

# Responding to Neglected Patients' Needs Through Innovation

Spring Gombe-Götz

Policy Advocacy Manager

8 November, 2018

Federal Parliament Belgium

*Best Science for the Most  
Neglected*

**DNDi**

Drugs for Neglected Diseases *initiative*

HDTV  
by evertz

# 15 years ago: the creation of DNDi

**1999**

**Nobel Peace Prize** awarded to MSF, who commits the prize money to the Drugs for Neglected Diseases Working Group

**2003**

Initial meeting in **Nairobi**

**DNDi creation with founding partners:**

- Kenya Medical Research Institute, Kenya
- Institut Pasteur, France
- Indian Council of Medical Research, India
- Médecins Sans Frontières
- Ministry of Health, Malaysia
- Oswaldo Cruz Foundation (Fiocruz), Brazil
- WHO –TDR (Special Programme for Research and Training in Tropical Diseases) as a permanent observer





# Dynamic portfolio: New disease areas, new models...

Neglected diseases

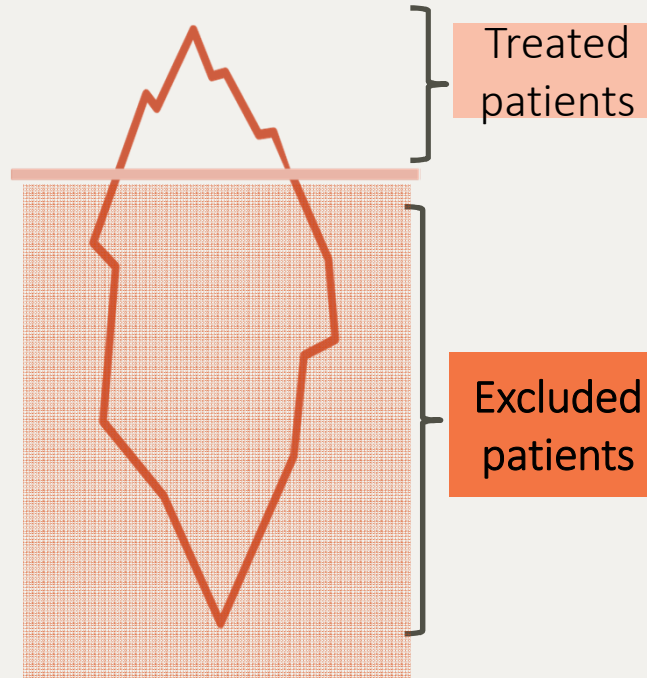
**Mycetoma**



Testing ravuconazole

Neglected patients

**Hepatitis C**



Public health approach

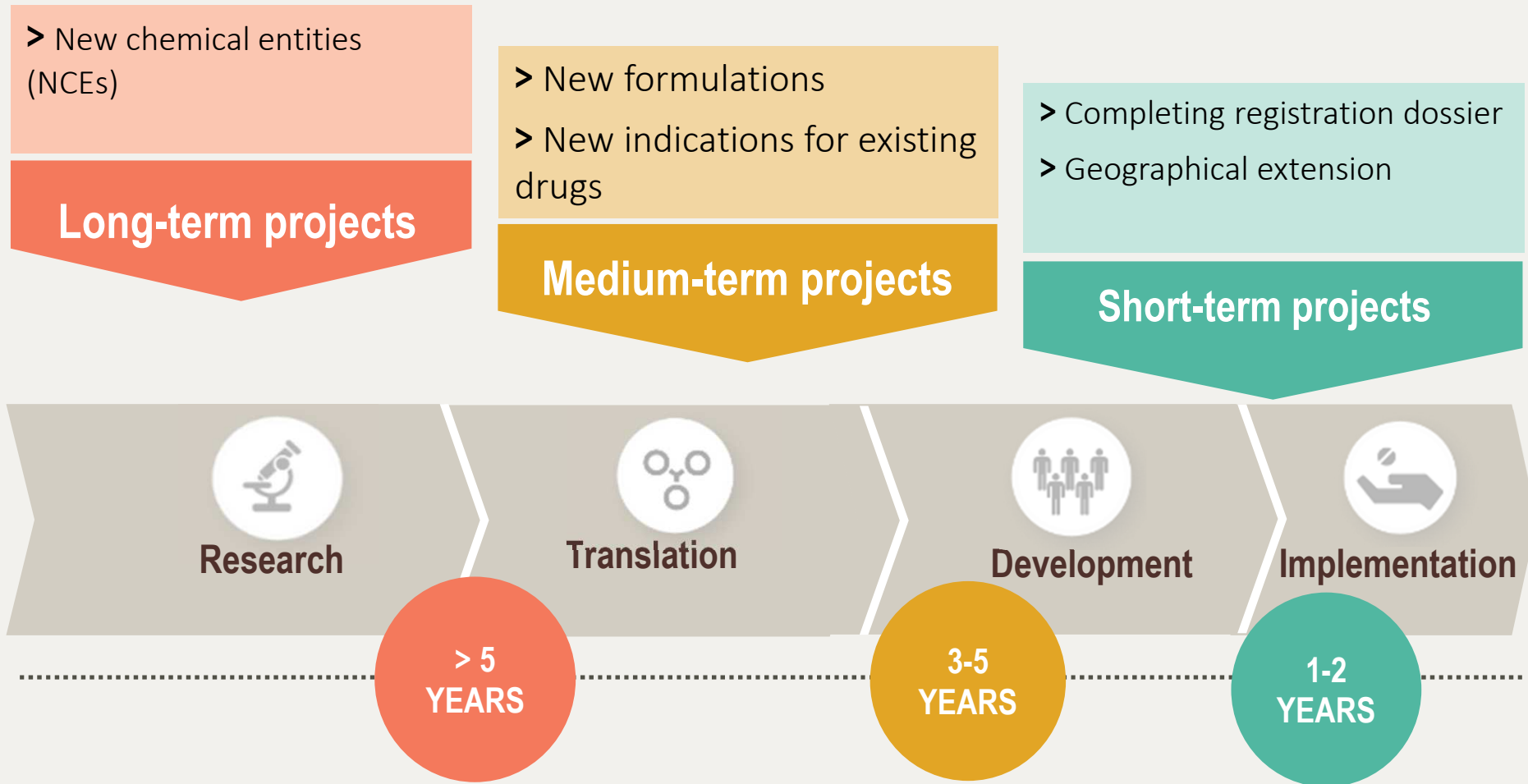
Neglected models

**Antimicrobial resistance**

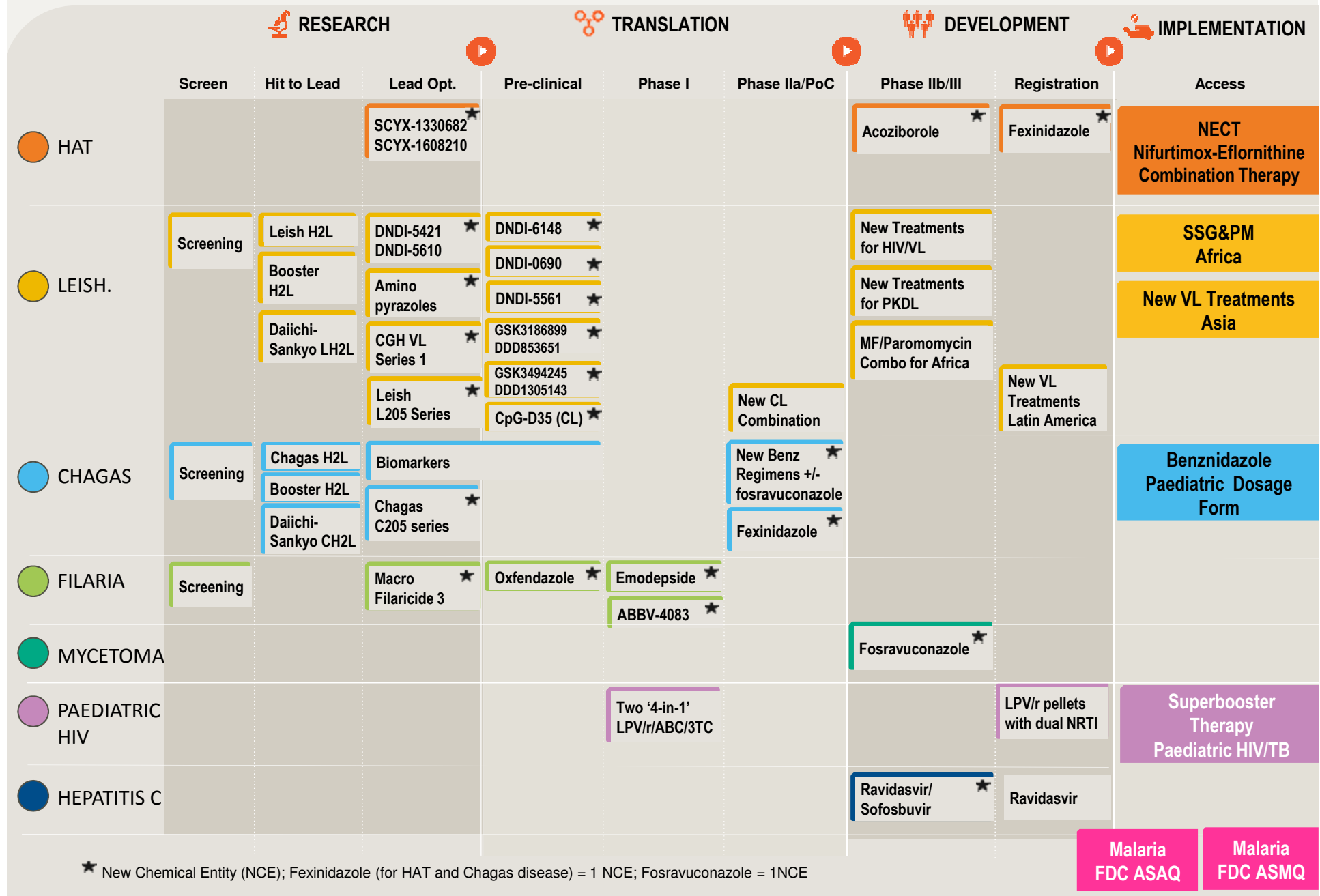


Incubation of GARD

# Short-, medium- and long-term approaches to address immediate patient needs and deliver innovative medicines



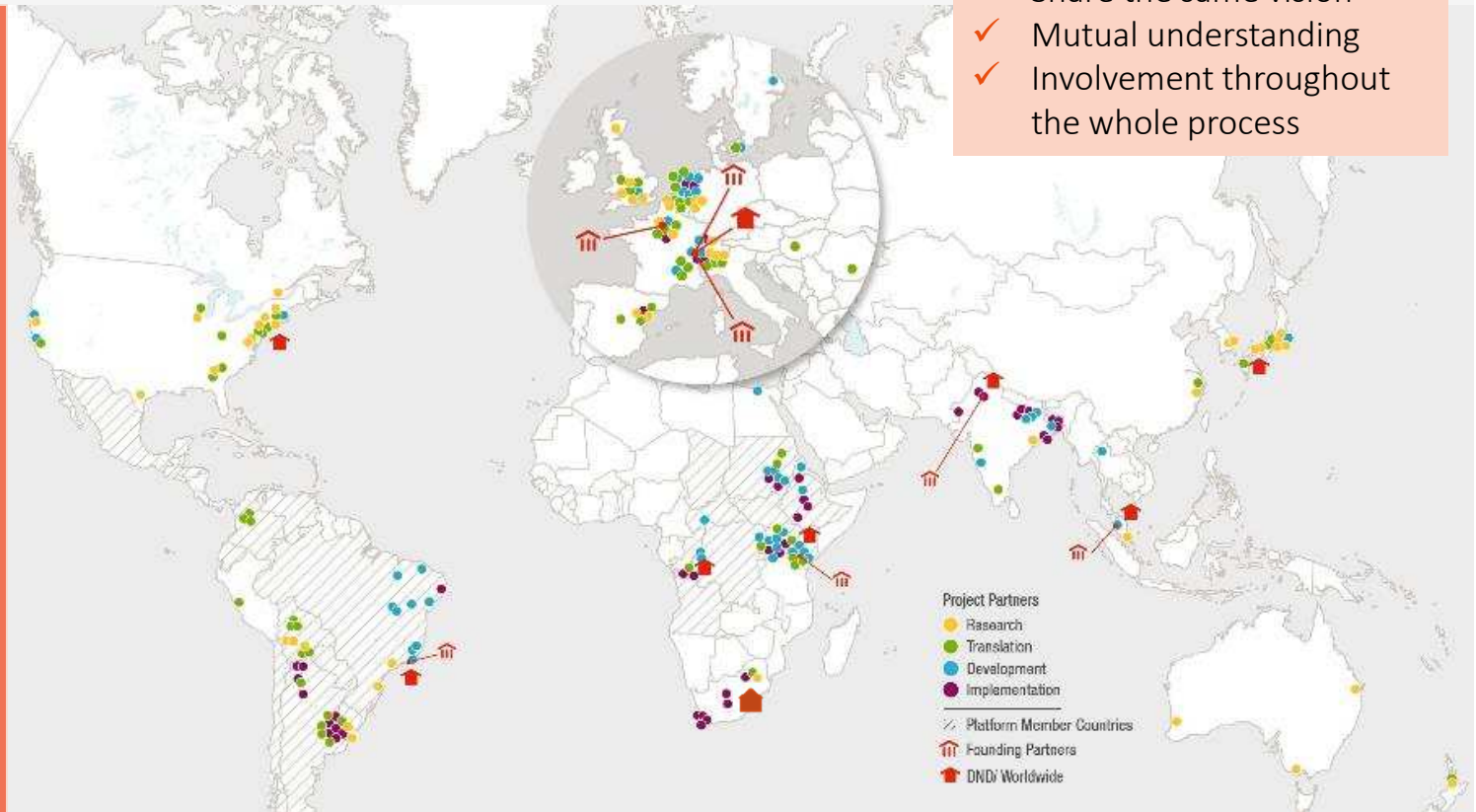
# DNDi portfolio: Improve existing treatments & develop NCEs



# DNDi's success is only possible through innovative partnerships

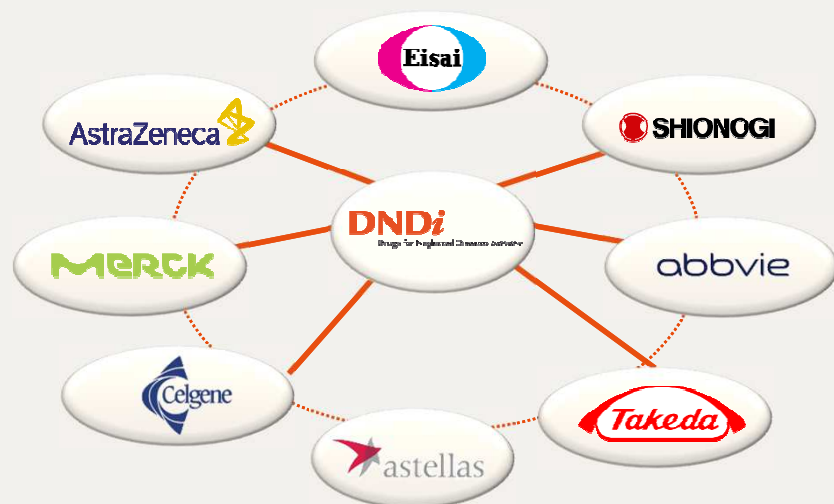
## Over 170 Partnerships Worldwide

Biotechs  
Pharmaceutical  
companies  
PDPs  
International  
organisations  
NGOs  
CROs  
Universities  
Research Institutes

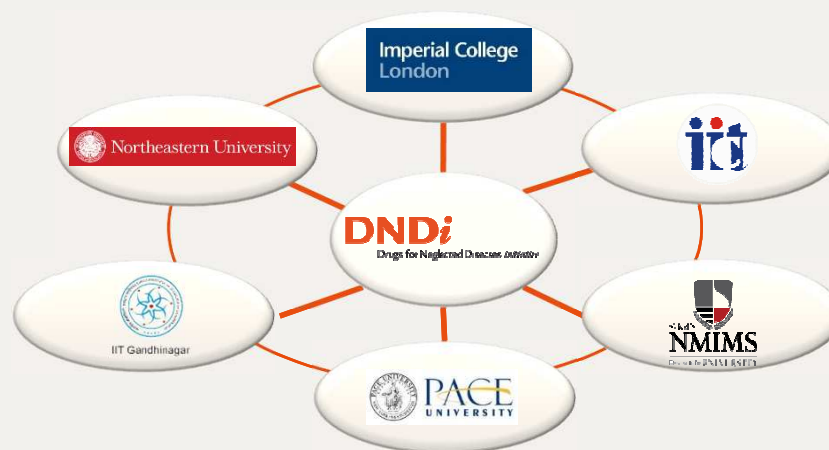


# Open innovation to speed up drug discovery.

NTD Drug Discovery Booster, *Est. 2015*



Open Synthesis Network, *Est. 2016*



The Mycetoma Open Source project (MycetOS),  
*Est. 2017*

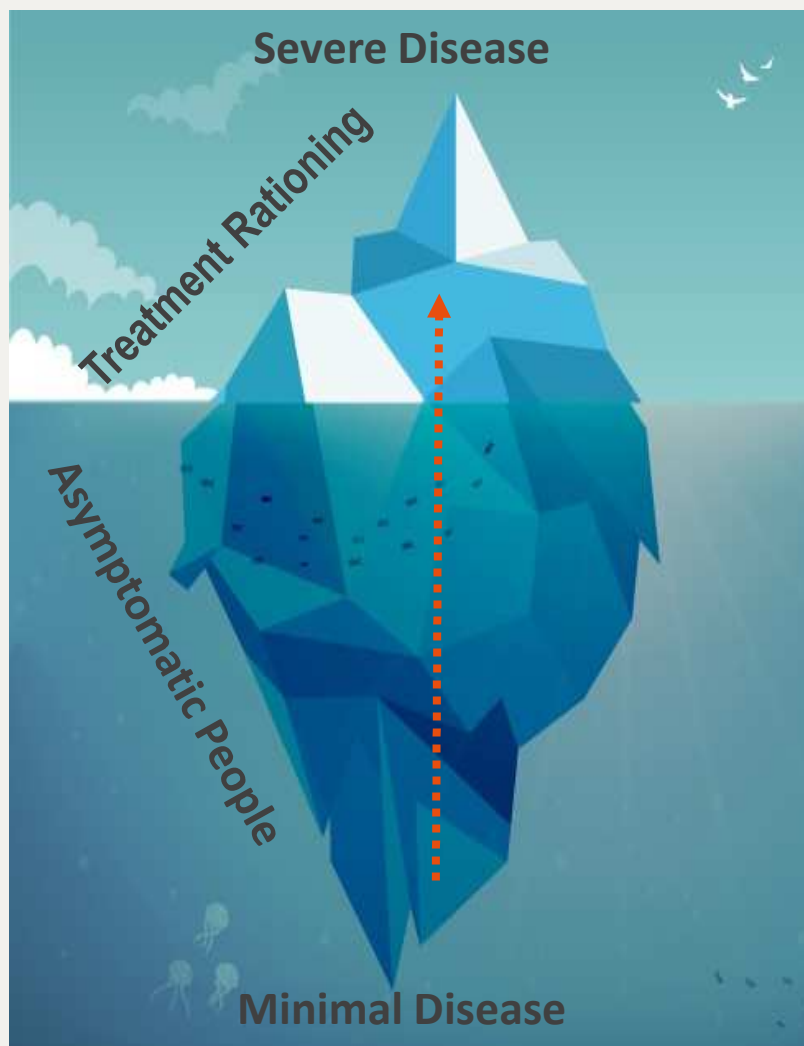


# For each disease, a Target Product Profile to guide all decisions: **HCV TPP**

<sup>8</sup> Characteristics	Ideal	Acceptable
Efficacy	>95% SVR	>= 95% SVR
Safety	No side effects	Minimal side effects
Pangenotypic	Yes	Yes
Treatment duration	Same regimen F0-F4 10 weeks (8 wks ?) F0-F3 12 weeks F4	12 weeks F0-F3 12 weeks with RBV F4 24 weeks without RBV F4
Populations	Mono & HIV co-infected polytransfused & people who inject drugs	Mono & HIV co-infected ; polytransfused & people who inject drugs
Dosing	FDC once a day	Two tablets once a day (+/-RBV)
Drug-Drug Interactions	None in HIV/HCV	Manageable in HIV/HCV
Monitoring	POC diagnosis, Triage (F?), SVR12	POC diagnosis, Triage (F?), SVR12, Minimal safety monitoring
Cost	~ US\$ 300 by 2017	~ US\$ 300 by 2020



# DNDi HCV strategic objectives:



- Develop new, affordable, pan-genotypic TT for HCV
- Simplify HCV test & treat strategies and develop innovative models of care to support scale up
- Improve access (IP, regulatory, pricing, etc.) and affordability of HCV TT in countries

# DNDi Hepatitis C Strategy: 3 pillars

**1**

**Accelerate  
R&D**

Accelerating the development  
of **promising drug  
candidates**



**with**



Pharma  
companies



Governments

**2**

**Catalyse  
ACCESS**

Supporting **affordable  
access to all DAAs**



**with**



Pharma  
companies  
Civil Society  
organisations



Governments

**3**

**Simplify  
TREATMENT  
STRATEGIES**

Working with health  
providers to scale –  
up treatment



**with**



Primary  
healthcare  
doctors



Non  
Governmental  
Organizations

# A mature and dynamic portfolio, with strong partnerships and donor support



**39** R&D projects  
in **8** disease areas  
with **7** treatments delivered



GARDP: new initiative  
**created** by WHO and DNDi  
and **incubated** by DNDi



**20** entirely  
new chemical entities  
(NCEs) in the pipeline



**210** staff  
**Half** in endemic countries  
Close to **1,000** people  
working on DNDi projects



EUR **532 million** raised  
**equally** from public  
and private sources



**4** disease-specific clinical  
trial platforms  
Several technology  
transfers

# 7 new treatments delivered since 2007



- ✓ Easy to use
- ✓ Affordable
- ✓ Field-adapted
- ✓ Non-patented



2007 **ASAQ**  
Malaria  
>500 million patients  
reached



2008 **ASMQ**  
Malaria  
Used in Africa and Asia



2009 **NECT**  
Sleeping sickness  
100% of stage-2 patients



2010 **SSG&PM**  
Visceral leishmaniasis in E Africa  
**Now 1st line in all countries**



2011 **PAEDIATRIC BENZNIDAZOLE**  
Chagas disease  
**Two sources developed**



2011 **NEW VL TREATMENT ASIA**  
Visceral leishmaniasis in Asia  
**Support to elimination  
programme**



2016 **SUPERBOOSTER THERAPY**  
Paediatric HIV  
**Recommended by WHO**



# Sleeping sickness, two new oral treatments to change the history of the disease



15 years ago

Melarsoprol

Toxic, resistant

Eflornithine

Not available

Since 2009

NECT

Improved therapy



2018

Fexinidazole

Oral (10 days)



2021

Acoziborole

Single-dose, oral



# The Global Antibiotic R&D Partnership (GARDP)

Created by WHO and DNDi, incubated by DNDi

Antimicrobial resistance is a major and rapidly growing global public health challenge, with estimates of up to 700,000 deaths per year.

## Focus:

- Drug-resistant bacterial infections for which adequate treatment is not available.
- Address global health priorities that reflect the realities of clinical practice.

## Scope:

- Global including low- and middle-income countries.



# GARDP's priorities and programmes

---

Developing and delivering new or improved antibiotic treatments for which , while endeavouring to ensure sustainable access.

## 2023 objectives

Develop 4 new treatments through:

- Improving existing antibiotics
- Developing new chemical entities.

Build a robust pipeline of pre-clinical and clinical candidates end to end.

Actively support appropriate use of and access to new antibiotic treatments.

**Neonatal sepsis:** developing treatments for highly drug-resistant infections in babies.

**Paediatric antibiotics:** exploring ways to optimize current and develop new antibiotics for children.

**Sexually-transmitted infections:** develop a new treatment for drug-resistant gonorrhoea and other STIs.

**Memory recovery and exploratory:** revive and evaluate old knowledge and abandoned projects, support early research.

## How much is it to develop a treatment for neglected patients?

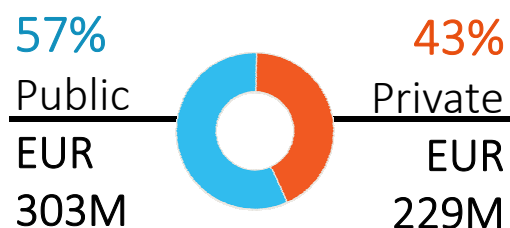
By putting the specific needs of patients at the centre of the innovation process, the Drugs for Neglected Diseases initiative along with its partners has proved that it is possible to address the medical needs of the poorest populations by developing quality, adapted, and affordable treatments. DNDi's cost of development ranges from EUR 10-40 million for a combination therapy made of already existing drugs, and EUR 100-151 million for a new treatment made from scratch.

DISEASE	DRUG	 Research	Early safety and proof-of-concept trials in patients	Larger scale safety and efficacy trials	Post-registration trials for additional data	TOTAL COST
COMBINATION THERAPIES						
MALARIA	ASAQ FDC	Not needed for combinations of approved drugs		€7m	€5.5m	€12.5m
SLEEPING SICKNESS	NECT			€3.6m	€3.2m	€6.8m
VISCERAL LEISHMANIASIS	SSG & PM			€9.4m	€2.2m	€11.6m
NOVEL DRUGS						
SLEEPING SICKNESS	Fexinidazole	€7.2m	€4.5m	€28.5m	€15.1m*	€55.3m
SLEEPING SICKNESS	SCYX-7158	€22.2m	€3.6m	€18.5m	€15.1m*	€59.4m

\*Projected estimates until 2020



# The support of our donors: €532M received out of €730M needed by 2023



## STRICTLY RESTRICTED FUNDING (23%) – € 121M

- Bill & Melinda Gates Foundation (€53M) – (\$63.3M)
- Japan GHIT Fund (€20.2M)
- Wellcome Trust (€16.2M)
- European Union – FP5,6,7& EDCTP (€13.6M)
- Médecins Sans Frontières (€10.2M)
- USA – NIH/NIAID (€1.6M) – (\$1.8M)
- United Kingdom – DFID (€1.6M) – (£1.2M)
- Switzerland – Canton de Genève (€1.9M)
- UBS Optimus Foundation (€1.5M) – (CHF 2M)
- Switzerland – SDC (€0.7M) – (CHF 0.9M)
- The Global Fund – AMFm (€0.5M)
- Ruta’N Medellin (€0.3M)
- Kalacore (€0.3M)

## UNRESTRICTED FUNDING (48%) - € 255M

- United Kingdom – DFID (€136.4M) – (£113M)
- Médecins Sans Frontières (€83.5M)
- Switzerland – SDC (€17.4M) – (CHF 19.2M)
- Spain – AECID (€12M)
- Other Private Foundations - Rockefeller, Slim, Starr, FINEP, Moreau, BBVA (€5.6M)

## PORTFOLIO FUNDING (29%)- € 156M

- Bill & Melinda Gates Foundation (€50.6M) – (\$60.4M)
- Netherlands – DGIS (€33M)
- France – AFD & MAEE (€16.3M)
- Unitaid (€12.5M) – (\$14.4M)
- Germany – KfW & GTZ (€20.1M)
- USAID (€9.4M) – (\$10M)
- Fundación Mundo Sano (PRV) (€6.2M)
- Medicor Foundation (€3.8M)
- WHO/TDR (€2.6M)
- Norway – NORAD (€2.8M)
- Brazil - BNDES & MoH (€0.4M)

**Note: Does not include GARDP funding**

# Public leadership is needed for a favourable environment

- Sustainable financing
- Identification of R&D needs for better priority setting
- Adapted regulatory environment at national & regional levels
- IP environment to catalyse innovation and facilitate access





A photograph of a woman with short dark hair, wearing a grey and black patterned sweater, smiling as she feeds a young child. The child, wearing a purple long-sleeved shirt with a Hello Kitty design, is looking up at the woman with an open mouth. The woman is holding a spoon to the child's mouth. The background shows a wooden headboard and a white cloth.

**THANK  
YOU!**

A dark, semi-transparent overlay on the right side of the image, featuring a faint, stylized illustration of a person's head and shoulders. The DNDi logo is positioned in the bottom right corner of this overlay.

**DNDi**  
Drugs for Neglected Diseases *initiative*