



**Focused on
Growth and Innovation**

David Veitch, CEO

**UBS Virtual Global Healthcare Conference
Investor presentation – May 19, 2020**



At a glance

- Well funded, commercial-stage biotech company with significantly growing cash flows from commercialized products
- Focused in the areas of oncology and infectious diseases
- 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

Products / Product candidates / Indication		Preclinical	Phase 1	Phase 2	Phase 3	Market
Antifungals	Cresemba® (isavuconazole) Invasive aspergillosis and mucormycosis (U.S. and EU and several other countries) Invasive fungal infections (Japan)	intravenous and oral				
		intravenous and oral				
Antibiotics	Zevtera®/Mabelio® (ceftobiprole) Hospital- and community-acquired pneumonia (HAP, CAP) (major European and several non-European countries) Acute bacterial skin and skin structure infections (ABSSSI) Staphylococcus aureus (MSSA/MRSA) bacteremia (bloodstream infections)	intravenous				
		intravenous				
		intravenous				
Oncology	Derazantinib FGFR kinase inhibitor Intrahepatic cholangiocarcinoma (iCCA) – registrational study	oral				
	Urothelial cancer – monotherapy and combination with atezolizumab (Tecentriq®)*	oral				
	Gastric cancer (planned study start Q3 2020)	oral				
	Lisavanbulin (BAL101553) tumor checkpoint controller Glioblastoma (targeted, biomarker-driven phase 2 study, planned study start mid-2020)	oral				
	Glioblastoma – combination with radiotherapy	oral				
	BAL3833 panRAF/SRC kinase inhibitor Solid tumors	oral **				
Internal & external innovation		Research	Development			

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

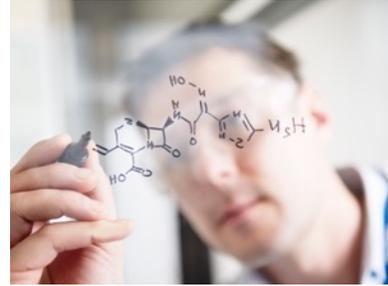
** pre-clinical reformulation activities ongoing.

Our strategy



Foster

Foster an agile organisation based on a dynamic and open culture



Focus

Focus on continuously increasing cash flow 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

Global coverage — Cresemba®



Over 100 countries covered by partnerships

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®)





Portfolio



Antifungal

Cresemba[®]
(isavuconazole)

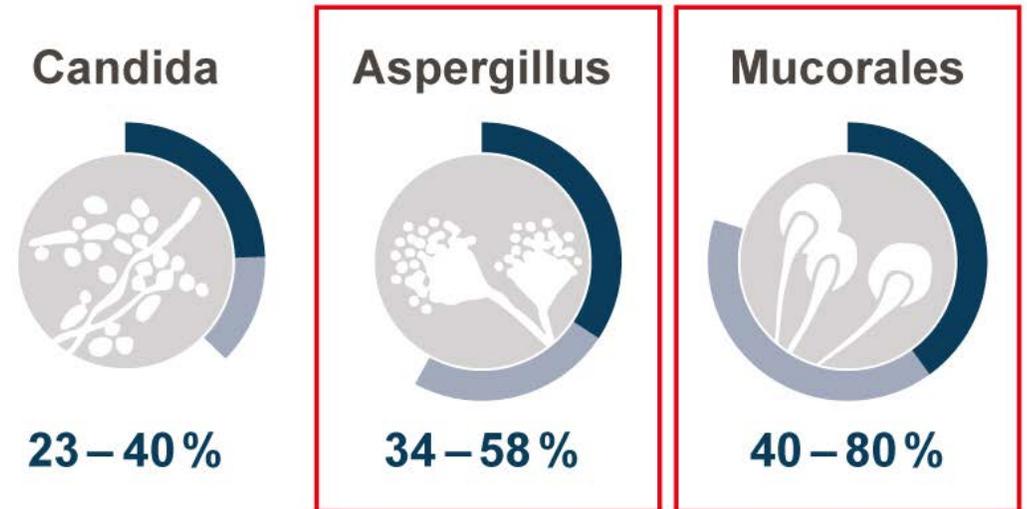
Invasive mold infections



The market — Invasive fungal infections

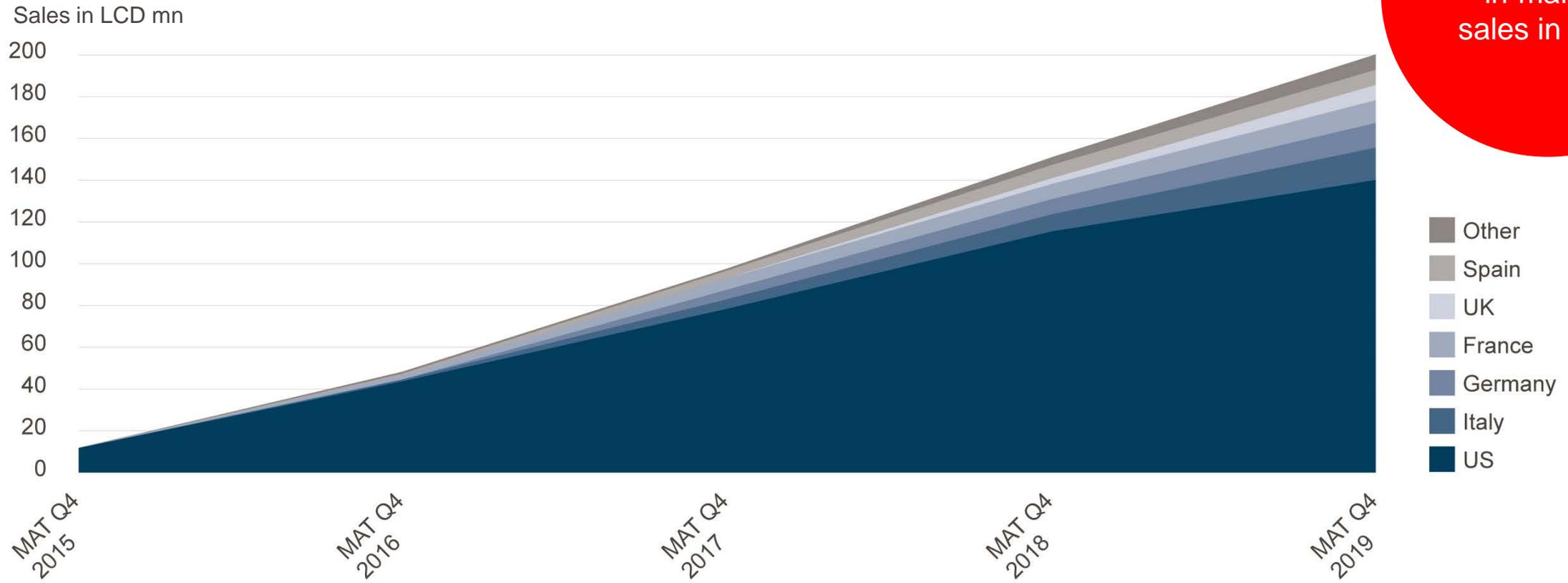
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand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



> USD 200 mn
“in-market”
sales in 2019

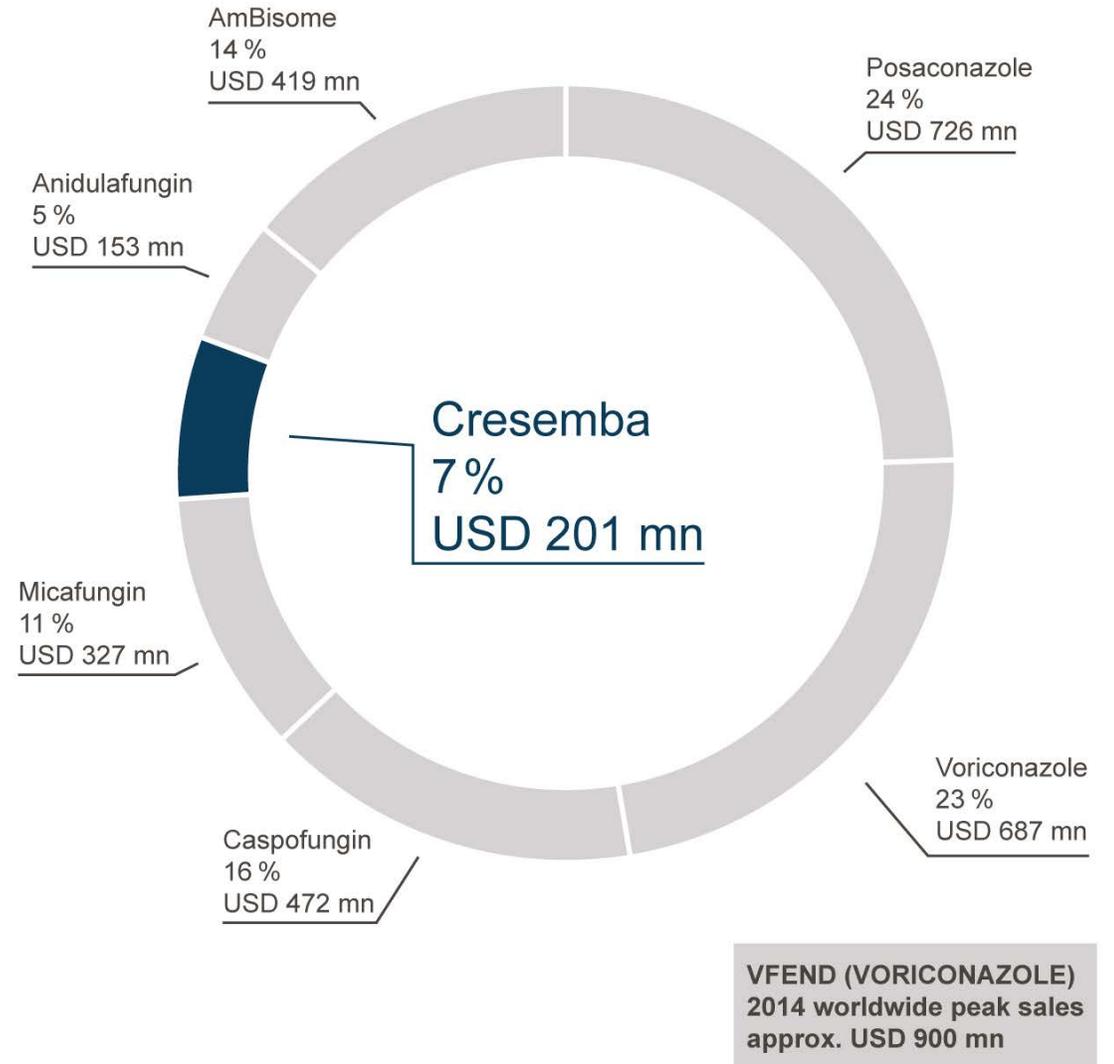
LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, Dec. 2019

Sales of best-in-class antifungals* by product

USD 3 bn sales (MAT Q4 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, Dec. 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

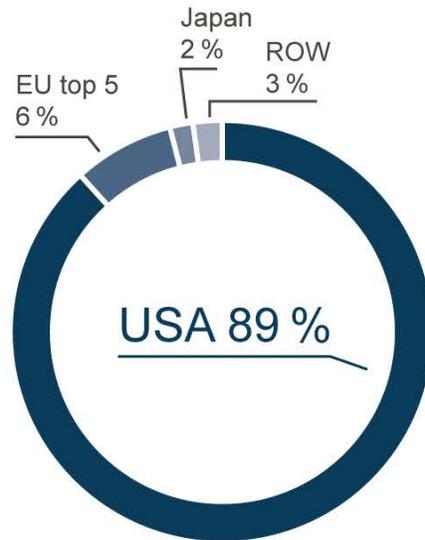
Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP), and community-acquired pneumonia (CAP). Not approved in the U.S.

MENA: Middle East and North Africa

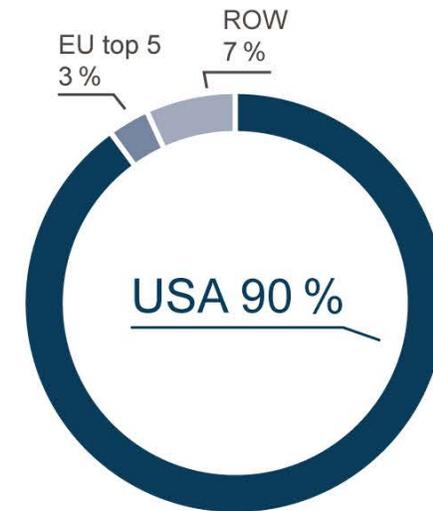


The hospital anti-MRSA antibiotic market — A USD 3 bn market* with the U.S. being the most important region

Daptomycin sales by region 2015 (before LOE)



Ceftaroline sales by region (MAT Q4 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, Dec. 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)² ongoing, on track to report topline results in H2 2021



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 textured surface. Some cells are larger and more prominent than others. The background is a dense network of fine, fibrous structures. The overall color scheme is dominated by shades of orange and yellow.

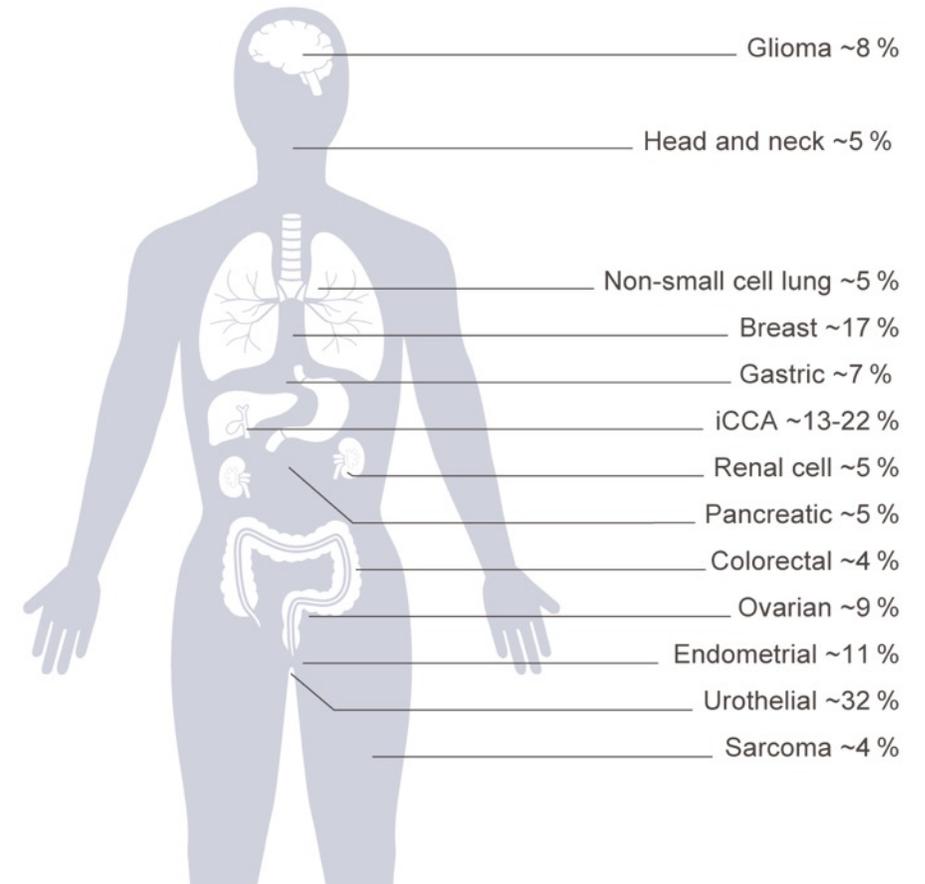
Oncology

Derazantinib

FGFR-driven tumors

Targeting FGFR-driven tumors as single agent and in combination with immunotherapy

- Small molecule, oral inhibitor of FGFR family of kinases
- 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 and VEGFR2 kinase
 - Safety profile: exploring relevance for potential combination therapies
- Two clinical studies ongoing (FIDES-01 in iCCA & FIDES-02 in urothelial cancer)
- Plan to start a multi-cohort phase 1/2 study (FIDES-03) in patients with advanced gastric cancer in Q3 2020



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

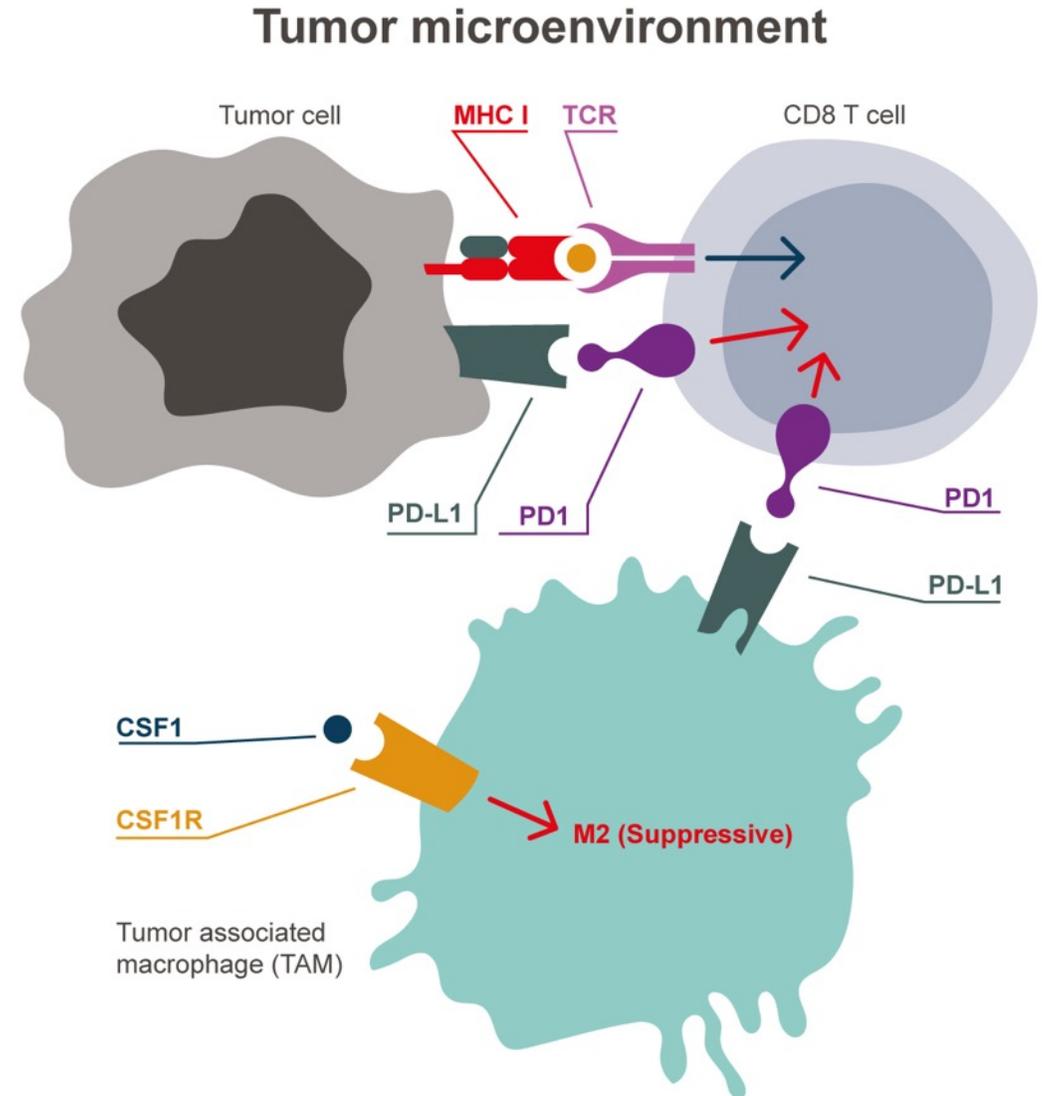
FGFR-inhibitors show differences in kinase-inhibition profiles¹

FGFR-inhibitor compound (Sponsor)	Parameter	FGFR1	FGFR2	FGFR3	FGFR4	CSF1R	VEGFR2
Derazantinib (Basilea)	Ratio to FGFR2 activity	4	1	4	77	3	6
Pemigatinib (Incyte)	Ratio to FGFR2 activity	3	1	4	39	231	62
Erdafitinib (Janssen)	Ratio to FGFR2 activity	2	1	2	13	95	6
Rogaratinib (Bayer)	Ratio to FGFR2 activity	5	1	6	18	116	48
Infigratinib (QED)	Ratio to FGFR2 activity	2	1	2	47	86	55
Futibatinib (Taiho)	Ratio to FGFR2 activity	2	1	2	18	NA	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

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=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

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 H2 2020

Cohort 2: Patients with FGFR2 gene mutations or amplifications

- 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

Clinical program in urothelial and gastric cancer

FIDES-02¹ | Urothelial Cancer

Multi-cohort Phase 1b/2 study of derazantinib monotherapy or in combination with atezolizumab (Tecentriq[®]) in patients with urothelial cancer expressing activating molecular FGFR aberrations

- Substudies (N≈300) in various treatment settings, including:
 - Post-chemotherapy/immunotherapy recurrence (second-line and post second-line)
 - First-line platinum-ineligible, PD-L1-low
 - Resistance to prior FGFR-inhibitor treatment
- Study conducted in multiple centers in Asia-Pacific, Europe and North America
- First interim data expected in H2 2020

FIDES-03 | Gastric Cancer

Multi-cohort Phase 1b/2 study of derazantinib as monotherapy or in combination therapy with standard of care or atezolizumab in patients with advanced HER2-negative gastric adenocarcinoma harboring FGFR genetic aberrations

- Substudies using derazantinib monotherapy or combination treatment, including:
 - Derazantinib monotherapy in various molecular subtypes
 - Combination of derazantinib and standard of care
 - Combination of derazantinib with atezolizumab (Tecentriq[®])
- Study will be conducted in multiple centers in Asia-Pacific, Europe and North America
- Expected start of enrolment in Q3 2020

¹ NCT04045613

Oncology

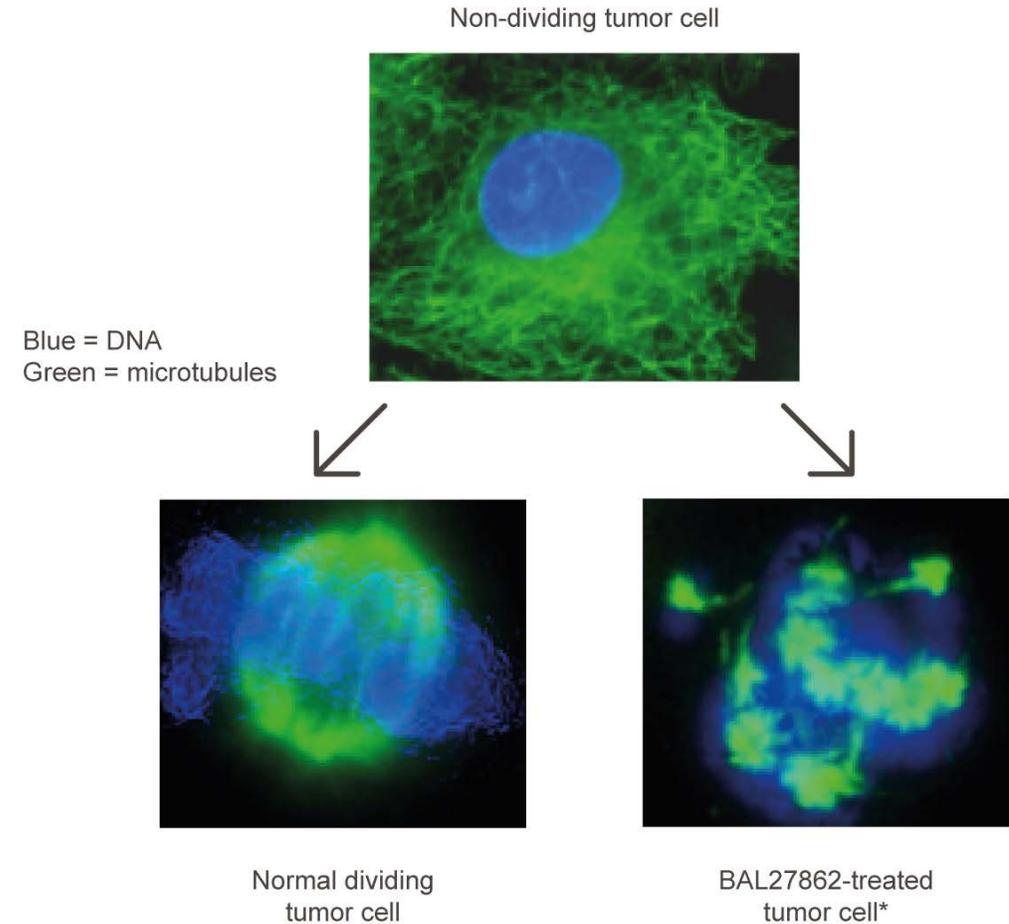
Lisavanbulin (BAL101553)

Glioblastoma
and other solid tumors



Novel tumor checkpoint controller crossing the blood-brain barrier

- Novel compound inducing tumor cell death through spindle assembly checkpoint activation
- Targeting diverse tumor types resistant to standard therapeutic approaches
- Flexible dosing potential, including daily oral dosing
- Comprehensive biomarker program to optimize patient selection
- Crosses the blood-brain barrier with potent activity in brain tumor models alone and in combination
- Clinical program focused on glioblastoma (GBM) using a biomarker-driven approach



* Lisavanbulin (BAL101553) is a prodrug of BAL27862

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

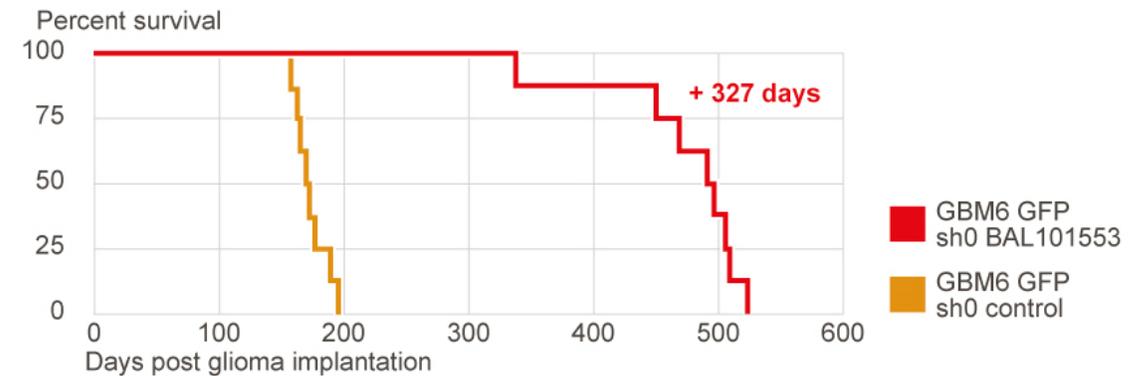
EB1 — A potential response-predictive clinical biomarker for lisavanbulin

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

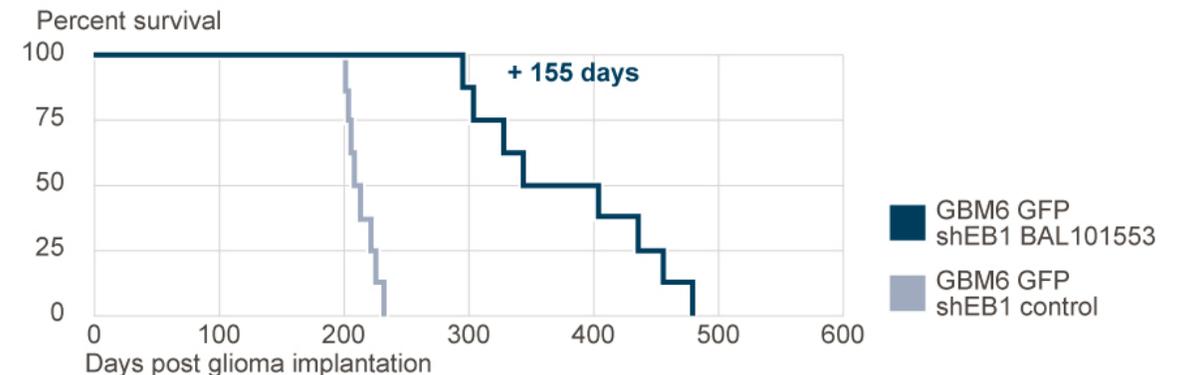
¹ Berges et al. EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after oral treatment with the novel tubulin-binding checkpoint activator BAL101553. Eur. J. Cancer 2018, 103, E61-62, A166

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

EB1-expressing GBM



EB1-downregulated GBM

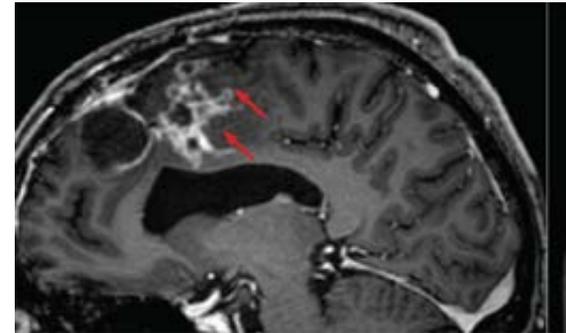


EB1 — A potential response-predictive clinical biomarker for lisavanbulin

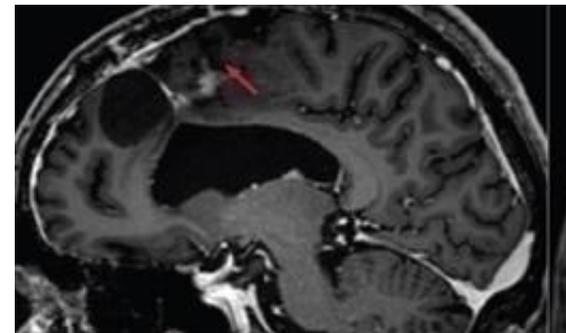
- EB1 (plus-end binding protein) is located on the microtubules and involved in microtubule dynamics and has been shown to be a response predictive marker for lisavanbulin in preclinical studies
- Strong EB1 staining was observed in a patient with an exceptional response to daily oral lisavanbulin in 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 in GBM, which is anticipated to start in mid-2020

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

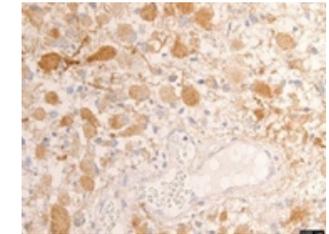
GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



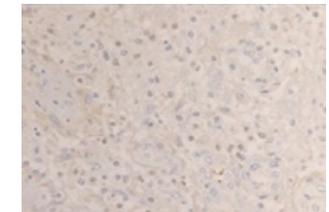
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



Non-responder



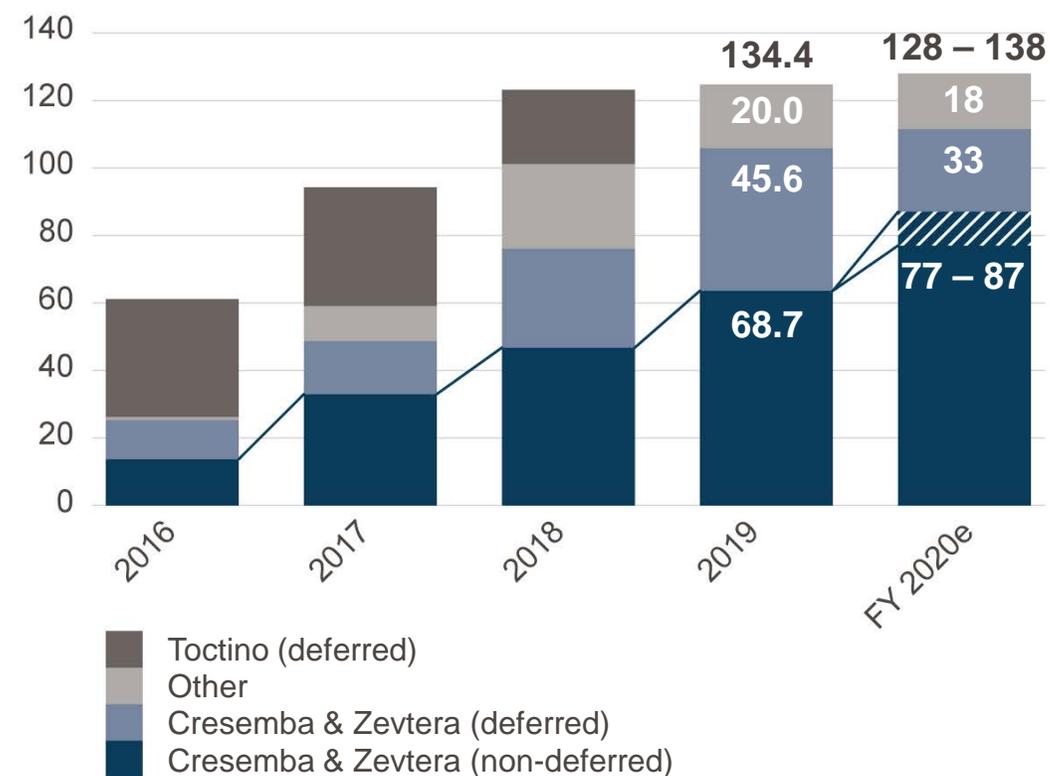
Financials



Financial guidance

In CHF mn	FY 2019 actuals	FY 2020 guidance
Total revenue	134.4	128-138
thereof: Contributions Cresemba® & Zevtera®		
non-deferred	68.7	77-87
deferred	45.6	33
Operating loss	17.2	20-30
Cash and financial investments	161.0	100-110

Strong increase in non-deferred revenue contributions
Y-o-Y, CHF mn



Outlook 2020 / 2021

Cresemba® & Zevtera® — Increasing cash flows
By the end of 2021, Cresemba to be on the market in 60 countries

	H1 2020	H2 2020	H1 2021	H2 2021
Isavuconazole		Complete patient enrolment in phase 3 study in Japan		Topline results from phase 3 study in Japan
Ceftobiprole			Complete patient enrolment in SAB phase 3 study	Topline results from SAB phase 3 study
Derazantinib	FIDES-01 (iCCA)	Complete patient enrolment in phase 2 registrational study (FGFR2 fusions)		Topline results (FGFR2 fusions)
		Interim data (other FGFR2 gene aberrations)		
	FIDES-02 (urothelial cancer)	Safety data and recommended phase 2 dose (RP2D) for derazantinib/Tecentriq combination and expansion into phase 2	Interim efficacy results in derazantinib Monotherapy	Interim efficacy results in combination therapy with Tecentriq
	FIDES-03 (gastric cancer)	✓ Clinical supply agreement with Roche in gastric cancer	Start of phase 1/2 study	Interim efficacy data
Lisavanbulin (Oral)		Full results of phase 1 study in glioblastoma	Interim data from phase 2 biomarker-driven glioblastoma study	Topline results from phase 2 biomarker-driven glioblastoma study
			Complete patient enrolment in phase 1 study in newly diagnosed glioblastoma	Interim data from phase 1 study in newly diagnosed glioblastoma

Disclaimer and forward-looking statements

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Focused on Growth and Innovation

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