Global Antibiotic Research and Development Partnership (GARDP): A model for public-health driven R&D and access

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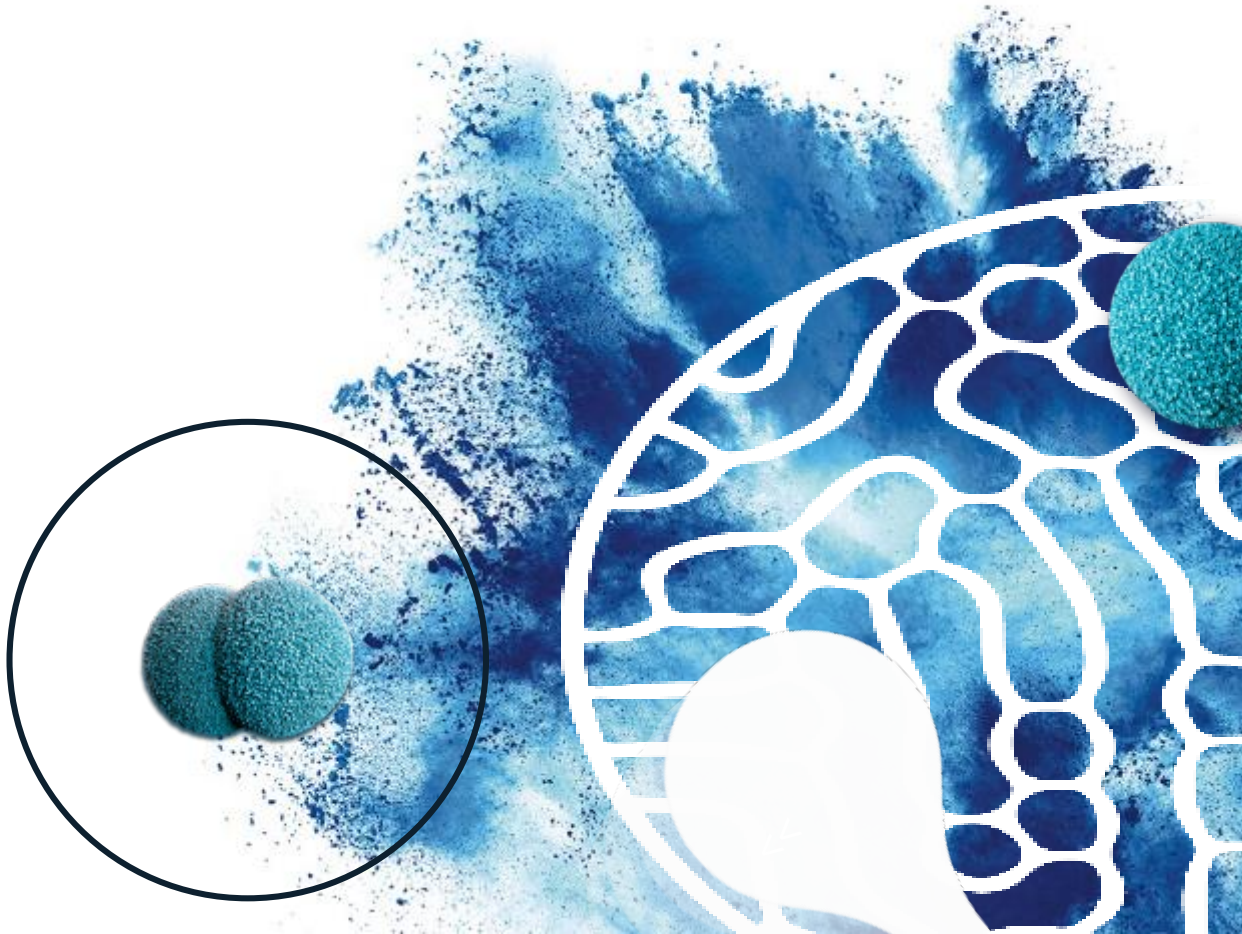


GARDP

Our vision:

All infections are treatable for everyone, everywhere

GARDP

Our mission

GARDP brings together the public and private sectors to develop new treatments for bacterial infections. We ensure responsible and sustainable access, addressing the public health impact of antibiotic resistance.

Our focus

Bacteria on the WHO priority pathogen list
Diseases and populations disproportionally affected by drug resistance
Late-stage clinical development and access

Our approach

INVEST IN LIVES IMPROVED & SAVED

GARDP is developing new and improved treatments for priority drug-resistant infections that cause hundreds of thousands of deaths every year.

INNOVATIVE PARTNERSHIPS

In four years, GARDP has formed more than 50 partnerships in 20 countries, built a solid base of knowledge and expertise, and created research programmes to deliver new treatments.

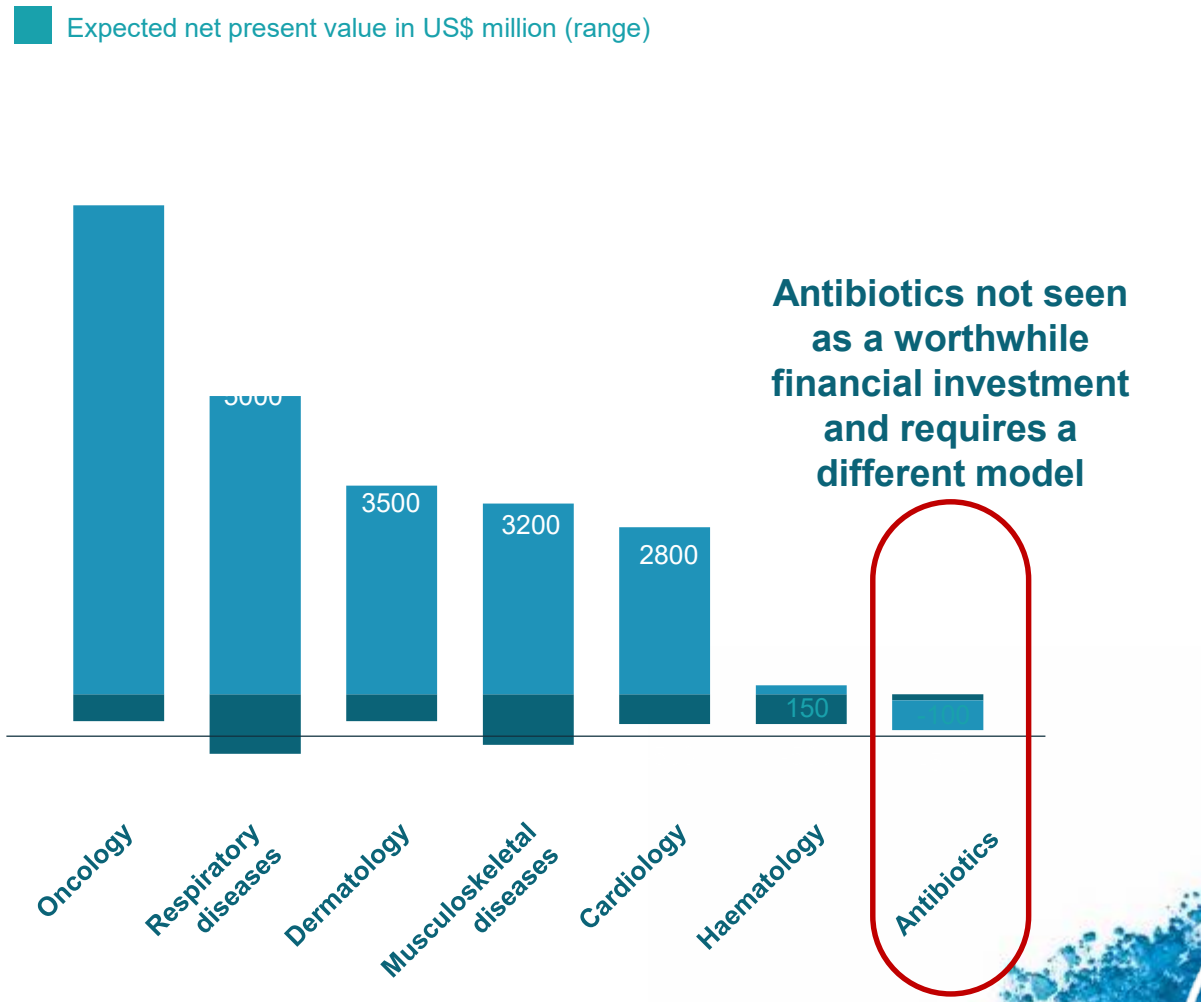
DELIVERING IMPACT

Through collaborations, GARDP has evaluated 100 substances for antibacterial activity, resulting in six drug candidates for infections that pose the greatest threat to health.

Why is there a crisis?

- The R&D is scientifically challenging as novelty in new drugs is relatively rare
- A blockbuster model is not sustainable for antibiotic R&D
- Antibiotic R&D is not an attractive investment; many in the private sector have difficulties raising capital and have left the field

Antibiotics: a high investment for public health but with no market return



Source: BCG for the German Federal Ministry of Health, ISDA, FDA
Nature drug Discovery: https://www.nature.com/articles/d41573-019-00130-8
PEW

Value of a not-for-profit model

The issue	Area of intervention	Desired outcome	Why a not-for-profit?
Meeting global public health needs	Objective and long-term public health portfolio approach Targeted PPPs, ensuring return on public investment	Ensuring new treatments are available long term	Can take a long term tailored, approach, not tied to any product
Best use of public money	Explore and invest in high-risk projects	Deliver 'most needed' treatments for Public Health	Less risk averse, do research in nontraditional setting
Ensuring availability and appropriate use	Obtain key indication approval, generate more real-world data	Good stewardship of new antibiotics, enhance portfolio	Can invest in areas of low commercial, high public health return
Ensuring equity, meeting specific challenges	R&D in populations with high burden & need, e.g. neonates	Addressing underserved areas	Address areas seen as too challenging by others
Addressing emerging threats	Conduct R&D in different, challenging geographies (LMICs) Expand LMIC access : licensing, registration, guidelines, support stewardship, supply	Accelerate access in high burden areas, enhance appropriate use	Can take a local and multilateral approach
Infrastructure for global R&D projects	Set up and support global networks and collaborations	Set up trial networks including high burden LMICs, strengthen research capacity	Working long term with regional partners in LMICs

GARDP's commitment 2020-2025

What will GARDP develop treatments for?

- Serious bacterial infections in hospitalized people
- Drug-resistant infections in children
- Neonatal sepsis in newborns
- Sexually transmitted infections

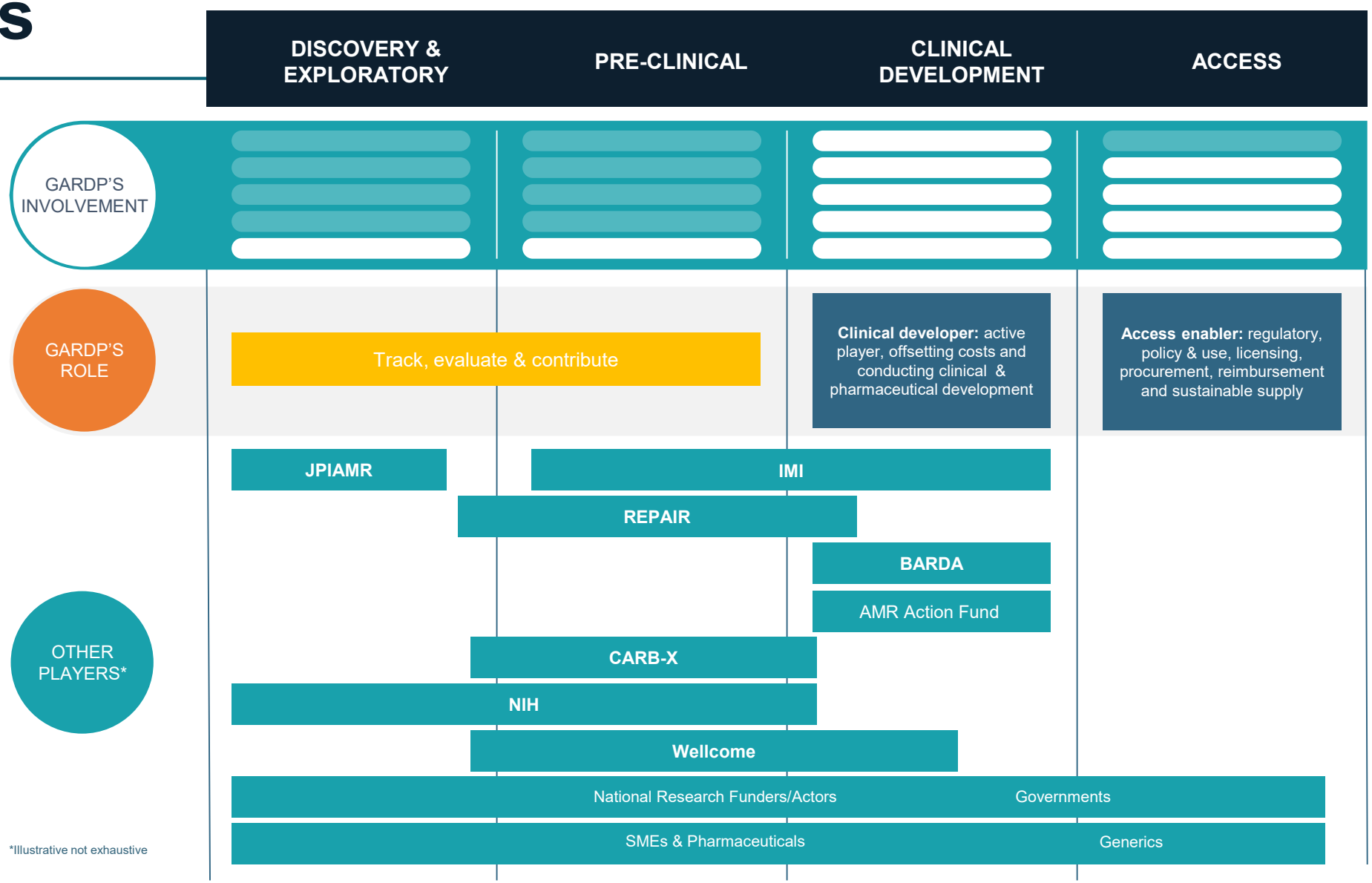
WHO priority pathogen list for which there is a critical and high need for new antibiotics.

How will GARDP accomplish its ambition?

- Focus on developing new and improved treatments in late-stage clinical development and ensuring responsible and sustainable access.

€500 million to accelerate development and delivery of **5 new treatments** that address **urgent public health needs**

GARDP is filling critical gaps



*Illustrative not exhaustive

Overview of achievements to date

Serious bacterial infections	• Partnered with **Venatorx Pharmaceuticals** to develop **a new drug candidate, cefepime-taniborbactam,** for serious bacterial infections in hospitalized adults, including those for which there are limited treatment options • Global complicated UTI study recommenced recruitment (53% of target); synopsis complete and feasibility assessment commenced for observational study on the management of carbapenem resistant infections in India and South Africa
Children's antibiotics	• Completed one of **the largest global studies on newborns** with **sepsis (**over **3,000** babies enrolled). Results will inform empiric trial that will evaluate combination treatments for neonatal sepsis (a leading cause of death among babies). • Completed a **safety evaluation of an existing antibiotic (fosfomycin)** for use in babies to treat drug-resistant infections, including neonatal sepsis • The **Paediatric Investigation Plan for Polymyxin B** with GARDP as sponsor was agreed by the EMA • GARDP and **Venatorx Pharmaceuticals agreed a paediatric programme for cefepime-taniborbactam with the EMA and FDA**
Sexually transmitted infections	• Partnered with **Entasis Therapeutics** to develop a **first-in-class treatment for gonorrhoea** • **Global phase III trial recruiting patients in most sites in US, the NL, after a Covid-19 pause, & South Africa, with activation pending in Thailand** • **Drug product manufacturing development activities progressed with registration batches ready to start in Q1 2021**
Discovery & exploratory research	• Screened **>65'000 compounds** and **evaluated more than 100 new and 'recovered' chemical entities** as part of our discovery and exploratory research programme to boost antibiotic R&D • Set up AMR discovery screening consortium and completed screening of compound libraries from 3 Japanese pharma companies
External scientific affairs & REVIVE	• **Launched REVIVE**, an online knowledge sharing platform on antimicrobial R&D and secured attendance of over **5,700 participants at 32 webinars** led by experts in the field • Expanded content including R&D sessions at conferences (bootcamps) and expert "viewpoints" on REVIVE website • Launched Antimicrobial R&D Encyclopaedia

What do we mean by drugs, treatments & sustainable access?

GARDP will develop five new treatments for bacterial infections which may be new, old or a combination of antibiotics by 2025.

5BY25

Antibiotic
Is a substance that either kills bacteria or stops them from growing.

Treatment
Is a formulation and/or regimens of either a single or combination of (antibiotic) drugs that is used to treat infections.

Sustainable access
Refers to treatments which are of required quality (i.e. relating to active compounds and manufacturing standards); affordable for patients and/or health systems; supplied in a timely and appropriate manner; and having the required stewardship in place to ensure they are used in a responsible manner (avoiding over-prescription and unnecessary use).

GARDP access framework for public health impact

Licensing	Regulatory	Public Health Policy & Use	Outsourcing Strategies	Procurement	Reimbursement Models
In & out-licensing supporting: ► Quality manufacturing ► Early access across high-burden countries ► Appropriate marketing	Collaboration with WHO & national regulators for: • Public health evidence (need and use) • Global registration • Label extension	• Early access programs • Guidelines to ensure appropriate access • Surveillance for resistance • Diagnostics for stewardship	• Cost of goods focus • Defining best practice in manufacturing • Maintaining a core network of partners	• Better understanding national needs in key high-burden countries • Facilitating cost-saving procurement mechanisms to support demand	• Non-volume based sustainable reimbursement models

GARDP's intensity of engagement will vary for each of these interventions and will include:

Advocating | Facilitating | Implementing

SECURE concept

SECURE: a collaborative initiative that aims to accelerate access to a portfolio of antibiotics to assist countries in addressing the silent pandemic of drug-resistant bacterial infections

PORTFOLIO: Focus on Reserve antibiotics and those antibiotics subject to frequent supply interruptions

- Driven by public health and clinical needs of participating countries

FOCUS: Antibiotic security, a key component of pandemic preparedness to prevent future health outbreaks

How will it work?

- Create **a dynamic portfolio of affordable essential antibiotic treatments** to manage drug-resistant infections
- Provide countries with **rapid access to portfolio**
- Countries that opt in to SECURE will be able to procure **a regular supply of quality antibiotics**, through market-shaping interventions
- Foster **optimal use of these antibiotics** by generating real-word data to support country adoption

Thank you

World Health Organization

GARDP

DNDi Drugs for Neglected Diseases Initiative

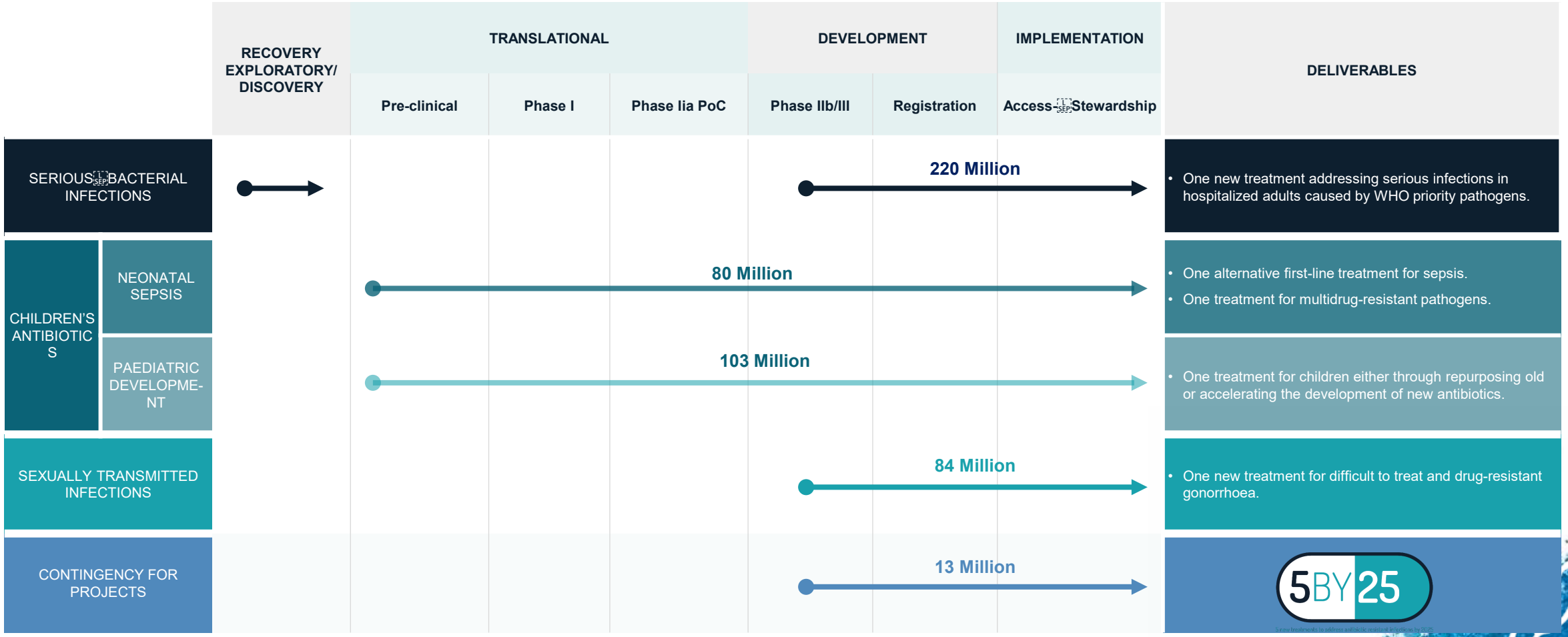
www.gardp.org

GARDP's pipeline *(April 2020)*

PROGRAMMES	DISCOVERY	TRANSLATIONAL			DEVELOPMENT		IMPLEMENTATION
		Pre-clinical	Phase 1	Phase 2a PoC	Phase 2b/3	Registration	Access-Stewardship
SERIOUS BACTERIAL INFECTIONS	HIPS library screening				Cefepime-taniborbactam (NCE) Venatorx Pharmaceuticals1		
	CALIBR ReFRAME library screening						
	Japanese Consortium library screening: Eisai, Takeda & Daiichi Sankyo						
CHILDREN'S ANTIBIOTICS – NEONATAL SEPSIS		Fosfomycin			Treatment regimen neonatal sepsis	Approved in EU, with Neo & Paeds label	
		Flomoxef				Approved in Asia: Neo & Paeds data	
		Amikacin				Approved globally: Neo & Paeds data	
CHILDREN'S ANTIBIOTICS – PAEDIATRIC DEVELOPMENT			Polymyxin B		Treatment options serious bacterial infections paediatric and neonates	Approved in USA, with limited Neo & Paeds label	
			Paediatric cefepime-taniborbactam (NCE) Venatorx Pharmaceuticals				
				Potential candidate A (NCE or repurposed			
SEXUALLY TRANSMITTED INFECTIONS					Zoliflodacin (NCE) Entasis Therapeutics Inc.		Access strategy Including manufacturing development
		Potential candidate B (NCE)				Approved in USA for CABP	

1 Development of cefepime-taniborbactam is sponsored by Venatorx Pharmaceuticals and has been funded in whole or in part with federal funds from NIAID/NIH/HHS and BARDA/ASPR/HHS in the United States and The Wellcome Trust in the United Kingdom

Estimated costs of GARDP portfolio – EUR 500M



It is currently estimated that 25 to 50 percent of the new projects, those assigned as serious bacterial infections and those not assigned yet, will be funded via cost sharing agreements. This is reflected in the above figures, but it does mean the costs could change significantly.