

# Investor Presentation

December 2017

Destiny  Pharma

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## Beyond Antibiotics

### Novel Drugs to Combat Bacterial Infection & Resistance



Clinical stage biotech, targeting antibiotic-resistant bacterial infections in hospitals



Lead drug asset, XF-73, has multiple drivers for adoption in an area of global unmet medical need, with a potentially expedited route to regulatory approval



XF-73 significantly de-risked, 5 successful clinical trials completed, no evidence of resistance. Next major clinical trial is Phase IIb



Proprietary, anti-microbial drug platform – “XF Drugs”



Market exclusivity, including robust IP protection that potentially extends into 2030s



Clearly defined value creation opportunity, significant addressable markets, including blockbuster potential for XF-73’s lead indication



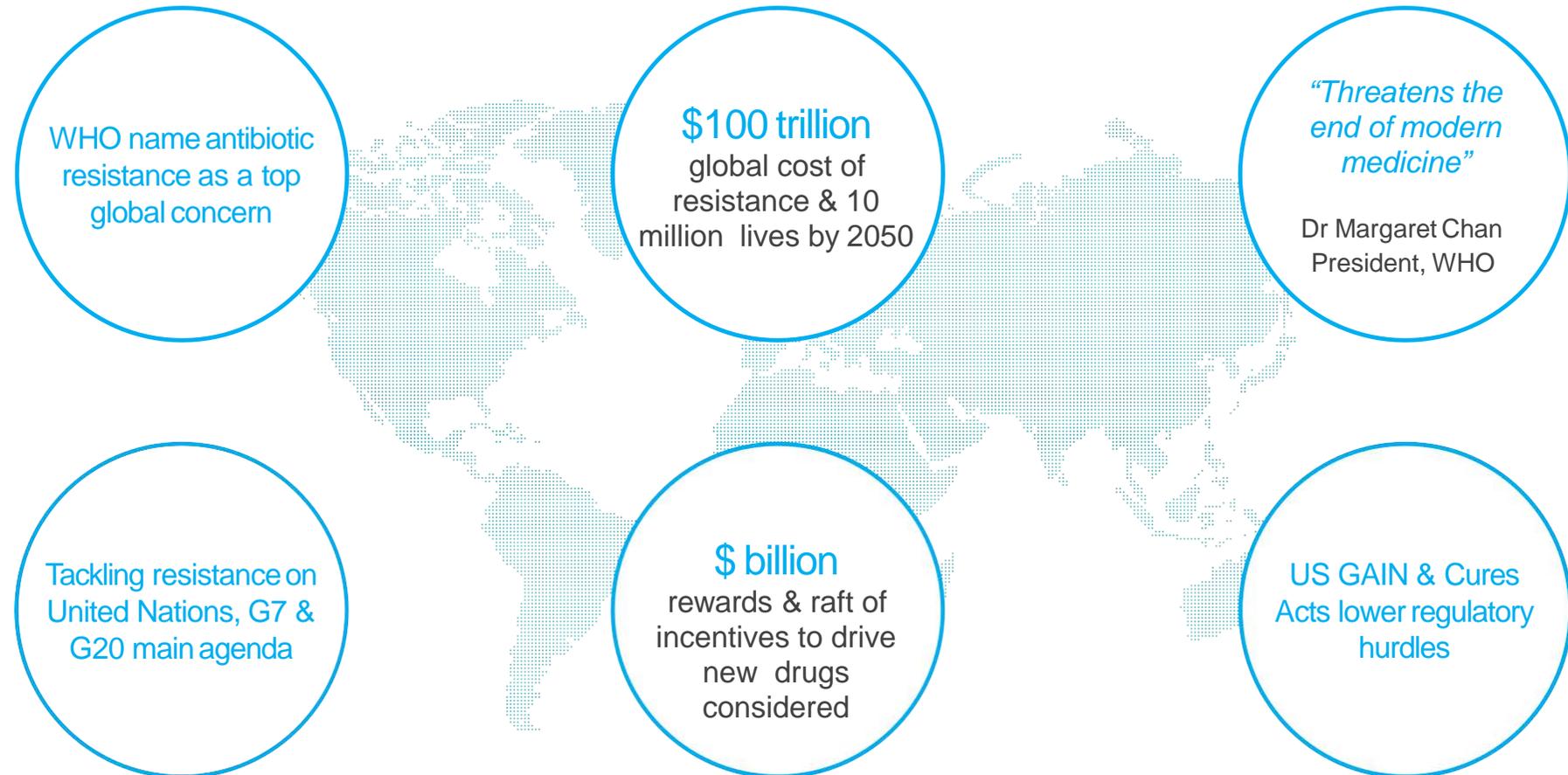
IPO in London in September 2017 raising £15.3m (London AIM:DEST). Additional £3m raised November 2017



Regional partnership deal signed with China Medical Systems November 2017

# Tackling Anti-Microbial Resistance

## A Global Imperative



XF Drugs address resistance and opens new preventative markets

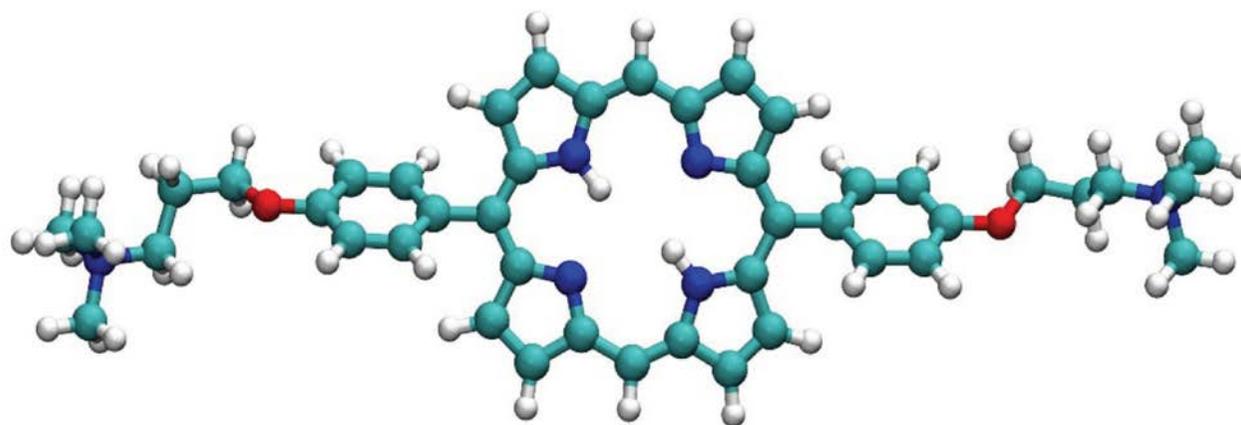
# XF Drug Platform

## Potential Solution to Antibiotic Resistance

	Antibiotic	XF Drug
Ultra-rapid, bacterial kill (within minutes)	⊗	⊙
MRSA unable to become resistant to drug action	⊗	⊙
Potential for widespread use	⊗	⊙
Kills all antibiotic resistant Gram positive bacteria tested	⊗	⊙
Kills any stage of bacterial growth – including bacterial Biofilms	⊗	⊙
FDA, QIDP & Fast Track status	⊙	⊙

### XF-73, Lead Drug from Platform

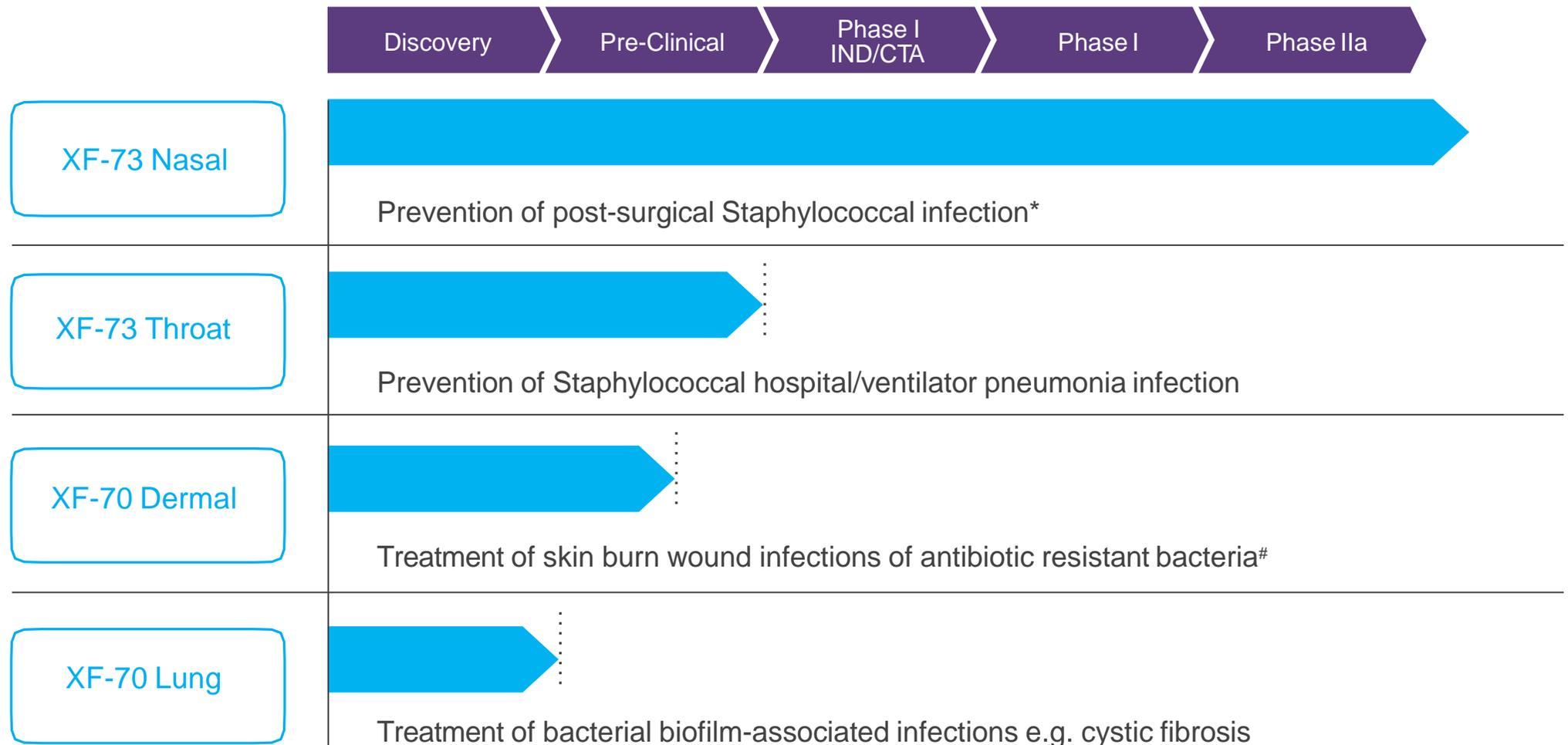
- INN = exeporfinium chloride
- Novel drug structure
- IP/market extensions into the 2030s
- 3 main steps, chemical synthesis
- Activity against selected Gram negative bacteria



Molecular structure of XF-73

# XF Drug Platform

## Potential Solution to Antibiotic Resistance

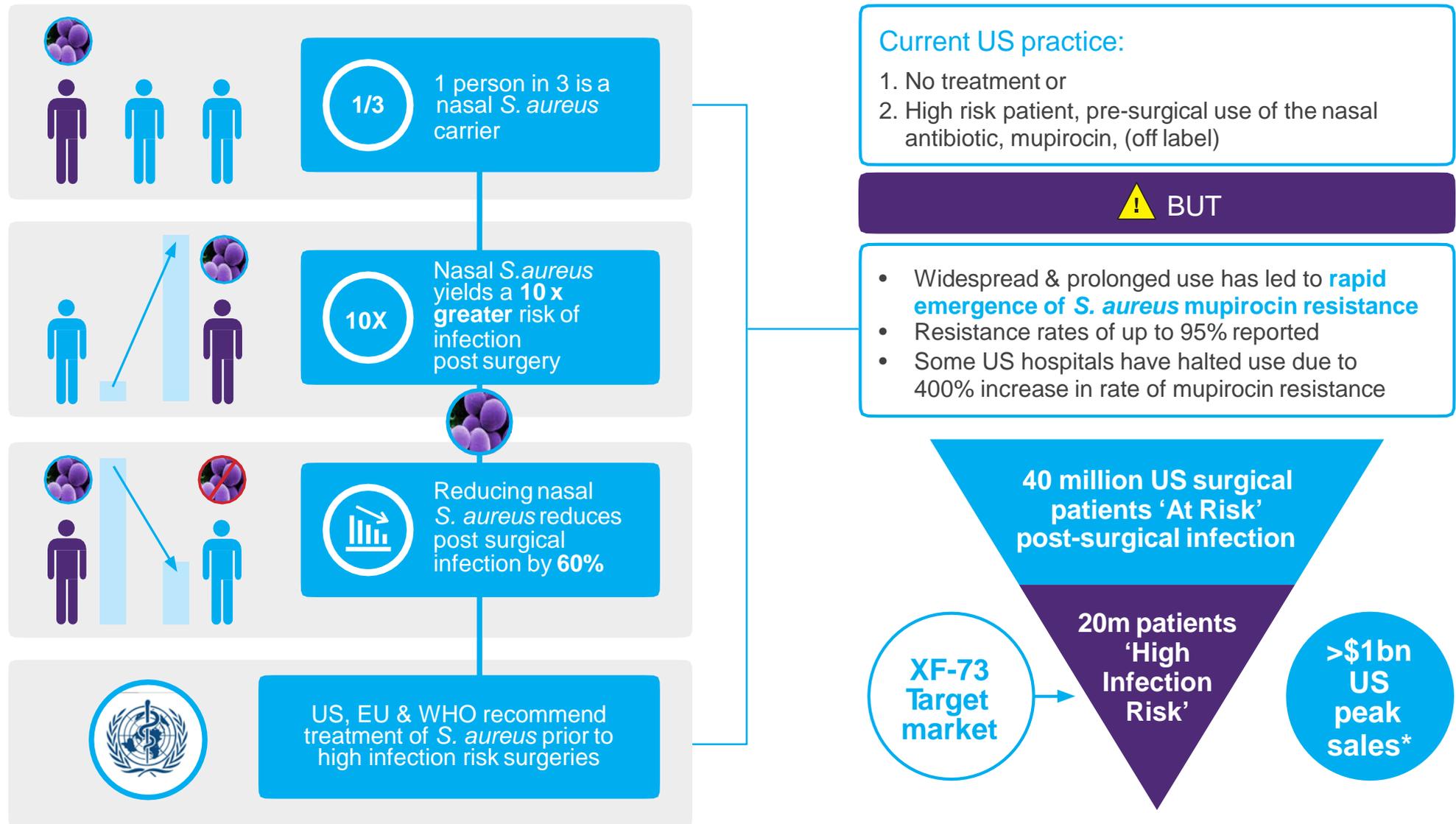


\* New US disease indication, QIDP designated by FDA, October 2015

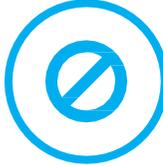
# Gram negative (*A. baumannii*, *P. aeruginosa*) & Gram positive (*E. aureus*) bacteria

# XF-73: Focus on 'Prevention of post-surgical *Staphylococcus aureus* infections'

## Blockbuster Opportunity



\* Benchmark to Bactroban, a mupirocin nasal ointment (GSK)

Drivers	Status	Drivers	Status
<p>Guidelines</p> 	<p>Recent guidelines (US SIS, SHEA, IDSA &amp; WHO) support increased use of preventative approaches, but antibiotic resistance recognised as an issue with mupirocin</p>	<p>Cost Effective</p> 	<p>Economic modelling studies demonstrate preventative approach to <i>S. aureus</i> infection more cost effective than treatment</p>
<p>Hospital Rankings</p> 	<p>US hospital administrators motivated to reduce infection rates to help push their ranking in published hospital listings</p>	<p>Financial Penalties</p> 	<p>US general, acute-care and short term hospitals with the highest MRSA infections will have 1% of their medicare reimbursements withheld</p>
<p>Litigation</p> 	<p>The direct costs of the medical malpractice liability system in the US are widely estimated to be on the order of \$20-\$30 billion per year</p>	<p>Governments</p> 	<p>Recent UN General Assembly* call for new drugs to tackle antibiotic resistance – recommendations for new incentives to come. On G7 &amp; G20 agendas too</p>

XF-73 Well Positioned for Value Based Pricing



XF-73 granted Qualified Infectious Disease Product (QIDP) status in November 2015

+5 years US market exclusivity

Fast Track status available

XF-73 will be the 1st drug approved for the newly defined indication of  
“Prevention of post-surgical Staphylococcal infections”

Only requires efficacy vs placebo  
for Phase II and Phase III studies  
Higher probability of success  
Lower cost/time to approval

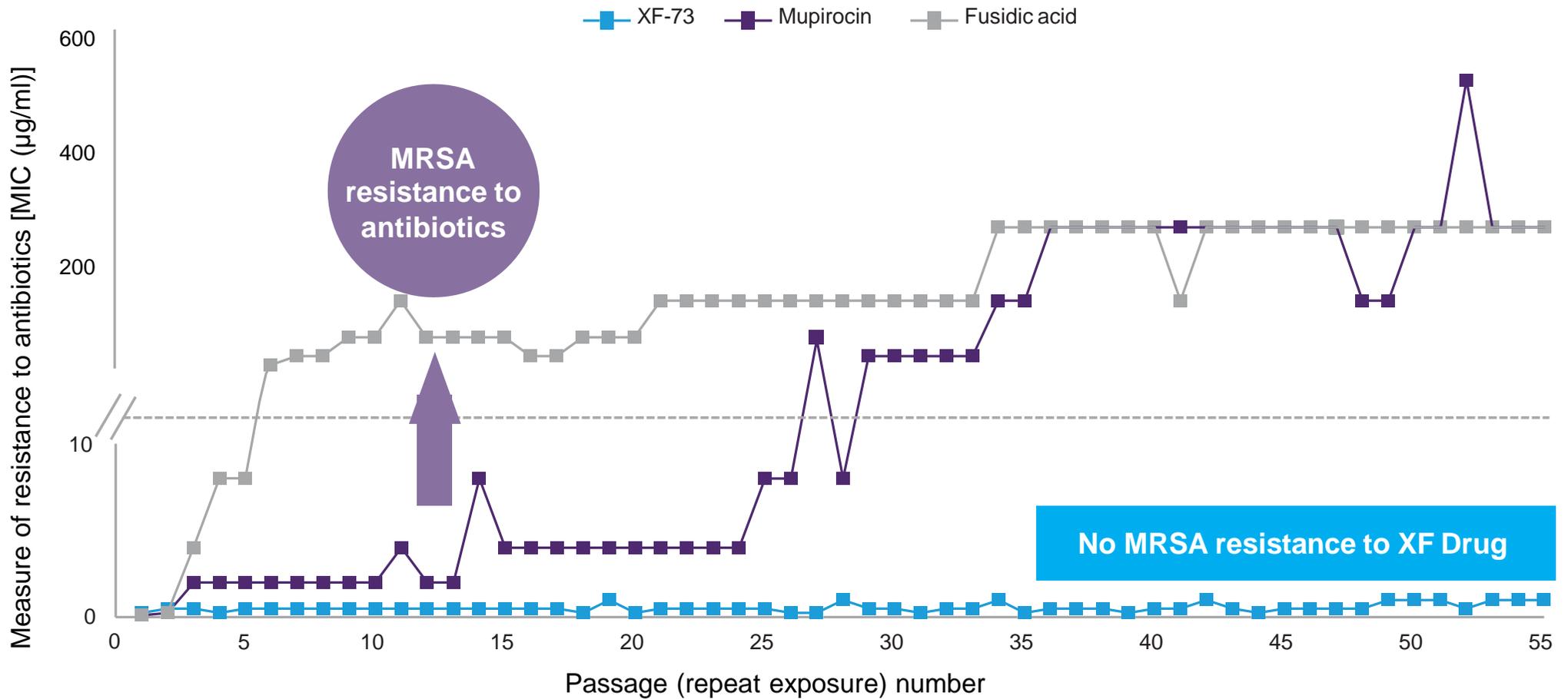
Barrier to new entrants  
who will need to test  
against XF-73

US Cures Act passed December 2016

Directs FDA to allow novel antimicrobial drug approval via a Limited Patient Pathway

# XF-73

No MRSA Resistance Seen, Unlike Antibiotics



Farrell, *et al.*; Investigation of the potential for mutational resistance to XF-73, Retapamulin, Mupirocin, Fusidic acid, Daptomycin and Vancomycin in MRSA isolates during a 55-Passage study. *Antimicrobial Agents & Chemotherapy* (2011); 55; (3) 1177-1181

# XF-73 Clinical Data\* Supports Resistance Profile

	XF-73 Potency (MIC) µg/ml
Nasal <i>S.aureus</i> swabs prior to XF-73	0.25 – 2.0
Nasal <i>S.aureus</i> swabs post XF-73	0.25 – 2.0

No significant change was noted in MIC pre and post XF-73 exposure



NO BACTERIAL RESISTANCE



1st clinical evidence supporting resistance profile

\* Samples from clinical trial XF-73B03, microbiological analysis report Oct 2016.

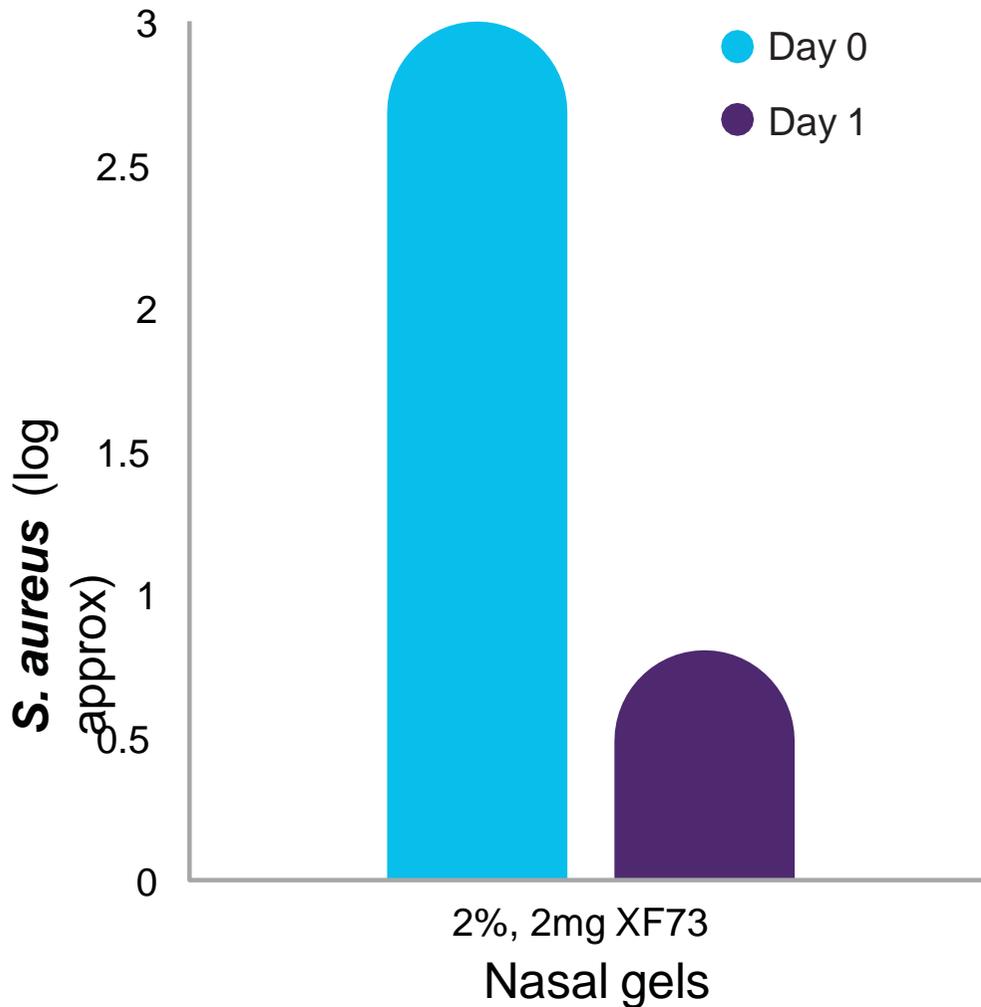
# XF-73 Program Significantly De-risked 5 Clinical Trials Completed



Antibiotic	Sponsor	no. subjects	Design & Results
<b>XF-73A01</b>	Destiny Pharma	23	1st in man, low dose (0.075mg/g), 5 days dosing, <b>safe</b>
<b>XF-73B01</b>	Destiny Pharma	45	Higher dose (0.5mg/g), anti- <i>S. aureus</i> effect, 5 days dosing, <b>dose response, safe</b>
<b>XF-73B02</b>	Destiny Pharma	32	Higher dose (2.0mg/g), <b>enhanced anti-<i>S. aureus</i> effect</b> , 5 days dosing, <b>safe</b>
<b>*XF-73B03</b>	Destiny Pharma	60	2 day dosing, lower viscosity gel, hospital-like procedure, <b>rapid anti-<i>S. aureus</i> nasal effect, safe</b>
<b>*DMID-11-0007</b>	US Government funded	56	5 day dosing, lower viscosity gel, hospital-like procedure, <b>rapid anti-<i>S. aureus</i> nasal effect, safe</b>

Safety & efficacy clinical data support progression to Phase IIb

\* Both studies placebo controlled & XF-73 applied as an intra-nasal gel achieved statistical difference for *S. aureus* reduction



**Safety summary:**

- 216 subjects
- Safe, well tolerated & 100% compliance
- No XF-73 detected in the bloodstream, demonstrates XF-73 targeted to bacteria in nose thereby reducing potential for toxicity in other tissues

**Anti-Staphylococcal efficacy summary:**

- 166 subjects XF-73 nasally dosed
- XF-73 decolonises *S. aureus* rapidly
- Works after 1 day of dosing (2 - 3 doses)
- Effect maintained during treatment

# XF-73 Achieved Rapid & Sustained Clinical *S. aureus* Nasal Reduction

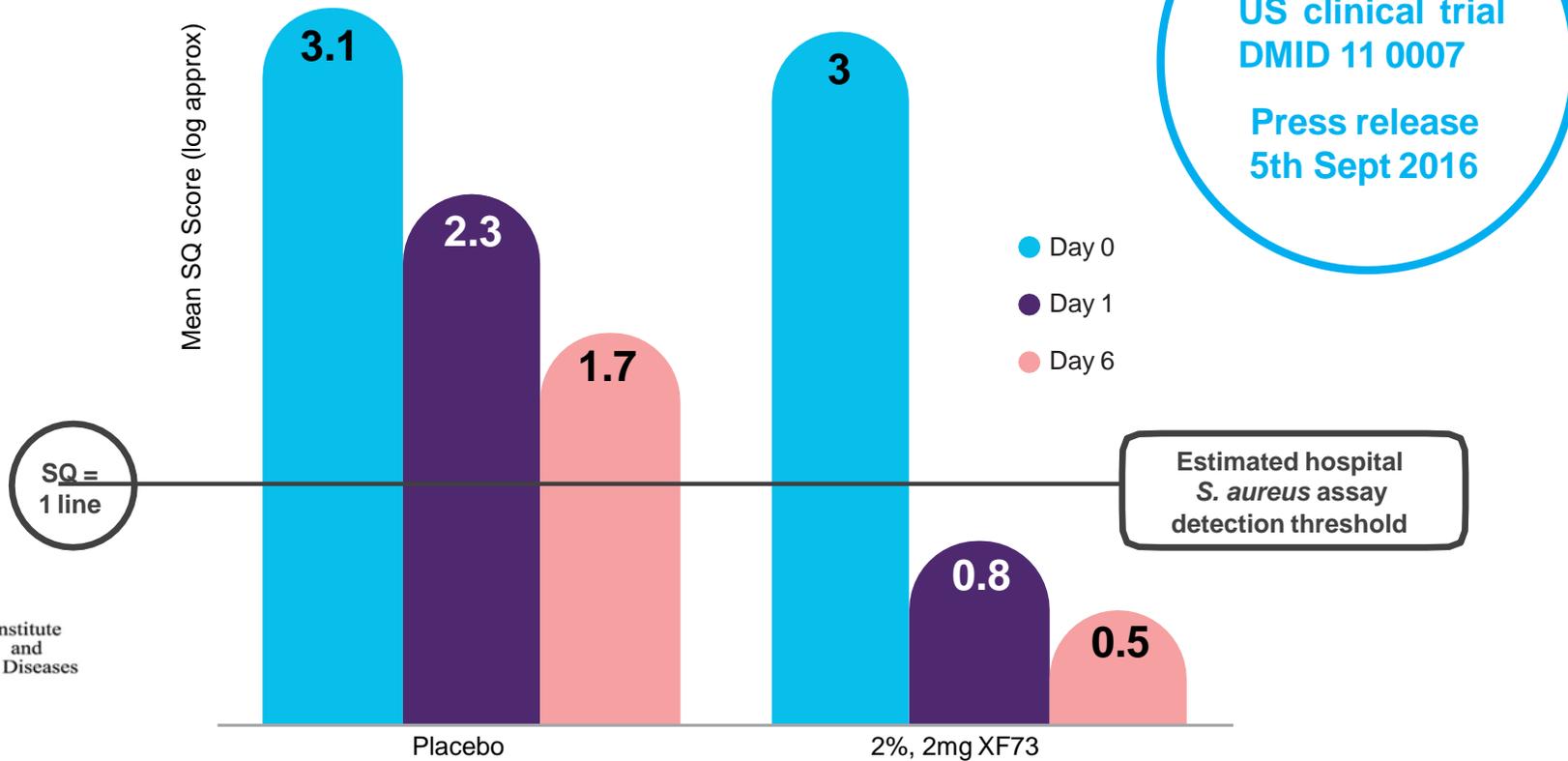
*S. aureus* load after 0, 1 & 5 days dosing



National Institutes of Health



National Institute of Allergy and Infectious Diseases



# National Institutes of Health has Verified the Clinical Antibacterial Efficacy of XF-73

Data from clinical trial DMID 11 0007 (Sept 2016)



56 volunteer study conducted by NIH's National Institute of Allergy and Infectious Diseases

Number of nasal *S. aureus* showed statistical significance from placebo for 2.0mg/g, XF-73 low viscosity gel via area under the curve (AUC)

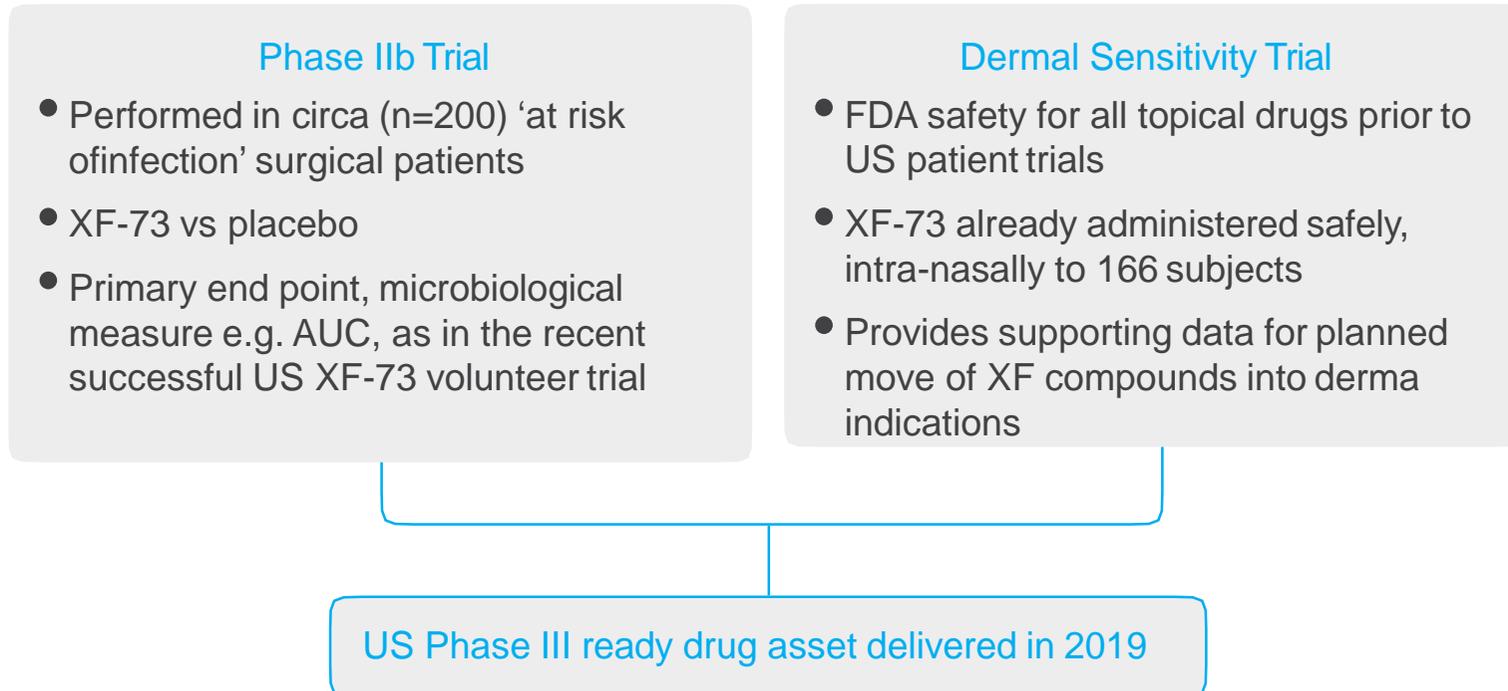
**Even though not powered to do so**

Days 1 to 6

\*p = 0.01

Lead formulation identified 2mg/g: XF-73, lower viscosity, nasal gel

\* Note, statistical significance acceptance limit commonly set at  $p \leq 0.05$



Strategy to continue focused development to approval

Partner at the right time under the right terms

## Collaboration with China Medical Systems (CMS)



- 
- In September 2017, Destiny announced it had entered into a framework development and commercialisation collaboration agreement with CMS
- 
- CMS is a specialty pharma company based in China, focusing on marketing, promotion and sales of prescription drugs and other medicinal products to hospitals nation-wide; 2016 sales of \$740 million
- 
- CMS will get full rights in China and certain other Asian countries, excluding Japan, to enable the development and commercialisation of Destiny's pipeline in the region
- 
- CMS made an additional £3 million equity investment on finalisation of agreement in November 2017. CMS also invested £3m in September IPO
- 
- The parties will coordinate and share data from their respective studies
- 
- Destiny will receive future milestones based on the achievement of sales targets by CMS and make a manufacturing margin on any product Destiny Pharma supplies
-

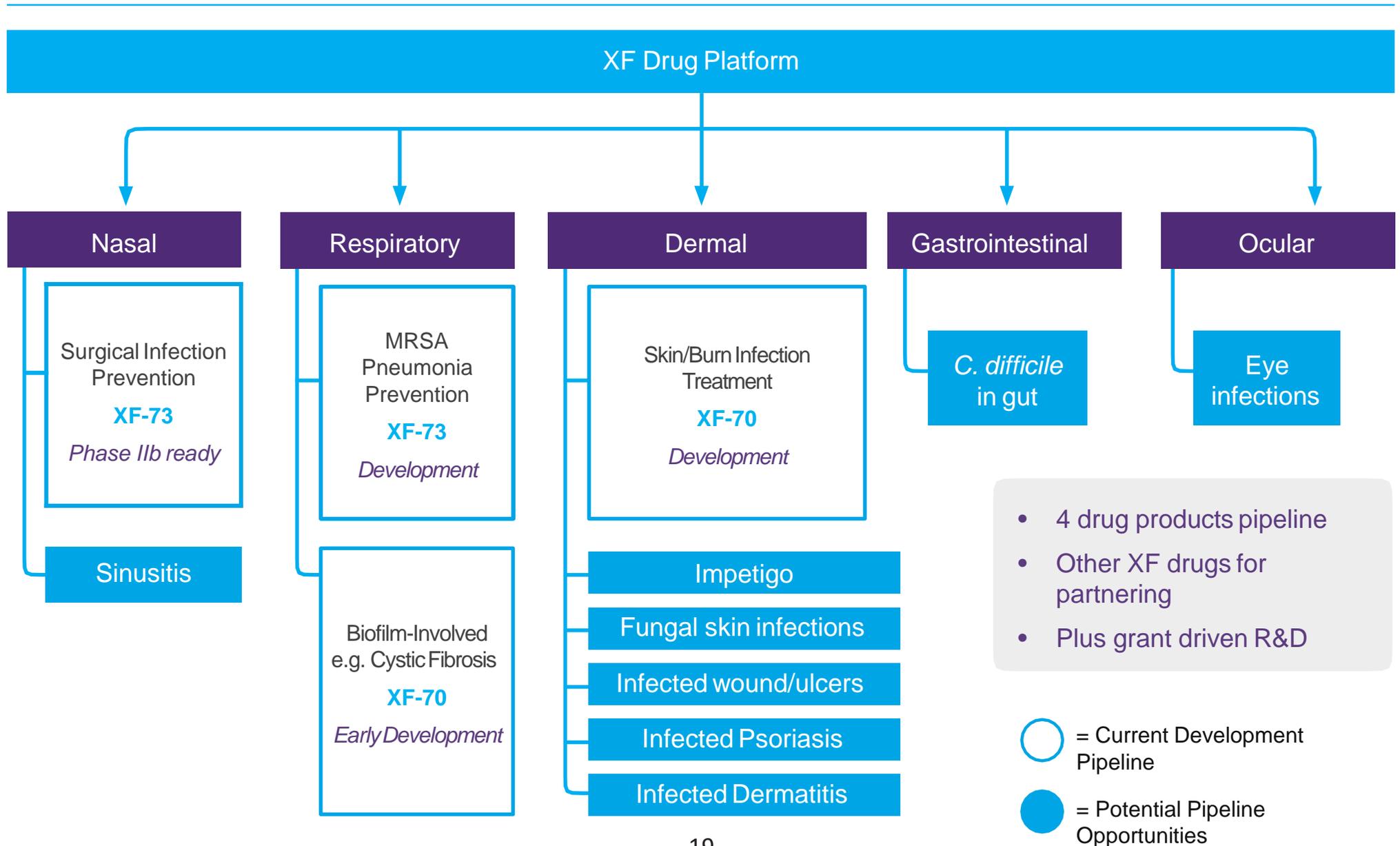
# Market Research\* Highlights Compelling XF-73 Target Product Profile



	Nasal antibiotic (mupirocin)	Nasal XF-73	Competitive Advantage of XF-73
Efficacy vs <i>S. aureus</i>	Majority	All strains	✔ Targeting of the whole <i>S. aureus</i> spectrum compelling
Resistance build up	Yes	No	✔ Removes concern of universal use; acceptable use in wider patient population
Tolerability	Congestion	No congestion	✔ Well tolerated – positive effect on compliance
Dosing prior to surgery	5 days	1 day	✔ Shortens pre-surgery time to treat; reduction in isolation time leading to cost savings
Drug Action	Traditional	Innovative	✔ Promotes interest; potential for superior efficacy and safety
Target Indication- “Prevention of post-surgical <i>S. aureus</i> ”	Unapproved	Approved	✔ Potential for uptake on hospital formulary; widening patient population

Attractive XF-73 profile for Surgeons/Treaters & Payers vs antibiotic  
Potential to save time, costs & lives

# XF-73 Achieved Rapid & Sustained Clinical *S. aureus* Nasal Reduction



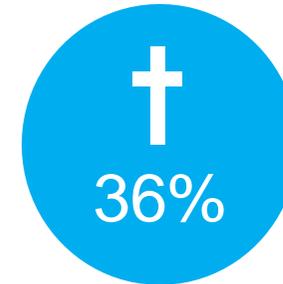
XF-73 indication expansion: Prevention of Ventilator Associated Pneumonia, *S.aureus*/MRSA



Market Size: \$0.5 billion  
market potential



*S. aureus* carriers have 15 X greater  
risk of developing *S. aureus* pneumonia  
(Paling 2017)



In-hospital mortality rate of  
*S. aureus* VAP is up to 36%



Cost of single MRSA  
VAP is >\$40,000



300,000 VAPs in US pa  
which cost up to  
\$1.5 billion per year



Next milestones:

- Production of formulations for throat delivery
- Testing for anti-*S. aureus* efficacy
- Additional safety studies to support clinical testing
- 1st in man clinical study

# Pipeline Products

## Additional XF Drug Programs

XF-70 - treatment for antibiotic resistant bacterial burn/skin wound infections:



\$1 billion existing dermal market



*S. aureus* burn wound model research conducted with the US Department of Defense



XF-70 effective at reducing *S. aureus* burden in wound after single dose

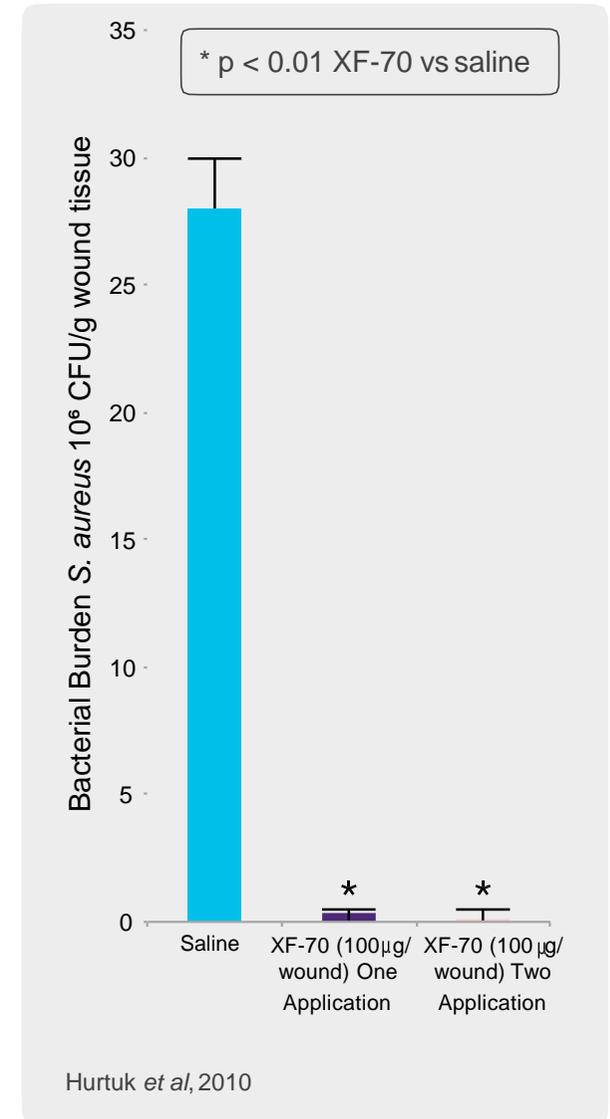


Potential for burn infection product for treatment of *A. baumannii*, *P. aeruginosa* & *S. aureus* (WHO 2017 serious concern Superbug strains)



### Next milestones:

- Synthesis of XF-70 drug batches
- Production of appropriate formulations
- Burn wound testing anti-bacterial efficacy
- Pre-clinical safety data pack
- 1st in man clinical study



# XF Drug Platform Market Exclusivity Potentially Extends into 2030s



US XF-73 QIDP status gives 5 years extra US market cover

- 3 patent families based on
  - Composition of Matter
  - Novel Mechanism of Action
  - Biofilm Action
- Portfolio of 93 granted & 4 pending patents in 3 patent families
- All major commercial territories covered

Potential 6 month paediatric extension

# Beyond Antibiotics

## Novel Drugs to Combat Bacterial Infection & Resistance



Well funded, clinical stage biotech, lead asset targets antibiotic-resistant bacterial infections in hospitals



Lead drug asset, XF-73, has multiple drivers for adoption in an area of global unmet medical need, with a potentially expedited route to regulatory approval



XF-73 significantly de-risked, 5 successful clinical trials completed, no evidence of resistance. Next major clinical trial is Phase IIb



Proprietary, anti-microbial drug platform – “XF Drugs”



Market exclusivity, including robust IP protection that potentially extends into 2030s



Clearly defined value creation opportunity, significant addressable markets, including blockbuster potential for XF-73's lead indication



# Appendix

# XF-73 Active Against AR bacteria: a serious concern for WHO

The image is a collage. On the left, a hand holds a smartphone. In the center, a document from the World Health Organization (WHO) is displayed, titled 'WHO priority pathogens list for R&D of new antibiotics'. The document lists 18 bacteria categorized into three priority levels: Critical, High, and Medium. Green checkmarks indicate that XF-73 is active against several of these bacteria, while a red X indicates it is not active against one. Surrounding the WHO document are several news articles from various sources, including The New York Times, BBC News, and The Telegraph, all reporting on the WHO's list of priority pathogens and the threat of superbugs.

**World Health Organization**

**WHO priority pathogens list for R&D of new antibiotics**

**Priority 1: CRITICAL**

1. *Acinetobacter baumannii*, carbapenem-resistant ✓
2. *Pseudomonas aeruginosa*, carbapenem-resistant ✓
3. *Enterobacteriaceae*, carbapenem-resistant, ESBL-producing (Not tested)

**Priority 2: HIGH**

1. *Enterococcus faecium*, vancomycin-resistant ✓
2. *Staphylococcus aureus*, methicillin-resistant, intermediate and resistant ✓
3. *Helicobacter pylori*, clarithromycin-resistant (Not tested) ✓
4. *Campylobacter* spp., fluoroquinolone-resistant ✓
5. *Salmonellae*, fluoroquinolone-resistant ✗
6. *Neisseria gonorrhoeae*, cephalosporin-resistant, fluoroquinolone-resistant (Not tested)

**Priority 3: MEDIUM**

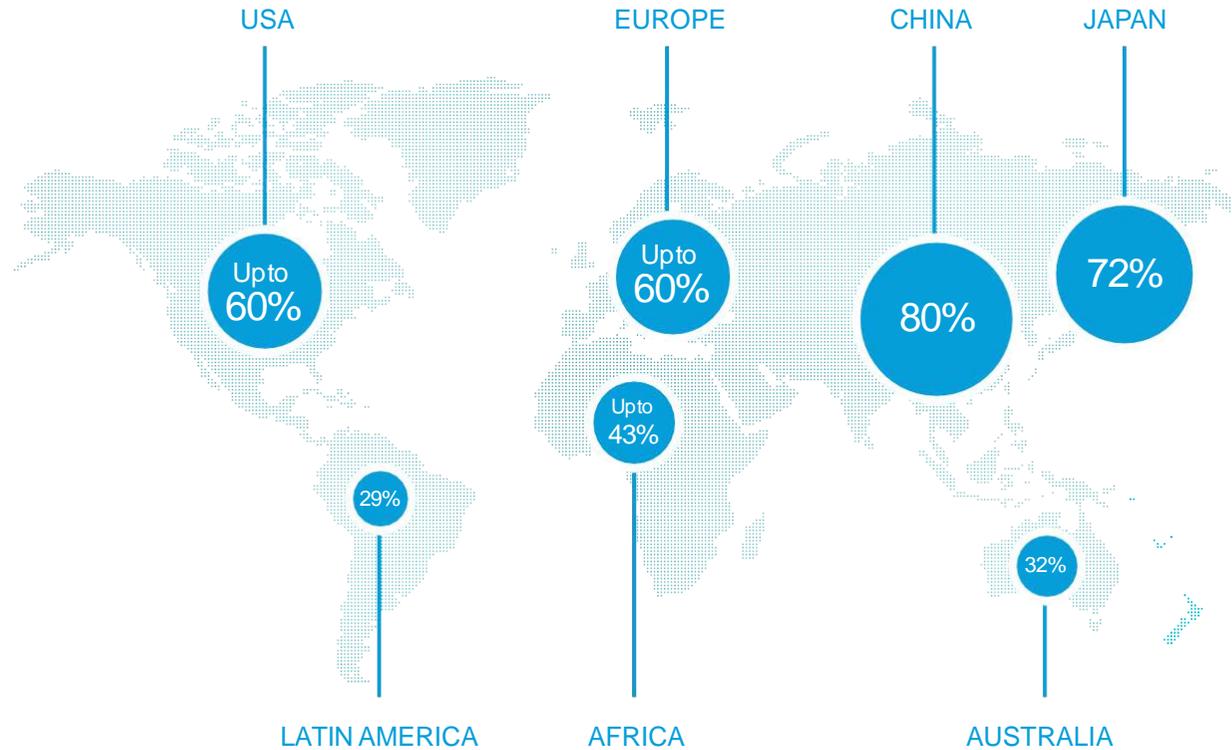
1. *Streptococcus pneumoniae*, penicillin-non-susceptible ✓
2. *Haemophilus influenzae*, ampicillin-resistant ✓
3. *Shigella* spp., fluoroquinolone-resistant ✓ (XF-70)

**News Articles:**

- The New York Times:** 'Deadly, Drug-Resistant 'Superbugs' Pose Huge Threat, W.H.O. Says'
- BBC News:** 'World's most threatening superbugs ranked in new list'
- The Telegraph Science:** 'Superbugs which pose greatest threat to humanity identified by World Health Organisation'
- WHO Website:** 'WHO raises alarm over drug-resistant superbugs'

# Staphylococcus aureus (SA)

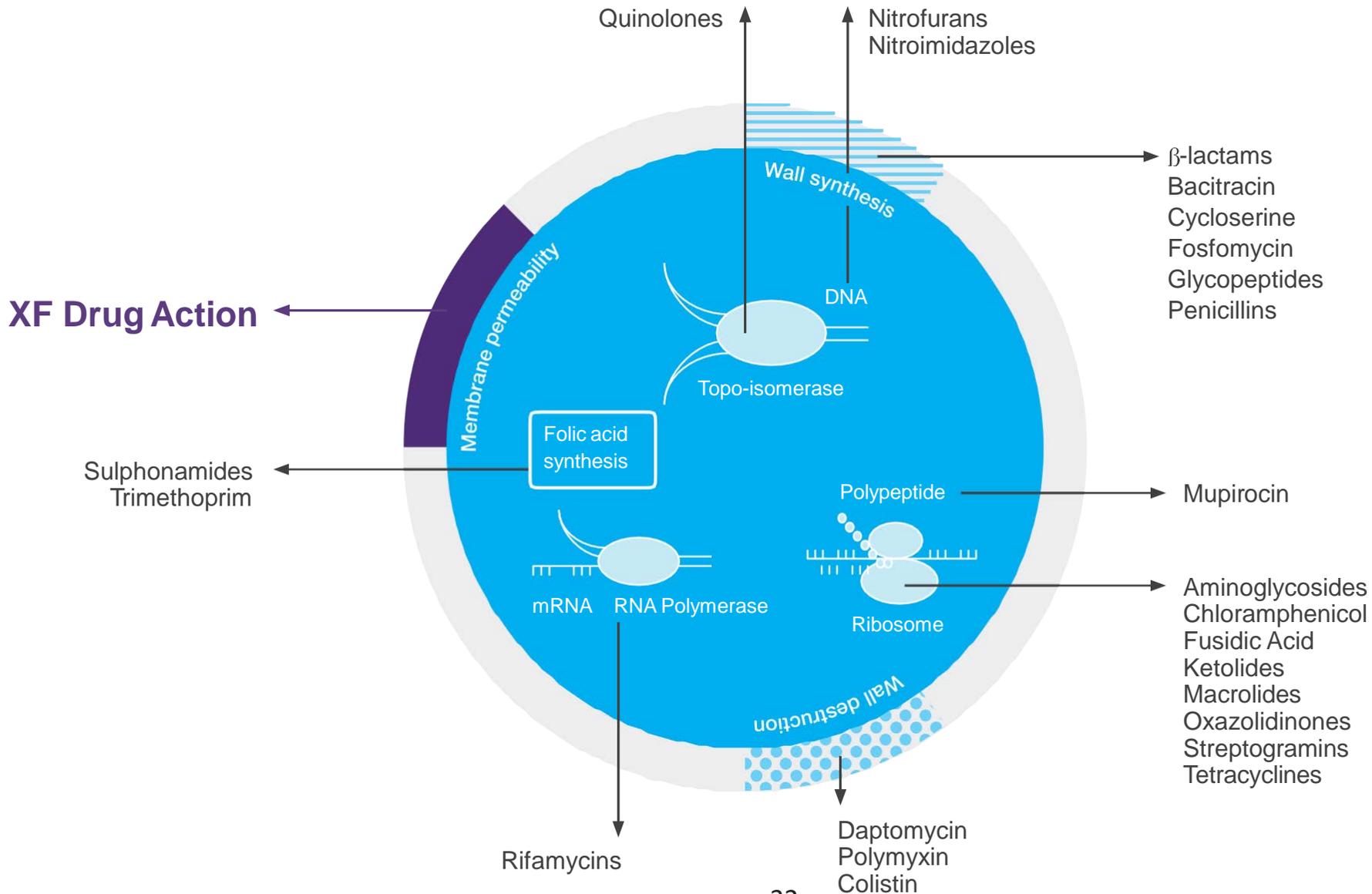
## Major Cause of Hospital Infection



- SA hospital infections cost US economy alone up to \$10 billion p.a.
- Significant percentage of SA is antibiotic resistant i.e. MRSA
- 40 million surgical patients p.a. in USA 'at risk' of SA infection

**XF-73 aims to address this global unmet medical need**

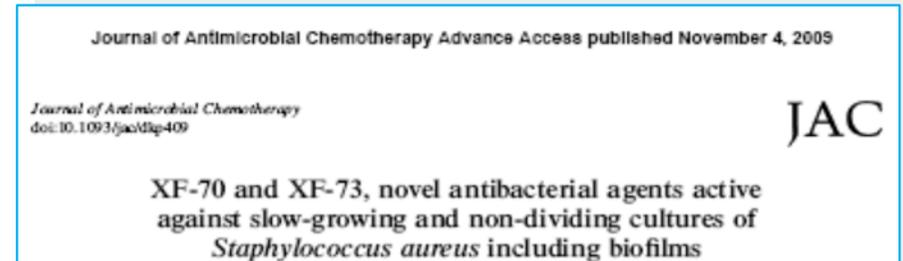
# Mechanisms Of Antibiotic/Antibacterial Drug Action



# XF Drug Unique Mechanism Of Action: Published

## XF-73 active against the bacterial cell membrane

- Intrinsic antibacterial action
- Rapid reduction in Membrane potential
- Loss intracellular ATP & K<sup>+</sup>
- Not accompanied by cell lysis



ndal<sup>1</sup>, William Rhys-Williams<sup>2</sup>, William Love<sup>2</sup>  
n Chopra<sup>1\*</sup>

<sup>1</sup>Molecular and Cellular Biology, University of Leeds,  
Science Park Square, Falmer, Brighton BN1 9SB, UK

009; revised 13 October 2009; accepted 14 October 2009

eria exhibit tolerance to many antibiotics. However,  
a in all growth phases. We sought to examine whether  
d XF-73, which have rapid membrane-perturbing activity



**ABSTRACT**  
The antibacterial activity of XF-73, a dicationic porphyrin drug, was investigated against a range of Gram-positive and Gram-negative bacteria with known antibiotic resistance profiles, including resistance to cell wall synthesis, protein synthesis, and DNA and RNA synthesis inhibitors as well as cell membrane-active antibiotics. Antibiotic-sensitive strains for each of the bacterial species tested were also included for comparison purposes. XF-73 was active [minimum inhibitory concentration (MIC) 0.25–6 mg/L] against all of the Gram-positive bacteria tested, irrespective of the antibiotic resistance profile of the isolates, suggesting that the mechanism of action of XF-73 is unique compared with the major antibiotic classes. Gram-negative activity was lower (MIC 1 mg/L to >64 mg/L). Minimum bactericidal concentrations data



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