



Focused on Growth and Innovation

Investor presentation
January 2020



Disclaimer and 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.

Experienced leadership team



David Veitch CEO

Joined 2014

Previous roles:



Adesh Kaul CFO

2009



Marc Engelhardt MD, Ph.D. CMO

2010



Gerrit Hauck Ph.D. CTO

2018



Laurenz Kellenberger Ph.D. CSO

2000

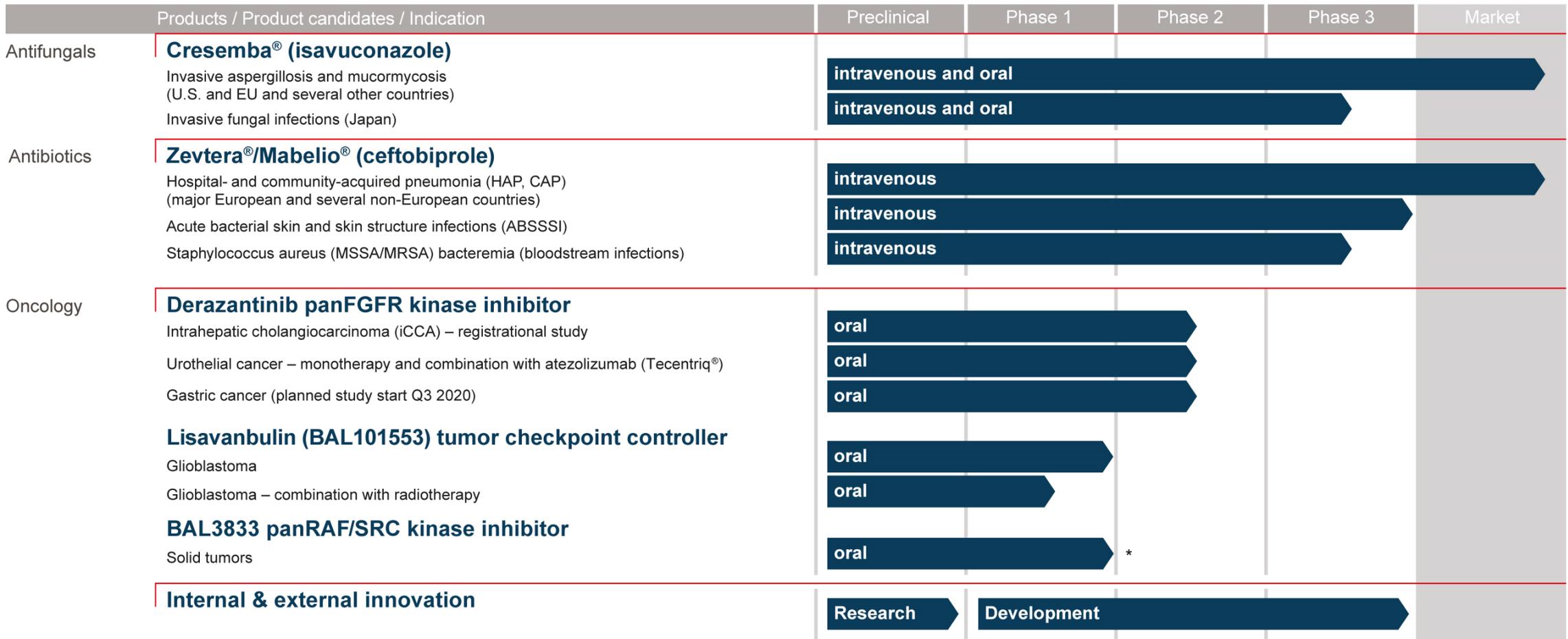


At a glance

- Well funded, commercial-stage biotech company with significantly growing cash revenues
- Focused in the areas of oncology, hospital antifungals and hospital antibiotics
- Potential for sustainable growth and value creation based on commercialized brands and an innovative pipeline
- Experienced people with the proven expertise to take compounds from research to market
- Two revenue generating hospital anti-infective brands, Cresemba® and Zevtera® and three oncology drug candidates in development
- Recognized ability to establish and manage partnerships in both the development and commercial phase, providing access to international markets
- Listed on SIX Swiss Stock Exchange, SIX: BSLN
- Based in life sciences hub, Basel, Switzerland



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



* pre-clinical reformulation activities ongoing.

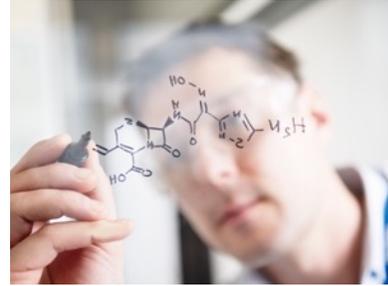
Tecentriq® is a registered trademark of Hoffmann-La Roche Ltd.

Our strategy



Foster

Foster an agile organisation based on a dynamic and open culture



Focus

Focus on continuously increasing revenues from our two commercial-stage hospital anti-infective brands, Cresemba[®] and Zevtera[®]



Leverage

Leverage our expertise in bringing drugs from research to market by utilising appropriate partnerships with established organisations



Invest

Invest in our clinical portfolio of targeted, small molecule, oncology drug candidates and the phase 3 ceftobiprole program



Innovate

Continue to broaden our R&D pipeline through both internal and external innovation

The company we keep — established strong partnerships

License partners



Europe (excl. Nordics), China
Asia-Pacific, Russia, Turkey
and Israel (Cresemba®)



U.S. (Cresemba®)



Japan (Cresemba®)



China (Zevtera®)

Distribution partners



Europe (excl. Nordics), Israel
(Zevtera®)



MENA region
(Cresemba® and Zevtera®)



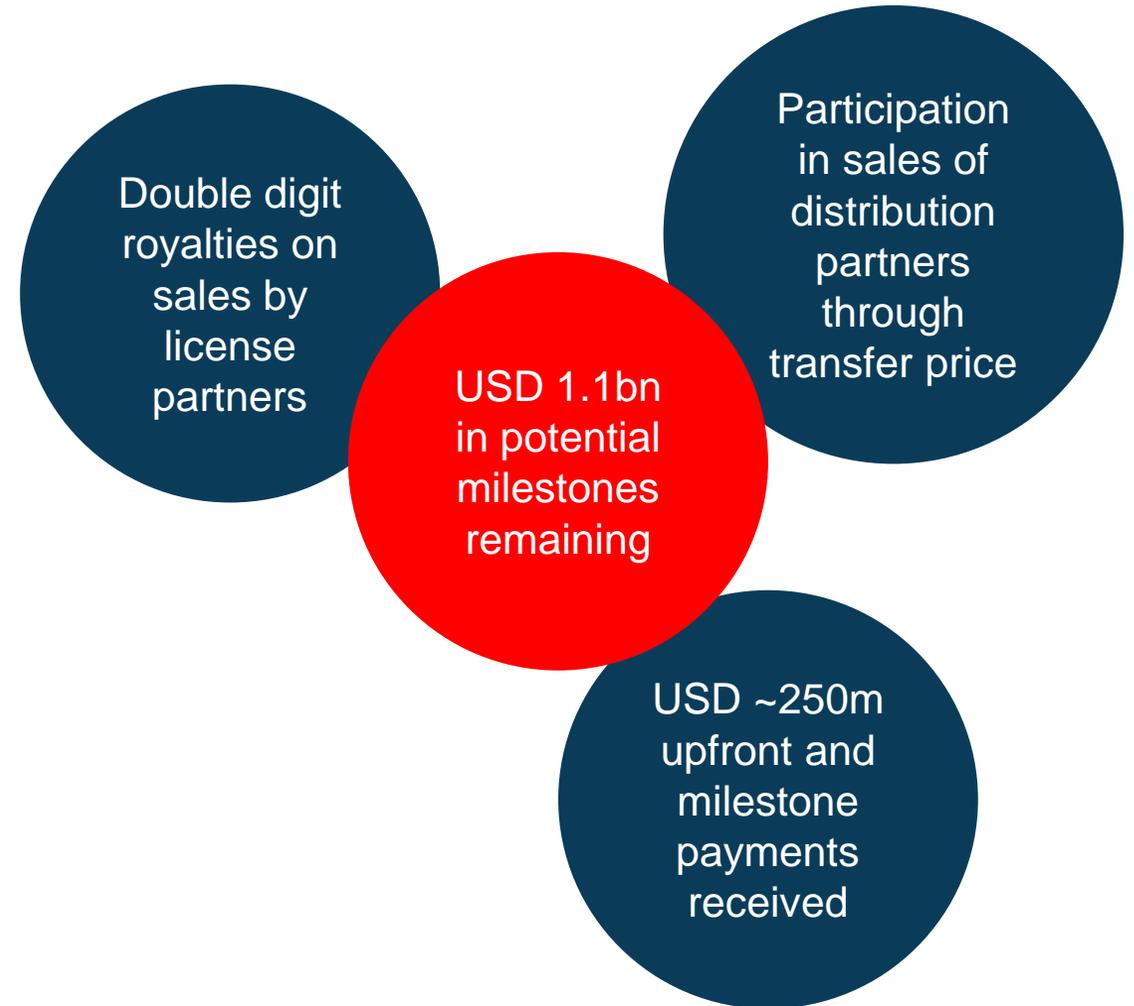
LatAm
(Cresemba® and Zevtera®)



Nordics
(Cresemba® and Zevtera®)



Canada
(Cresemba® and Zevtera®)



Five reasons to invest



Growth

Well funded with increasing and sustainable revenues through commercialized brands



Prospects

Opportunity to share in pipeline value creation and proven approach to the successful commercialization of products around the world



Leadership

Experienced team working in an agile culture able to turn pipeline projects into revenue generating brands



Partnerships

Proven ability to build successful partnerships in research, development and commercialization with leading academic, governmental and industrial organisations

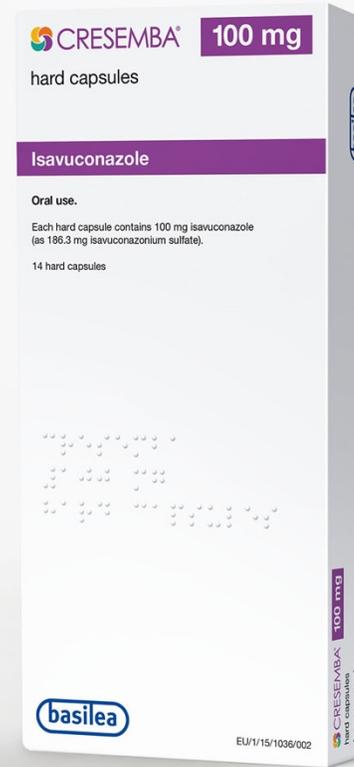


Focus

One of the few biopharmaceutical companies in the world focused on the development and commercialization of targeted oncology small molecules and new antibiotics and antifungals



Portfolio



Antifungal

Cresemba[®]
(Isavuconazole)

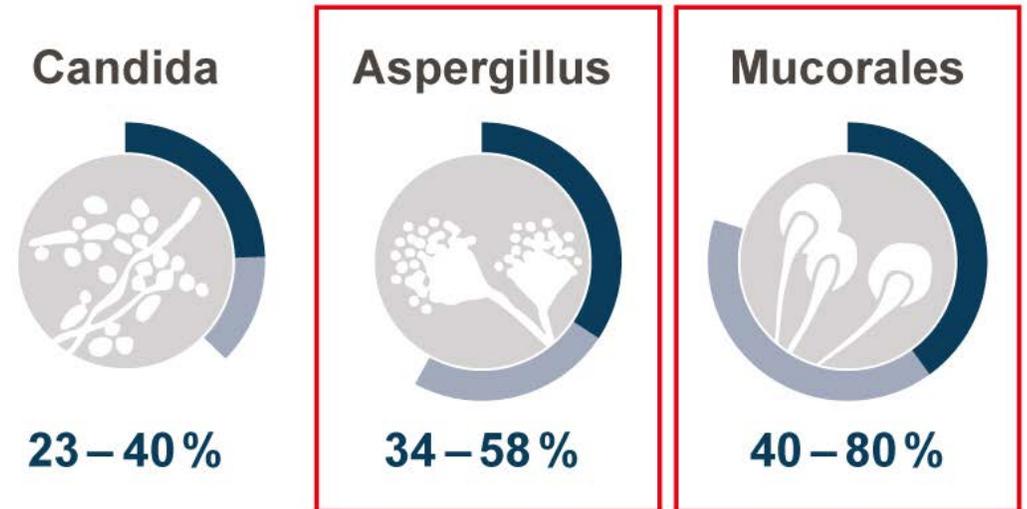
Invasive mold infections



The market — invasive fungal infections

- 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

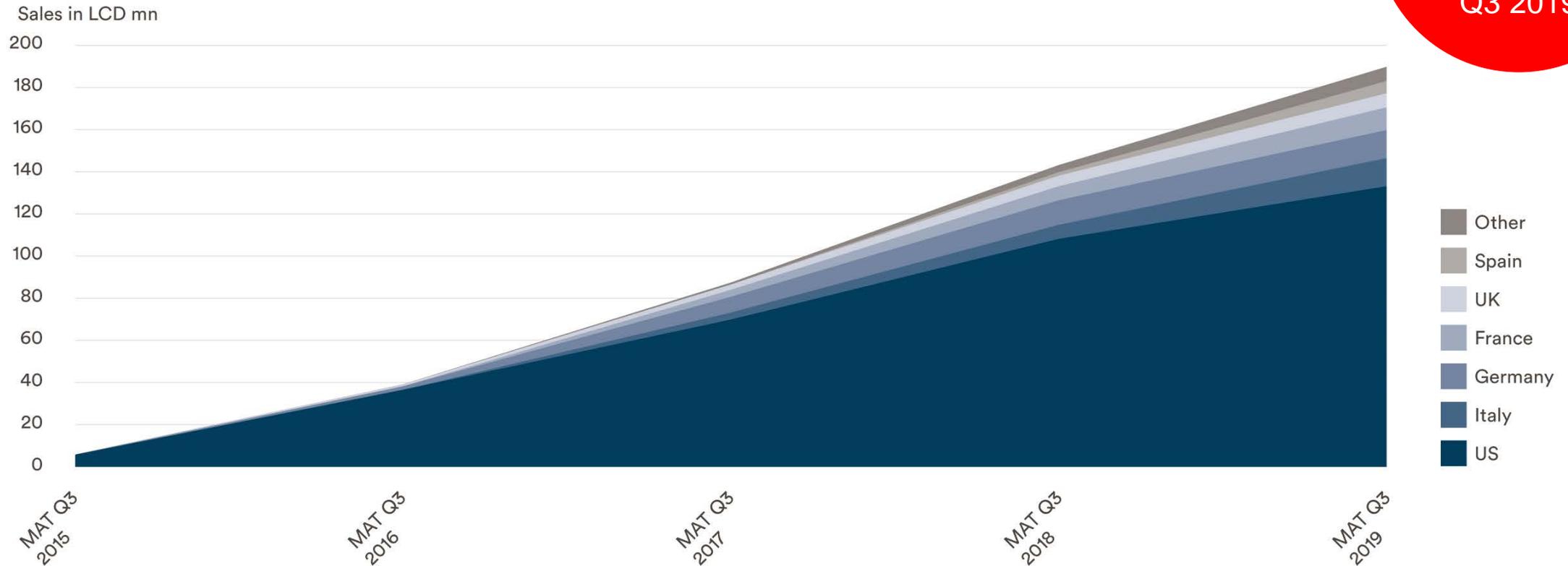
Mortality rates for invasive fungal infections**



**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 190mn
“in-market”
sales in MAT
Q3 2019



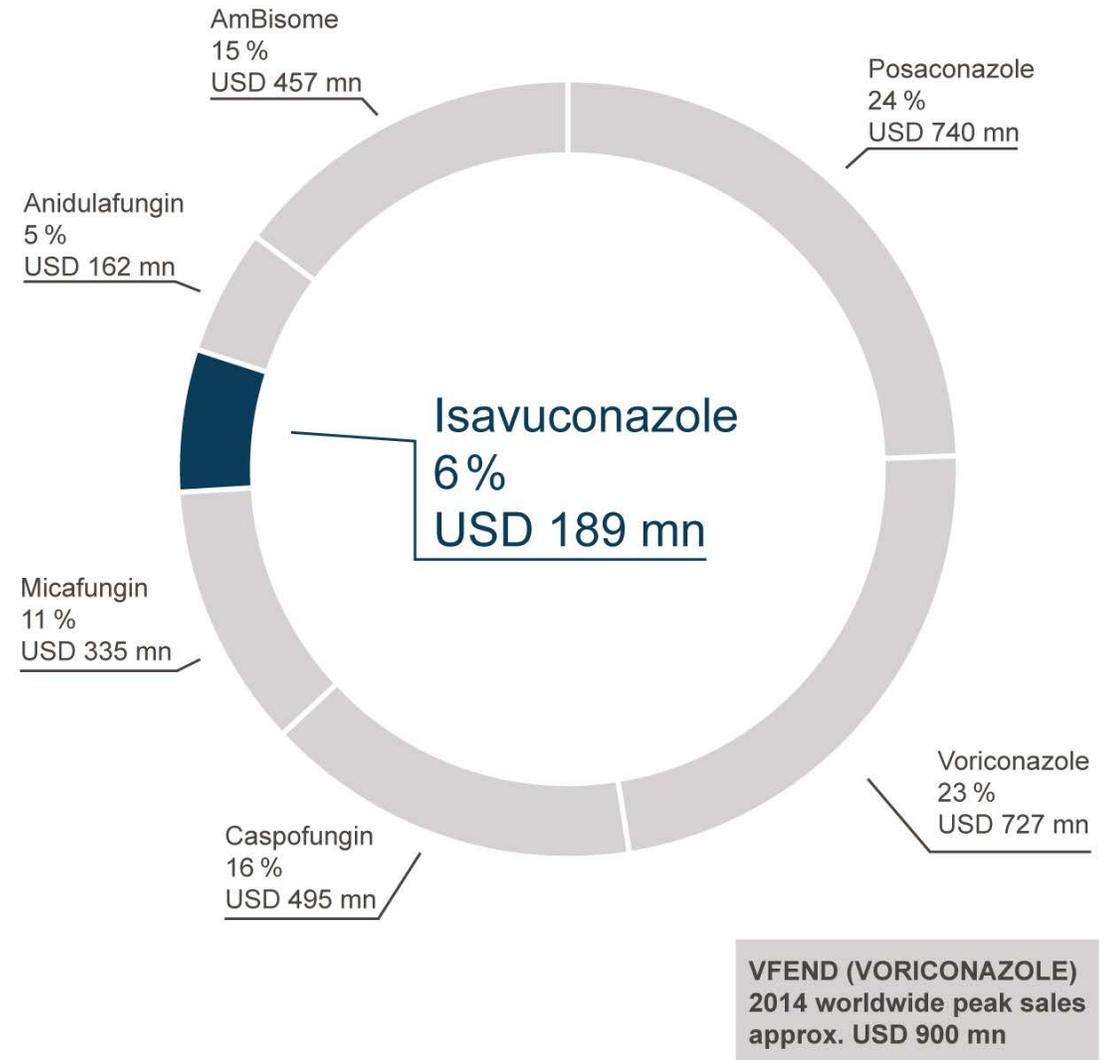
LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, Sept. 2019

Sales of best-in-class antifungals* by product

USD 3.0 bn sales (MAT Q3 2019)

- Potential to increase Cresemba® (isavuconazole) market share
 - Anticipate to be launched in 60 countries by end-2021
 - Exclusivity through 2027 in the U.S. and potential pediatric exclusivity extension to 2027 (from 2025) in the EU

* 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, Sept. 2019

Antibacterial

Zevtera[®] / Mabelio[®]
(Ceftobiprole)

Severe bacterial infections



Zevtera[®] — an introduction

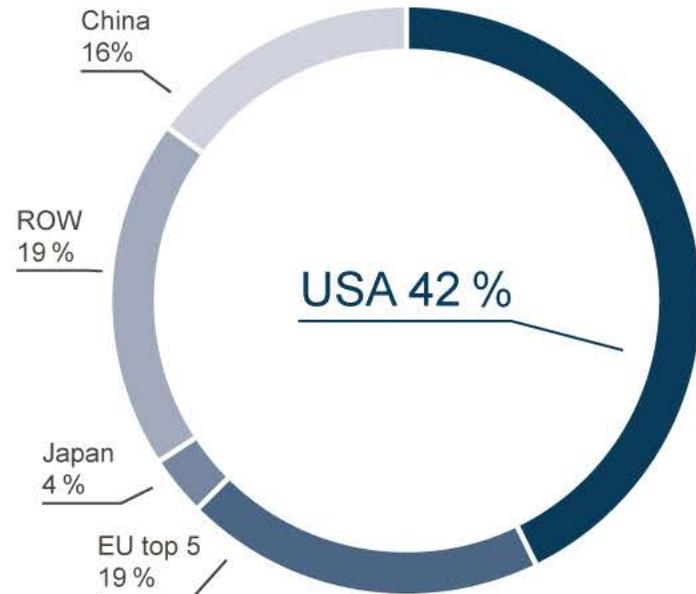
- Broad-spectrum anti-MRSA cephalosporin (including Gram-negative bacteria)
- Rapid bactericidal activity
- Potential to replace antibiotic combinations
- Early improvement in HAP, particularly in patients with MRSA, and CAP, including high-risk patients
- Cephalosporin class safety profile
- Marketed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

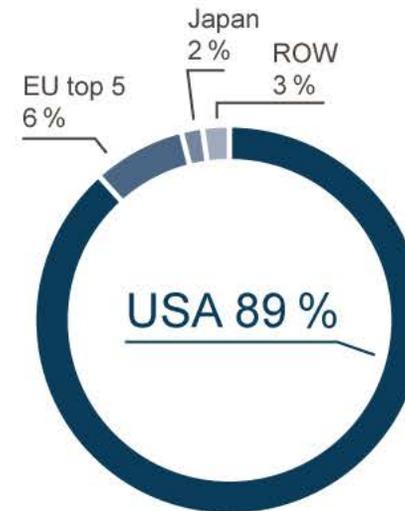


The hospital anti-MRSA antibiotic market — A USD 3.1bn market with the U.S. being the most important region

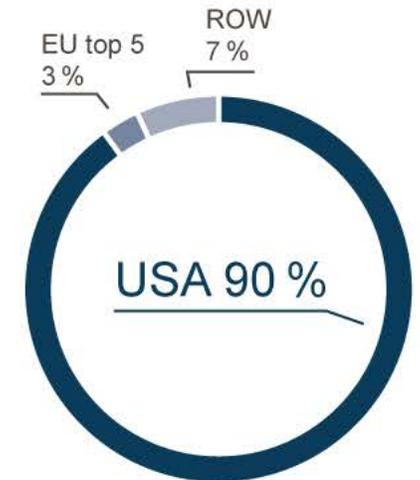
Global anti-MRSA hospital antibiotics sales*
USD 3.1bn (MAT Q3 2019)



Daptomycin sales
by region 2015
(before LOE)



Ceftaroline sales
by region
(MAT Q3 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, Sept. 2019

Strategy for accessing the U.S. market

- Two cross-supportive studies under FDA Special Protocol Assessment (SPA)
- Acute Bacterial Skin and Skin Structure Infections (ABSSSI) successfully completed¹



- *Staphylococcus aureus* bacteremia (SAB) ongoing, anticipated to report topline results in H2 2021²



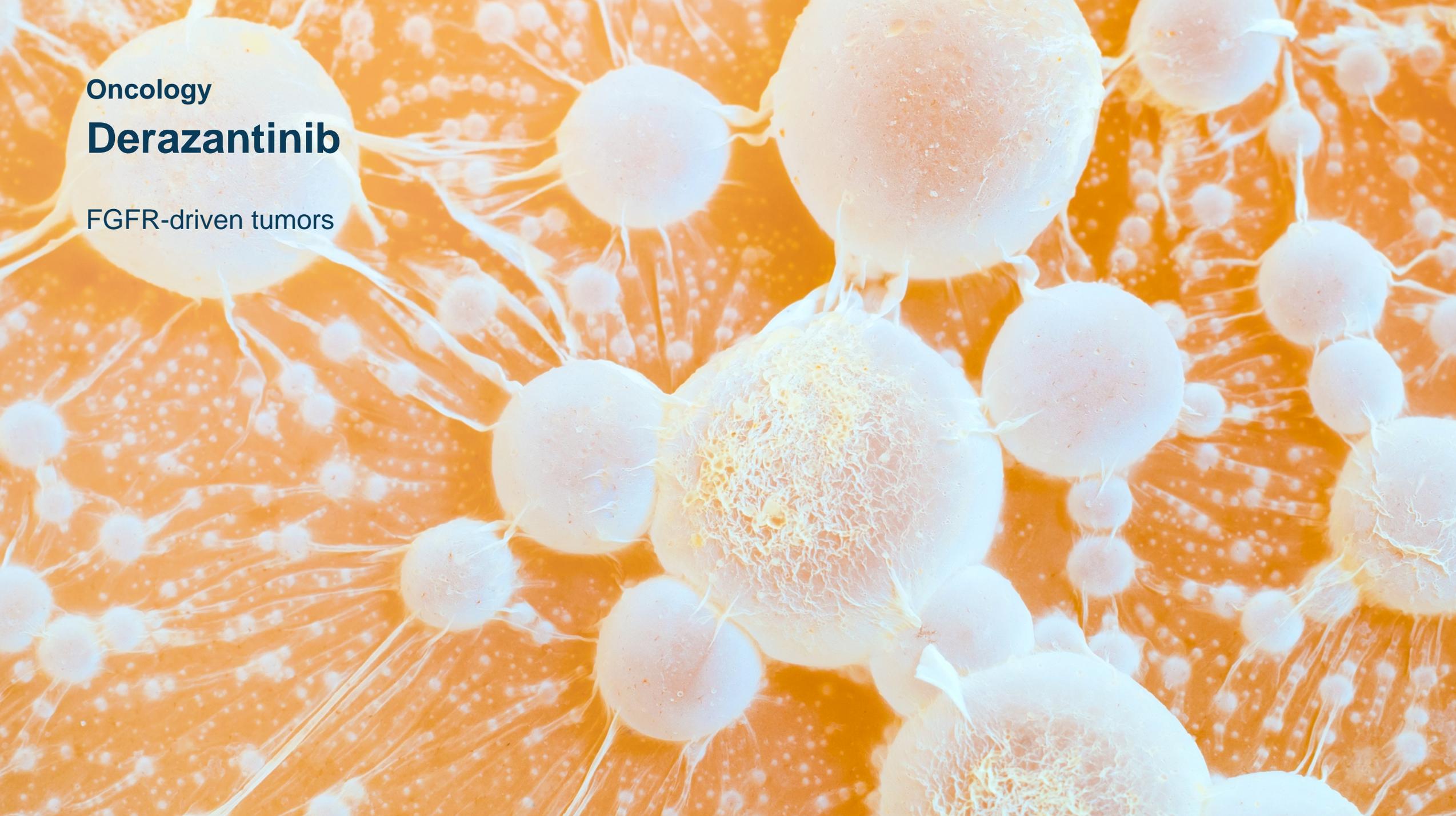
¹ NCT03137173

² NCT03138733

- Phase 3 program largely funded 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

A microscopic image of cells, likely cancer cells, with an orange overlay. The cells are spherical and have a fibrous, web-like structure extending from them. The background is a warm, orange color, and the cells are in various stages of growth and division. The text is overlaid on the top left of the image.

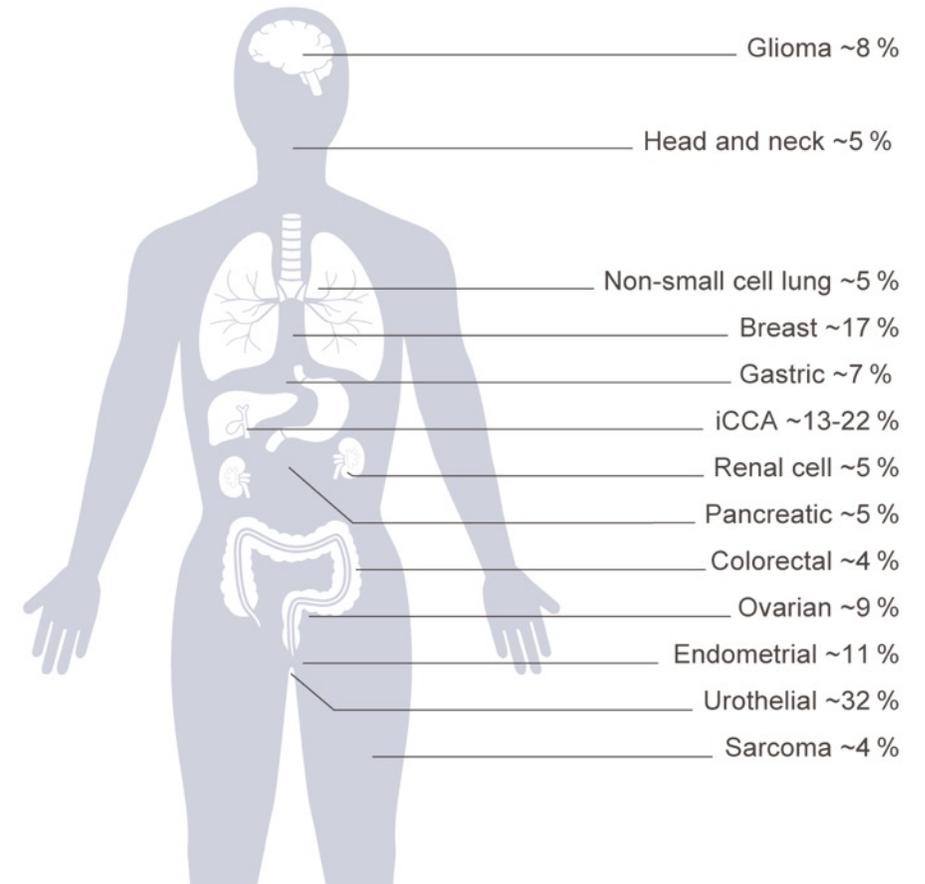
Oncology

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) and VEGFR2 (Vascular Endothelial Growth Factor Receptor 2) 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

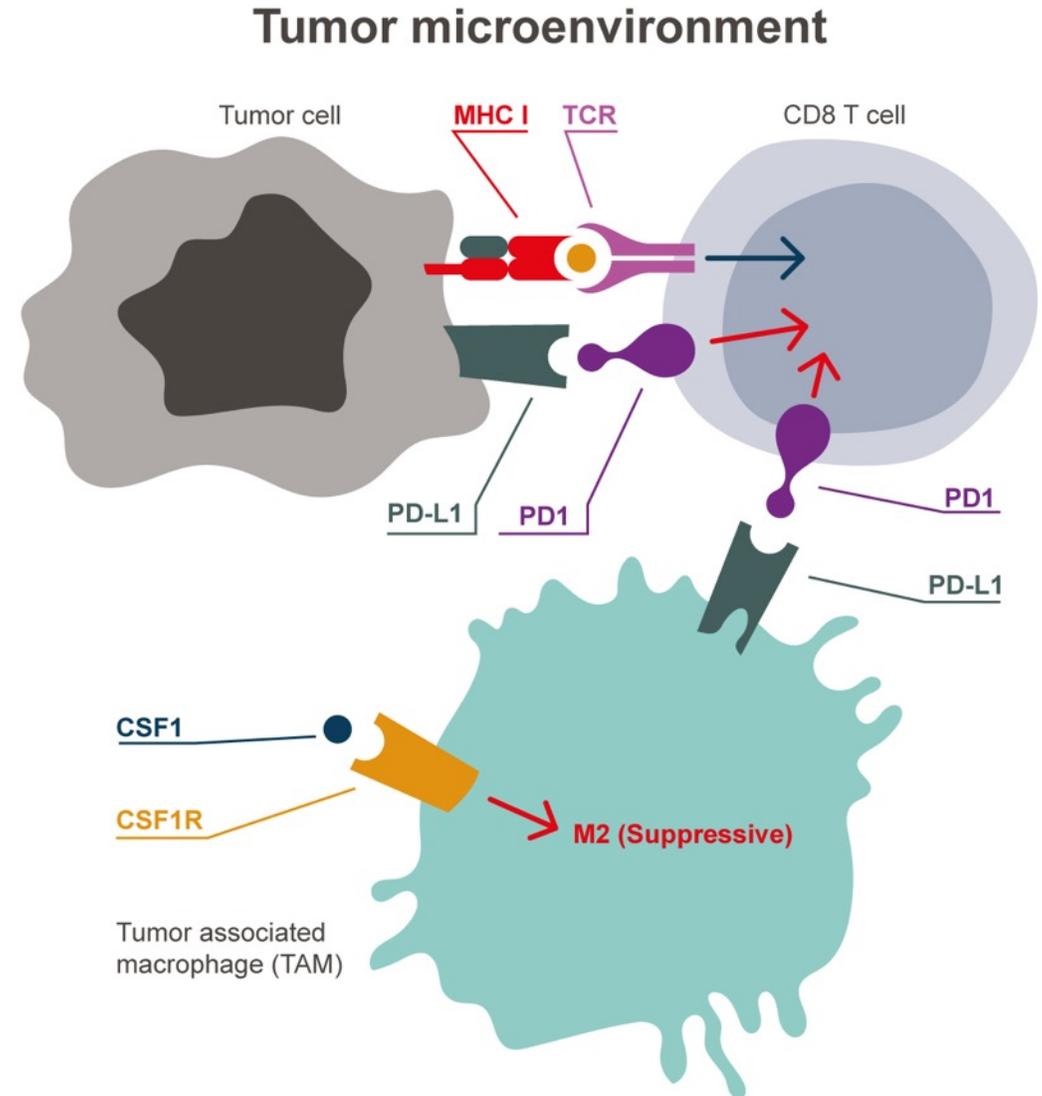
FGFR-inhibitors show differences in kinase-inhibition profiles¹

FGFR-inhibitor compound (Sponsor)	Parameter	FGFR1	FGFR2	FGFR3	FGFR4	CSF1R
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

¹ McSheehy et al. Derazantinib (DZB): A dual FGFR/CSF1R-inhibitor active in PDX-models of urothelial cancer. Mol Cancer Ther. 2019 (18) (12 Supplement) LB-C12

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



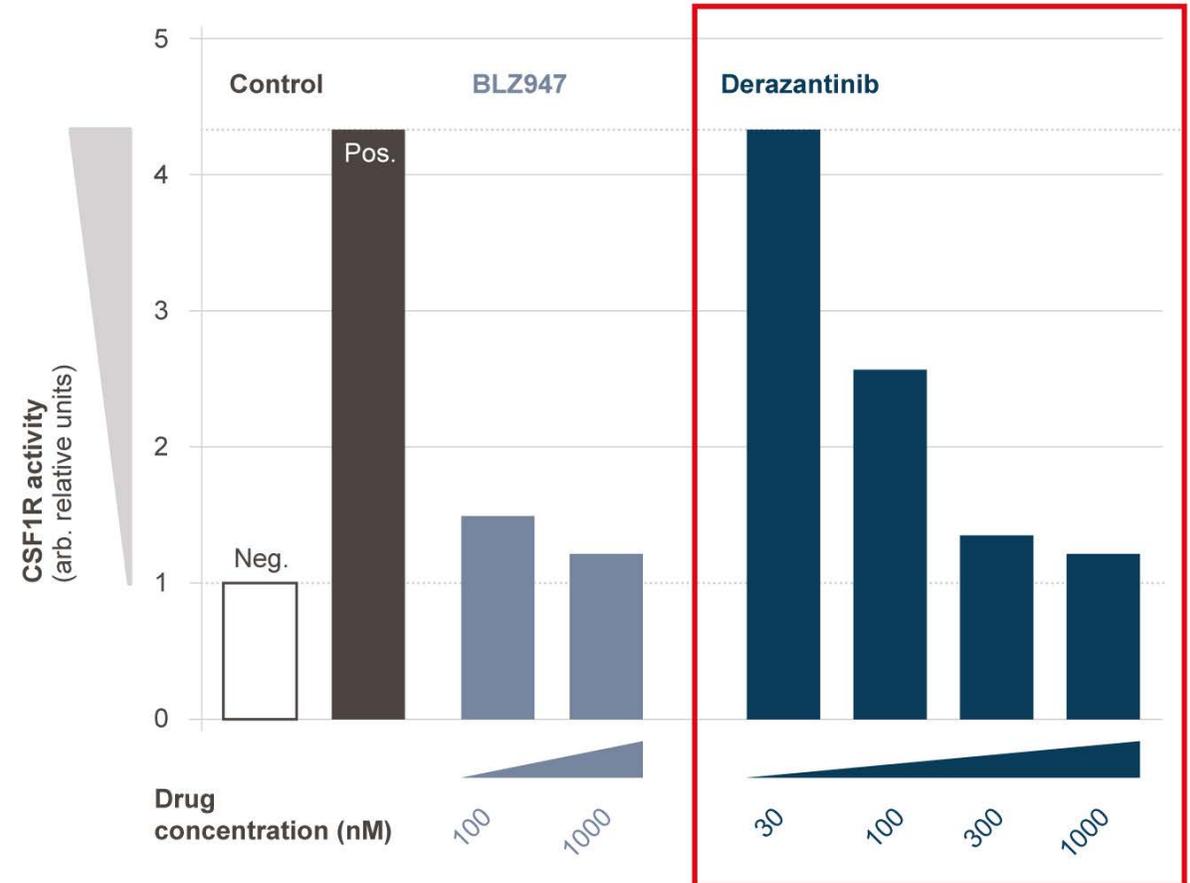
¹ X. Zheng et al. Redirecting tumor-associated macrophages to become tumoricidal effectors as a novel strategy for cancer therapy. *Oncotarget*. 2017;8(29):48436-48452

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 inhibits mouse macrophage CSF1R activity

- Derazantinib treatment reduced CSF1-stimulated CSF1R activation (pCSF1R) in a concentration-dependent manner
- The maximum effect is similar to the specific CSF1R inhibitor BLZ945
- Derazantinib active-concentration (IC₅₀ of 120 nM) is achievable in patients

Inhibition of CSF1R activity



Method: bone-marrow-derived mouse macrophages were starved overnight, treated with CSF1 for 3 min, with or without pre-incubation with BLZ945 or DZB, and then extracted for subsequent immunoblot. The graph shows the quantification of the experiment based on densitometric analysis of the immunoblots

¹ McSheehy et al. Derazantinib (DZB): A dual FGFR/CSF1R-inhibitor active in PDX-models of urothelial cancer. Mol Cancer Ther. 2019 (18) (12 Supplement) LB-C12

FGFR-inhibitors show differences in safety profiles

	Cholangiocarcinoma				Urothelial cancer	
	DZB ¹ (N=44)	INF ² (N=71)	FUT ³ (N=45)	PEM ⁴ (N=146)	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 safety events	Phosphorus↑ Nausea Vomiting	Phosphorus↑ Fatigue Stomatitis	Phosphorus↑ Constipation AST↑	Phosphorus↑ Alopecia Diarrhoea	Diarrhoea Alopecia Constipation	Phosphorus↑ Stomatitis Dry mouth
Blood phosphorus↑†	59%	73%	80%	60%	31%	73%
Fatigue†	43%	49%	NR	42%	32%	≥21%
Alopecia†	20%	38%	NR	49%	NR	≥27%
Dry eye/xerophthalmia†	16%	32%	NR	25%	NR	≥19%
Central serous retinopathy	0%	NR	NR	4%	NR	21%
Alanine aminotransferase (ALT) ↑	30%	NR	31%	NR	NR	41% ⁷
Hand-foot syndrome/PPE	0%	27%	22%	>5%**	NR	≥22%
Nail toxicities	<5%	NR	NR	42%	NR	52%
Stomatitis	11%	45%	22%	35%	34%	≥55%

Sources: ¹ Droz Dit Busset et al., ESMO 2019 and Basilea data on file; ² Javle et al., ESMO 2018; ³ Meric-Bernstam et al, ESMO WC GI Cancer, 2018;

⁴ Vogel, et al., ESMO 2019; 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;

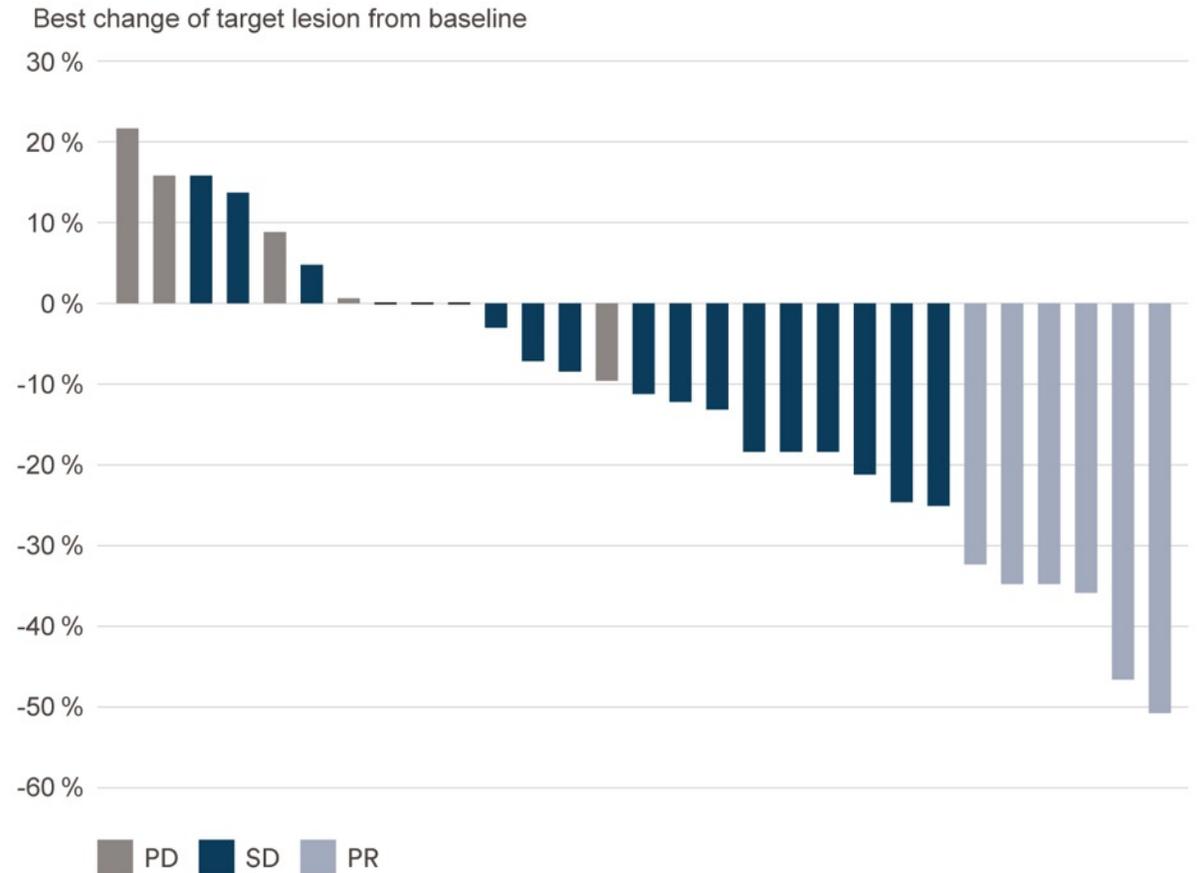
PPES: Palmar-plantar erythrodysesthesia; NR: not reported; QD: daily; Q3W/Q4W: every 3/4 weeks; w: weeks.

* Drug-related events reported only; † assumed FGFR inhibitor class-effect; **AE frequency not reported, but 8/146 (5.5%) patients were reported with dose interruptions due to PPE

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 — 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

Oncology

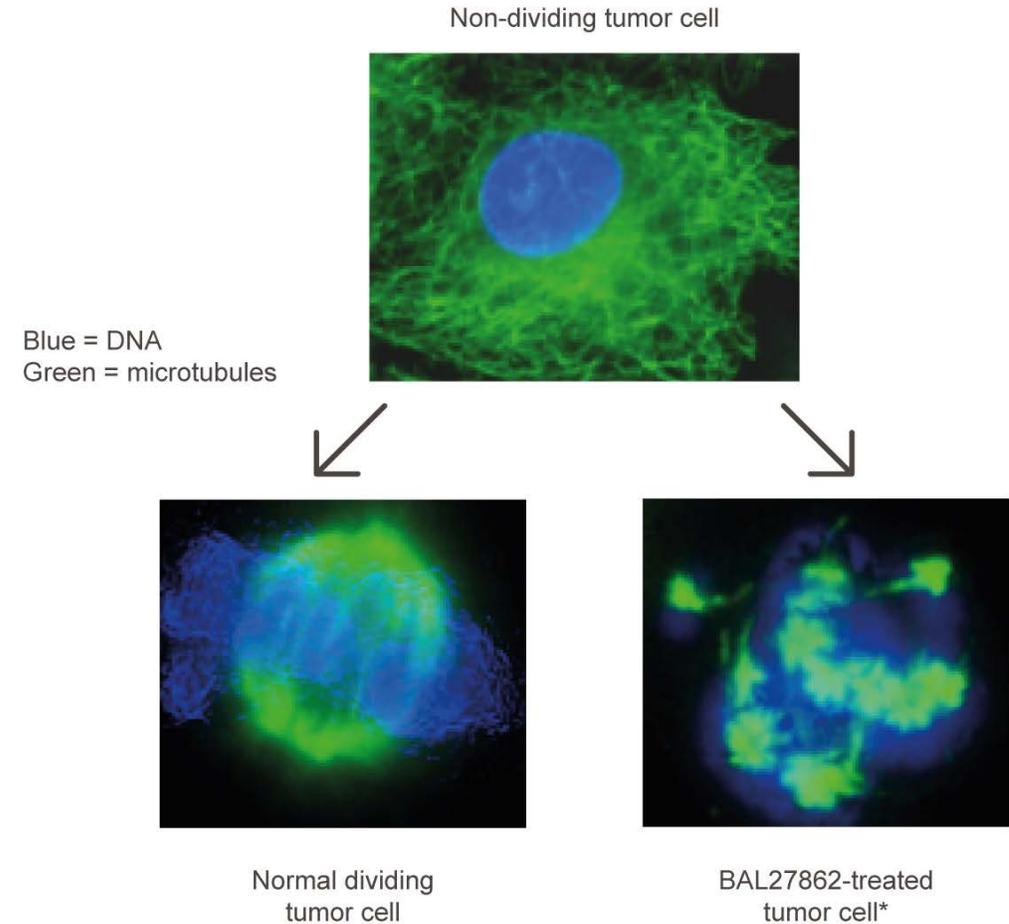
Lisavanbulin (BAL101553)

Glioblastoma
and other solid tumors



Lisavanbulin — 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 selection

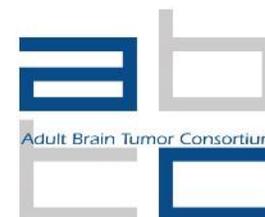


* Lisavanbulin (BAL101553) is a prodrug of BAL27862

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

Lisavanbulin — focus on a selected, biomarker-driven approach

- Two recent phase 1/2 clinical studies:
 - Concluded patient enrolment in phase 1 dose escalation study¹ (daily oral) in patients with recurrent glioblastoma or high-grade glioma in August 2019
 - Concluded patient enrolment in phase 2a expansion study using weekly 48-hour intravenous (i.v.) administration in patients with recurrent GBM or with platinum-resistant ovarian cancer in December 2019²
- Profound objective responses in GBM across the two studies
 - More than 80% reduction of the GBM tumor area observed in two patients with glioblastoma
 - GBM tumor tissue of one of these patients displayed strong expression of EB1
- Plan to start a phase 2 oral biomarker-driven glioblastoma study in mid-2020
- Ongoing 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 NCT02490800

² NCT02895360

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

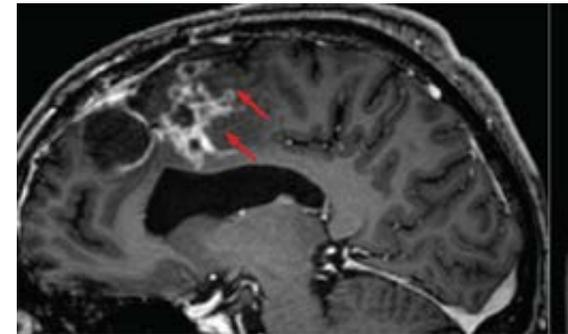
EB1 — A potential response-predictive clinical biomarker for lisavanbulin

- Strong EB1 staining was observed in a patient with an exceptional response to daily oral lisavanbulin in the phase 1 dose-escalation study in recurrent GBM¹
 - Patient ongoing for >20 months
 - >80% reduction in GBM tumor size
- Potential utility of EB1 and other biomarkers to support a biomarker-driven clinical program, which is currently being planned
- Predictive of response to lisavanbulin in mouse models²

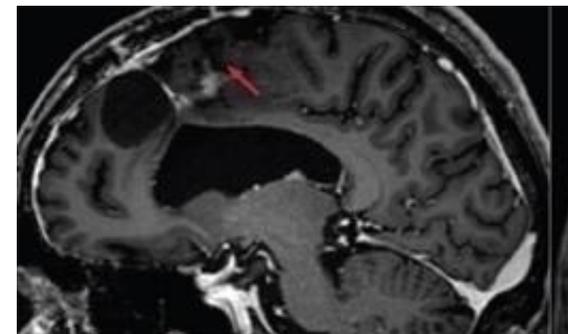
¹ Lopez et al. Phase 1/2a study of once daily oral BAL101553, a novel tumor checkpoint controller, in adult patients with progressive or recurrent glioblastoma or high-grade glioma. JCO 2019;37:15 suppl, 2025

² Berges et al. EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after oral treatment with the novel tubulin-binding checkpoint activator BAL101553. Eur. J. Cancer 2018, 103, E61-62, A166

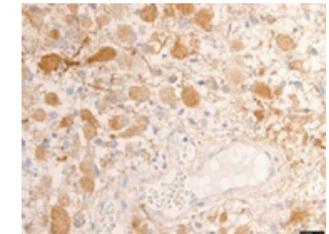
GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



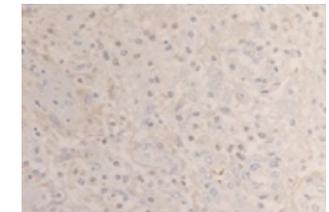
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



Non-responder



Financials

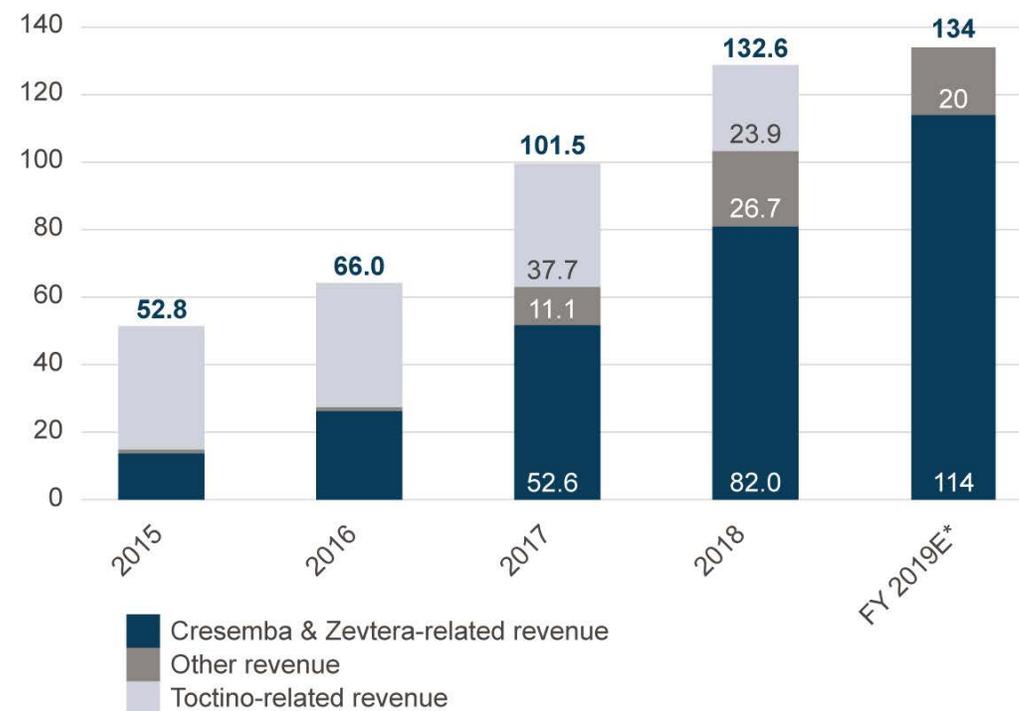


Financials

In CHF mn	FY 2018 actuals	FY 2019 guidance	FY2019E*
Total revenue	132.6	128 – 133	134
thereof: Contributions Cresemba & Zevtera	82.0	105 – 110	114
Operating loss	24.1	22 – 27	
Net operating cash consumption	79.2	60 – 65	
Cash and financial investments	223.0	-	161

* The audited full financial statements as well as the annual report 2019 will be published on February 18, 2020. The final audited revenue for 2019 and the cash position as of year-end 2019 may differ from the preliminary reported numbers.

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



Focus 2019 and 2020

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 FGFR2 gene aberrations			Interim data from iCCA in other FGFR2 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
				Plan to start a phase 1/2 study in gastric cancer
Lisavanbulin (BAL101553)		✓ Completed patient enrolment in phase 1 study arm for recurrent glioblastoma (oral)		Plan to start a phase 2 biomarker-driven glioblastoma study (oral)
		✓ Completed interim data review of phase 2a study in ovarian cancer and glioblastoma (48-hr. i.v.)		
				Complete patient enrolment in phase 1 study in newly diagnosed glioblastoma (oral)

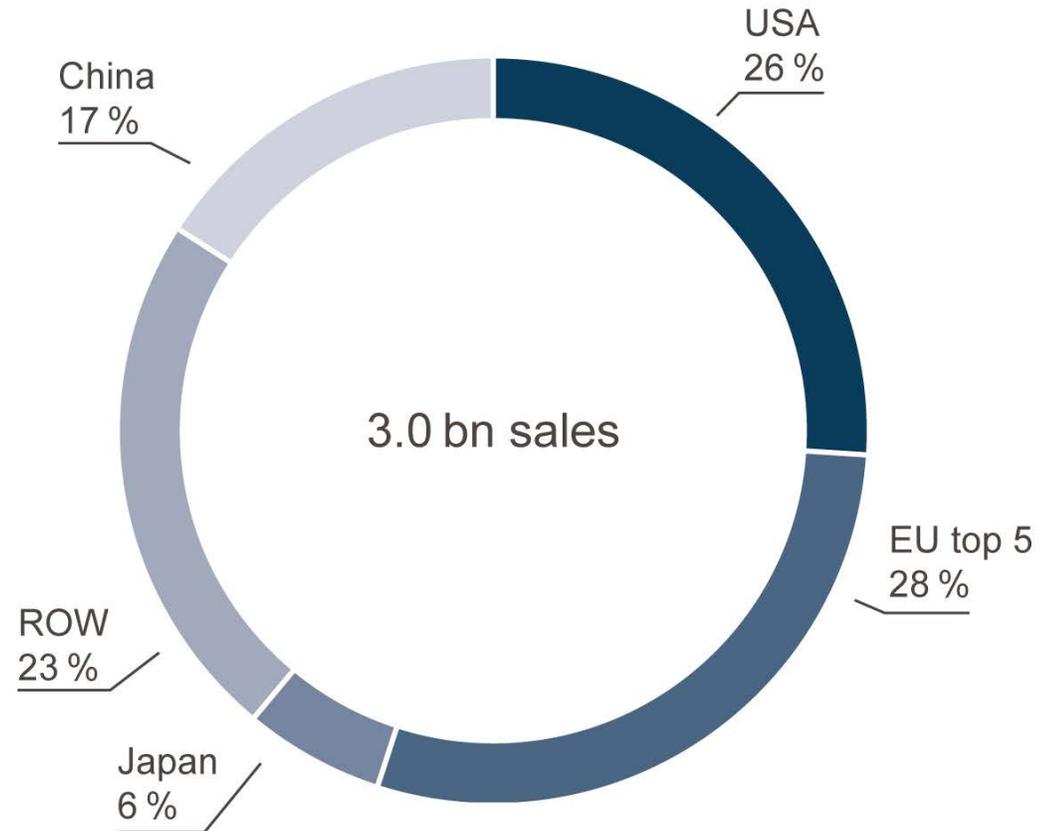


“Patients are at the heart
of what we do”

Appendix

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 2019)

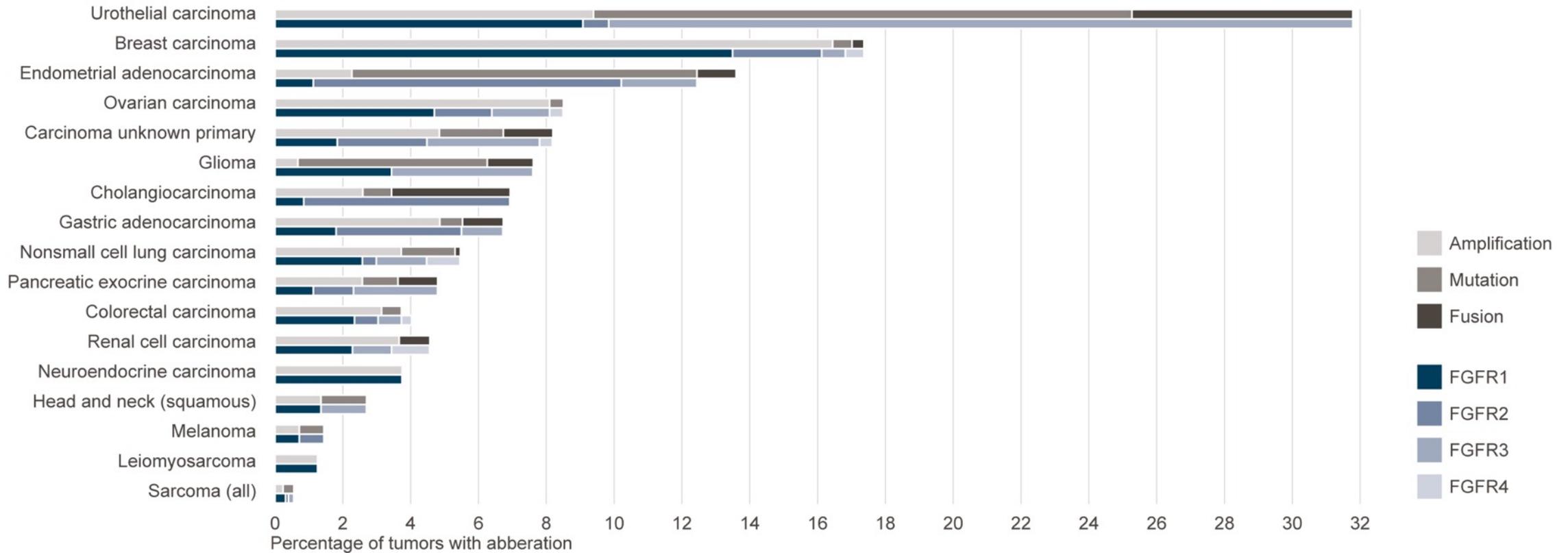


* Best-in-class antifungals: isavuconazole, posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin

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

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

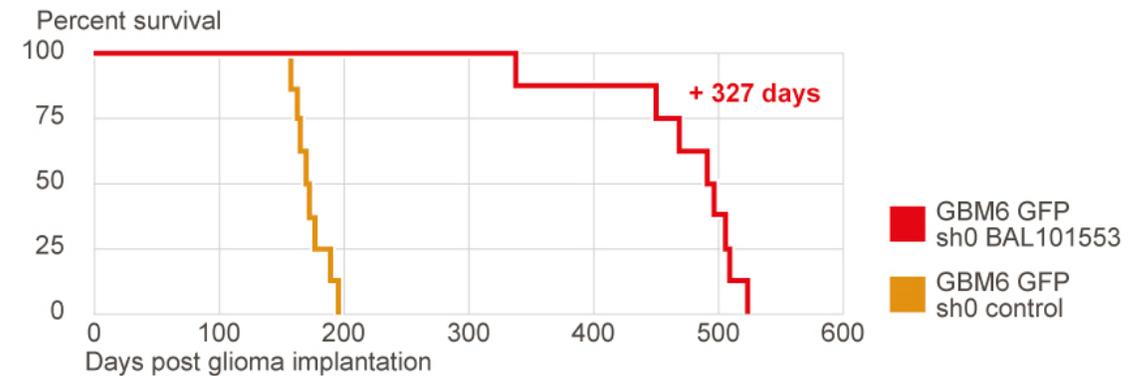
EB1 — A potential response-predictive clinical biomarker for lisavanbulin

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

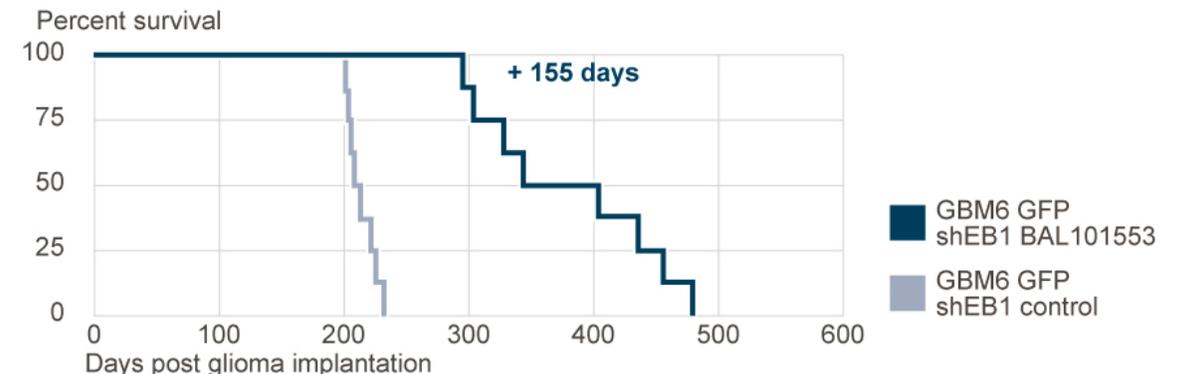
¹ Berges et al. EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after oral treatment with the novel tubulin-binding checkpoint activator BAL101553. Eur. J. Cancer 2018, 103, E61-62, A166

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

EB1-expressing GBM



EB1-downregulated GBM





Oncology

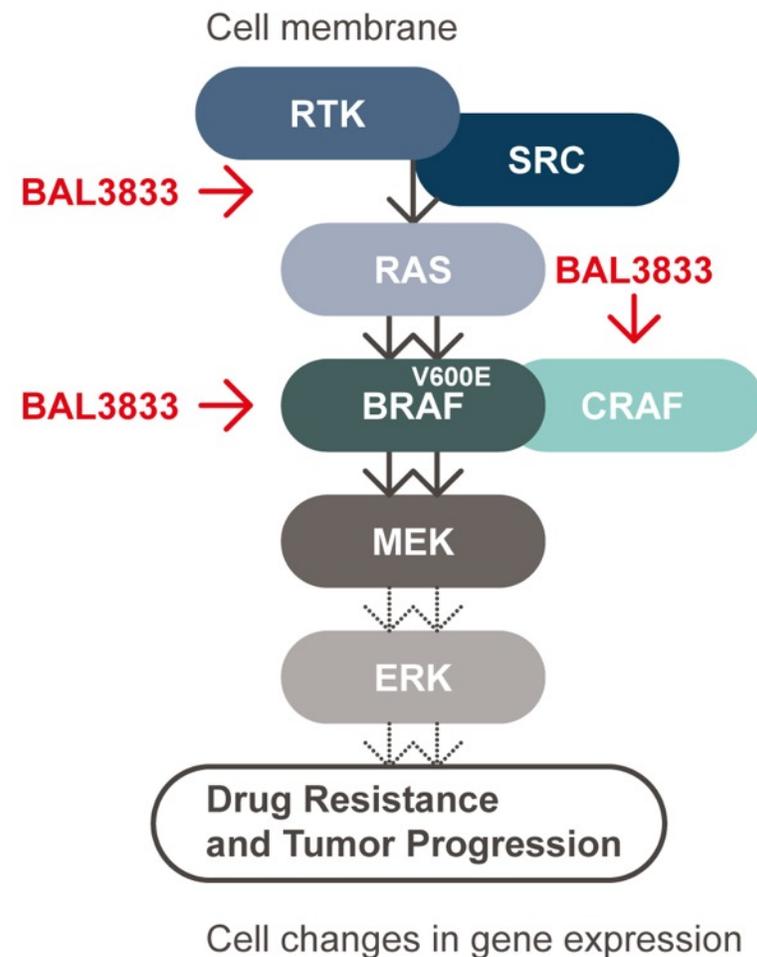
BAL3833

Melanoma and
RAS-driven tumors

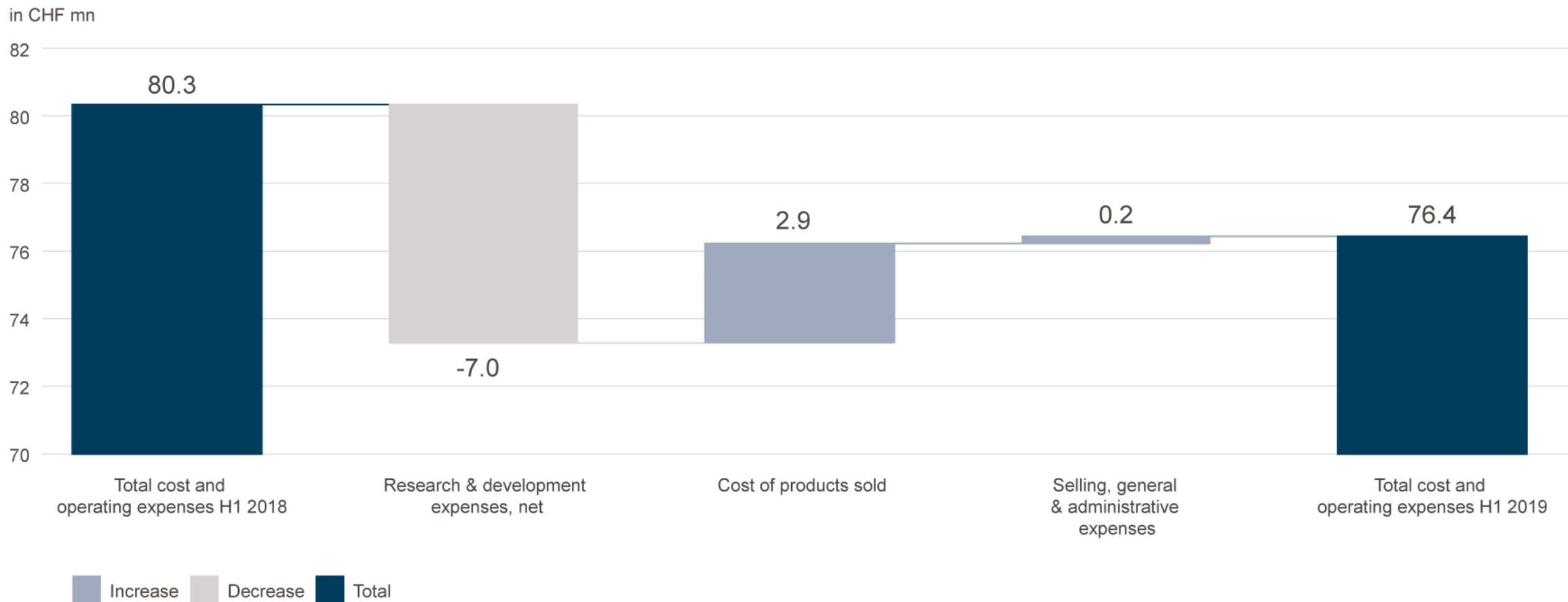
BAL3833 — panRAF/SRC kinase inhibitor

- 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



Cost and operating expenses H1 2019 versus H1 2018



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:** 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



Focused on Growth and Innovation

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