New Drugs for Neglected Diseases
New Hope for Forgotten Patients

An Overview of DNDi

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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

(1975-2004)

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

1.3%
21 new drugs for neglected diseases

Tuberculosis: 3 new drugs

98.7%
1,535 new drugs for other diseases

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)

7 worldwide offices
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

- Long-term projects
  - Existing chemical libraries
  - New lead compounds
- Medium-term projects
  - New formulations (fixed-dose combinations)
  - New indications of existing drugs
- Short-term projects
  - Completing registration dossier
  - Geographical extension

- Discovery
  - S
  - LS
  - LO

- Preclinical
- Clinical
- Access to Patients
**Project Portfolio – End of 2010**

**Discovery**
- Discovery Activities
  - Compound mining
  - Chemical classes
  - Target-based
  - Screening

**Pre-clinical**
- Nitroimidazole backup (HAT)
- Oxaborole (HAT)
- Alternative formulations of Amphotericin B (VL)
- Nitroimidazole (VL)
- Drug combination (Chagas)
  - K777 (Chagas)

**Clinical**
- Fexinidazole (HAT)
- Combination therapy (VL in Asia)
- Combination therapy (VL in Africa)
  - AmBisome®
  - Miltefosine
- Combination therapy (VL in Latin America)
- Pediatric benznidazole (Chagas)
- Azoles E1224 (Chagas)

**Available**
- 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

**Discovery Activities**
- Compound mining
- Chemical classes
- Target-based
- Screening

**Chagas LO Consortium**
- Scynexis
- Pace Univ.
- CDCO
- Epichem
- Murdoch Univ.
- FUOP

**VL LO Consortium**
- Advinus
- CDRI
- NECT
- Nifurtimox - Eflornithine Co-Administration Stage 2 HAT
- VL Combi. Therapy Africa - SSG/PM

**K777** (Chagas)

**a robust pipeline**

**6 to 8 new treatments by 2014**

24/01/11
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

**Intervention / field trials**
To demonstrate feasibility & generate data for adoption
NECT field
Brazil ASMQ

**Founding partners**
MSF, KEMRI, ICMR, Fiocruz etc

**Platforms**
LEAP, HAT, FACT, Chagas

**Implementers**
NCP, WHO, NGOs (MSF)

**Other international partners**
WHO NTD, MSF logistics:
NECT in 9 countries (+Bayer, SA)

**Pharmas**
SA : 50 m ASAQ treatments distributed

Supporting advocacy to international audiences & endemic countries

24/01/11
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