Full-year results 2018 presentation

February 19, 2019
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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

Sales in LCD mn

MAT Q3 2016  |  MAT Q4 2016  |  MAT Q1 2017  |  MAT Q2 2017  |  MAT Q3 2017  |  MAT Q4 2017  |  MAT Q1 2018  |  MAT Q2 2018  |  MAT 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

Number of launched countries


x2 x3
>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
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, ortavancin 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 derazantinib\(^1\) versus <10% for SoC\(^2,3\)
  - Progression-Free Survival (PFS) approx. 6 months\(^1\) versus 3 months for SoC\(^2,3\)
- Manageable safety profile and low discontinuation rate\(^1,4\)

Registralional 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
\(^3\) 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
Derazantinib — Significant potential beyond iCCA and urothelial cancer

Frequency of FGFR aberrations across different tumor types

- Glioma ~8%
- Head and neck ~5%
- Non-small cell lung ~5%
- Breast ~17%
- Gastric ~7%
- iCCA ~13-22%
- Renal cell ~5%
- Pancreatic ~5%
- Colorectal ~4%
- Ovarian ~9%
- Endometrial ~11%
- Urothelial ~32%
- Sarcoma ~4%

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 inhibitors

• 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 avelumab in patients with urothelial cancer

Sources:
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

\(^1\) The 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

<table>
<thead>
<tr>
<th>In CHF mn</th>
<th>FY 2018</th>
<th>FY 2017</th>
<th>Change</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total revenue</td>
<td>132.6</td>
<td>101.5</td>
<td>+31%</td>
</tr>
<tr>
<td>thereof: Product and contract revenue</td>
<td>105.9</td>
<td>90.3</td>
<td>+17%</td>
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</table>

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

<table>
<thead>
<tr>
<th>In CHF mn</th>
<th>FY 2018</th>
<th>FY 2017</th>
<th>Change</th>
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</thead>
<tbody>
<tr>
<td>Other revenue</td>
<td>26.5</td>
<td>10.8</td>
<td>+145%</td>
</tr>
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Note: Consolidated figures in conformity with U.S. GAAP; rounding was consistently applied.
# Cost and operating expenses

## In CHF mn

<table>
<thead>
<tr>
<th></th>
<th>FY 2018</th>
<th>FY 2017</th>
<th>Change</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total cost and operating expenses</td>
<td>156.7</td>
<td>118.6</td>
<td>+32%</td>
</tr>
</tbody>
</table>

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

- **FY 2017:** 55.1mn
  - Cost of products sold: 54.5
  - R&D expenses: 9.0
  - S,G&A expenses: 55.1
- **FY 2018:** 104.9mn
  - Cost of products sold: 31.4
  - R&D expenses: 20.3
  - S,G&A expenses: 104.9

Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently
### Financial summary 2018 and 2017

<table>
<thead>
<tr>
<th>In CHF mn</th>
<th>FY 2018</th>
<th>FY 2017</th>
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</thead>
<tbody>
<tr>
<td>Operating loss</td>
<td>(24.1)</td>
<td>(17.1)</td>
</tr>
<tr>
<td>Net loss</td>
<td>(31.4)</td>
<td>(19.4)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>In CHF mn</th>
<th>FY 2018</th>
<th>FY 2017</th>
</tr>
</thead>
<tbody>
<tr>
<td>Net cash used for operating activities</td>
<td>(79.2)</td>
<td>19.0</td>
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</tbody>
</table>

<table>
<thead>
<tr>
<th>In CHF mn</th>
<th>Dec 31, 2018</th>
<th>Dec 31, 2017</th>
</tr>
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<tbody>
<tr>
<td>Cash and financial investments</td>
<td>223.0</td>
<td>310.7</td>
</tr>
</tbody>
</table>

**Note:** Consolidated figures in conformity with U.S. GAAP; rounding applied consistently
## Financial guidance 2019

<table>
<thead>
<tr>
<th>In CHF mn</th>
<th>FY 2019 guidance</th>
<th>FY 2018 actuals</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total revenue</td>
<td>128 - 138</td>
<td>132.6</td>
</tr>
<tr>
<td>Contributions from Cresemba &amp; Zevtera</td>
<td>100 - 110</td>
<td>82.0</td>
</tr>
<tr>
<td>Operating loss</td>
<td>20 - 30</td>
<td>24.1</td>
</tr>
<tr>
<td>Net operating cash consumption</td>
<td>55 - 65</td>
<td>79.2</td>
</tr>
</tbody>
</table>

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

- **2015**: 52.8
- **2016**: 66.0
- **2017**: 101.5
- **2018**: 132.6
- **2019E**: 128 - 138

- Red: Toctino-related revenue
- Green: All other revenue
- Blue: Cresemba & Zevtera-related revenue
Potential for sustainable growth and value creation based on commercialized products and differentiated pipeline

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

* pre-clinical reformulation activities initiated

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

<table>
<thead>
<tr>
<th>H1 2019</th>
<th>H2 2019</th>
<th>H1 2020</th>
<th>H2 2020</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Ceftobiprole</strong></td>
<td>Top line results from phase 3 ABSSSI study</td>
<td></td>
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<tr>
<td><strong>Derazantinib</strong></td>
<td>Interim analysis of phase 2 registrational study in iCCA FGFR2 fusions</td>
<td>Complete patient enrolment in phase 2 registrational study in iCCA</td>
<td>Top line results from phase 2 registrational study in iCCA</td>
</tr>
<tr>
<td></td>
<td>Collaboration with Roche in urothelial cancer</td>
<td></td>
<td>Interim data from first cohort(s) in urothelial cancer</td>
</tr>
<tr>
<td></td>
<td>Expand ongoing iCCA study in other FGFR gene aberrations</td>
<td>Start phase 2 study in urothelial cancer</td>
<td>Interim data from iCCA in other FGFR gene aberrations</td>
</tr>
<tr>
<td><strong>BAL101553</strong></td>
<td>Complete patient enrolment in phase 1 study arm for recurrent glioblastoma (oral)</td>
<td>Top line results from phase 2a study in ovarian cancer and glioblastoma (48-hr. i.v.)</td>
<td>Complete patient enrolment in phase 1 study in newly diagnosed glioblastoma</td>
</tr>
</tbody>
</table>
Q & A