


Best Science for the Most Neglected

# New tools for neglected diseases in Africa


## What are the best regulatory pathways?



REGISTERING NEW DRUGS: THE AFRICAN CONTEXT

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Clinical Development Director

**DNDi**  
Drugs for Neglected Diseases Initiative

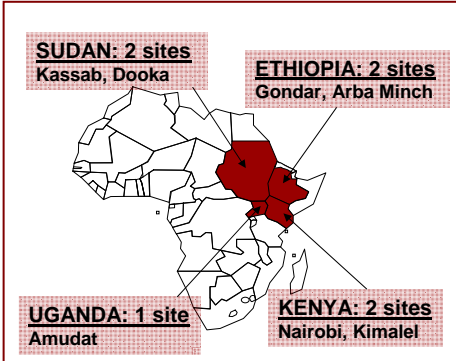


African Union NEPAD COHRED

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# Clinical Trials and future registration ?

## Visceral Leishmaniasis



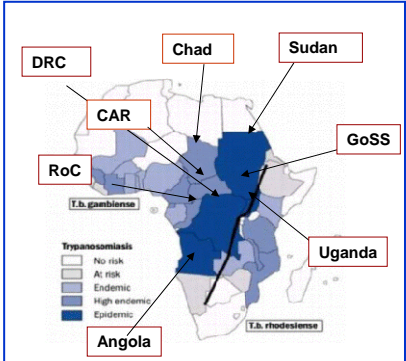
**SUDAN: 2 sites**  
Kassab, Dooka

**ETHIOPIA: 2 sites**  
Gondar, Arba Minch

**UGANDA: 1 site**  
Amudat

**KENYA: 2 sites**  
Nairobi, Kimala

## HAT

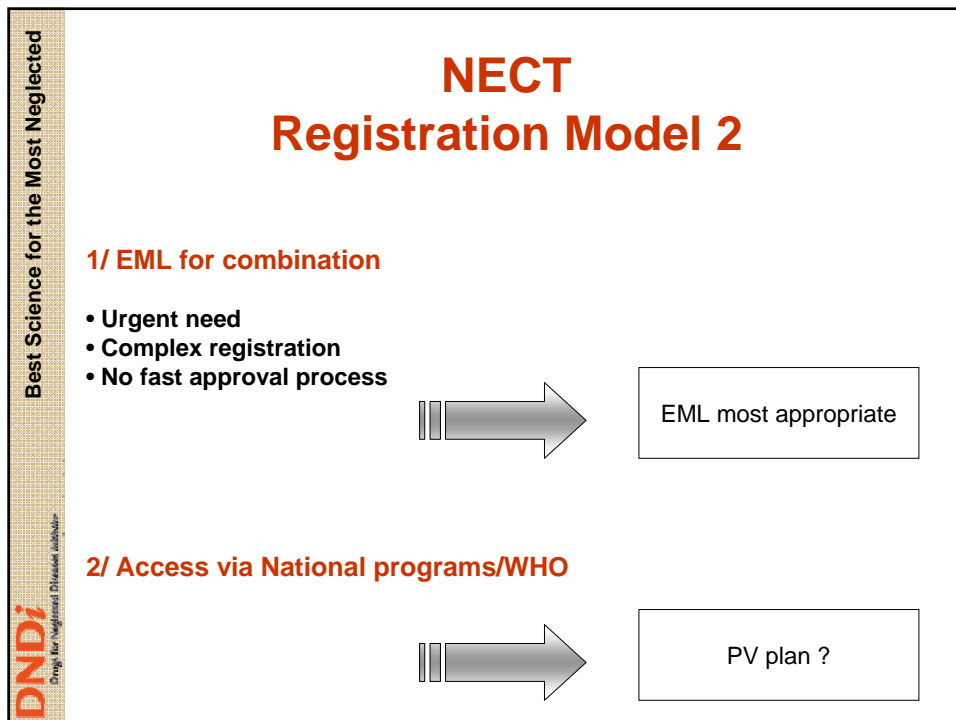
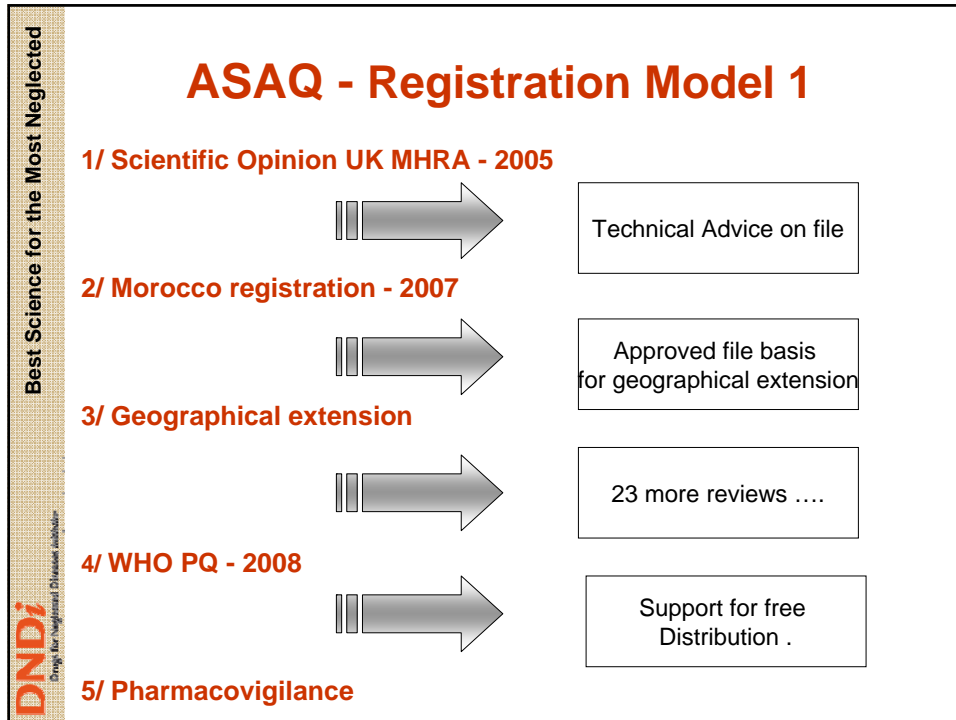


DRC Chad Sudan CAR GoSS RoC Uganda Angola

**Trypanosomiasis**

- No risk
- At risk
- Endemic
- High endemic
- Epidemic

T.b. gambiense T.b. rhodesiense



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**The case of human African trypanosomiasis (HAT)**

- Only endemic in sub-Saharan Africa;
  - number of cases (~10,000 reported; BUT 50 – 70,000 estimated)
  - few cases elsewhere (sporadically travelers)
- No market at all; few drugs available
- Limited knowledge on the disease – little experience in conducting clinical trials
  - Major logistic and capacity constraints
  - Access to patients (remote, scattered, insecurity)
- Clinical development methodology not well established
  - Diagnosis and staging, Test of Cure, surrogate markers, .

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**Fexinidazole: a first NCE for HAT**

- **Objective:**
  - **timely** registration in endemic countries for rapid access
  - of **safe, effective and quality** drug

 **Appropriate scientific benefit / risk assessment**

- **Different options exist**
  - First filing through stringent western regulatory authorities
    - What is the expertise of these RA in assessing benefit/risk for HAT?
  - Direct registration in endemic countries
    - Can we ensure timely expert quality assessment of the whole submission dossier ?
- **Preferred option:** joint evaluation at all stages
  - Build joint working group on methodological and clinical issues in the development of a drug for HAT with all stakeholders
  - Enhance, facilitate collaboration between western regulators and endemic countries' ones


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## Endemic countries of reference

- Start with country with highest prevalence?
- Start with country with highest regulatory resources
- All ?

 **Combination ideal**  
Requires mutual recognition process

**HAT NCE**  
**could represent a good model to test new mechanisms**

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## The George Institute Report methodology

- Expert Group with representatives from:
  - Regulatory bodies in sub-Saharan Africa, EMEA, FDA, ASEAN, WHO
- Interviews of other main stakeholders
  - PDPs, WHO, Pharma, Regulatory representatives from some sub-Saharan African countries, as well as EMEA, FDA
  - Draft version presented at DNDi Stakeholders meeting (Nairobi June 2009)
  - Comments integrated
  - Final review by Expert Group