




Focused on Growth and Innovation

Investor presentation
June 2020





“Patients are at the heart
of what we do”

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Executive summary



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

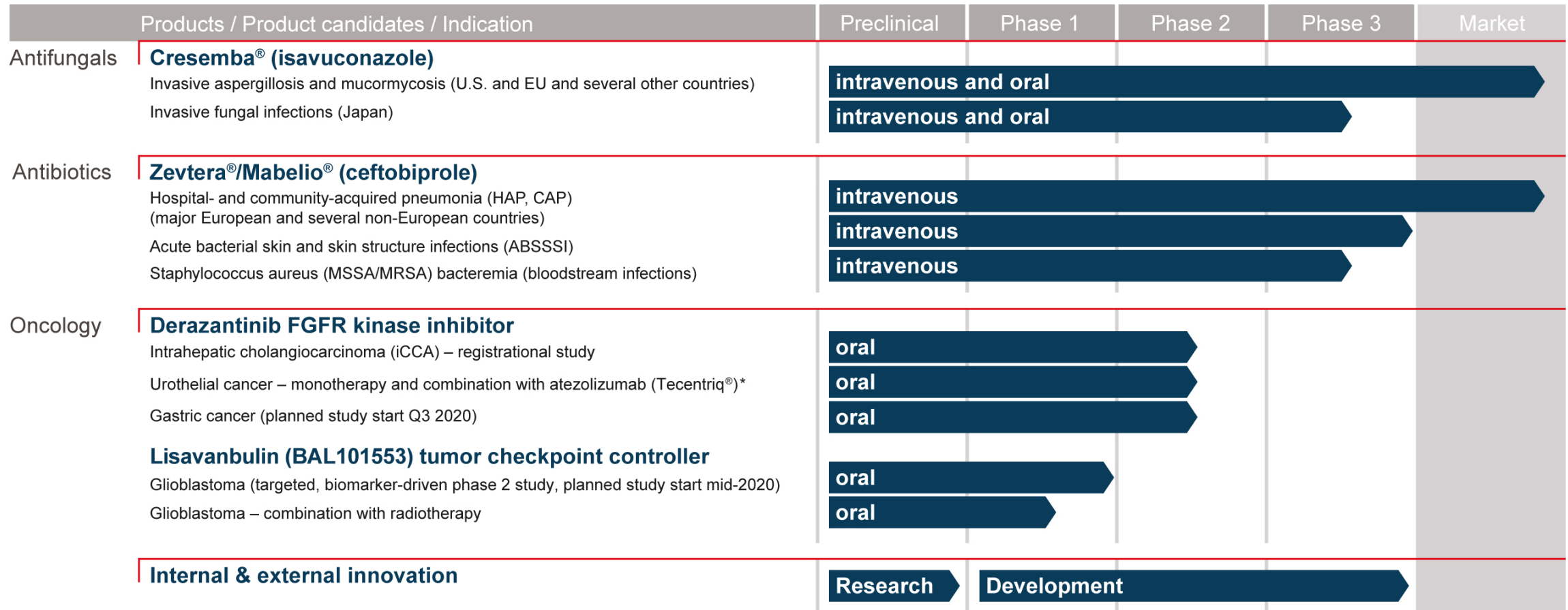


At a glance

- Well funded, commercial-stage biotech company with significantly growing cash 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 two clinical oncology drug candidates
- 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



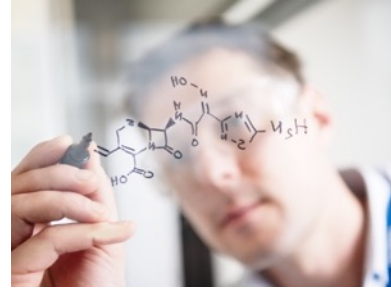
* Tecentriq® is a registered trademark of Hoffmann-La Roche Ltd.

Our strategy



Foster

Foster an agile organisation based on a dynamic and open culture



Focus

Focus on continuously increasing 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

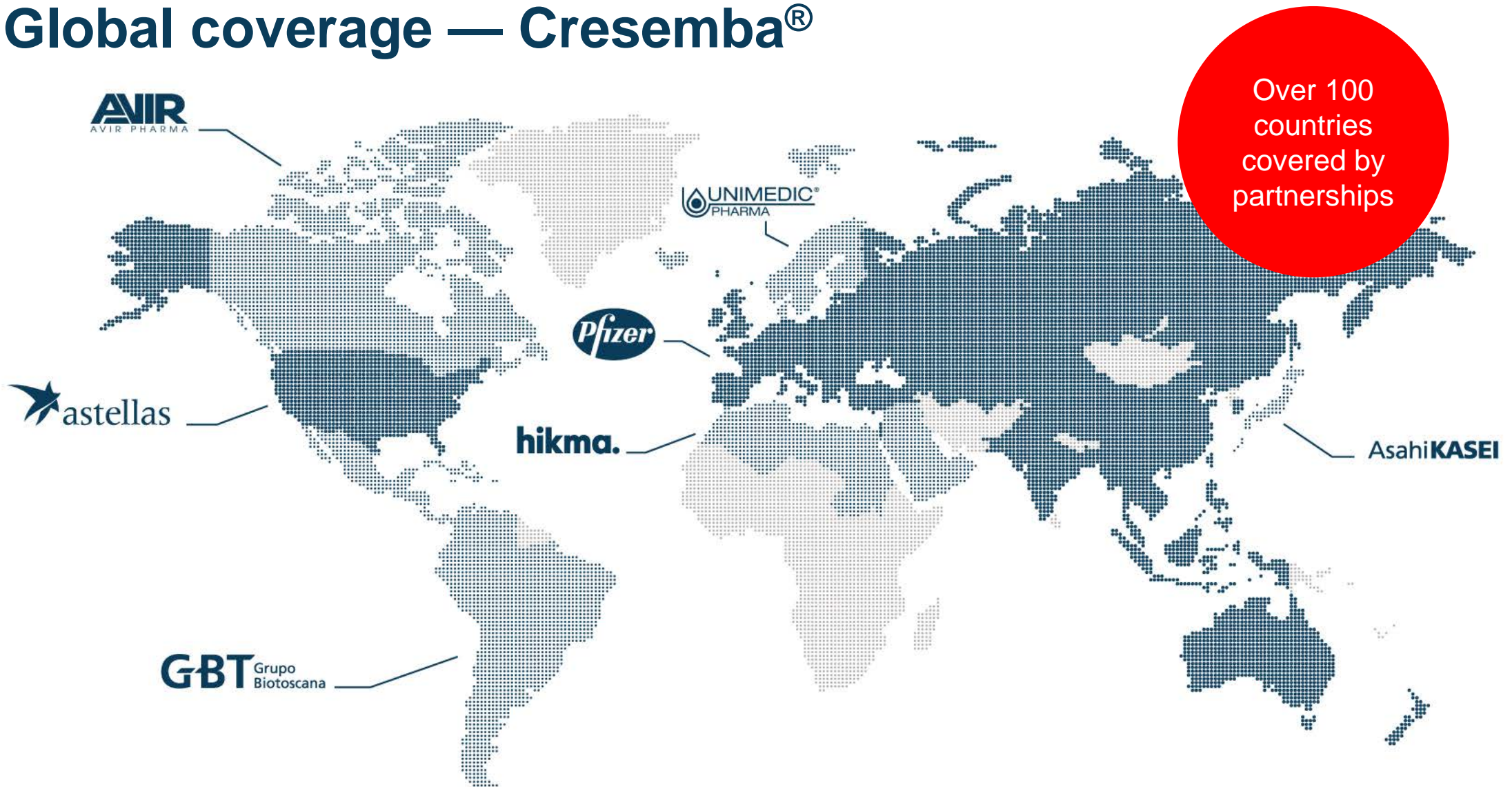
Invest in our clinical portfolio of targeted, small molecule, oncology drug candidates and the phase 3 ceftobiprole program



Innovate

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

Global coverage — Cresemba®



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®)

Double-digit
percentage
royalties on
sales by
license
partners

USD 1.1 bn
in potential
milestones
remaining

Participation
in sales of
distribution
partners
through
transfer price

USD ~250 mn
upfront and
milestone
payments
received



**Five reasons
to invest**



Five reasons to invest



Growth

Well funded with increasing and sustainable cash flow 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)

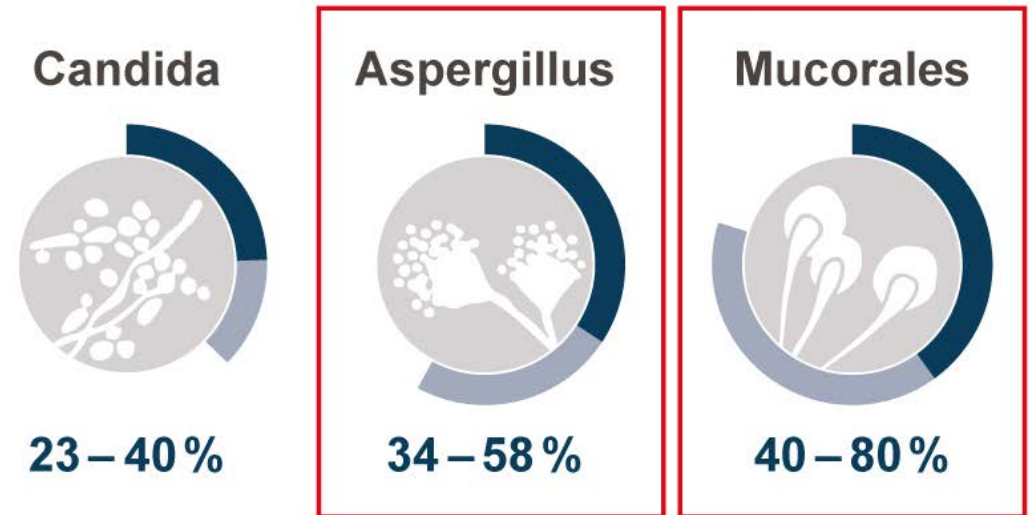
Invasive mold infections



The market — Invasive fungal infections

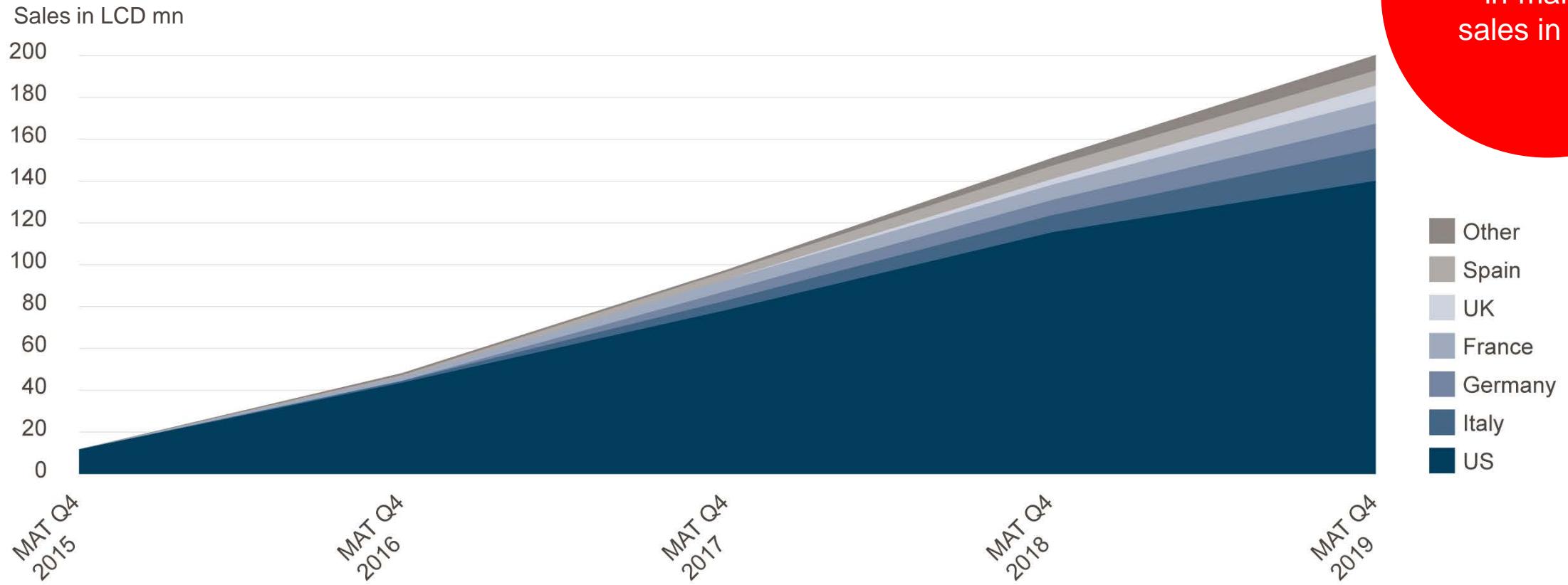
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand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



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

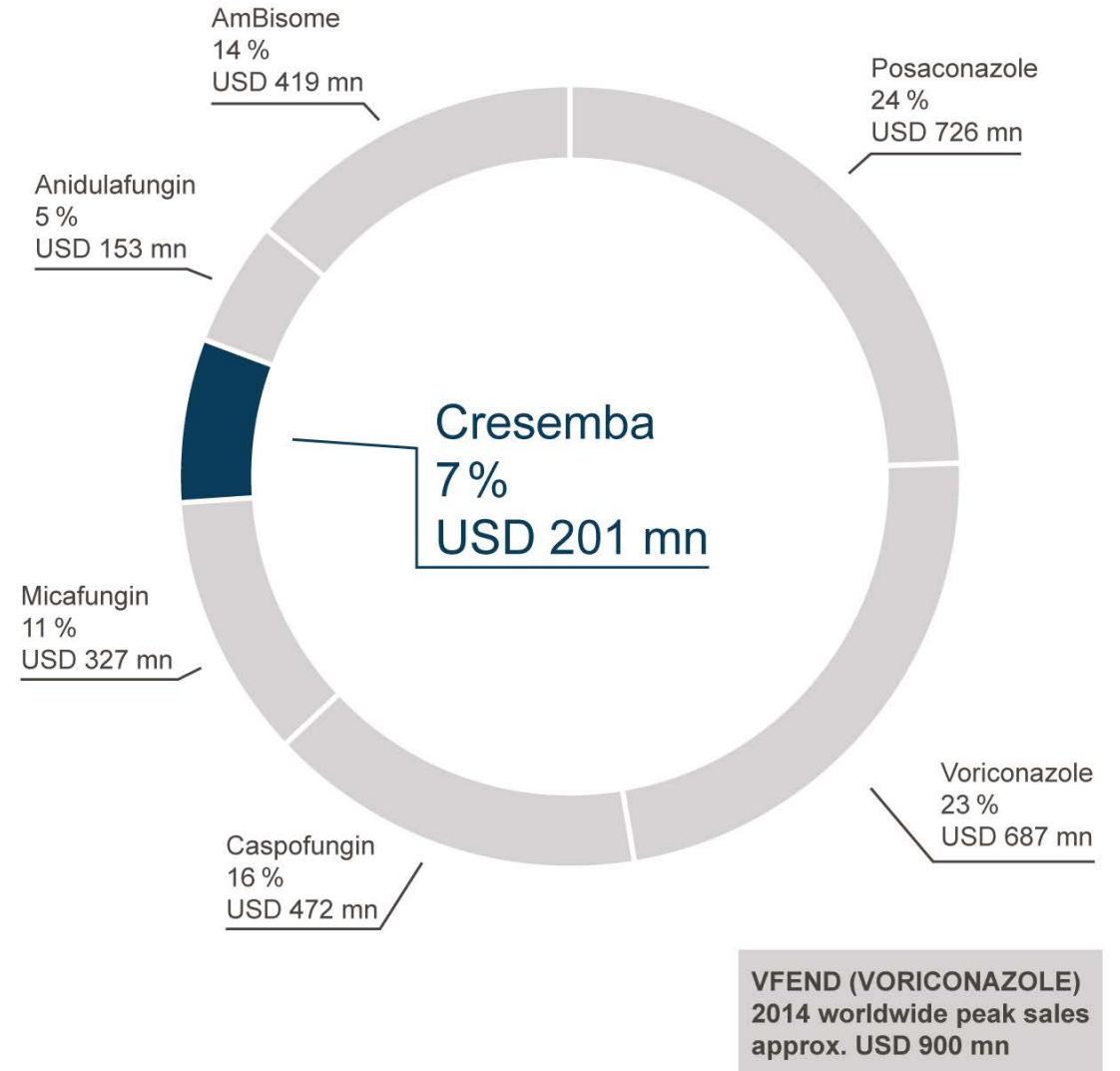
LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, Dec. 2019

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

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 without restriction 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)

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

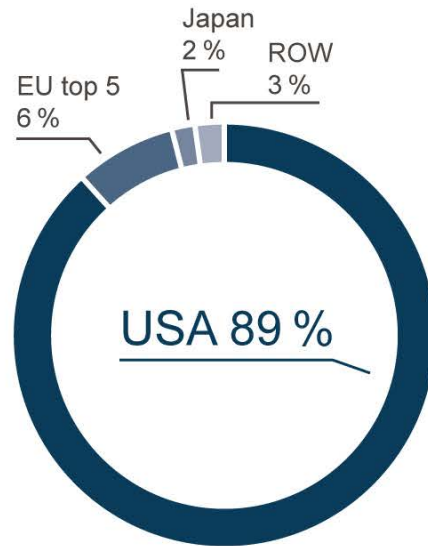
Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-associated pneumonia (VAP), and community-acquired pneumonia (CAP). Not approved in the U.S.

MENA: Middle East and North Africa

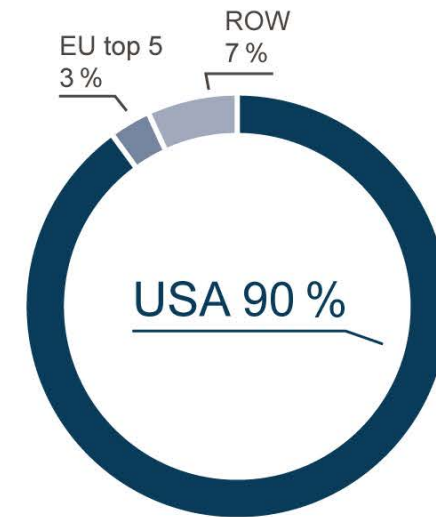


The hospital anti-MRSA antibiotic market — A USD 3 bn market* with the U.S. being the most important region

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, topline results from phase 3 study expected in Q1 2022



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

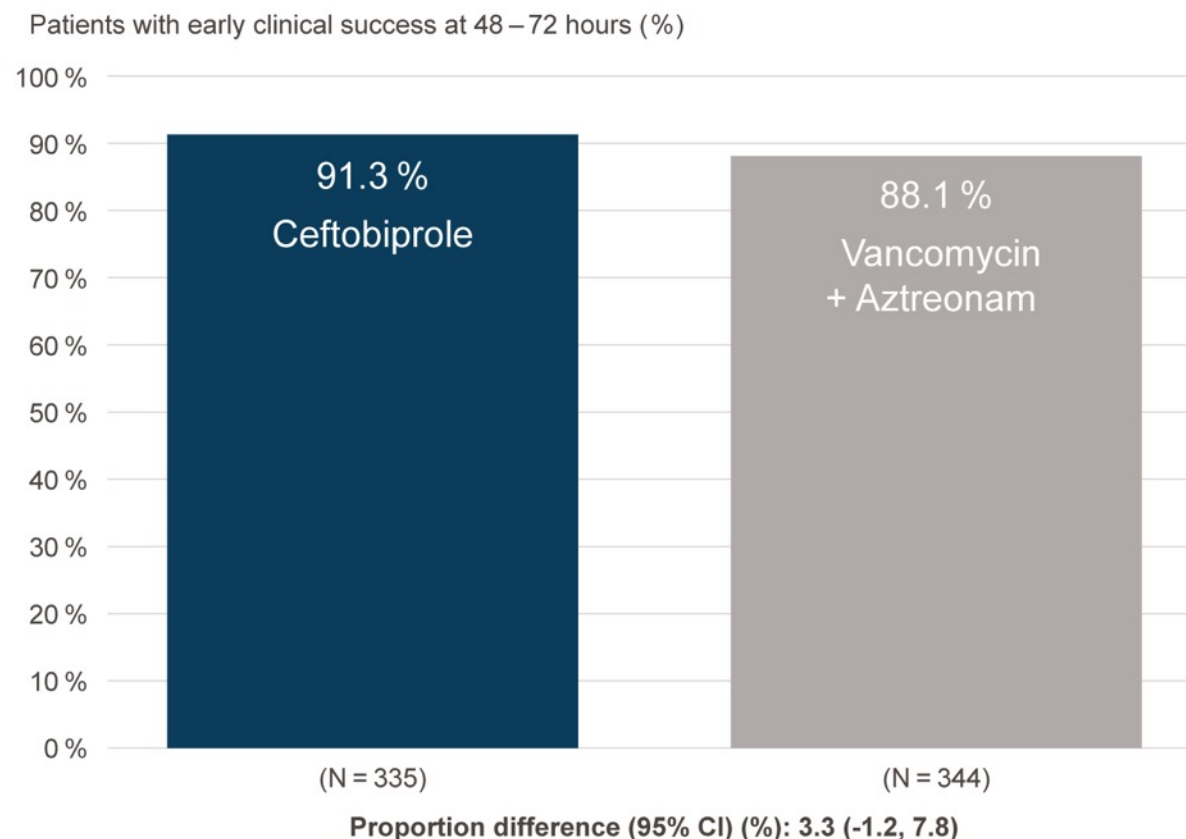
Ceftobiprole — Positive topline phase 3 results reported in ABSSSI

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

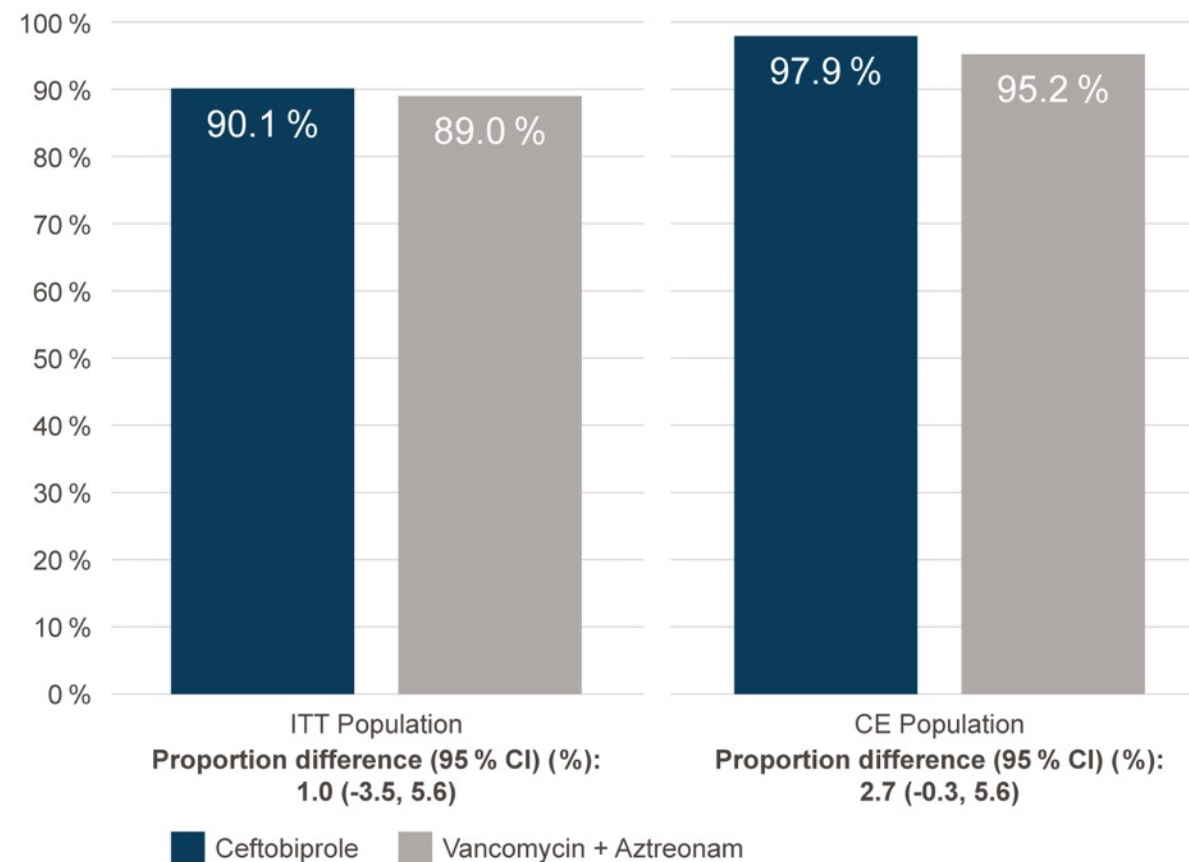
Key topline study¹ results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints




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

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.

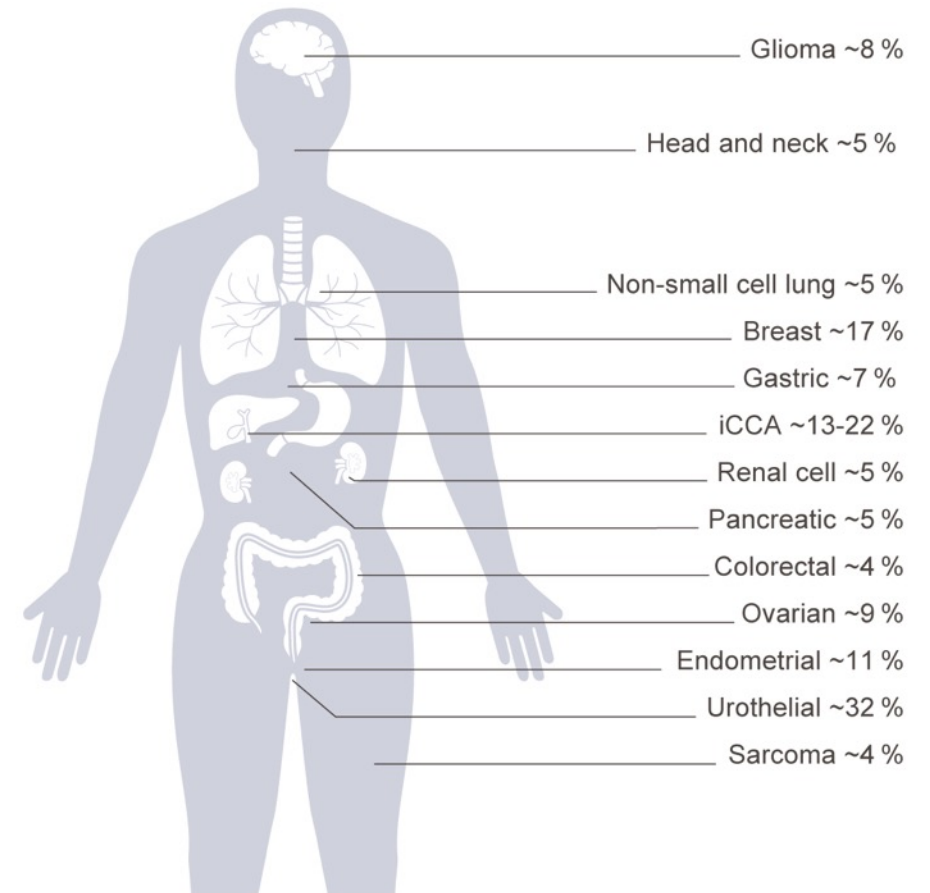
Oncology

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

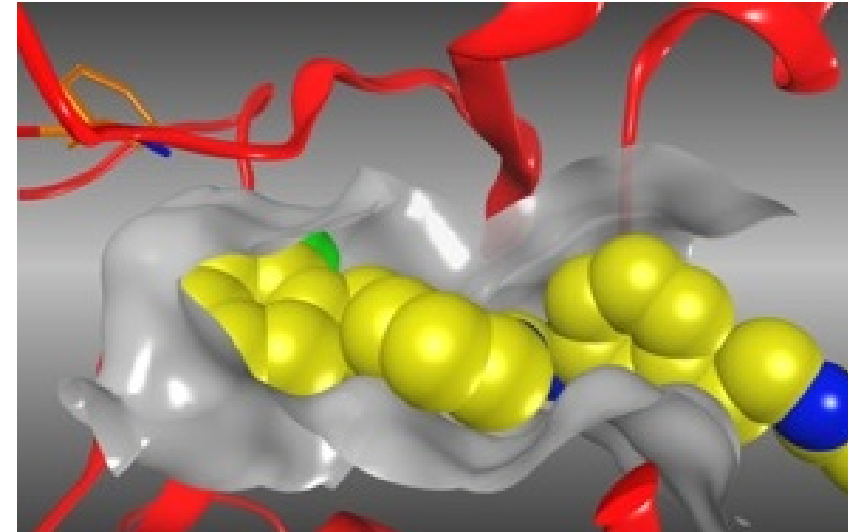
| FGFR-inhibitor compound (Sponsor) | Parameter | FGFR1 | FGFR2 | FGFR3 | FGFR4 | CSF1R | VEGFR2 |
|-----------------------------------|-------------------------|-------|-------|-------|-------|-------|--------|
| Derazantinib (Basilea) | Ratio to FGFR2 activity | 4 | 1 | 4 | 77 | 3 | 6 |
| Pemigatinib (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

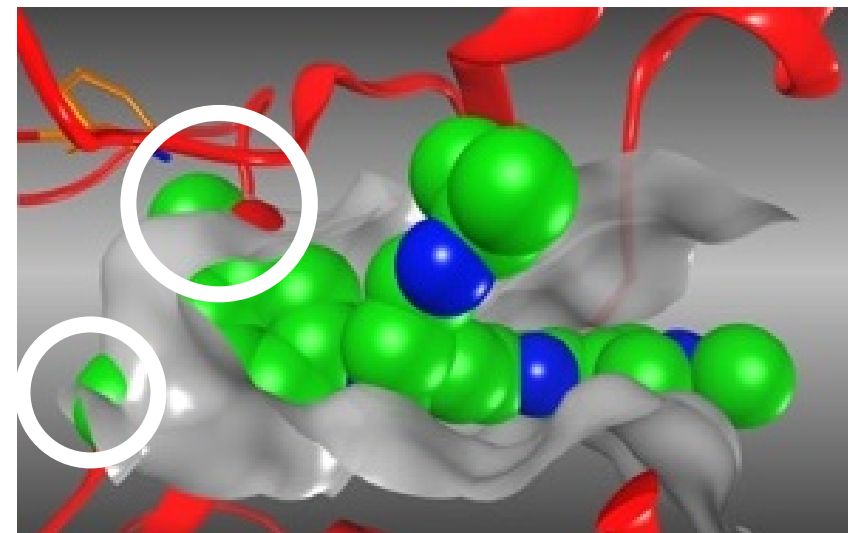
In-silico analysis of derazantinib binding to CSF1R

- Crystal structures 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 derazantinib¹

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



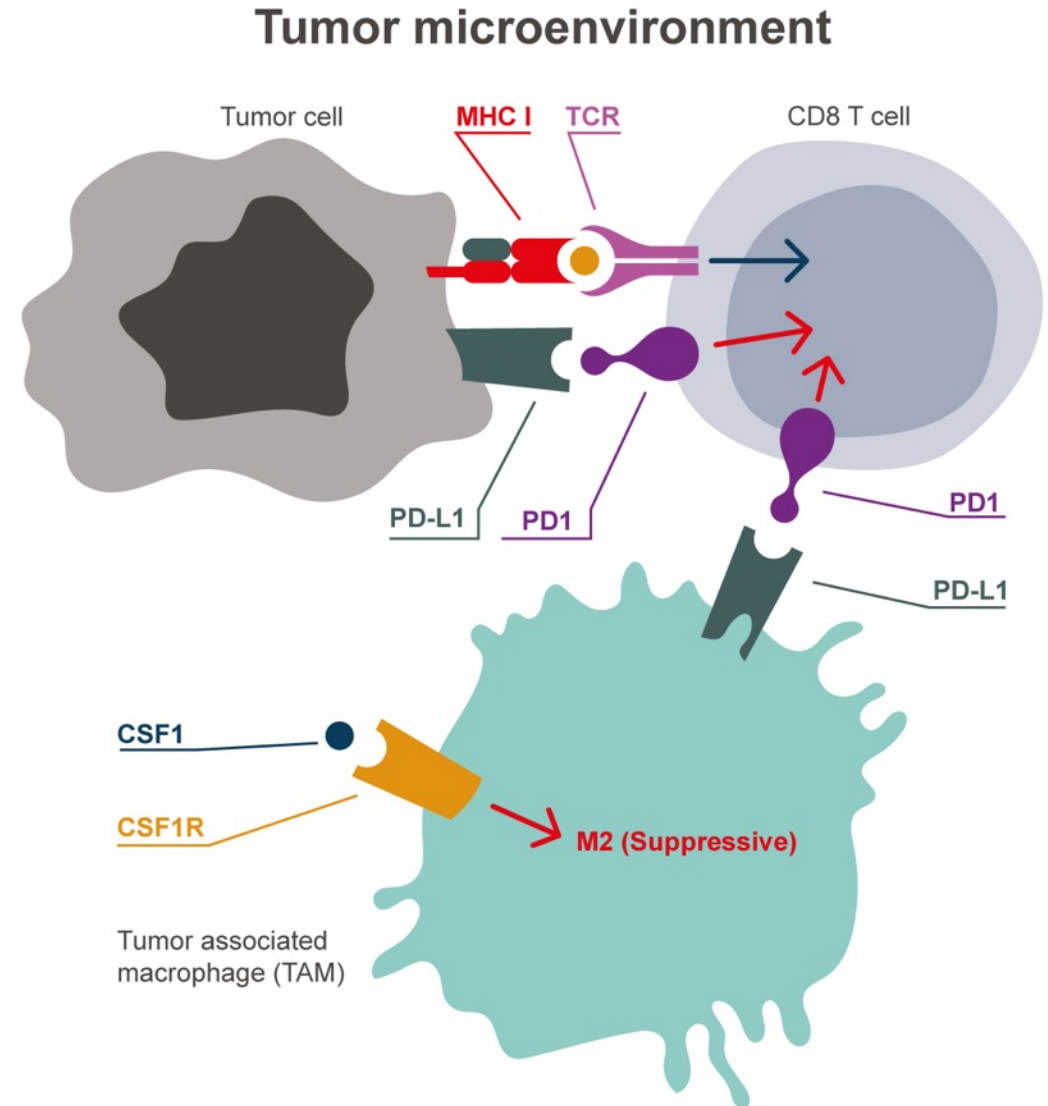
Derazantinib (yellow) fits to smaller active site pocket of CSF1R (grey/red)



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

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



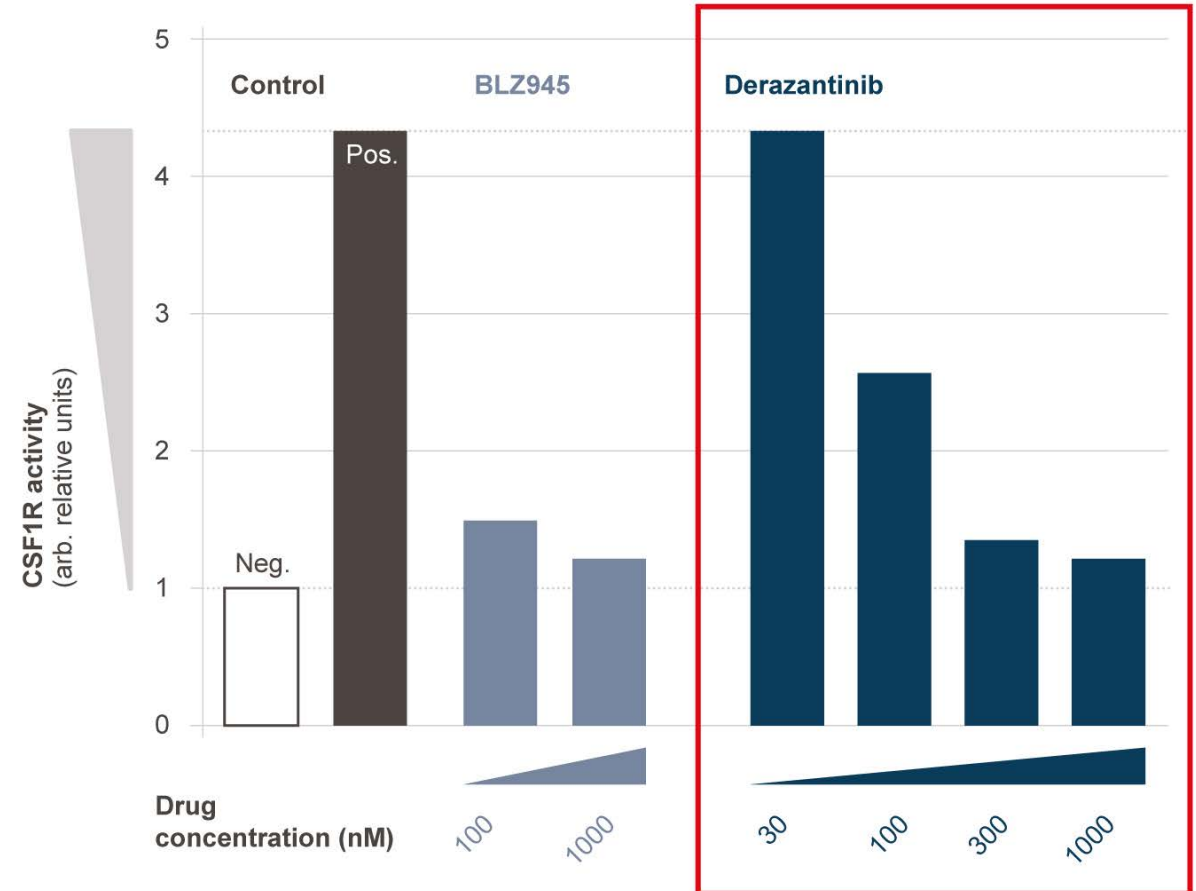
¹ X. Zheng et al. Redirecting tumor-associated macrophages to become tumoricidal effectors as a novel strategy for cancer therapy. *Oncotarget*. 2017;8(29):48436-48452

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

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 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=67) | PEM ⁴ (N=146) | PEM ⁵ (N=108) | ERD ⁶ (N=87) |
| Dosing regimen | 300mg QD | 125mg Q4W QD for 3w | 20 mg QD | 13.5mg Q3W QD for 2w | 13.5mg Q3W QD for 2w | 8 mg QD (titration to 9mg) |
| Most frequent safety events | Phosphorus↑ Nausea Vomiting | Phosphorus↑ Fatigue Stomatitis | Phosphorus*↑ Diarrhea* Dry mouth* | Phosphorus↑ Alopecia Diarrhoea | Diarrhoea Alopecia Constipation | Phosphorus↑ Stomatitis Fatigue |
| Blood phosphorus↑† | 59% | 73% | 88% | 60% | 31% | 76% |
| Fatigue† | 43% | 49% | NR | 42% | 32% | 54%# |
| Alopecia† | 20% | 38% | NR | 49% | 40% | 26% |
| Dry eye/xerophthalmia† | 16% | 32% | NR | 35%# | NR | 28%# |
| Retinopathy¶ | 0% | NR | 9% | 6%‡ | NR | 25% |
| Alanine aminotransferase (ALT) ↑ | 30% | NR | NR | 43%** | NR | 41%** |
| Hand-foot syndrome/PPE | 0% | 27% | 18% | 15% | NR | 26% |
| Nail toxicities | <5% | NR | 42% | 43%# | NR | 41%# |
| Stomatitis | 11% | 45% | NR | 35% | 34% | 56% |

Sources: ¹ Droz Dit Busset et al., ESMO 2019 and Basilea data on file; ² Javle et al., ESMO 2018; ³ Goyal et al., ASCO 2020; ⁴ Pemazyre™ U.S. Prescribing Information (April 2020);

⁵ Necchi, et al., ESMO 2018; ⁶ Balversa™ U.S. prescribing information (April 2019).

† assumed FGFR inhibitor class-effect; *futibatinib treatment-related adverse events

includes various and different adverse reactions; for details see Pemazyre™ U.S. Prescribing Information (April 2020) and Balversa™ U.S. prescribing information (April 2019);

¶ Refers to reported adverse events of Retinal Pigment Epithelial Detachment (RPED) for pemigatinib, Central Serous Retinopathy (CSR)/RPED for erdafitinib and CSR for futibatinib

‡ reported incidence is from 466 patients who received Pemazyre™ across clinical trials;

** based on reported laboratory abnormalities, regardless of causality.

Abbreviations: DZB: derazantinib, INF: infigratinib (BJG398), FUT: futibatinib (TAS-120), PEM: pemigatinib (INCB54828), ERD: erdafitinib; PPE: Palmar-plantar erythrodysesthesia; NR: not reported; QD: daily; Q3W/Q4W: every 3/4 weeks; w: weeks.

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

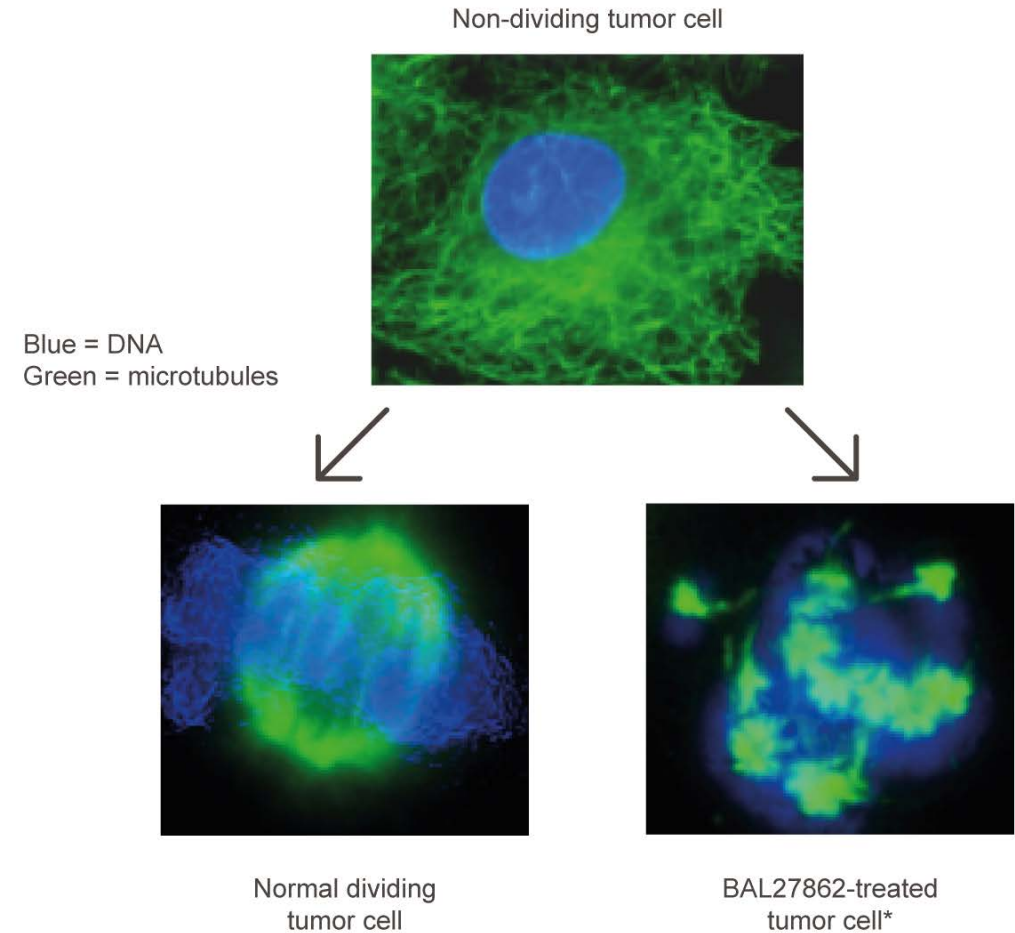
Lisavanbulin (BAL101553)

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

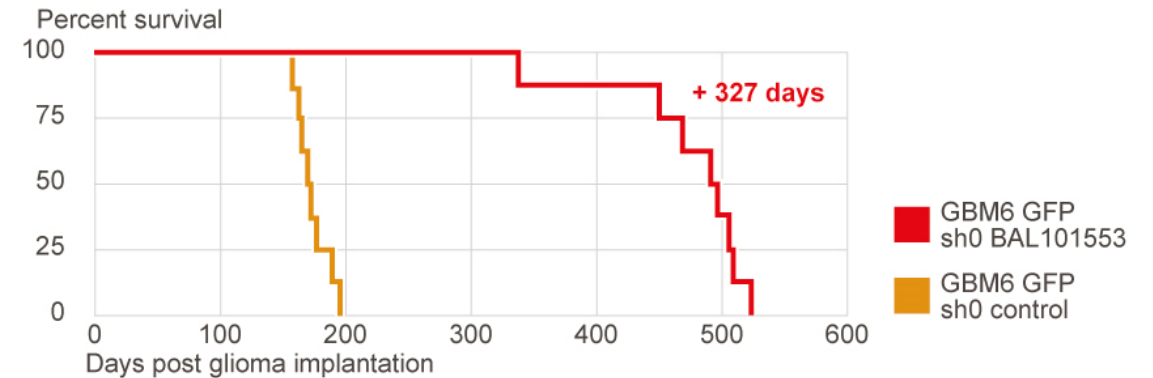
EB1 — A potential response-predictive clinical biomarker for lisavanbulin

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

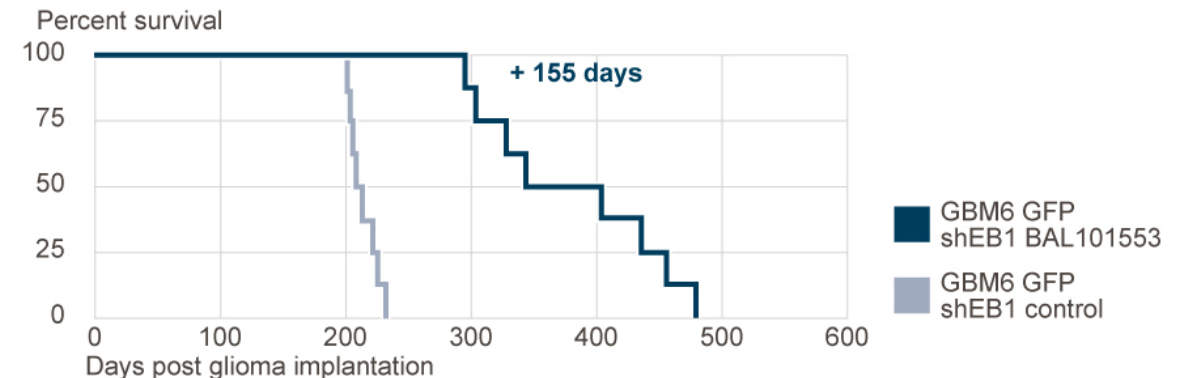
¹ Berges et al. EB1-dependent long survival of glioblastoma cancer stem-like cell tumor-bearing mice after oral treatment with the novel tubulin-binding checkpoint activator BAL101553. Eur. J. Cancer 2018, 103, E61-62, A166

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

EB1-expressing GBM



EB1-downregulated GBM

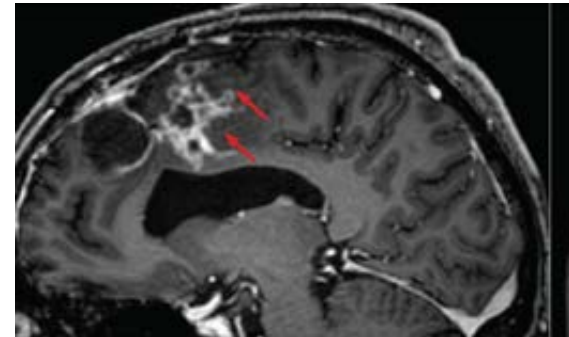


EB1 — A potential response-predictive clinical biomarker for lisavanbulin

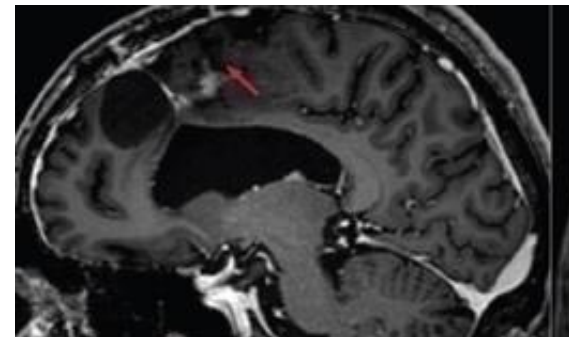
- EB1 (plus-end binding protein) is located on the microtubules and involved in microtubule dynamics and has been shown to be a response predictive marker for lisavanbulin in preclinical studies
- Strong EB1 staining was observed in a patient with an exceptional response to daily oral lisavanbulin in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

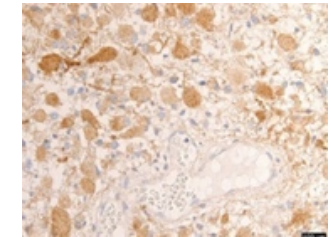
GBM tumor size reduction in an exceptional responder and EB1 staining of GBM tissue compared to non-responding patients



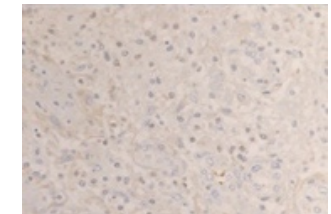
Baseline (May 2018)



Post Cycle 12 (April 2019)



Responder



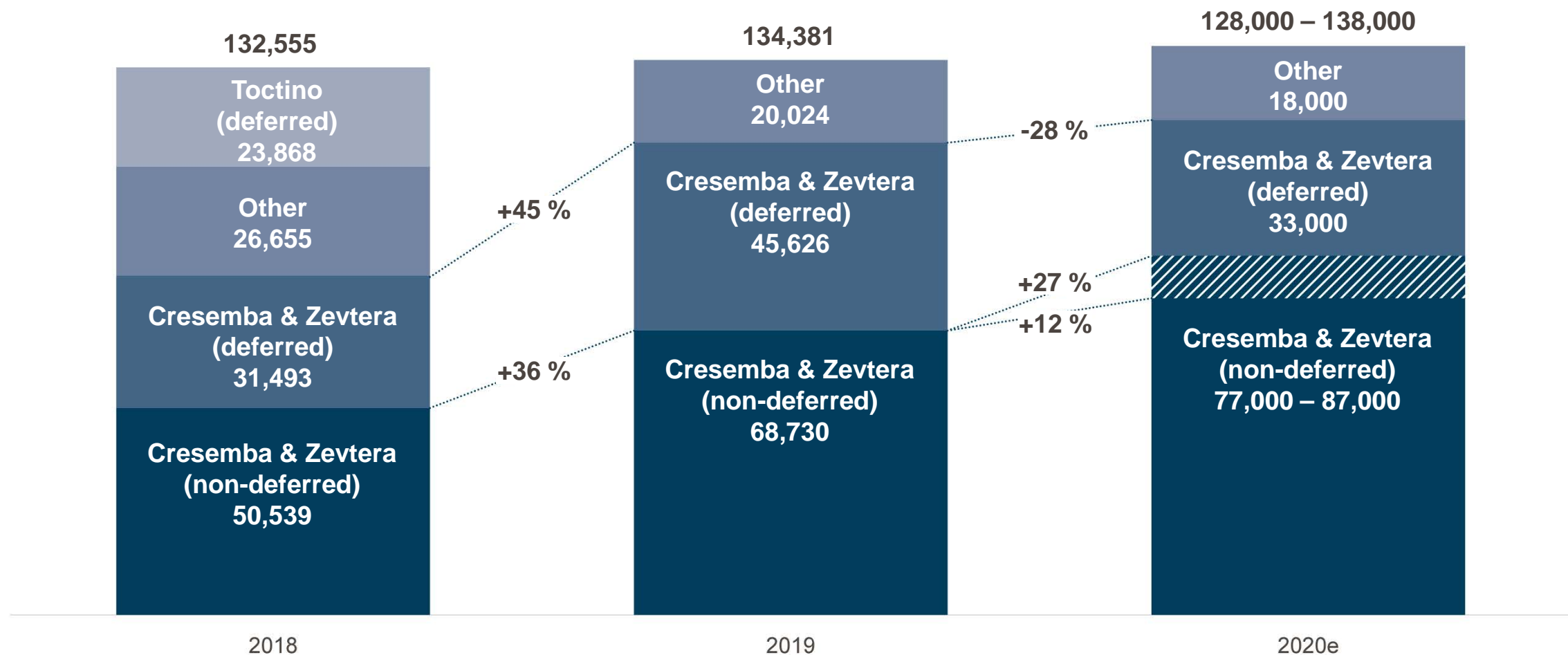
Non-responder



Financials

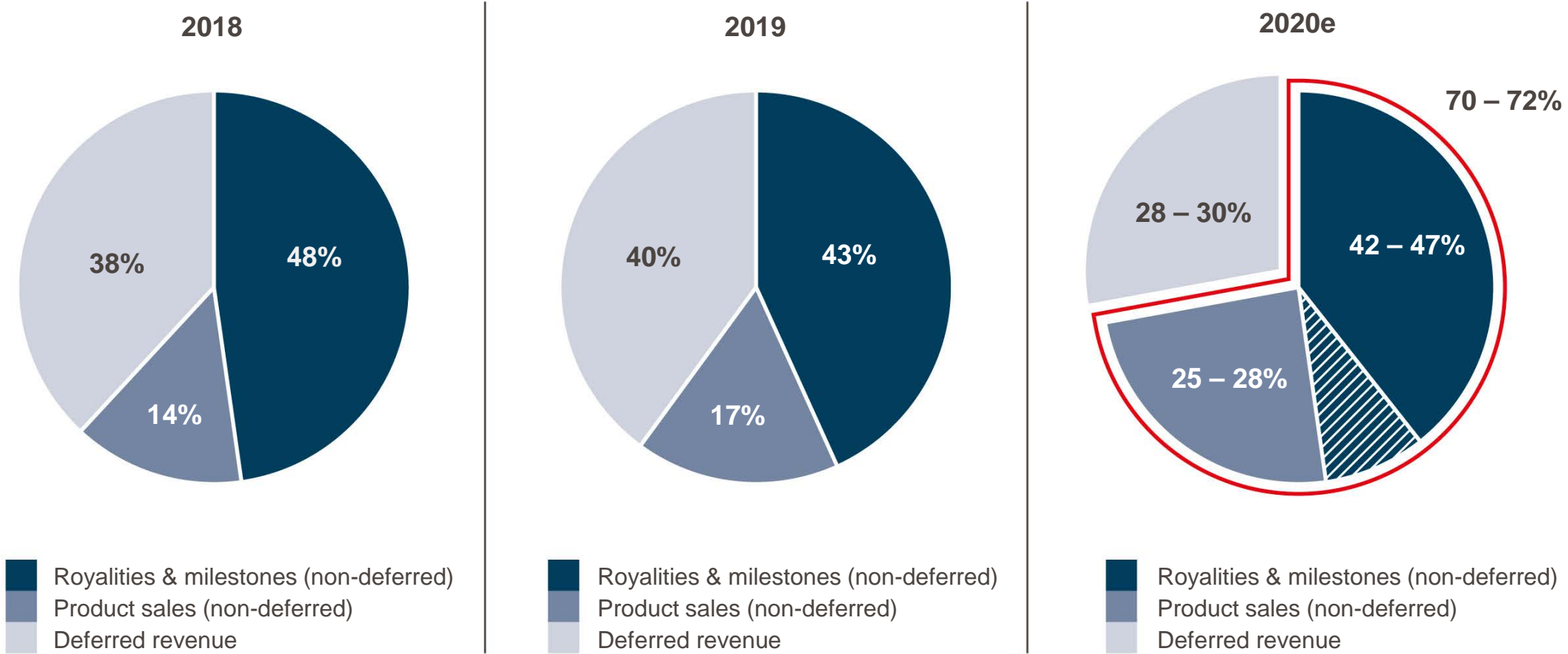


Continued double-digit growth in non-deferred Cresemba® & Zevtera®-related revenues (in CHF, thousands)



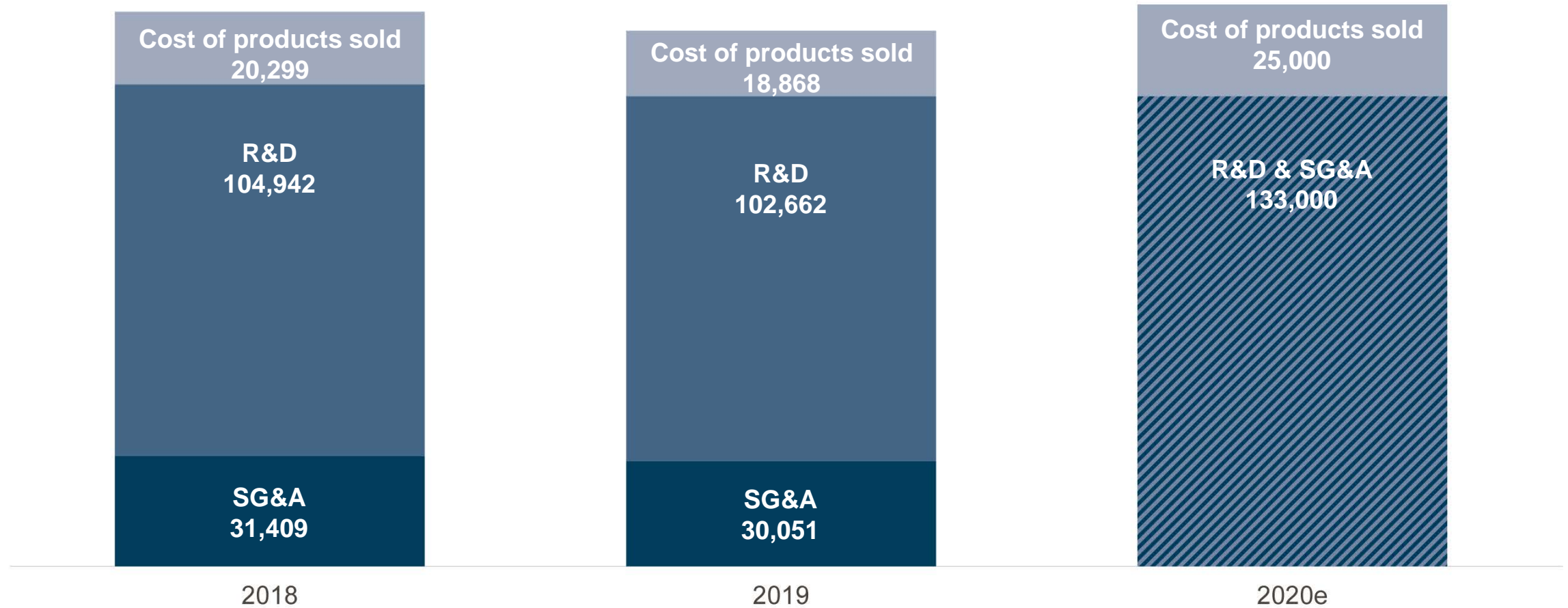
Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently

Cresemba® and Zevtera® -related revenue mix shifting towards non-deferred revenue



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

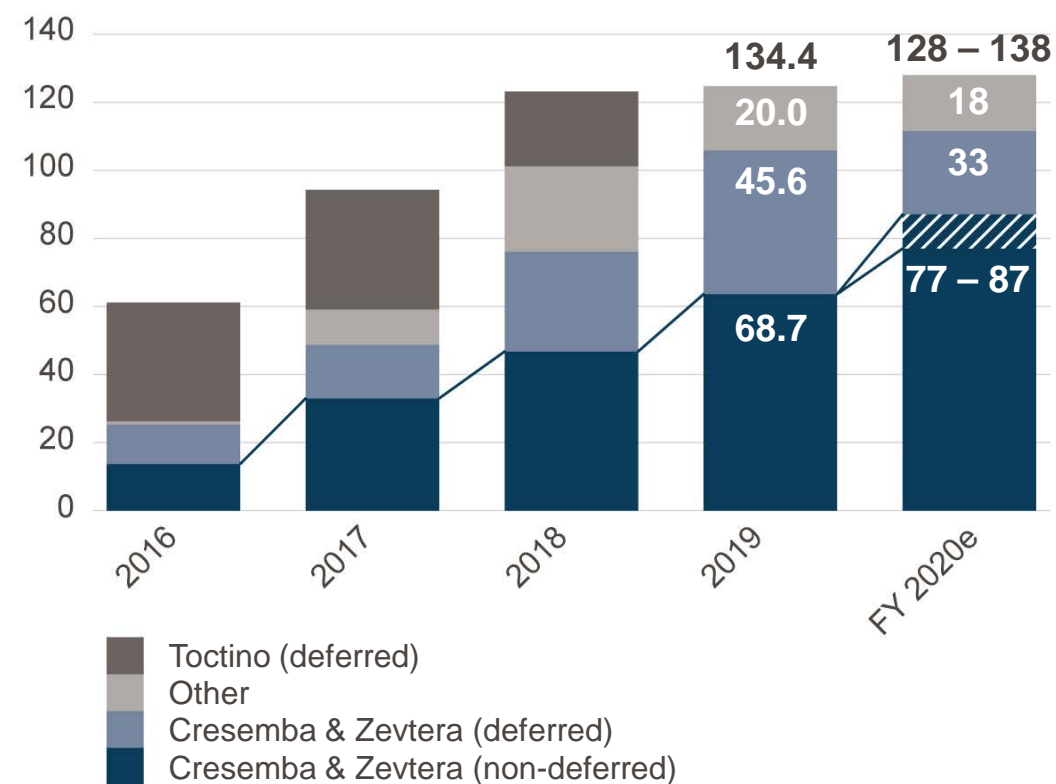
Flat R&D + SG&A expenses (in CHF, thousands)



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

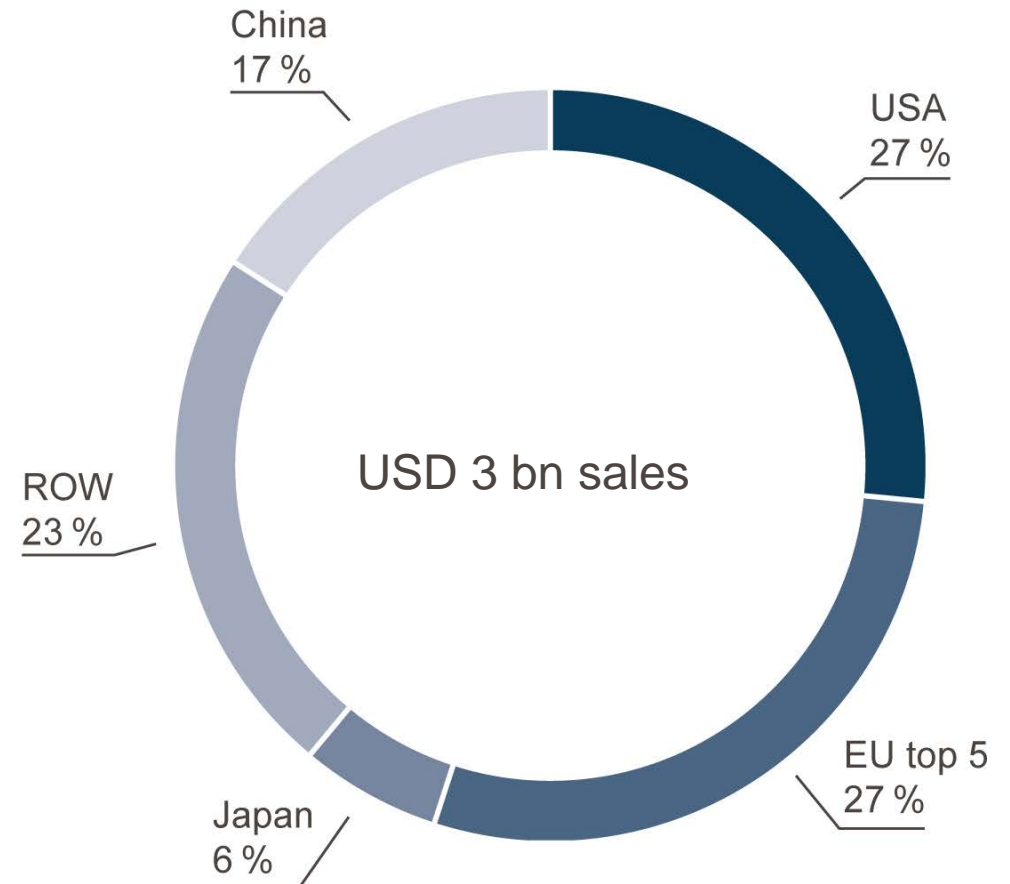
| | H1 2020 | H2 2020 | H1 2021 | H2 2021 |
|----------------------------|--|---|---|--|
| Isavuconazole | | Complete patient enrolment in phase 3 study in Japan | | Topline results from phase 3 study in Japan |
| Ceftobiprole | | | | Complete patient enrolment in SAB phase 3 study |
| Derazantinib | FIDES-01 (iCCA) | Complete patient enrolment in phase 2 registrational study (FGFR2 fusions) Topline results (FGFR2 fusions) | | |
| | | Interim data (other FGFR2 gene aberrations) | | Topline results (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 |
| | ✓ 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 | Start phase 2 biomarker-driven glioblastoma study | 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 | |

Appendix

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

USD 3 bn sales of best-in-class antifungals*
(MAT Q4 2019)

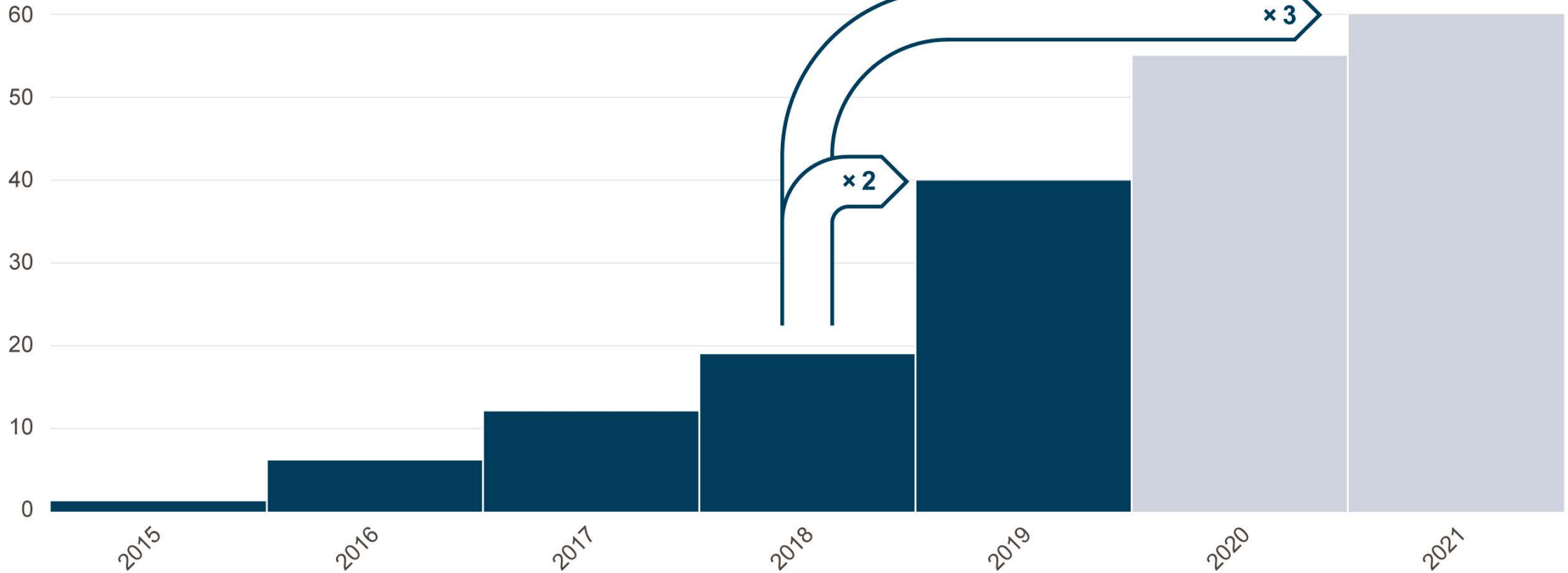
* Best-in-class antifungals: isavuconazole, posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin



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

Cresemba[®] — Strong global roll out

Number of launched countries at year-end



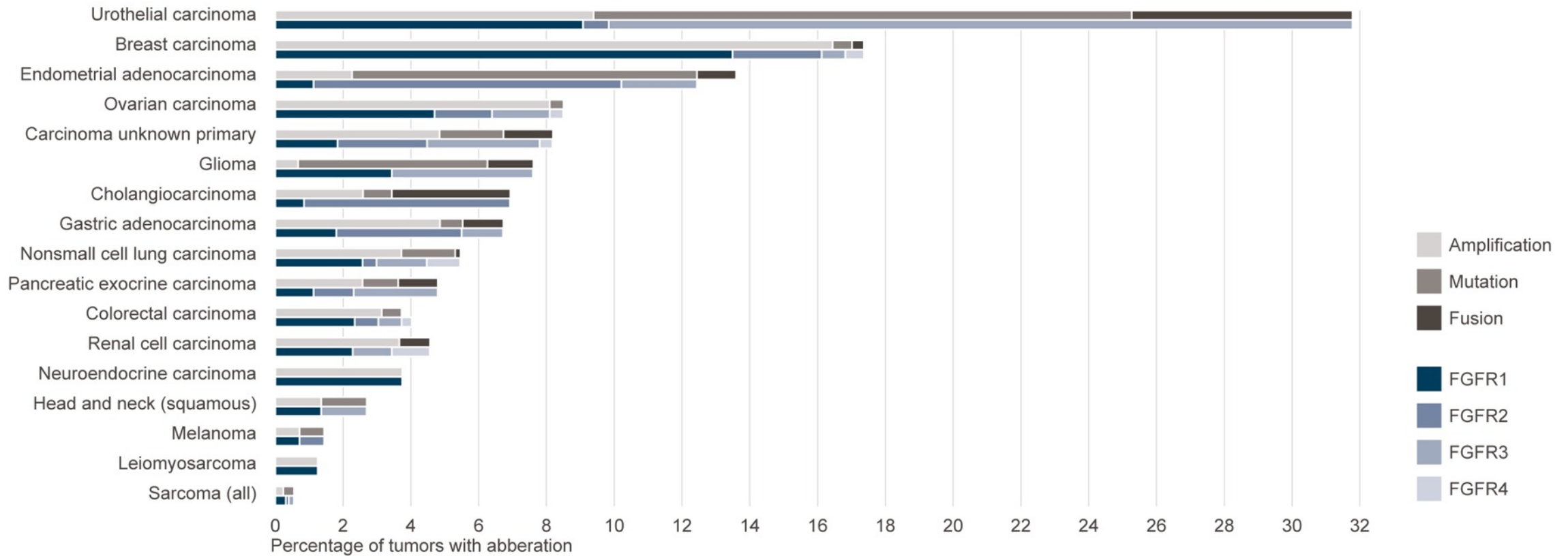
Phase 3 study with ceftobiprole in the treatment of patients with SAB



- **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

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

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

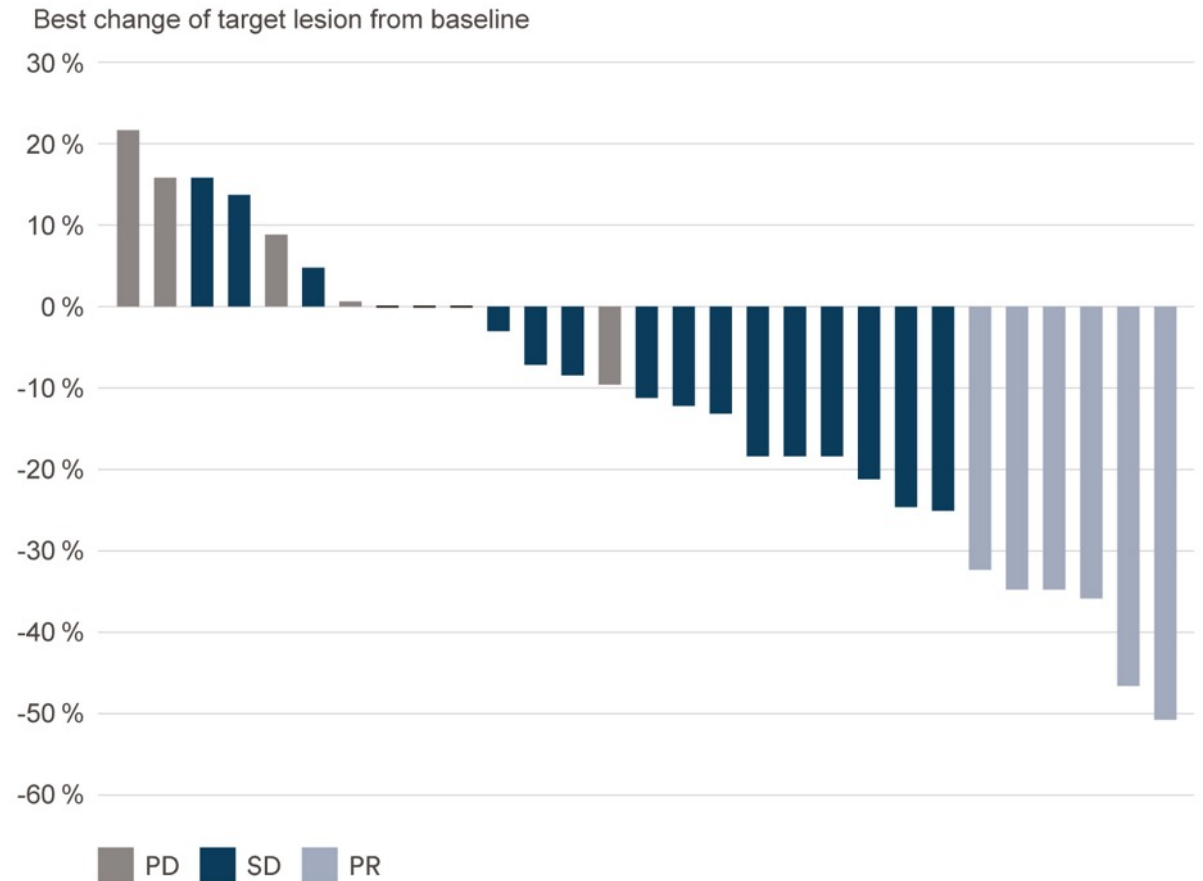
- Derazantinib as single agent and in combination with atezolizumab (Tecentriq[®]) in patients with advanced urothelial cancer testing positive for mutations or fusions of FGFR1, FGFR2 or FGFR3 genes
- The subgroup of patients with low PD-L1 expression have limited clinical benefit from the treatment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

Glossary

- ABSSSI: Acute bacterial skin and skin structure infections
- SAB: *Staphylococcus aureus* bacteremia
- FGFR: Fibroblast Growth Factor Receptor
- iCCA: Intrahepatic cholangiocarcinoma
- CSF1R: Colony-stimulating Factor 1 Receptor
- VEGFR2: Vascular Endothelial Growth Factor Receptor 2
- GBM: Glioblastoma multiforme

Disclaimer and forward-looking statements

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