

**BEST SCIENCE**  
FOR THE MOST NEGLECTED

**DNDi**

Drugs for Neglected Diseases *initiative*

## GHIT PDP Webinar

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# Born on the frontlines of medical action



**DNDi was created in response to the frustration of clinicians and the desperation of patients faced with medicines that were ineffective, unsafe, unavailable, unaffordable, or that had never been developed at all.**

## **The root of the problem?**

The prevailing profit-oriented model for medical research and development (R&D) leaves little incentive to develop drugs for the poorest and most vulnerable communities.

**URGENT R&D NEEDS REMAIN UNADDRESSED**

# For neglected diseases, a fatal imbalance remains

1975- 1999

**1.1%** of the 1,393 new drugs  
were for neglected diseases  
that represent  
**12%** of the global disease burden

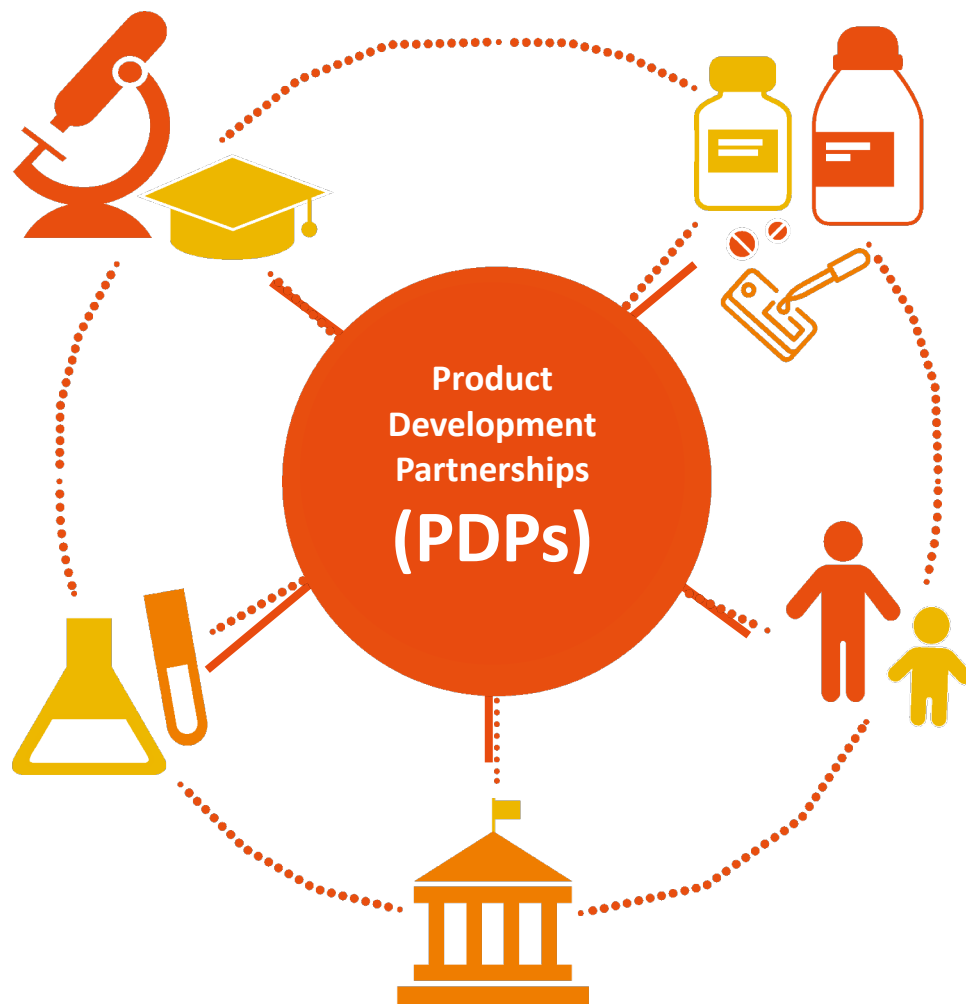


SOURCE: MSF & the DND Working Group, 2001. *Fatal Imbalance: The Crisis in R&D for Neglected Diseases*. Médecins Sans Frontières.

# The “virtual orchestra”: a collaborative partnership model of R&D

## ACADEMIA

We work together with universities, research institutes and national research centres all around the world



## TREATMENT AND DIAGNOSTIC PROVIDERS

We partner with treatment and diagnostic providers to ensure R&D responds to needs in the field, and to encourage rapid deployment of the new medical tools developed

## PHARMACEUTICAL INDUSTRY

Close collaboration with pharmaceutical companies, ranging from generics and biotechs to “Big Pharma” on projects spanning the whole drug R&D cycle from discovery to access and delivery

## PATIENTS AND COMMUNITIES

Patient and community participation is key, in our projects we closely work with community stakeholders

## HEALTH MINISTRIES

Important partnerships involving definition of needs, co-sponsorship of clinical studies, and working together to facilitate programme implementation



# 15 YEARS, 9 TREATMENTS, MILLIONS OF LIVES SAVED

We discover, develop, and accelerate access to urgently needed treatments for neglected patients, focusing on gaps for neglected tropical diseases and viral diseases that fuel cycles of poverty and disease in resource-constrained settings

## Treatments delivered



field-adapted and affordable treatments for 6 deadly diseases

## R&D pipeline replenished



- 20+ NCEs
- 4 million+ compounds screened
- 13 projects in Phase III and registration

A healthy pipeline of drug candidates for 8 deadly diseases

## Clinical trials conducted



An average of 20 active clinical studies per year

## Research networks established

- 5 clinical research networks
- 4 lead optimization consortia
- **800+ members** COVID-19 Clinical Research Coalition

## Global partnerships forged



200+ partner institutions in 40+ countries

Mobilizing any given year 1000+ partner FTEs **(65% in Africa)**

## Diverse global team mobilized



A diverse global team of 250+ staff

37 nationalities across 4 continents

A unique footprint in LMICS

## Policies influenced

DNDi's model, experience, and lessons learned documented and disseminated

## New organization to fight drug-resistant infections

DNDi joined forces with the World Health Organization (WHO) to create the **Global Antibiotic R&D Partnership (GARDP)**

## WHAT DRIVES THE NEGLECT?

# Our work is more urgent than ever



### Profit-driven R&D leads to:

- **Neglect to invest in unprofitable or challenging segments**, such as diseases of poverty including neglected tropical diseases, paediatric formulations, and gender-responsive R&D
- **Neglect to ensure timely, affordable, and equitable access** worldwide, including in resource-limited settings

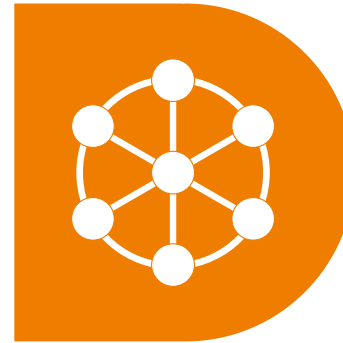
### Emerging trends will affect the most vulnerable people and the least resourced settings most acutely:

- **Pandemic-prone** diseases
- **Climate-sensitive** infectious diseases



### We innovate to save lives

**We discover and develop urgently needed treatments for neglected patients**, and work to ensure they are affordable, available, and adapted to the communities who need them



### We foster inclusive & sustainable solutions

**We work hand-in-hand with partners in low- and middle-income countries** to power our progress and strengthen innovation ecosystems that put people's needs first



### We advocate for change

**We speak out for policy change** to enable more effective and equitable R&D and access to the fruits of science for all people, no matter their income or where they live

# DNDi – Innovating to save lives

**We discover, develop, and accelerate access to urgently needed treatments for neglected patients, focusing on gaps for neglected tropical diseases and viral diseases that fuel cycles of poverty and disease in resource-constrained settings**



## SLEEPING SICKNESS

Accelerate sustainable disease elimination



## LEISHMANIASIS

Deliver safer, simpler treatments to save lives and reduce social stigma



## CHAGAS DISEASE

Contribute to eliminating Chagas as a public health problem



## FILARIA (RIVER BLINDNESS)

Advance progress toward breaking the cycle of transmission



## MYCETOMA

Prevent devastating amputation and disability



## HIV

Ensure access to lifesaving treatment for children and people with advanced HIV



## HEPATITIS

Help make treatment a reality for millions of people waiting for a cure



## COVID-19/ PANDEMIC PRONE DISEASES

Speed tools to save lives, especially in resource-limited settings



## NEW AREAS TO BE

EXPLORED: Dengue, schistosomiasis, snakebite



# Role of DNDi Japan office

## Japan Office activity

	DISCOVERY			TRANSLATION			DEVELOPMENT		IMPLEMENTATION
	Screen	Hit-to-lead	Lead optimization	Pre-clinical	Phase I	Phase IIa/ Proof-of-concept	Phase IIb/III	Registration	Treatment access
SLEEPING SICKNESS			SCYX-1330682 + SCYX-1608210				Acoziborole + Fexinidazole for <i>T.b. rhodesiense</i> +		Fexinidazole for <i>T.b.gambiense</i> * + Nifurtimox-eflornithine combination therapy (NECT)*
LEISHMANIASIS	Screening	Leishmaniasis Hit-to-lead	CF series +	DNDI-6174 +	DNDI-6148 +		New CL combination	New VL treatments (Latin America)	SSG&PM (East Africa)*
		NTD Drug Discovery Booster Hit-to-lead	S07 series +	GSK3494245 + DDD1305143 +	DNDI-0690 + GSK899 DDD853651 +		New treatments for PKDL	New treatments for HIV/VL	New VL treatments (South Asia)*
			DNDI-5421 + DNDI-5610 +		Novartis LXE408 + CpG-D35 for CL + GSK245 DDD130514 +		Miltefosine + paromomycin combination (Africa)		
			Amino pyrazoles +						
CHAGAS DISEASE	Screening	Chagas disease Hit-to-lead	Oxaborole profiling	Biomarkers		Fexinidazole +	New benznidazole regimens +		Benznidazole paediatric dosage forms*
		NTD Drug Discovery Booster Hit-to-lead	UW series +						
			Daiichi Sankyo series +						
FILARIA: RIVER BLINDNESS				Macrofilaricide CC6166 +	Oxfendazole +	Emodepside + TylAMac (ABBV-4083) +			
MYCETOMA							Fosravuconazole +		
HIV					5 FC (cryptococcal meningitis)			4-in-1 (ABC/3TC/LPV/r)	Super-booster therapy for children with HIV/TB* 2-in-1 LPV/r pellets and ABC/3TC or AZT/3TC
HEPATITIS C							Ravidasvir + sofosbuvir +	Ravidasvir +	
COVID 19		Hit-to-lead		Pre-Clinical			ANTICOV		
MALARIA ★									Fixed-dose combination ASMQ*



NCE (New chemical entity)



\* Treatments delivered by DNDi

## IMPACT OF GHIT ON DNDI

Summary of DNDi Japan projects (mostly **GHIT funded!**)

Start	Partner	Stage	Disease
2021	Mitsubishi Tanabe	HTL	Leish/Chagas
	Takeda	LO	Leish (VL)
	Eisai	PreC	Leish
	U Tokyo	Scr	Leish (VL)
2020	U Tokyo, GeneDesign	Clin	Leish (CL)
	Daiichi Sankyo	LO	Chagas
		Scr	AMR
	Daiichi Sankyo Novare	Scr	Leish/Chagas
2019	Mitsubishi Tanabe	Scr	Leish/Chagas
2018	Eisai Takeda	Scr	AMR
	Takeda	PreC	Leish (VL)
	Eisai Shionogi Takeda Astellas	HTL	Leish/Chagas (Booster III)
	Daiichi Sankyo Novare	Scr	Leish/Chagas
2017	Eisai	Clin	Mycetoma
	GeneDesign	PreC	Leish (CL)
	Eisai Shionogi Takeda	HTL	Leish/Chagas (Booster II)
	Daiichi Sankyo	HTL	Leish/Chagas
	Riken	TRP	Leish/Chagas

Start	Partner	Stage	Disease
2016	GeneDesign	PreC	Leish (CL)
	Daiichi Sankyo Novare	Scr	Leish/Chagas
	Daiichi Sankyo	Scr	Leish/Chagas
	Eisai	Scr	Leish/Chagas
2015	Eisai	Clin	Mycetoma
	Takeda	LO	Leish (VL)
	Eisai, Shionogi, Takeda	HTL	Leish/Chagas (Booster I)
2014	Osaka U, Gene Design, Nagasaki U	PreC	Leish (CL)
	Eisai	Clin	Chagas
2013	Eisai	Scr	Leish/Chagas
	Takeda	Scr	Leish/Chagas
	Kitasato U	Scr	Leish/Chagas
	IMC	Scr	Leish/Chagas
2012	GeneDesign	TRP	Leish/Chagas
	U Tokyo, JICA	CapB	Leish (VL) (Bangladesh)
2009	Eisai	Clin	Chagas
2006	Kitasato U	Scr, CapB	HAT, Leish
2005	Kitasato U	Scr	HAT