



**Focused on  
Growth and Innovation**

**Full-year results 2021**

February 15, 2022

**Webcast presentation**



**David Veitch**

Chief Executive Officer

Introduction



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# Speakers



**David Veitch**  
**CEO**



**Adesh Kaul**  
**CFO**



**Dr. Marc Engelhardt**  
**CMO**

# Key achievements

**FY  
2021**

## Strong financial results

- CHF 128.8 mn Cresemba & Zevtera non-deferred revenue (+65% growth)
- Operating profit of CHF 1.2 mn

## Progressed clinical studies

- Reported positive results from derazantinib study
- Ceftobiprole phase 3 enrolment completed early Jan 2022

## Continued commercial success

- 29% y-o-y increase of royalty income from Cresemba reflecting continued strong in-market sales growth

## Strategic transactions

- Divestment of Chinese R&D subsidiary
- CHF 46 mn gross proceeds from private placement
- 2022 convertible bonds reduced by CHF 23 mn

## Clinical pipeline expansion

BAL0891, a first-in-class mitotic checkpoint inhibitor

## USD 2.7 mn

Awarded CARB-X research grant for novel preclinical antibiotic

# Strategy update

- Separation of the anti-infectives and oncology businesses
- Basilea will be a leading anti-infectives company, focused on novel treatments for severe bacterial and fungal infections. We are uniquely positioned to create sustainable value, in an area of increasing unmet medical need, with our proven ability to advance anti-infective compounds from research, through development, to commercialization.
- Maximize value of the oncology portfolio through strategic transactions

# The Basilea anti-infectives value proposition

- **Significantly growing cash revenues** from Cresemba and Zevtera
- **Cresemba:**
  - 29% royalty income growth in 2021, > USD 300 mn in-market sales in 12-months to September 2021
  - Marketing approvals and launches expected in China and Japan in 2022
- **Zevtera:**
  - Topline results of ceftobiprole phase 3 SAB study expected around mid-2022
  - Potential to file NDA for U.S. end of 2022
  - U.S. is the most important MRSA market ~ 80–90% of global potential
  - QIDP designation provides 10 years market exclusivity from approval
- **Preclinical assets:**
  - A number of preclinical programs, including DXR inhibitor (CARB-X funded)
  - Focus on external sourcing of additional anti-infective compounds
- **Sustainable profitability** from 2023

# Basilea will optimize the value of the oncology portfolio

- Multiple data readouts for derazantinib and lisavanbulin in 2022
- BAL0891 (TTK/PLK1) preparing to enable start of a phase 1 study in mid-2022
- Two preclinical oncology programs
- Exploring a range of transaction options for either a portfolio of assets, or as individual asset transactions, in order to maximize the value of the oncology portfolio

**Adesh Kaul**

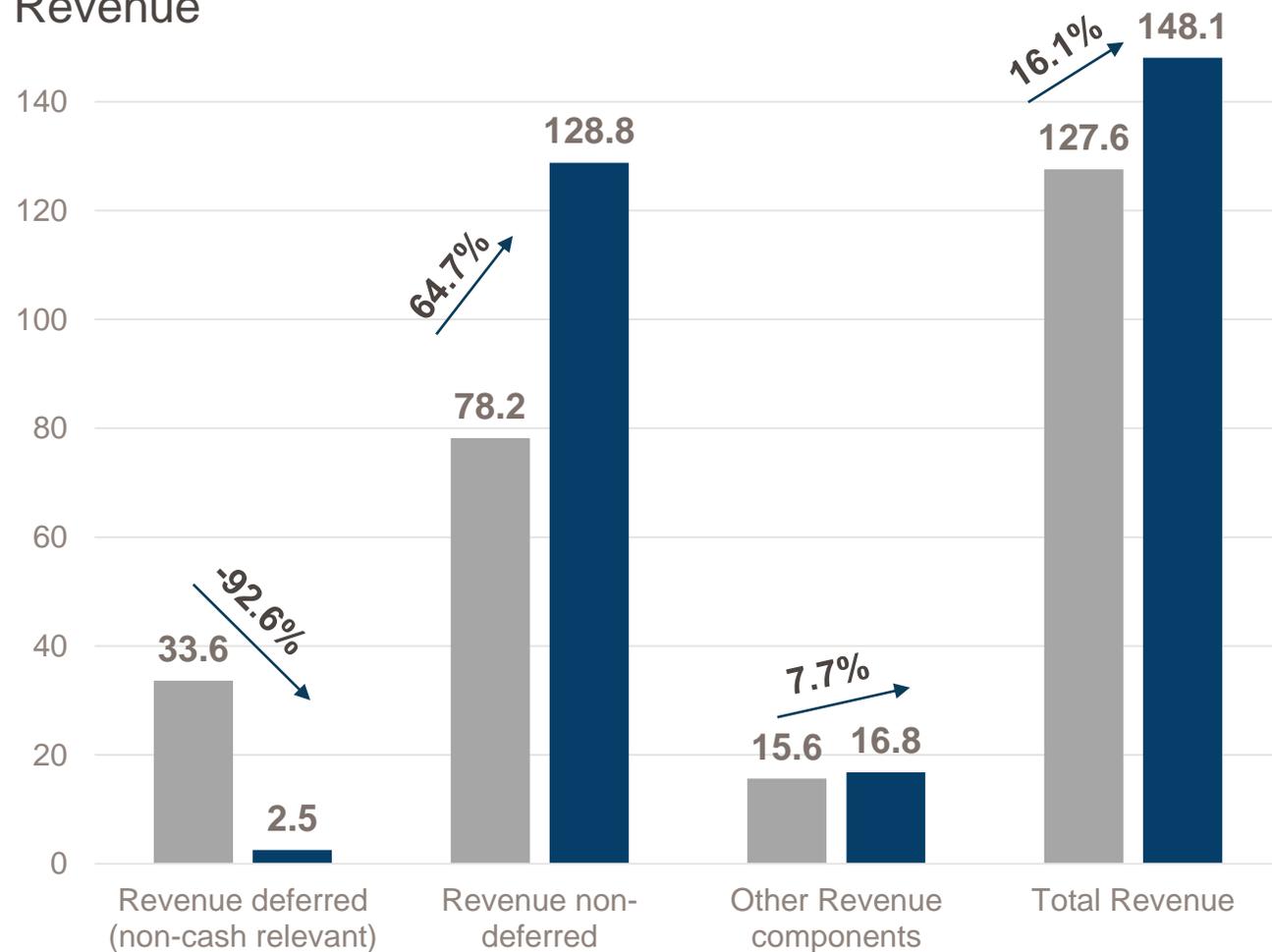
Chief Financial Officer

Financial update &  
commercial

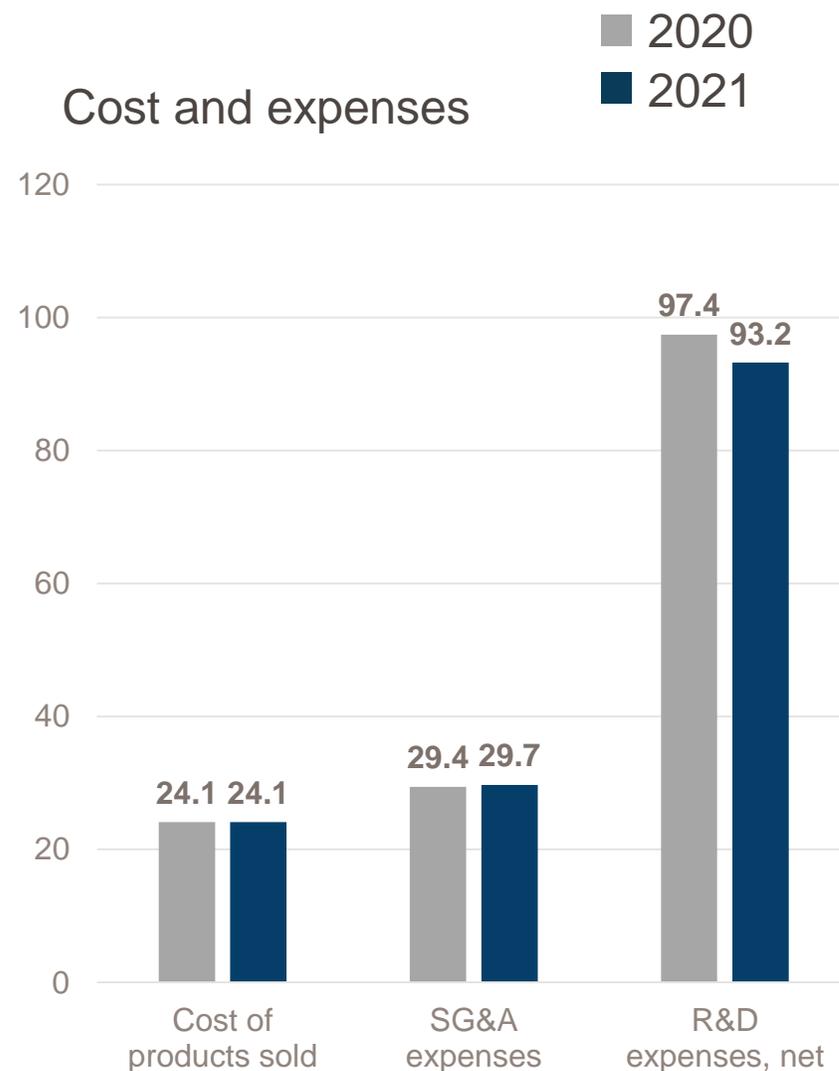


# Financial summary, in CHF mn (1/2)

## Revenue



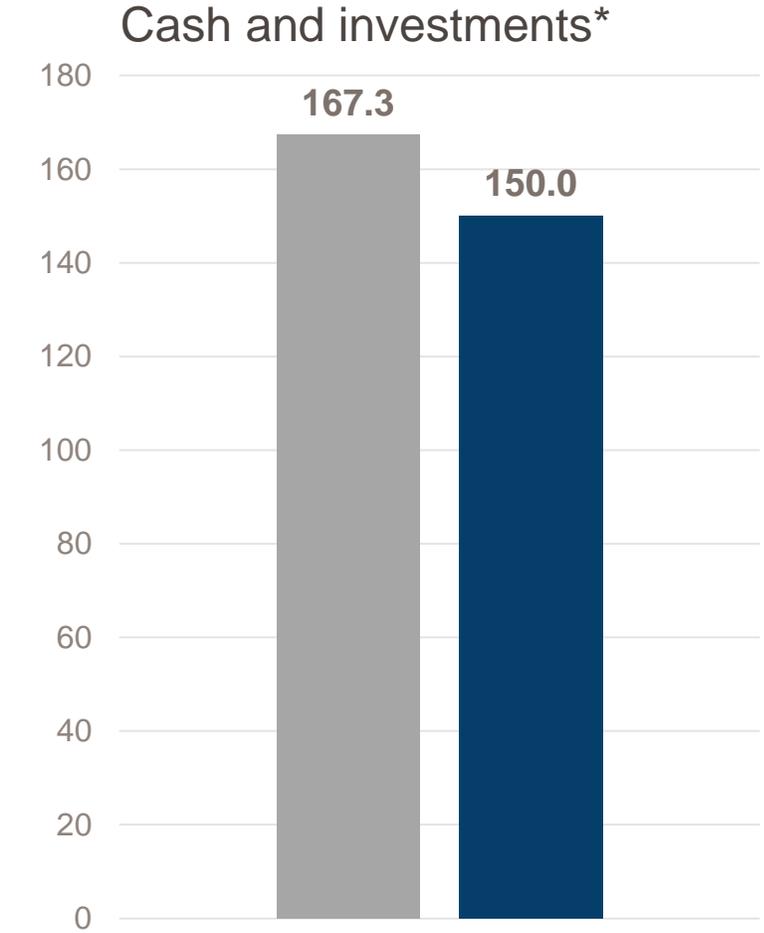
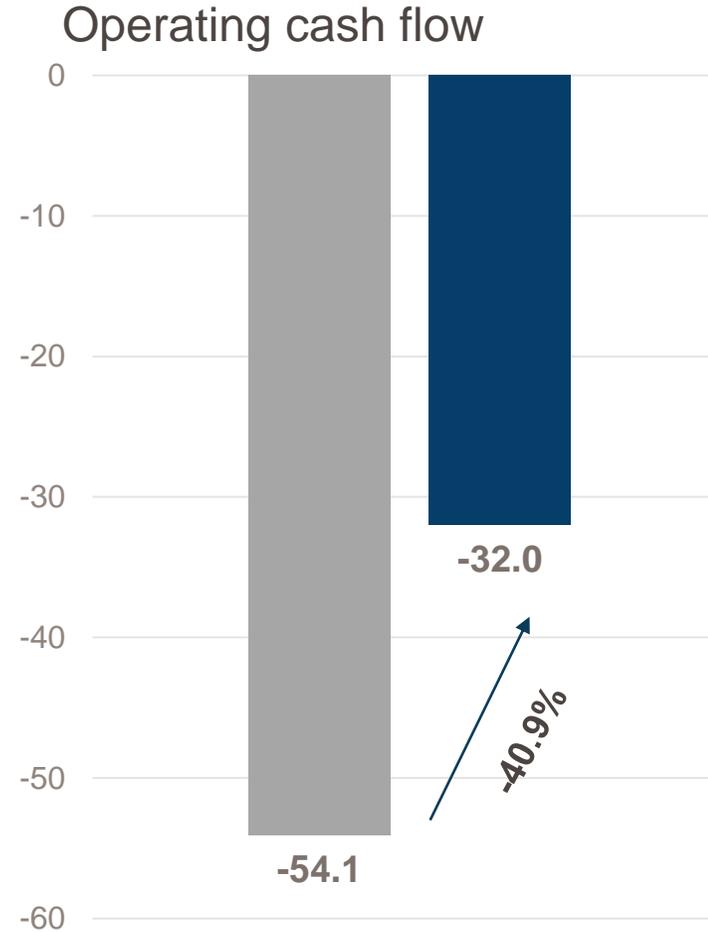
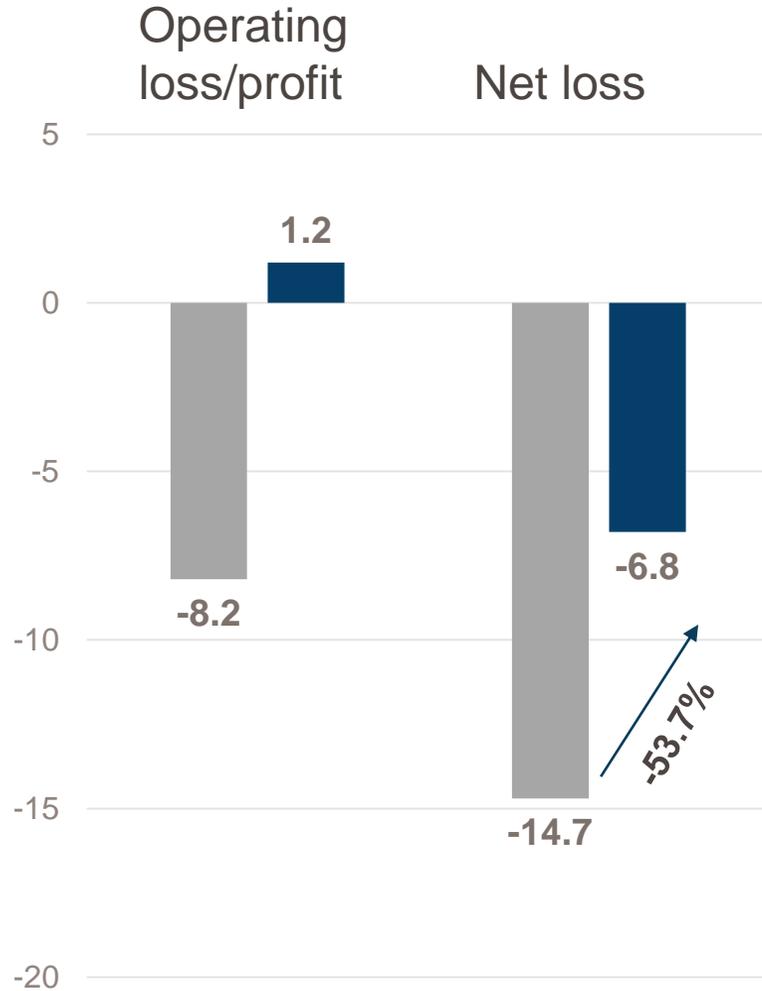
## Cost and expenses



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

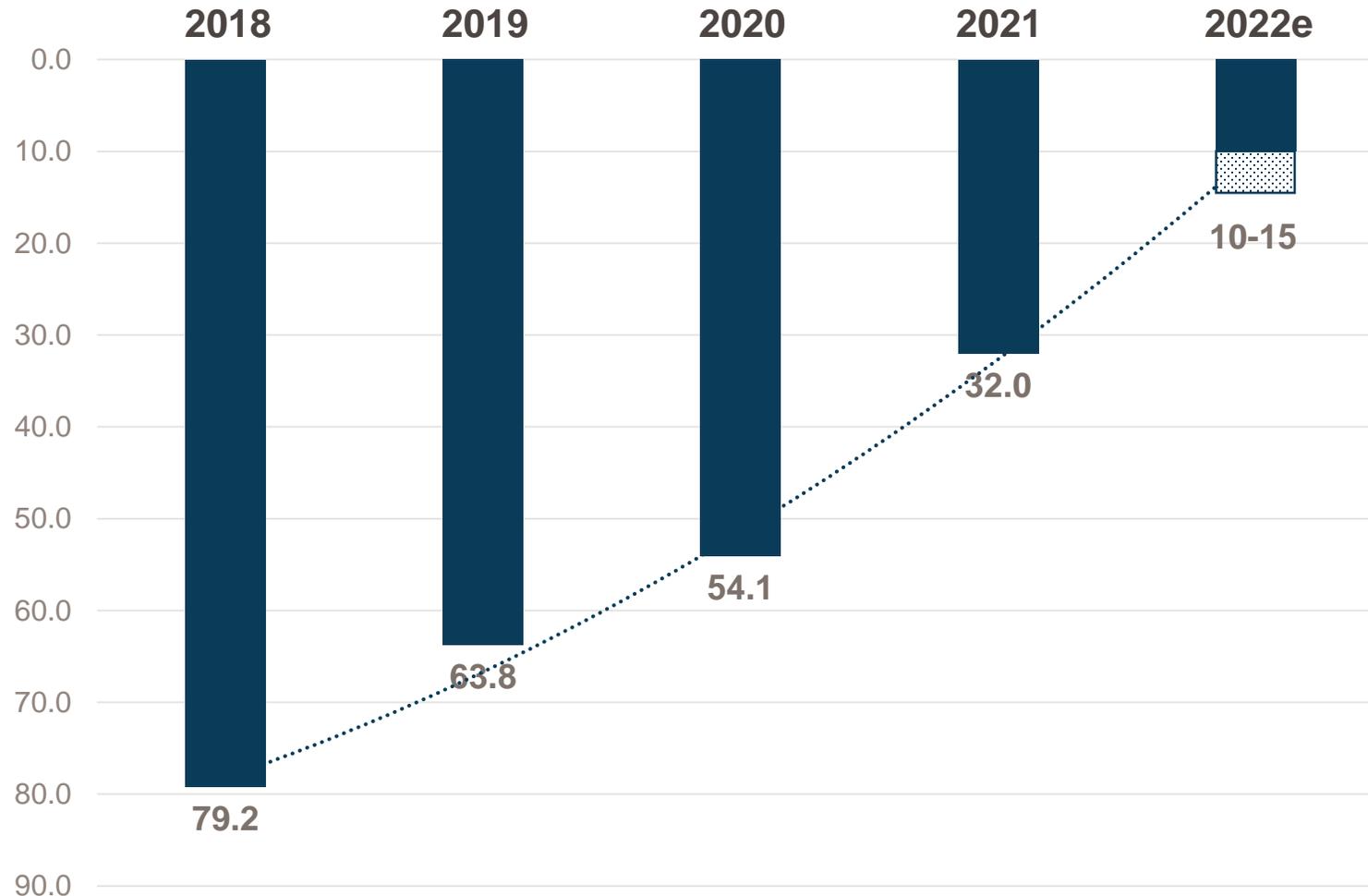
# Financial summary, in CHF mn (2/2)

■ FY 2020  
■ FY 2021



Note: Consolidated figures in conformity with U.S. GAAP; rounding applied consistently, \*Cash, cash equivalents, restricted cash and investments

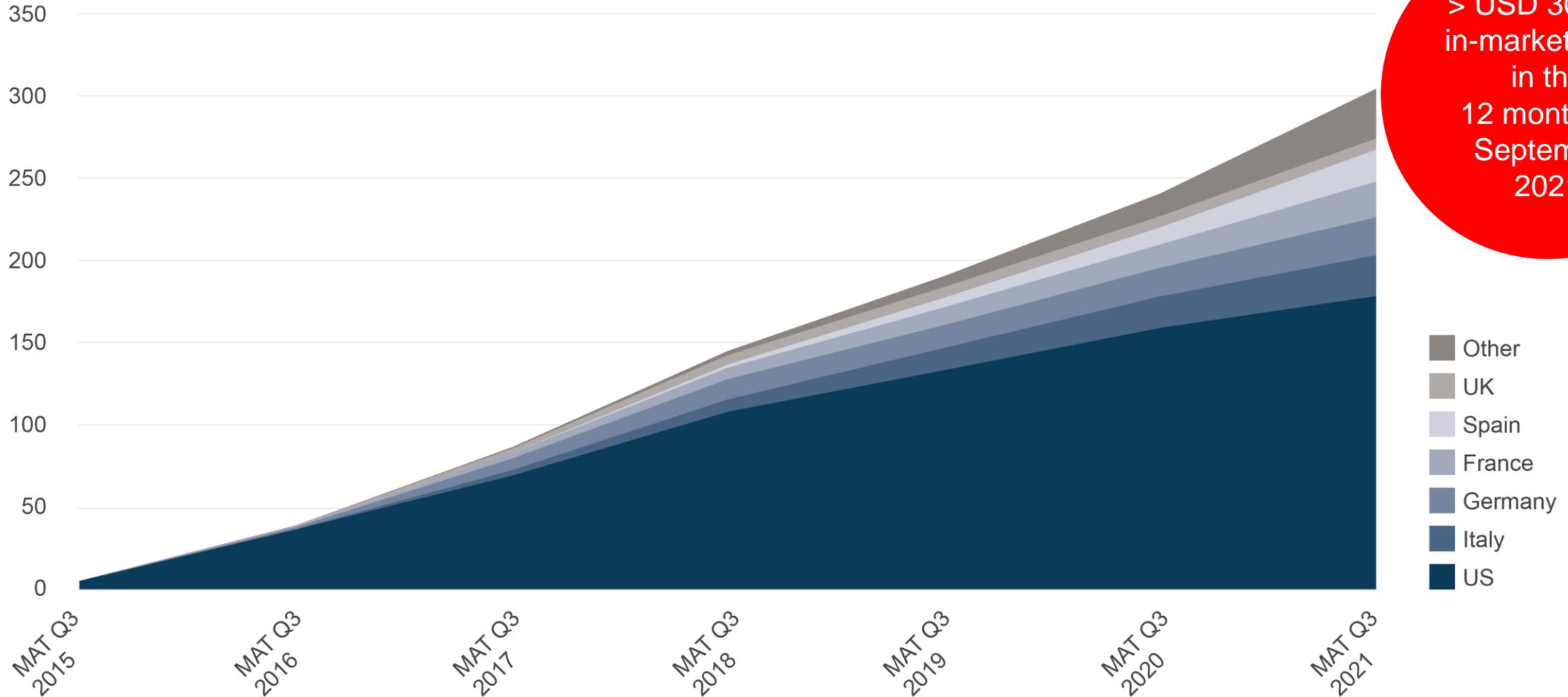
# Net cash used in operating activities



**Sustainable  
profitability from 2023  
onwards**

# Cresemba continues strong in-market sales uptake

Sales in USD mn



> USD 300 mn  
in-market sales  
in the  
12 months to  
September  
2021

MAT: Moving annual total; Source: IQVIA, September 2021

# 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®)



Russia and the Eurasian  
Economic Union  
(Zevtera®)

Double-digit  
percentage  
royalties on  
sales by  
license  
partners

>USD 1 bn  
in potential  
milestones  
remaining

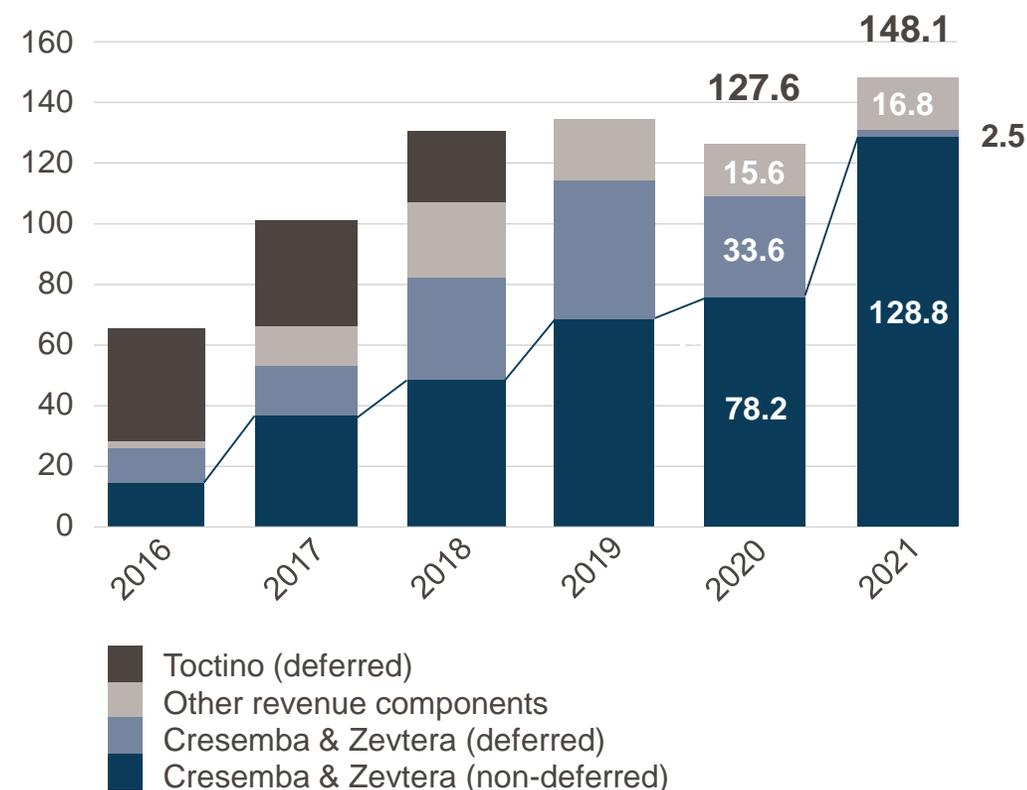
Participation  
in sales of  
distribution  
partners  
through  
transfer price

>USD 295 mn  
upfront and  
milestone  
payments  
received

# 2021 revenue and year-end cash-position exceed financial guidance

In CHF mn	FY 2021 (actual)	FY 2021e (guidance)	FY 2020 (actual)
Total revenue	148.1	134 – 144	127.6
thereof: Contributions Cresemba & Zevtera			
non-deferred	128.8	115 – 125	78.2
deferred	2.5	2.5	33.6
Operating profit/(loss)	1.2	(7 – 17)	(8.2)*
Cash and investments#	150 [173##]	142 - 147 [165 – 170##]	167.3

Continued strong double-digit growth in Cresemba & Zevtera non-deferred revenue contributions Y-o-Y, CHF mn



\* Including CHF 15mn one-off gain from sale and lease back transaction

# Cash, restricted cash and investments

##Excluding impact from reduction of the outstanding convertible bonds in 2021

# Guidance: Sustainable profitability from FY 2023 expected

In CHF mn	FY 2023e (guidance)	FY 2022e* (guidance)	FY 2021 (actual)
Cresemba & Zevtera related revenue	-	98 – 104	131.4
Royalty income	-	~ 59	53.2
Total revenue	-	106 – 112	148.1
Cost of products sold Operating expenses	- -30% vs. 2022	21 – 24 ~ 110	24.1 122.9
Operating (loss)/profit	> 0	(20 – 25)	1.2
Net cash used in operating activities	Cash flow positive	10 – 15	32.0

Decrease in Cresemba & Zevtera related revenue 2022 vs. 2021 due to lower expected milestone payments

\* 2022 guidance does not include the potential impact from strategic transactions on the oncology assets

**Dr. Marc Engelhardt**

Chief Medical Officer

Development  
update

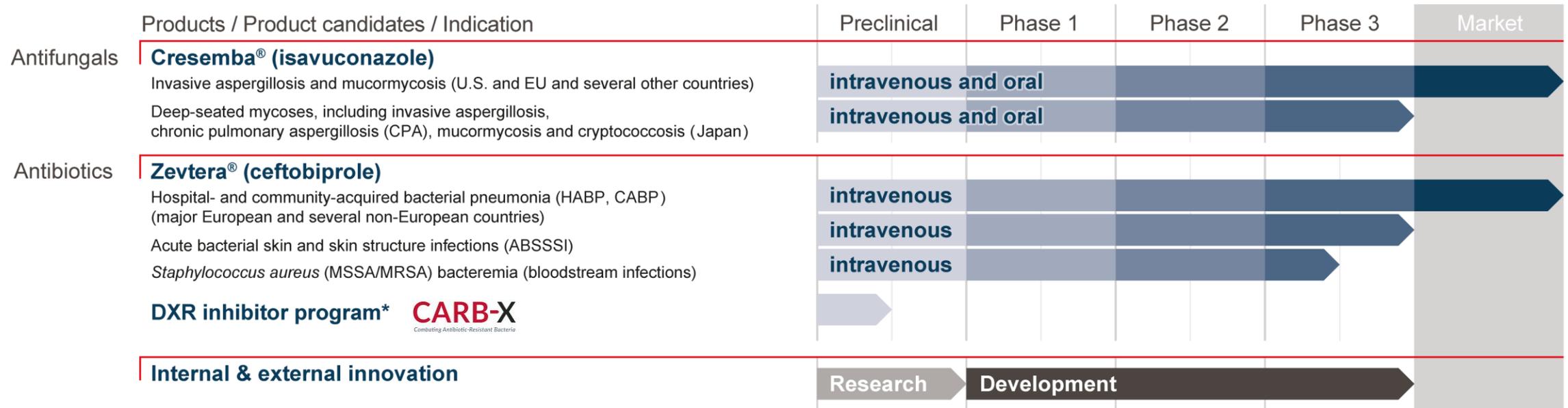


# Anti-infectives

Severe bacterial and fungal infections



# Pipeline: Anti-infectives



\*CARB-X's funding for this project is sponsored by Cooperative Agreement Number IDSEP160030 from ASPR/BARDA and by awards from Wellcome Trust and Germany's Federal Ministry of Education and Research. The content is solely the responsibility of the authors and does not necessarily represent the official views of CARB-X or any of its funders.

# Ceftobiprole - Strategy for accessing the U.S. market

- Two cross-supportive phase 3 studies under FDA Special Protocol Assessment (SPA)
- Phase 3 program largely funded by BARDA (~70% of total program costs; up to USD ~134 mn)

1. Acute Bacterial Skin and Skin Structure Infections (ABSSSI)<sup>1</sup>, successfully completed



2. *Staphylococcus aureus* bacteremia (SAB)<sup>2</sup>, patient enrolment completed in January 2022, topline results expected around mid-year 2022



- Qualified Infectious Disease Product (QIDP) designation extends U.S. market exclusivity to 10 years from approval

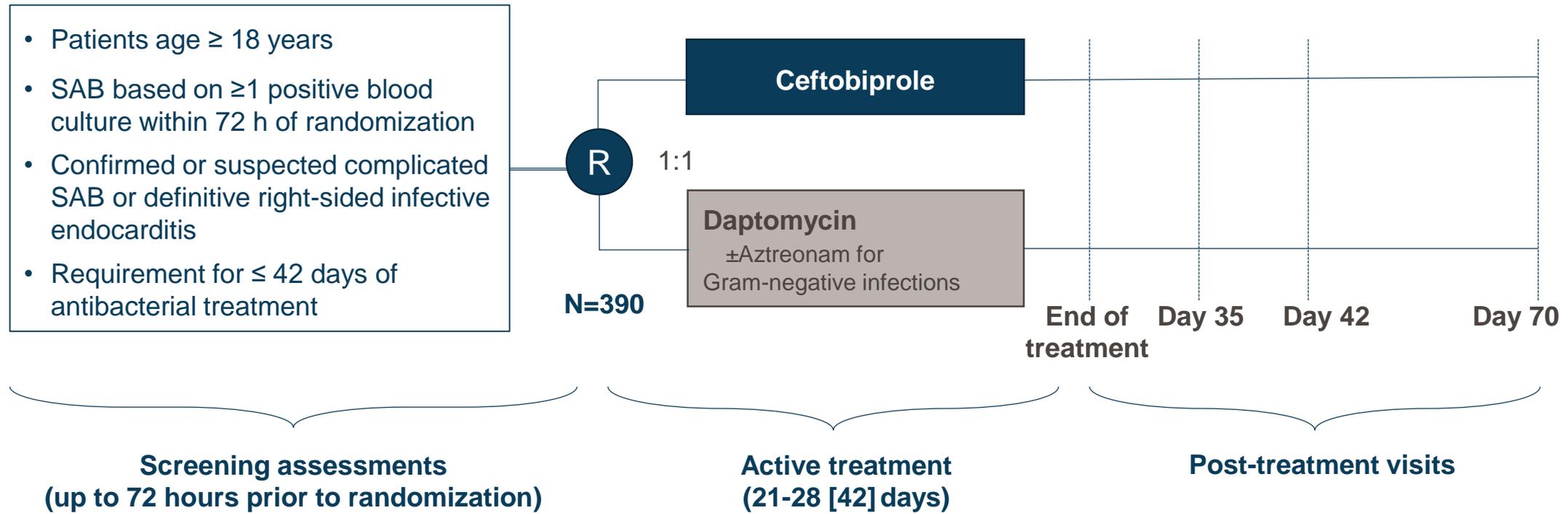
<sup>1</sup> Overcash JS et al. Clin Infect Dis. 2021;73:e1507-e1517. (NCT03137173)

<sup>2</sup> Hamed K et al. Future Microbiol. 2020;15:35-48. (NCT03138733)

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



ERADICATE is the largest randomized study conducted for registrational purposes of a new antibiotic treatment in *Staphylococcus aureus* bacteremia



Adapted from Hamed K et al. Future Microbiol. 2020;15:35-48

# SAB – an area with high medical need

- Nearly 120,000 *S. aureus* bloodstream infections in the U.S. (in 2017)<sup>1</sup>
- ERADICATE targets complicated SAB, characterized by concomitant or metastatic infections such as bone, joint or heart valve infections; persistent bacteremia; or bacteremia in patients on dialysis
- Substantial morbidity and approximately 20% 30-day mortality<sup>2</sup>
- Limited antibiotic treatment options with only two approved treatments for SAB in the U.S. that cover both MSSA and MRSA, i.e. vancomycin and daptomycin

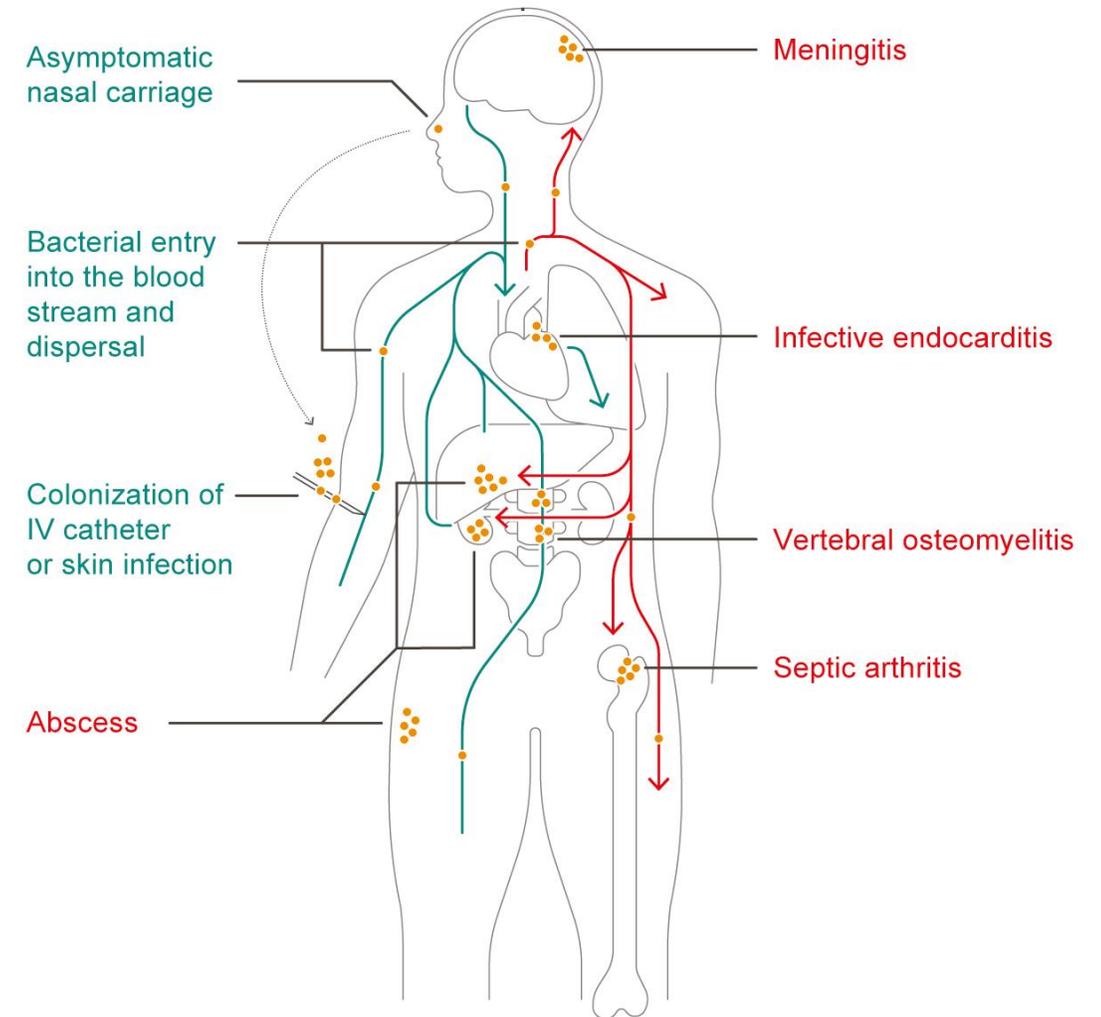
<sup>1</sup> MMWR, 2019;68:214–219.

<sup>2</sup> Hamed K et al. Future Microbiol. 2020;15:35-48.

MRSA: methicillin-resistant *Staphylococcus aureus*

MSSA: methicillin-susceptible *Staphylococcus aureus*

## Causes and consequences of SAB



Adapted from Edwards AM et al. Trends Microbiol. 2011;19:184-190.

# Ceftobiprole key attributes for SAB treatment

- Advanced generation cephalosporin with broad-spectrum bactericidal activity against Gram-positive organisms, including MRSA and MSSA, and Gram-negative organisms<sup>1</sup>
- Efficacy demonstrated in Phase 3 clinical studies in acute bacterial skin and skin structure infections, and pneumonia<sup>1,2</sup>
- Superior activity profile in multiple in vivo models of serious infection compared to vancomycin and daptomycin<sup>3</sup>
- Low propensity for resistance development<sup>1</sup>
- Established safety profile consistent with the cephalosporin class, demonstrated in both adult and pediatric patients<sup>1,2,4</sup>

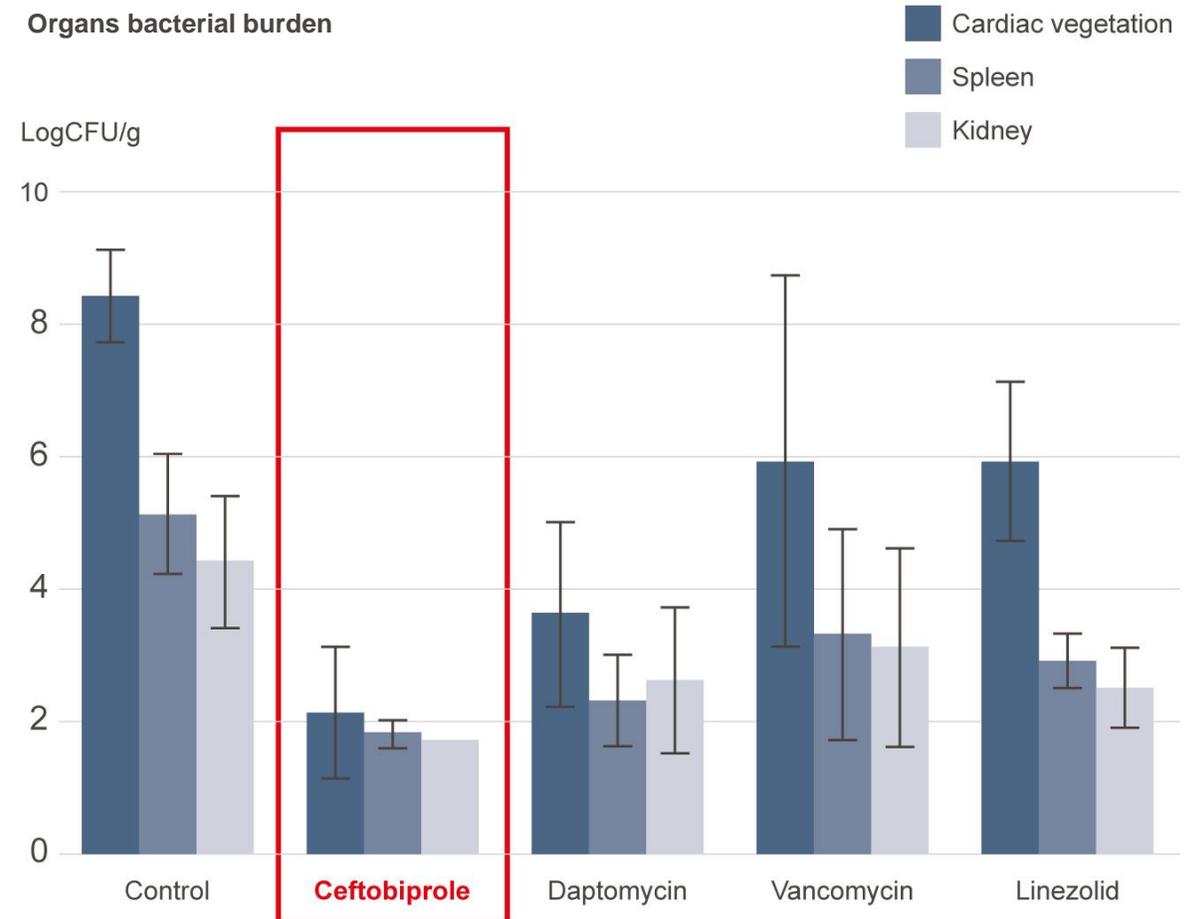
<sup>1</sup>Syed YY. *Drugs*. 2014;74:1523-1542.

<sup>2</sup>Overcash JS et al. *Clin Infect Dis*. 2021;73:e1507-e1517.

<sup>3</sup>Tattevin P et al. *Antimicrob Agents Chemother*. 2010;54:610-613.

<sup>4</sup>Rubino CM et al. *Pediatr Infect Dis J*. 2021;40:997-1003.

## Comparative efficacy in a rabbit model of endocarditis



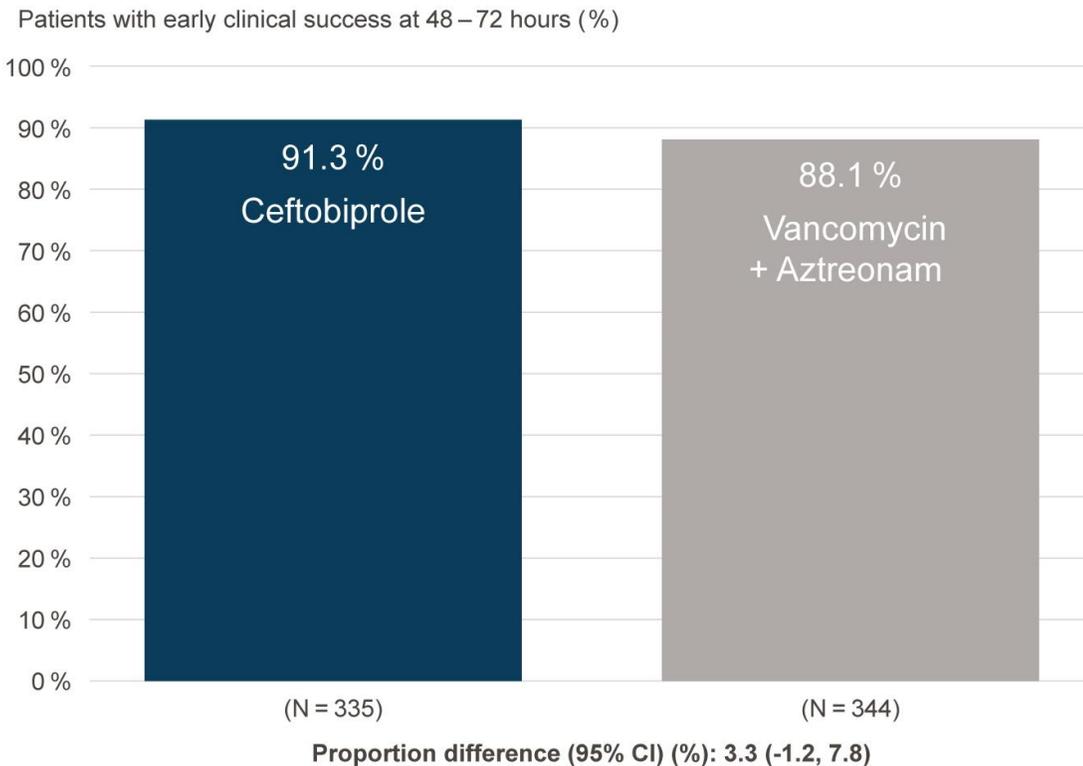
Organism titers in cardiac vegetations, spleens and kidneys of untreated and antibiotic treated rabbits infected with MRSA<sup>3</sup>

# Phase 3 study with ceftobiprole in the treatment of patients with ABSSSI

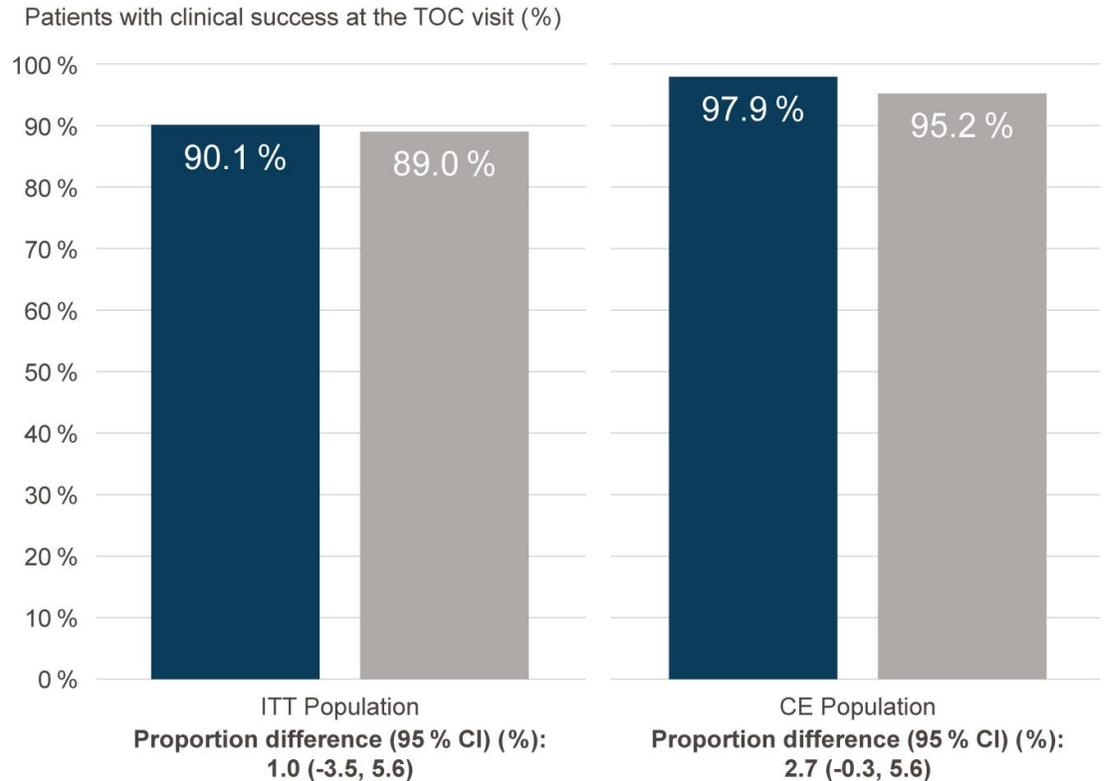


Demonstrated non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints

## Early clinical response at 48-72h after start of treatment (ITT population)



## Investigator-assessed clinical success at test-of-cure (TOC) 15-22 days after randomization (ITT, CE populations)



ITT, intent-to-treat  
Pre-defined limit of non-inferiority = lower limit of 95% CI for difference > -10%

A microscopic image showing a cluster of cells. The cells are generally spherical and have a granular, light-colored appearance. One cell in the lower-middle part of the image is highlighted with a bright yellow, textured overlay, suggesting it is the focus of the study. The background is a warm, orange-yellow color.

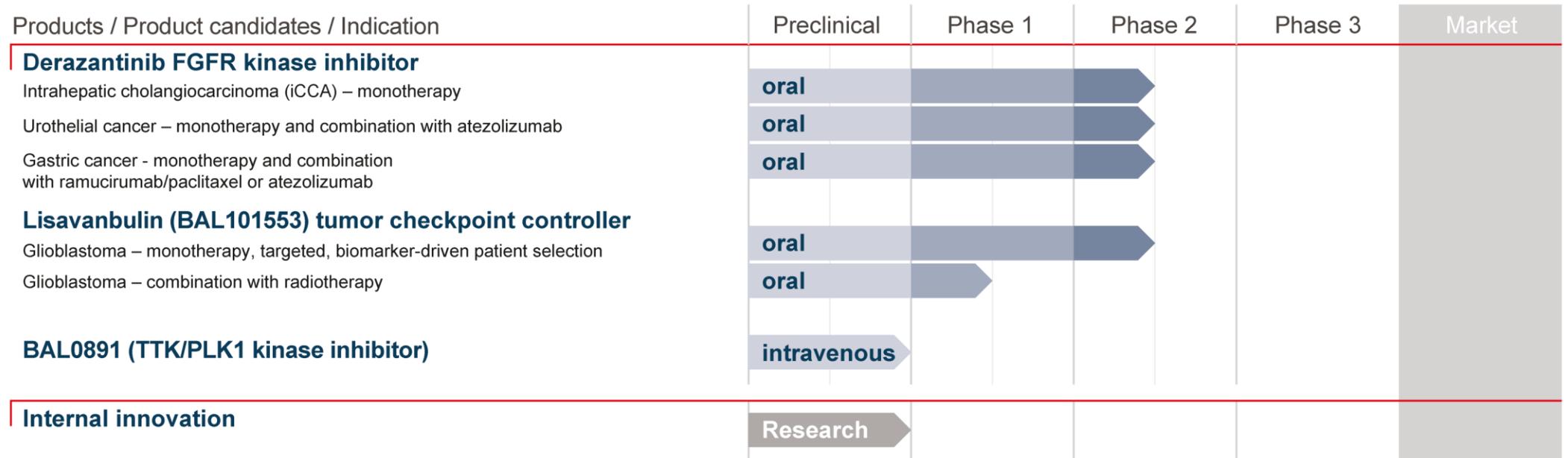
# Oncology

FGFR-driven tumors

EB1-positive glioblastoma

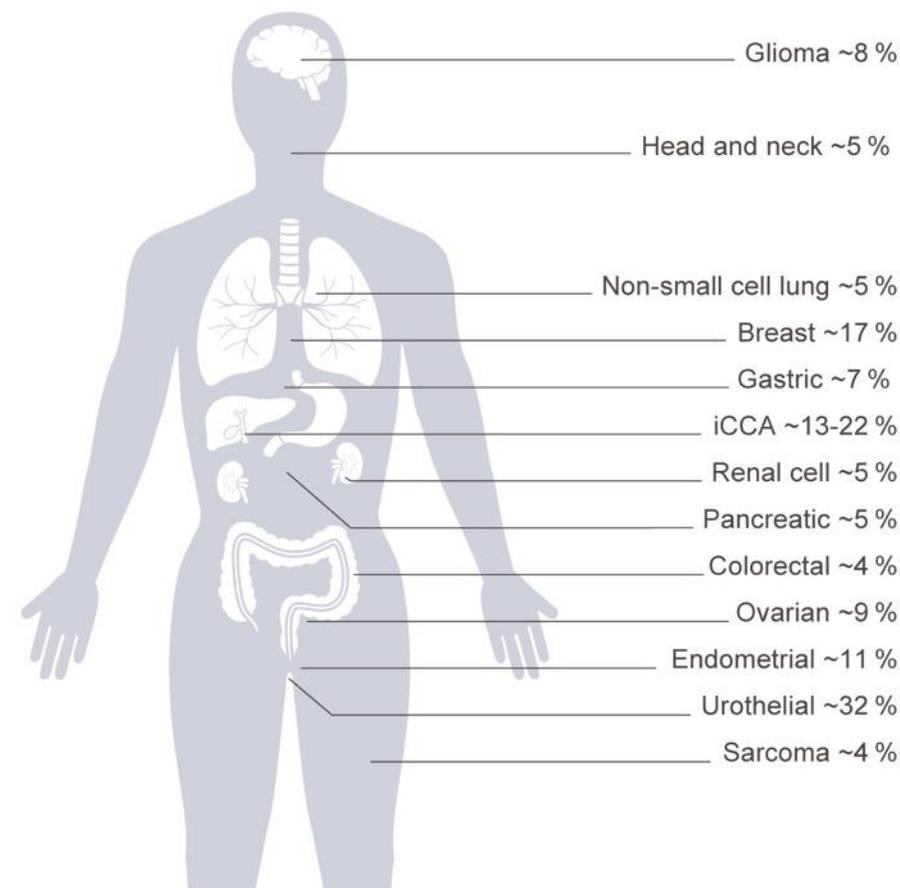
TTK/PLK1 kinase inhibition

# Pipeline: Oncology



# Targeting FGFR-driven tumors with derazantinib

- Small molecule, oral inhibitor of FGFR family of kinases
- Development strategy focused on achieving differentiation by leveraging unique properties of derazantinib
  - Kinase inhibition profile: potential advantage of additional targets of derazantinib such as CSF1R and VEGFR2 kinase
  - Safety profile: exploring relevance for potential combination therapies
- Focus on two clinical studies:
  - FIDES-01 (Ph 2) in intrahepatic cholangiocarcinoma (iCCA)
  - FIDES-03 (Ph 1/2) in gastric cancer



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

# Derazantinib clinical program in iCCA – FIDES-01

Phase 2 studies with FGFR-inhibitors in intrahepatic cholangiocarcinoma (iCCA)

Variable	Derazantinib <sup>1</sup> FIDES-01 Cohort 1	Infigratinib <sup>2</sup> (QED)	Pemigatinib <sup>3</sup> (Incyte) FIGHT-202	Futibatinib <sup>4</sup> (Taiho) FOENIX- CCA2
N	103	108	108	103
Objective response rate	21%	23%	37%	42%
Disease control rate	76 %	84%	82%	83%
Median progression-free survival	8.0 months	7.3 months	7.0 months	9.0 months

Derazantinib <sup>5</sup> FIDES-01 Cohort 2*	Pemigatinib <sup>6</sup> (Incyte) FIGHT-202
23	20
9%	0%
74%	40%
7.3 months	2.1 months

- FGFR2 fusions/rearrangements
- FGF/R non-fusion genetic alterations

- Derazantinib continues to show a well-manageable safety profile, with low rates of retinal side effects, stomatitis, hand-foot syndrome and nail toxicity.
- Overall, these results underscore the favorable benefit to risk profile of derazantinib as a monotherapy in iCCA (a form of bile duct cancer)

1. Droz Dit Busset et al., ESMO 2021 and Basilea data on file. 2. Javle et al. J Clin Oncol 39, no. 3\_suppl (January 20, 2021) 265-265. 3. Abou-Alfa et al. J Clin Oncol 39, no. 15\_suppl (May 20, 2021) 4086-4086.

4. Goyal et al. Cancer Res 2021; 81, 13 Supplement, pp. CT010. 5. Javle et al., J Clin Oncol 40, no. 4\_suppl (February 01, 2022) 427-427. 6. Abou-Alfa et al. Lancet Oncol 2020;21(5):671-684.

\*Interim analysis, based on investigator assessments.

# Derazantinib clinical program in gastric cancer – FIDES-03

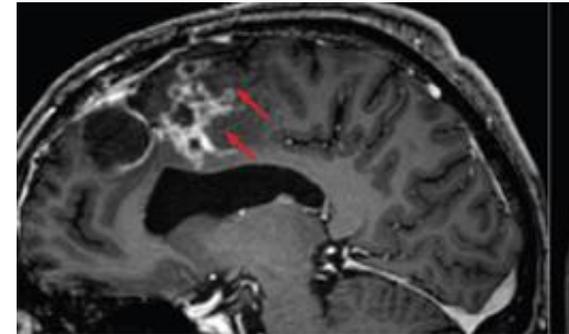
Multi-cohort Phase 1b/2 study of derazantinib as monotherapy or in combination therapy with standard of care (ramucirumab/paclitaxel) or atezolizumab in patients with advanced HER2-negative gastric adenocarcinoma harboring FGFR genetic aberrations

- Substudies using derazantinib monotherapy or combination treatment, including:
  - Derazantinib monotherapy in various molecular subtypes
  - Combination of derazantinib with ramucirumab/paclitaxel
  - Combination of derazantinib with atezolizumab
- Clinical supply agreement with Roche for atezolizumab
- Clinical trial collaboration and supply agreement with Lilly for ramucirumab
- Interim results in derazantinib monotherapy and recommended phase 2 dose of derazantinib in combination with ramucirumab/paclitaxel expected H1 2022

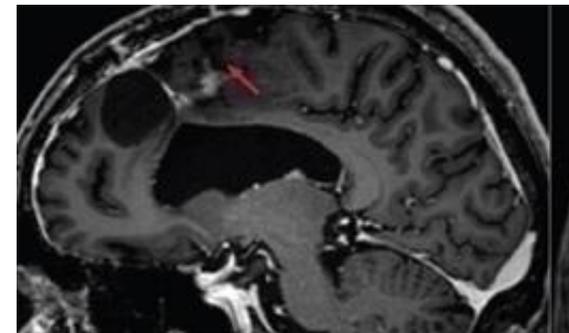
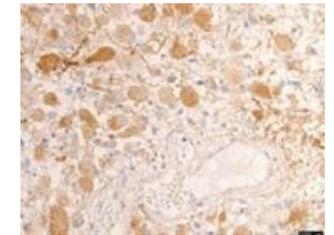
# Lisavanbulin biomarker-driven phase 2 study in recurrent glioblastoma

- EB1 is located on the microtubules, involved in microtubule dynamics and was shown a response predictive marker for lisavanbulin in preclinical studies
- Results from phase 1 study with daily oral lisavanbulin in patients with recurrent glioblastoma (n= 20):<sup>1, 2</sup>
  - Three patients with EB1-positive glioblastoma
  - Two of the EB1-positive patients with long-lasting clinical benefit, ongoing for more than 3 years
    - One exceptional response with >80% reduction in glioblastoma tumor size
  - No clear clinical benefit for EB1-negative patients
- Phase 2 interim results expected H1 2022

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



Baseline (May 2018)



Post Cycle 12 (April 2019)

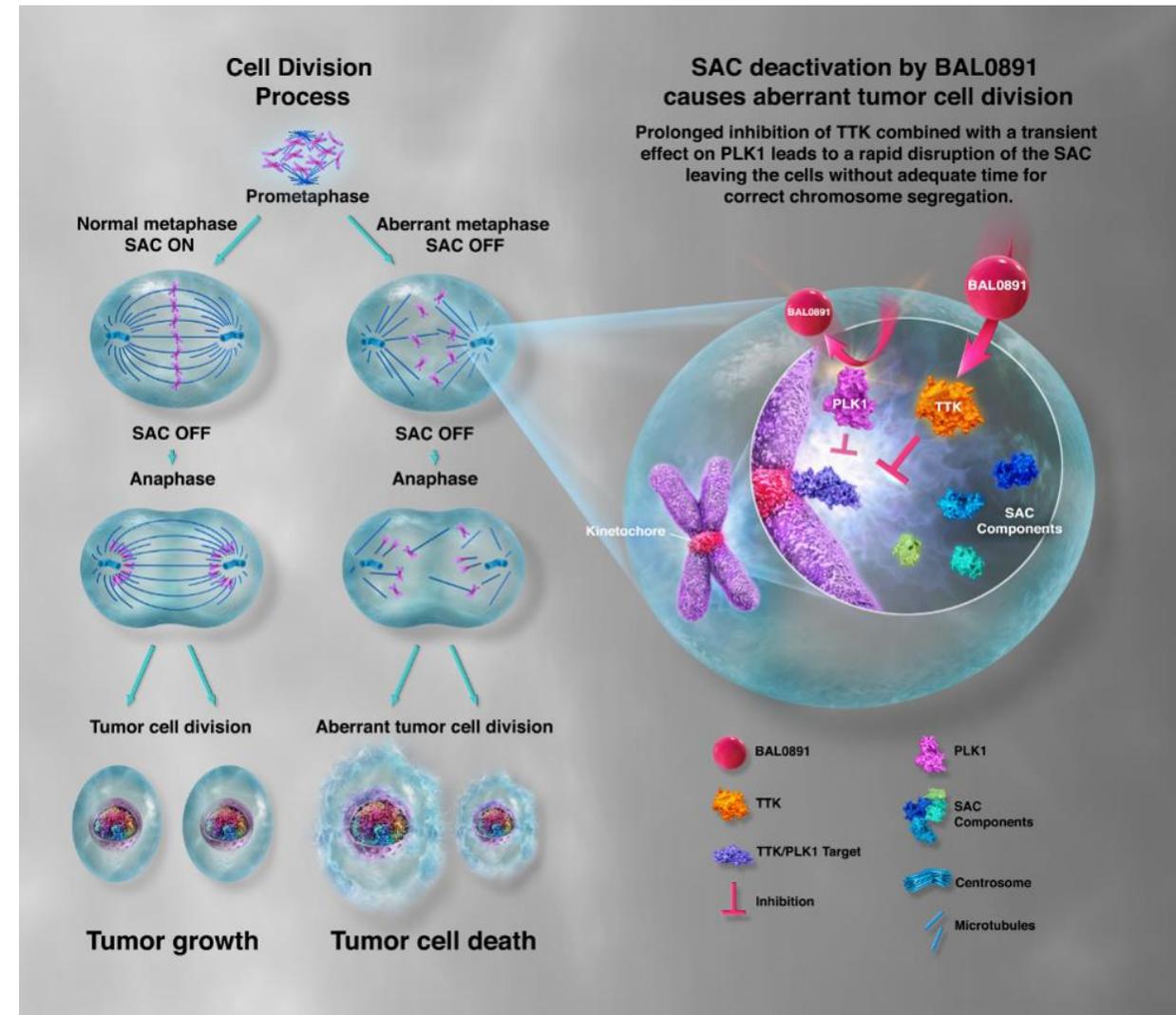


<sup>1</sup> Lopez et al. JCO 2019;37,15 suppl, 2025 (NCT02490800)

<sup>2</sup> Tiu et al. JCO 2021;39,15 suppl, TPS2068 (NCT02490800)

# BAL0891: a first-in-class mitotic checkpoint inhibitor

- Unique dual inhibitor of threonine tyrosine kinase (TTK) and polo-like kinase 1 (PLK1)
  - Dual action of BAL0891, with prolonged TTK and transient PLK1 inhibition, leads to a rapid disruption of the spindle assembly checkpoint (SAC)
  - Cells are pushed through mitosis without adequate time for correct chromosome alignment and segregation
  - Activity results in aberrant tumor cell division leading to tumor cell death
  - Potent single-agent anticancer activity in preclinical models of human cancer
- FDA approved IND in December 2021
- Preparing to enable start of phase 1 study in patients with solid tumors mid-2022



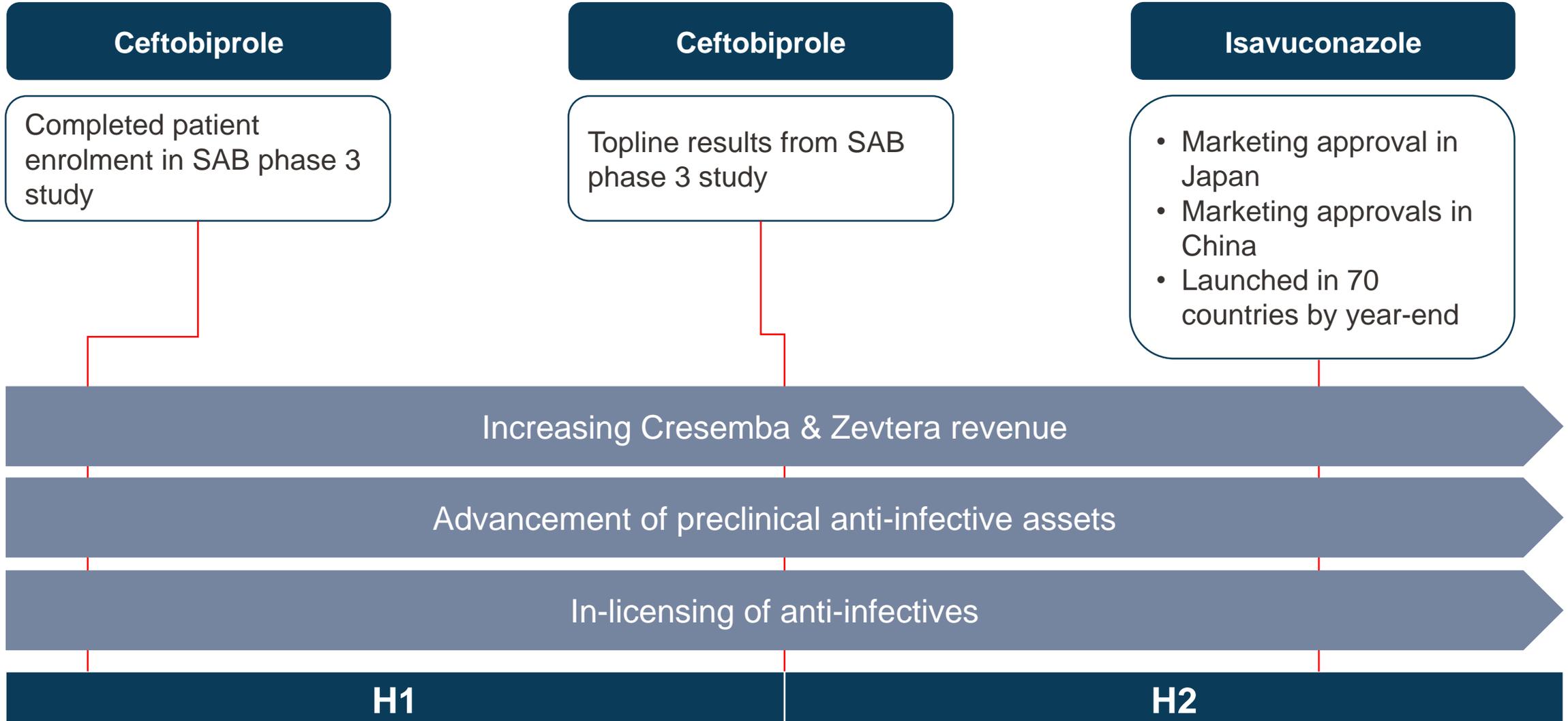
**David Veitch**

Chief Executive Officer

Outlook



# Outlook 2022: Anti-infectives



# Outlook 2022: Oncology

- Multiple data readouts for derazantinib
  - FIDES-01 (iCCA) cohort 2 topline results in FGFR2 non-fusion patients
  - FIDES-03 (gastric cancer) interim results monotherapy and combination treatment with ramucirumab/paclitaxel
- Lisavanbulin (EB1-positive recurrent glioblastoma) phase 2 interim and topline results
- BAL0891 (TTK/PLK1) preparing to enable start of a phase 1 study in mid-2022
- 2 preclinical oncology programs in development
- Strategic transactions in order to maximize the value of oncology portfolio



# Q & A



# Thank you

# Glossary

- ABSSSI: **A**cute **b**acterial **s**kin and **s**kin **s**tructure **i**nfections
- CARB-X: **C**ombating **A**ntibiotic-**R**esistant **B**acteria Biopharmaceutical **A**ccelerator
- CSF1R: **C**olony-**s**timulating **f**actor **1** receptor
- FGFR: **F**ibroblast **g**rowth **f**actor **r**eceptor
- FIDES: **F**ibroblast growth factor inhibition with **d**erazantinib in **s**olid tumors
- iCCA: **I**ntrahepatic **c**holangi**c**arcinoma
- IND: **I**nvestigational **n**ew **d**rug
- MSSA: **M**ethicillin-**s**usceptible ***S**ta**p**hylococcus **a**ureus*
- MRSA: **M**ethicillin-**r**esistant ***S**ta**p**hylococcus **a**ureus*
- NDA: **N**ew **d**rug **a**pplication
- ORR: **O**bjective **r**esponse **r**ate
- PFS: **P**rogression-**f**ree **s**urvival
- PLK1: **P**olo-**l**ike **k**inase **1**
- SAB: ***S**ta**p**hylococcus **a**ureus* **b**acteremia
- SAC: **S**pindle **a**ssembly **c**heckpoint
- TTK: **T**hreonine **t**yrosine **k**inase
- VEGFR2: **V**ascular **e**ndothelial **g**rowth **f**actor **r**eceptor **2**



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