



Creating anti-infective opportunities

“Patients are at the heart of what we do”

Investor presentation

April 9, 2024



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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



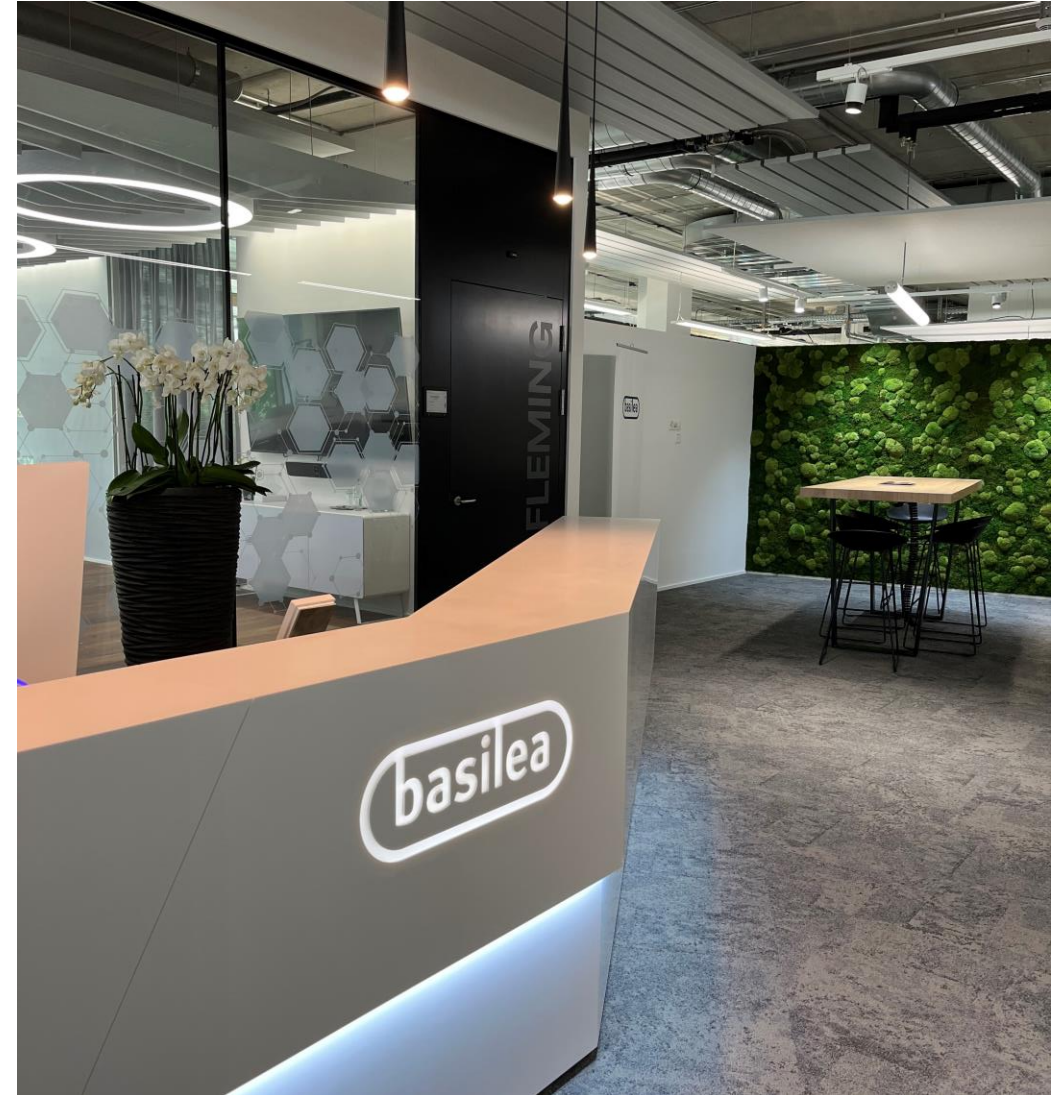
Laurenz Kellenberger Ph.D. CSO

2000

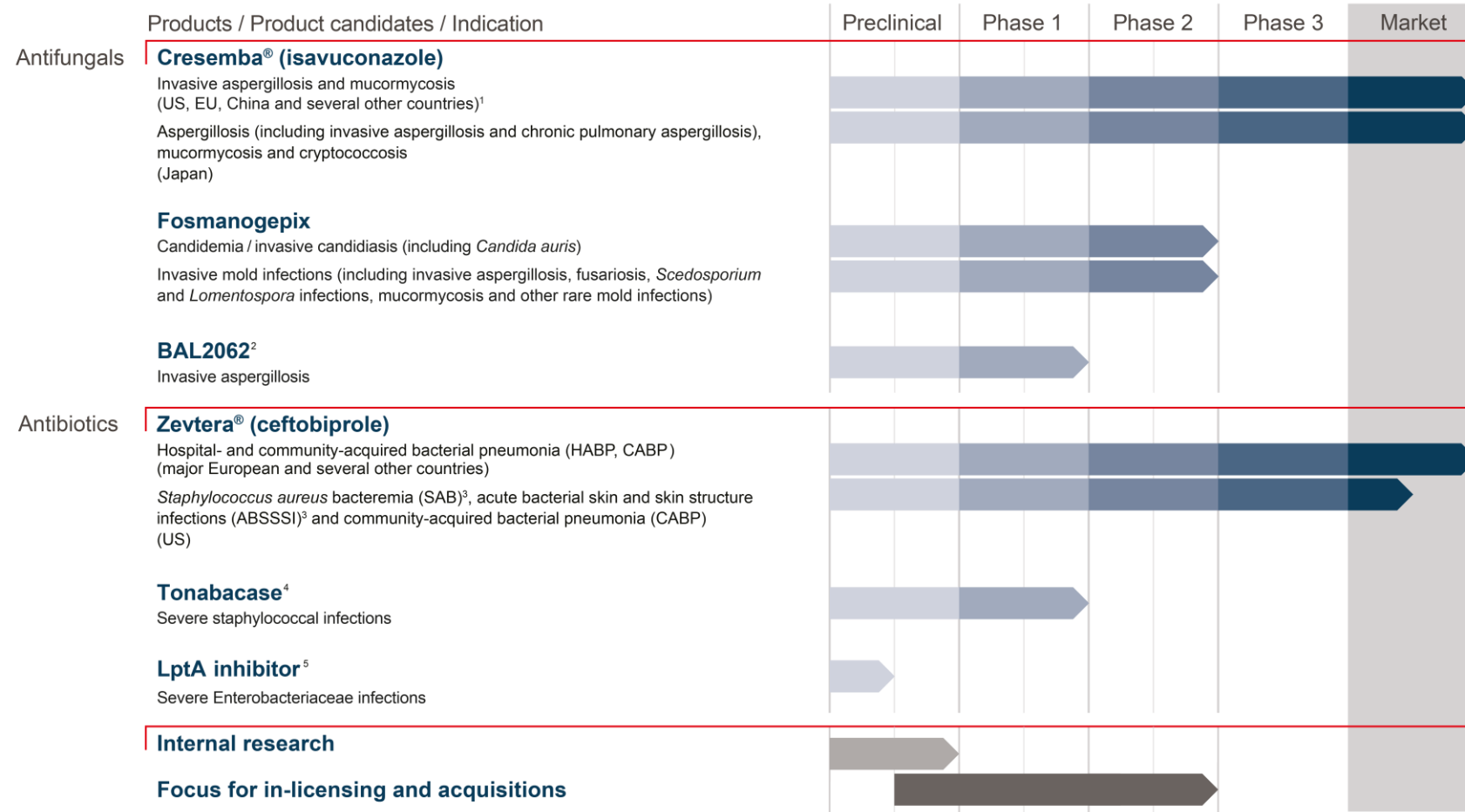


At a glance

- Focus on the treatment of severe bacterial and fungal infections
- Recognized ability to establish and manage partnerships in both the development and commercial phase
- Cresemba® and Zevtera® – two revenue generating hospital anti-infective brands
- Commercial products complemented by clinical and preclinical programs, including
 - **Fosmanogepix**, a phase-3-ready broad-spectrum antifungal
 - **BAL2062**, an antifungal for the potential treatment of invasive aspergillosis
 - **Tonabacase**, an endolysin antibacterial for the potential treatment of severe staphylococcal infections
- Profitable biotech company listed on SIX Swiss Stock Exchange, SIX: BSLN
- Located in the Basel area life sciences hub, Switzerland



Innovative anti-infective pipeline



1 The registration status and approved indications may vary from country to country.

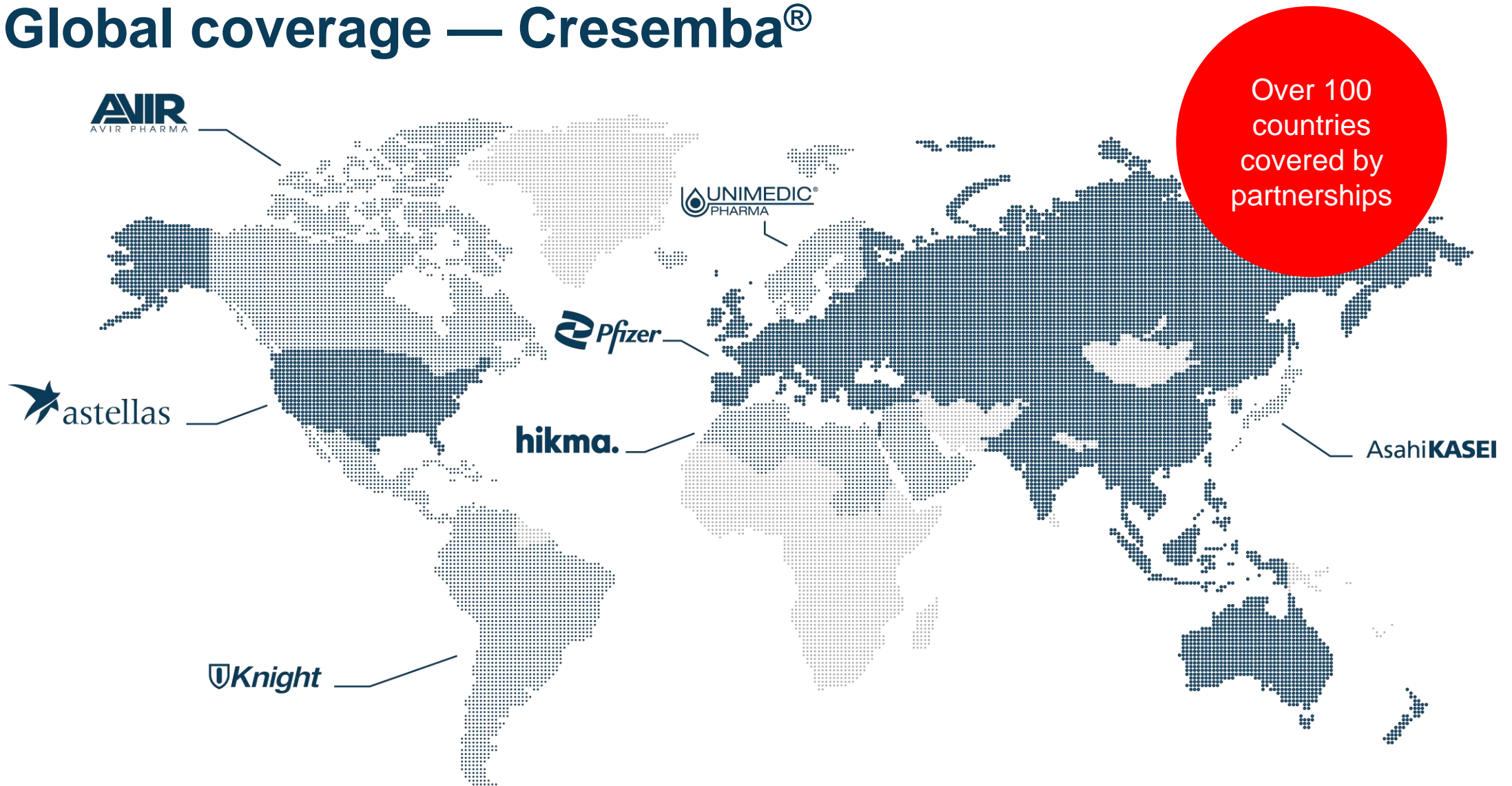
2 Formerly GR-2397

3 Phase 3 program was funded in part with federal funds from the US Department of Health and Human Services (HHS); Administration for Strategic Preparedness and Response (ASPR); Biomedical Advanced Research and Development Authority (BARDA).

4 Exclusive option to in-license upon completion of preclinical profiling

5 CARB-X's funding for this project is provided in part with federal funds from the US Department of Health and Human Services (HHS); Administration for Strategic Preparedness and Response; Biomedical Advanced Research and Development Authority; Antibacterials branch; under agreement number 75A50122C00028; and by awards from Wellcome (WT224842) and Germany's Federal Ministry of Education and Research (BMBF).

Global coverage — Cresemba®



The company we keep — Established strong partnerships

License partners



Europe (excl. Nordics), China
Asia-Pacific, Russia, Turkey
and Israel (Cresemba®)



US (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

>CHF 1 bn
in potential
milestones
remaining

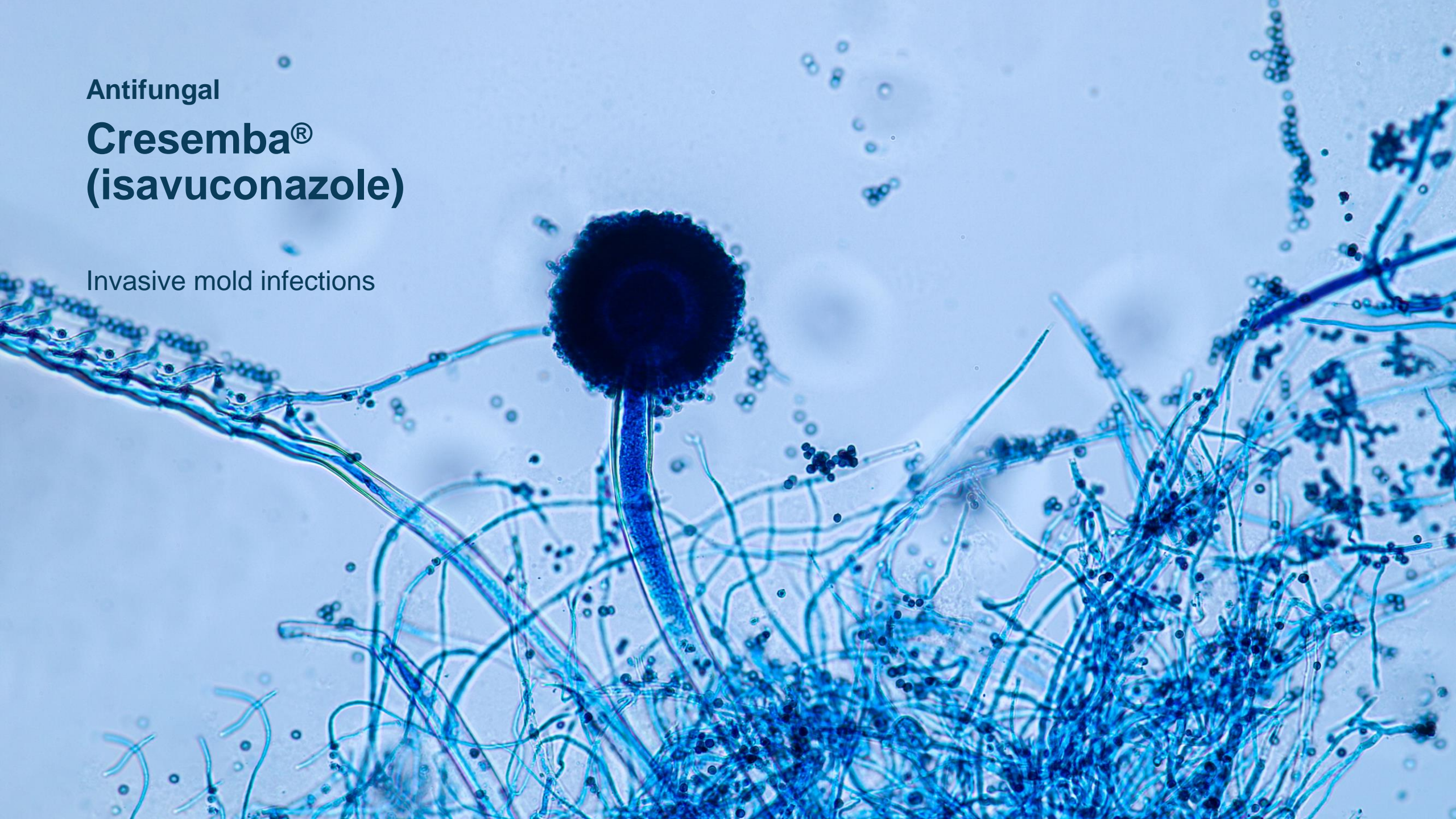
Participation
in sales of
distribution
partners
through
transfer price

>CHF 355 mn
upfront and
milestone
payments
received

Antifungal

**Cresemba®
(isavuconazole)**

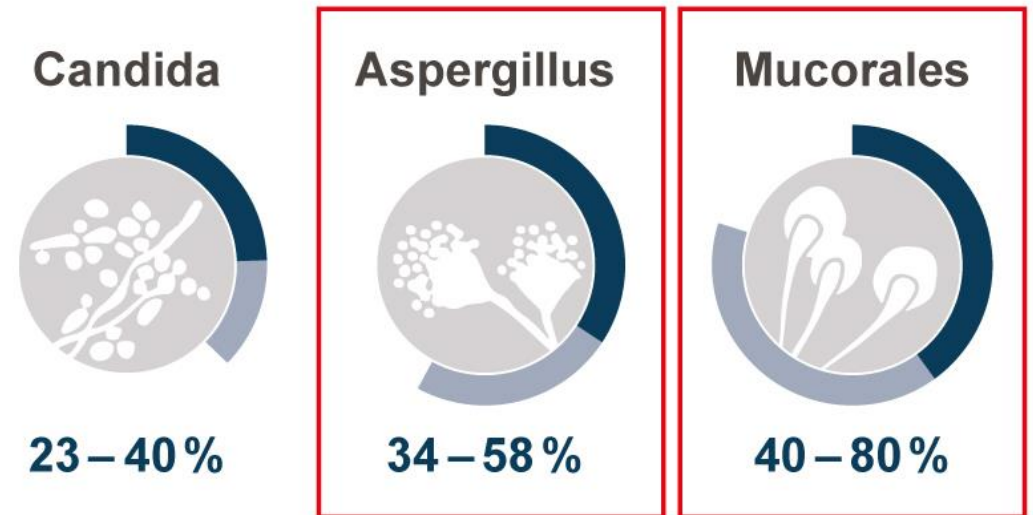
Invasive mold infections



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

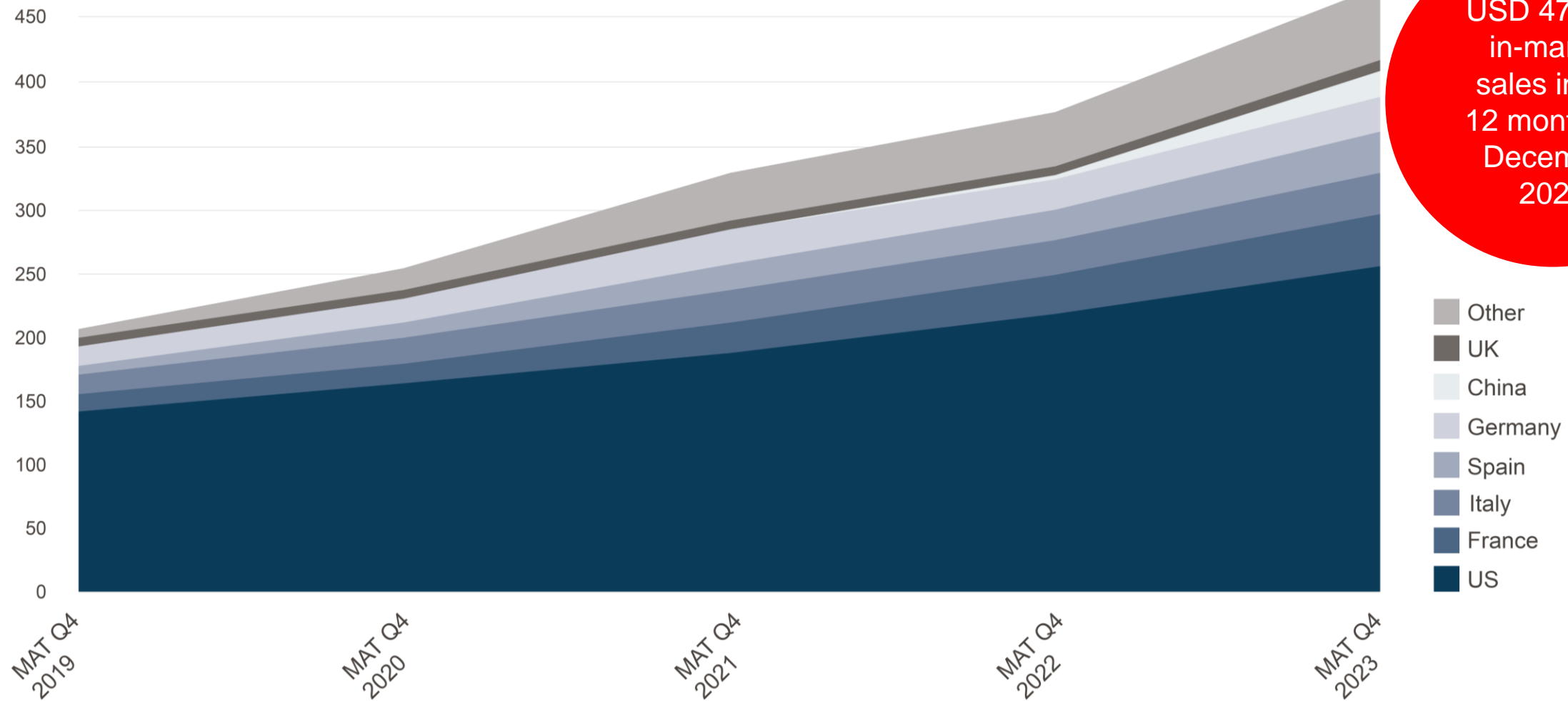
Mortality rates for invasive fungal infections**



**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

Sales in USD mn



USD 473 mn
in-market
sales in the
12 months to
December
2023

MAT: Moving annual total; Source: IQVIA Analytics Link, December 2023

Global sales of best-in-class antifungals* by product

USD 2.9 bn sales (MAT Q4 2023)

Significant potential to increase Cresemba® (isavuconazole) global market share

- Launched in 71 countries
- Pediatric label extension in US granted in December 2023; market exclusivity extended to September 2027
- Pediatric label extension in EU anticipated in 2024; would lead to market exclusivity extension by two years to October 2027

* Best-in-class antifungals: Cresemba (isavuconazole), posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin, rezafungin

VFEND (VORICONAZOLE)
2014 worldwide peak sales
approx. USD 900 mn



MAT: Moving annual total; Source: IQVIA Analytics Link, December 2023, rounding consistently applied

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.

Antifungal
Fosmanogepix

Invasive yeast and mold infections

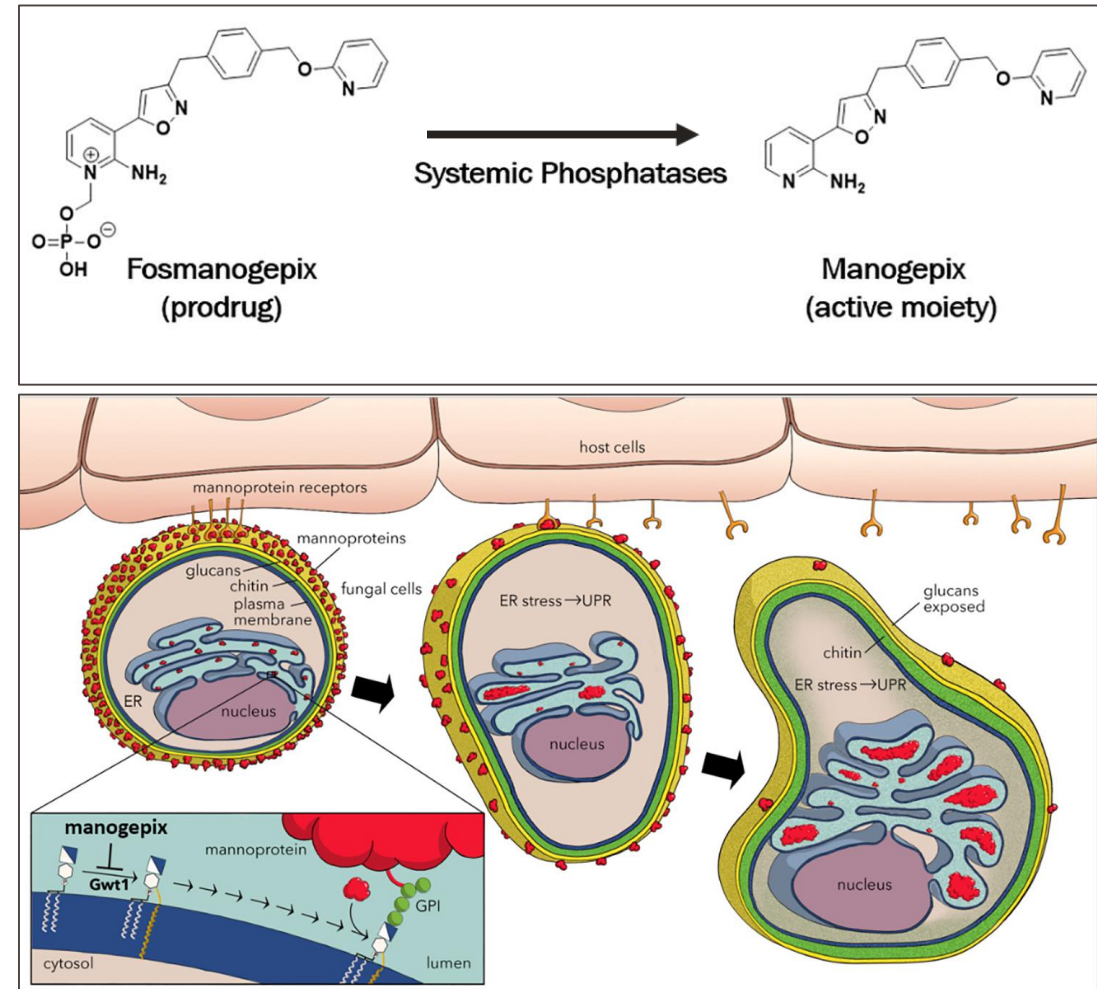


Fosmanogepix – A highly attractive antifungal asset

- First-in-class, intravenous and oral antifungal with a novel mechanism of action
- Broad spectrum antifungal activity against yeasts, molds and dimorphic fungi, including *Candida auris*, azole-resistant *Aspergillus* spp. and *Fusarium* spp.
- Three successfully completed phase 2 studies for the treatment of
 - Candidemia, including *Candida auris*
 - Mold infections
- Phase-3-ready for yeast and mold infections with first phase 3 study in candidemia / invasive candidiasis expected to start mid-2024
- Potential to become our next lead commercial product and mid-term value driver
- Asset acquired from Pfizer, which maintains the right of first negotiation for commercialization

Overview

- Fosmanogepix is the prodrug of manogepix
- Novel mechanism of action
- Inhibition of the protein Gwt1 impedes the production of cell wall mannoproteins, causing cell wall fragility, fungal cell death and decreased potential for biofilm formation
- Potent broad-spectrum activity against resistant yeasts, molds and dimorphic fungi, including azole-resistant phenotypes
- IV and oral availability enables treatment in both inpatient and outpatient settings
- US FDA fast track status, QIDP and orphan drug designations



Friedman DZP, Schwartz IS. Infect Dis Clin North Am. 2023;37:593-616.

Shaw KJ, Ibrahim AS. J Fungi (Basel). 2020; 6:239

Addressing high unmet medical needs

- Fast track status by the US FDA for invasive candidiasis, invasive aspergillosis, scedosporiosis, fusariosis, mucormycosis, cryptococcosis, and coccidioidomycosis
- Addressing emerging resistance issues in yeast infections including *Candida auris* and azole resistant *Aspergillus* spp.
- Potent activity against mold infections including difficult-to-treat *Fusarium* and *Scedosporium* spp.
- Wide tissue distribution enabling treatment of disseminated infections including CNS
- Favorable drug-drug interaction profile
- *In-vivo* synergism with liposomal amphotericin B and echinocandins may provide utility for the most difficult-to-treat infections

Hoenigl M, Sprute R, Egger M, et al. *Drugs*. 2021;81:1703-1729.

Winston DJ, Young PA, Schlamm HT, Schiller GJ. *Clin Infect Dis*. 2023:ciad309.

Gebremariam T, Gu Y, Alkhazraji S, et al. *Antimicrob Agents Chemother*. 2022;66:e0038022.

Addressing high unmet medical needs (cont)

	Fosmanogepix IV and Oral	Ibrexafungerp Oral	Olorofim Oral	Rezafungin IV	
Fungal pathogens					
<i>Candida spp.*</i>	Potent activity	Potent activity	No activity	Potent activity	
<i>Aspergillus spp.†</i>	Potent activity	Potent activity	Potent activity	Potent activity	
<i>Mucorales‡</i>	Variable activity	No activity	No activity		
<i>Fusarium spp.</i>	Potent activity	No activity	Variable activity		
<i>Scedosporium spp.</i>	Potent activity	Variable activity	Potent activity		
<i>Lomentospora spp.</i>	Potent activity	Variable activity	Potent activity		
<i>Cryptococcus spp.</i>	Potent activity		No activity	No activity	
Endemic molds§	Potent activity	Potent activity	Potent activity		
Other rare molds¶	Variable activity, Potent activity, Potent activity, Potent activity, Potent activity	Potent activity, Potent activity, Variable activity, No activity, No activity	No activity, Potent activity, Variable activity, Potent activity, Potent activity		
Other rare yeasts¶¶	Potent activity		No activity		

* including *C. albicans*, *C. auris*, *C. dubliniensis*, *C. glabrata*, *C. krusei*, *C. lusitanae*, *C. parapsilosis*, *C. tropicalis*. Fosmanogepix not active against *C. krusei*.
 † including *A. calidoustus*, *A. fumigatus* (including azole-resistant), *A. flavus*, *A. lentulus*, *A. nidulans*, *A. niger*, *A. terreus*, *A. tubingensis*.
 ‡ including *Cunninghamella spp.*, *Lichtheimia spp.*, *Mucor spp.*, *Rhizopus spp.*
 § including *Blastomyces dermatitidis*, *Coccidioides immitis*, *Histoplasma capsulatum*.
 ¶ including *Alternaria alternata*, *Cladosporium spp.*, *Paecilomyces variotii*, *Purpureocillium lilacinum*, *Scopulariopsis spp.*, *Rasamsonia spp.*
 ¶¶ including *Trichosporon asahii*, *Exophiala dermatitidis*, *Malassezia furfur*.

Adapted from Hoenigl M, Sprute R, Egger M et al. *Drugs*. 2021;81:1703-1729.

Proprietary information of Basilea Pharmaceutica International Ltd, Allschwil – not for distribution

Planned global phase 3 program

Candidemia / Invasive candidiasis

- Randomized, double-blind, non-inferiority study
 - Approximately 450 patients
- Fosmanogepix IV (oral step-down fosmanogepix) vs caspofungin IV (oral step-down to fluconazole)
- Primary endpoints
 - FDA: Survival at 30 days
 - EMA: Overall response at end-of-study treatment
- Protocol and initial Health Authority approvals obtained
- Expected study start mid-2024

Invasive mold infections (IMI)

- Randomized, open-label study including non-controlled salvage treatment arm
 - Approximately 200 patients
- Cohorts of invasive mold disease including IMI caused by:
 - *Aspergillus* spp.
 - *Fusarium* spp.
 - *Scedosporium* spp.
 - *Lomentospora prolificans*
 - Mucorales fungi, or
 - Other multi-drug resistant molds
- Fosmanogepix IV or oral vs best available therapy
- Endpoints include survival and overall response
- Expected study start end-2024

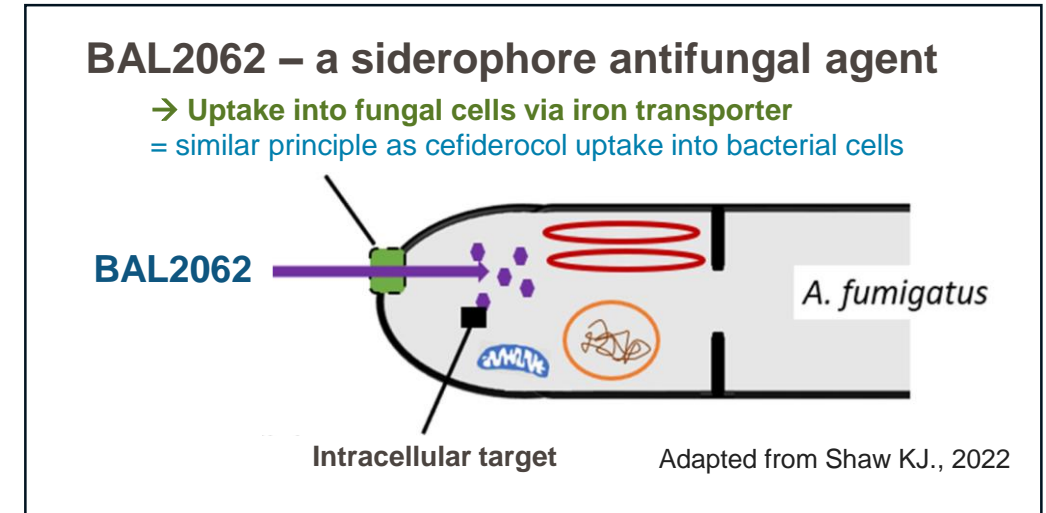
Antifungal
BAL2062

Invasive *Aspergillus* infections



Profile of BAL2062

- First-in class antifungal with novel mechanism of action for intravenous administration
- Rapid fungicidal activity in vitro against *Aspergillus* spp.¹
- Lack of cross resistance with marketed antifungal agents²
- Active against azole- and amphotericin B-resistant *Aspergillus* spp.²
- Active in invasive pulmonary aspergillosis animal models¹
- Low propensity for CYP-450 mediated drug-drug interactions²

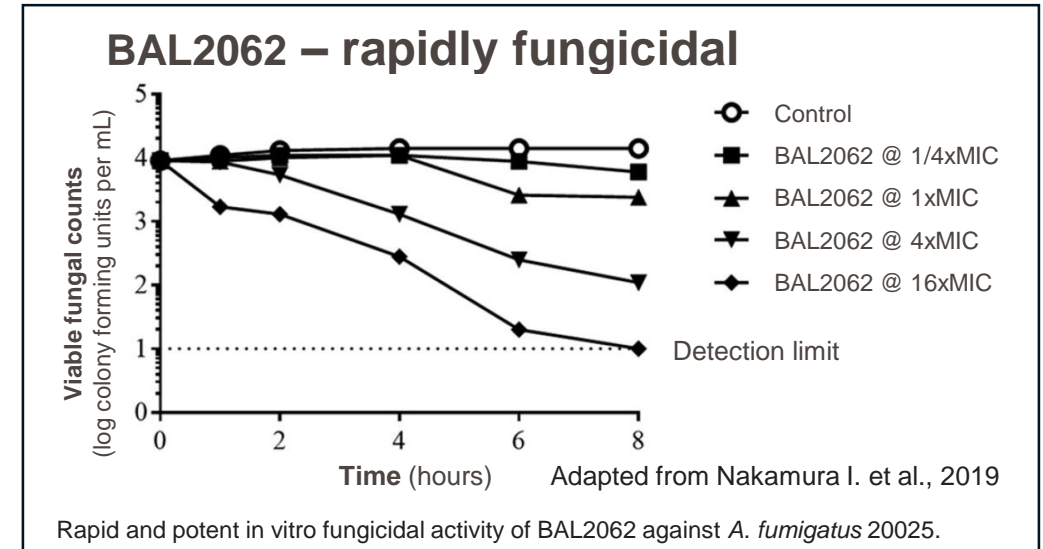


¹ Nakamura I, Ohsumi K, Takeda S, et al. Antimicrob Agents Chemother. 2019;63:e02689-18.

² Shaw KJ. J Fungi (Basel). 2022; 8:909.

Profile of BAL2062 cont.

- Clinical safety and tolerability demonstrated in phase 1 study¹
- Potential for enhanced clinical efficacy addressing unmet medical needs in invasive aspergillosis and other invasive fungal infections
- Plan to start clinical phase 2 program in H1 2025 based on results from additional preclinical profiling studies
- QIDP, Orphan Drug and Fast Track designations granted from the FDA for invasive aspergillosis²



¹ Mammen MP, Armas D, Hughes FH, et al. Antimicrob Agents Chemother. 2019;63:e00969-19.

² Shaw KJ. J Fungi (Basel). 2022; 8:909.

Antibacterial
Zevtera[®]
(ceftobiprole)

Severe bacterial infections



Zevtera[®] — An introduction

- Broad-spectrum hospital anti-MRSA cephalosporin (including Gram-negative bacteria)
 - Rapid bactericidal activity
 - Potential to replace antibiotic combinations
 - Efficacy demonstrated in phase 3 clinical studies in SAB, ABSSSI and pneumonia^{1, 2, 3}
 - Low propensity for resistance development¹
 - Safety profile consistent with the cephalosporin class safety profile, demonstrated in both adult and pediatric patients^{1, 2, 3, 4}
- Marketed in selected countries in Europe, Latin America, the MENA-region and Canada
- US FDA approval in April 2024

Approved in major European countries & several non-European countries for both hospital-acquired bacterial pneumonia (HABP), excluding ventilator-associated pneumonia (VAP), and community-acquired bacterial pneumonia (CABP). Indicated in the US for the treatment of adult patients with Staphylococcus aureus bloodstream infections (bacteremia) (SAB), including right-sided infective endocarditis, and adult patients with acute bacterial skin and skin structure infections (ABSSSI) and for adult and pediatric patients (3 months to less than 18 months old) with community-acquired bacterial pneumonia (CABP).



¹ Syed YY. Drugs. 2014;74:1523-1542 and Basilea data on file.

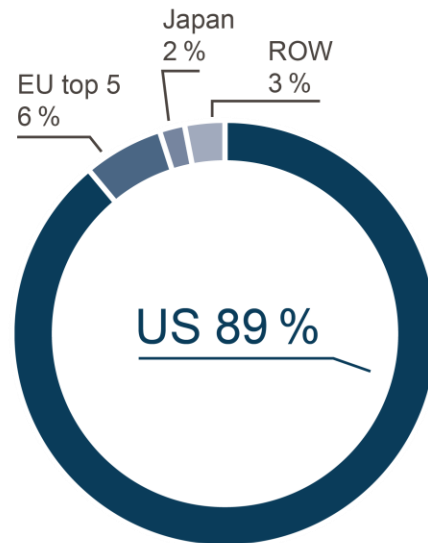
² Overcash JS et al. Clin Infect Dis. 2021;73:e1507-e1517.

³ Holland TL et al. N Engl J Med 2023;389:1390-1401.

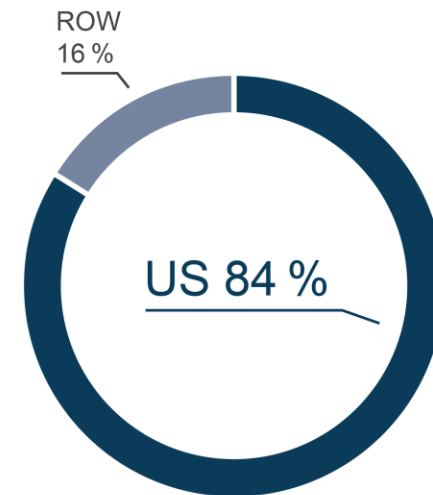
⁴ Rubino CM et al. Pediatr Infect Dis J. 2021;40:997-1003.

The hospital anti-MRSA antibiotic market — A USD 2.4 bn market* with the US being the most important region

Daptomycin sales by region
(2015, before LOE)



Ceftaroline sales by region
(MAT Q4 2023)



* Vancomycin, linezolid, teicoplanin, daptomycin, tigecycline, telavancin, ceftaroline, dalbavancin, ceftobiprole, oritavancin and tedizolid (daptomycin and tigecycline are partial sales in the US in IQVIA data)

MRSA: Methicillin-resistant *Staphylococcus aureus*; LOE: Loss of exclusivity; ROW: Rest Of World; MAT: Moving annual total; Source: IQVIA Analytics Link, December 2023

Ceftobiprole — Strategy for accessing the US market

- FDA approved three indications on April 3, 2024:
 1. *Staphylococcus aureus* bacteremia (SAB)¹, including right-sided endocarditis
 2. Acute bacterial skin and skin structure infections (ABSSSI)²
 3. Community-acquired bacterial pneumonia (CABP, adult and pediatric)³
- Phase 3 program largely funded by BARDA (~USD 112 million, or approximately 75 percent of the costs related to the SAB and ABSSSI phase 3 studies, regulatory activities and non-clinical work)
- Qualified Infectious Disease Product (QIDP) designation extends US market exclusivity to 10 years from approval
- Commercialization planned through partnership
 - Partnership expected around mid-year 2024



¹ Holland TL et al. N Engl J Med 2023;389:1390-1401.

² Overcash JS et al. Clin Infect Dis. 2021;73:e1507-e1517.

³ Nicholson SC et al. International Journal of Antimicrobial Agents 2012 (39), 240-246.

SAB – An area with high medical need

- Nearly 120,000 *S. aureus* bloodstream infections in the US (in 2017)¹
- 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 mortality²
- Limited antibiotic treatment options with only two approved treatments for SAB in the US that cover both MSSA and MRSA, i.e. vancomycin and daptomycin

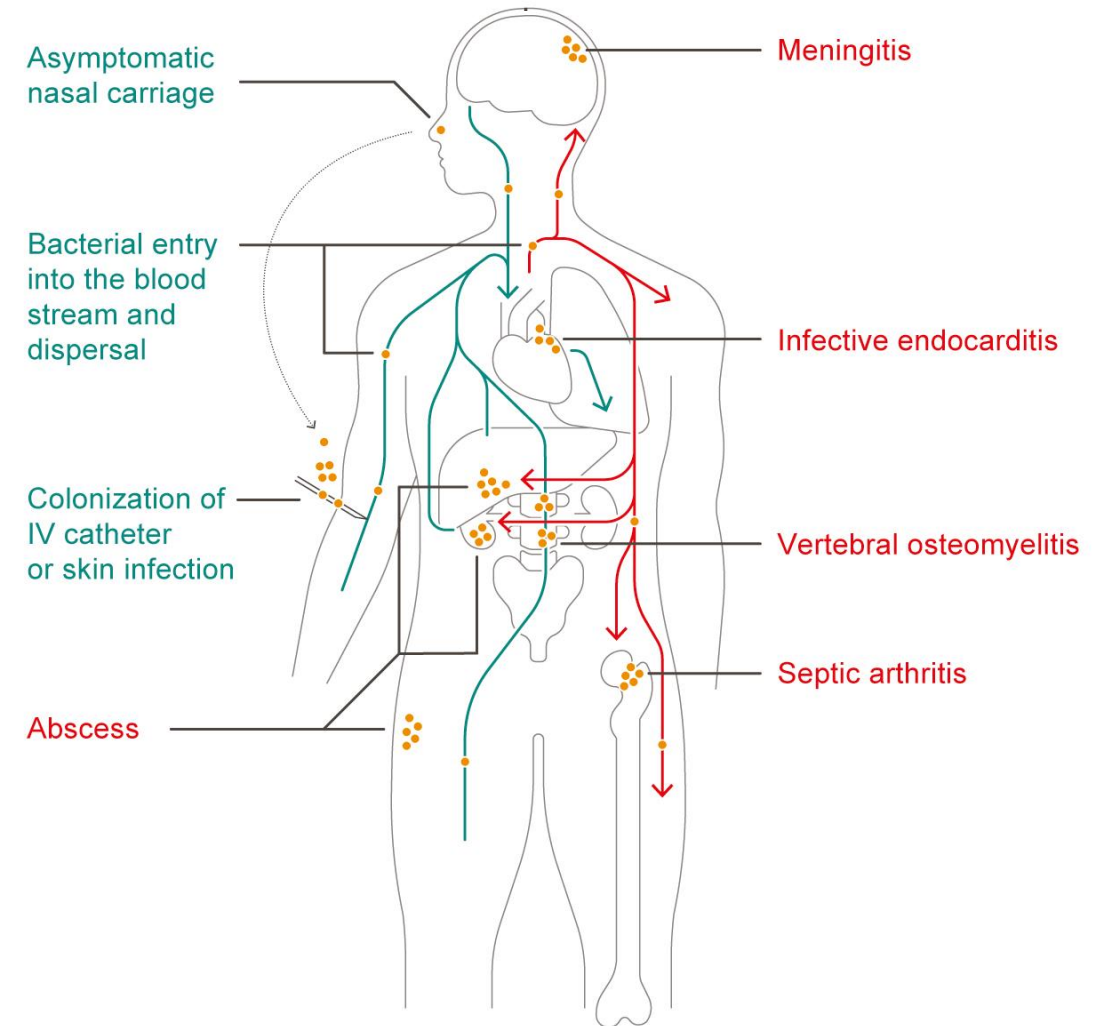
¹ MMWR, 2019;68:214–219.

² 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.

ERADICATE — The largest phase 3 registrational study conducted in SAB¹

- ERADICATE is the largest phase 3 study conducted for registrational purposes of a new antibiotic treatment in *Staphylococcus aureus* bacteremia.
- The randomized, double-blind, multicenter phase 3 study was a global study performed in 60 study centers in 17 countries from August 2018 to March 2022.
- 390 patients were randomized to ceftobiprole or daptomycin, with or without intravenous aztreonam for coverage of Gram-negative pathogens, for up to 42 days of treatment.
- Patient characteristics in the 387 patients included in the modified intent-to-treat (mITT) population were balanced between the treatment groups.
- Primary objective of demonstrating non-inferiority compared to daptomycin was achieved, similar outcomes observed for secondary endpoints.

¹ Holland TL et al. N Engl J Med 2023;389:1390-1401.

Ceftobiprole — Place in therapy

- Excellent treatment option in difficult-to-treat patients presenting to the hospital with severe infections, especially when the clinician suspects involvement of Gram-positive pathogens including *Staphylococcus aureus*
- Single agent first-line bactericidal broad-spectrum therapy with proven efficacy in SAB, ABSSSI and CABP, enabling to treat these vulnerable patients effectively early in their disease to achieve recovery
- Ceftobiprole is differentiated versus competitors in various clinically important aspects, including:
 - The strong, bactericidal activity against MSSA and MRSA
 - A robust Gram-negative coverage
 - Efficacy demonstrated in pulmonary infections in phase 3 studies
 - The safety profile reflecting the cephalosporin class
 - The low propensity for resistance development

Focused launch in area of highest unmet medical need with opportunities for broader utilization

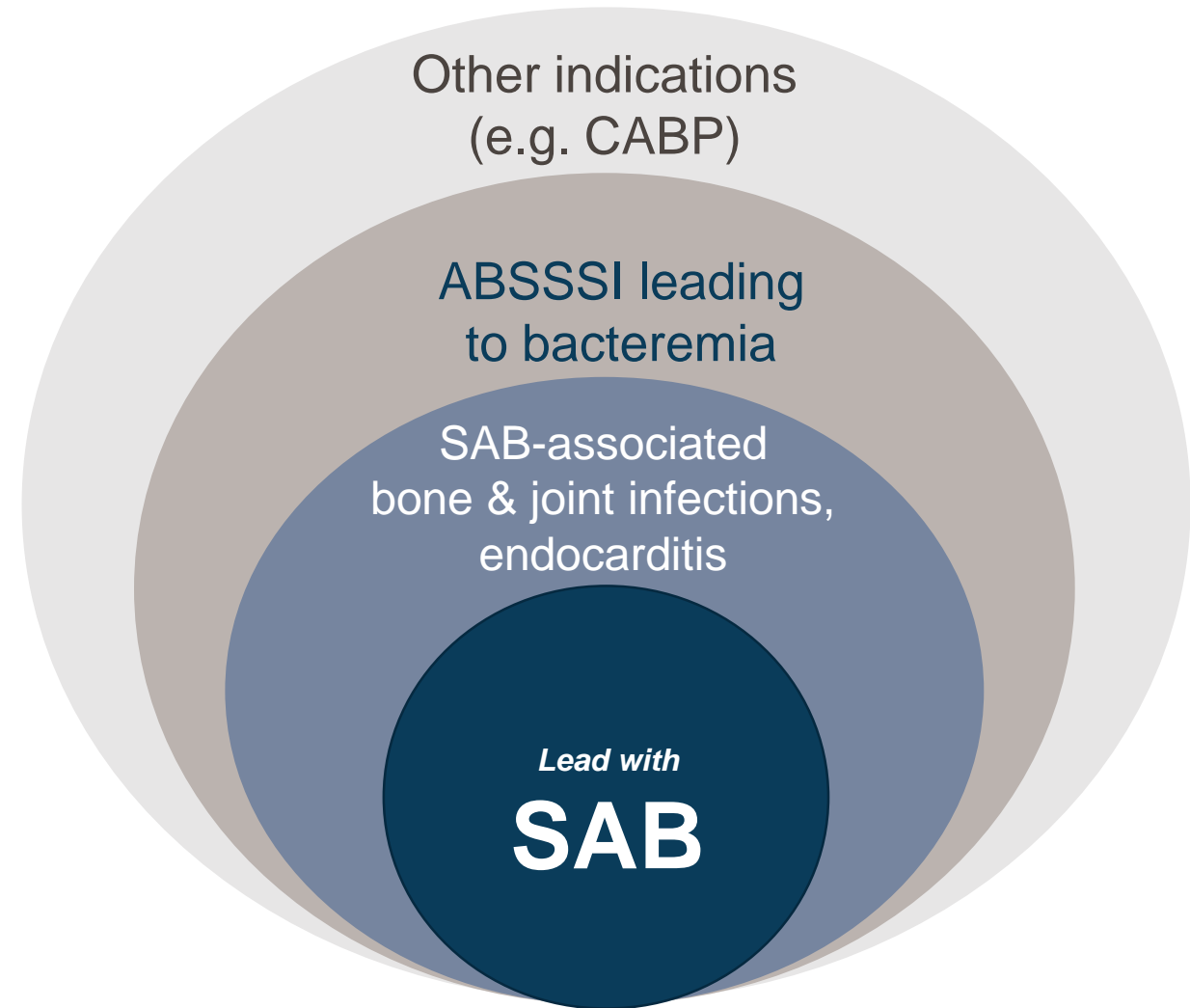
Patient numbers in the United States

- *Staphylococcus aureus* bacteremia (SAB): 120,000 cases¹
- Acute bacterial skin and skin structure Infections (ABSSSI): >600,000 hospitalizations per year²
- Community-acquired bacterial pneumonia (CABP): >1,500,000 hospitalizations per year³

¹ Kourtis AP et al. MMWR Morb Mortal Wkly Rep. 2019;68:214-219.

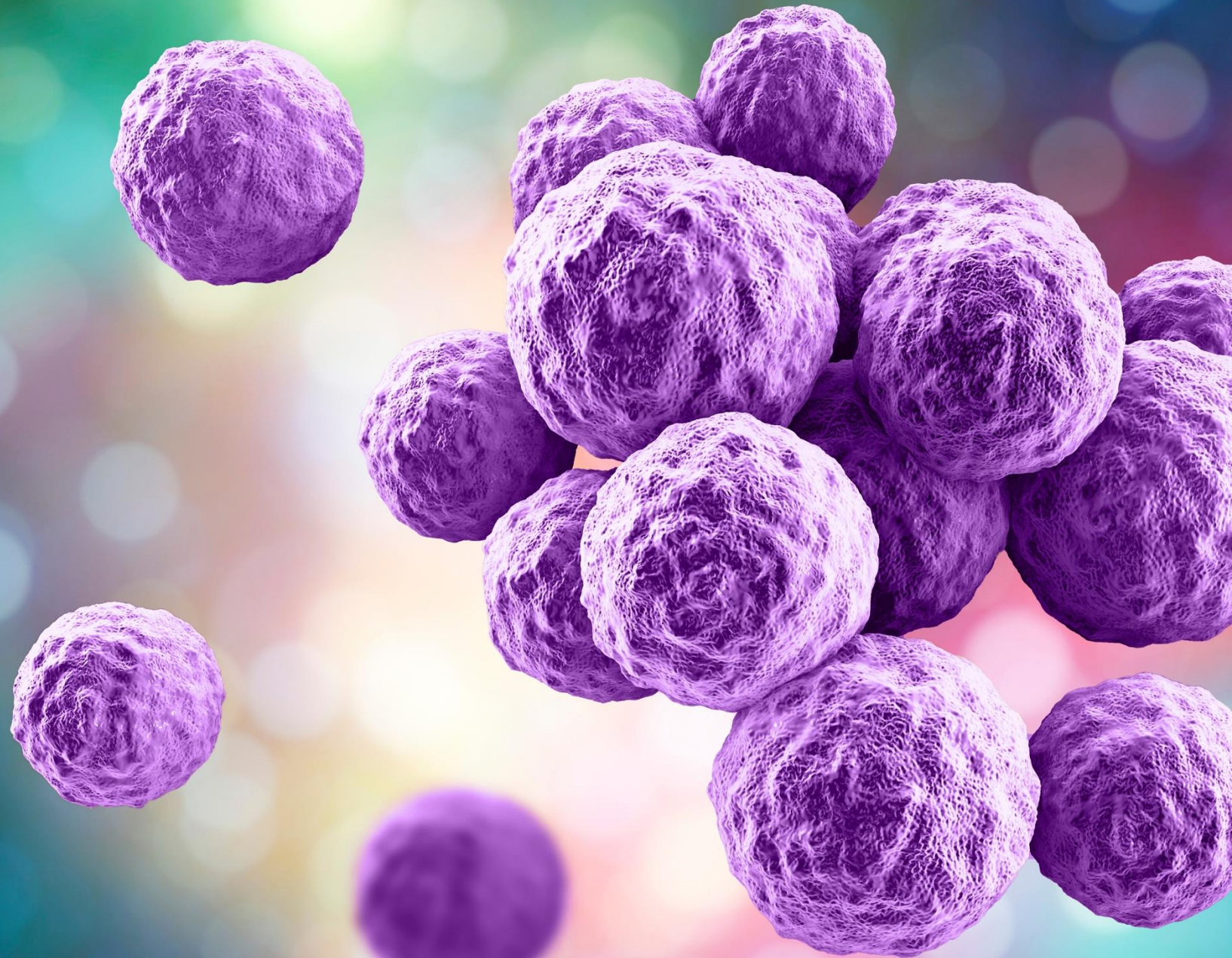
² Edelsberg J et al. Emerg Infect Dis. 2009;15:1516-8.

³ Ramirez JA et al. Clin Infect Dis. 2017;65:1806-1812.



Antibacterial
Tonabacase

Severe staphylococcal infections



Endolysins — Overview

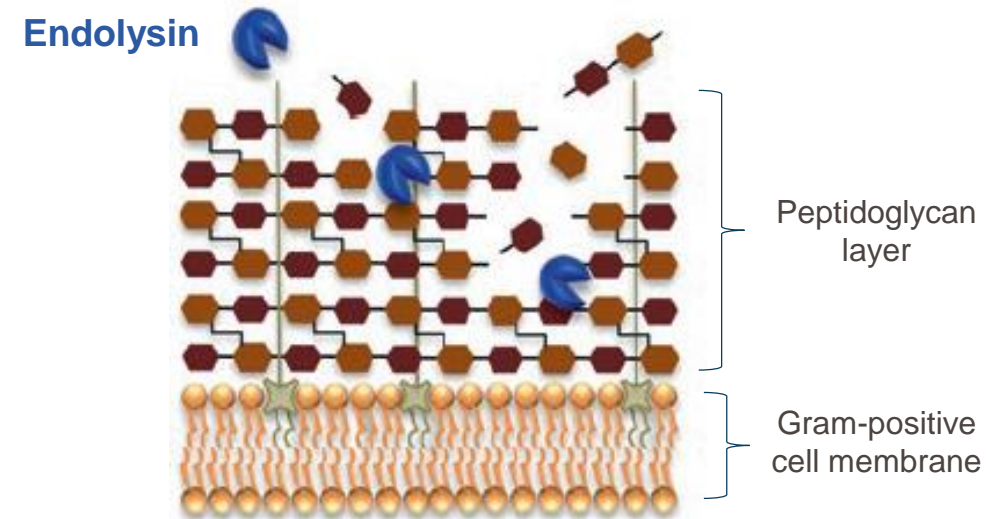
- Endolysins are recombinant proteins from bacteriophages
 - Responsible for cell wall cleavage and bacterial cell lysis
- Pathogen-specific spectrum
- Advantages over conventional antibiotics^{1, 2}
 - Rapid killing of bacterial cells
 - Effective against multidrug-resistant bacteria and biofilms
 - Low risk of resistance development
 - Minimal off-target damage preventing significant microbiome disruption
 - Synergy with standard-of-care antibiotics
 - Typically used in combinations

¹ Liu H, Hu Z, Li M et al. J Biomed Sci 2023 (30), 29

² Abdelrahman F, Easwaran M, Daramola O et al. Antibiotics 2021 (10) 124

³ Dams D, Briens Y Adv Exp Med Biol. 2019:1148:233-253

Schematic representation of endolysin effects on Gram-positive bacteria



Reference: Dams D and Briens Y, 2019³

Profile of tonabacase

- Potential first-in-class antibiotic of the endolysin class for the treatment of severe staphylococcal infections
 - Potential for superiority in combination therapy vs. standard-of-care antibiotics
- Activity in *S. aureus* infection models
 - Activity against MRSA and MSSA¹
- Clinical safety and tolerability demonstrated in phase 1 studies^{2, 3}
 - Potential to administer multiple doses of tonabacase
- Plan to start clinical phase 2 program in 2025 upon completion of preclinical profiling
 - Evaluation license and exclusive option to license upon successful completion of preclinical profiling

¹ Kim NH, Park WB, Cho JE et al. Antimicrob Agents Chemother 2018 (62), e00731-18
² Jun SY, Jang J, Yoon S. et al. Antimicrob Agents Chemother 2017 (61), e02629-16
³ Wire MB, Jun SY, Jang IJ et al. Antimicrob Agents Chemother 2022 (66), e01842-21
⁴ Huang DB, Gaukel E, Kerzee N, et al. Antimicrob Agents Chemother 2021 (65), e00508-21

Reduction of MRSA counts (cardiac vegetations, kidneys, and spleen) in left-sided endocarditis model



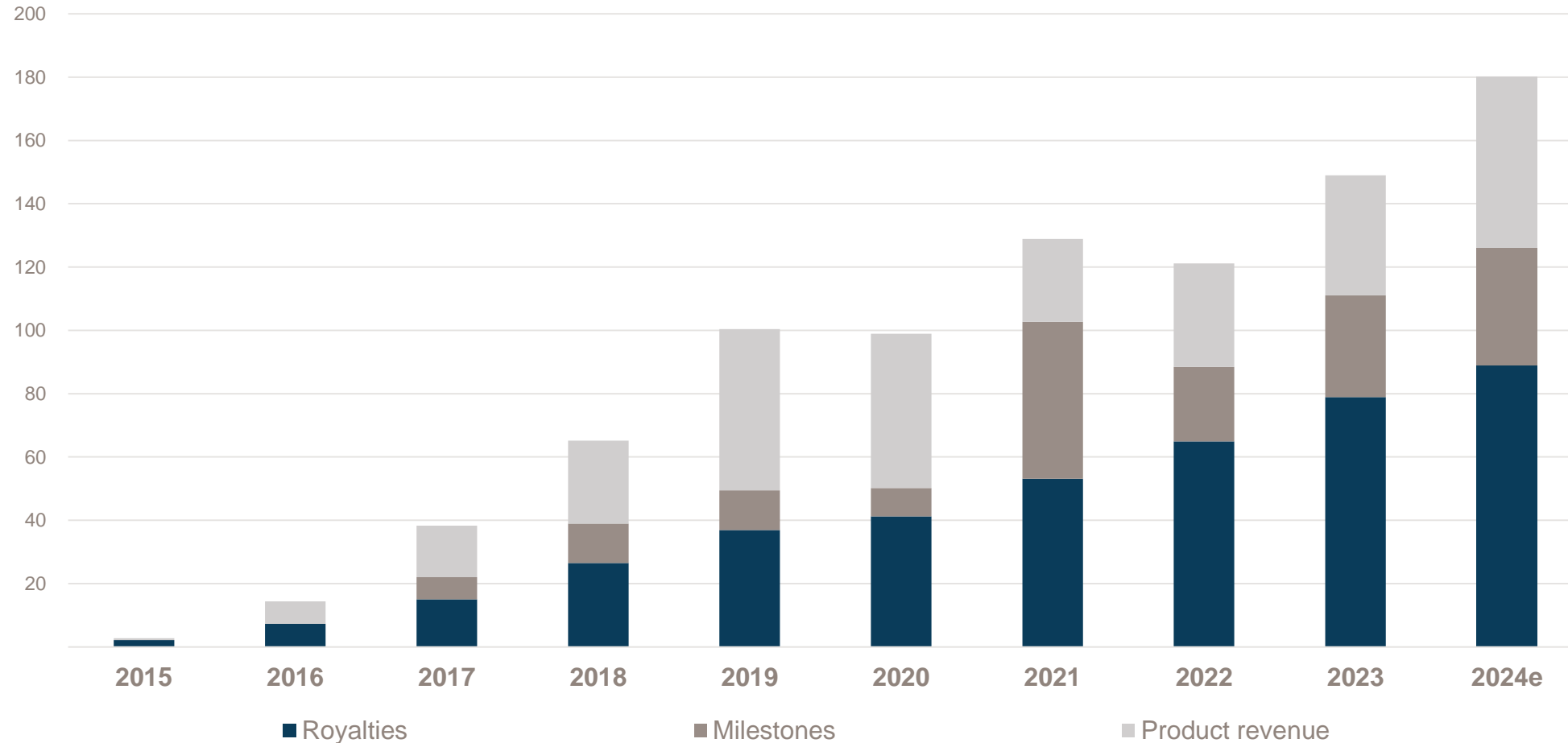
CFU: Colony forming unit; Adapted from Huang DB, Gaukel E, Kerzee N, et al. 2021⁴



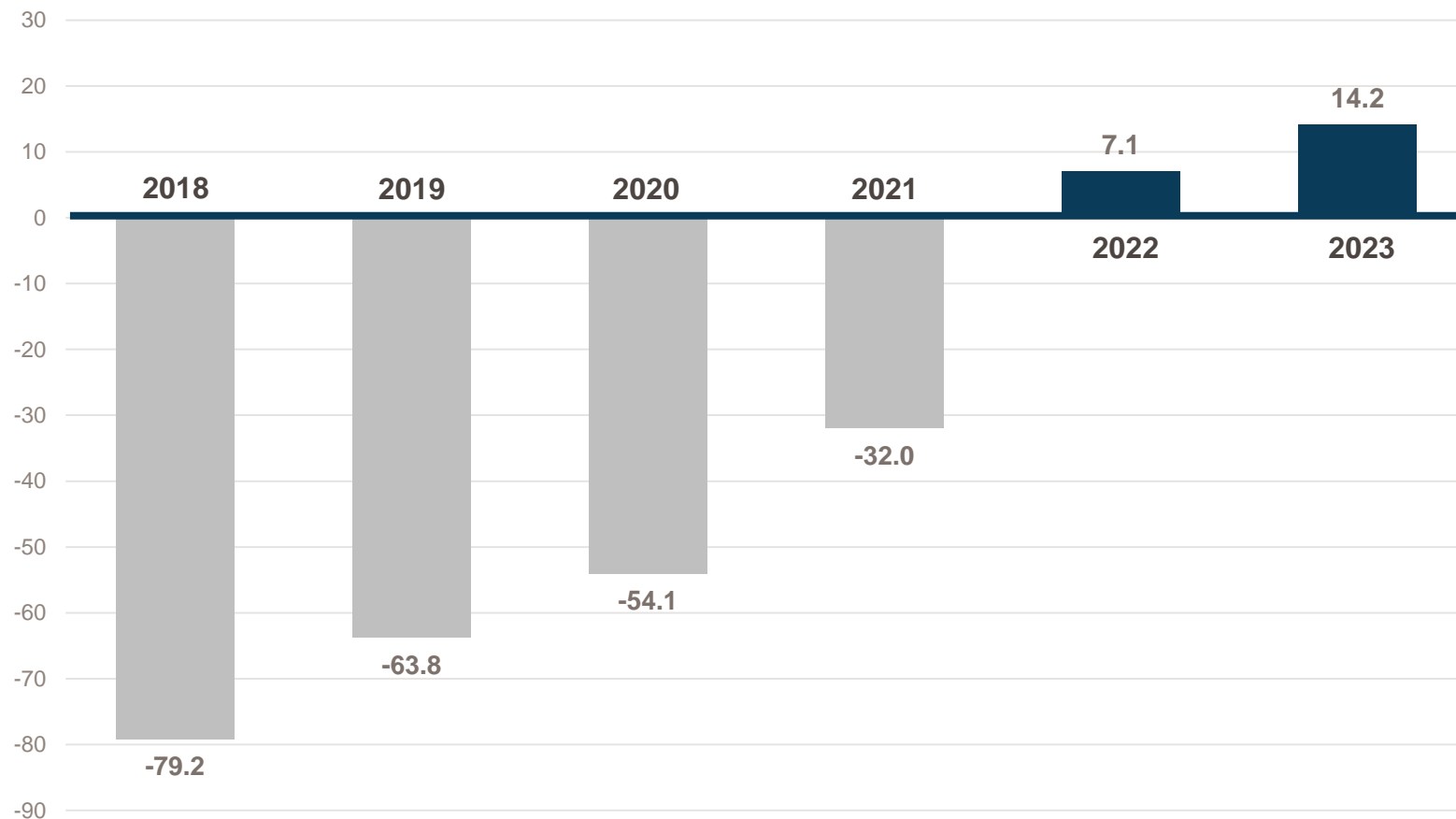
Financials & Outlook



Cresemba and Zevtera-related revenue breakdown (in CHF mn)

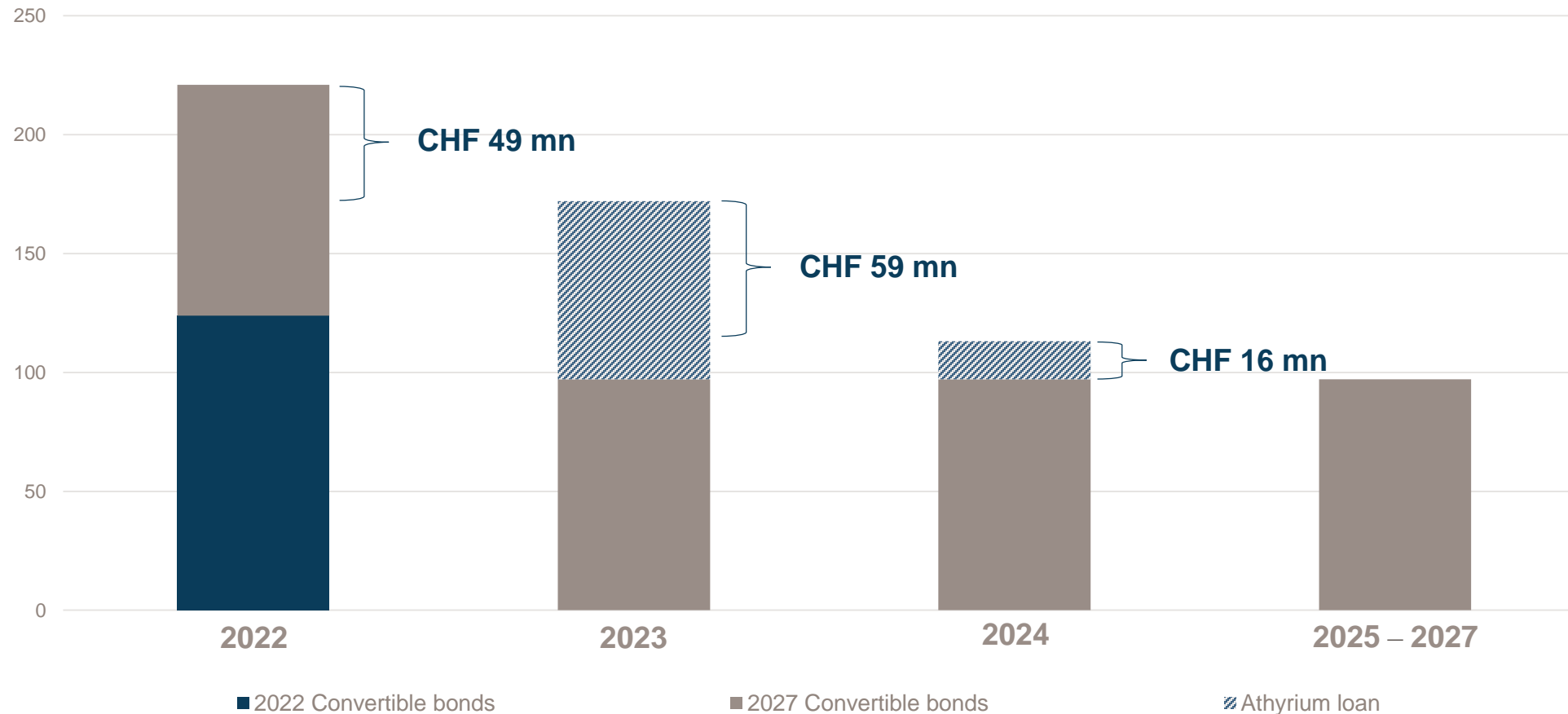


Cash flows from operating activities (in CHF mn)



Note: Consolidated figures in conformity with US GAAP; rounding applied consistently

CHF 124 mn non-dilutive debt level reduction 2022-2024



Note: Figures (in CHF mn) as of the beginning of the fiscal year; rounding applied consistently

2024 Guidance – 20% increase in Cresemba and Zevtera-related revenue and more than doubling of net profit

In CHF mn	FY 2024 guidance*	FY 2023
Cresemba and Zevtera related revenue	~180	150.3
<i>of which royalty income</i>	~89	78.9
Total revenue	~183	157.6
Cost of products sold	~33	26.8
Operating expenses	~120	111.6
Operating profit	~30	19.2
Net profit	~25	10.5

* Excluding the impact of in-licensing and acquisitions

Note: Consistent rounding was applied.

Key milestones

	Product	H2 2023	H1 2024	H2 2024
Antibacterials	Ceftobiprole (Zevtera)	US NDA submission ✓	US FDA approval ✓	
		US NDA accepted for review ✓	Executing US partnership (mid-2024)	
	Tonabacase	Evaluation license & exclusive option to license ✓		Decide on definitive licensing option
Antifungals	Isavuconazole (Cresemba)	Pediatric submissions ✓ Decision on US pediatric extension ✓	Decision on EU pediatric extension	
	Fosmanogepix	Acquisition of rights ✓	Initiate phase 3 study in candidemia / invasive candidiasis (mid-2024)	Initiate phase 3 study in mold infections (around year-end)
	BAL2062	Acquisition of rights ✓		

Increasing Cresemba & Zevtera revenue

In-licensing and acquisition of anti-infectives

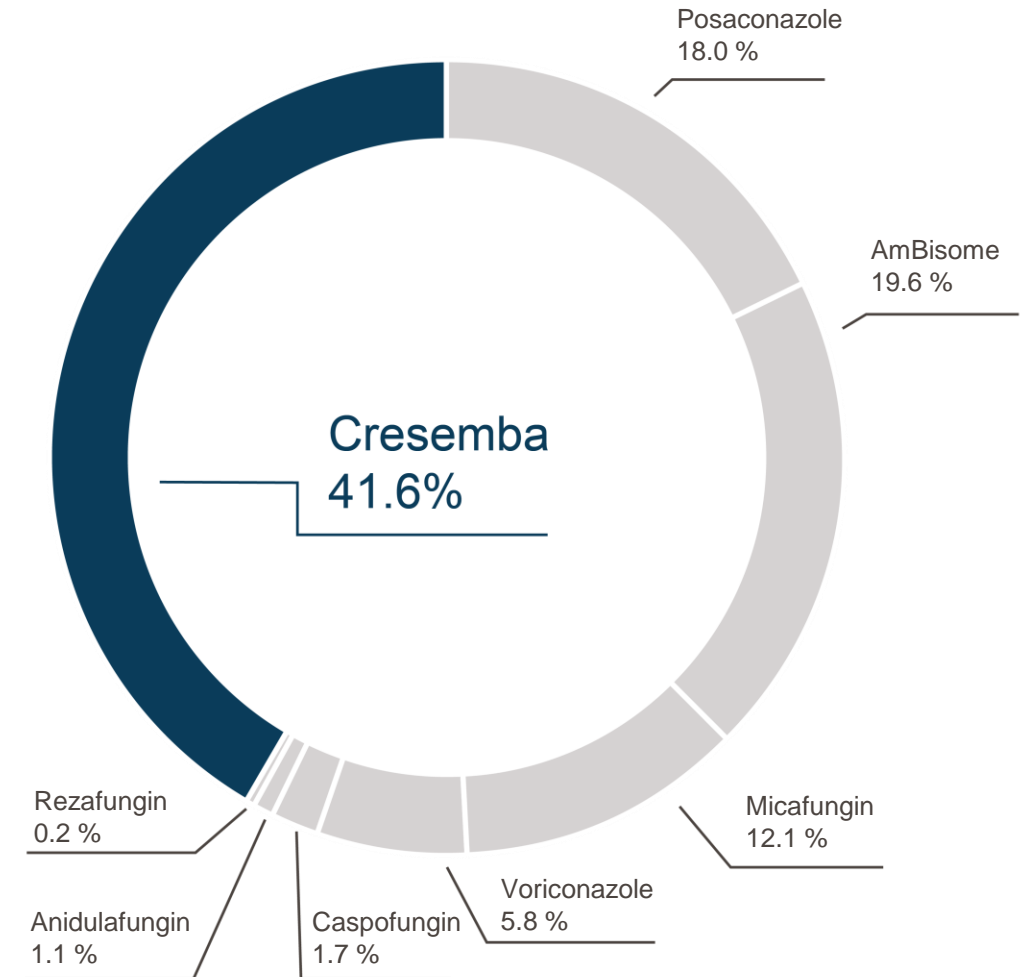
Advancement of preclinical anti-infective assets

Appendix

Cresemba has become the market leader in the US in terms of value

- Consistently increased market share among best-in-class antifungals* since launch to around 42% by December 2023**

* Best-in-class antifungals: Cresemba (isavuconazole), posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin, rezafungin

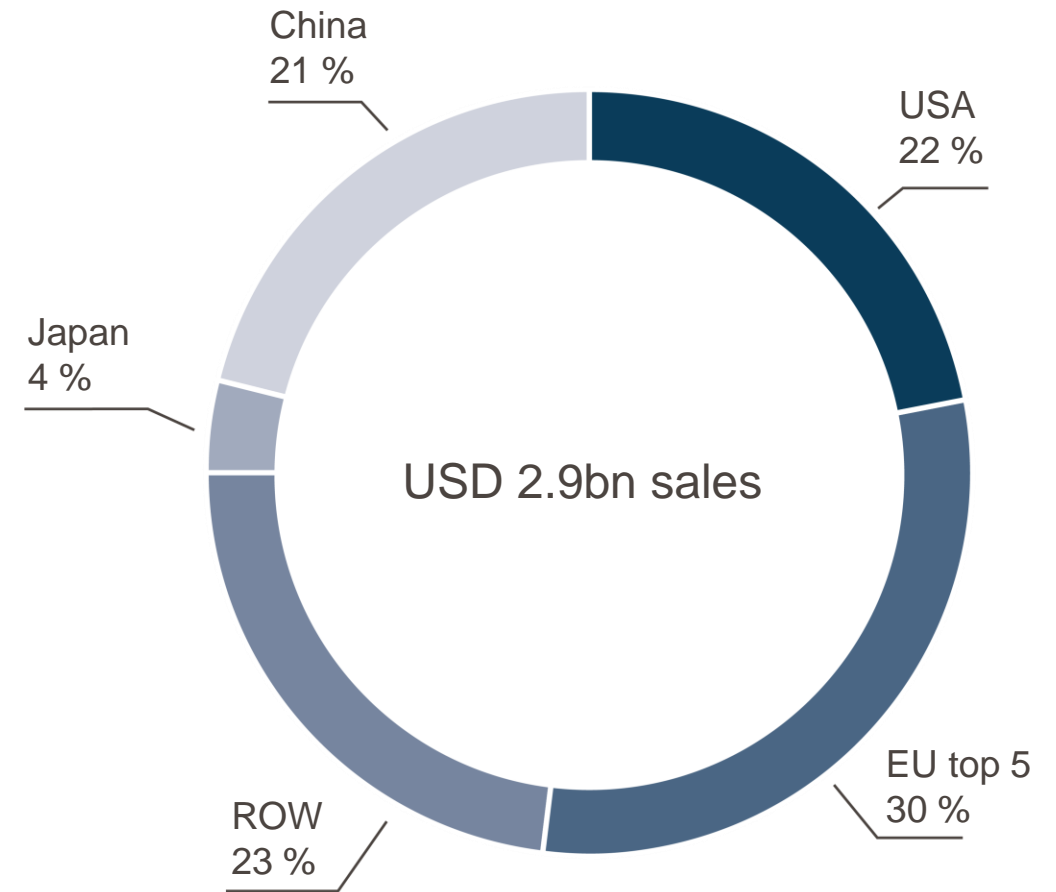


**Market share based on MAT Q4 2023, in-market sales reported as moving annual total (MAT) in US dollar; rounding consistently applied. Source: IQVIA Analytics Link, December 2023

Significant growth potential for Cresemba

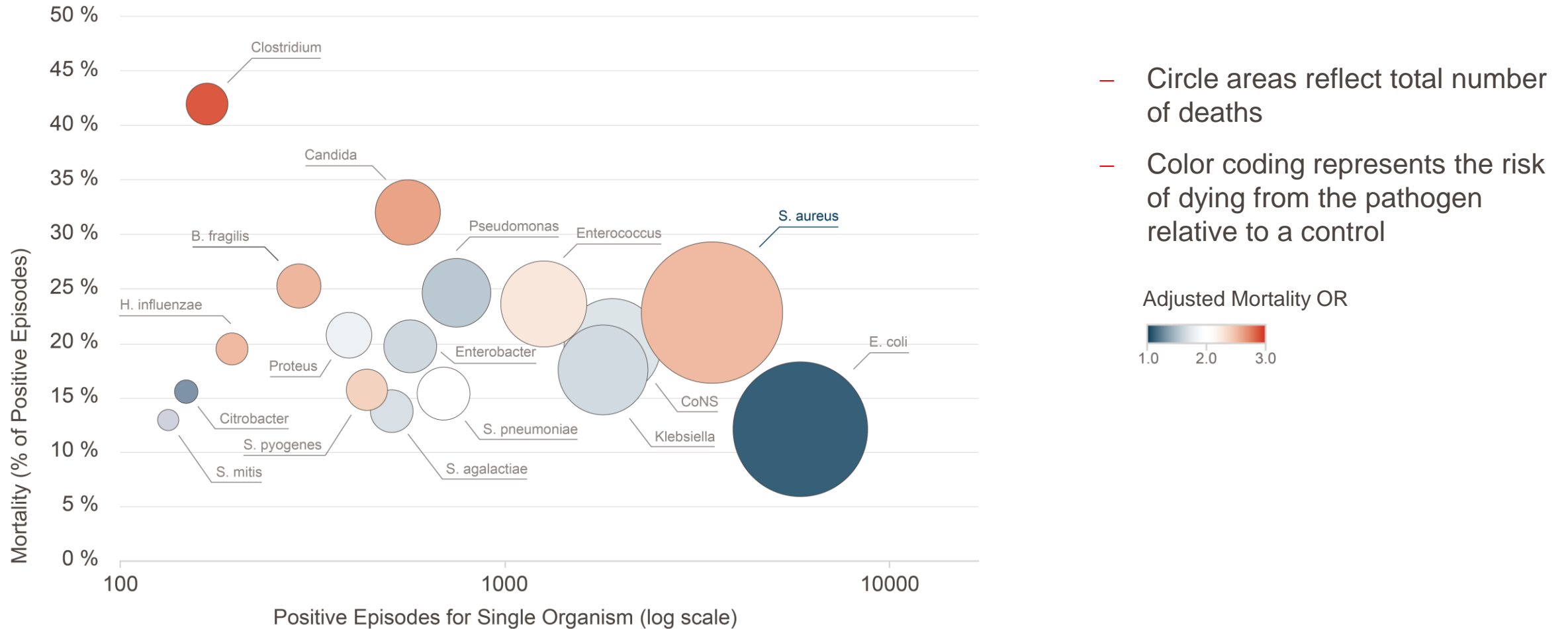
- USD 2.9 bn sales of best-in-class antifungals* (MAT Q4 2023)
- Recently launched in Japan and China, representing 25% of global potential

* Best-in-class antifungals: Cresemba (isavuconazole), posaconazole, voriconazole, AmBisome, anidulafungin, caspofungin, micafungin, rezafungin



MAT: Moving annual total; Source: IQVIA Analytics Link, December 2023

SAB — Highest disease burden among bloodstream infections



Adapted from: Verway M et al. J Clin Microbiol. 2022;60:e0242921.

Completed clinical phase 1/2 program

Seven phase 1 studies in healthy subjects

- Established dose range and safety/tolerability profile
- >90% oral bioavailability
- No significant food effect
- Broad tissue distribution to relevant target organs (mass balance study)
- Low propensity for CYP3A4 inhibition

Fosmanogepix treatment up to 6 weeks

One phase 1 B study in neutropenic patients with AML

- Consistent safety and tolerability profile

Fosmanogepix treatment up to 2 weeks

Three phase 2 studies in patients with candidemia, candidemia with *C. auris*, and invasive mold infections

- Proof of concept achieved based on survival and clinical success rates adjudicated by an independent review committee
- Safety and tolerability characterized by drug-related adverse events of headache, dizziness, fatigue, nausea and vomiting

Fosmanogepix treatment up to 6 weeks

More than 300 subjects treated with fosmanogepix

Shaw KJ, Ibrahim AS. J Fungi (Basel). 2020; 6:239.

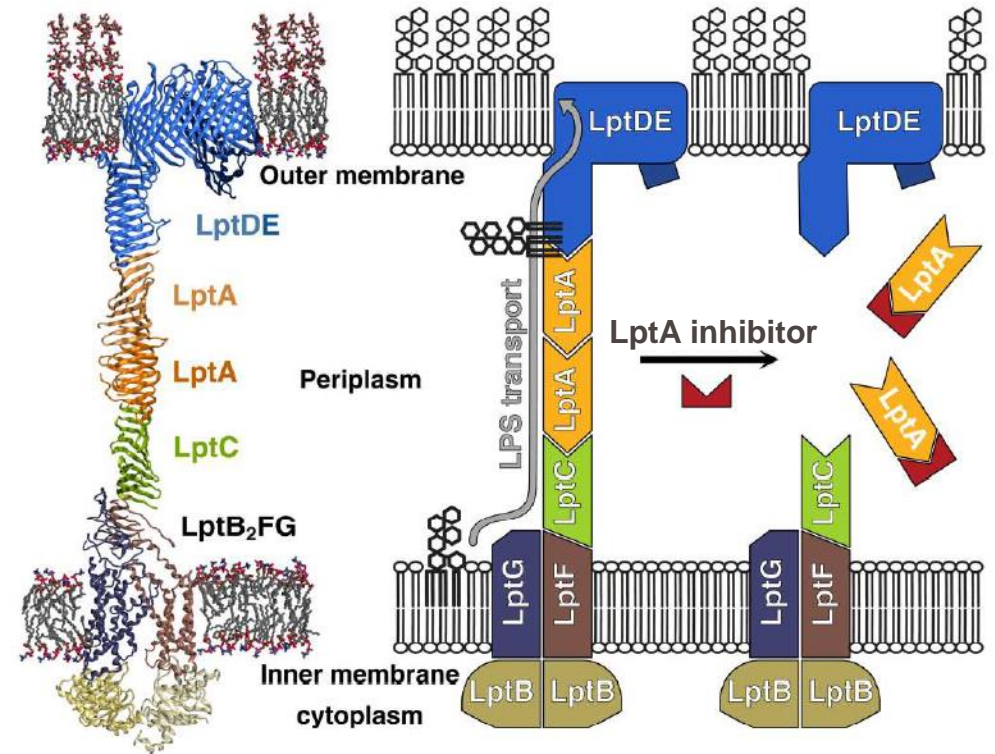
Hodges MR, Ople E, Wedel P, et al. Antimicrob Agents Chemother. 2023;67:e0162322.

Vazquez JA, Pappas PG, Boffard K, et al. Antimicrob Agents Chemother. 2023;67(5):e0141922.

Pappas PG, Vazquez JA, Oren I, et al. J Antimicrob Chemother. 2023:dkad256

LptA inhibitor

- New class of antibiotic
- Selective spectrum antibiotic with potent activity against Gram-negative bacteria of the Enterobacteriaceae family
- Small molecule peptidomimetic with a novel mode of action
 - Selectively targets LptA an essential part of the lipopolysaccharide (LPS) transport bridge
 - Inhibition of LptA results in a loss of the integrity of the outer cell membrane
 - Rapid killing of the bacteria
- Activity in *Klebsiella pneumoniae* lung infection model
- Potential treatment for severe infections caused by Enterobacteriaceae
- IND filing expected around year-end 2025
- Program supported by grant award from CARB-X



Adapted from Schuster et al., Sci. Adv. 9 (2023)

Proprietary information of Basilea Pharmaceutica International Ltd, Allschwil – not for distribution

In-licensing focus



Partner of choice in the anti-infectives space

- ✓ Strong and proven R&D capabilities to bring drugs from research to market
- ✓ Cost-effective business model
- ✓ Experience in accessing non-dilutive funding incentives
- ✓ Financial strength and strong cash flow generation from commercialized brands

Antifungals

- Novel mechanisms of action
- Addressing areas of highest unmet medical needs
- Gaining benefits through orphan drug pathways
- Novel formulations

Commonalities

- Addressing serious hospital infections with increasing medical need
- Innovative & differentiated assets with potential for successful commercialization
- In-licensing assets from late stage research through to end of phase 2

Antibacterials

- Traditional and non-traditional approaches
- Potential for demonstrating superiority
- Balance development risks to optimize market access/label

Glossary

- ABSSSI: **A**cute **b**acterial **s**kin and **s**kin **s**tructure infections
- AML: **A**cute **M**yeloid **L**eukemia
- BARDA: **B**iomedical **A**dvanced **R**esearch and **D**evelopment **A**uthority
- CABP: **C**ommunity-**a**cquired **b**acterial **p**neumonia
- CARB-X: **C**ombating **A**ntibiotic-**R**esistant **B**acteria **B**iopharmaceutical **A**ccelerator
- CNS: **C**entral **N**ervous **S**ystem
- CYP-450: **C**ytochrome-**P-450** enzymes
- EMA: **E**uropean **M**edicines **A**gency
- FDA: **U**S **F**ood and **D**rug **A**dministration
- HABP: **H**ospital-**a**cquired **b**acterial **p**neumonia
- IMI: **I**nvasive **m**old infections
- i.v.: **I**ntravenous
- mITT: **M**odified **i**ntent-**t**o-**t**reat
- LPS: **L**ipopolysaccharide
- MSSA: **M**ethicillin-**s**usceptible ***S**taphylococcus **a**ureus*
- MRSA: **M**ethicillin-**r**esistant ***S**taphylococcus **a**ureus*
- NDA: **N**ew **D**rug **A**pplication
- OR: **O**dds ratio
- PD: **P**harmacodynamic
- PK: **P**harmacokinetic
- PDUFA: **P**rescription **D**rug **U**ser **F**ee **A**ct
- QIDP: **Q**ualified **I**nfectious **D**isease **P**roduct
- SAB: ***S**taphylococcus **a**ureus* **b**acteremia
- SPA: **S**pecial **P**rotocol **A**ssessment
- US GAAP: **U**nited **S**tates **G**enerally **A**ccepted **A**ccounting **P**riniples
- VAP: **V**entilator-**a**ssociated **p**neumonia

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**Creating anti-infective
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