Positive topline results ERADICATE phase 3 study

Webcast presentation

June 28, 2022

David Veitch

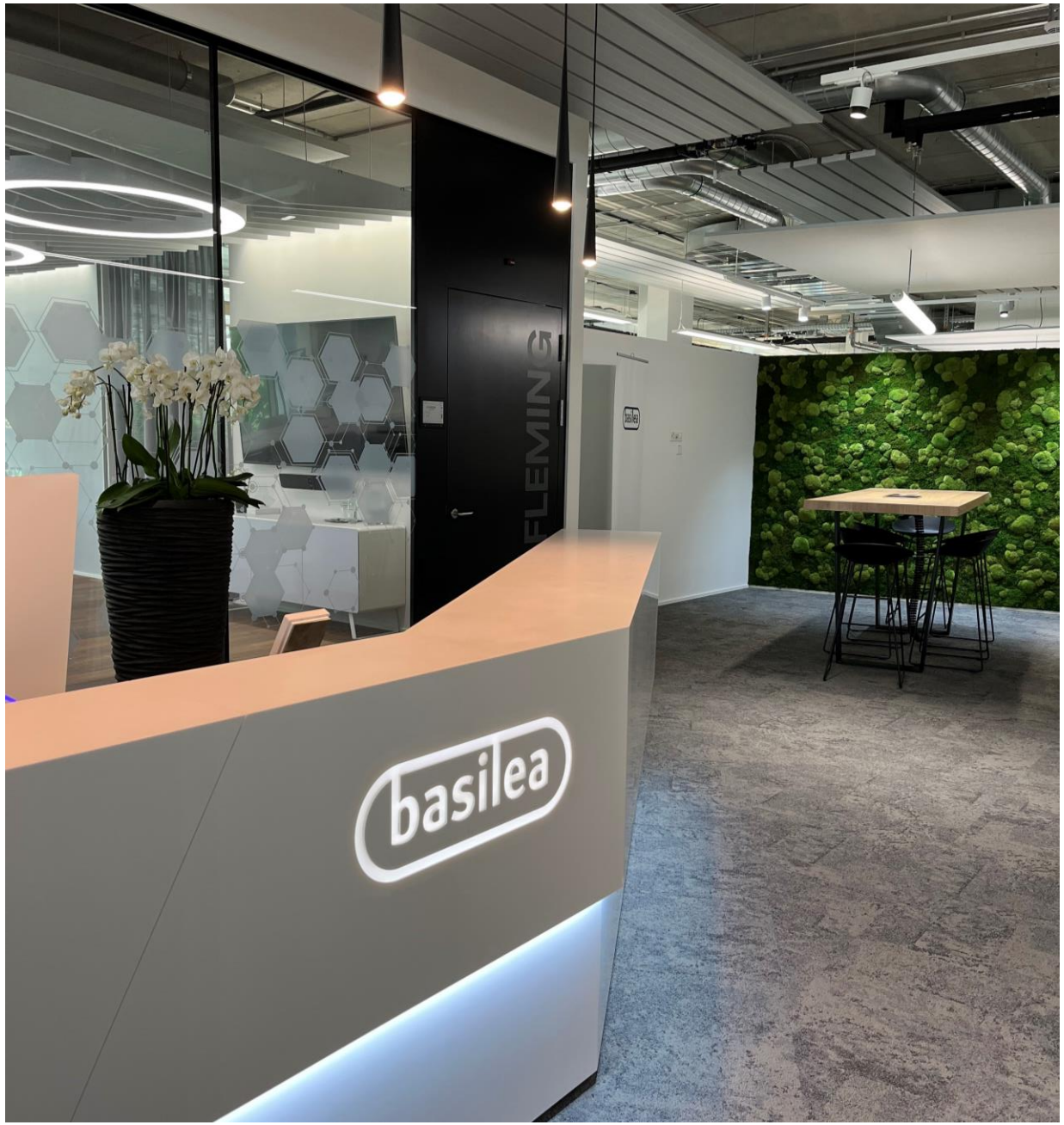
Chief Executive Officer

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. Derazantinib, lisavanbulin, BAL0891 and their uses are investigational and have not been approved by a regulatory authority for any use. Efficacy and safety have not been established. The information presented should not be construed as a recommendation for use. The relevance of findings in nonclinical/preclinical studies to humans is currently being evaluated.

At a glance

- Focused on the treatment of serious bacterial and fungal infections
- Well funded, with two revenue generating hospital anti-infective brands, Cresemba® and Zevtera®, complemented by preclinical pipeline
- Recognized ability to establish and manage partnerships in both the development and commercial phase, providing access to international markets
- Experienced team with the proven expertise to take compounds from research to market
- Listed on SIX Swiss Stock Exchange, SIX: BSLN
- Based in the Basel area life sciences hub, Switzerland



Dr. Marc Engelhardt

Chief Medical Officer

Strategy for accessing the U.S. market

- Two cross-supportive phase 3 studies under FDA Special Protocol Assessment (SPA)
  1. Acute bacterial skin and skin structure infections (ABSSSI)[1], successfully completed
  2. *Staphylococcus aureus* bacteremia (SAB)[2], successfully completed (topline results reported)

TARGET ABSSSI Study

ERADICATE SAB Study

- Phase 3 study in community-acquired bacterial pneumonia (CABP) previously completed[3]
  - Additionally explore the possibility of gaining approval for CABP as a third indication

- New Drug Application (NDA) submission planned around year-end 2022
- Qualified Infectious Disease Product (QIDP) designation extends U.S. market exclusivity to 10 years from approval
- Commercialization planned through partnership

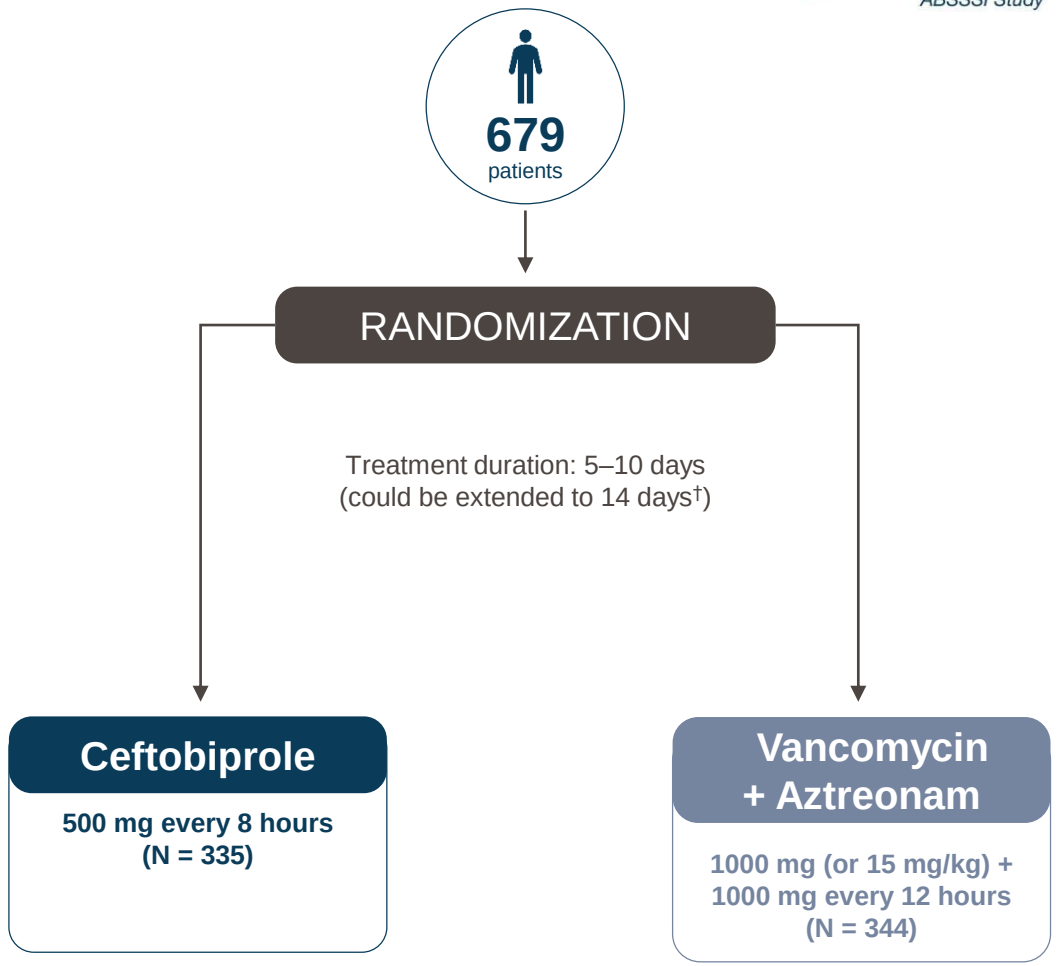
- The phase 3 program has been funded in part (~70% of total program costs; up to USD ~134 mn) with Federal funds from the Department of Health and Human Services; Office of the Assistant Secretary for Preparedness and Response; Biomedical Advanced Research and Development Authority, under Contract No. HHSO100201600002C

BARDA

[1] Overcash JS et al. Clin Infect Dis. 2021;73:e1507-e1517. (NCT03137173)
[2] Hamed K et al. Future Microbiol. 2020;15:35-48. (NCT03138733)
[3] Nicholson SC et al. International Journal of Antimicrobial Agents 2021 (39), 240-246

TARGET (ABSSSI) study design

- Double-blind, randomized, controlled, multicenter (32 sites in the U.S., Bulgaria, Hungary and Ukraine), phase 3 study
- Primary objective: Compare the safety and efficacy of ceftobiprole with vancomycin plus aztreonam in skin and skin structure infections[1]



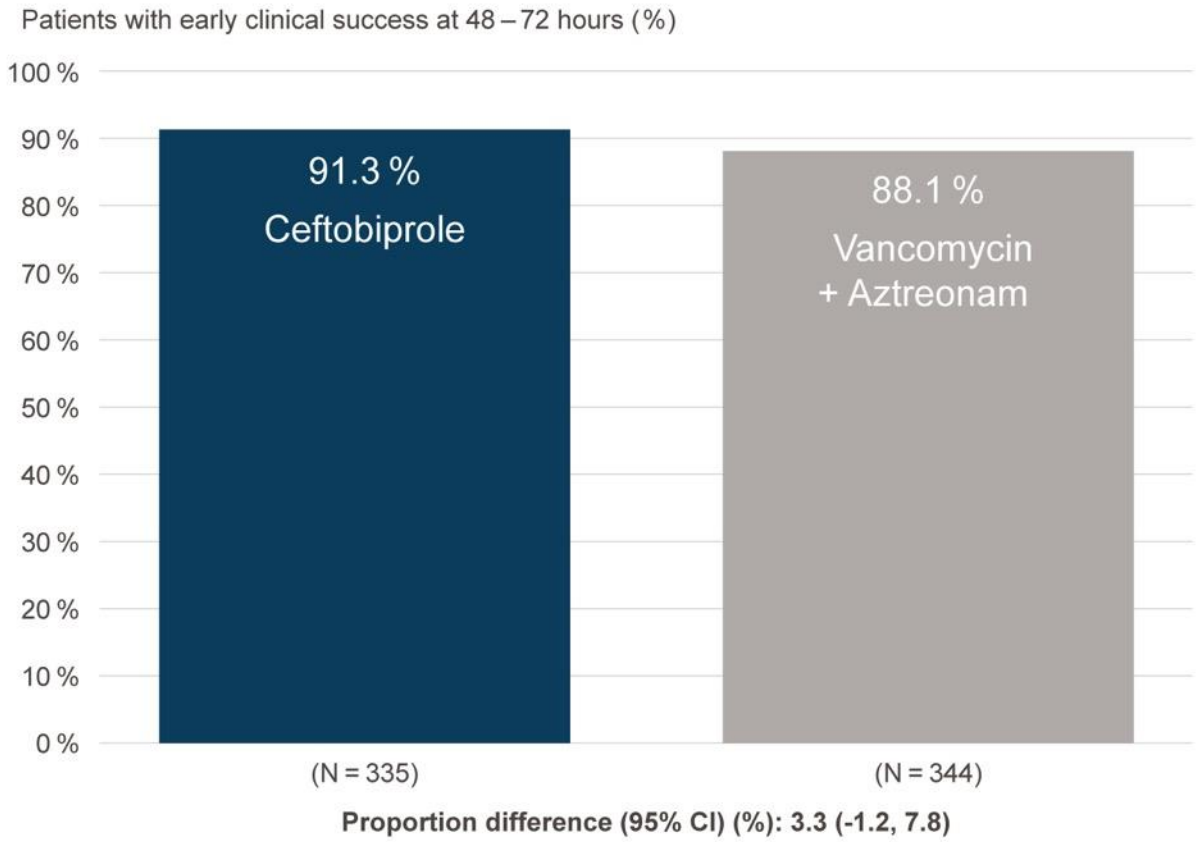
Stratified by study site and type of ABSSSI;
ABSSSI, acute bacterial skin and skin structure infections.

[1]Overcash JS, et al. Clinical Infectious Diseases 2020; 73:e1507-17.

TARGET (ABSSSI)
Positive results reported

Results showing non-inferiority of ceftobiprole to vancomycin plus aztreonam for the primary and secondary endpoints[1]

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

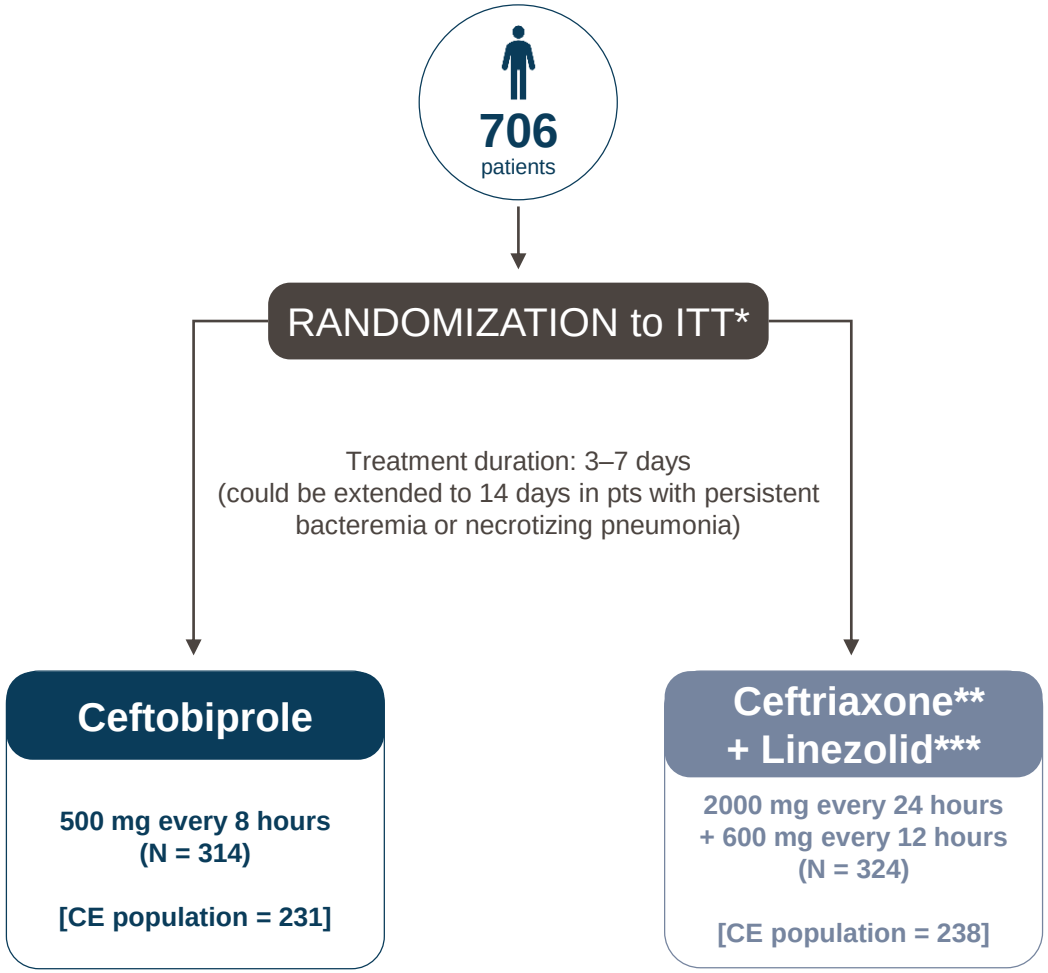


ITT: intent-to-treat
Pre-defined limit of non-inferiority = lower limit of 95 % CI for difference > -10 %

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

CABP phase 3 study design

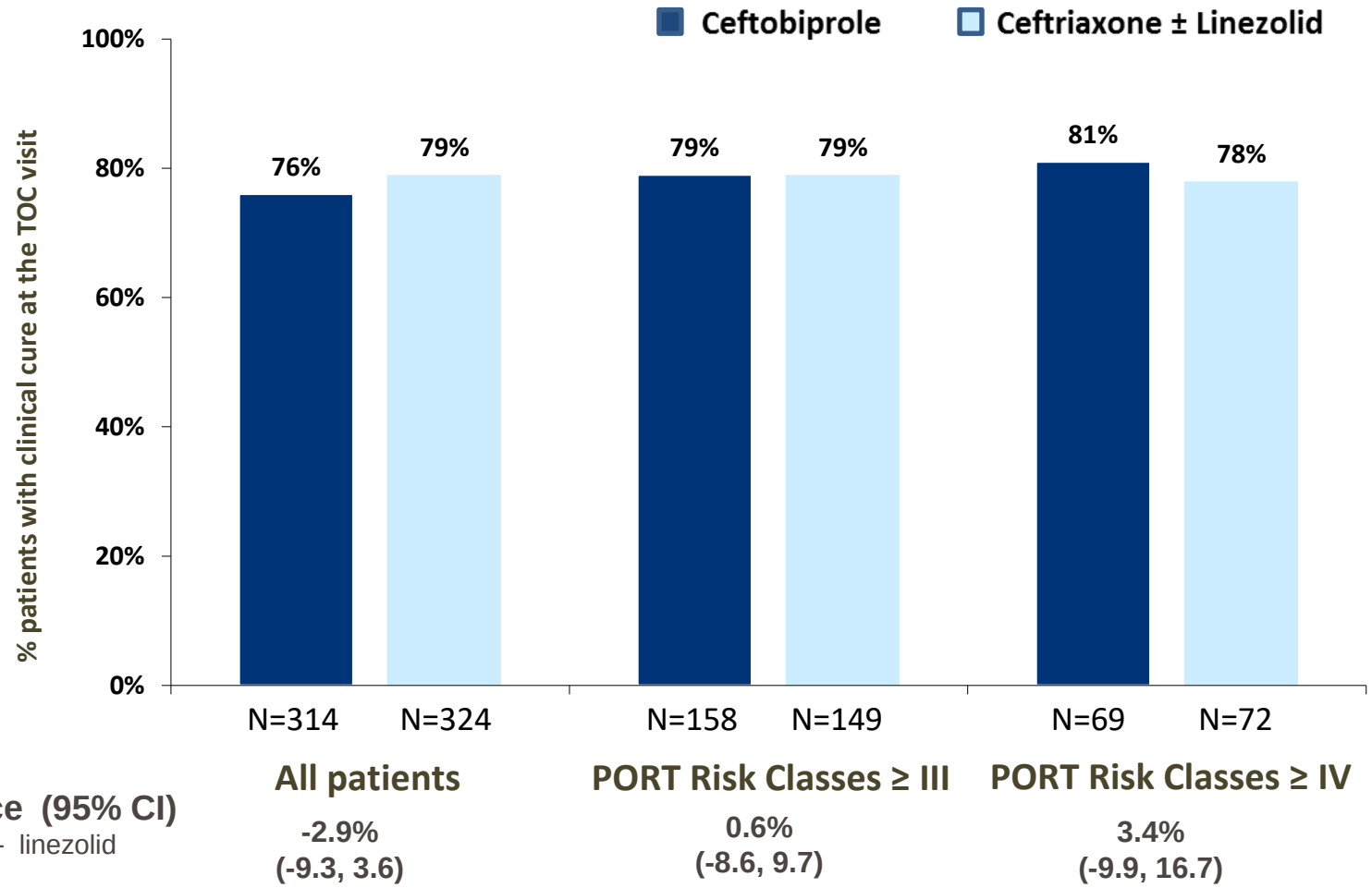
- Double-blind, randomized, controlled, multicenter (103 sites in the U.S., Europe, Latin America, Hong Kong, China, South Korea and Taiwan), phase 3 study
- Primary objective: Non-inferiority of ceftobiprole medocaril compared with ceftriaxone with/without linezolid (comparator) with respect to the clinical cure rate in subjects hospitalized with community-acquired bacterial pneumonia (CABP) at the test-of-cure (TOC) visit (ITT and CE populations)



* Stratified: Pneumonia Severity Index (<90 versus ≥91); need for anti-staphylococcal therapy
** After 3 days patients could switch oral therapy
*** If MRSA suspected by the investigator

Nicholson SC et al. Int J Antimicrob Agents. 2012 39:240-6.

CABP: primary endpoint is achieved
Clinical cure at TOC (ITT analysis) – predefined endpoint



Between group difference (95% CI)
Ceftobiprole minus Ceftriaxone+/- linezolid

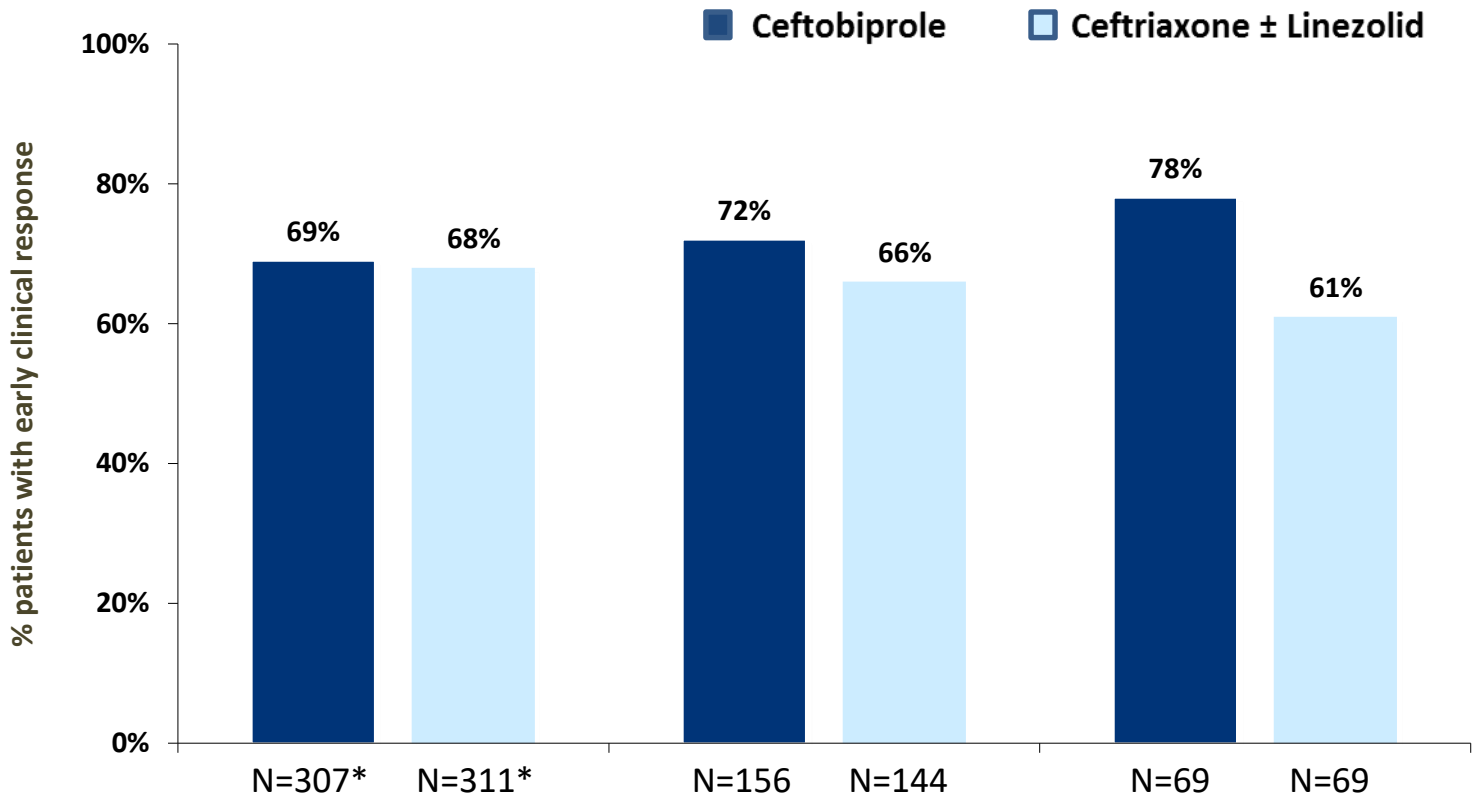
All patients
-2.9%
(-9.3, 3.6)

PORT Risk Classes ≥ III
0.6%
(-8.6, 9.7)

PORT Risk Classes ≥ IV
3.4%
(-9.9, 16.7)

Nicholson SC et al. Int J Antimicrob Agents. 2012 39:240-6.

CABP: Post-hoc analysis: revised FDA endpoint achieved
Early clinical response (ITT)



Between group difference (95% CI) Ceftobiprole minus ceftriaxone+/- linezolid	All patients	PORT Risk Classes ≥ III	PORT Risk Classes ≥ IV
	0.6% (-6.8, 7.9)	5.8% (-4.7, 16.3)	17.4% (2.3, 32.5)

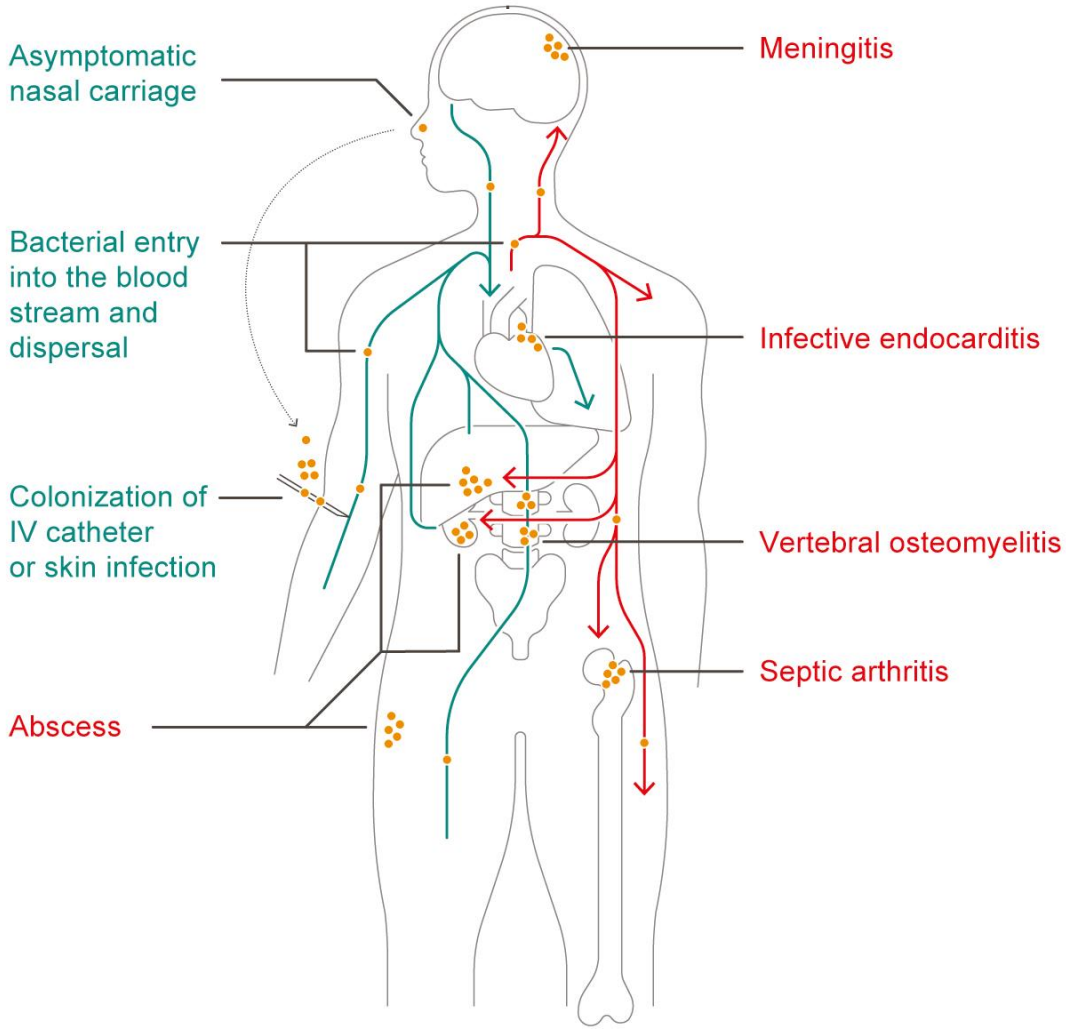
*Analysis population is a sub-population of the ITT population that could be assessed for the revised FDA early endpoint

Welte, T. *et al.* 2014 ECCMID Poster eP431

SAB – an area with high medical need

- Nearly 120,000 *S. aureus* bloodstream infections in the U.S. (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 mortality[2]
- 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

Causes and consequences of SAB



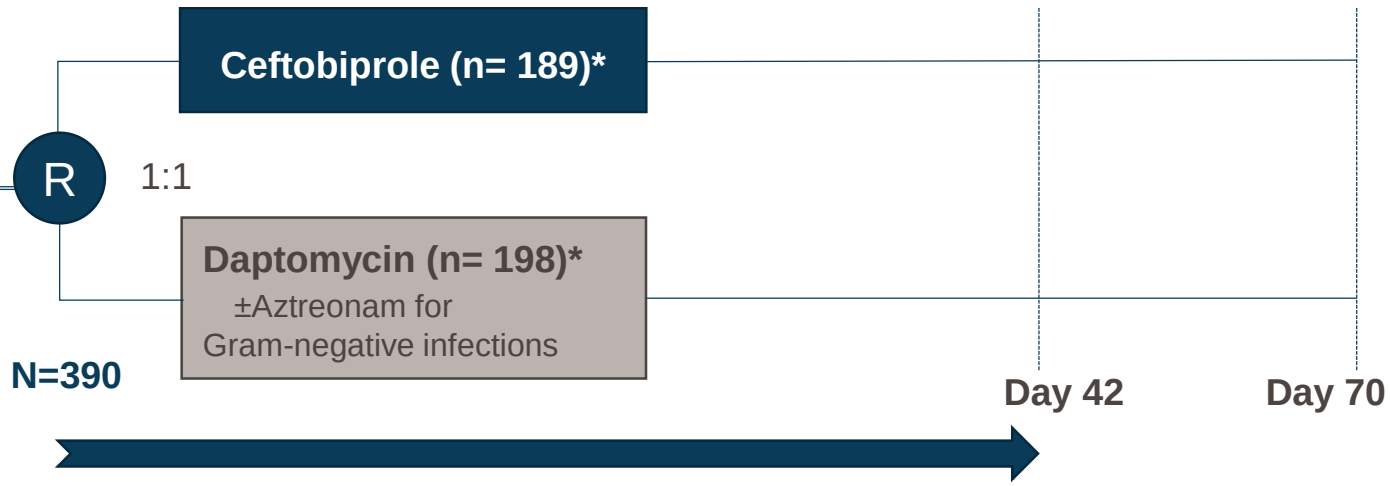
Adapted from Edwards AM et al. Trends Microbiol. 2011;19:184-190.

[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*

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

ERADICATE (390 patients) is the largest randomized study conducted for registrational purposes of a new antibiotic treatment in *Staphylococcus aureus* bacteremia

- Patients age ≥ 18 years
- SAB based on ≥1 positive blood culture within 72 h of randomization
- Confirmed or suspected complicated SAB or definitive right-sided infective endocarditis
- Requirement for ≤ 42 days of antibacterial treatment



Screening assessments (up to 72 hours prior to randomization)

Active treatment (up to 42 days)

Primary endpoint assessment

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

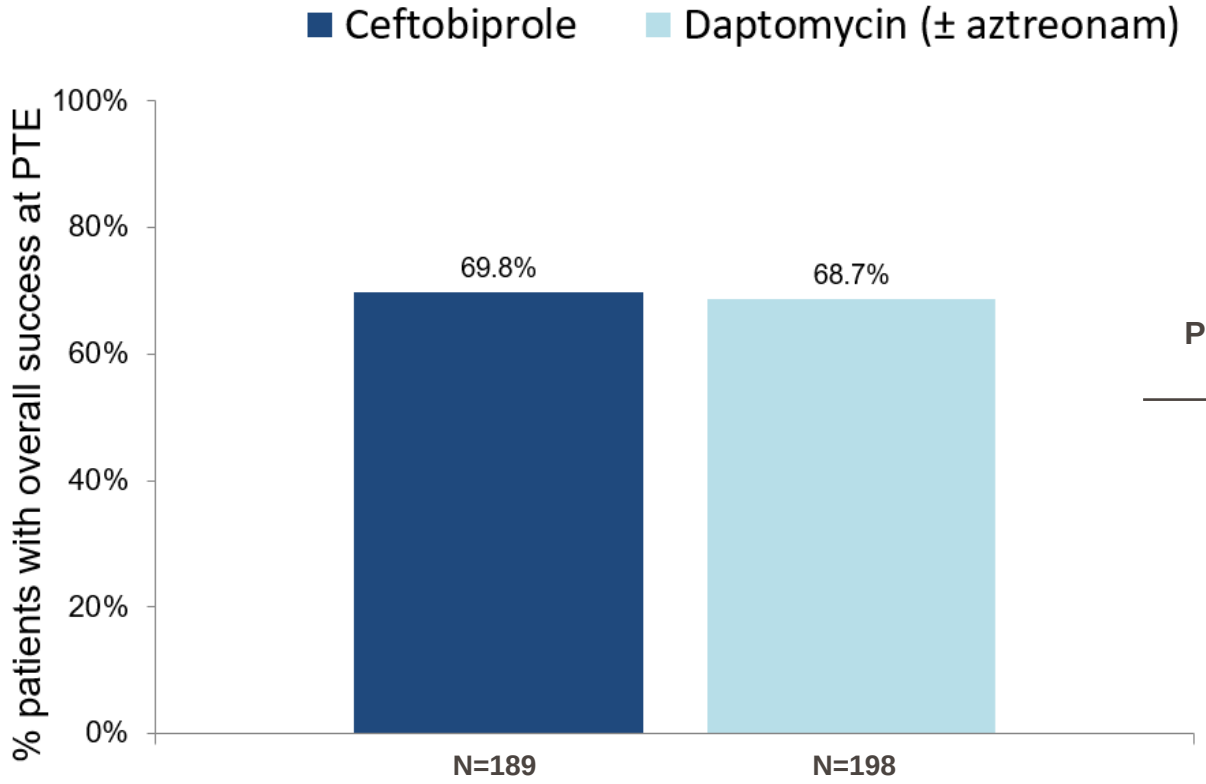
Study overview

Primary objective	To demonstrate non-inferiority of ceftobiprole to daptomycin ± aztreonam in patients with *S. aureus* bacteremia (SAB), including infective endocarditis (IE) at a non-inferiority margin of 15%
Study design	Multi-country, randomized (1:1), double-blind, non-inferiority study
Study population	390 adult patients with complicated SAB, including patients undergoing chronic intermittent hemodialysis, patients with persistent SAB, skin infections, septic arthritis, septic thrombophlebitis, septic pulmonary emboli, abdominal or thoracic abscesses, osteomyelitis, cerebral or epidural abscesses, or right-sided infective endocarditis
Primary endpoint	Overall success at the PTE visit (70 days after randomization) assessed by an independent Data Review Committee (DRC) in the modified intent-to-treat (mITT) population: Overall success required: • Survival • Absence of metastatic foci or other SAB complications • Resolution or improvement of SAB-related signs and symptoms • *S. aureus* bloodstream clearance. Criteria for treatment failure included the use of systemic concomitant non-study antibiotics exceeding predefined thresholds
Secondary endpoints	All-cause mortality, microbiological eradication, development of metastatic foci or other complications of SAB, time to *S. aureus* bloodstream clearance, safety and tolerability.

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

Primary endpoint is achieved

DRC assessed overall success at PTE in mITT population



Proportion difference (95% CI) (%):
2.0 (-7.1, 11.1)
Pre-defined lower bound of non-inferiority = lower limit of 95% CI for difference >-15%

Between group difference (95% CI)	**2.0%***
Ceftobiprole minus daptomycin ± aztreonam	**(-7.1, 11.1)**

DRC: Data review committee; PTE: Post-treatment evaluation
*Cochran-Mantel-Haenszel (CMH) weights method adjusted for actual stratum (dialysis status and prior antibacterial treatment use)

ERADICATE: Other results

- Secondary efficacy endpoints including all-cause mortality, new complications of SAB and overall success in the clinically evaluable population were consistent with the primary study outcome
- Ceftobiprole was well tolerated and the overall rate of adverse events was similar between the two treatment groups
- The observed safety and tolerability profile was consistent with previous phase 3 studies and the post-marketing experience with ceftobiprole
  - As expected, gastrointestinal side effects were more frequent with ceftobiprole

Conclusions

- The ERADICATE study in patients with complicated SAB met the primary and secondary efficacy objectives, supporting the efficacy and safety of ceftobiprole in this indication
- Ceftobiprole was well tolerated and the observed safety profile consistent with previous phase 3 studies and the post-marketing experience with ceftobiprole
- Cross-supportive data package consisting of successful TARGET phase 3 study and successful ERADICATE phase 3 study
  - Special protocol assessment agreement achieved with FDA for both studies
- The study results support an NDA filing, which is planned around year end 2022
- Basilea will seek approval for SAB and ABSSSI
  - In addition, Basilea will explore the possibility for gaining approval for CABP as a third indication

Ceftobiprole key attributes

- Advanced generation cephalosporin with broad-spectrum bactericidal activity against Gram-positive organisms, including MRSA and MSSA, and Gram-negative organisms[1]
- Efficacy demonstrated in Phase 3 clinical studies in *Staphylococcus aureus* bacteremia, acute bacterial skin and skin structure infections, and pneumonia[1, 2]
- Low propensity for resistance development[1]
- Established safety profile consistent with the cephalosporin class, demonstrated in both adult and pediatric patients[1, 2, 3]

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

David Veitch

Chief Executive Officer

Outlook

Strategy focused on anti-infectives

Significantly growing cash revenues from Cresemba and Zevtera:

Cresemba

- 29% royalty income growth in 2021
- > USD 324 mn global in-market sales in 12-months to December 2021
- 2022: launched in China and regulatory decision expected in Japan

Zevtera

- Preparing to file NDA for U.S. around year-end 2022
- U.S. is the most important MRSA market ~80–90% of global potential
- Qualified infectious disease product (QIDP) designation provides 10 years market exclusivity from approval
- Commercialization in U.S. planned with a partner

Preclinical assets

- A number of preclinical programs, including DXR inhibitor (CARB-X funded) and a potential first-in-class broad spectrum antifungal
- Focus on external sourcing of additional preclinical and clinical anti-infective compounds

Sustainable profitability from 2023

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.

Outlook 2022

Ceftobiprole

Completed patient enrolment in phase 3 SAB study (ERADICATE) ✓

Isavuconazole

- Marketing approval in Japan
- Marketing approval in China ✓
- Launched in 70 countries by year-end

Ceftobiprole

Published topline results of phase 3 SAB study (ERADICATE) ✓

Ceftobiprole

File U.S. NDA for SAB and ABSSSI; explore CABP as additional indication

Increasing Cresemba (isavuconazole) & Zevtera (ceftobiprole) revenue

Advancement of preclinical anti-infective assets

In-licensing of anti-infectives

Strategic transactions in order to maximize the value of oncology portfolio

H1 22	H2 22

Q & A

Thank you

Glossary

- ABSSSI: **A**cute **b**acterial **s**kin and **s**kin **s**tructure **i**nfections
- CABP: **C**ommunity-**a**cquired **b**acterial **p**neumonia
- CARB-X: **C**ombating **A**ntibiotic-**R**esistant **B**acteria Biopharmaceutical **Accelerator**
- DRC: **D**ata **r**eview **c**ommittee
- 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
- ORR: **O**bjective **r**esponse **r**ate
- PTE: **P**ost-**t**reatment **e**valuation
- SAB: ***S**taphylococcus **a**ureus* **b**acteremia
- TOC: **T**est-**o**f-**c**ure

Focused on Growth and Innovation

Hegenheimermattweg 167b
4123 Allschwil
Switzerland

info@basilea.com
www.basilea.com

Appendix

Analysis populations

- **Intent-to-treat population (ITT):** All randomized patients with patients analyzed according to the study medication assigned
- **Safety population:** All randomized patients who received any amount of study medication with patients analyzed according to the first medication actually received
- **Modified intent-to-treat population (mITT):** Subset of ITT population who have received any amount of study medication, and have a blood culture positive for *S. aureus* at baseline based on a central microbiology laboratory assessment or based on unequivocal evidence of a baseline blood culture positive for *S. aureus* at the local laboratory
- **Clinically evaluable population (CE):** Subset of mITT population who have complied with important aspects of the study, e.g., no major protocol violations and not indeterminate outcome