J.P. Morgan Healthcare Conference

David Veitch, CEO
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Disclaimer and forward-looking statements

Forward looking statements

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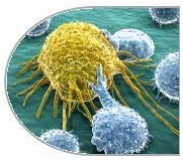
Basilea — At a glance

- Revenue-generating, commercial-stage Swiss biotech company with solid cash position (YE2018 ~CHF 223mn)
- Focused in the areas of oncology, hospital antibiotics and hospital antifungals



- Two marketed anti-infective brands (Cresemba and Zevtera) and three oncology drug candidates in development
- Potential for sustainable growth and value generation based on increasing revenues and selective investments into internal and external innovation

- Founded in 2000 as spin-off from Roche
- Listed on the SIX Swiss Stock Exchange since 2004 (SIX: BSLN)
- Based in life sciences hub Basel (Switzerland); approx. 220 employees

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

PRODUCTS / INDICATION PRODUCT CANDIDATES / TARGET POPULATION	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	MARKET
ANTIFUNGALS					
Cresemba® (isavuconazole)					
Invasive aspergillosis and mucormycosis (U.S. and EU and several other countries)			i. v. and oral		
Invasive fungal infections (Japan)			i. v. and oral		
ANTIBIOTICS					
Zevtera®/Mabelio® (ceftobiprole)					
Hospital- and community-acquired pneumonia (HAP, CAP) (major European and several non-European countries)			i. v.		
Acute bacterial skin and skin structure infections (ABSSSI)			i. v.		
Staphylococcus aureus (MSSA / MRSA) bacteremia (bloodstream infections)			i. v.		
ONCOLOGY					
Derazantinib (BAL087) panFGFR kinase inhibitor					
Intrahepatic cholangiocarcinoma (iCCA) – registrational study			oral		
Other solid tumors			oral		
BAL101553 tumor checkpoint controller					
Ovarian cancer, glioblastoma			48 hr. i. v.		
Glioblastoma (ongoing), solid tumors (completed)			oral		
Glioblastoma – combination with radiotherapy			oral		
BAL3833 panRAF/SRC kinase inhibitor					
Solid tumors			oral *		
Internal & external innovation		Research	Development		

* additional pre-clinical activities planned

Established strong partnerships to fully exploit commercial potential of Cresemba® and Zevtera®

License partners

- **Pfizer,** for Europe (ex. Nordics), China, Asia-Pacific, Russia, Turkey and Israel *(Cresemba)*
- **Astellas,** for the U.S. *(Cresemba)*
- **Asahi Kasei Pharma,** for Japan *(Cresemba)*
- **CR Gosun,** for China *(Zevtera)*

Distribution partners

- **Correvio (formerly Cardiome),** for Europe (ex. Nordics), Israel *(Zevtera)*
- **Hikma,** for MENA region *(Cresemba and Zevtera)*
- **Grupo Biotoscana,** for LatAm *(Cresemba and Zevtera)*
- **Unimedic,** for Nordics *(Cresemba and Zevtera)*
- **Avir,** for Canada *(Cresemba and Zevtera)*

>100 countries covered by partnerships — Received ~ USD 240mn in total upfront and milestone payments

Ongoing participation

- Double-digit royalties on sales by license partners
- Participation through transfer price structure in sales by distribution partners
- USD 1.1bn in total potential regulatory and sales milestones

Unless otherwise indicated, the partners commercialize both Cresemba and Zevtera

Antifungal

Cresemba®
(isavuconazole)

- Invasive mold infections
- Marketed in the U.S. and Europe

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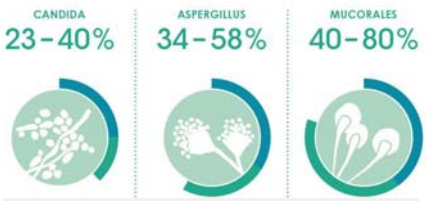
Invasive fungal infections — An area of continued high unmet medical need

- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand
- Mucorales infections on the rise – doubled from 2000 to 2013
- Limitations of current therapies (spectrum of activity, toxicity, effective plasma levels) drive the need for new agents

Mortality rates for invasive fungal infections**



**Kullberg/Arendrup *N Engl J Med* 2015, Baddley *Clin Infect Dis* 2010, Roden *Clin Infect Dis* 2005, Greenberg *Curr Opin Infect Dis* 2004

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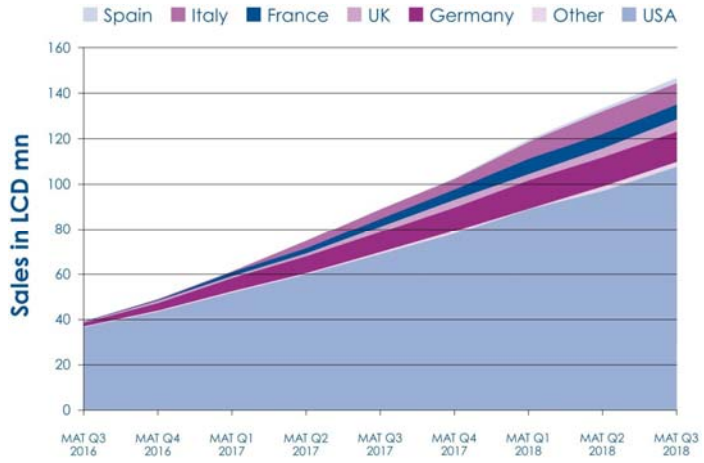
Cresemba continues strong sales uptake in established and new markets

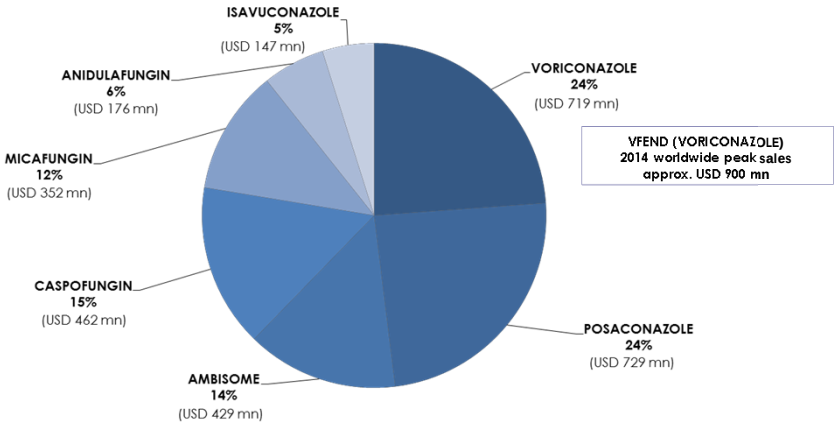
> USD 145 mn annual in-market sales by Q3 2018



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

Sales of best-in-class antifungals* by product

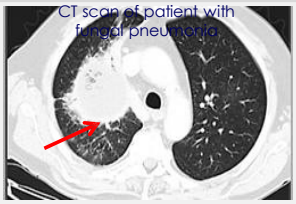
USD 3.0 bn sales (MAT Q3 2018)



* 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, September 2018

Cresemba — Differentiated by spectrum, safety and tolerability

CRESEMBA® (ISAVUCONAZOLE)


CT scan of patient with fungal pneumonia

- Broad spectrum of activity against molds, including emerging molds (mucorales)
- Consistent plasma levels
- Statistically fewer drug-related adverse events and treatment-emergent adverse events (liver, skin, eye) in invasive aspergillosis patients vs. voriconazole in SECURE phase 3 study
- Can be administered in patients with renal impairment
- Manageable drug-drug interaction profile
- Once daily maintenance dose, i.v./oral treatment

- ECIL-6 guideline: Cresemba recommended for the first-line treatment of invasive aspergillosis in leukemia and hematopoietic stem cell transplant patients. ECIL states that isavuconazole is as effective as voriconazole with a better safety profile.

ECIL: The European Conference on Infections in Leukaemia

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Cresemba — Marketed in the EU and U.S. and further country launches planned

For full U.S. prescribing information see: www.cresemba.com

- Marketed in major European countries by Pfizer
- Marketed in the U.S. by Astellas
  - USD 117mn (+35% Y-o-Y) net sales guidance for FY18/19 (ending Mar 2019)
  - CHF 10mn sales milestone triggered in Q4 2018
- Many launches in countries outside of the EU and U.S. anticipated 2019 and beyond
- Exclusivity through 2027 in the U.S. and potential pediatric exclusivity extension to 2027 (from 2025) in the EU

Approved in Europe for the treatment of adults with: invasive aspergillosis and mucormycosis for whom amphotericin B is inappropriate

Approved in the U.S. for the treatment of adults with: invasive aspergillosis and invasive mucormycosis

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Antibacterial

Zevtera®/Mabelio® (ceftobiprole)

European box/vials
Ceftobiprole is not approved in the U.S.

- Hospital* and community-acquired pneumonia
- Marketed in major European countries, Argentina, Canada , Peru and Saudi-Arabia

* HAP (excluding VAP)

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Zevtera/Mabelio — A fast-acting hospital antibiotic with activity against a broad range of bacteria

Approved in major European countries & several non-European countries for both hospital-acquired pneumonia (HAP), excluding ventilator-acquired pneumonia (VAP), and community-acquired pneumonia (CAP)

Not approved in the U.S.

- 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 major European markets, Argentina, Canada, Peru and Saudi Arabia

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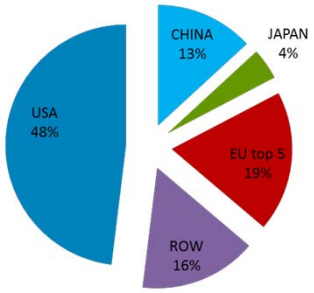
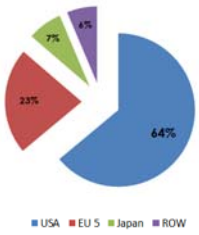
Anti-MRSA hospital antibiotics market — A USD 3.1bn market with the U.S. being the most important region

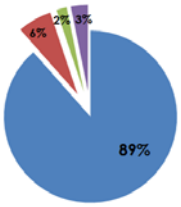
Global anti-MRSA hospital antibiotics sales*
USD 3.1bn (MAT Q3 2018)



Linezolid sales by region 2014 (before LOE)



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

Ceftobiprole — Strategy for accessing the important U.S. market providing attractive risk-return profile

- U.S. registration requires two cross-supportive phase 3 studies
  - FDA has approved Special Protocol Assessments for ABSSSI and SAB phase 3 studies
  - ABSSSI and SAB studies started in 2018
- Few approved SAB agents available, with limitations, mainly related to resistance or tolerability
- For SAB, ceftobiprole has potential to be positioned as a rapidly cidal agent against both MSSA and MRSA with the favourable safety profile of a cephalosporin
- BARDA funding of up to USD 128mn (~70% of the total estimated program costs) to support U.S. phase 3 program*
- QIDP designation (SAB, ABSSSI, CABP): exclusivity extended to 10 years upon approval

*The project is funded in part with Federal funds from the Department of Health and Human Services; Office of the Assistant Secretary for Preparedness and Response; Biomedical Advanced Research and Development Authority (BARDA) under Contract No. HSO100201600002C

SAB: *Staphylococcus aureus* bacteremia; **ABSSSI:** acute bacterial skin and skin structure infection; **CABP:** community-acquired bacterial pneumonia

Oncology

Derazantinib (BAL087)

panFGFR kinase inhibitor
for various solid tumors

Derazantinib — Potential first-in-class FGFR kinase inhibitor in intrahepatic cholangiocarcinoma (iCCA)

- 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
- FGFR genetic aberrations have been identified as important therapeutic targets for various cancers, including iCCA, bladder, breast, gastric and lung cancers[1, 2]
- Opportunity in iCCA, an indication with high unmet need and globally increasing incidence
- Strong data foundation generated to support potential expedited FDA approval in iCCA
- Orphan drug designation granted by FDA and EMA

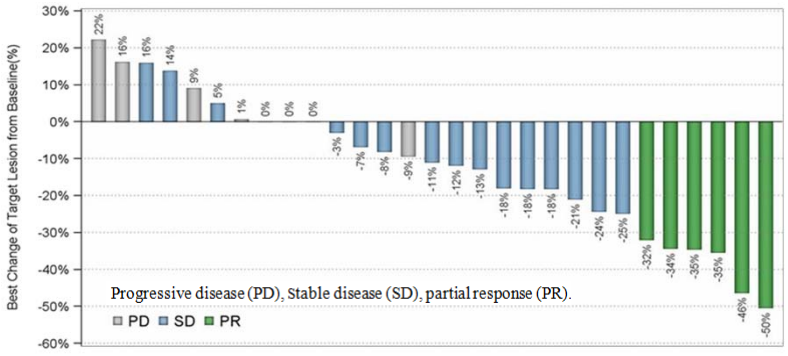
Sources:
1 R. Porta et al. FGFR a promising druggable target in cancer: Molecular biology and new drugs. Critical Reviews in Oncology/Hematology 2017 (113), 256-267;
2 I. S. Babina, N. C. Turner. Advances and challenges in targeting FGFR signalling in cancer. Nature Reviews Cancer 2017 (17), 318-332

Derazantinib — Favorable clinical data (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 derazantinib[1] versus <10% for SoC[2]
  - Progression-Free Survival (PFS) approx. 6 months[1] versus 3 months for SoC[2]
- Manageable toxicity and low discontinuation rate[3]
- Registrational phase 2 study in iCCA patients ongoing, interim analysis expected in early 2019

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; 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
3 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 — Established proof-of-concept in iCCA in phase 1/2a study

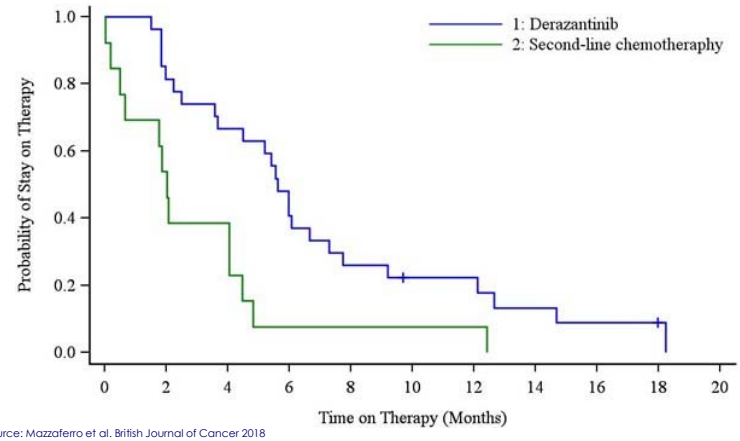


- Subgroup analysis of 29 patients with FGFR2-fusion positive iCCA:
  - Objective response rate of 21%
  - In 72% of patients, tumor response or disease stabilization for ≥16 weeks was achieved*
  - Manageable safety profile, representative of class of drugs

Source: Mazzaferro et al. British Journal of Cancer 2018; *Mazzaferro et al. J Clin Oncol 2017;35 suppl: abstract 4017

Derazantinib — Time on treatment supports clinical benefit in FGFR2-fusion positive iCCA

Intrapatient comparison of time on study with derazantinib compared to pre-study second-line chemotherapy



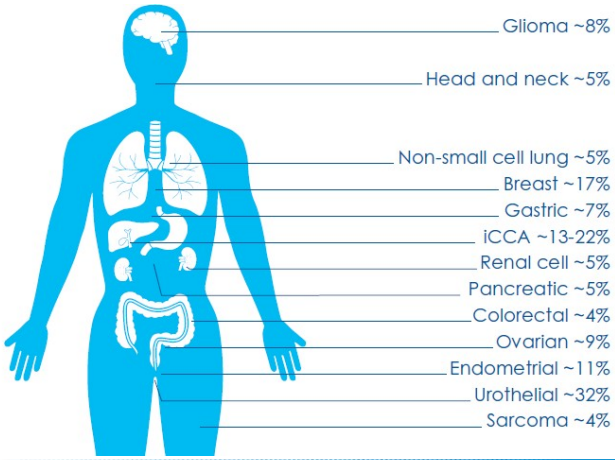
Source: Mazzaferro et al. British Journal of Cancer 2018

Derazantinib — iCCA registrational phase 2 study ongoing

- **Design:** Multi-national, open-label, non-comparative study
- **Enrolment:** 100 adult patients
- **Indications:** Intrahepatic cholangiocarcinoma (iCCA) with FGFR2 fusions (2nd-line)
- **Main inclusion criteria:**
  - Adult subjects with locally advanced (inoperable) or metastatic iCCA whose tumors harbor FGFR2 gene fusions and who received at least one prior regimen of systemic therapy
  - Measurable disease by RECIST 1.1
- **Intervention**: 300 mg oral ARQ 087 once daily
- **Primary endpoint:** Objective Response Rate (ORR)
- **Secondary endpoints**: Progression-free survival (PFS), Overall Survival (OS), Duration of response (DoR), Safety

Derazantinib — Significant potential beyond iCCA

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

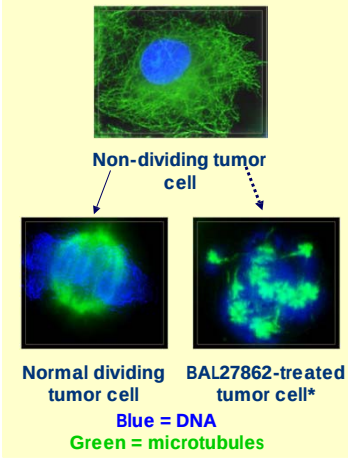
Oncology

BAL101553

Treatment-refractory solid tumors, including glioblastoma

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

Bachmann AACR 2015



- Novel compound inducing tumor cell death through checkpoint activation
- Destabilizing the microtubule scaffold through a novel target-binding site
- Targeting diverse tumor types resistant to standard therapeutic approaches
- Crosses the blood-brain barrier with potent activity in brain tumor models alone and in combination
- Flexible dosing potential, including daily oral dosing
- Comprehensive biomarker program to optimize patient and tumor selection

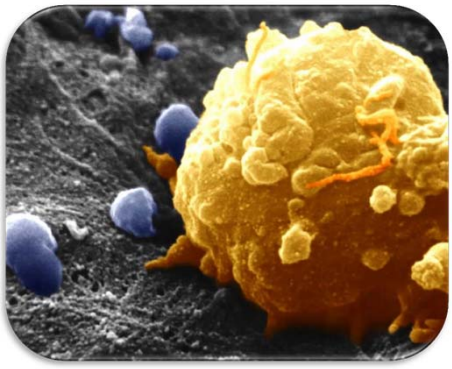
* BAL101553 is a prodrug of BAL27862

BAL101553 — Three ongoing clinical studies

- Phase 2a expansion (weekly 48-hour i.v.) in patients with recurrent glioblastoma or platinum-resistant ovarian cancer
  - Anticipated to complete in H2 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]

Adult Brain Tumor Consortium

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



Oncology

BAL3833

Treatment-refractory solid tumors, including metastatic melanoma and RAS-driven tumors

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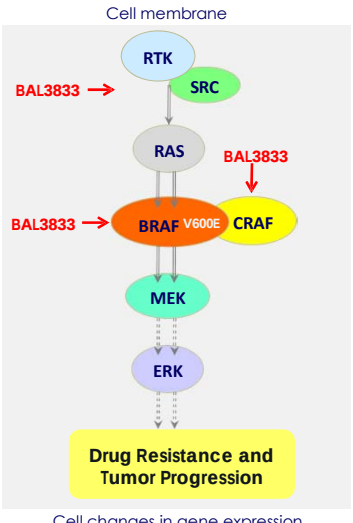
BAL3833 — panRAF/SRC kinase inhibitor



- 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 being initiated

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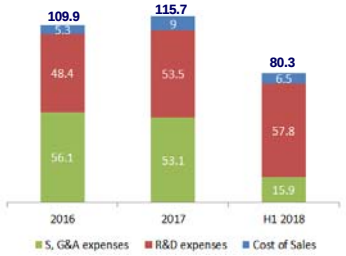
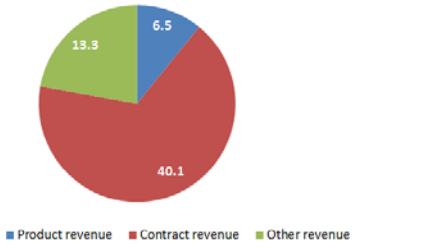
Key financials HY 2018 — Strong revenue growth and selective investments in pipeline

In CHF mn, *except for number of shares*	**H1 2018**	H1 2017	FY 2017
Cash and financial investments	**247.3**	253.1	310.7
Total revenue	**59.9**	46.2	101.5
Total operating expenses	**80.3**	65.3	115.7
Net loss	**22.5**	20.6	19.4
Operating Loss	**20.4**	19.1	14.1
Shares outstanding: 11.9mn (as of June 30, 2018)			

H1 2018 - Savings on S,G&A, Investments in R&D



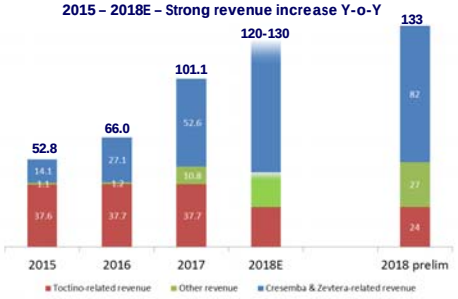
H1 2018 – Total Revenue CHF 59.9mn (+30% Y-o-Y)



Financial guidance 2018 (in CHF mn)

	Updated FY 2018 guidance	**H1 2018 Actuals**	**Initial FY 2018 guidance**	**FY 2017 Actuals**
Total revenue	**120 - 130**	59.9	105 - 115	101.5
Contributions from Cresemba & Zevtera	**75 - 85**	27.8	60 - 65	52.6
Operating loss	**25 - 35**	20.4	10 - 20	14.1
Cash burn	**~7 avg/month**	~10 avg/month	na	+19.0

2015 – 2018E – Strong revenue increase Y-o-Y



H2 2018E – Significant reduction in operating loss expected

Strong financial position provides flexibility towards achieving multiple key value inflection milestones

- Cash and financial investments of ~CHF 223mn by year-end 2018
- Positive cash-flow from commercialized products Cresemba and Zevtera
- Near-term operating cash-flow expected to further improve based on strongly growing product sales and largely stable operating expenses
- BARDA covering approximately 70% of ceftobiprole phase 3 program cost through non-dilutive funding
- Convertible bond not maturing before December 2022, providing sufficient flexibility for conversion, re-financing or re-payment

Focus 2018/2019

Cresemba® & Zevtera®/Mabelio® Increase cash-generating revenues

H1 2018	H2 2018	H1 2019	H2 2019
Ceftobiprole Start ABSSSI phase 3 study	**Ceftobiprole** Start *Staphylococcus aureus* bacteremia phase 3 study		**Ceftobiprole** Top line results from phase 3 ABSSSI study
		Derazantinib Interim analysis of phase 2 registrational trial in iCCA	**Derazantinib** Start phase 2 development in FGFR-driven solid cancer types
BAL101553 Publication of oral and 48-hr. i.v. phase 1 results in solid tumors at ASCO	**BAL101553** Start phase 2a study in ovarian cancer and glioblastoma (48-hr. i.v.)		**BAL101553** Top line results from phase 2a study in ovarian cancer and glioblastoma (48-hr. i.v.)
		BAL101553 Complete enrollment in oral phase 1 study arm for recurrent glioblastoma	
	BAL3833 Completed phase 1 dose-escalation study		

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