New Drugs for Neglected Diseases
New Hope for Forgotten Patients
An Overview of DNDi
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January 24, 2011
A Fatal Imbalance

Tropical diseases (including malaria) and tuberculosis account for:
- 12% of the global disease burden
- Only 1.3% of new drugs developed

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 Frontières (MSF)
- Institut Pasteur France
- WHO/TDR (permanent observer)

7 worldwide offices
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

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Best Science for the Most Neglected
Project Portfolio – End of 2010

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

**Pre-clinical**
- HAT LO
  - Scynexis
  - Pace Univ.
- VL LO
  - Advinus
  - CDRI
- Chagas LO
  - Advinus
  - Epichem
  - Murdoch Univ.
  - FUOP

**Clinical**
- Fexinidazole (HAT)
- Combination therapy (VL in Asia)
  - AmBisome®
  - Miltefosine
- Combination therapy (VL in Africa)
- Combination therapy (VL in Latin America)
- Paediatric 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

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

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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

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

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

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

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

**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!