

# New Drugs for Neglected Diseases

## New Hope for Forgotten Patients



An Overview of DNDi

Dr. Florence Camus-Bablon  
Senior Access Advisor

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**DNDi**

Drugs for Neglected Diseases *initiative*

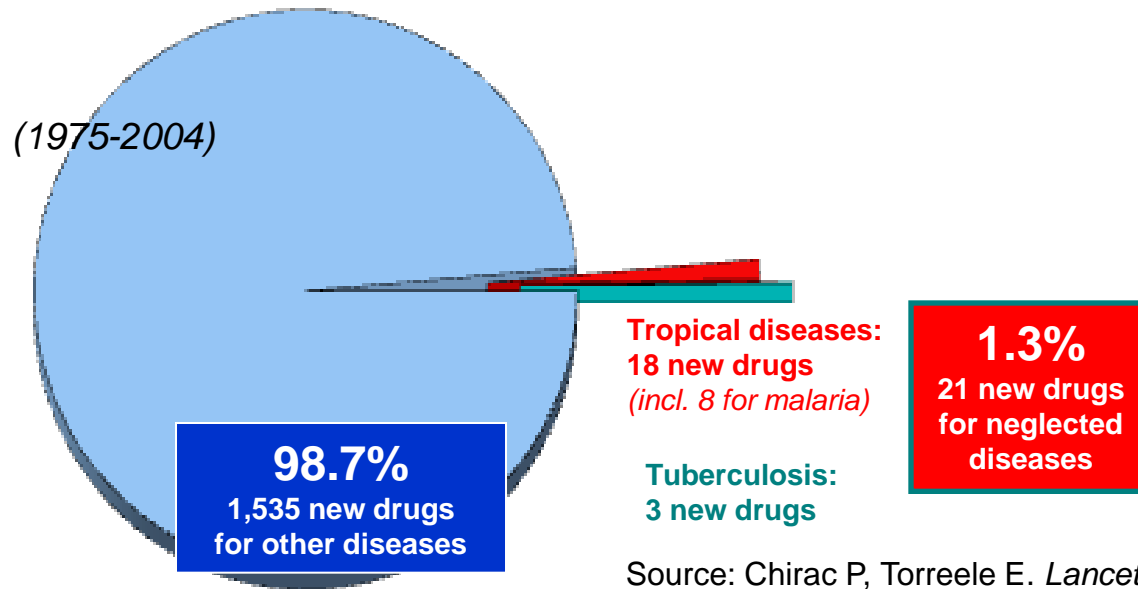
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# A Fatal Imbalance

Tropical diseases (including malaria) and tuberculosis account for:

- 12% of the global disease burden
- Only 1.3% of new drugs developed



Tropical diseases:  
18 new drugs  
(incl. 8 for malaria)

Tuberculosis:  
3 new drugs

**1.3%**  
21 new drugs  
for neglected  
diseases

Source: Chirac P, Torreele E. *Lancet*. 2006 May 12; 1560-1561.

# Responding to the Needs of Patients Suffering from Neglected Diseases...



Malaria



Visceral Leishmaniasis (VL)



Sleeping Sickness (HAT)



Chagas Disease

# DNDi: An innovative R&D model

- Non-profit drug research & development (R&D) organization founded in 2003
- Address the needs of the most neglected patients
- Harness resources from public institutions, private industry and philanthropic entities

## ● 7 Founding Partners

- Indian Council for Medical Research (ICMR)
- Kenya Medical Research Institute (KEMRI)
- Malaysian MOH
- Oswaldo Cruz Foundation Brazil
- Medecins Sans Frontieres (MSF)
- Institut Pasteur France
- WHO/TDR (permanent observer)



# Vision

A collaborative  
Patients' needs-driven  
Virtual  
Non-profit drug R&D organisation  
To develop new treatments  
Against the most neglected  
Communicable Diseases





# DNDi objectives: A patient & country-needs driven *initiative*

To develop and deliver 6-8 new treatments for NTD, based upon needs identified by endemic country stakeholders.

With country stakeholders, to support recommendation and implementation of these new treatments to facilitate equitable access.



# DNDi Portfolio-Building Model

- Existing chemical libraries
- New lead compounds

**Long-term projects**

- New formulations (fixed-dose combinations)
- New indications of existing drugs

**Medium-term projects**

- Completing registration dossier
- Geographical extension

**Short-term projects**



# Project Portfolio – End of 2010



## Discovery Activities

- Compound mining
- Chemical classes
- Target-based
- Screening

HAT LO Consortium  
 • *Scynexis*  
 • *Pace Univ.*

VL LO Consortium  
 • *Advinus*  
 • *CDRI*

Chagas LO Consortium  
 • *CDCO*  
 • *Epichem*  
 • *Murdoch Univ*  
 • *FUOP*

**a robust pipeline**

## Major Collaborators

- Sources for hit and lead compounds:  
*GSK, Anacor, Merck, Pfizer, Novartis (GNF, NITD), GATB,...*
- Screening Resources:  
*Eskitis, Institut Pasteur Korea, Univ. Dundee,...*
- Reference screening centres:  
*LSHTM, Swiss Tropical Institute, University of Antwerp*

Nitroimidazole backup (HAT)

Oxaborole (HAT)

Alternative formulations of Amphotericin B (VL)

Nitroimidazole (VL)

Drug combination (Chagas)

K777 (Chagas)

*Exploratory*

Fexinidazole (HAT)

Combination therapy (VL in Asia)

Combination therapy (VL in Africa)  
 • AmBisome®  
 • Miltefosine

Combination therapy (VL in Latin America)

Paediatric benznidazole (Chagas)

Azoles E1224 (Chagas)

*Exploratory*

**ASAQ**

(Malaria)  
 Fixed-Dose Artesunate/ Amodiaquine

**ASMQ**

(Malaria)  
 Fixed-Dose Artesunate/ Mefloquine

**NECT**

Nifurtimox - Eflornithine  
 Co-Administration  
 Stage 2 HAT

**VL Combi. Therapy**  
 Africa - SSG/PM

**6 to 8 new treatments by 2014**



# Access: DNDi Guiding Principles

Driven by goals to :

1. Facilitate equitable access to new treatments
2. Transition, in long term, new treatments to natural implementers, i.e., M of H, NCP, WHO, NGOs, medical practitioners

**Access strategy:**

**Pragmatic**

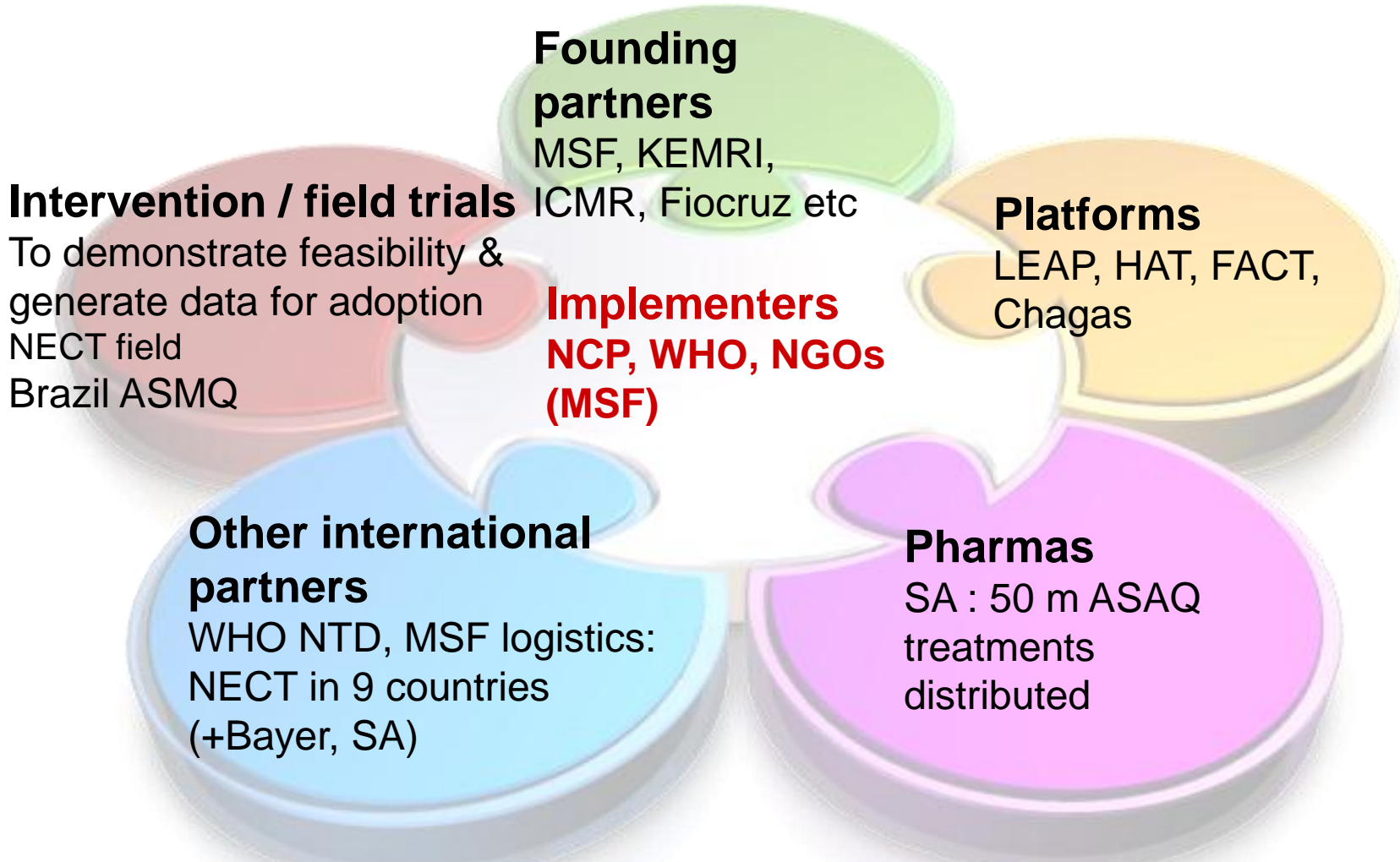
**Focused on most pressing “actionable” access barriers**

**Within DNDi expertise / mandate**

**To facilitate rapid implementation and relevant use**

**For max health impact**

# DNDi facilitates access via 5 main mechanisms: early involvement of partners



**Supporting advocacy to international audiences & endemic countries**

# RD Platforms / Networks

## Involved in development from the start

**Aim:** To strengthen clinical research capacity & Assist GCP clinical development for specific diseases in endemic areas, i.e. HAT in Central Africa, VL in East Africa, Chagas in LA, FACT.

**Include:** Endemic region academics, NCP / MOH, regulatory officials, NGOs / MSF, WHO / Coordinated by DNDi.

**Mandate:** To evaluate, validate and facilitate registration & adoption of new treatments.

**FACT advisory group**



# 7-Year Results

- **2 new malaria treatments: ASAQ and ASMQ FDC**
- **1 new sleeping sickness combination: NECT**
- **1 new visceral leishmaniasis combination for Africa: SSG/PM**
- **Largest pipeline** ever for the kinetoplastid diseases
- Clinical research platforms
- On track to deliver new treatments per business plan



By working together in a creative way,  
we can bring innovation to neglected  
patients!



[www.dndi.org](http://www.dndi.org)

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