

Partnering with PDPs for the Most Neglected Diseases



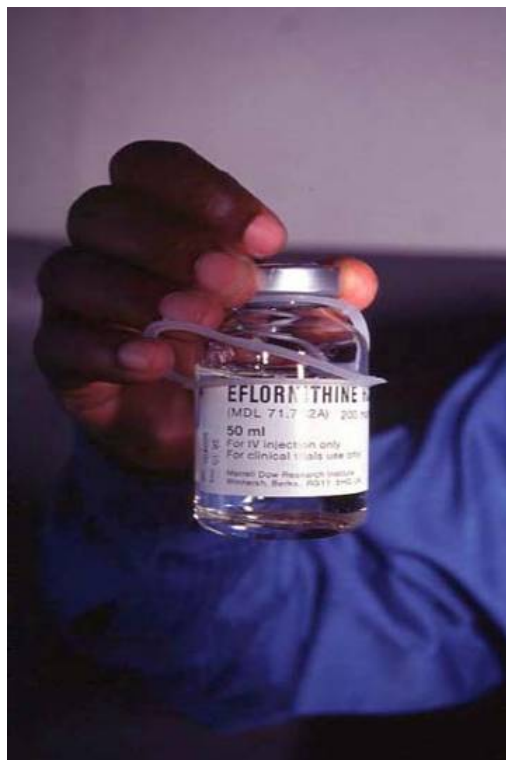
An Overview of DNDi & Its Partnerships

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Neglected Diseases: Current Treatment Limitations



Melarsoprol



Eflornithine

- Ineffective (resistance)
- Toxic
- Expensive
- Painful when delivered
- Difficult to use
- Not adapted to the field
- Not registered in endemic regions
- Restricted by patents

We Need Safe, Effective, Easy-to-Use Drugs

A New Model for Drug Development: DNDi

- Non-profit drug research & development (R&D) organization founded in 2003
- Addressing the needs of the most neglected patients
- Harnessing 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 support offices

Coordination team
Geneva + consultants

USA

DRC

Brazil

Kenya

India

Malaysia

Japan

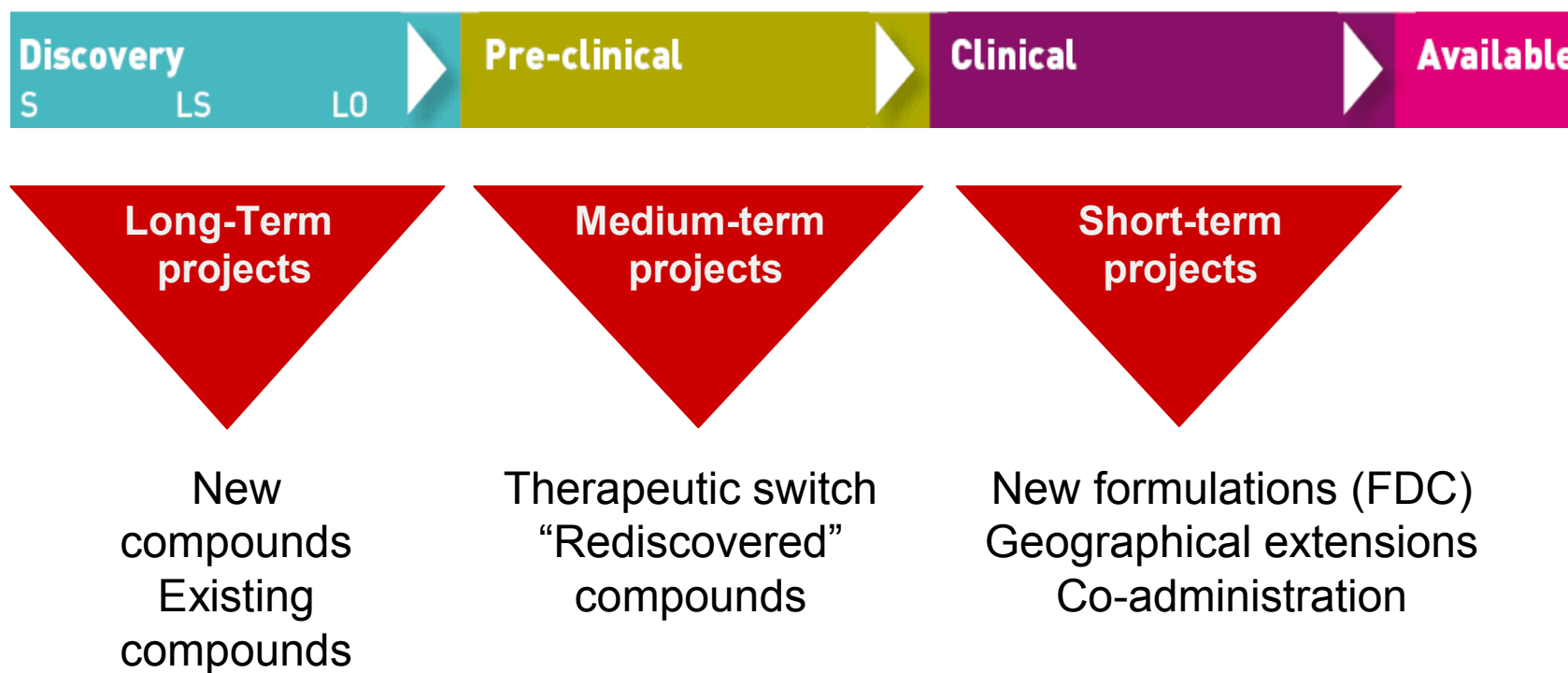


DNDi Portfolio-Building Model

Mission

- Deliver 6 - 8 new **treatments** by 2014 for tool-deficient neglected diseases
- Establish a robust pipeline for future needs

Strategy



3 New Treatments Developed So Far

2007

ASAQ (Malaria)
Fixed-Dose
Artesunate/
Amodiaquine



Partners

sanofi-aventis
(France)

2008

ASMQ (Malaria)
Fixed-Dose
Artesunate/
Mefloquine



Farmanguinhos
(Brazil)
Cipla
(India)

2009

NECT
Nifurtimox -
Eflornithine
Co-Administration
(HAT)

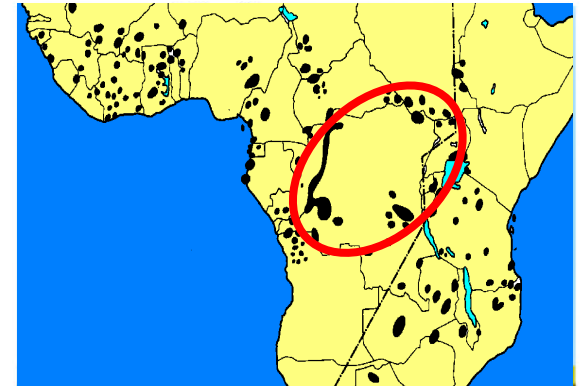


**National Control
Programs**
MSF
WHO

- Easy to Use
- Affordable
- Field-Adapted
- Non-Patented

Human African Trypanosomiasis (HAT) or Sleeping Sickness

- **60 million at risk** in sub-Saharan Africa
 - Primarily affects rural, remote populations
 - 3 major epidemics in 20th century – affecting up to 50% in affected villages
- Transmitted by the tsetse fly; caused by protozoal parasite *Trypanosoma brucei*
- **Difficult to diagnose**; most patients go undiagnosed until late stage of disease
 - Late stage: parasites have crossed blood-brain barrier (BBB)
- **Disease is fatal if untreated**



Existing HAT Treatments: Major Flaws

Melarsoprol: toxic yet widely used

- Arsenical drug
- 1st discovered in 1940s
- increasing resistance

1 in 20 patients die due to treatment.



Eflornithine: safe, effective, but....

- Difficult to transport

*Treatment kit containing 2 adult treatments weighs **37.6 kg** (80 lbs).*

- Difficult to administer

Treatment requires 56 slow, IV infusions every 6 hours for 14 days.



DNDi's HAT Strategy



Short-term projects

Improving existing treatments:

⇒ Nifurtimox-Eflornitine Combination Therapy (NECT)

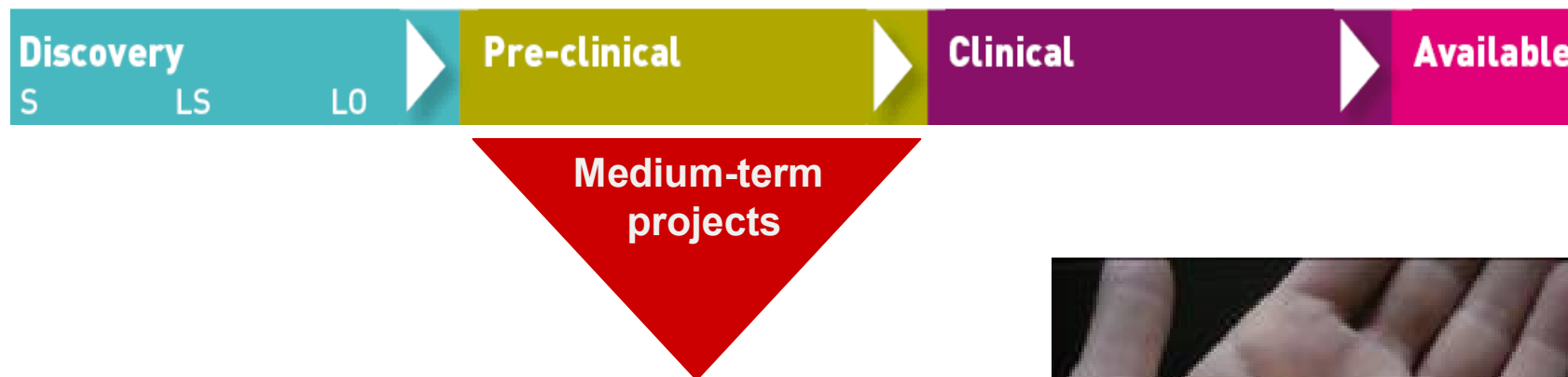
- Simplified treatment – less infusions, shorter course, safe & efficacious
- Added to WHO Essential Medicines List - May 2009

Key partners include:

- ⇒ sanofi-aventis (eflornitine)
- ⇒ Bayer (nifurtimox)
- ⇒ WHO
- ⇒ National Control Programs
- ⇒ MSF (Doctors Without Borders)
- ⇒ Swiss Tropical Institute



DNDi's HAT Strategy



Rediscovering existing compounds:

⇒ **Fexinidazole:**

- “rediscovered” by DNDi after extensive review of existing data
- entering into Ph I clinical studies in 2009



Key partners include:

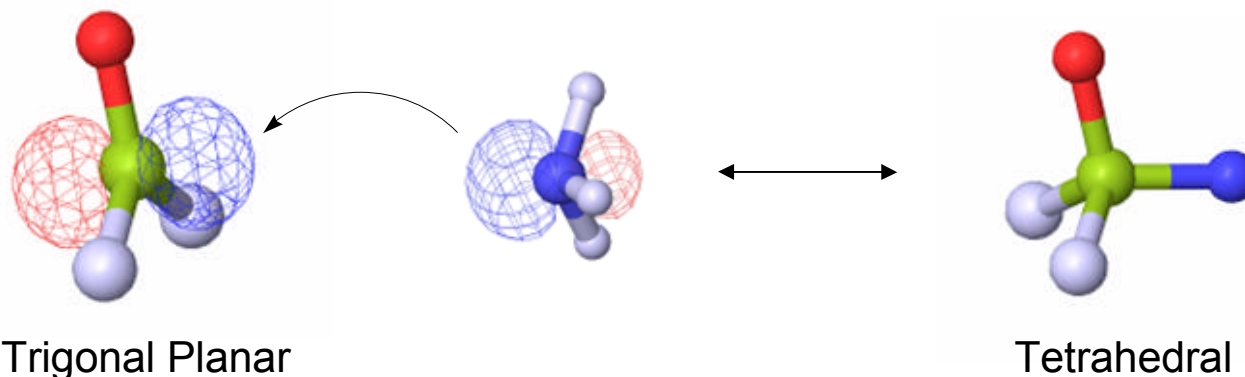
- ⇒ **Swiss Tropical Institute**, ...
- ⇒ **sanofi-aventis** will insure the manufacturing, registration and distribution of the product

DNDi's HAT Strategy



Long-Term projects

New drugs:



⇒ Oxaboroles:

- innovative chemistry with potent anti-protozoal activity
- clinical candidate expected by Q4 2009

Key partners include:

- **Anacor Pharmaceutical:** access to compounds and know-how
- **Scynexis:** lead optimization

DNDi Business Model

DNDi guidelines for partnerships:

- **To develop treatments free of any rights** (i.e. royalty-free worldwide exclusive license for neglected diseases) so as to **insure affordability** for patients
- **To provide treatments responding to the medical needs** of the patients in endemic countries (simple administration regimen, stability, pricing)
- **To actively contribute to the use of the product in endemic countries** through planned access campaigns, phase 4 monitoring, etc.
- **To make all information generated about the product during its development freely available** to the public and the scientific community

By working together in a creative way,
PDPs, large and small pharma, and the
public sector can bring innovation to
neglected patients!



www.dndi.org