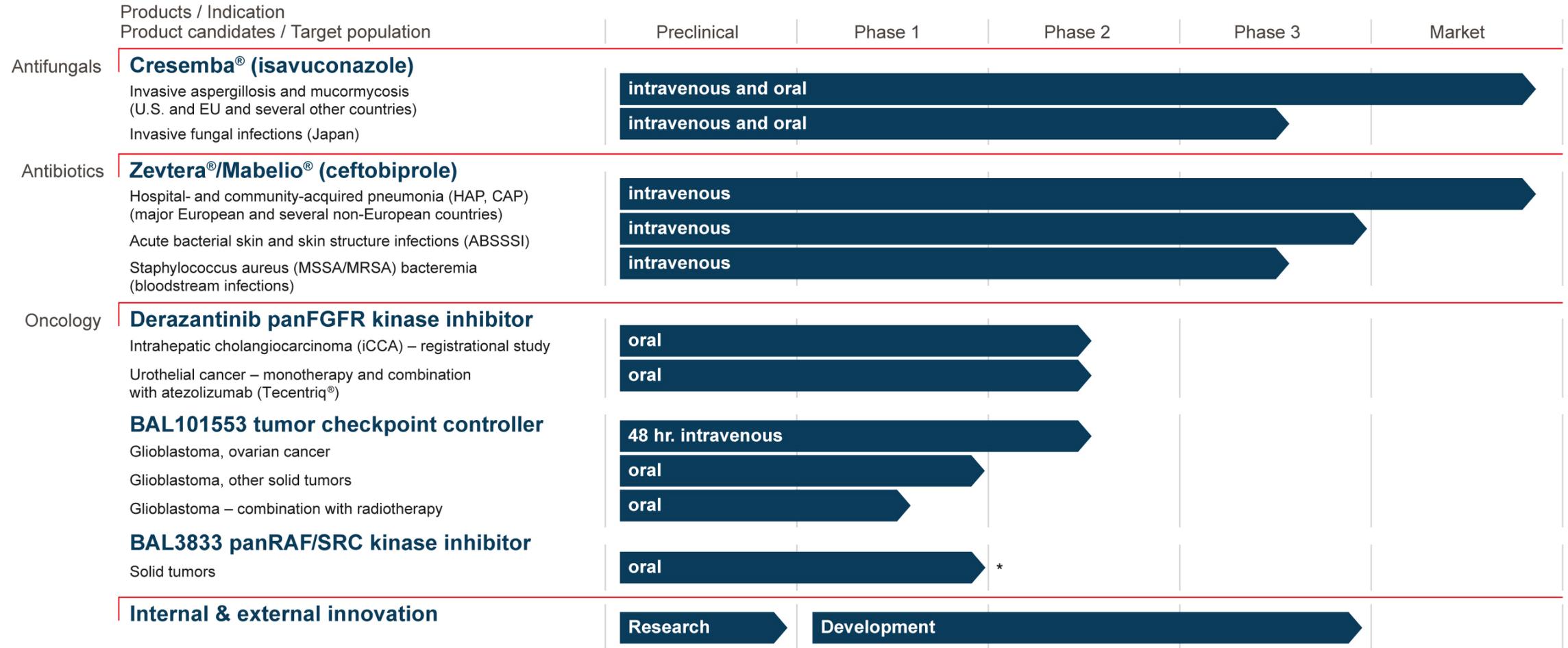
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Basilea at a glance

- 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

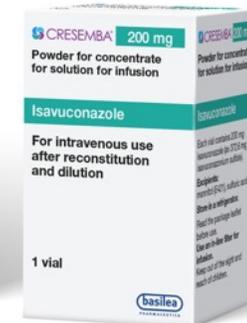
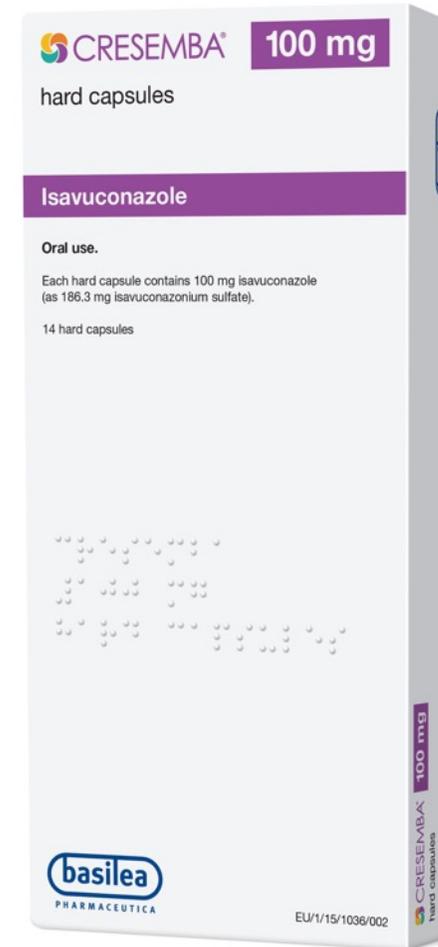
- Double-digit royalties on sales by license partners
- Participation in sales of distribution partners through transfer price
- ~USD 245mn upfront and milestone payments received
- USD 1.1bn in potential milestones remaining



Antifungal

Cresemba® (isavuconazole)

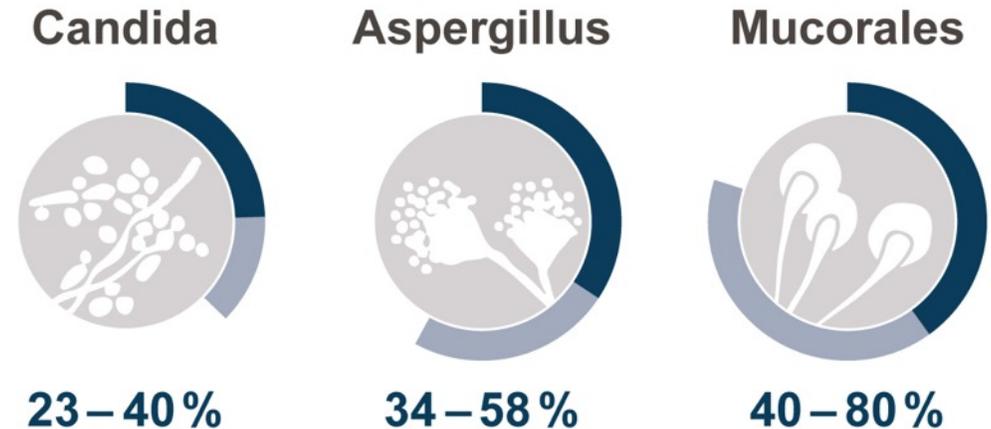
Invasive mold infections



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

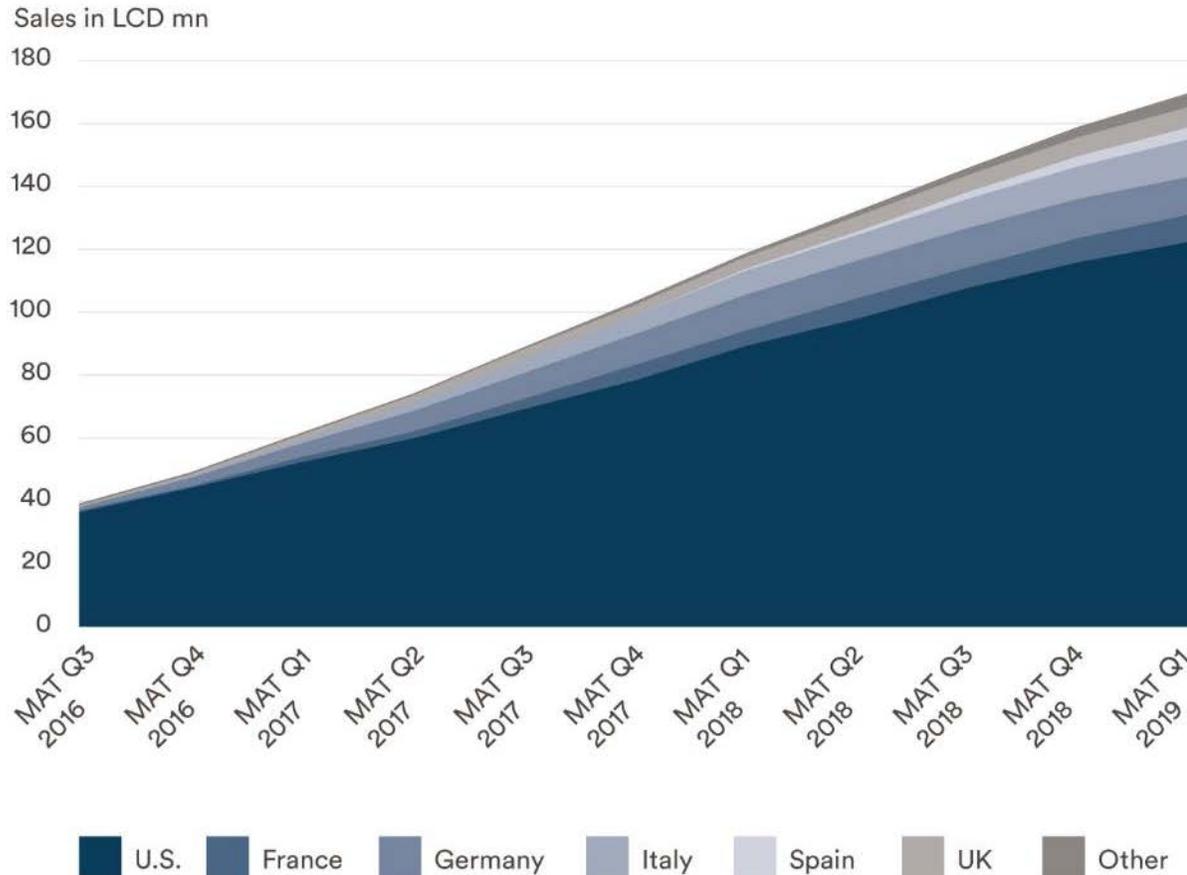
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand
- 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

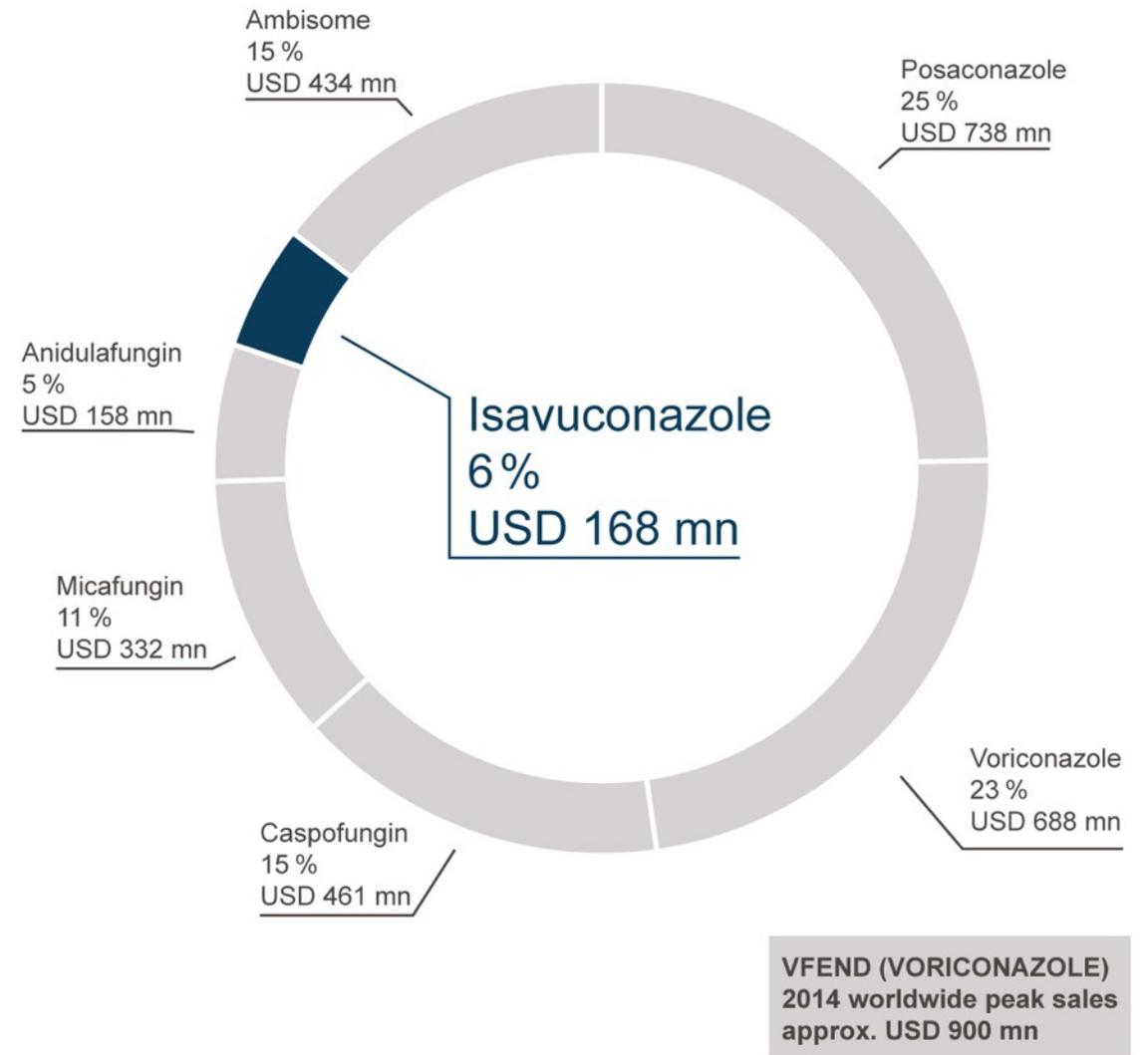


Approx. USD 170mn in-market sales in MAT Q1 2019

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.

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

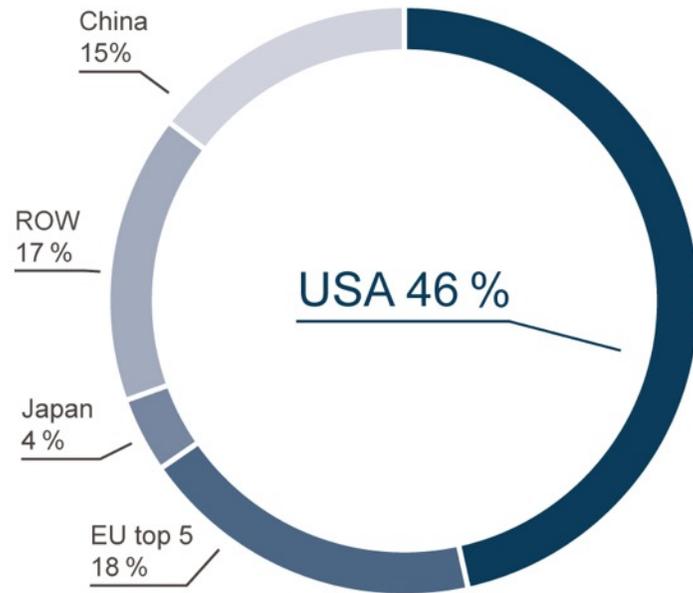
- Broad-spectrum anti-MRSA cephalosporin (including Gram-negative bacteria)
- Rapid bactericidal activity
- Potential to replace antibiotic combinations
- Early improvement in HAP, particularly in patients with MRSA, and CAP, including high-risk patients
- Cephalosporin class safety profile
- Marketed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

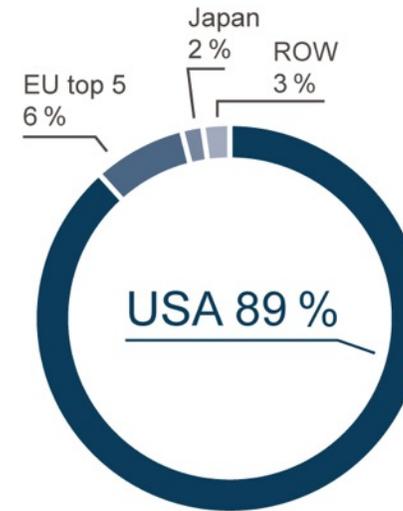


Anti-MRSA hospital antibiotics market — A USD 3.1bn market with the U.S. being the most important region

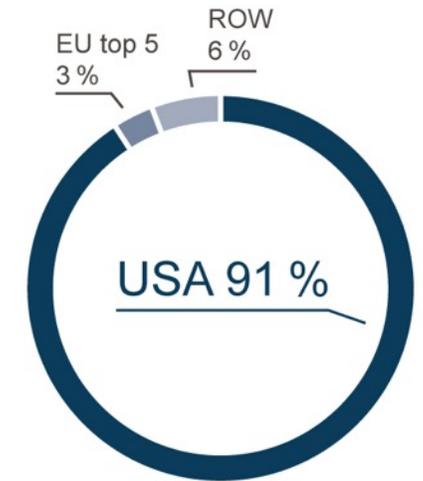
Global anti-MRSA hospital antibiotics sales*
USD 3.1bn (MAT Q1 2019)



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



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



- Partial funding of phase 3 program by BARDA (up to USD 128mn, ~70% of total program costs)



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

¹ NCT03137173

² NCT03138733

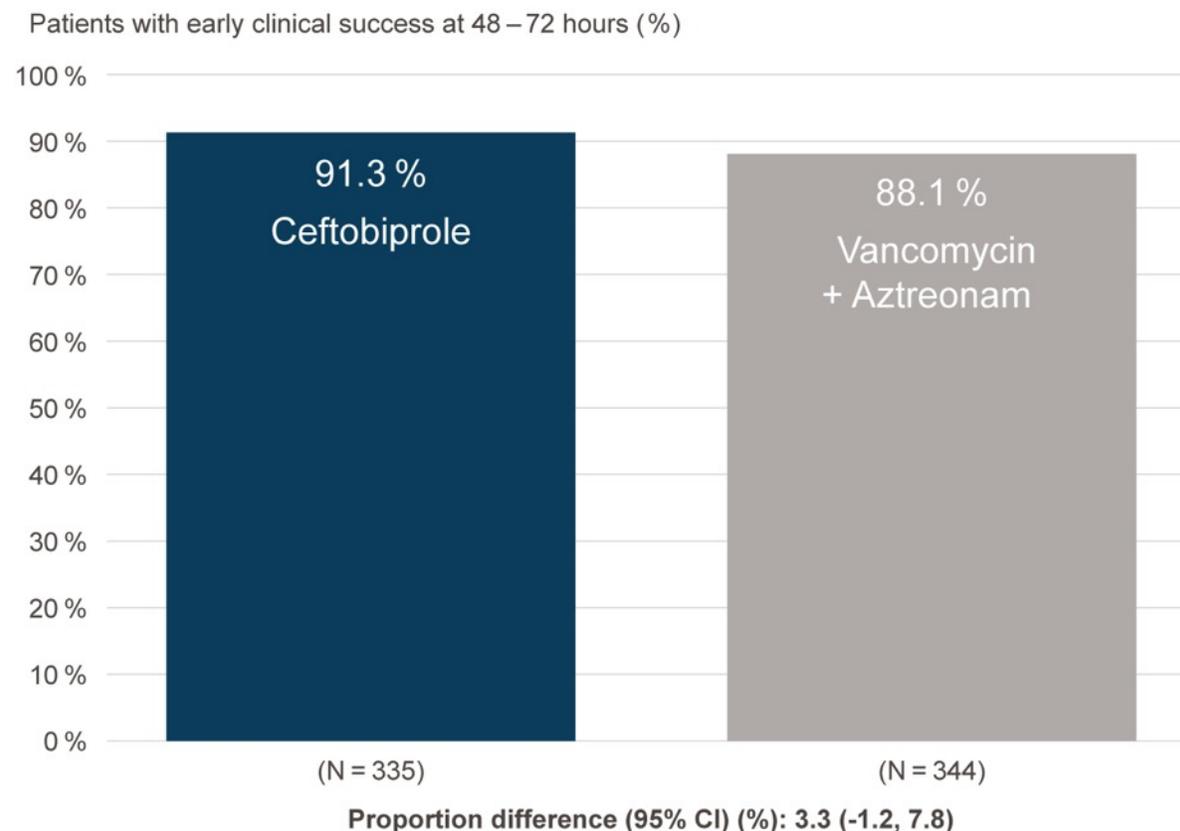
Ceftobiprole — positive topline phase 3 results reported in ABSSSI

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

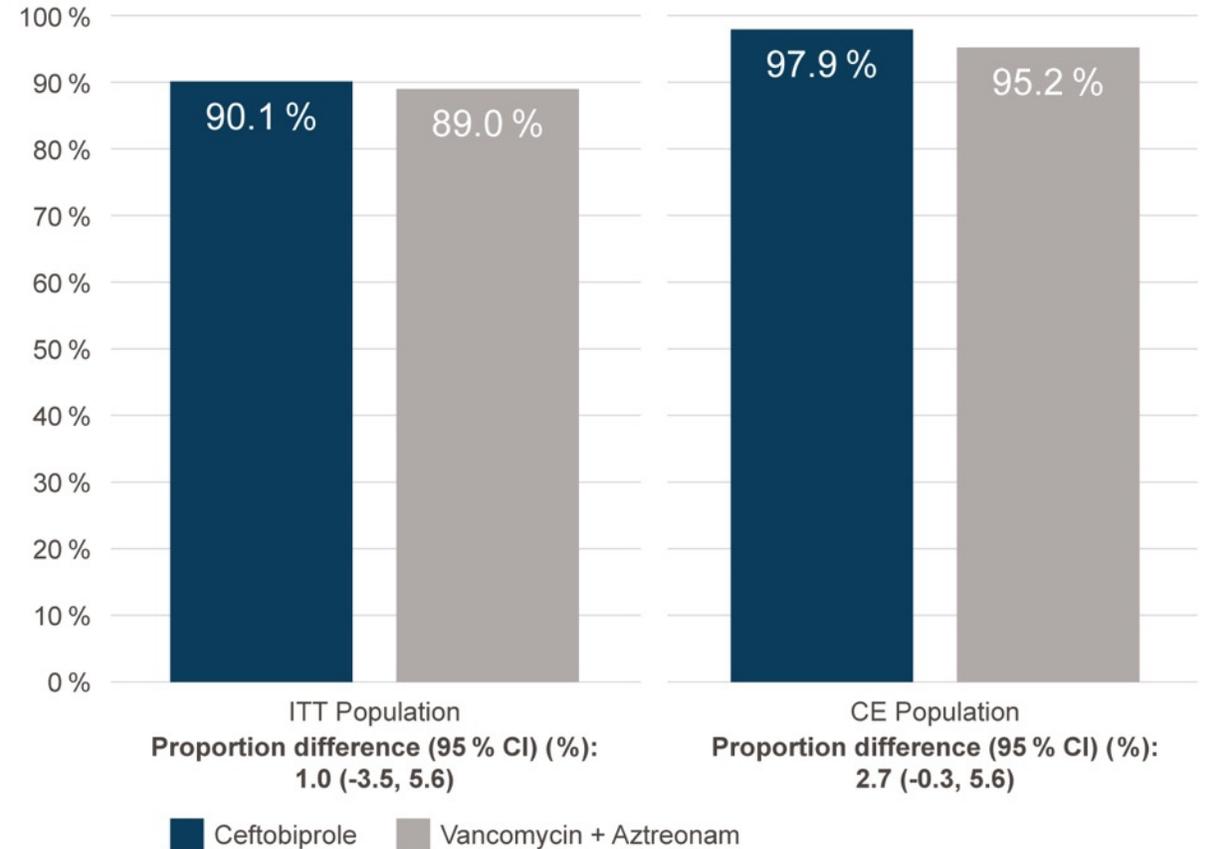


¹ NCT03137173

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

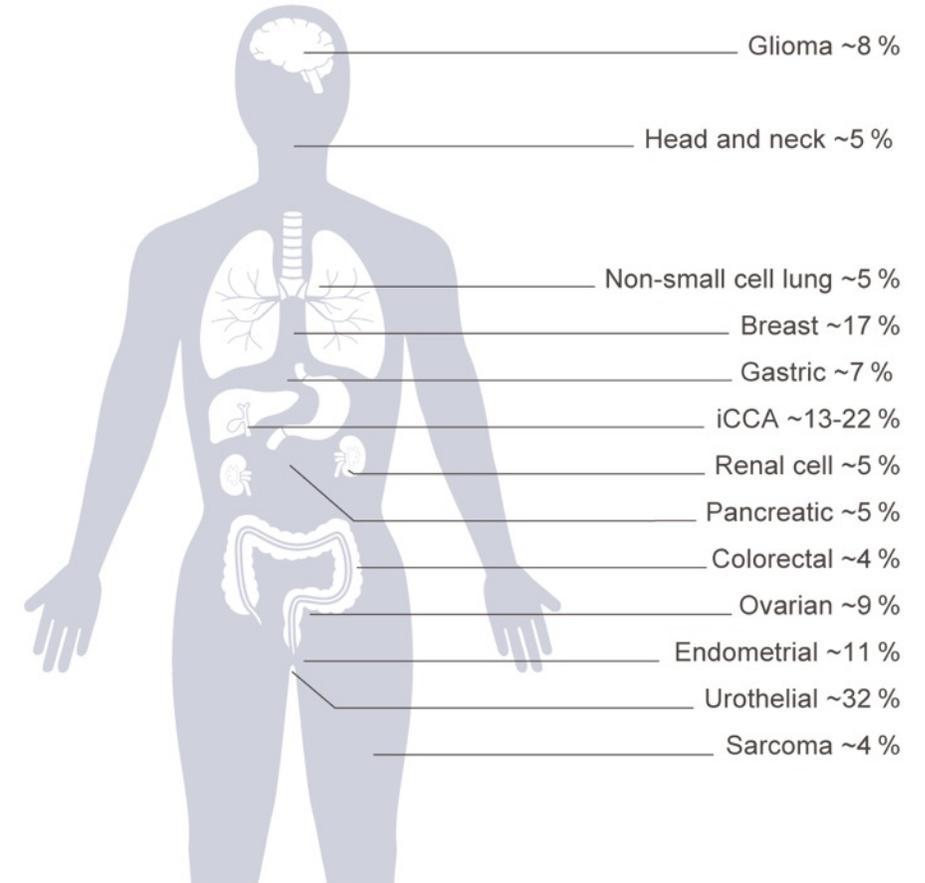
Oncology

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

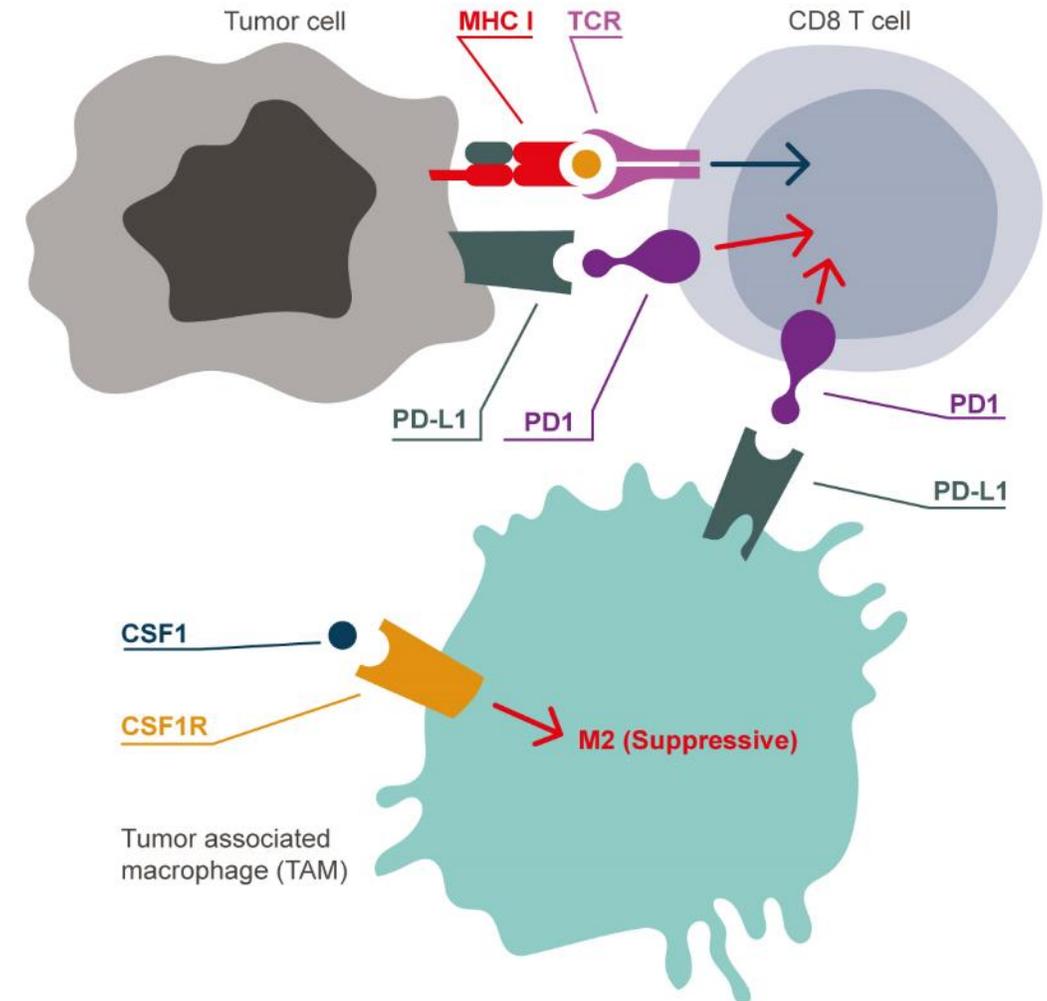
Potential therapeutic relevance of CSF1R-inhibition

- 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

Tumor microenvironment



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

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	41% ⁷
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; ⁷ Balversa™ U.S. prescribing information (April 2019) based on reported laboratory abnormalities N=86 patients, regardless of causality.

Abbreviations: DZB: derazantinib, INF: infigratinib (BJG398), FUT: futibatinib (TAS-120), PEM: pemigatinib (INCB54828), ERD: erdafitinib;

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

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

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

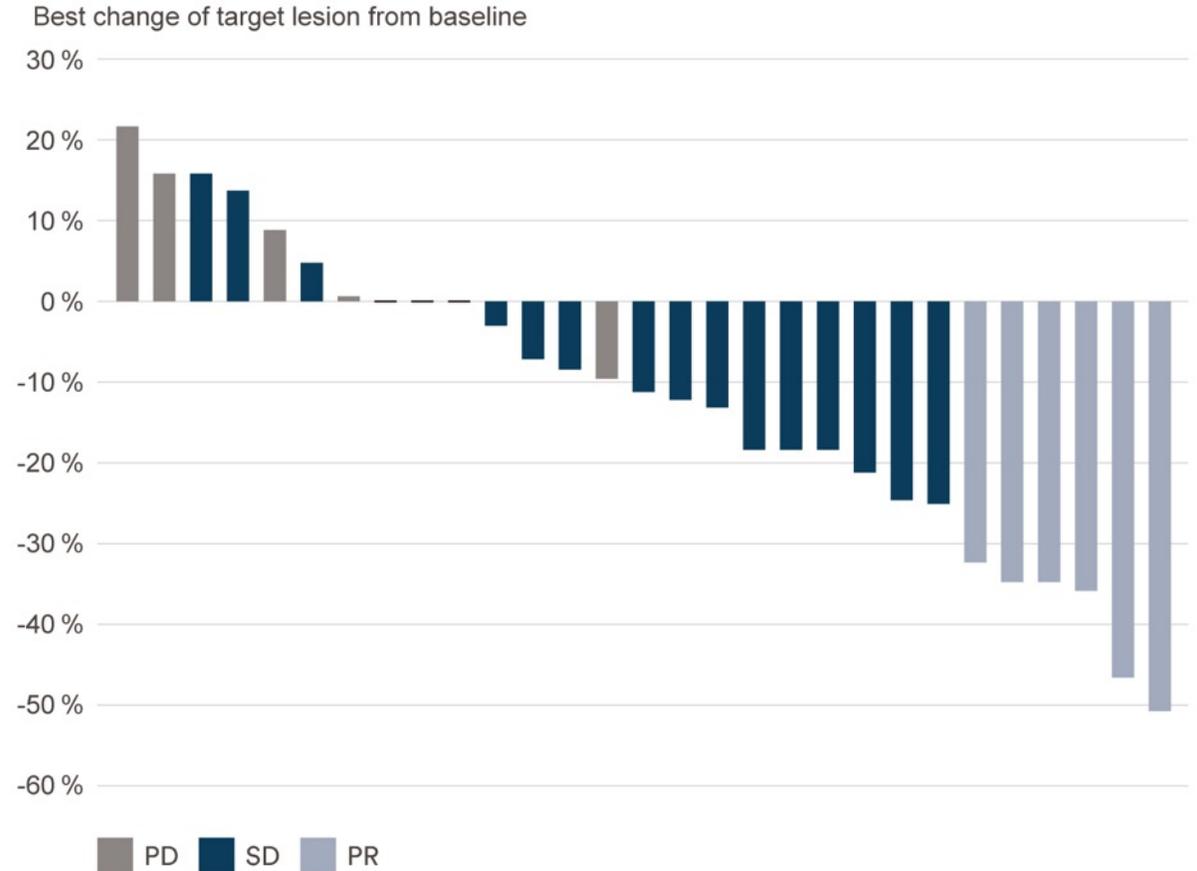
- Derazantinib as single agent and in combination with atezolizumab (Tecentriq[®]) in patients with advanced urothelial cancer testing positive for mutations or fusions of FGFR1, FGFR2 or FGFR3 genes
- The subgroup of patients with low PD-L1 expression have limited clinical benefit from the treatment 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

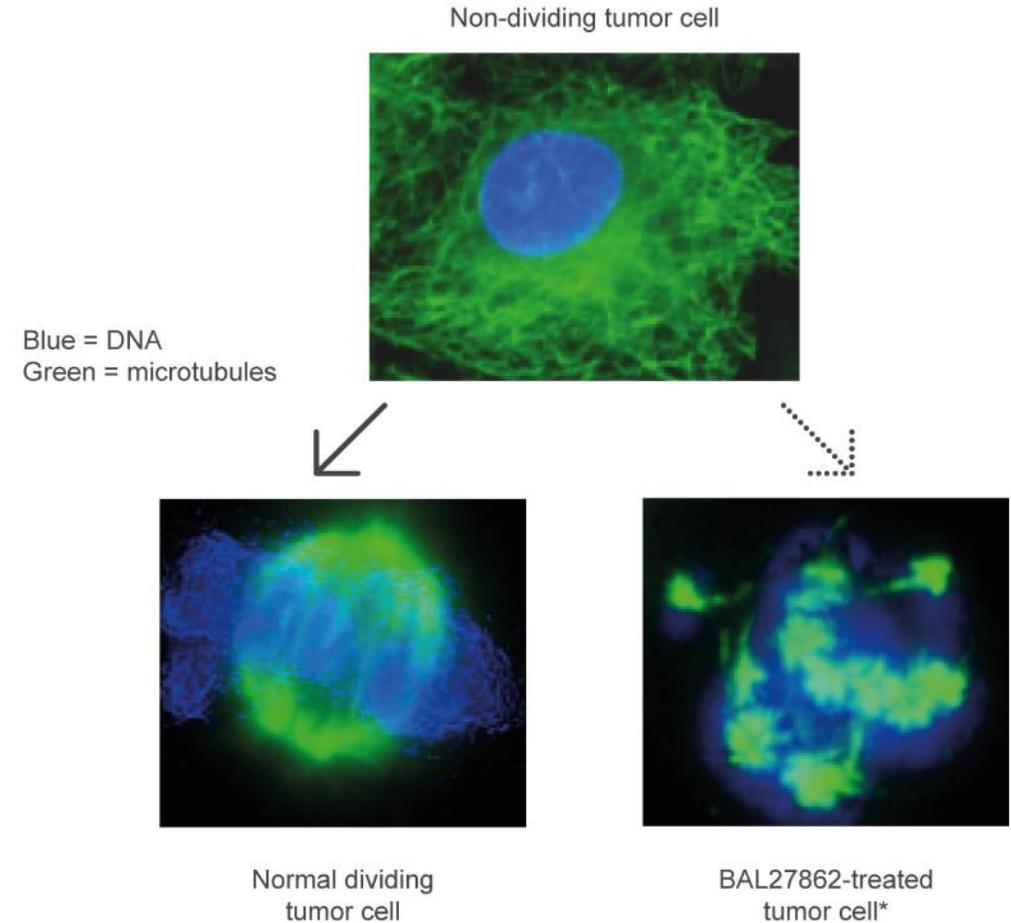
BAL101553

Glioblastoma and ovarian cancer



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

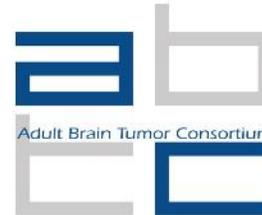
- Novel compound inducing tumor cell death through spindle assembly checkpoint activation
- Destabilizing the microtubule scaffold through a novel target-binding site
- Targeting diverse tumor types resistant to standard therapeutic approaches
- Crosses the blood-brain barrier with potent activity in 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

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)

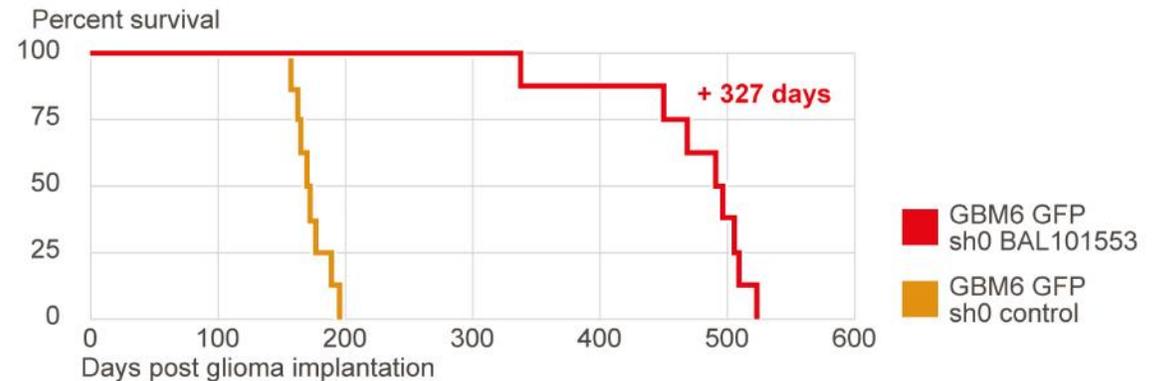
EB1 — A potential response-predictive clinical biomarker for BAL101553

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

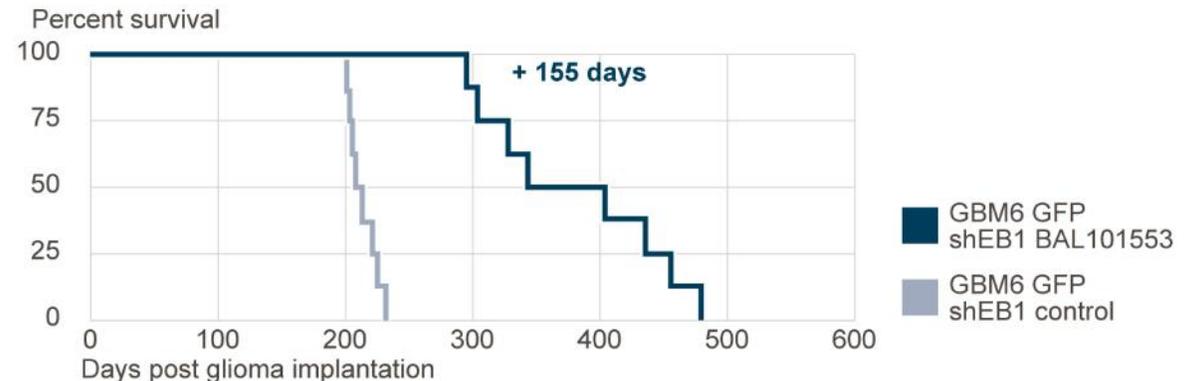
¹ Berges et al. Eur. J. Cancer 2018, 103,E61-62

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

EB1-expressing GBM



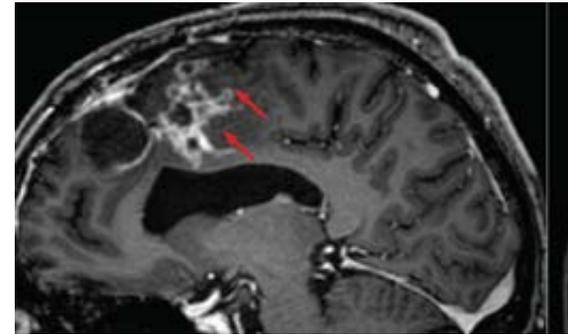
EB1-downregulated GBM



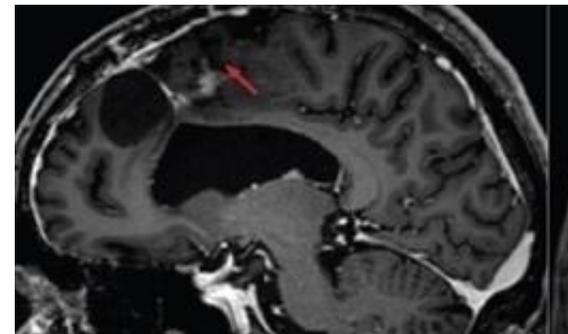
EB1 — A potential response-predictive clinical biomarker for BAL101553

- 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

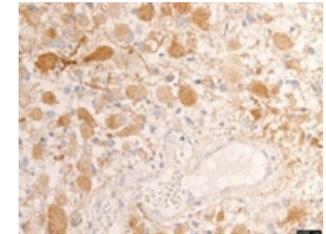
GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



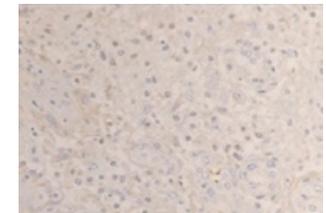
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



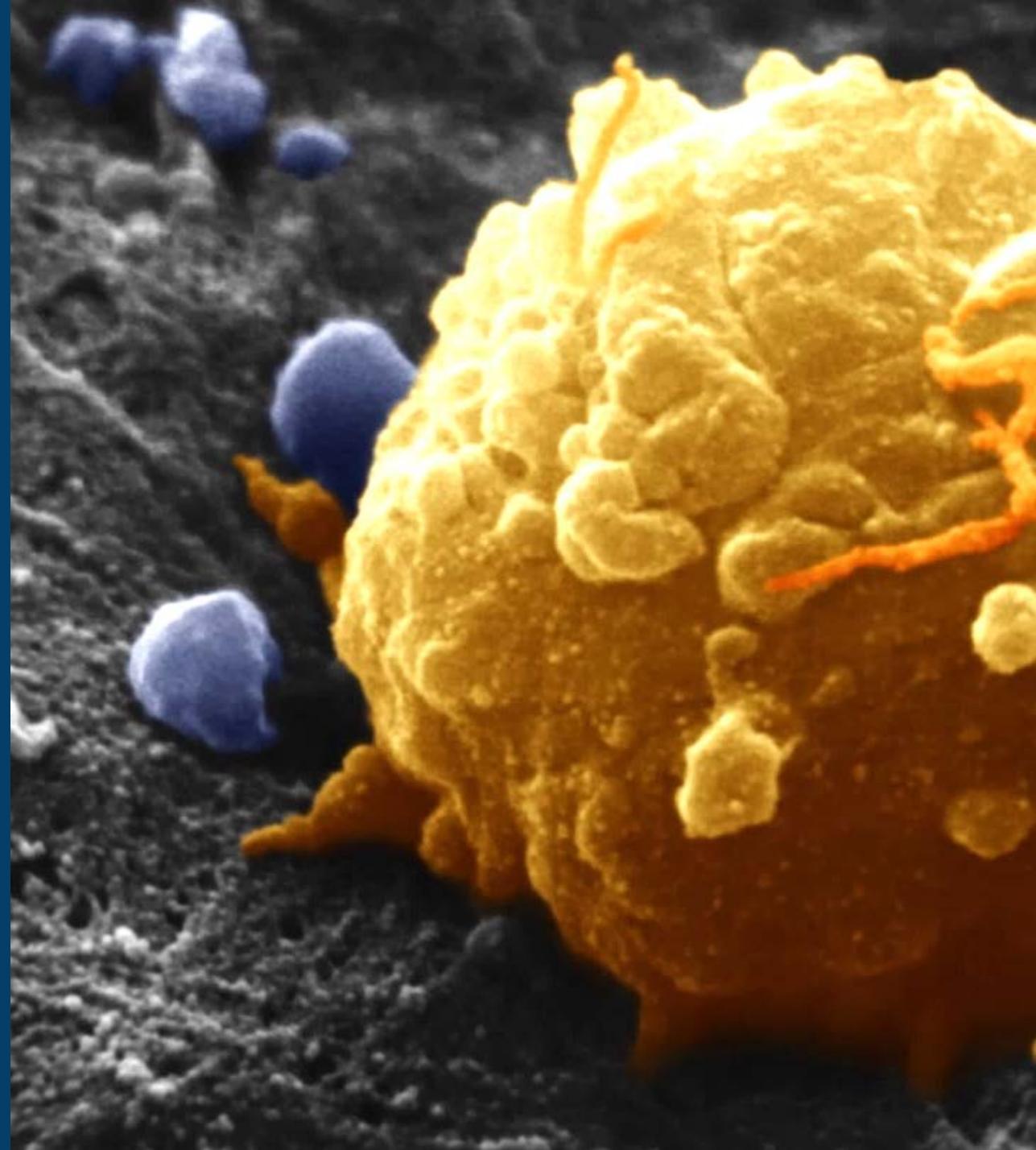
Non-responder

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

Oncology

BAL3833

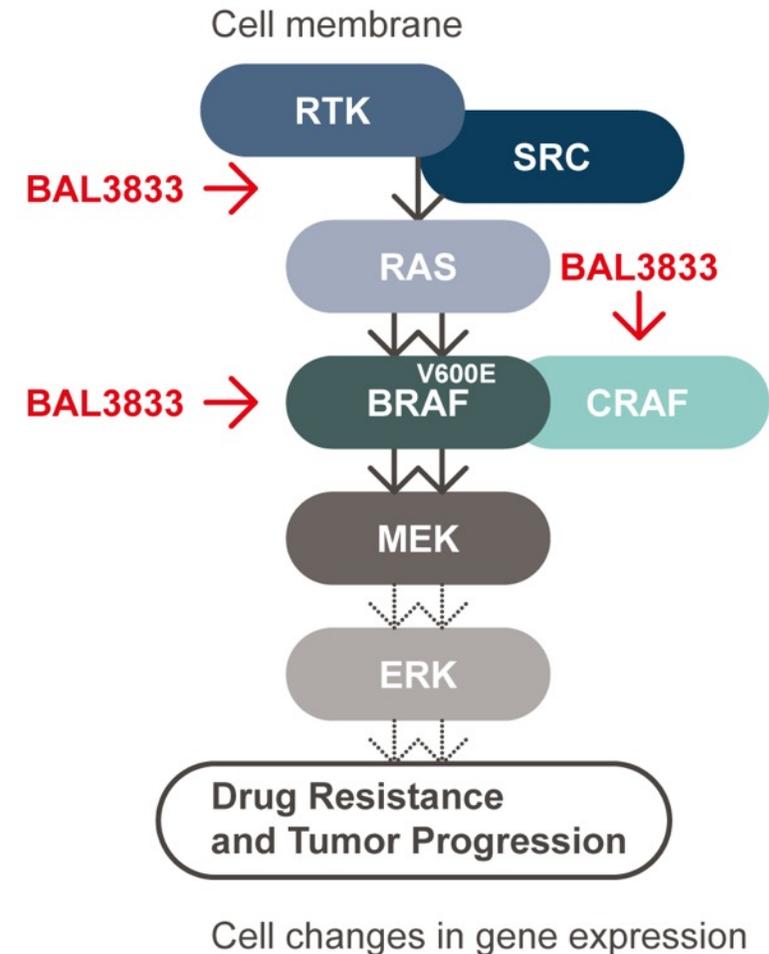
Melanoma and RAS-driven tumors



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

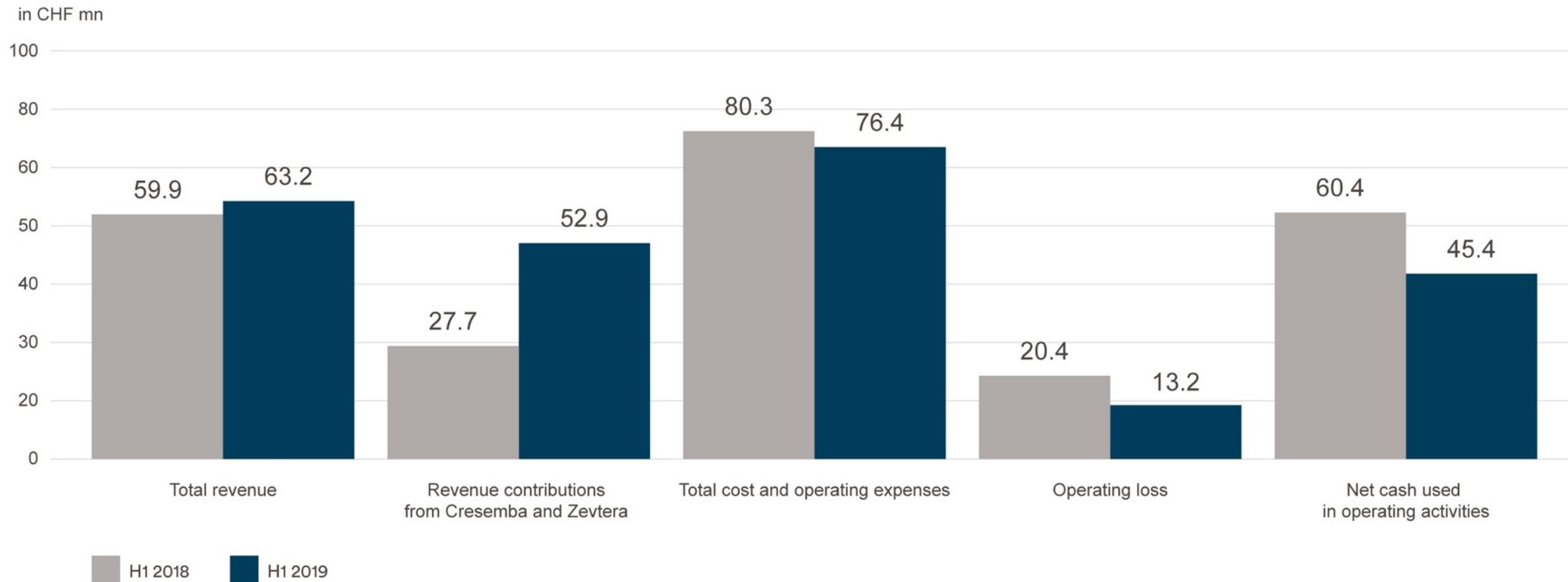
¹ NCT02437227





Financials

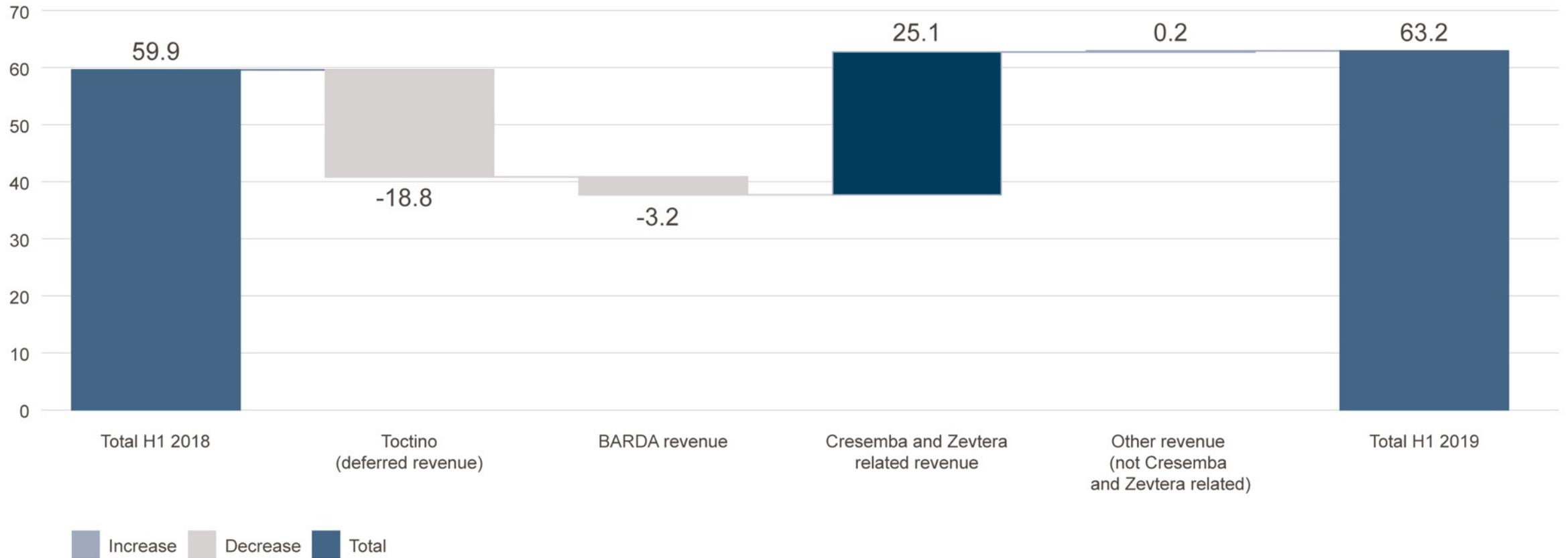
Financial summary H1 2019 and H1 2018



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

Revenue H1 2019 versus H1 2018

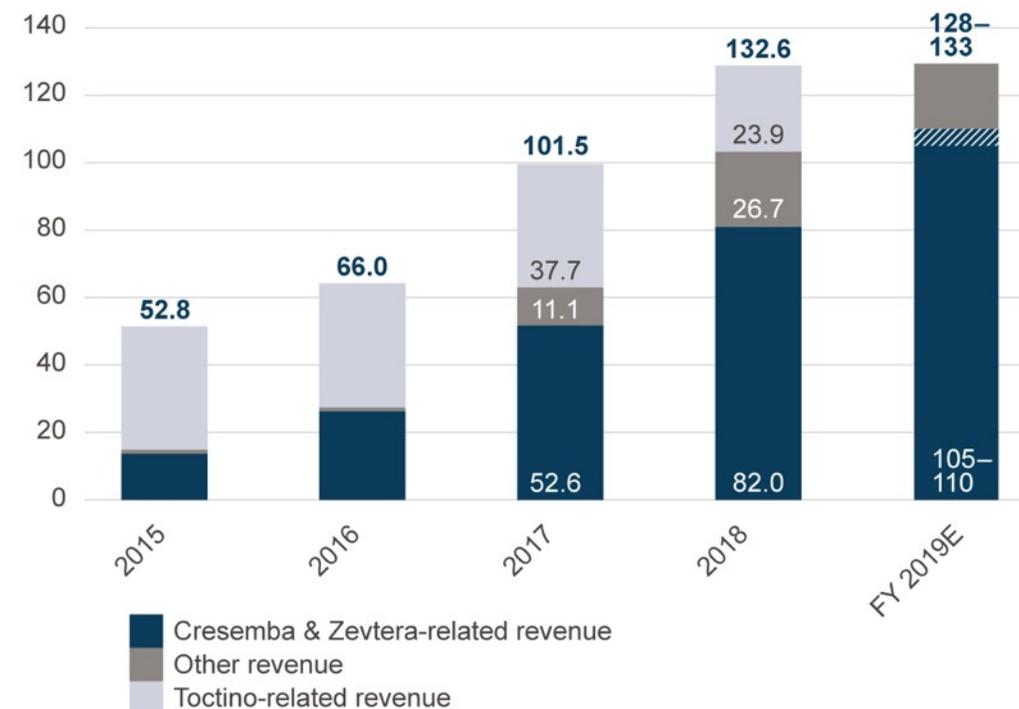
in CHF mn



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)



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