

New Drugs for Neglected Diseases

New Hope for Forgotten Patients



An Overview of DNDi

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DNDi

Drugs for Neglected Diseases *initiative*

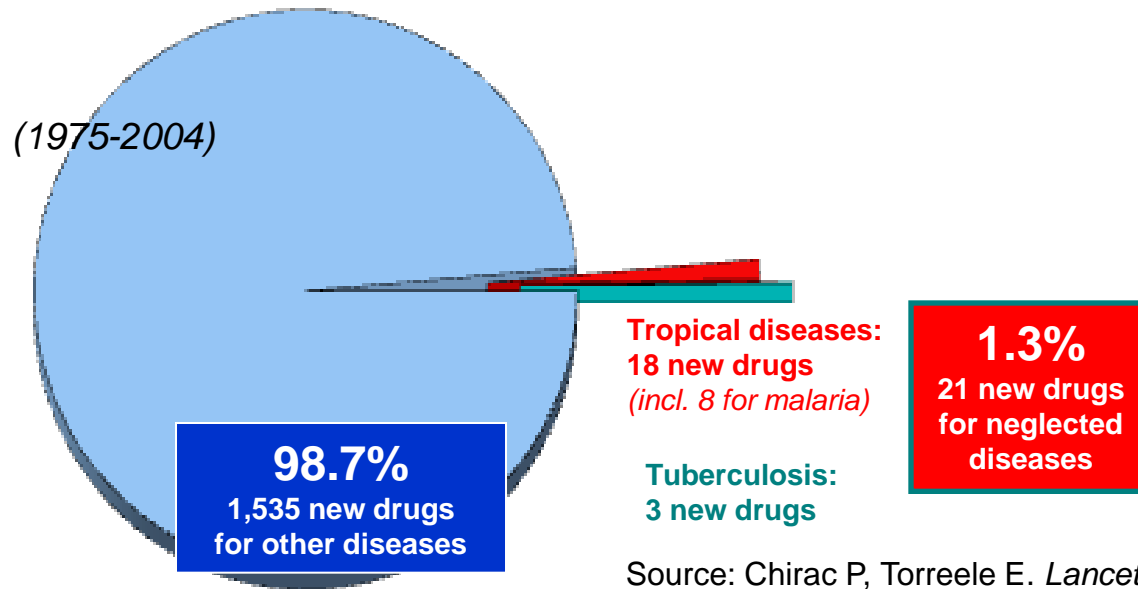
DNDi

Drugs for Neglected Diseases *initiative*

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

- 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

- 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*

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

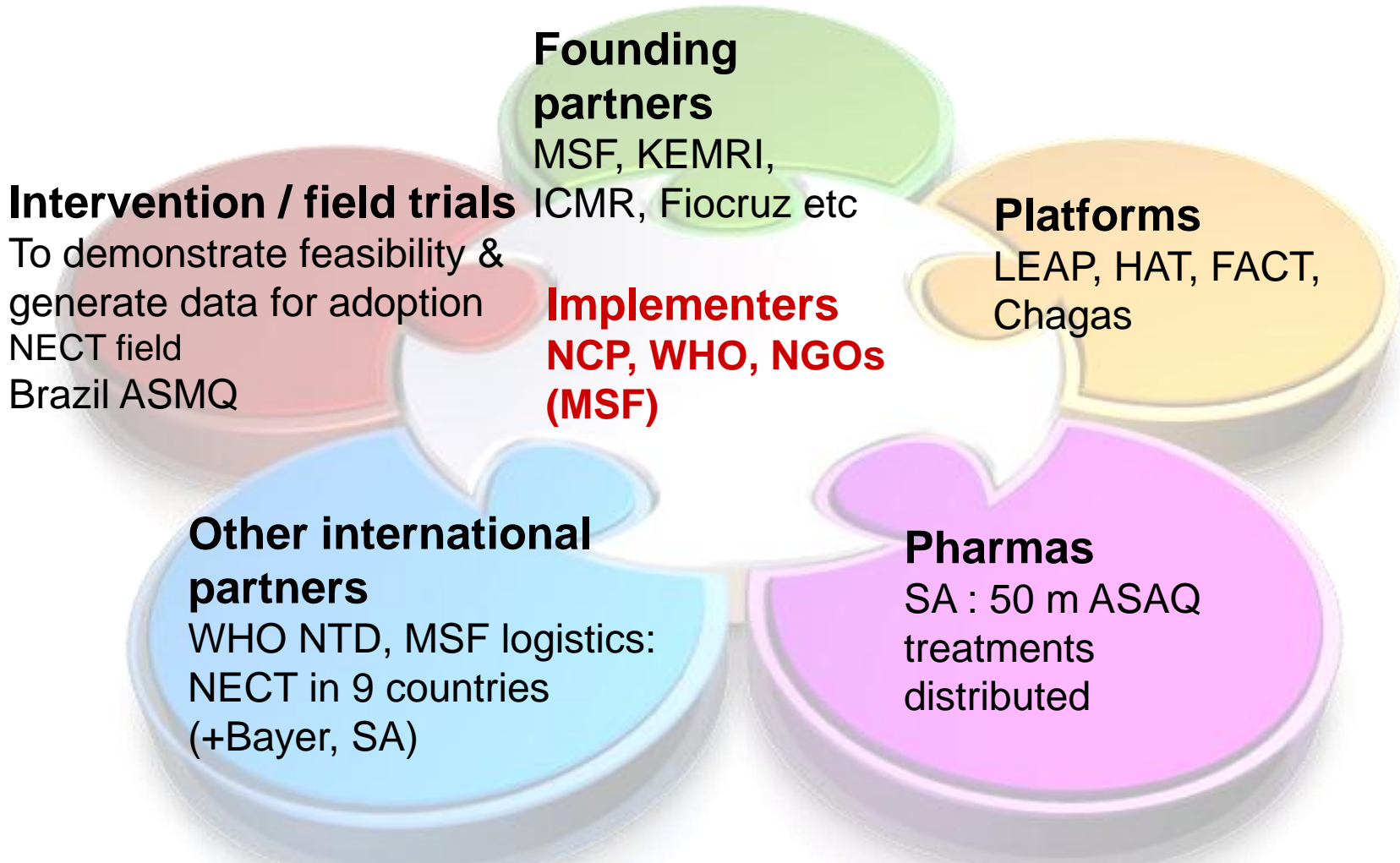
Focused on most pressing “actionable” access barriers

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

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