

**Best Science for the Most Neglected**  
DNDi  
Drugs for Neglected Diseases Institute

## New tools for neglected diseases in Africa

*What are the best regulatory pathways?*



REGISTERING NEW DRUGS: THE AFRICAN CONTEXT

Dr Bernard Pécoul  
Executive Director  
Dr Nathalie Strub Wourgaft  
Clinical Development Director

**DNDi**  
Drugs for Neglected Diseases Institute




**Best Science for the Most Neglected**  
DNDi  
Drugs for Neglected Diseases Institute

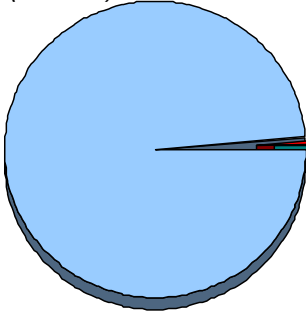
## A Fatal Imbalance

Tropical diseases (including malaria) and tuberculosis account for 12% of the global disease burden

- But only 1.3% of new drugs developed



(1975-2004)



Category	Number of New Drugs
Tropical diseases (incl. 8 for malaria)	18
Tuberculosis	3
<b>Total for neglected diseases</b>	<b>21</b>

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

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

## Product Development Partnerships (PDPs): Fill the Gaps in R&D for neglected diseases

Best Science for the Most Neglected

DNDi  
Drugs for Neglected Diseases Initiative

## Combined PDP pipeline includes 143 candidates

### 104 biopharmaceutical candidates in development...

Stage	Count	Percentage
Pre Clinical	59	57%
Phase I	14	14%
Phase II	12	12%
Phase III	10	10%
Registration	2	2%
Launched	6	6%

Legend: Drugs (orange), Vaccines (green), Microbicides (red)

### ... and 39 diagnostic & vector control candidates

#### Diagnostics

Stage	Count	Percentage
Feasibility	7	26%
Test Development	7	26%
Evaluation	6	22%
Demonstration	1	4%
Country Adoption	6	22%

Legend: CD4 (black), FIND (grey), IDRI (orange)

#### Vector control

Stage	Count
Early Stage	5
In Development	7

Legend: IVCC (yellow)

Notes: Includes products not funded by Gates Foundation.  
 Biopharmaceutical candidates in development include: IAVI, IPM, IVI, GATB, Aeras, MMV, MVI, MVP, PVS, DNDi, IOWH, PDVI, HHVI.  
 Source: PDPs

Slides source from: **BILL & MELINDA GATES foundation** & **BCG** THE BOSTON CONSULTING GROUP

Best Science for the Most Neglected

**Shared objectives**

- Develop treatments or vaccines **primarily** dedicated to patients from endemic countries with no or poor access to the best effective and safe treatment, or to prophylaxis options
- For some as DNDi, the mission goes beyond , i.e strenghtening existing research capacities and up to ensuring that implementation takes place

DNDi  
Drugs for Neglected Diseases Initiative

Best Science for the Most Neglected

**DNDi**


**A patient-needs driven & innovative R&D model**

- Deliver **6 - 8 new treatments by 2014** for sleeping sickness, Chagas disease, leishmaniasis and malaria
- Establish a **robust pipeline** for future needs
- Use and strengthen existing **capacity in disease-endemic countries**

● **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)

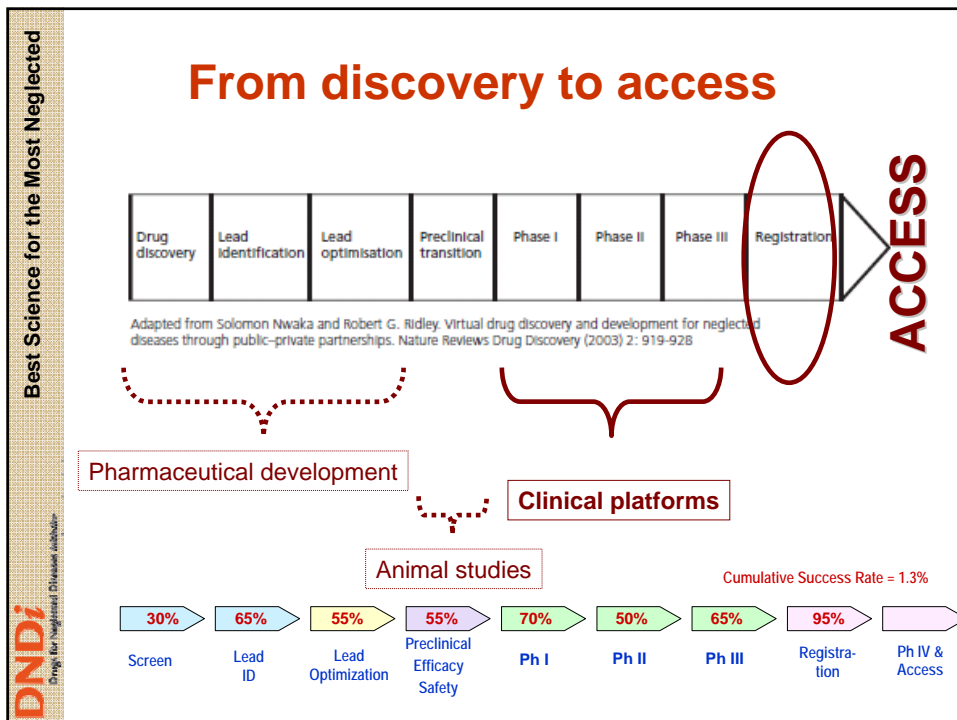
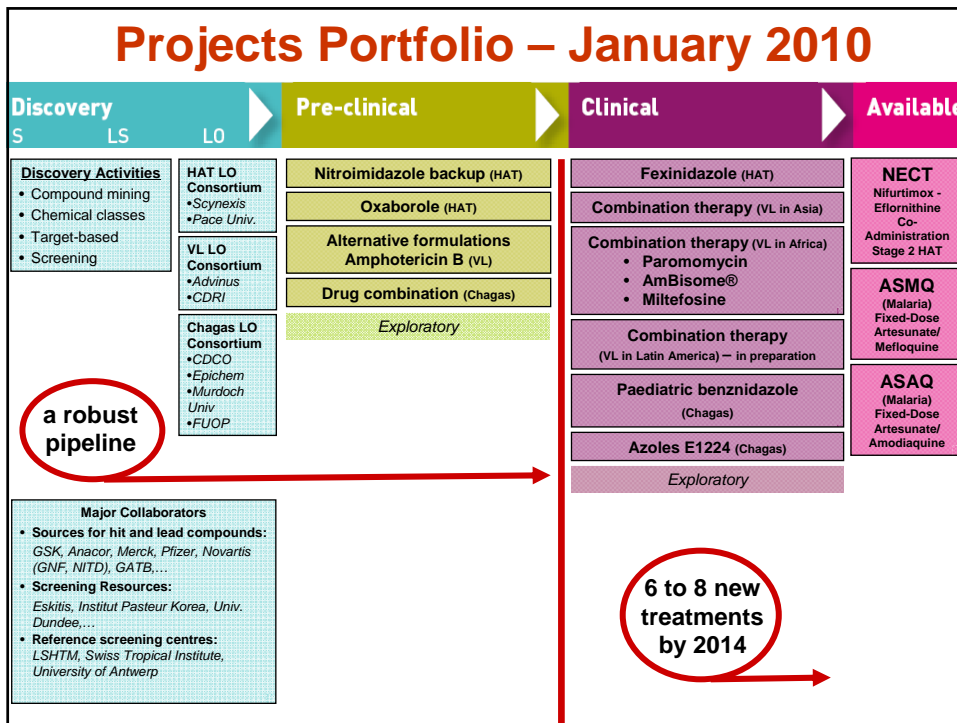
7 worldwide offices

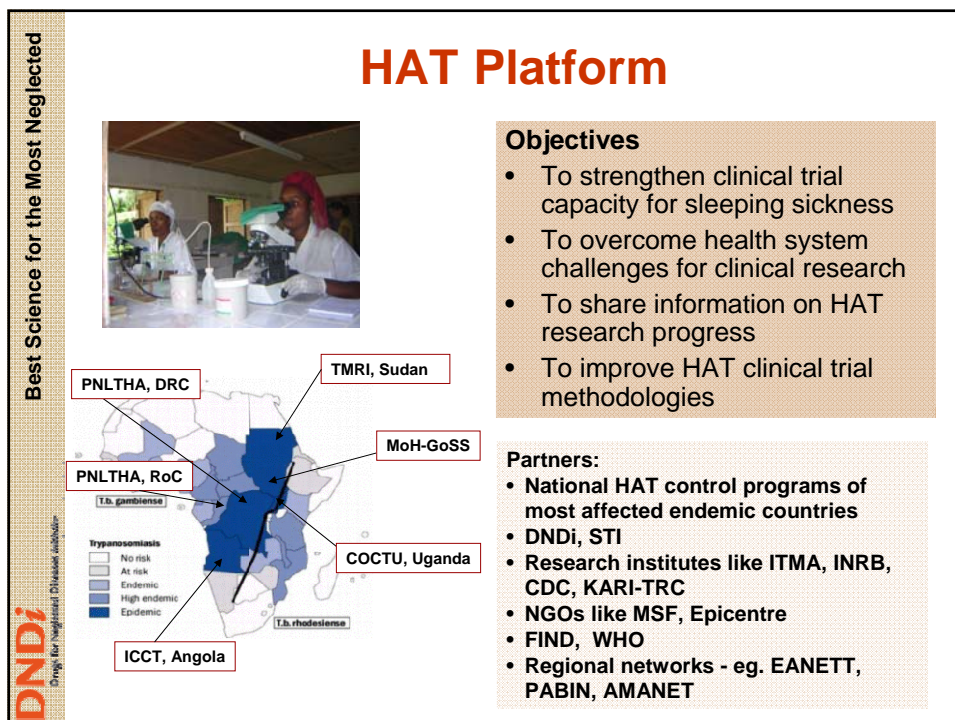
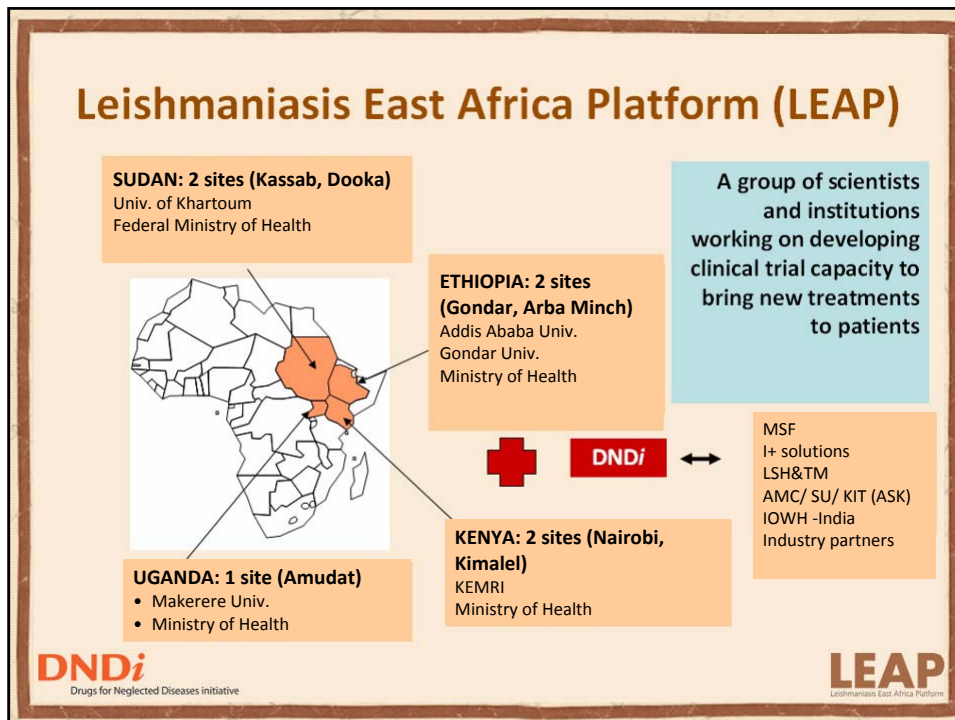


USA, Brazil, DRC, Kenya, India, Malaysia, Japan

Geneva Coordination Team + consultants

DNDi  
Drugs for Neglected Diseases Initiative





Best Science for the Most Neglected

**2 examples of successful registration**



ASAQ



NECT

DNDi  
Drugs for Neglected Diseases Initiative

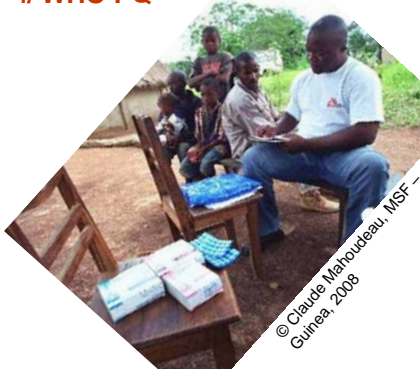
Best Science for the Most Neglected

**ASAQ**  
**Registered & manufactured in Africa**

**Innovative partnership with sanofi-aventis**

- A FDC of artesunate-amodiaquine
- Not patented, at cost in public market
- Manufactured in Marocco
- 2008: Prequalified by WHO
- Registered in 25 African countries
- 25 million distributed in 2009
- Ambitious field monitoring program (Pharmacovigilance)
- Technology transfer on going to an African manufacturer

**Registration Model:**  
1/ Scientific Opinion UK MHRA  
2/ Morocco registration  
3/ Geographical extension  
4/ WHO PQ



© Claude Mahriveau, MSF - Guinea, 2008

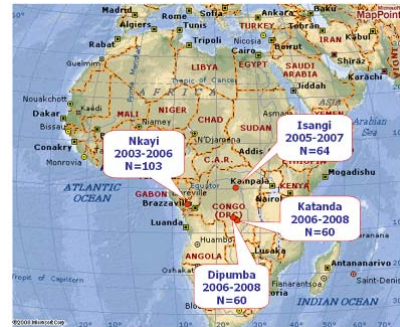
DNDi  
Drugs for Neglected Diseases Initiative

## NECT

### An improved option therapy developed in Africa & implemented in 4 endemic countries

**NECT (nifurtimox-eflornithine combination therapy) :  
A simplified, safe & effective treatment for stage 2 HAT**

- Pivotal Phase III trial in DRC NECT included into WHO Essential Medicines List (May 2009)
- NECT-FIELD study initiated in 6 clinical sites in DRC
- Implementation with WHO and national programmes



**Registration Model:**  
1/ EML for combination  
2/ Access via National programs/WHO

## But the landscape is evolving ...

- Up to now, the majority of treatments submitted for approval in Africa, were already approved drugs (EU, USA) or generic drugs
- New Chemical Entities (NCEs), vaccines, combination treatments now being developed to respond to the specific needs in developing countries
- African regulatory agencies will have to perform regulatory assessment of new treatments never evaluated before
- How can this be achieved the most efficiently?

**Best Science for the Most Neglected**

**Need for new pathways for registering innovative drugs for Africa**

- Increased participation of endemic countries within existing mechanisms
- Regional centres of excellence to support strengthening of African regulatory agencies



**DNDi**  
Drug for Neglected Diseases Initiative

**DNDi**  
Drug for Neglected Diseases Initiative

**The GEORGE INSTITUTE**  
for International Health

**Best Science for the Most Neglected**

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



**www.dndi.org**

**DNDi**  
Drug for Neglected Diseases Initiative