



**Focused on
Growth and Innovation**

**“Patients are at the heart
of what we do”**

Investor presentation

September 1, 2023



Table of contents

- Executive summary
- Portfolio
 - Antifungal
 - Cresemba® (isavuconazole)
 - Antibiotic
 - Zevtera® (ceftobiprole)
- Financials & Outlook
- Appendix





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



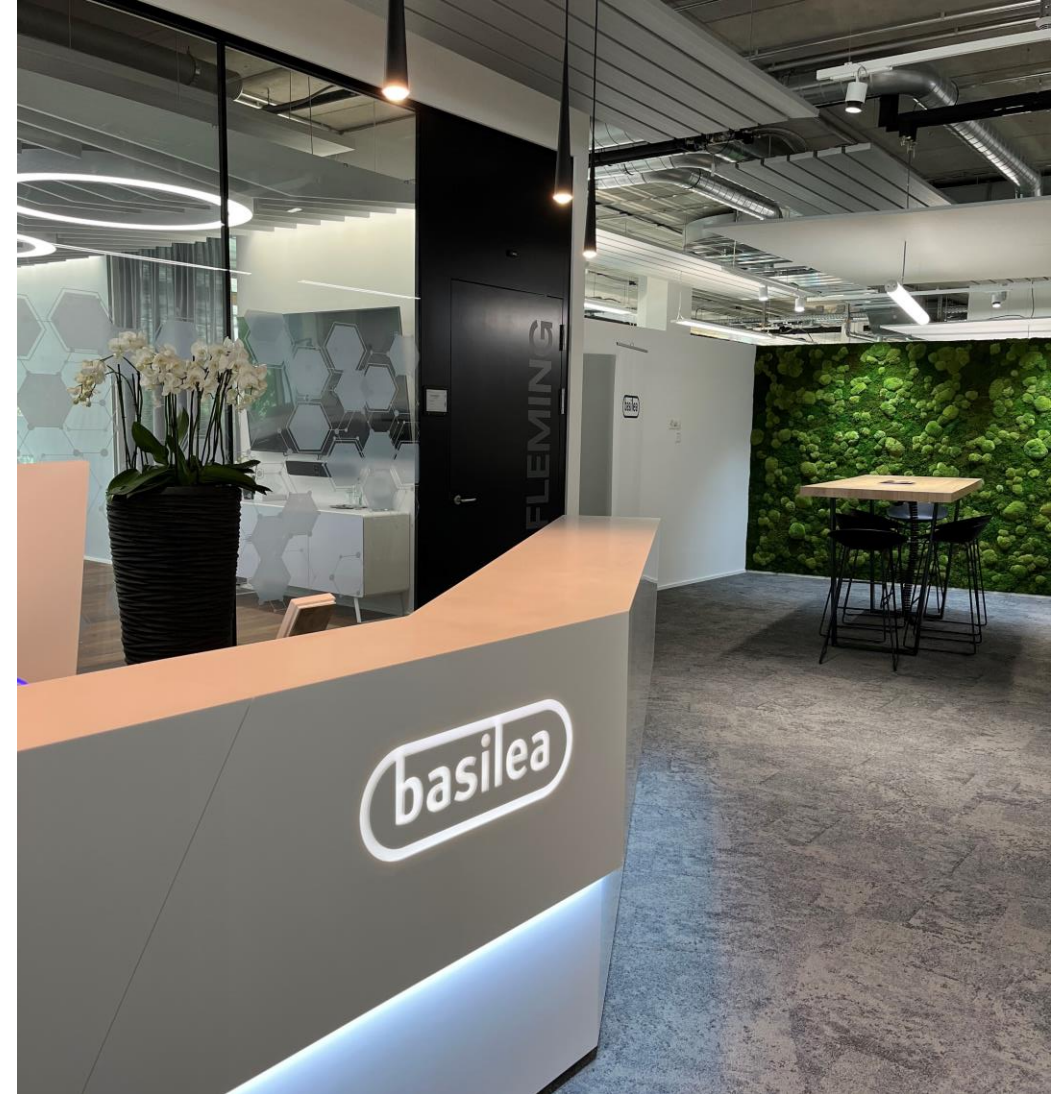
**Laurenz
Kellenberger**
Ph.D. CSO

2000

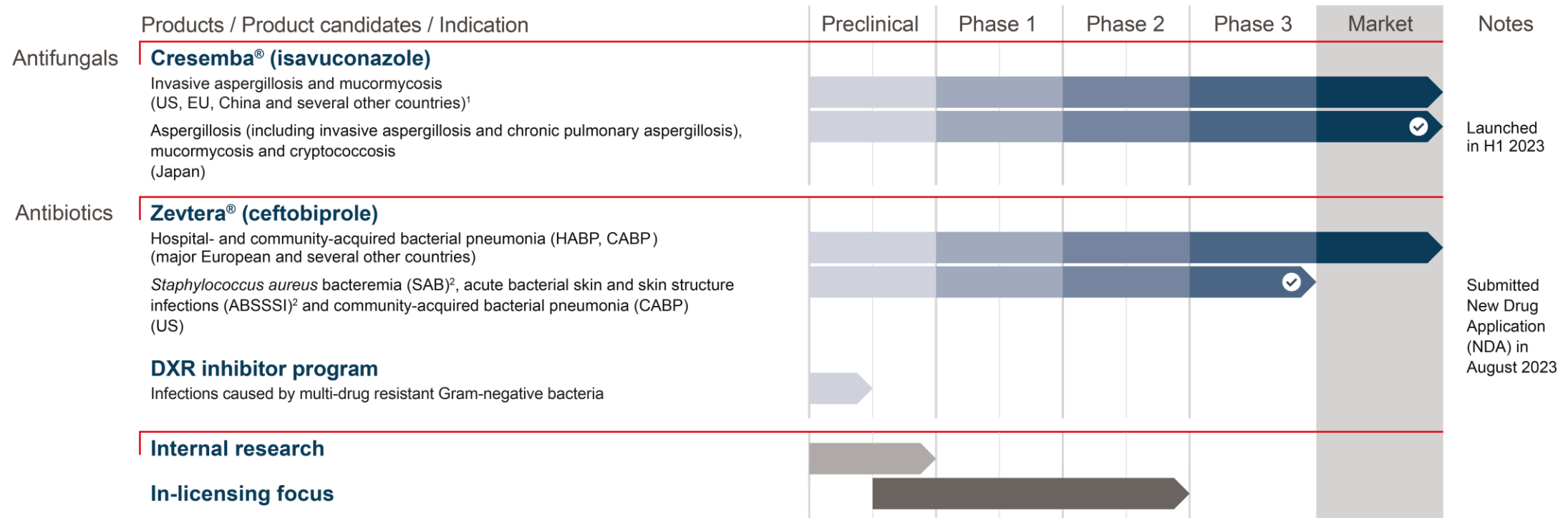


At a glance

- 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 programs which are in an earlier stage of development
- Profitable company listed on SIX Swiss Stock Exchange, SIX: BSLN
- Located in the Basel area life sciences hub, Switzerland



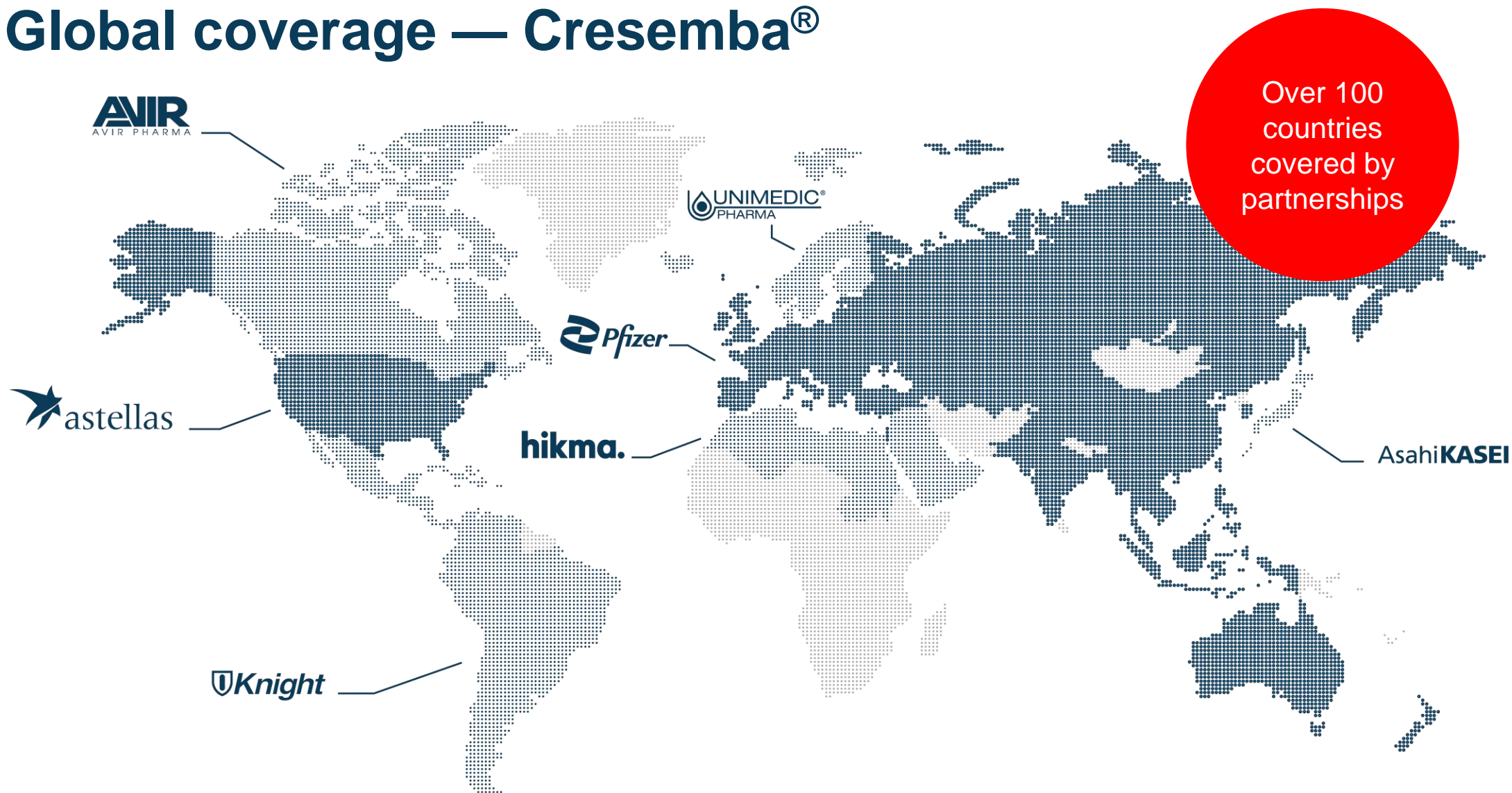
Potential for sustainable growth and value creation



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

² 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).

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)

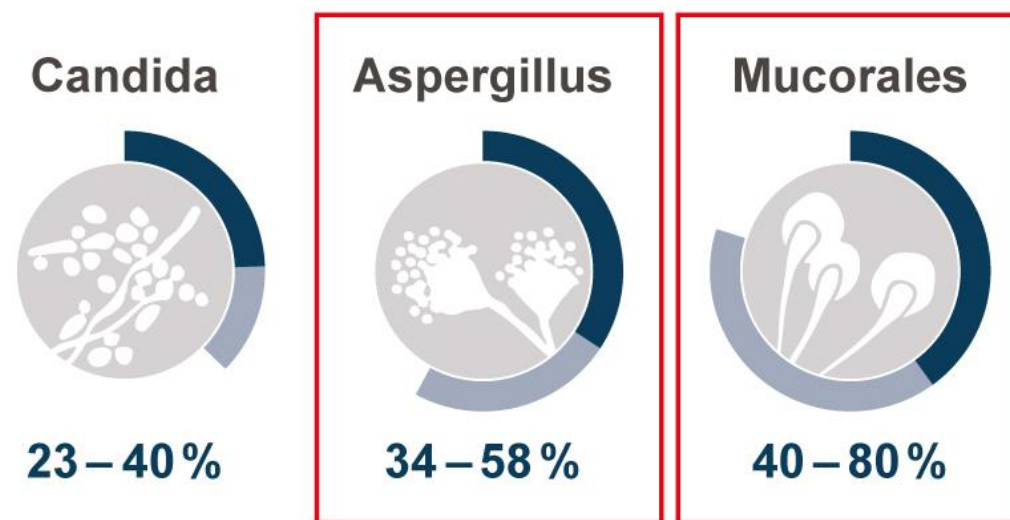
Invasive mold infections



The market — Invasive fungal infections

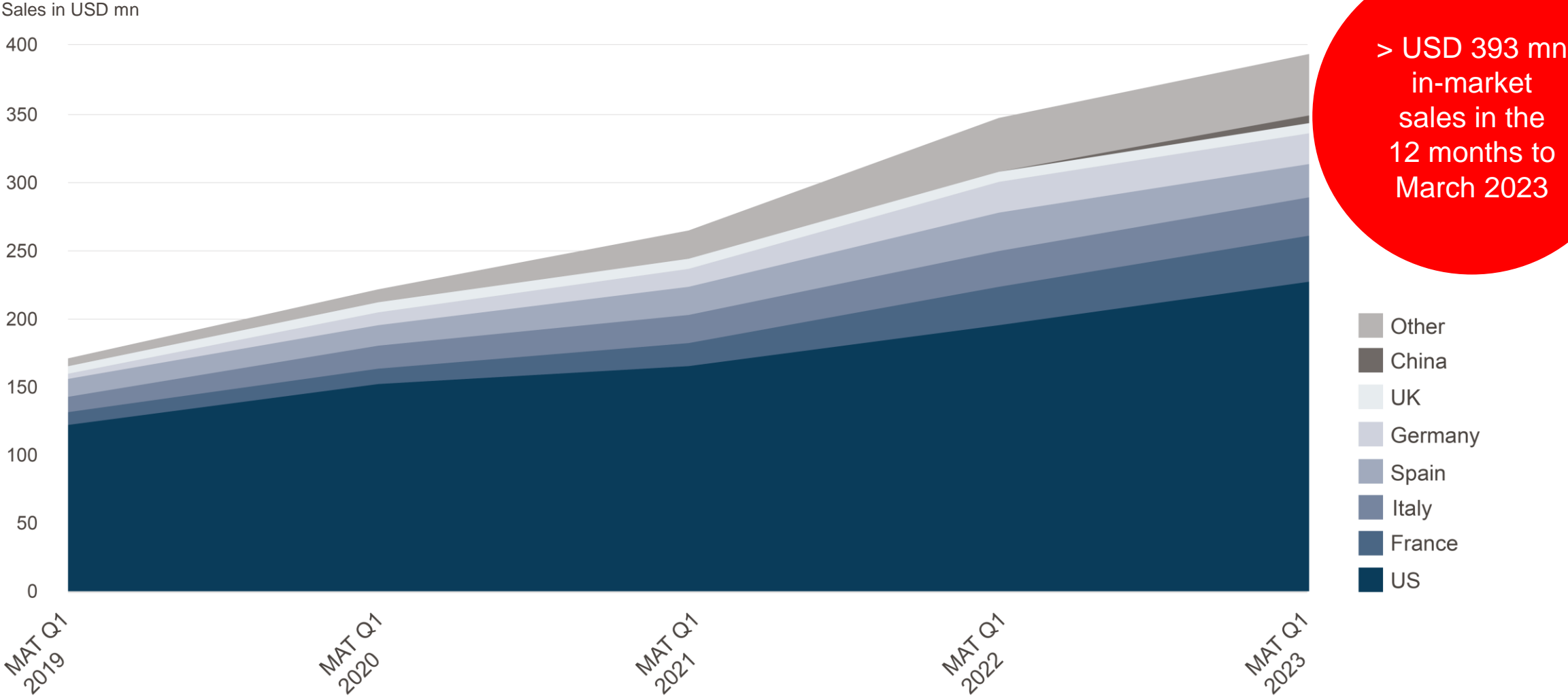
- Severe, potentially life-threatening infections mainly affecting immunocompromised patients
- An important cause of morbidity and mortality in cancer patients undergoing intensive chemotherapy regimens
- Rising number of immunocompromised patients (cancer and transplantations) driving therapeutic demand
- 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

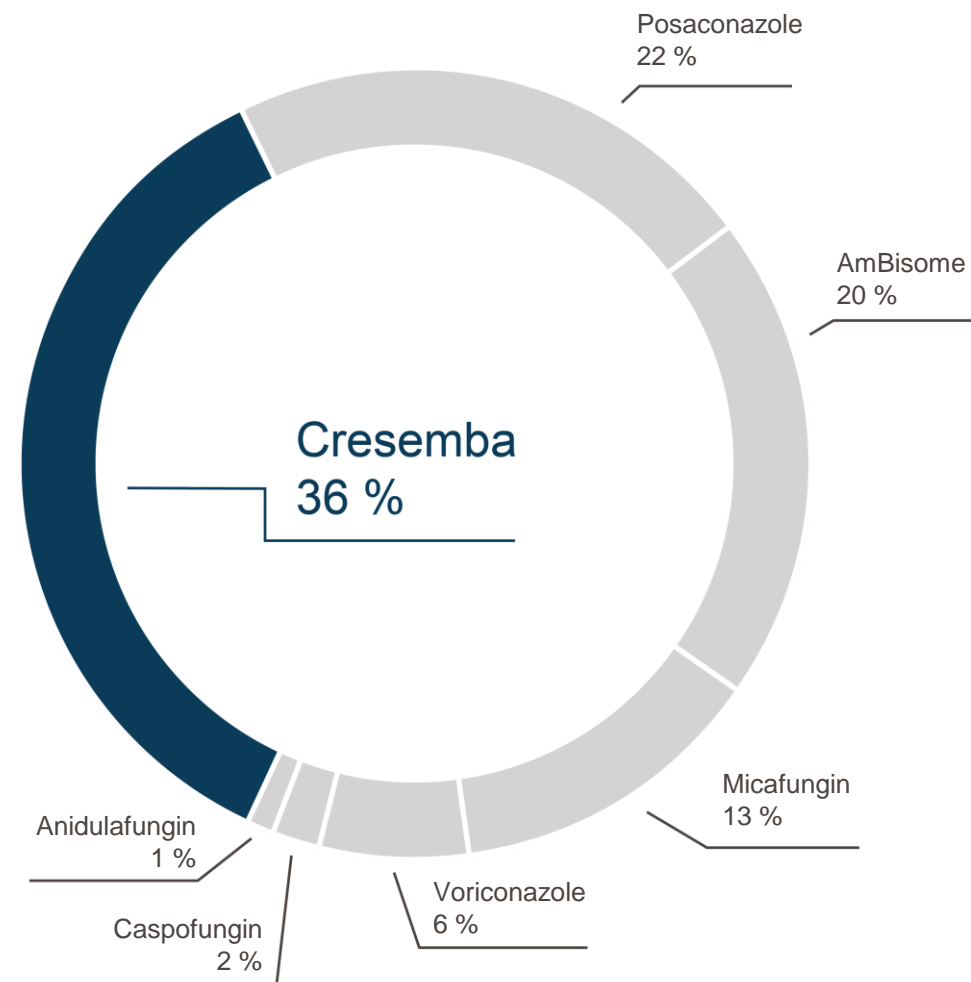


MAT: Moving annual total; Source: IQVIA Analytics Link, March 2023

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 36% by March 2023**

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



**Market share based on MAT Q1 2023, in-market sales reported as moving annual total (MAT) in US dollar; rounding consistently applied. Source: IQVIA Analytics Link, March 2023

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

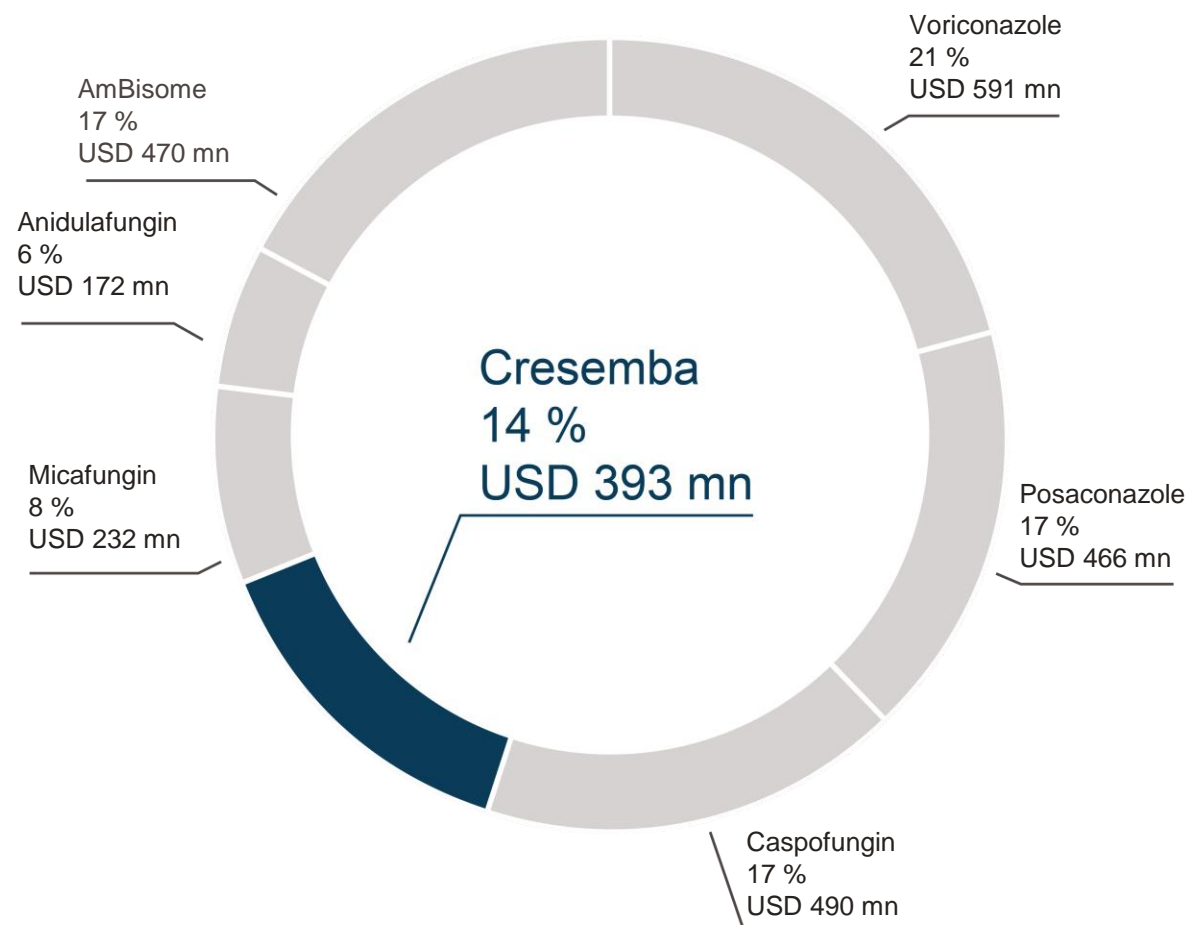
USD 2.8 bn sales (MAT Q1 2023)

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

- Launched in 67 countries by H1 2023
- Pediatric label extension in US anticipated in December 2023; subsequently market exclusivity extension by six months to September 2027
- Pediatric label extension in EU anticipated in 2024, which would extend market exclusivity by two years to October 2027

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

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



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

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

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.

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 NDA submitted in August 2023

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). Not approved in the US

MENA: Middle East and North Africa



Focused on Growth and Innovation



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

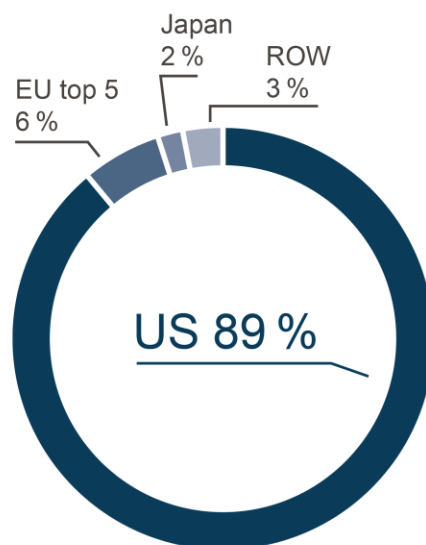
² Overcash JS et al. Clin Infect Dis. 2021;73:e1507-e1517.

³ Holland TL et al., Open Forum Infect. Dis. 2022, 9: (S931–S932).

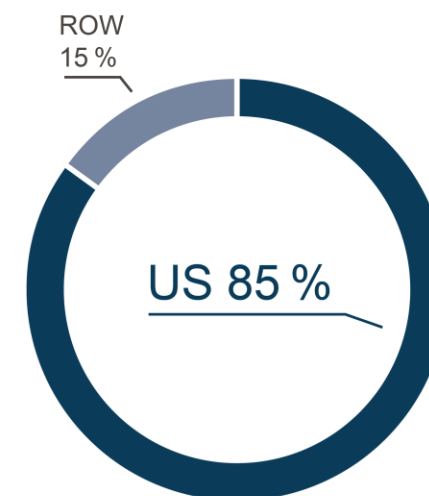
⁴ Rubino CM et al. Pediatr Infect Dis J. 2021;40:997-1003.

The hospital anti-MRSA antibiotic market — A USD 2.6 bn market* with the US being the most important region

Daptomycin sales by region
(2015, before LOE)



Ceftaroline sales by region
(MAT Q1 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, March 2023

Ceftobiprole — Strategy for accessing the US market

— NDA submitted in August 2023 for three indications:

1. *Staphylococcus aureus* bacteremia (SAB)¹
2. Acute bacterial skin and skin structure Infections (ABSSSI)²



3. Previously completed phase 3 study in community-acquired bacterial pneumonia (CABP) as a third indication³

- FDA decision on NDA expected in Q2 2024
- 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 to be secured prior to regulatory decision



¹ Holland TL et al., Open Forum Infect. Dis. 2022, 9: (S931–S932).

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

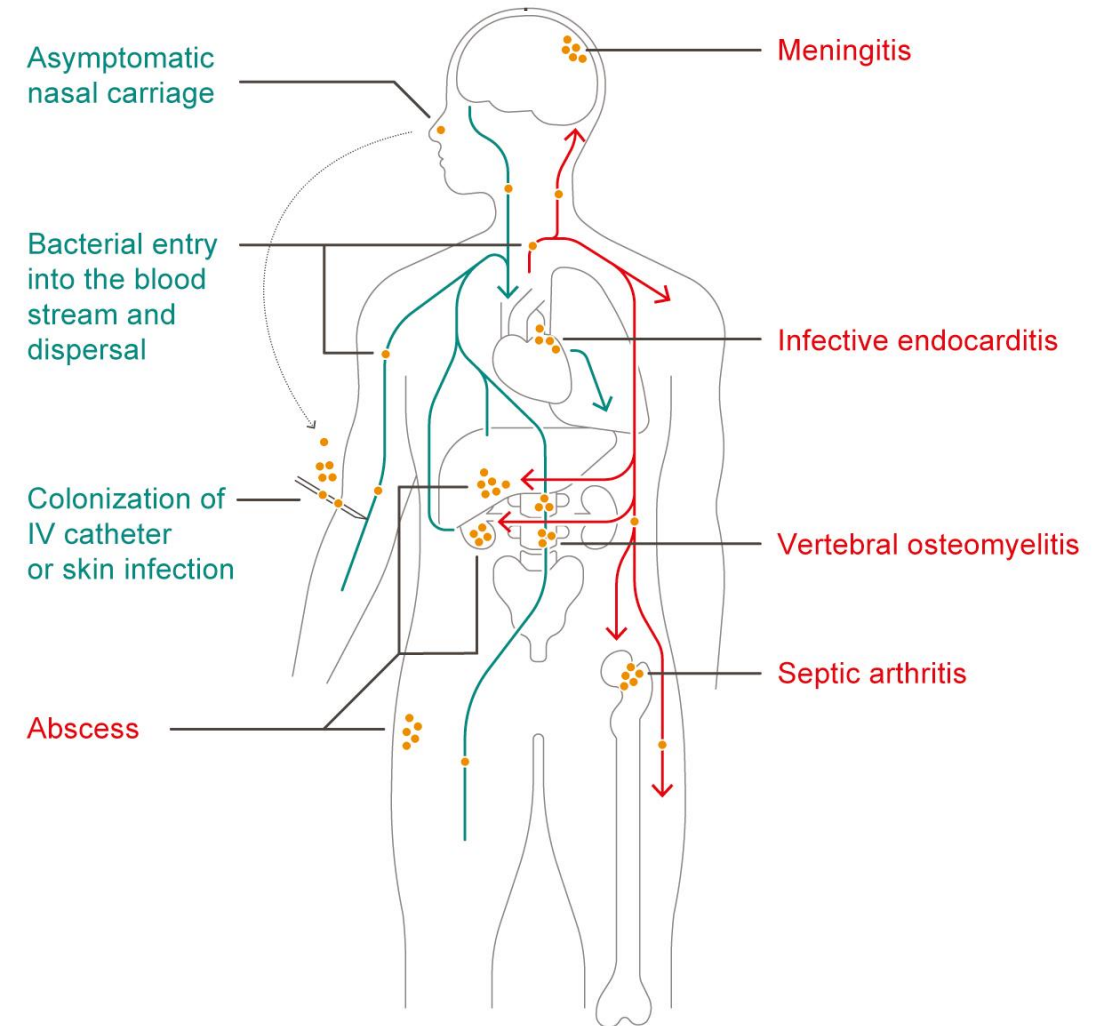
¹ MMWR, 2019;68:214–219.

² Hamed K et al. Future Microbiol. 2020;15:35-48.

MRSA: methicillin-resistant *Staphylococcus aureus*

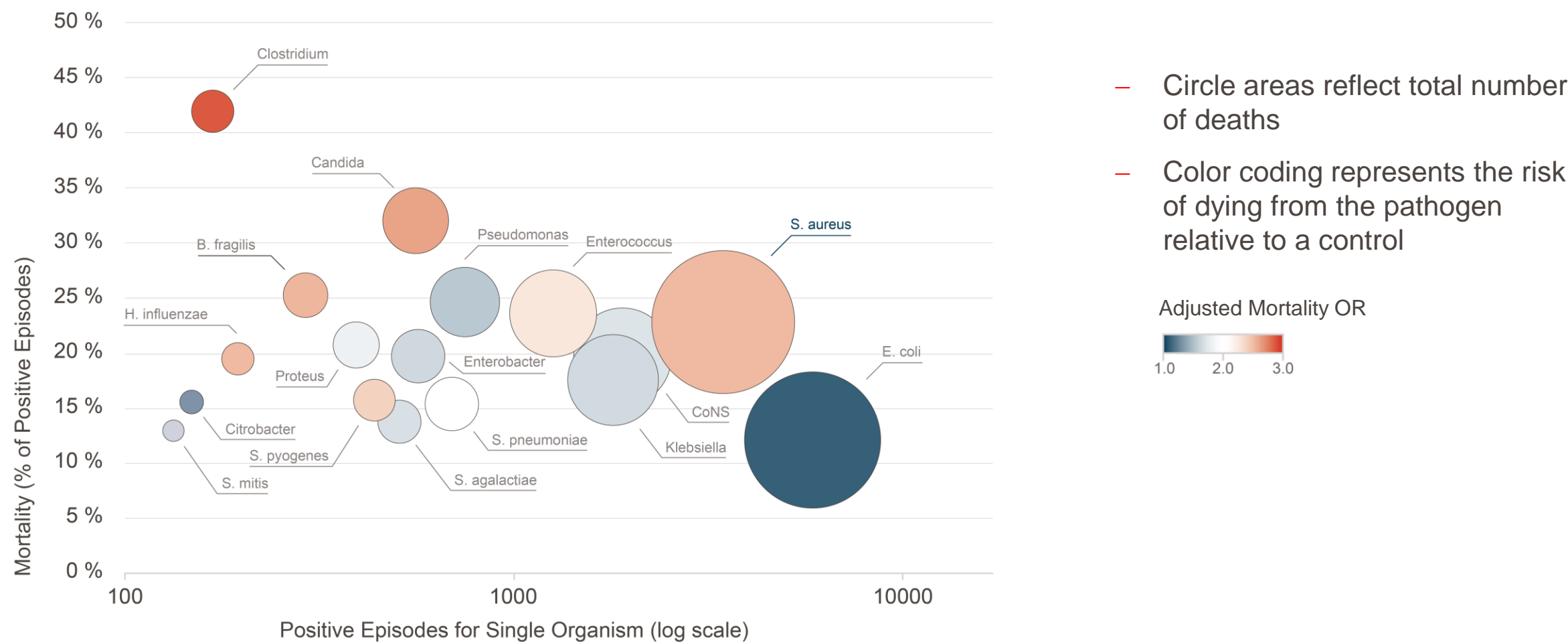
MSSA: methicillin-susceptible *Staphylococcus aureus*

Causes and consequences of SAB



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

SAB — Highest disease burden among bloodstream infections



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

ERADICATE — The largest phase 3 registrational study conducted in SAB

- 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.

Ceftobiprole — Place in therapy

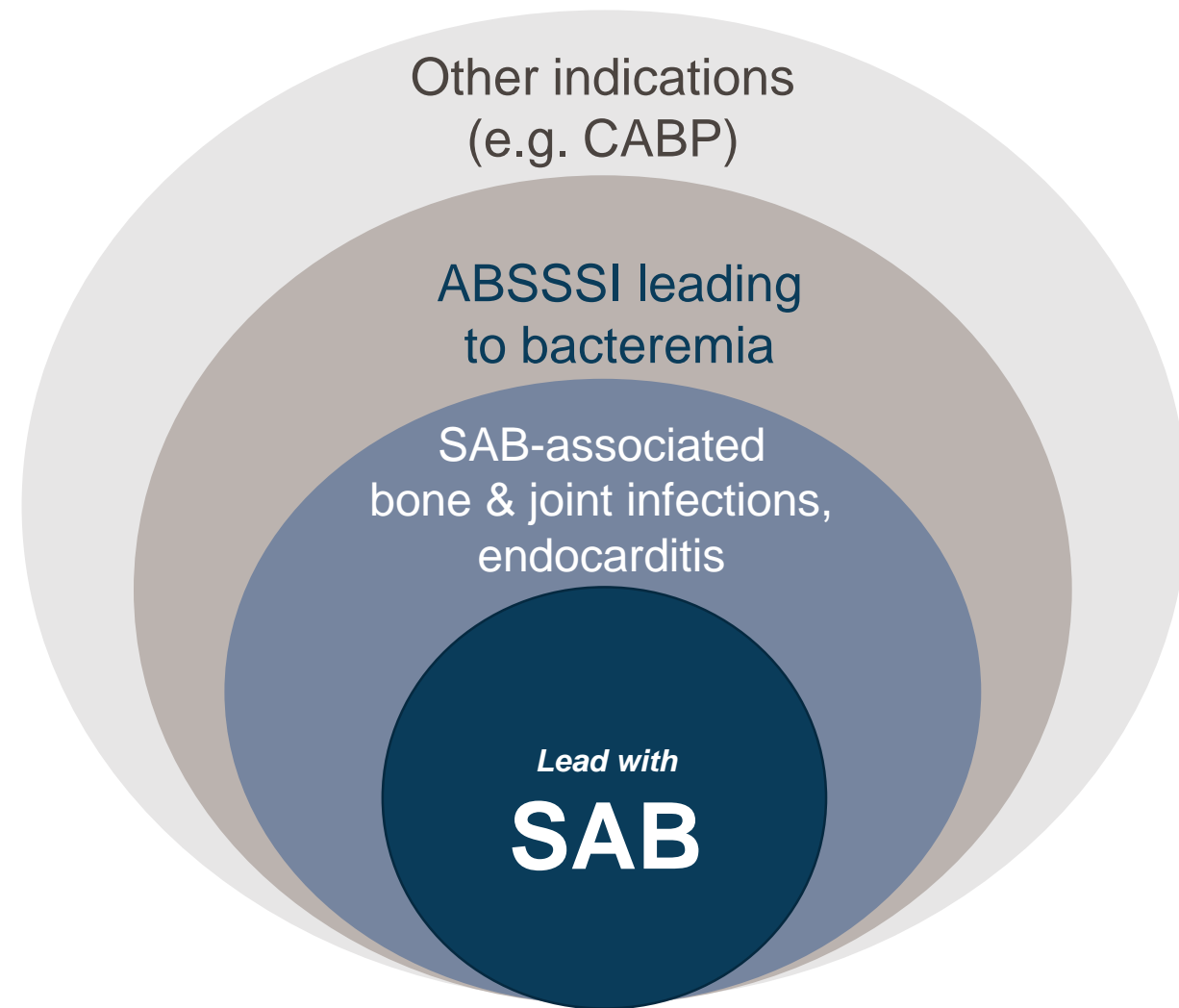
- Excellent treatment option in difficult-to-treat patients presenting to the hospital with severe infections, especially when the clinician suspects involvement of Gram-positive pathogens including *Staphylococcus aureus*
- Single agent first-line bactericidal broad-spectrum therapy with proven efficacy 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.





Financials & Outlook

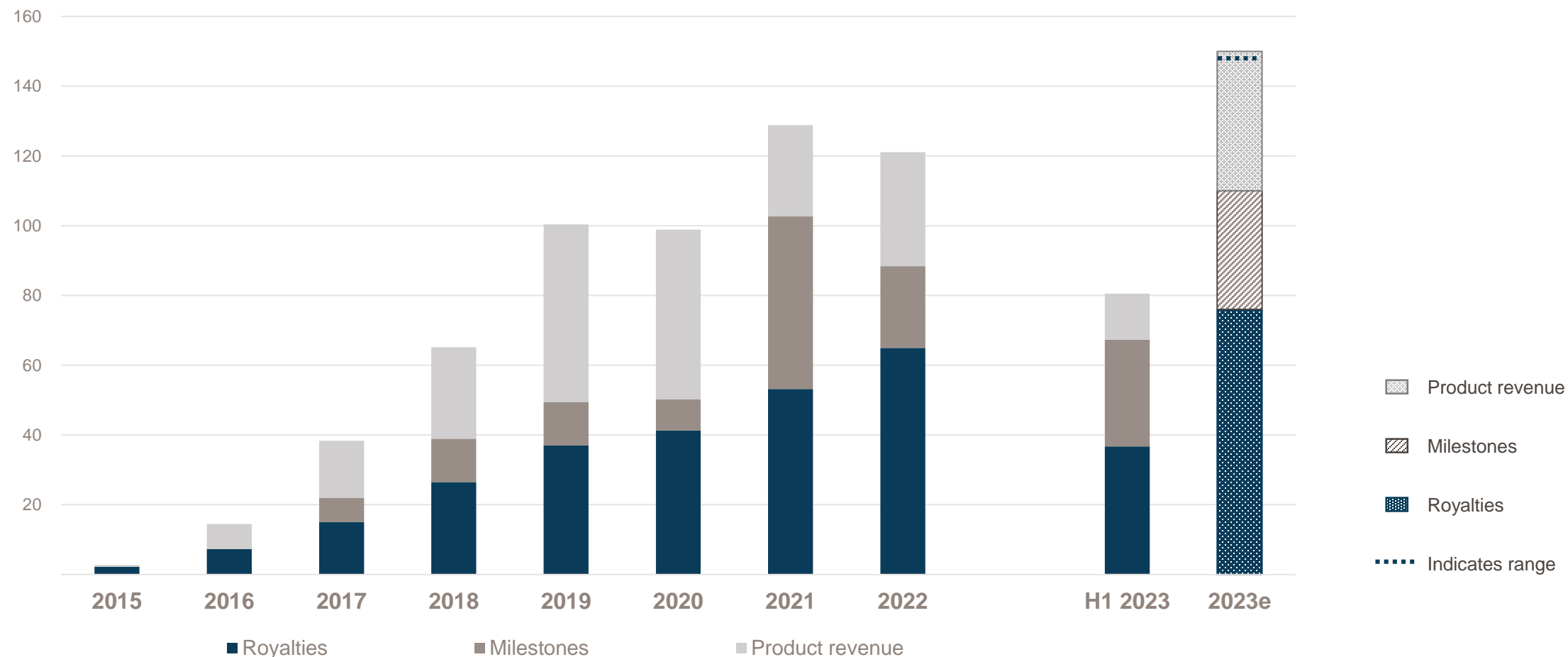


Strong financial half-year results 2023

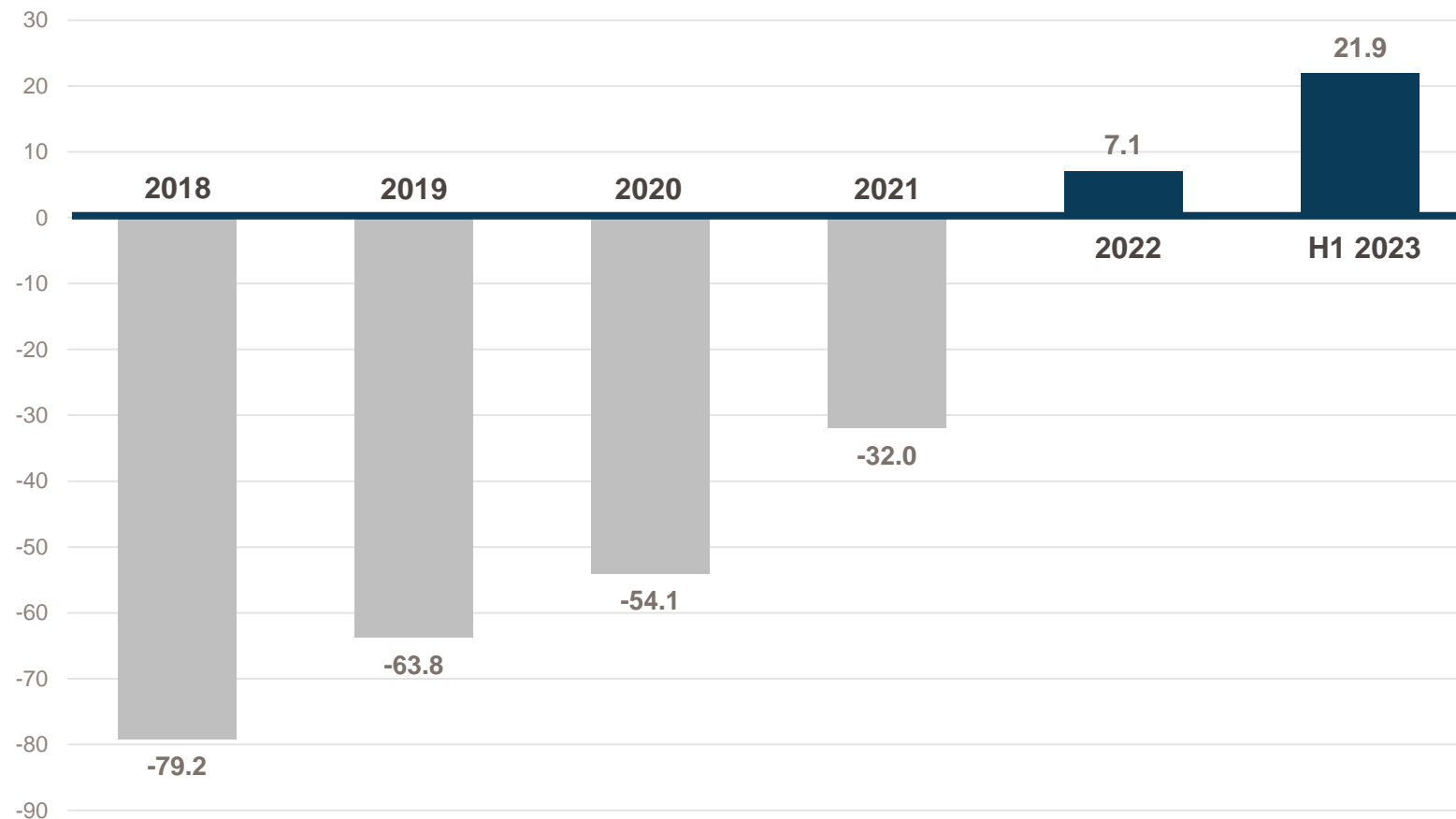
In CHF mn	H1 2023	H1 2022
Cresemba & Zevtera related revenue	80.5	51.2
Royalty income	36.7	28.9
Total revenue	84.9	57.6
Cost of products sold	10.0	14.9
Operating expenses	38.0	52.8
Operating profit/loss	36.9	(10.0)
Net profit/loss	31.8	(12.2)

Note: Consistent rounding was applied.

Cresemba & Zevtera related revenue breakdown (in CHF mn)

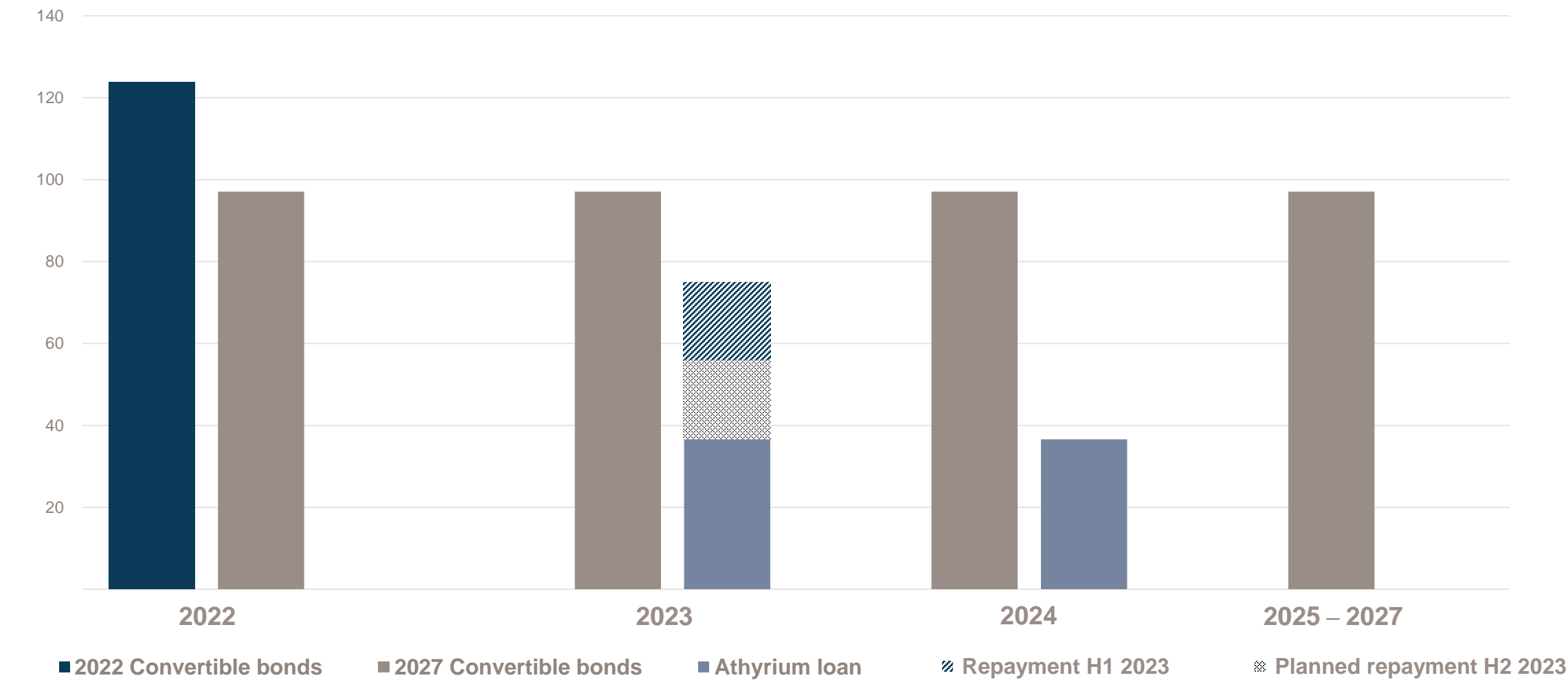


Cash flows from operating activities (in CHF mn)



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

Continued reduction of debt level (in CHF mn)



Note: Figures as of the beginning of the fiscal year; rounding applied consistently

Increased revenue and profit guidance for FY 2023

In CHF mn	FY 2022	FY 2023* (previous guidance)	FY 2023* (new guidance)
Cresemba & Zevtera related revenue	122.3	145 – 148	147 – 150
Royalty income	65.0	~74	~76
Total revenue	147.8	155 – 158	157 – 160
Cost of products sold Operating expenses	24.6 104.6	25 – 28 ~80	25 – 27 ~80
Operating profit	18.5	45 – 50	50 – 55
Net profit	12.1	36 – 41	41 – 46

*Excluding the impact of in-licensing activities

Note: Consistent rounding was applied.

Key milestones

Product	H1 2023	H2 2023	H1 2024
Ceftobiprole (Zevtera)		US NDA submission (August) ✓	Regulatory decision in the US (Q2)
			Executing US partnership (before regulatory decision)
Isavuconazole (Cresemba)	Launched in Japan ✓	Pediatric submissions ✓	

Increasing Cresemba & Zevtera revenue

In-licensing of anti-infectives (2023 and beyond)

Advancement of preclinical anti-infective assets

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

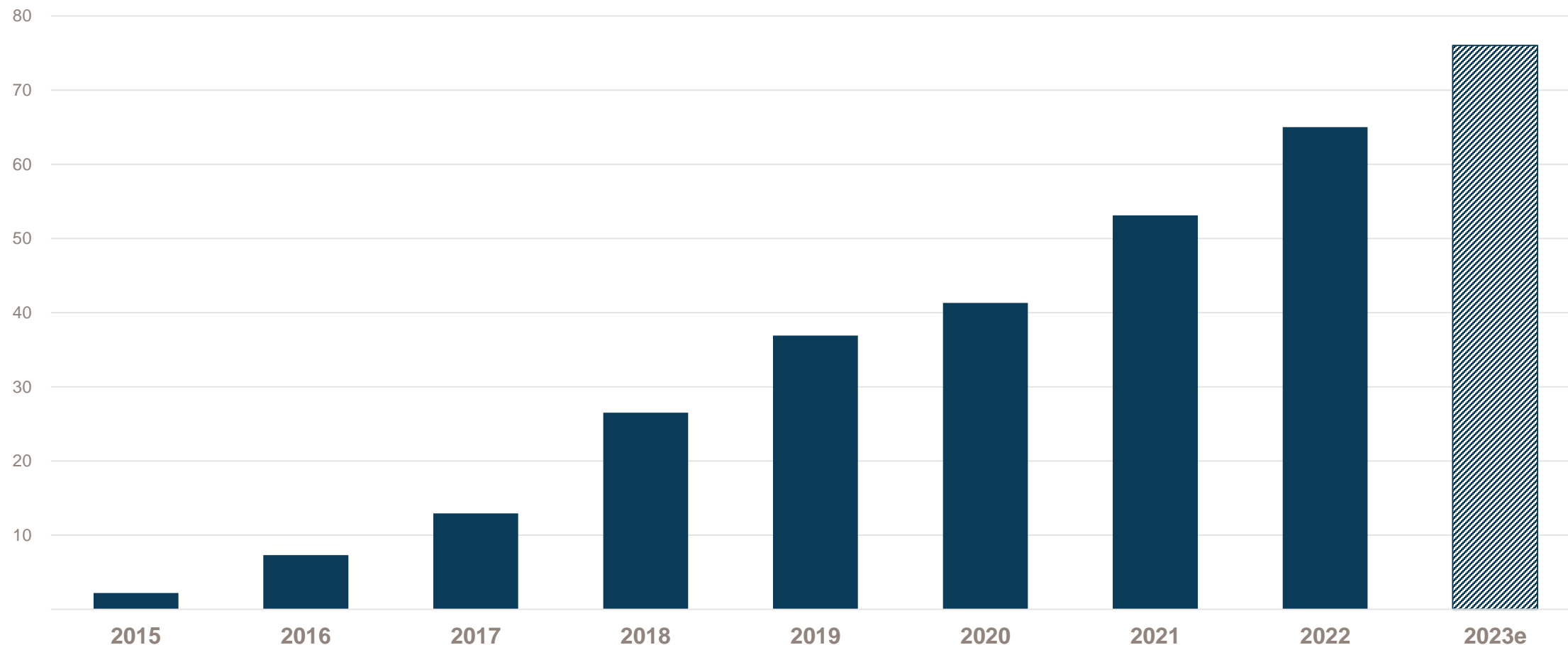
- Addressing serious hospital infections with increasing medical need
- Innovative & differentiated assets with potential for successful commercialization
- In-licensing assets from late stage research through to end of phase 2

Antibacterials

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

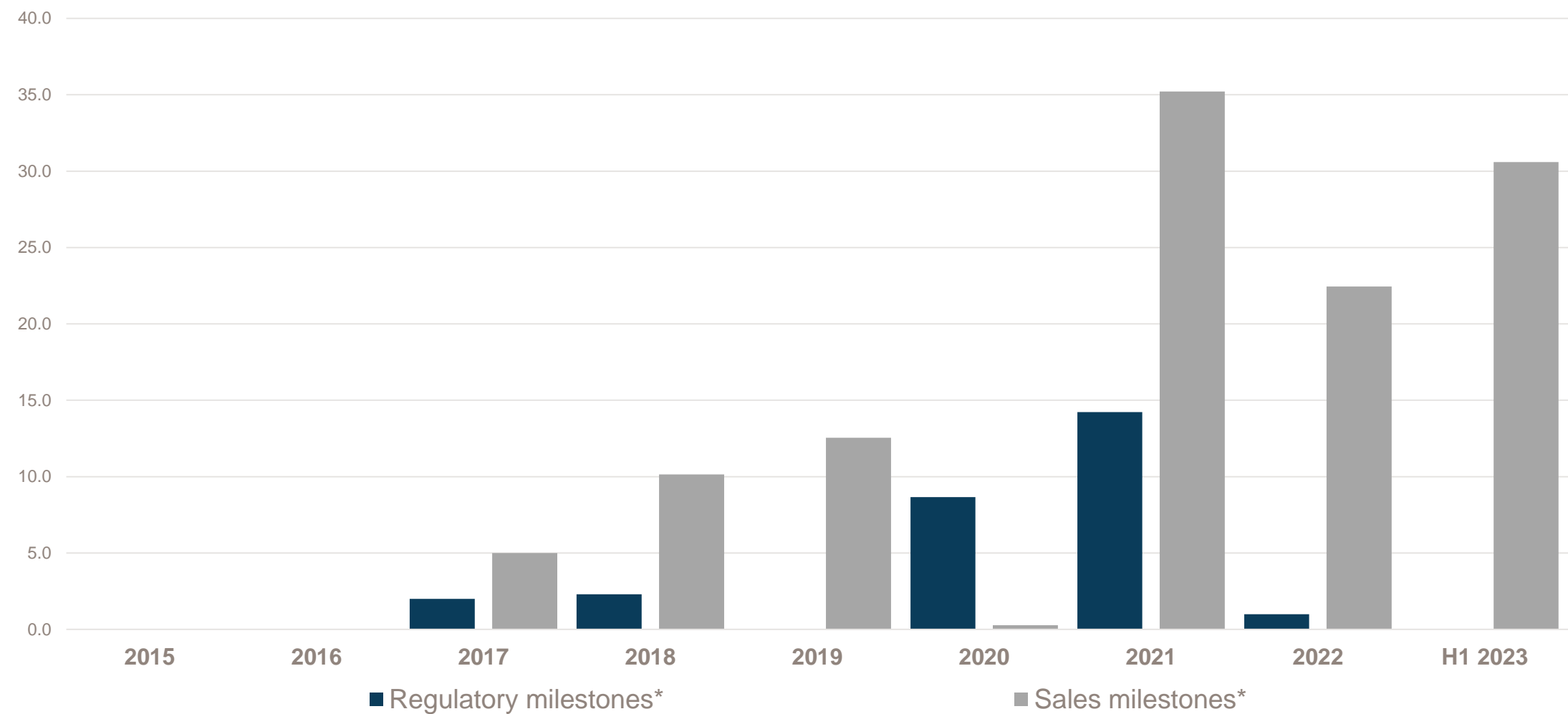
Appendix

Cresemba royalty income growth reflects continued commercial success in key territories (in CHF mn)



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

Regulatory and sales milestones (in CHF mn)

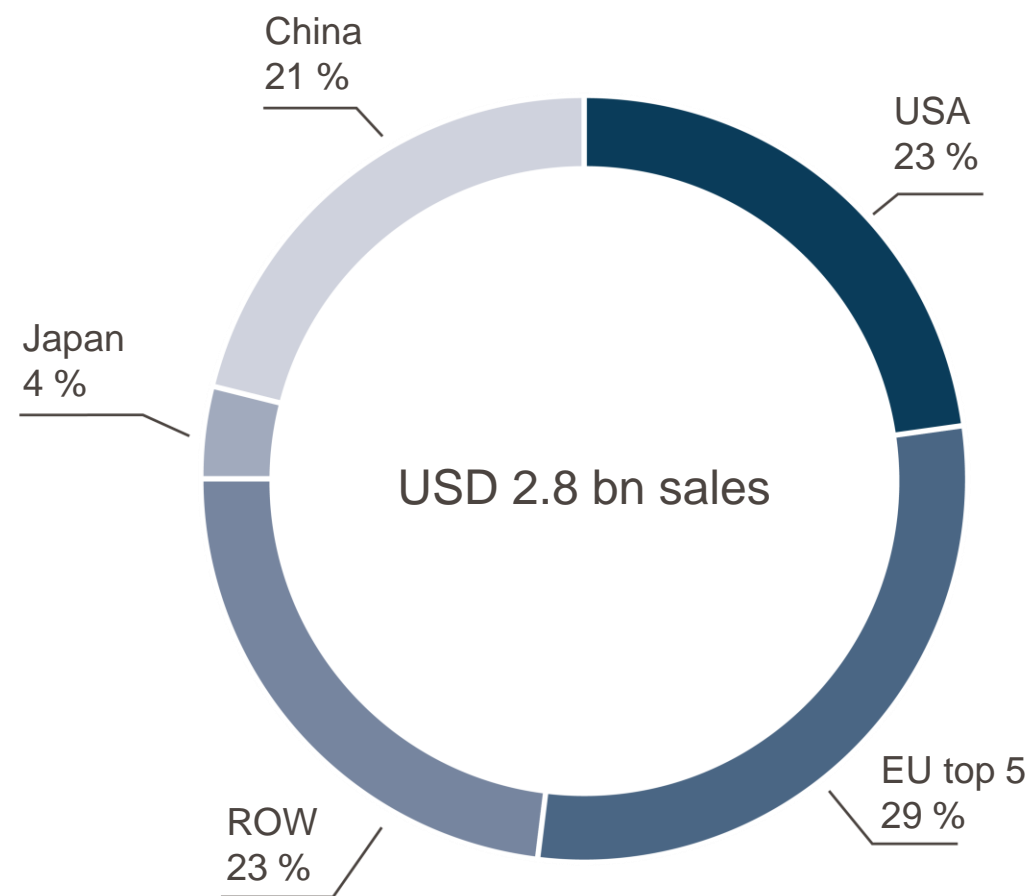


*Combined from license partners and distributors

Significant growth potential for Cresemba

- USD 2.8 bn sales of best-in-class antifungals* (MAT Q1 2023)
- Recently launched in Japan and China, representing 25% of global potential

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



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

Cresemba pediatric development program

- A pediatric development plan comprising 2 clinical studies was agreed with the FDA and the EMA
- Successful completion of the plan potentially results in 2 years additional market exclusivity in Europe and 6 months additional market exclusivity in the USA
- Clinical studies were undertaken in collaboration with Basilea's US partner Astellas and completed enrollment in August 2022

Study	Age group	Study design	Study identifier
Study 1 9766-CL-0046	1 – 17 years i.v. and capsules*	Phase 1, open-label, multicenter, non-comparative pharmacokinetics and safety study of intravenous and oral isavuconazole sulfate > Study completed in 2019 (49 patients enrolled)	Clinicaltrials.gov NCT03241550†
Study 2 9766-CL-0107	1 – 17 years i.v. and capsules*	Phase 2, open-label, non-comparative, multicenter study to evaluate the safety and tolerability, efficacy and pharmacokinetics of isavuconazonium sulfate for the treatment of invasive aspergillosis or invasive mucormycosis > Study completed in 2022 (31 patients enrolled)	Clinicaltrials.gov NCT03816176

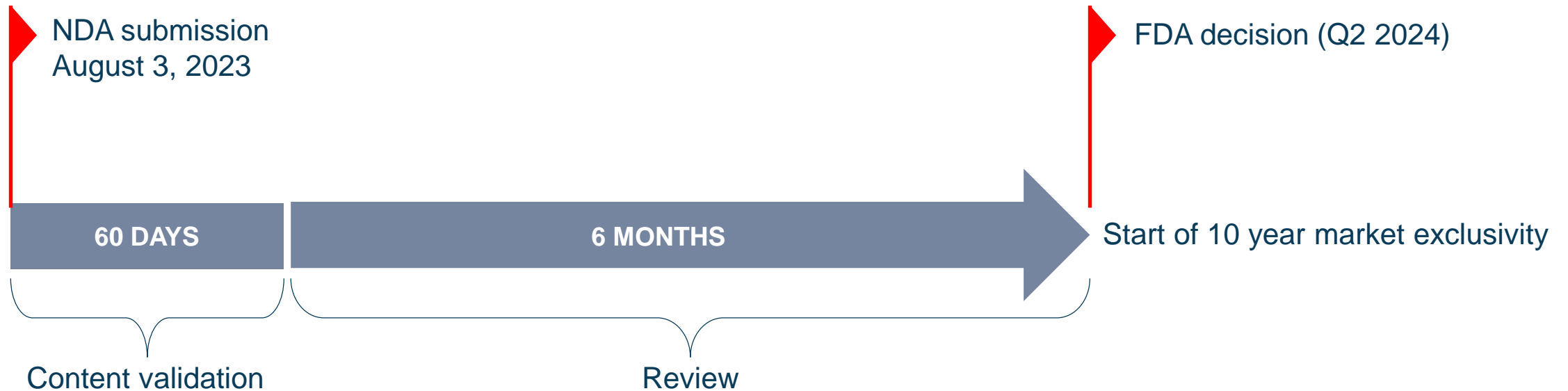
*Capsules only studied in children 6 years or older

†Arrieta AC, et al. Safety, Tolerability, and Population Pharmacokinetics of Intravenous and Oral Isavuconazonium Sulfate in Pediatric Patients. Antimicrob Agents Chemother. 2021;65:e0029021.

Ceftobiprole — FDA's NDA review process for a Qualified Infectious Disease Product

Ceftobiprole was granted QIDP status for SAB, ABSSSI and CABP

QIDP status provides 6 month priority review and extends market exclusivity to 10 years



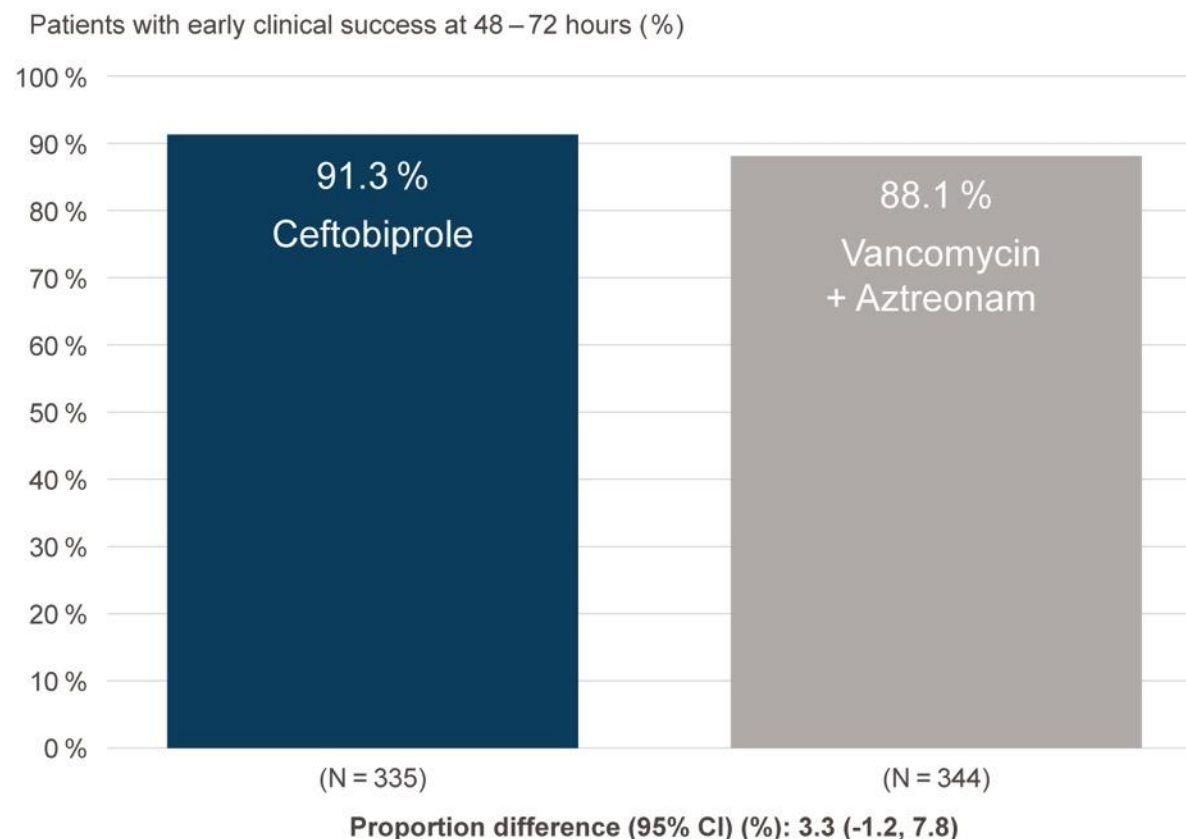
Ceftobiprole — Positive phase 3 results reported in ABSSSI

Results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints¹



¹ Overcash JS et al. Clin Infect Dis. 2021;73:e1507-e1517.

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



ITT: intent-to-treat

Pre-defined limit of non-inferiority = lower limit of 95 % CI for difference > -10 %

Ceftobiprole — Positive phase 3 results reported in ABSSSI

Key topline study¹ results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints

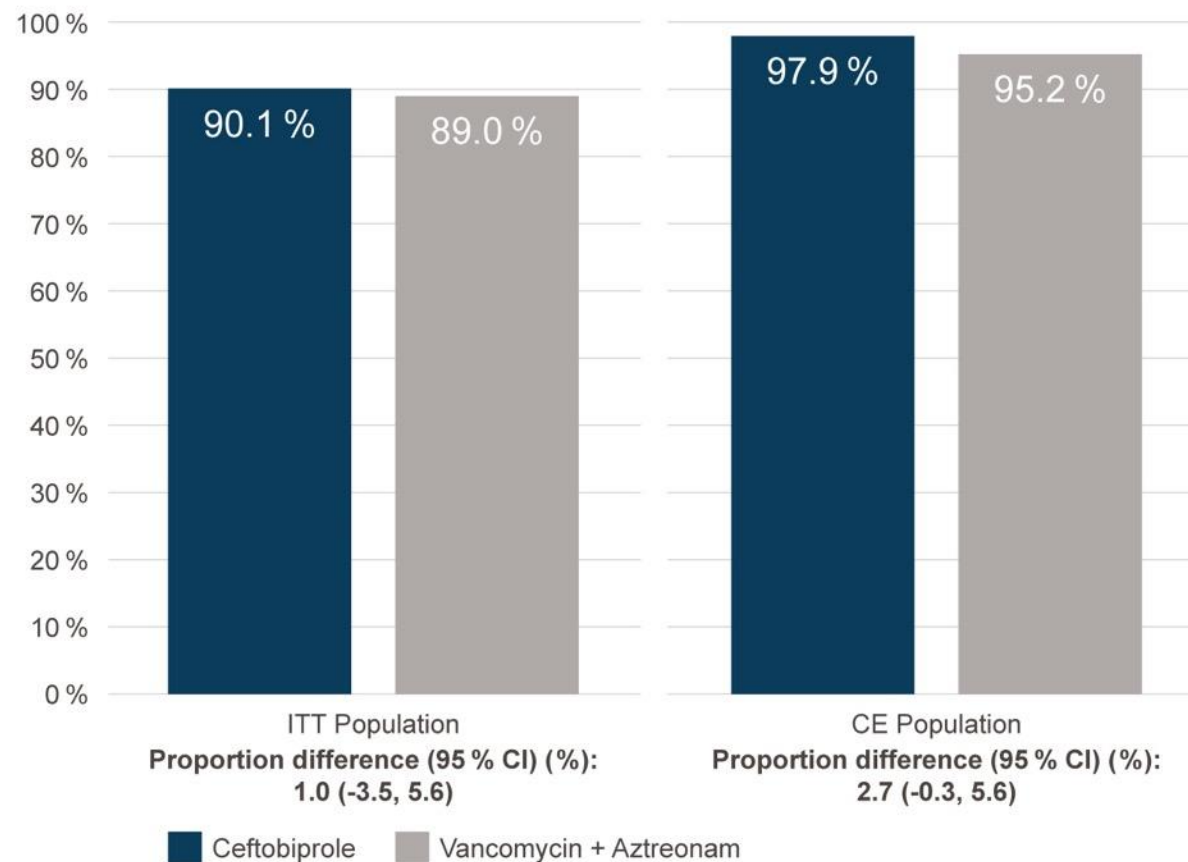


¹ NCT03137173

ABSSSI: Acute bacterial skin and skin structure infections

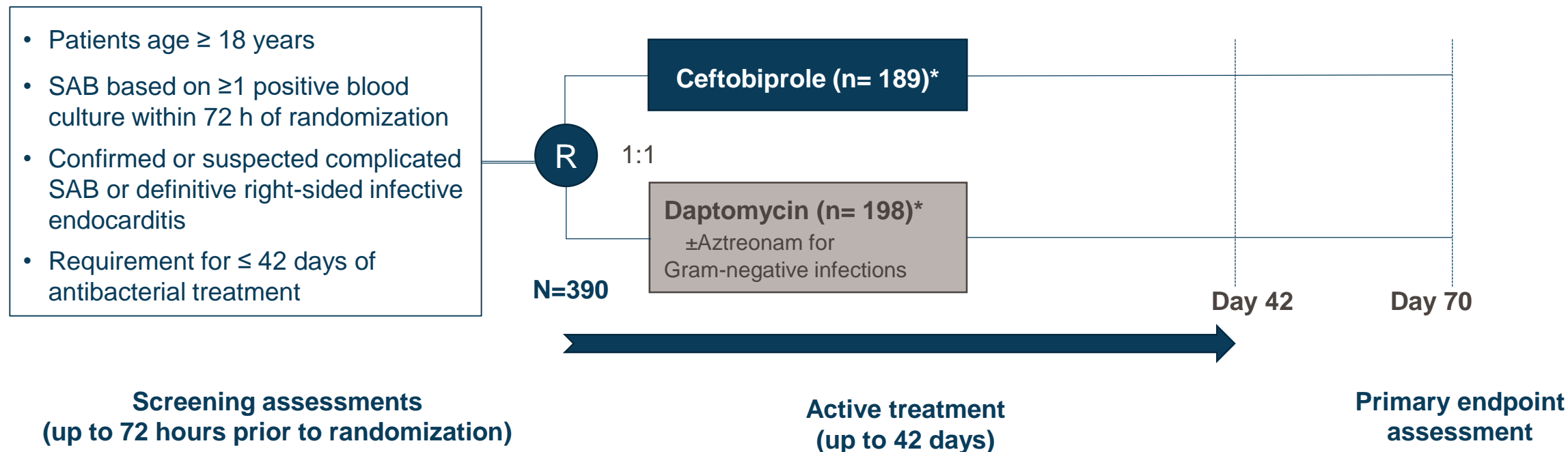
Investigator-assessed clinical success at test-of-cure (TOC) 15-22 days after randomization (ITT, CE populations)

Patients with clinical success at the TOC visit (%)



CE: clinically evaluable; ITT: intent-to-treat

ERADICATE — SAB Study design



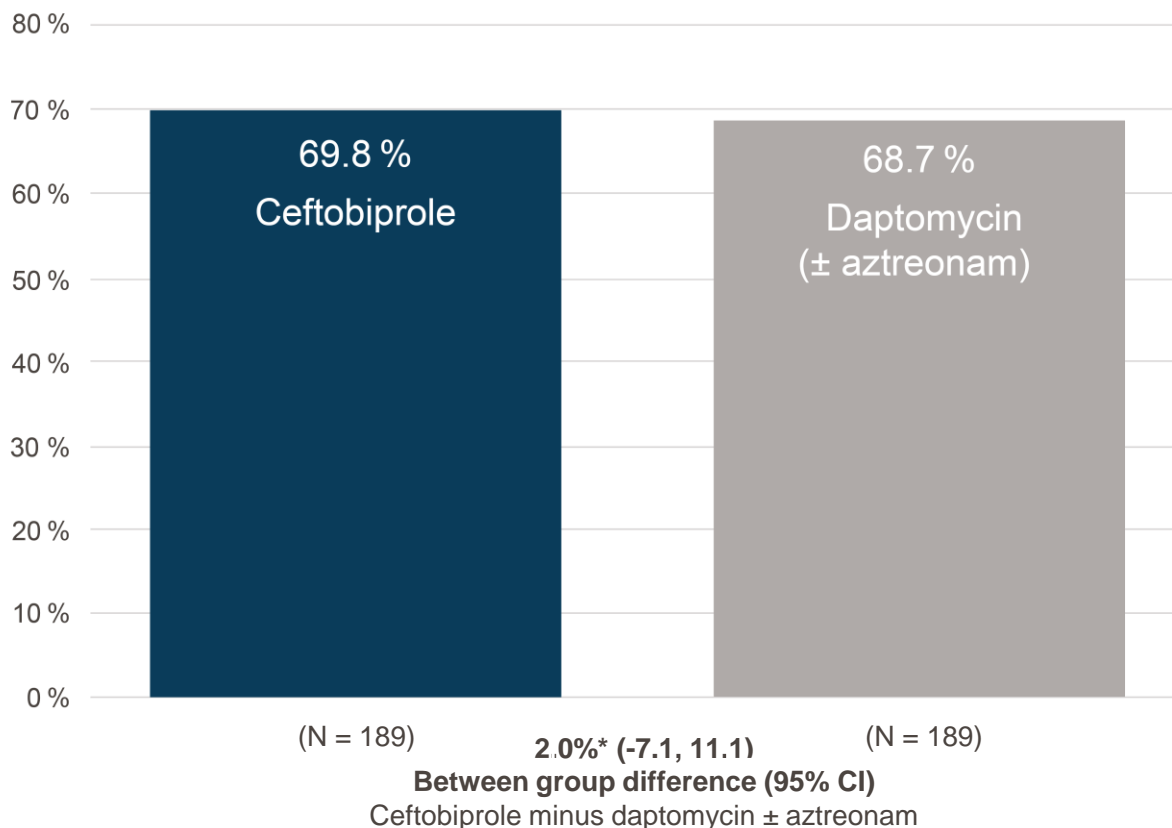
Adapted from Hamed K et al. Future Microbiol. 2020;15:35-48

*Ceftobiprole was administered 500 mg q6h on Day 1-8 and 500 mg q8h from Day 9 onwards. Daptomycin was administered at 6mg/kg up to 10 mg/kg q24h. Three patients in the ITT population were excluded from the modified intent-to-treat population (mITT): One patient was randomized but not dosed, and two patients did not have a positive *S. aureus* blood culture at baseline

Primary endpoint in SAB is achieved

(DRC assessed overall success at PTE in mITT population)

% Patients with overall success at PTE

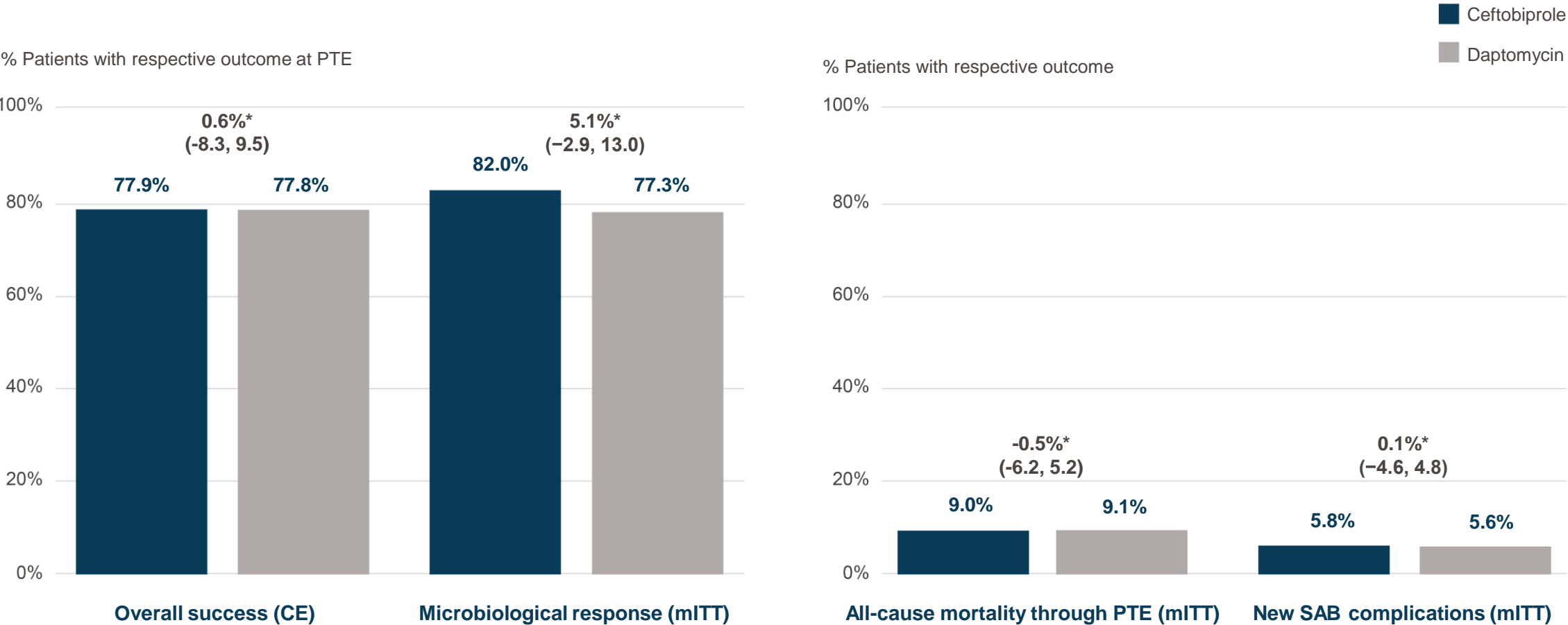


- Non-inferiority demonstrated based on the pre-defined non-inferiority margin of 15%
- Consistent results in key subgroups and various categories of underlying conditions:
 - MSSA or MRSA bloodstream infections at baseline
 - Skin and skin structure infections
 - Abdominal abscesses
 - Chronic dialysis
 - Septic arthritis
 - Osteomyelitis
 - Definite right-sided infective endocarditis
 - Patients with persistent SAB

DRC: Data review committee; PTE: Post-treatment evaluation visit at 70 days post-randomization

*Cochran-Mantel-Haenszel (CMH) weights method adjusted for actual stratum (dialysis status and prior antibacterial treatment use)

Secondary efficacy outcomes in SAB are similar



* Between-group difference (95%CI) of ceftobiprole minus daptomycin (± aztreonam), adjusted for actual stratum (dialysis status and prior antibacterial treatment use) using Cochran-Mantel-Haenszel weights method. CE: Clinically evaluable population.

ERADICATE — Further SAB results

- Median time to *Staphylococcus aureus* bloodstream clearance
 - MSSA: 3 days with ceftobiprole and 4 days with daptomycin
 - MRSA: 5 days for both ceftobiprole and daptomycin
- Emergence of resistance under treatment was observed in three patients on daptomycin.
No emergence of resistance under treatment was observed with ceftobiprole
- Observed ceftobiprole safety and tolerability profile is consistent with previous phase 3 studies and the post-marketing experience
- Ceftobiprole was well tolerated and overall rate of adverse events similar between the ceftobiprole and daptomycin groups; gastrointestinal side effects were more frequent with ceftobiprole (mainly driven by mild to moderate nausea)

Glossary

–	ABSSSI:	Acute b acterial s kin and s kin s tructure infections
–	BARDA:	B iomedical A dvanced R esearch and D evelopment A uthority
–	CABP:	C ommunity-acquired b acterial p neumonia
–	CE:	C linically e valuable
–	CARB-X:	C ombating A ntibiotic- R esistant B acteria Biopharmaceutical A ccelerator
–	DRC:	D ata review c ommittee
–	EMA:	E uropean M edicines A gency
–	FDA:	US F ood and D rug A dministration
–	HABP:	H ospital-acquired b acterial p neumonia
–	ITT:	I ntent- T o- T reat
–	i.v.:	I ntravenous
–	mITT:	M odified intent-to-treat
–	MSSA:	M ethicillin-susceptible <i>Staphylococcus aureus</i>
–	MRSA:	M ethicillin-resistant <i>Staphylococcus aureus</i>
–	NDA:	N ew D rug A pplication
–	OR:	O dds ratio
–	PTE:	P ost-treatment e valuation
–	QIDP:	Q ualified I nfectious D isease P roduct
–	SAB:	<i>Staphylococcus aureus</i> 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-associated p neumonia

Disclaimer and forward-looking statements

This communication, including the accompanying oral presentation, contains certain forward-looking statements, including, without limitation, statements containing the words “believes”, “anticipates”, “expects”, “supposes”, “considers”, and words of similar import, or which can be identified as discussions of strategy, plans or intentions. Such forward-looking statements are based on the current expectations and belief of company management, and are subject to numerous risks and uncertainties, which may cause the actual results, financial condition, performance, or achievements of Basilea, or the industry, to be materially different from any future results, performance, or achievements expressed or implied by such forward-looking statements. Such factors include, among others, the following: the uncertainty of pre-clinical and clinical trials of potential products, limited supplies, future capital needs and the uncertainty of additional funding, compliance with ongoing regulatory obligations and the need for regulatory approval of the company’s operations and potential products, dependence on licenses, patents, and proprietary technology as well as key suppliers and other third parties, including in preclinical and clinical trials, acceptance of Basilea’s products by the market in the event that they obtain regulatory approval, competition from other biotechnology, chemical, and pharmaceutical companies, attraction and retention of skilled employees and dependence on key personnel, and dependence on partners for commercialization of products, limited manufacturing resources, management’s discretion as to the use of proceeds, risks of product liability and limitations on insurance, uncertainties relating to public health care policies, adverse changes in governmental rules and fiscal policies, changes in foreign currency and other factors referenced in this communication. Given these uncertainties, prospective investors are cautioned not to place undue reliance on such forward-looking statements. Basilea disclaims any obligation to update any such forward-looking statements to reflect future events or developments, except as required by applicable law.



Focused on Growth and Innovation

**Hegenheimermattweg 167b
4123 Allschwil
Switzerland**

**info@basilea.com
www.basilea.com**

All rights reserved.
© 2023 Basilea Pharmaceutica International Ltd, Allschwil