DND*i* Partners' Meeting – Rio

DNDi
Drugs for Neglected Diseases *initiative*

Innovation & Access for Neglected Populations

A dynamic approach towards 2023

Dr Bernard Pécoul,
DNDi Executive Director

DNDi
Drugs for Neglected Diseases *initiative*

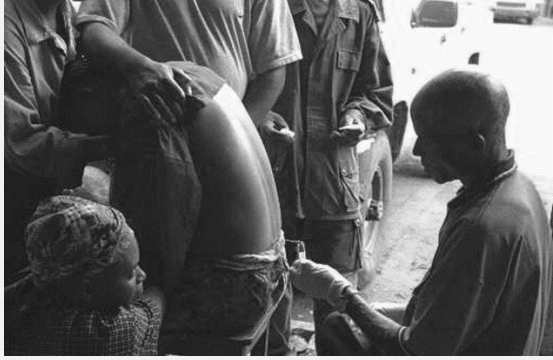
Origins of DND*i*

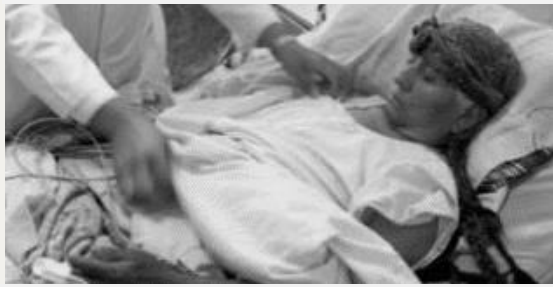
1999

- First meeting to describe the lack of R&D for neglected diseases
- MSF commits the Nobel Peace Prize money to the DND Working Group
- JAMA article: *'Access to essential drugs in poor countries - A Lost Battle?'*

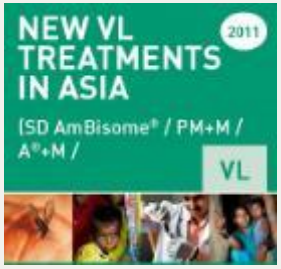
July 2003

- Creation of DND*i*
- Founding partners:
  - *Institut Pasteur, France*
  - *Indian Council of Medical Research, India*
  - *Kenya Medical Research Institute, Kenya*
  - *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*





7 new treatments delivered, recommended, implemented

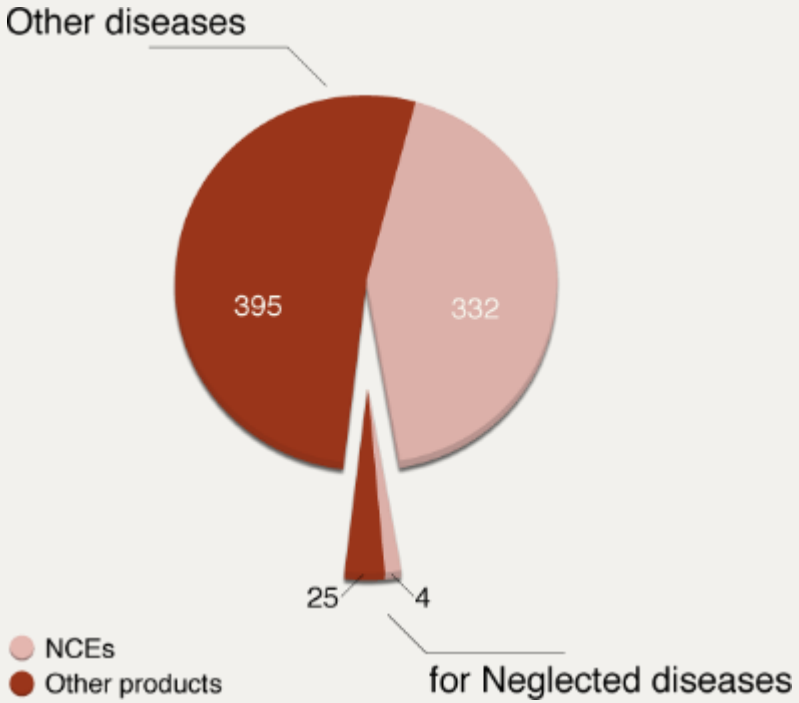


- ✓ Easy to use
- ✓ Affordable
- ✓ Field-adapted
- ✓ Non-patented

- **30 projects, 8 diseases areas**
- **13 entirely new chemical entities (NCEs)**
- **Over 160 partnerships,** most in endemic countries
- **160 staff**, half in endemic countries & 700 people working on DNDi projects
- **EUR 400 million** raised equally from public and private sources
- **4 regional disease-specific clinical trial platforms/ networks** and several technology transfers

Fatal imbalance still exists, an adapted R&D response is required

756 products developed (excluding vaccines & biologicals) (2000-2011) *



* Source: Pedrique B et al. The drug and vaccine landscape for neglected diseases (2000-11): a systematic assessment. *Lancet Global Health*, Early Online Publication, 24 Oct 2013.

Business Plan Review

Extensive **consultation through Regional Offices and with key stakeholders** and **partners to assess:**

- Lessons learned from DND*i* experience
- R&D landscape evolution
- Patient needs and gaps
- Future trends

The R&D landscape for neglected patients has changed but large gaps still remain

1. **R&D priorities** do not sufficiently originate from **low- and middle-income countries**
2. Patients' **needs are not prioritized** (e.g. Ebola, mycetoma, etc.)
3. **Innovation is not linked to equitable access** even when there is commercial incentive to drive innovation (e.g. HCV)
4. **Market incentives** aligned with IP/exclusivity do not adequately address health needs in LMICs (e.g. AMR)

These are the fundamental challenges for the future of biomedical innovation.

An unchanged vision, with a broader mission

- Develop new drugs or new formulations of existing drugs for **people suffering from neglected diseases**
- Maintain commitment to **most neglected diseases** and take on new disease areas
- **Strengthen capacities** in a sustainable manner
- Adopt a more **dynamic portfolio approach** with new **operating models**

Unchanged strategy: Improving treatments with existing drugs and delivering New Chemical Entities

New chemical entities (NCEs)

New formulations

New indications for existing drugs

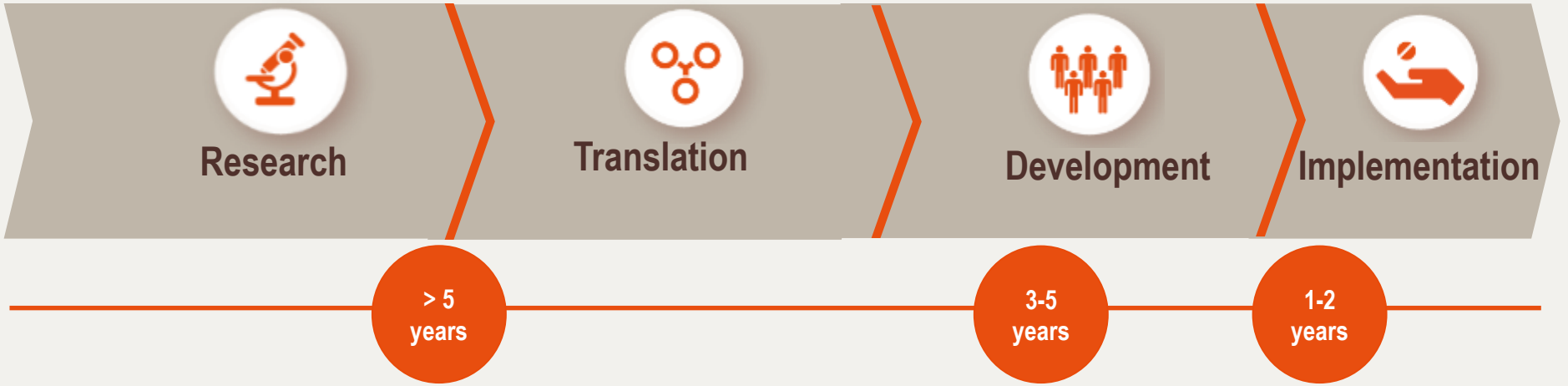
Completing registration dossier

Geographical extension

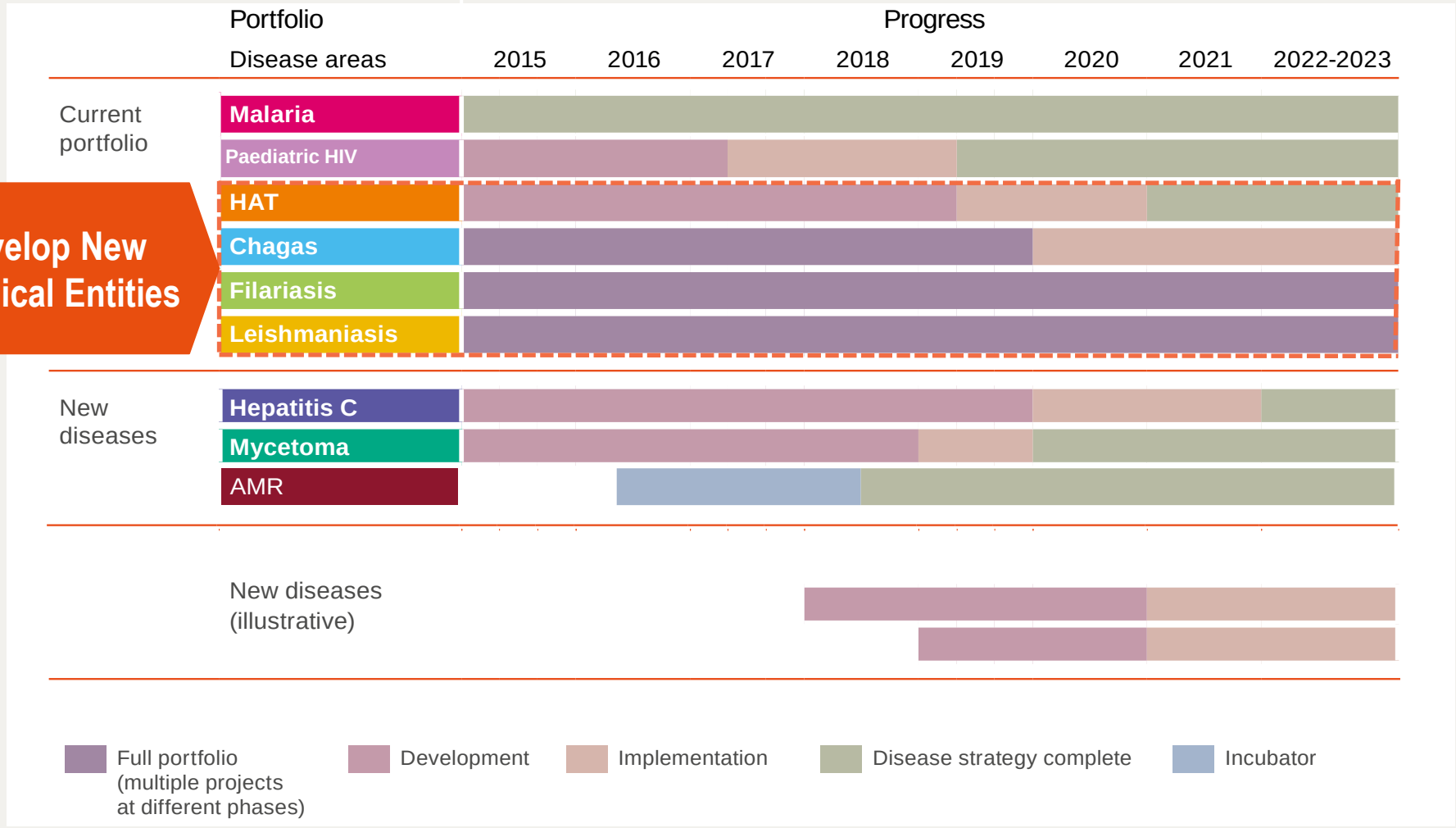
Long-term projects

Medium-term projects

Short-term projects



Most neglected diseases remain at the core, with new diseases taken on progressively



By 2023: Deliver 16 to 18 treatments with EUR 650 million

2016
7 treatments delivered

2023
16-18 treatments

2023
9 -11 additional treatments delivered

Influence the R&D landscape for neglected patients

- Political leadership for **needs-driven R&D**
- Creation of a **global fund and mechanism**
- Evidence on alternative **R&D models**

Develop treatments for people suffering from neglected diseases

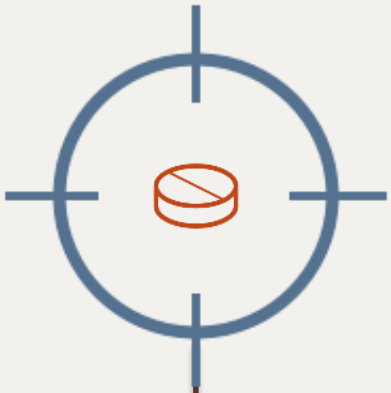
- Deliver 16-18 **treatments**
- **3 new chemical entities (NCEs)**
- ~10 disease areas
- **Focus on access and measure impact**

Strengthen research capacity, led by Regional Offices

- **R&D platforms** in disease-endemic countries
- **Regionally-driven** initiatives
- **Patient access** to treatments
- **Transfer of technology**

Develop treatments for patients suffering from neglected diseases

Sleeping sickness: Two new treatments in development to support sustainable elimination



13 years ago

Melarsoprol:

Toxic, resistant

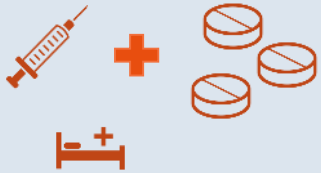
Eflornithine:

Not available

Since 2009

NECT

Improved therapy



2018?

Fexinidazole

Oral treatment (10 days)

Future objective

SCYX-7158

Single-dose, oral treatment

Leishmaniasis: Improving treatments with existing drugs

13 years ago

Treatment limitations:

- Toxic
- Painful
- Resistance
- Not registered
- Expensive
- Long duration
- Not field adapted

Since 2010

SSG & PM for VL in Africa

Since 2011

New treatments for VL in Asia
(SD Ambisome®, Paromomycin + Miltefosine combination)

2016-17

A new first-line treatment for VL in Latin America

By 2023

- Treatment for **HIV/VL**
- Treatment for **PKDL**
- Treatment combination for **CL**

Leishmaniasis: Towards new, safe, and effective treatments issued from drug discovery

< 2016

Drug discovery

- 6 new series from DNDi or partners
- Selection of an immune modulator (CpG) for **Cutaneous Leishmaniasis (CL)**

2016+

Progress with New Chemical Entities

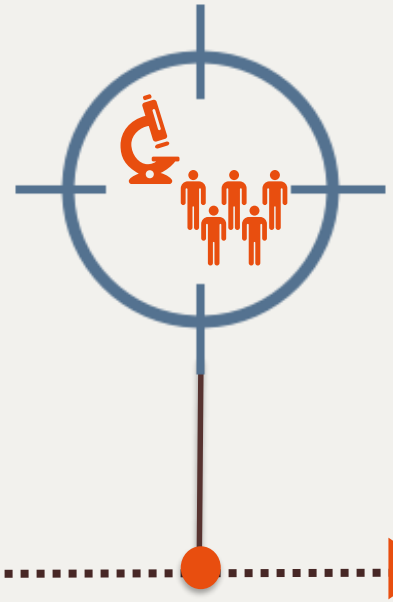
- Anfoleish for **CL**
- 3 NCEs entering pre-clinical development

By 2023

To deliver:

- A new oral treatment for **VL** and/or
- A CpG for **CL**

Chagas disease: improve existing treatments and strong effort in drug discovery



13 years ago

Benznidazole
Nifurtimox

Treatment limitations

- Toxic
- Limited efficacy
- Lack of availability
- No paediatric formulation

2011

Paediatric dosage form of benznidazole

- age-adapted
- easy-to-use
- affordable

2016-2020

Shorter, simplified treatment

- Fexinidazole (NCE)
- New benznidazole regimens

By 2023

Bring NCEs into development stage

Chagas: How to address the major access gap?

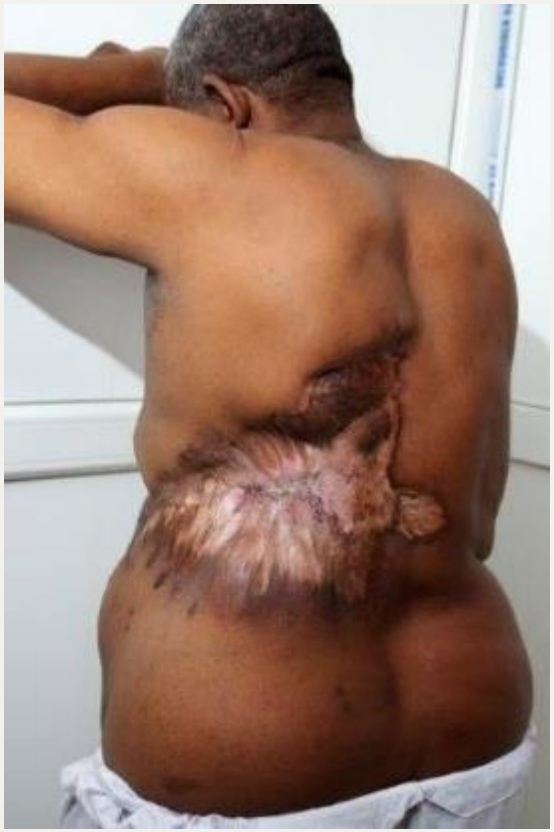
Less than 1% of patients treated

- With Chemo/Mundo Sano, register adult & paediatric BZN with US FDA, in Latin American countries
- Availability and affordability: multiple sources
- Access plan with the Global Chagas Coalition and other partners
- Pilot projects to boost access to diagnosis and treatment

Dynamic portfolio: New disease areas, new models….

Neglected diseases

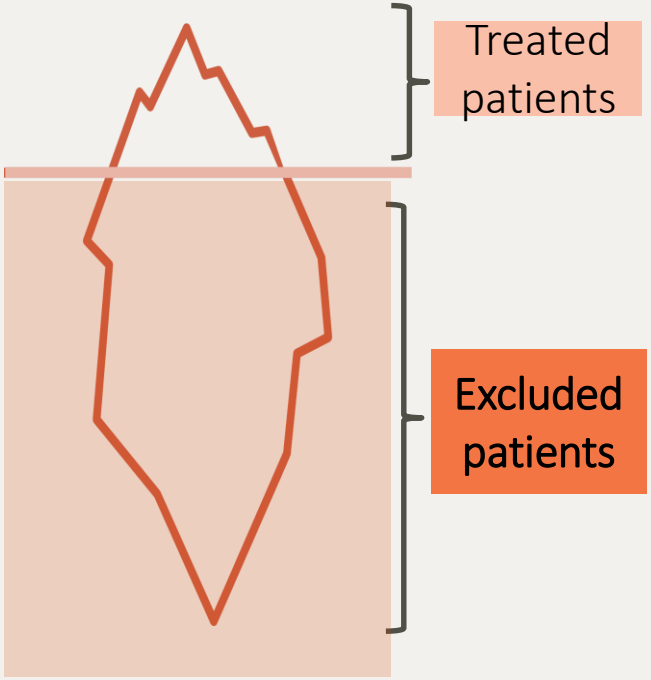
Mycetoma



Testing ravuconazole

Neglected patients

Hepatitis C



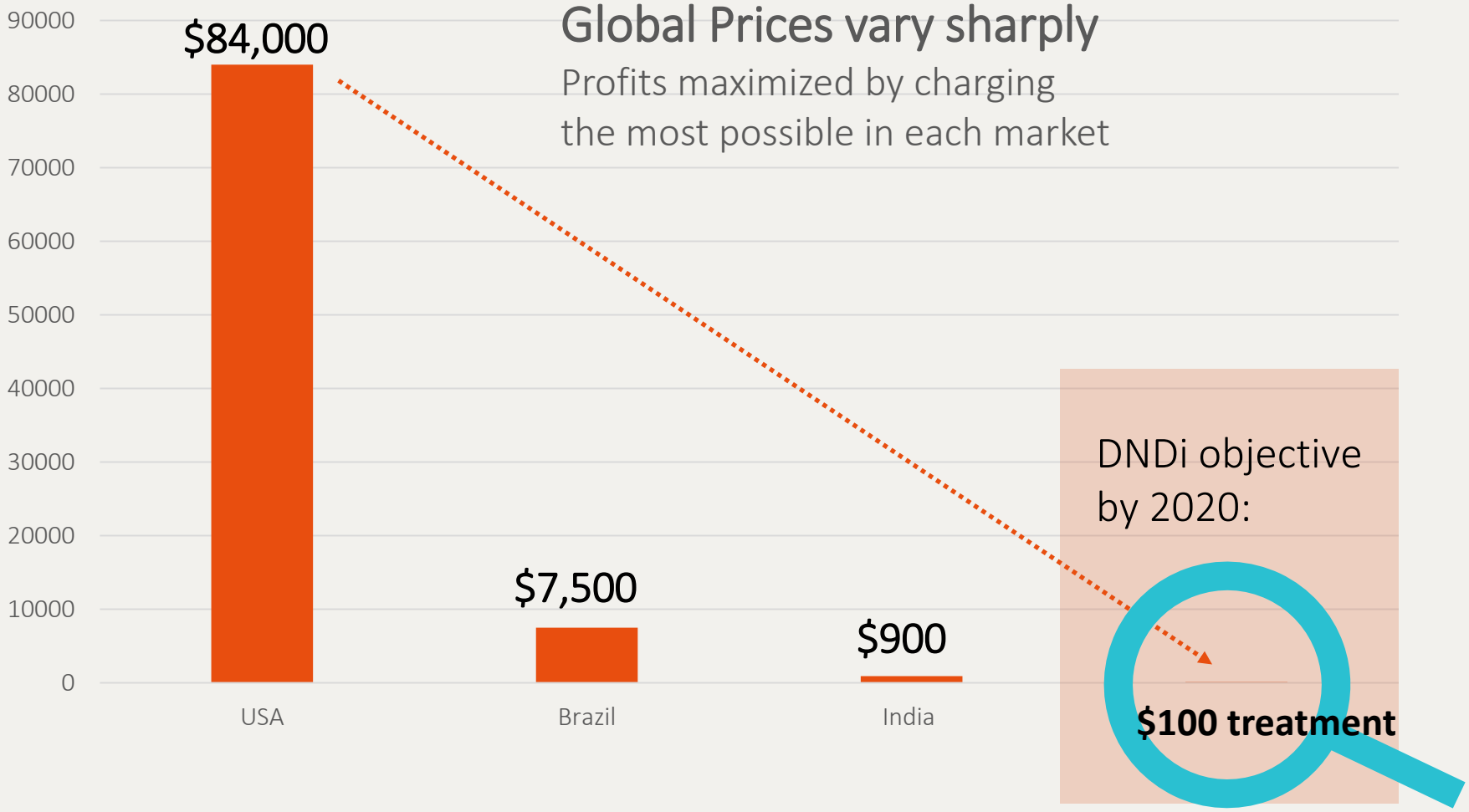
Public health approach

Neglected models

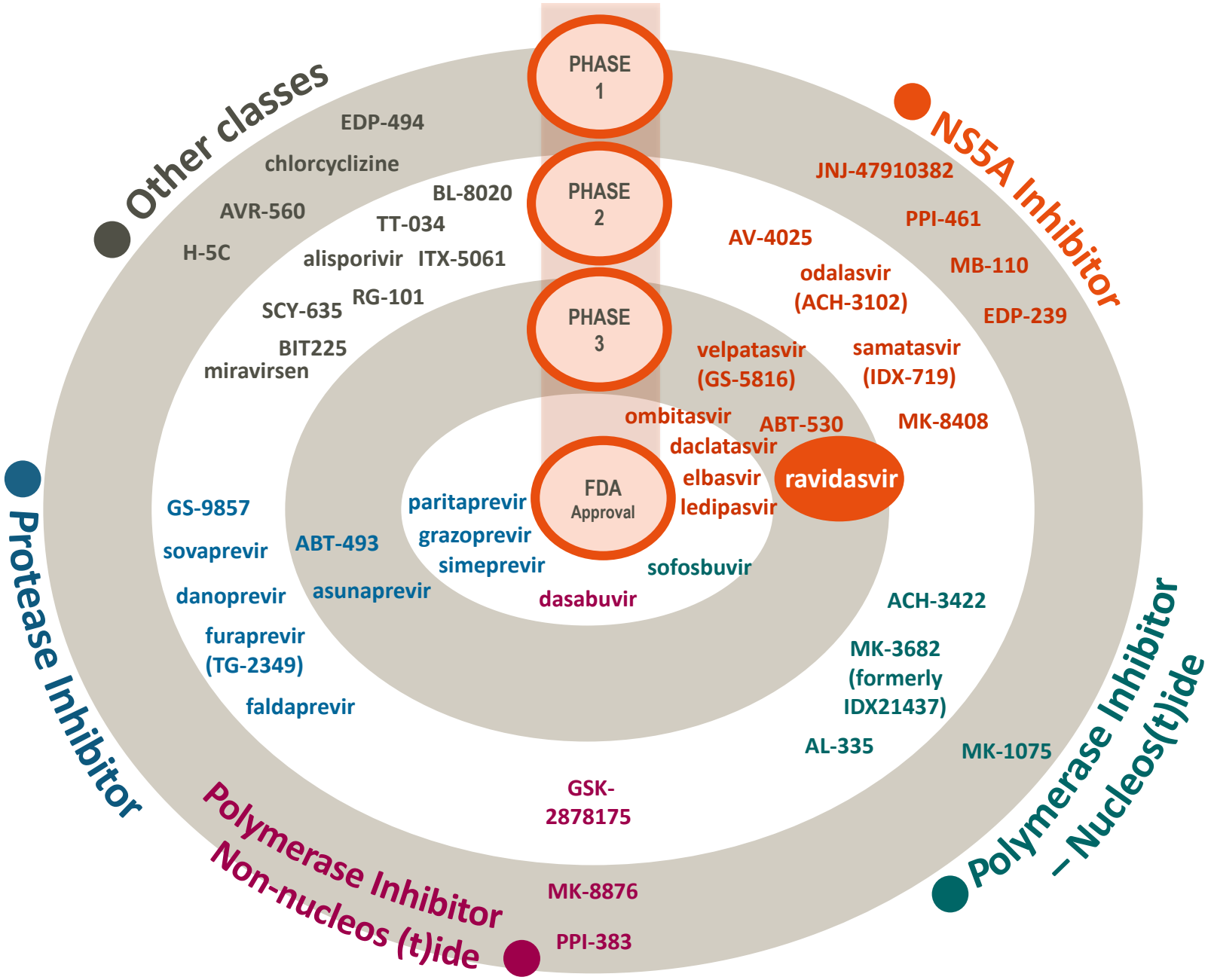
Antimicrobial resistance

Incubation of GARD

Hepatitis C: exorbitant price prevents public health approach



Abundant R&D pipeline… but many drug candidates abandoned



A pan-genotypic treatment for less than $300

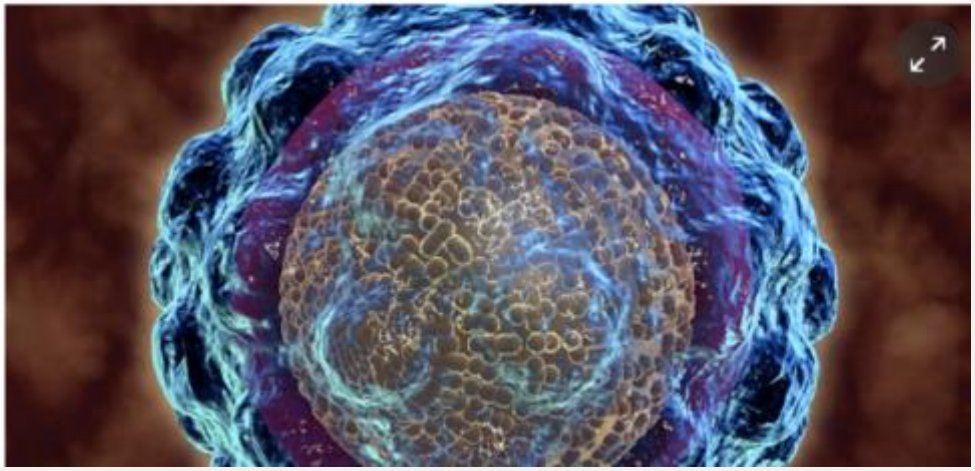
- DND*i*, Pharco and Presidio agreement to test combination of sofosbuvir + ravidasvir
- Partnership with Malaysia and Thailand to conduct Phase II/III multicentre study (900 patients)
- Using innovative licensing agreement or TRIPS flexibilities

theguardian

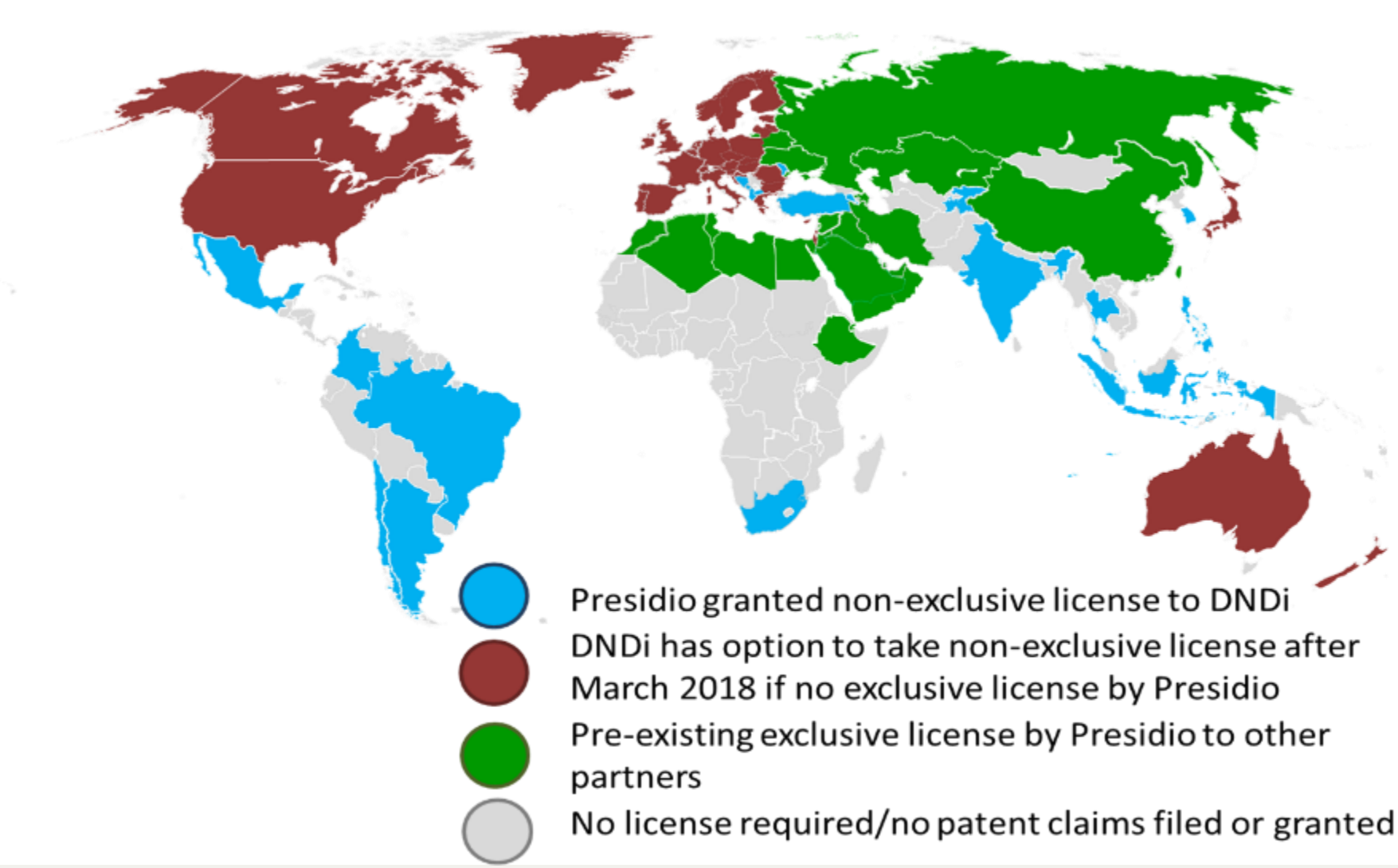
April 13, 2016

Hepatitis C treatment for under $300 coming soon

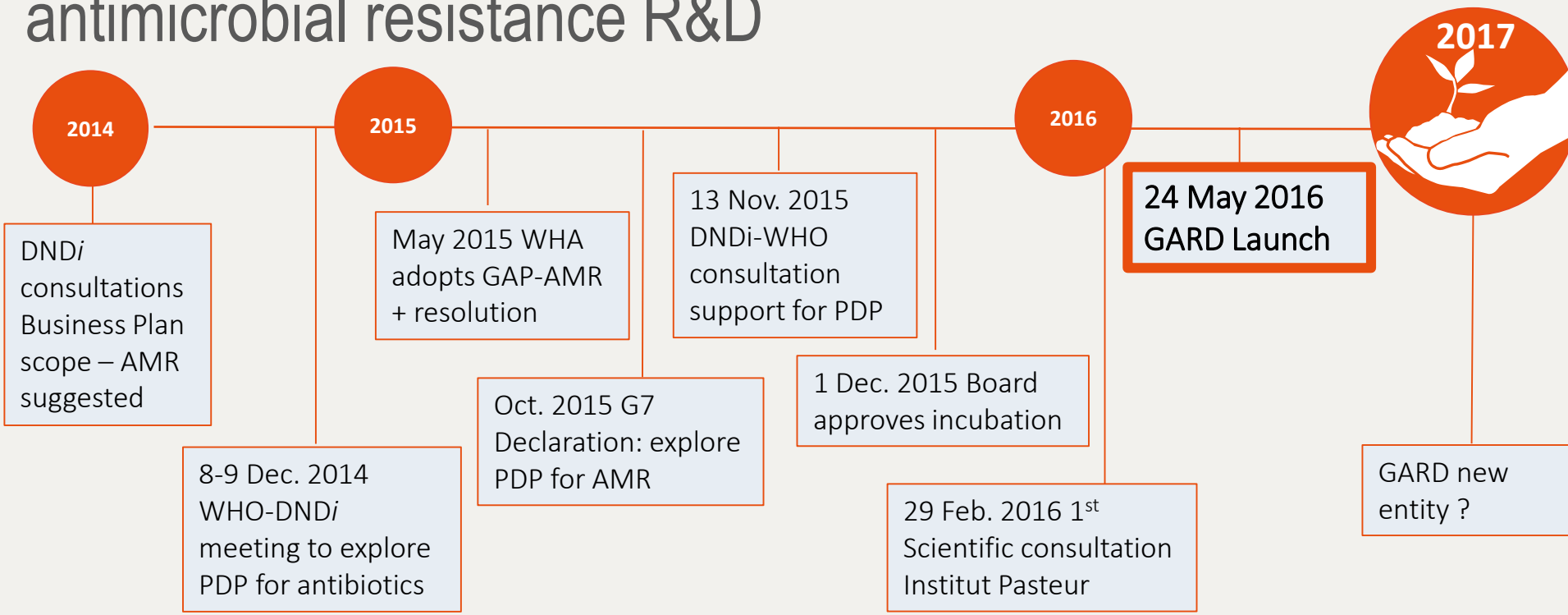
Drugs for Neglected Diseases initiative says drug successfully tested in Egypt could be available within 18-24 months



An innovative licensing agreement for ravidasvir that covers a very large territory



DNDi & WHO to collaborate to incubate GARD for antimicrobial resistance R&D



EUR 2.2 million of the required EUR 3 million seed funding committed to date:

- Federal Ministry of Health of Germany
- The Netherlands' Ministry of Health Welfare and Sports
- South African Medical Research Council
- United Kingdom Department for International Development
- Médecins Sans Frontières

GARD: Vision & Objectives

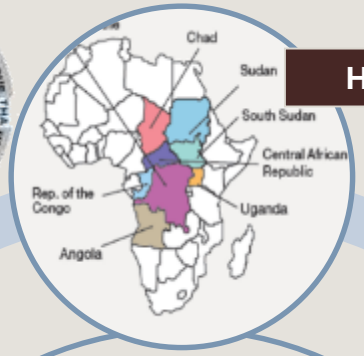
In cooperation with the public and private sectors:

- develop new antibiotic treatments addressing AMR
- promote their responsible use for *sustainable* access

by setting up a not-for-profit product development partnership that will focus on global health needs, and ensuring any new product is adapted to resource-limited settings.

Strengthen research capacity, led by Regional Offices

Using & strenghtening research capacities in endemic regions



HAT

A Key Role for Regional Disease Platforms

Defining patient needs and Target Product Profile (TPP)

Strengthening local capacities

Conducting clinical trials (Phase II/III studies)

Facilitating Registration of new therapies

Accelerating implementation of new therapies, ensure therapies reach patients

CHAGAS

LEISHMANIASIS

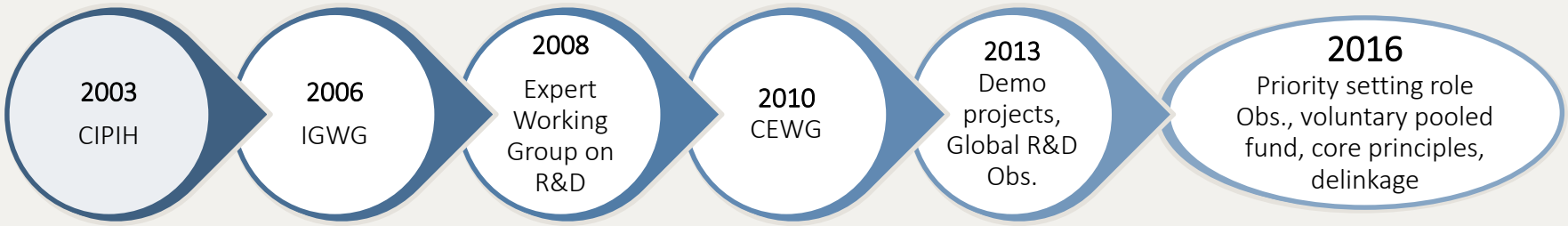
LEISHMANIASIS

LEAP
LEISHMANIASIS
EAST AFRICA PLATFORM

Influence the R&D landscape for neglected patients

Innovation & Access on the political agenda like never before

13 years of discussions at WHA, with 6 resolutions (2003-2016)



Connect the dots

- R&D Blueprint for Emerging Pathogens
- July 2016: UN High-Level Panel on Access to Medicines
- September 2016: UN High-Level Meeting on AMR

Need to develop an overarching framework: priority-setting, sustainable funding, and principles

Funding for R&D initiatives

NIH National Institutes of Health

wellcome trust

USAID FROM THE AMERICAN PEOPLE

ruta n MEDELLÍN

GHIT Fund
Global Health Innovative Technology Fund

BILL & MELINDA GATES foundation

BNDES The Brazilian development bank

DFID

UNITAID

EUROPEAN COMMISSION

EDCTP

...and others

Global Biomedical R&D Fund and Mechanism
For innovations of Public Health importance
governed by public leadership

Global Health R&D Observatory
Priority-Setting, Monitoring, Coordination

De-linkage

Open Innovation

Licensing for Access

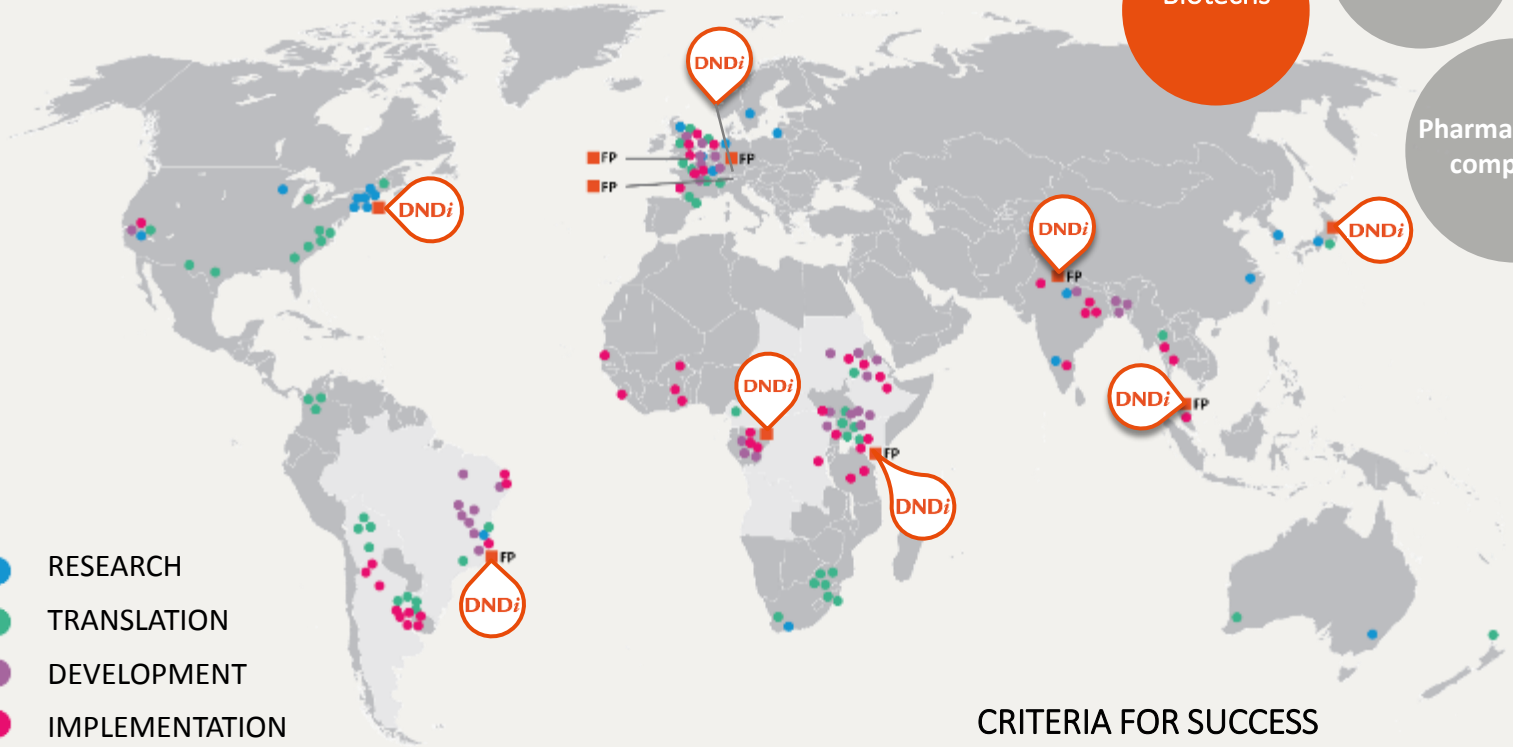
AMR

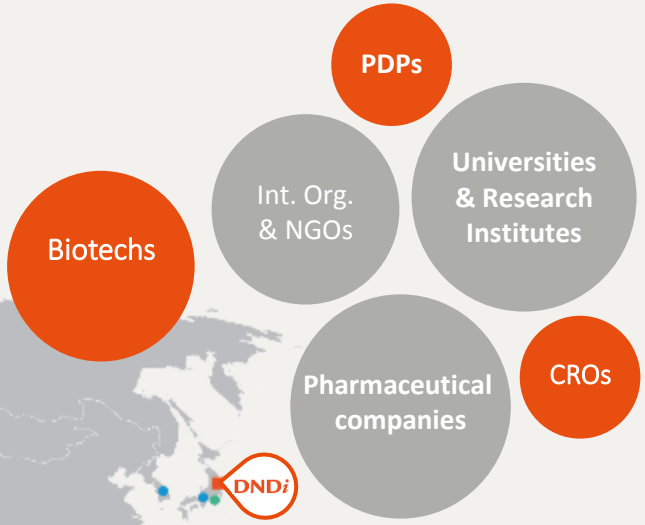
Emerging Infections (incl. Ebola)

Poverty Related / Neglected Diseases

DND*i*'s success is only possible through innovative partnerships

Over 160 partnerships worldwide

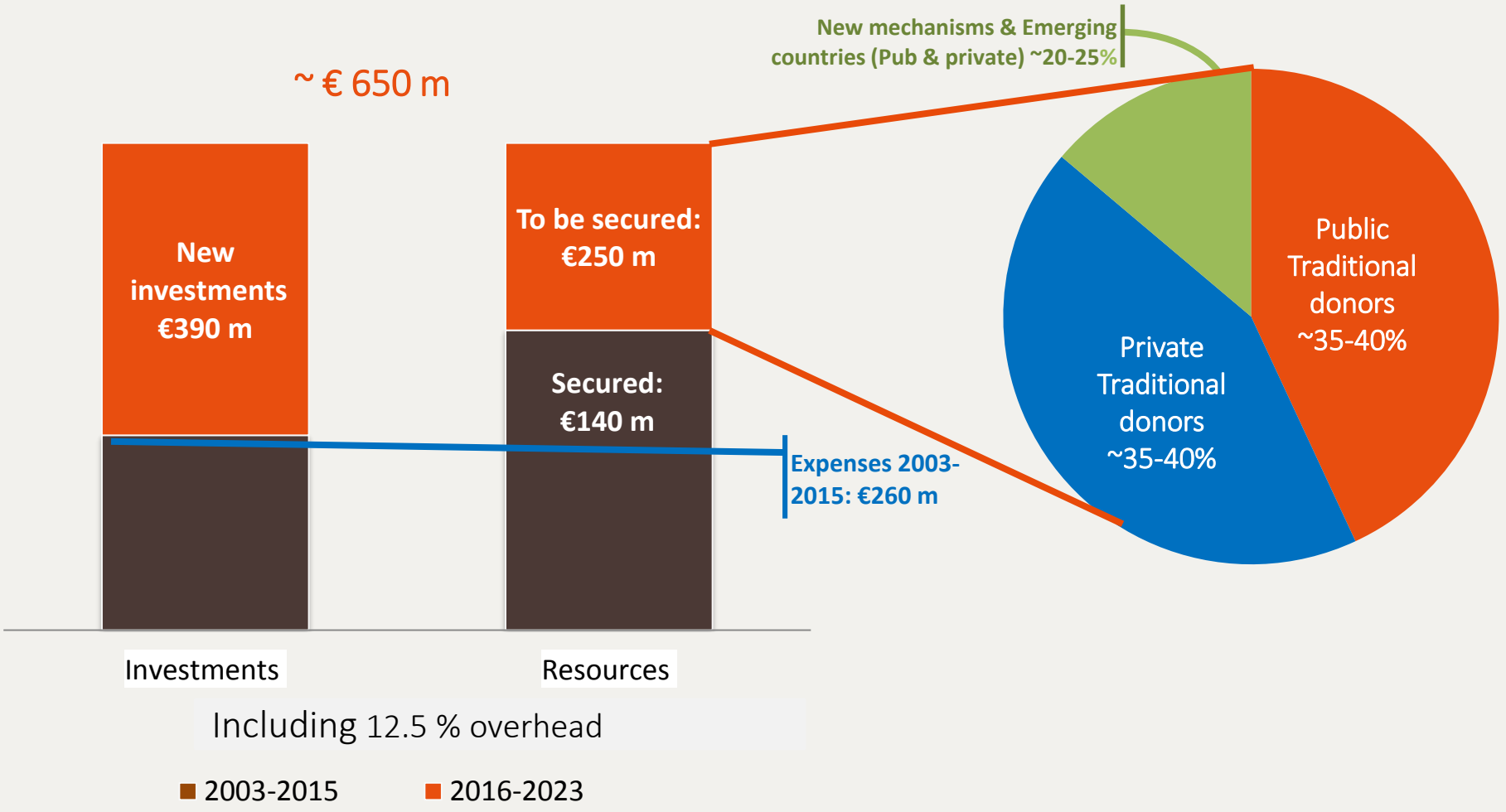




CRITERIA FOR SUCCESS

- ✓ Share the same vision
- ✓ Mutual understanding
- ✓ Involvement throughout the whole process

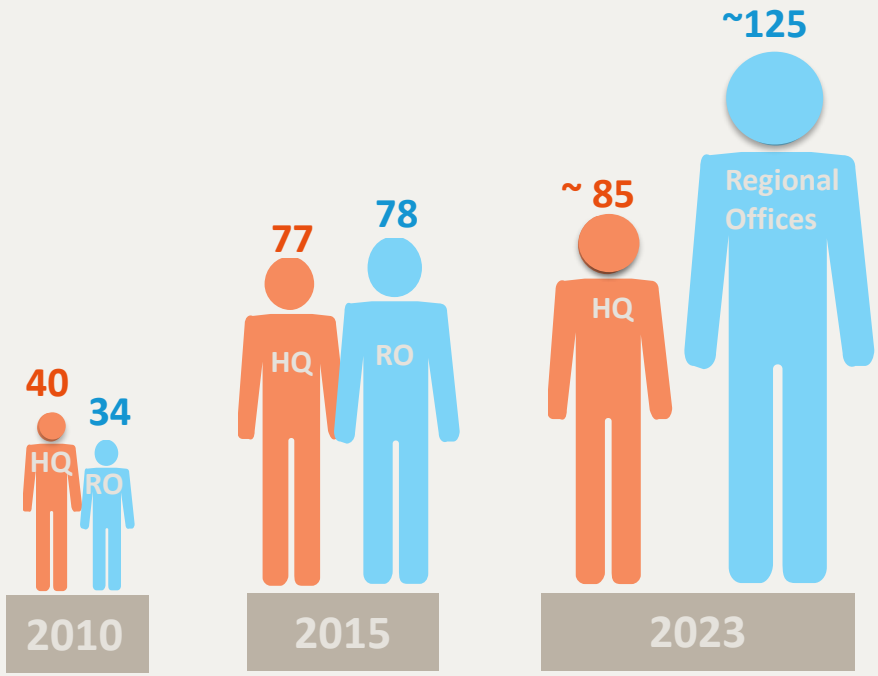
EUR 400M secured out of EUR 650M to deliver 16-18 treatments by 2023



Diversification of donors

- 50% public - 50% private
- max. 25% per donor

The people behind the work… in proximity to patients



x 4

DND*i* staff

Partner staff


Give neglected patients a voice.
They exist and must be heard.
Thank you.
DNDi
Drugs for Neglected Diseases initiative

Extra slides

Responding to the Needs of Patients Suffering from Neglected Diseases…

DNDi's PRIORITY:
Neglected Patients

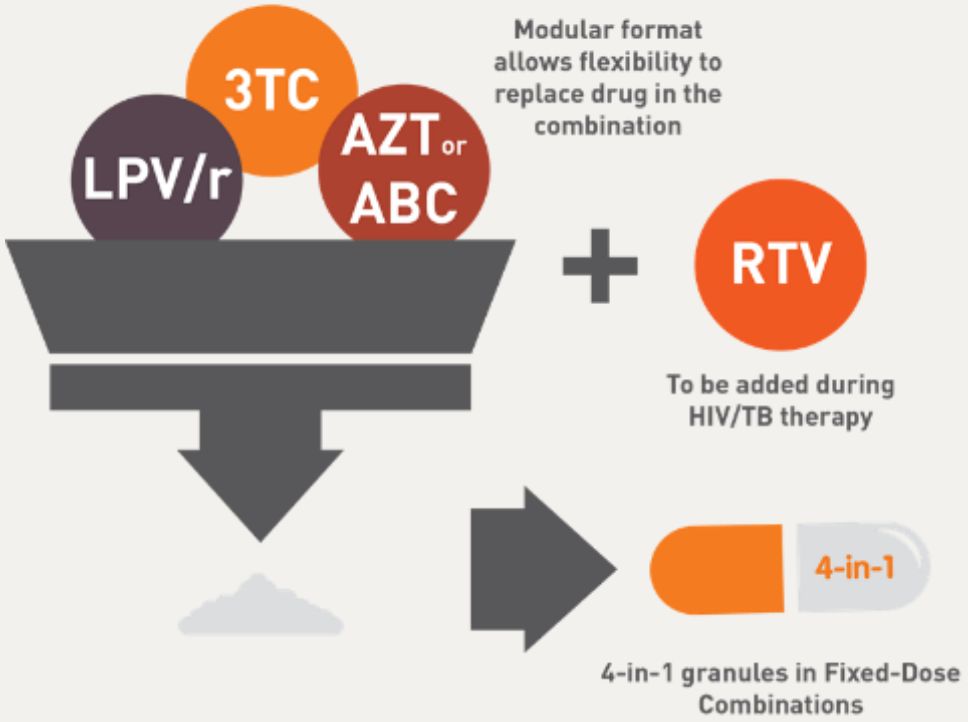
…from Bench to Bedside

For each disease, a Target Product Profile to guide all decisions (example of paediatric HIV)

IDEAL CHARACTERISTICS (TPP)

- 4 ARVs in one
- Simple to open and use with water, milk, food
- Good taste
- No fridge needed
- Suitable for infants (<2 months - 3 years)
- TB-treatment compatible
- Affordable for governments

PROCESS



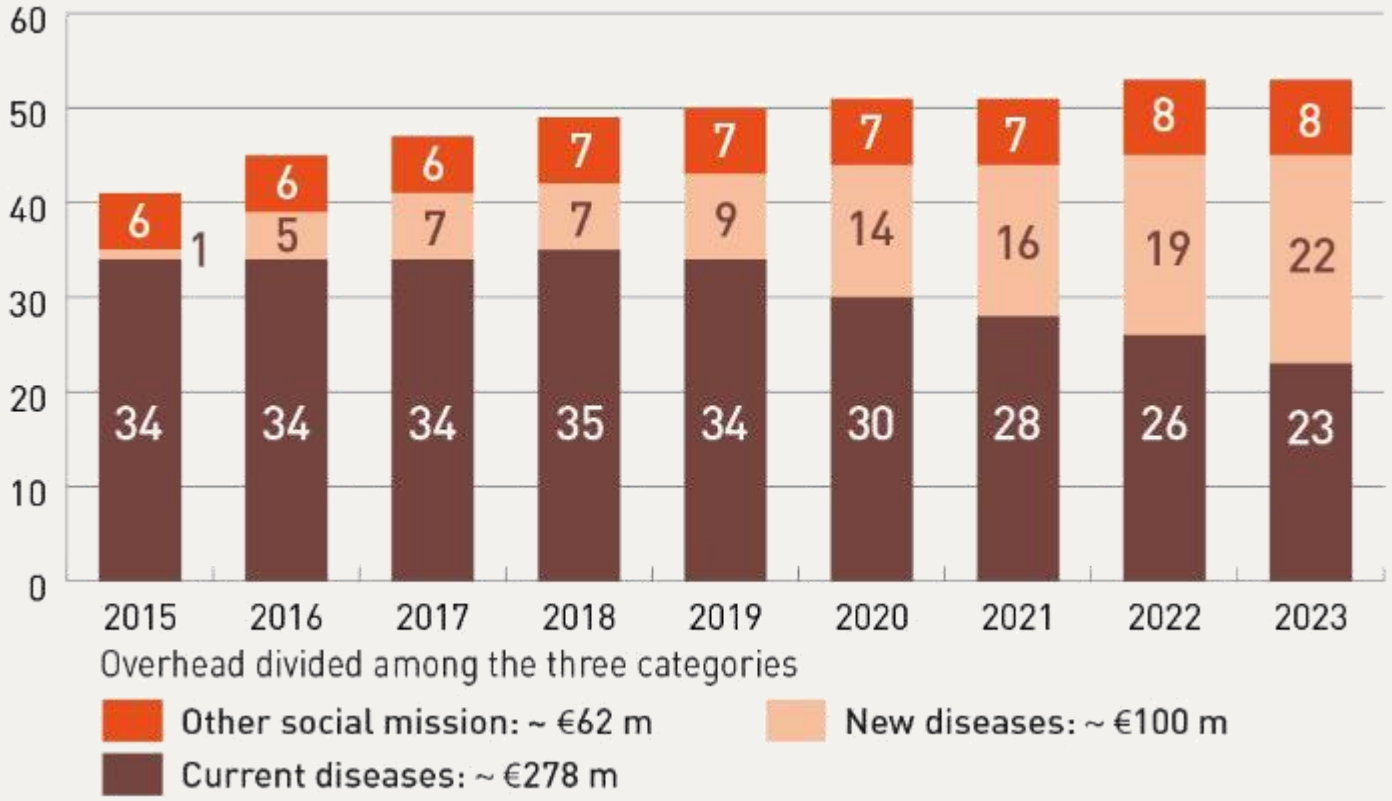
DND*i* Business Plan 2015-2023

A dynamic approach to address patient needs

Pipeline focus can quickly be adapted to:

- stay aligned with changes in the environment
- rapidly respond to urgent patient needs
- address specific regional needs

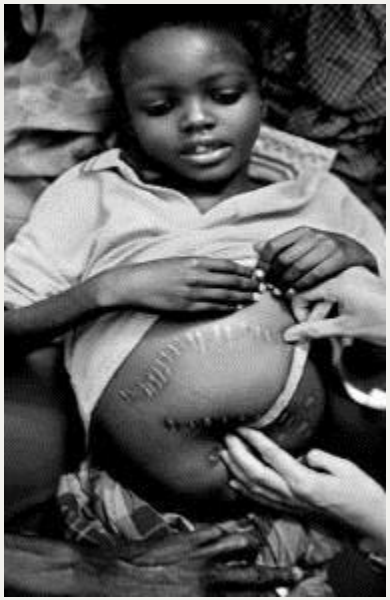
Growth is controlled as new diseases come on board

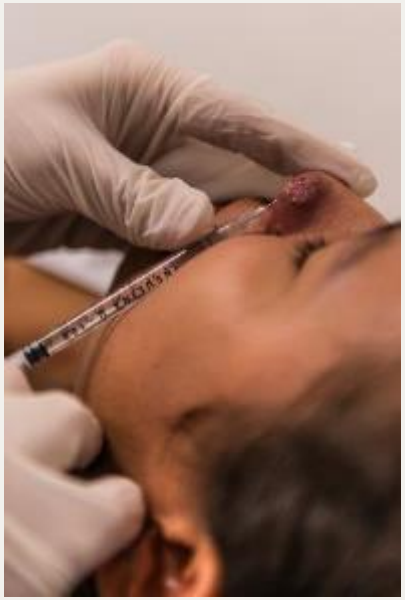


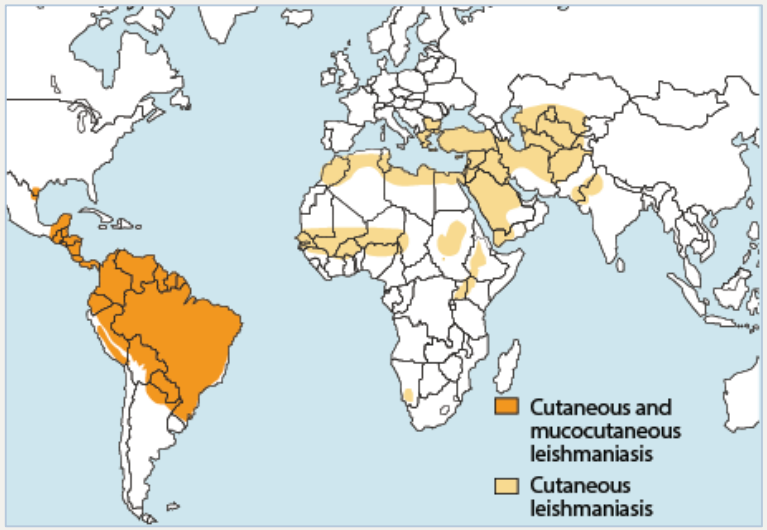
Budget projections EUR 48-50 million per year.

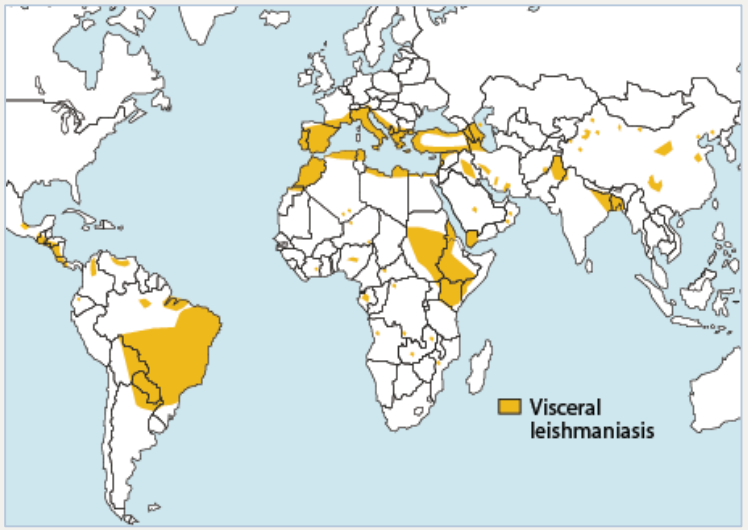
Leishmaniasis: 350 million people living at risk

- **200,000 – 400,000** new cases of visceral leishmaniasis each year
- **700,000 – 1,300,000** new cases of cutaneous leishmaniasis each year
- About **48,000** deaths due to visceral leishmaniasis in 2012
- **3,373,599 DALYs**
- A lack of surveillance systems and frequency of disease in **remote areas and marginalized population** means that it is **difficult to estimate the true incidence of leishmaniasis and the case-fatality of visceral leishmaniasis**.
- Present IN 4 CONTINENTS









Chagas landscape/ DND*i* activities 2016