

Full-year results 2018 presentation

February 19, 2019

Disclaimer and forward-looking statements

Forward looking statements

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Introduction

Key achievements

David Veitch
Chief Executive Officer

Key achievements in 2018

- Significantly **increased revenue contributions** from antifungal **Cresemba[®]** (isavuconazole) and antibiotic **Zevtera[®]/Mabelio[®]** (ceftobiprole)
- **Strengthened R&D pipeline** by in-licensing clinical oncology drug candidate **derazantinib** and **preclinical selective inhibitors** of a **kinase** involved in controlling cell division
- **Enrolling patients into both phase 3 studies** required for a New Drug Application for antibiotic **ceftobiprole** in the **important U.S. market**
- **Progressed** drug candidate **BAL101553** into phase 2a development, currently being evaluated in patients with **glioblastoma** and advanced **ovarian cancer**



Good start in 2019

- **Reported positive interim results** from **registrational phase 2 study** with **derazantinib** in intrahepatic cholangiocarcinoma (**iCCA**)
- **Collaboration** to explore **derazantinib** in **combination** with Roche's PD-L1-blocking **immune-checkpoint inhibitor** atezolizumab (Tecentriq[®]) in patients with **urothelial cancer**



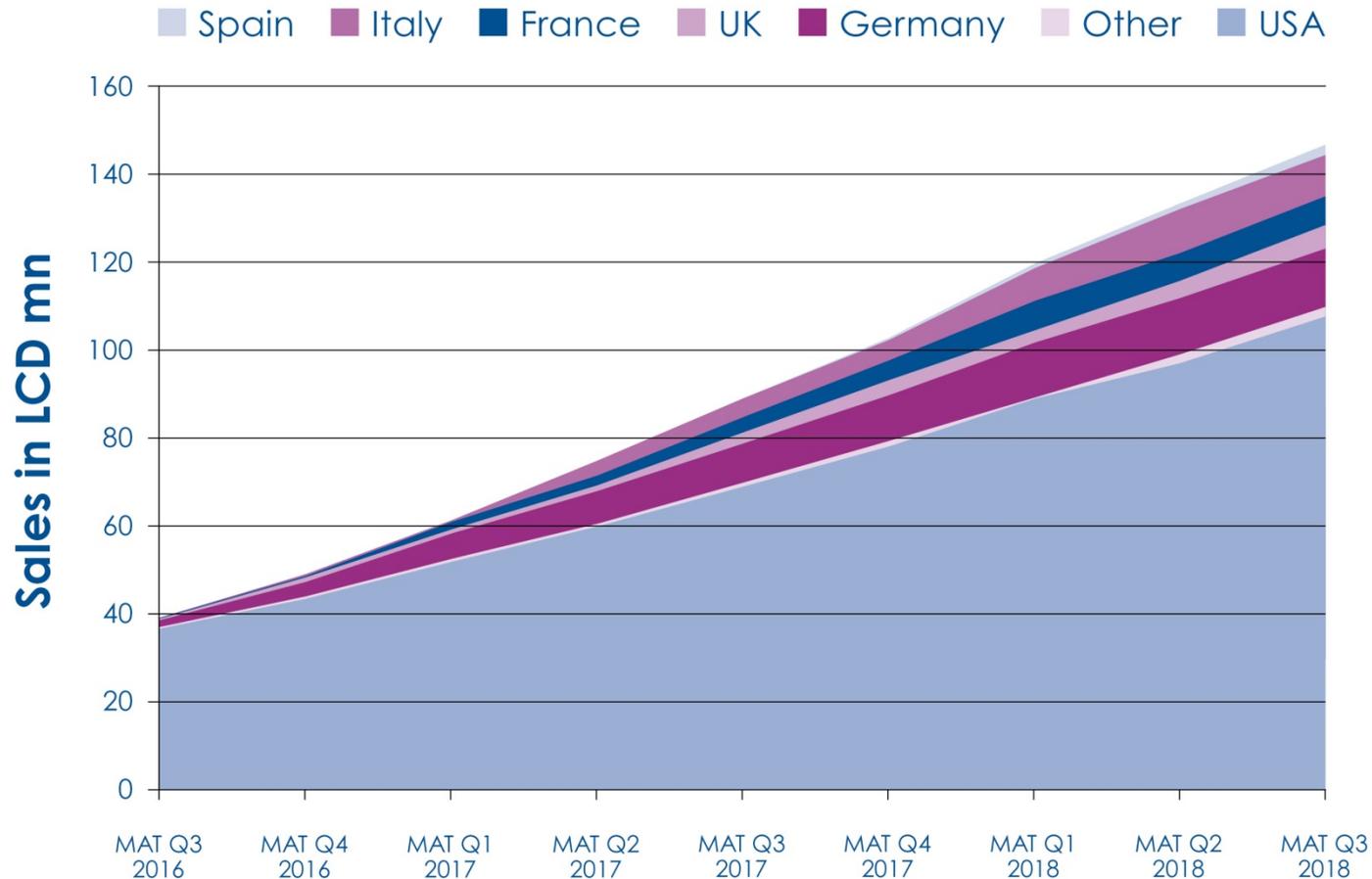


Commercial & partnering activities

Adesh Kaul
Chief Corporate
Development Officer

Cresemba continues strong sales uptake in established and new markets

USD 147 mn annual in-market sales by Q3 2018



LCD: USD corrected for currency fluctuations; MAT: Moving annual total; Source: IQVIA, September 2018

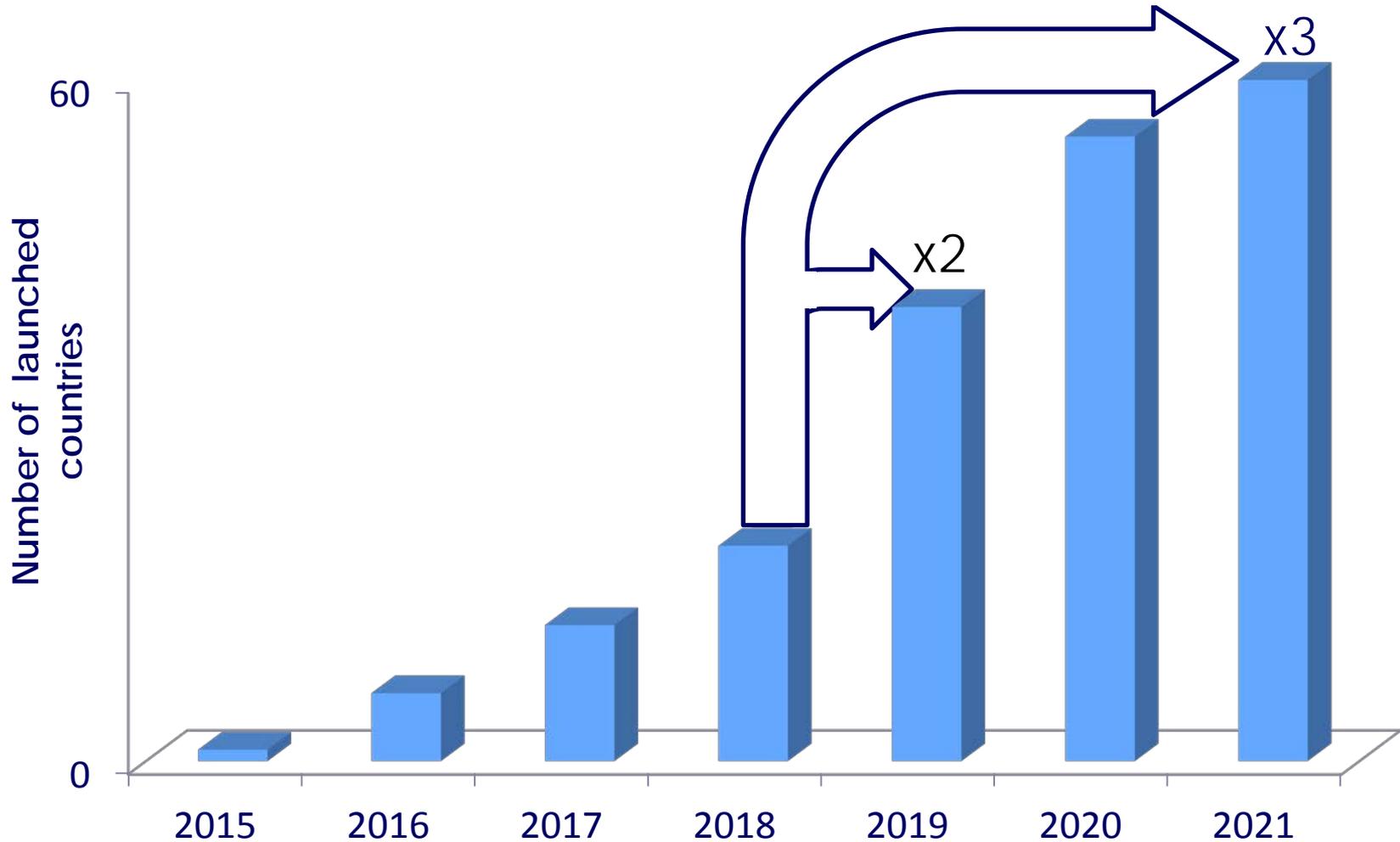


Milestones achieved

- **Pfizer:** USD 3mn upfront payment received
(Cresemba license extension to China and APAC)
- **Grupo Biotoscana:** CHF 2mn regulatory milestone payment received
(Cresemba approval in first Latin American market)
- Continued strong **Cresemba sales** triggered milestones
 - **Astellas:** CHF 10mn based on U.S. sales
 - **Pfizer:** USD 5mn based on European sales, January 2019



Cresemba – strong global roll out

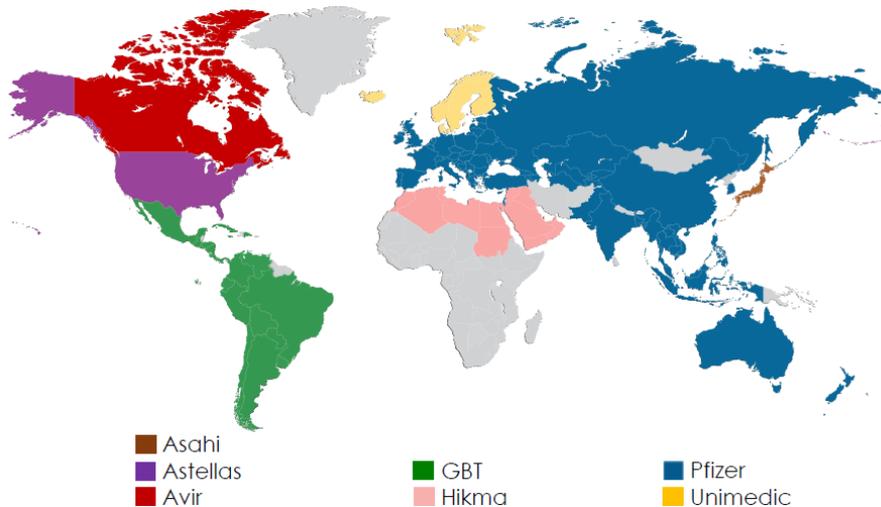


>100 countries covered by partnerships — USD 1.1bn in total potential milestones outstanding

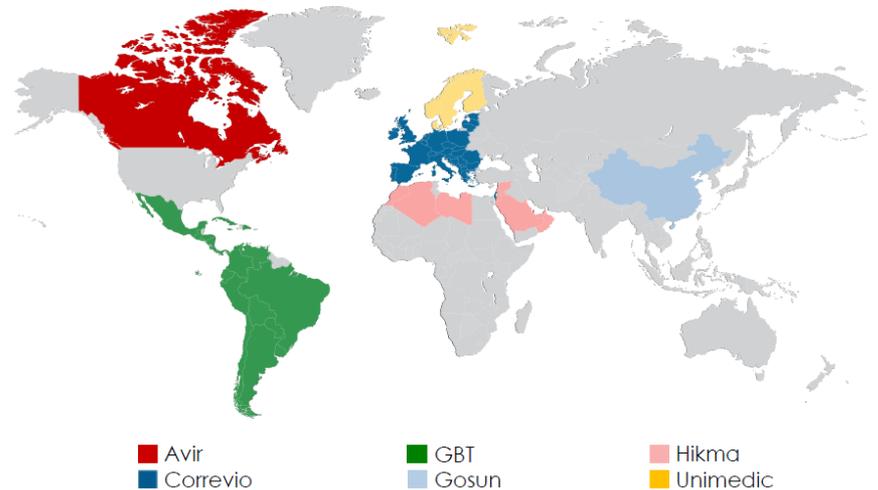
Ongoing participation

- Double-digit royalties on sales by license partners
- Participation through transfer price structure in sales by distribution partners
- Approximately USD 240mn upfront and milestone payments received; USD 1.1bn in potential milestones outstanding

Our Global Partnerships: Cresemba



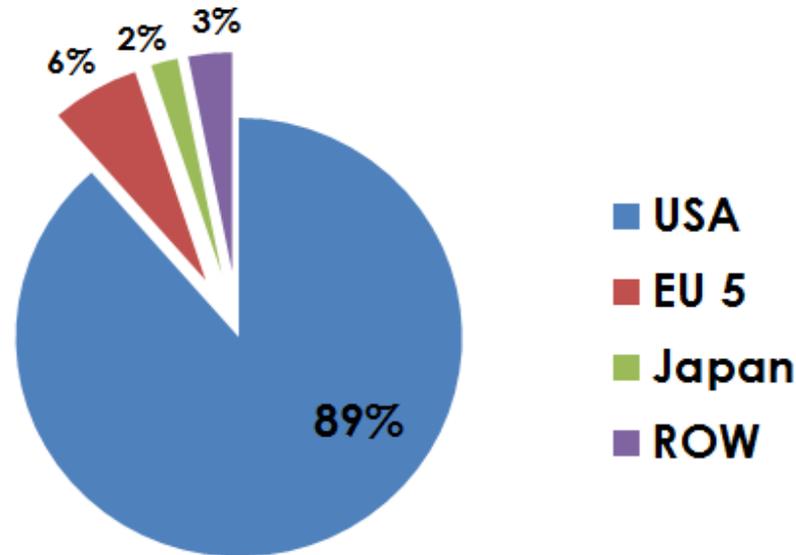
Our Global Partnerships: Zevtera



Anti-MRSA hospital antibiotics market — U.S. is the most important region

Global anti-MRSA hospital antibiotics sales*
USD 3.1bn (MAT Q3 2018)

Daptomycin sales by region 2015 (before LOE)



* 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, September 2018





Clinical development update

Dr. Marc Engelhardt
Chief Medical Officer

Enrolling patients into two phase 3 studies to gain U.S. regulatory approval for ceftobiprole

- Two cross-supportive studies, conducted under FDA Special Protocol Assessment



Acute Bacterial Skin and Skin Structure Infections
(anticipated to complete in H2 2019)



Staphylococcus aureus bacteremia
(anticipated to complete in H2 2021)

- 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



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 Inc.
 - panFGFR kinase inhibitor with strongest activity against FGFR1, 2 and 3
 - Exploring therapeutic potential of additional targets of derazantinib, including targets not addressed by other selective FGFR inhibitors, such as CSF1R (Colony-stimulating Factor 1 Receptor) kinase
- Strong data foundation generated to support potential accelerated FDA approval in intrahepatic cholangiocarcinoma (iCCA), an indication with high unmet need and globally increasing incidence
- Orphan drug designation in iCCA granted by FDA and EMA
- Collaboration with Roche to study derazantinib and immune-checkpoint inhibitor atezolizumab (Tecentriq®) in a clinical study in urothelial cancer



Derazantinib — potential for accelerated approval with solid clinical data in iCCA

Favorable clinical data from completed phase 1/2 study

- Promising anti-tumor efficacy and clinical safety shown in biomarker-driven clinical study in patients with FGFR2-gene-fusion expressing iCCA
- Derazantinib efficacy 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 and low discontinuation rate^{1,4}

Registrational phase 2 study, ongoing

- 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
- Final data to be presented mid-2020

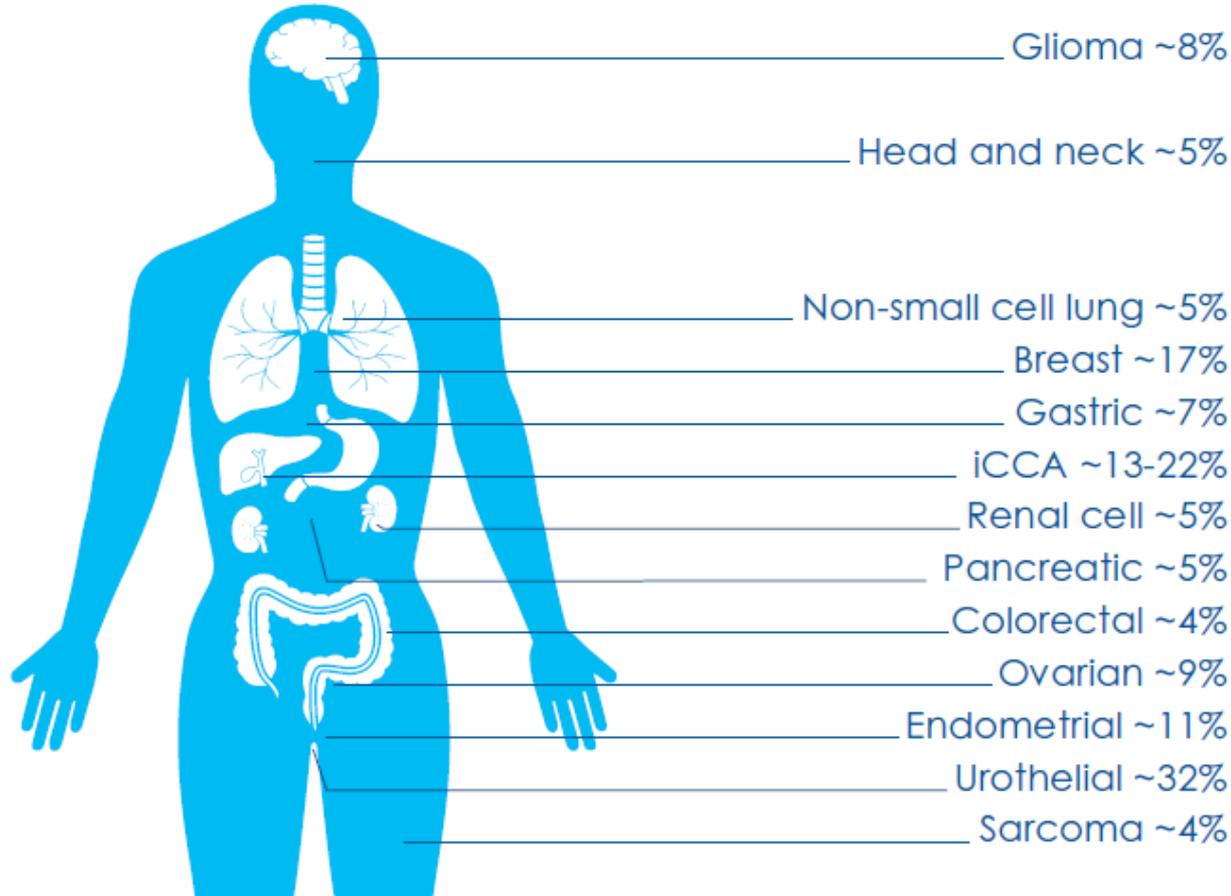
Sources:

- 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 tumours. *British Journal of Cancer* 2017 , 1-8



Derazantinib — Significant potential beyond iCCA and urothelial cancer

Frequency of FGFR aberrations across different tumor types

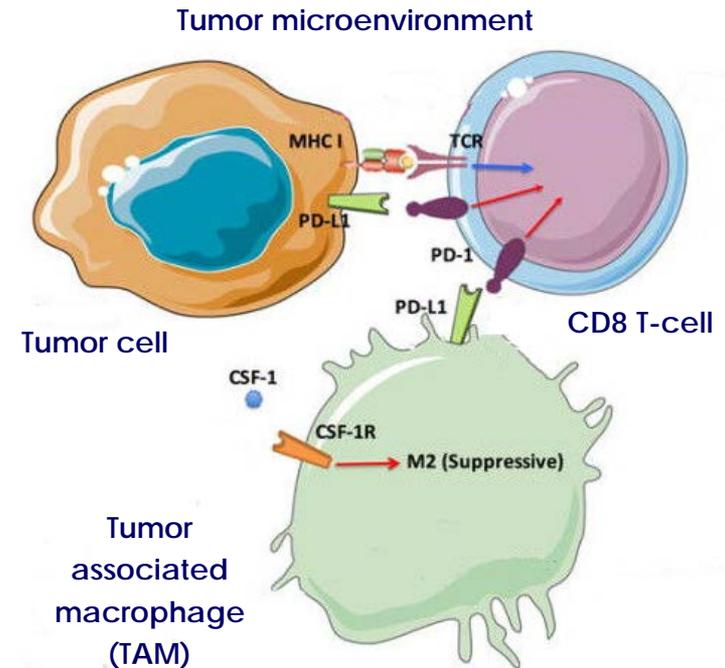


Source:
Helsten et al., Clin Cancer Res 2016 (22), 257-267;
FGFR2 fusions in iCCA: Graham et al. Hum Pathol
2014 (45), 1630-1638; Jain et al. JCO Precis Oncol
2018 (2) 1-12



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 collaboration with Roche to study a combination of derazantinib and Roche's PD-L1-blocking immune-checkpoint inhibitor atezolizumab in patients with urothelial cancer



Blocking CSF1/CSF1R has the potential to reprogram tumor-promoting macrophages and enhance the response to immune checkpoint (PD1/PD-L1) inhibitors.²

Sources: 1. X. Zheng et al. Redirecting tumor-associated macrophages to become tumoricidal effectors as a novel strategy for cancer therapy. *Oncotarget*. 2017;8(29):48436-48452
2. Graph adapted from: A. Ghasemzadeh et al. New Strategies in Bladder Cancer: A Second Coming for Immunotherapy. *Clin Cancer Res*. 2016;22(4):793-801

BAL101553 — three ongoing clinical studies

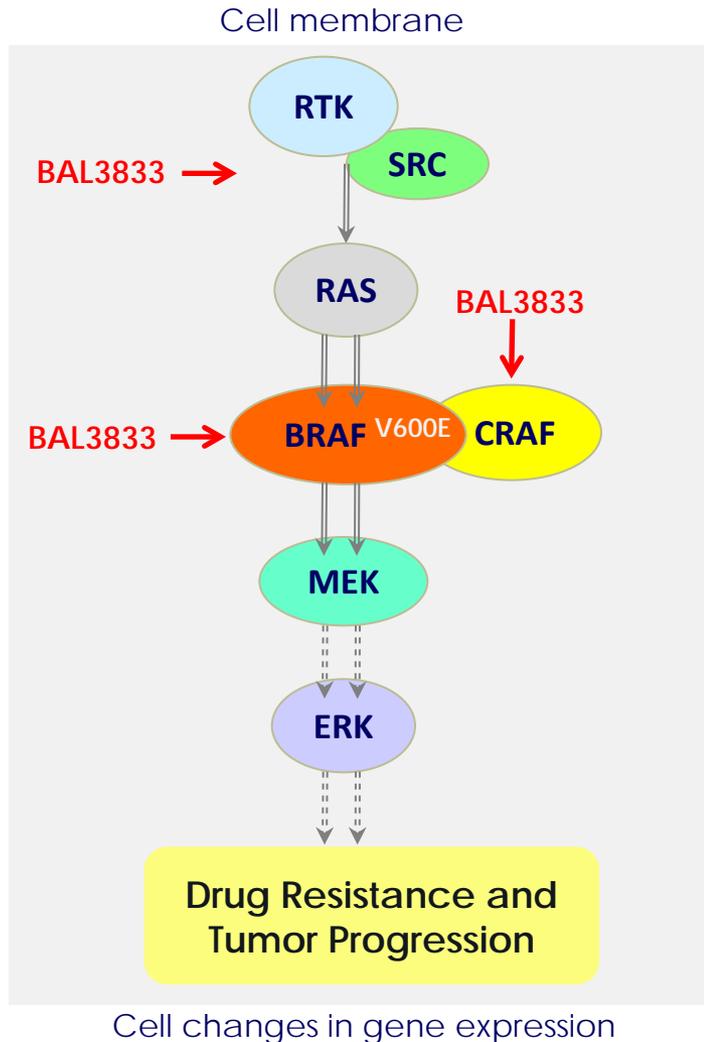
- Phase 2a expansion (weekly 48-hour i.v.) in patients with recurrent glioblastoma or platinum-resistant ovarian cancer
 - Anticipated to complete around year-end 2019
- Phase 1 dose escalation (daily oral) in patients with recurrent glioblastoma
 - Anticipated to complete in H1 2019
- Phase 1 study (daily oral) in combination with radiotherapy in patients with newly diagnosed glioblastoma in collaboration with the Adult Brain Tumor Consortium (ABTC)¹
 - Anticipated to complete patient enrolment mid-2020



¹The ABTC is funded by the U.S. National Cancer Institute (NCI)



BAL3833 — panRAF/SRC kinase inhibitor



- In-licensed novel, oral, small molecule drug from consortium around Wellcome Trust & Institute of Cancer Research (ICR)
- Dual-targeting kinase inhibitor
- Targets resistance mechanisms associated with approved BRAF inhibitors (including vemurafenib and dabrafenib)
- Resistance-reversal activity in BRAF/MEK inhibitor- and immunotherapy-resistant melanoma models
- Potential in diverse non-melanoma tumor types:
 - e.g. RAS-driven tumors
 - Expanded biomarker program to aid tumor selection
- Phase 1 dose-escalation study completed
 - Broad dose range investigated, maximum tolerated dose (MTD) was not defined
 - Pre-clinical activities to explore alternative formulations initiated



Financials

Donato Spota
Chief Financial Officer

Revenue

In CHF mn	FY 2018	FY 2017	Change
Total revenue	132.6	101.5	+31%
<i>thereof: Product and contract revenue</i>	105.9	90.3	+17%

In CHF mn	FY 2018	FY 2017	Change
Revenue contributions from Cresemba and Zevtera	82.0	52.6	+56%
<i>Product revenue</i>	26.2	16.3	+61%
<i>Royalties</i>	26.5	15.0	+77%
<i>Milestone payments</i>	15.4	7.0	+120%
<i>Other contract revenue</i>	13.9	14.2	-2.1%

In CHF mn	FY 2018	FY 2017	Change
Other revenue	26.5	10.8	+145%

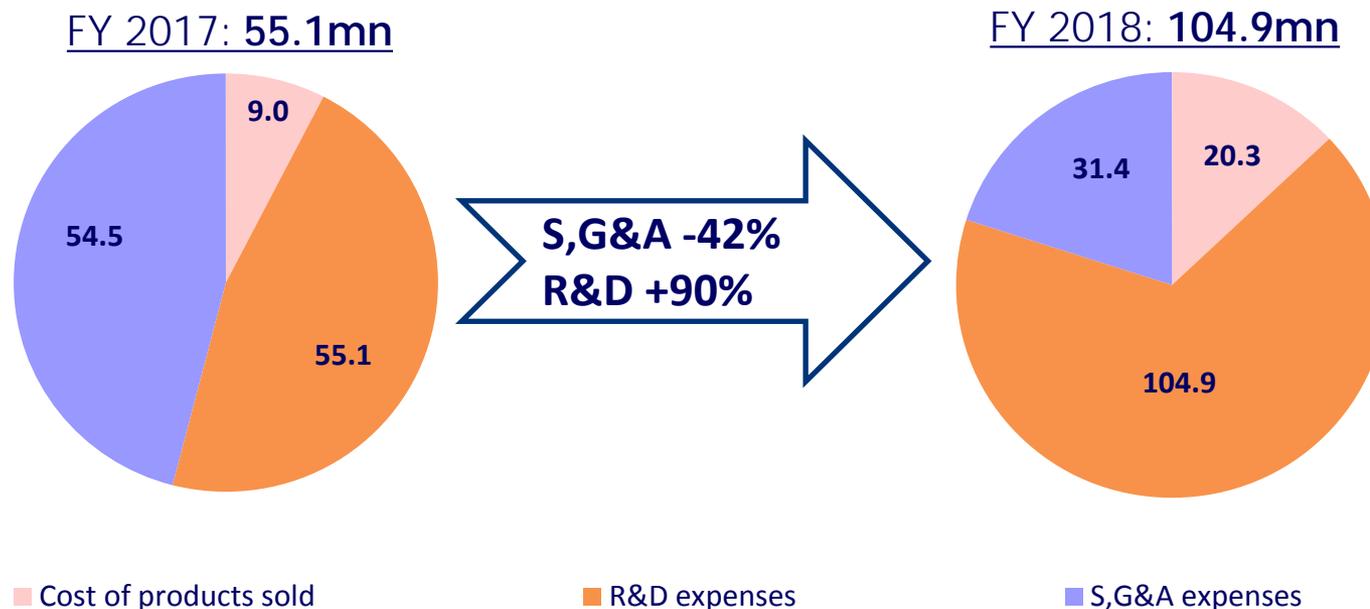
Note: Consolidated figures in conformity with U.S. GAAP; rounding was consistently applied



Cost and operating expenses

In CHF mn	FY 2018	FY 2017	Change
Total cost and operating expenses	156.7	118.6	+32%

R&D investments drive change in operating expenses (CHF mn)



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



Financial summary 2018 and 2017

In CHF mn	FY 2018	FY 2017
Operating loss	(24.1)	(17.1)
Net loss	(31.4)	(19.4)

In CHF mn	FY 2018	FY 2017
Net cash used for operating activities	(79.2)	19.0

In CHF mn	Dec 31, 2018	Dec 31, 2017
Cash and financial investments	223.0	310.7

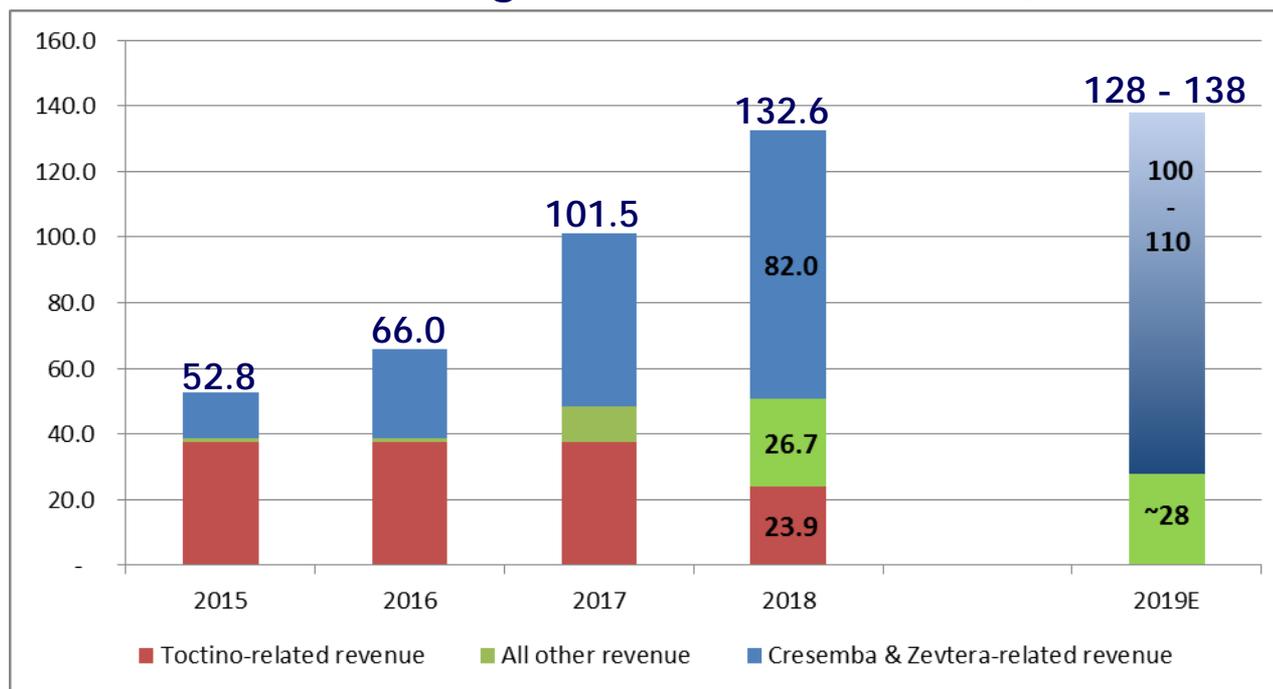
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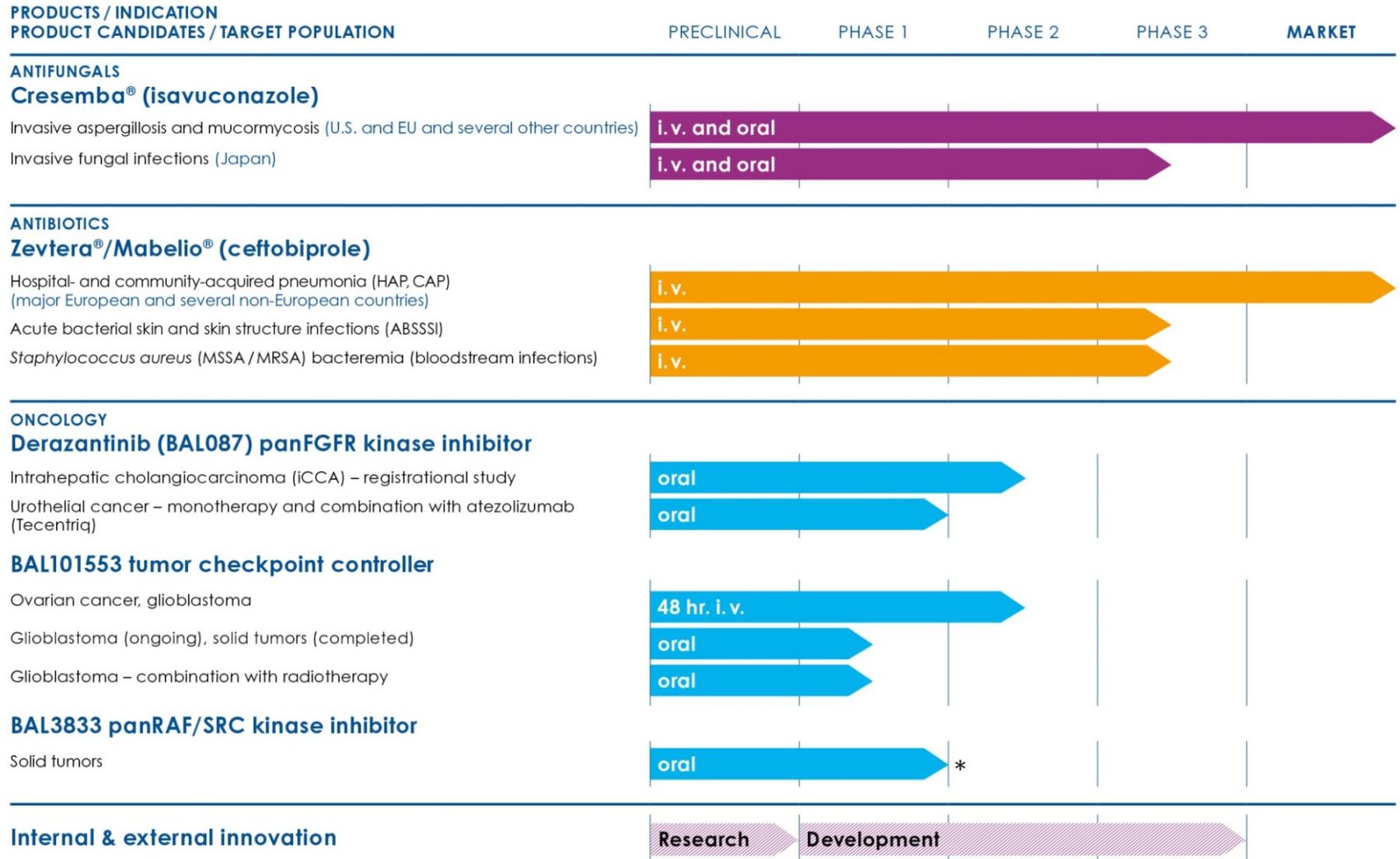
Financial guidance 2019

In CHF mn	FY 2019 guidance	FY 2018 actuals
Total revenue	128 - 138	132.6
Contributions from Cresemba & Zevtera	100 - 110	82.0
Operating loss	20 - 30	24.1
Net operating cash consumption	55 - 65	79.2

2015 – 2019E – Strong revenue increase Y-o-Y, CHF mn



Potential for sustainable growth and value creation based on commercialized products and differentiated pipeline



* pre-clinical reformulation activities initiated

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

Top line results from phase 3 ABSSSI study

Derazantinib

✓ Interim analysis of phase 2 registrational study in iCCA FGFR2 fusions

✓ Collaboration with Roche in urothelial cancer

Expand ongoing iCCA study in other FGFR gene aberrations

Complete patient enrolment in phase 2 registrational study in iCCA

Start phase 2 study in urothelial cancer

Top line results from phase 2 registrational study in iCCA

Interim data from first cohort(s) in urothelial cancer

Interim data from iCCA in other FGFR gene aberrations

BAL101553

Complete patient enrolment in phase 1 study arm for recurrent glioblastoma (oral)

Top line results from phase 2a study in ovarian cancer and glioblastoma (48-hr. i.v.)

Complete patient enrolment in phase 1 study in newly diagnosed glioblastoma



Q & A

