Strengthening Capacity for Research and Development in Developing Countries: **The DND*i* Experience**

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At the ReAct Annual Conference,
Machakos, Kenya
19th September 2017

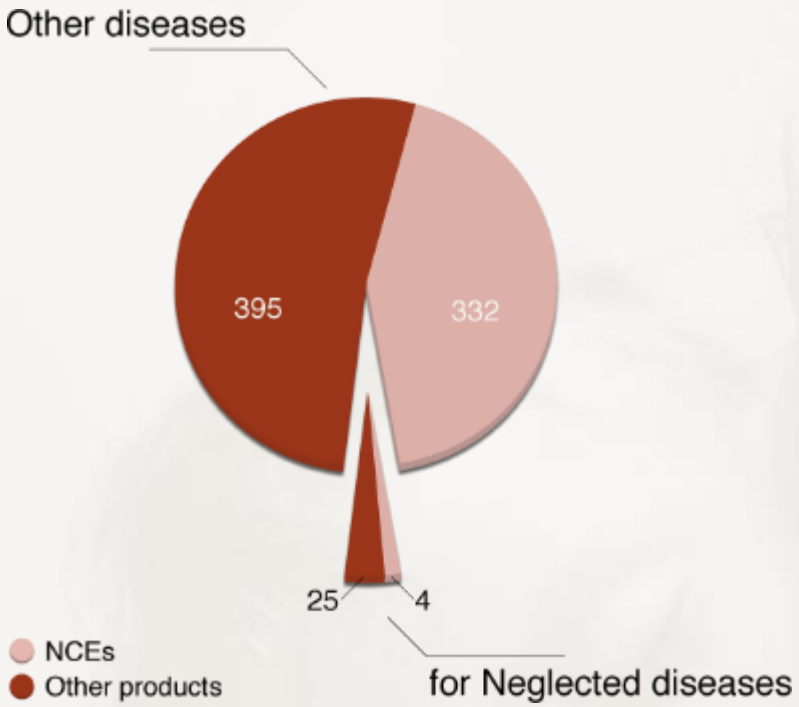
About DNDi

DNDi AFRICA
Drugs for Neglected Diseases *initiative*

Drugs for Neglected Diseases *initiative* (DND*i*) is a collaborative, patients' needs-driven, non-profit drug research and development (R&D) organization that is developing new treatments for neglected diseases

The fatal imbalance for NTD R&D exists,

756 products developed (excluding vaccines & biologicals) (2000-2011) *



* Source: Pedrique B et al. The drug and vaccine landscape for neglected diseases (2000-11): a systematic assessment. *Lancet Global Health*, Early Online Publication, 24 Oct 2013.

Until recently:

- R&D for neglected diseases was stagnant.
- The R&D landscape for neglected diseases was not evolving
- In endemic regions, there was little or no capacity for R&D for neglected diseases
- Pharma companies lacked interest in producing drugs for NTDs

One of the results of NTD R&D evolution was the not-for-profit PDPs e.g. DNDi, which aimed to fill R&D gaps and address the needs of neglected patients.

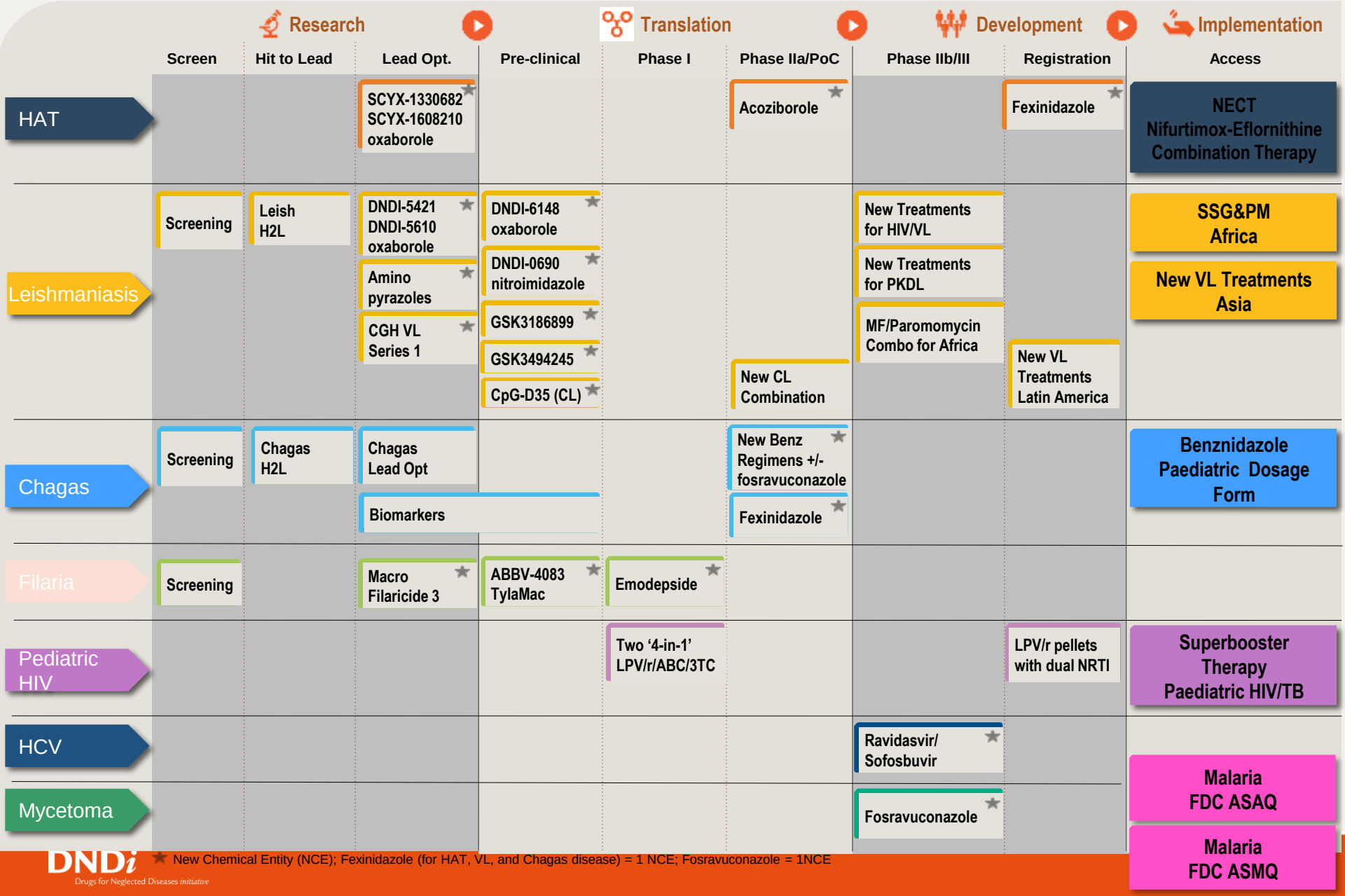
Responding to the Needs of Patients Suffering from Neglected Diseases…

DNDi's PRIORITY:
Neglected Patients

…from Bench to Bedside

DNDi R&D Portfolio June 2017

7 new treatments available and up to 16 new chemical entities in the pipeline



★ New Chemical Entity (NCE); Fexinidazole (for HAT, VL, and Chagas disease) = 1 NCE; Fosravuconazole = 1NCE

DNDi
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How we work

With Public
and Private Funding

DNDi

For underprivileged
patients

Conducts
research with:

Ministries
of Health

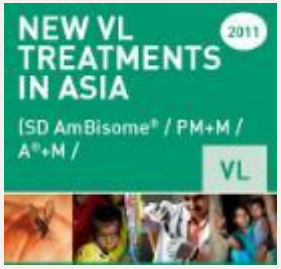
Public Research
Institutions

Biotechnology
& Pharmaceutical
Industries

To develop
and deliver treatments

Universities

7 new treatments delivered, recommended, implemented



- ✓ Easy to use
- ✓ Affordable
- ✓ Field-adapted
- ✓ Non-patented

- **30 projects, 9 diseases areas**
- **13 entirely new chemical entities (NCEs)**
- **Over 160 partnerships,** most in endemic countries
- **160 staff**, half in endemic countries & 700 people working on DNDi projects
- **EUR 475 million** raised equally from public and private sources
- **4 regional disease-specific clinical trial platforms/ networks** and several technology transfers

DND*i*'s success is only possible through innovative partnerships

PDPs
Int. Org. & NGOs
Universities & Research Institutes
Biotechs
CROs
Pharmaceutical companies

Over 160 partnerships worldwide

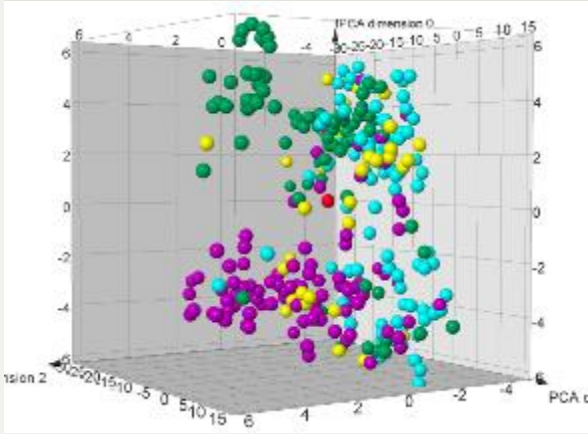
CRITERIA FOR SUCCESS

- ✓ Share the same vision
- ✓ Mutual understanding
- ✓ Involvement throughout the whole process

The NTD Drug Discovery Booster : Multilateral Partnership towards NCEs

- Objective: speed up the process and cut the cost of finding new treatments for leishmaniasis and Chagas disease
- Booster launched in 2015
- 3 Japanese pharma companies and AZ on board since the start
- Innovation: multilateral and cross-company comparative approach + iterative search
- Already 6 seed compounds submitted to the booster and > 1,600 analogues tested

SHIONOGI AstraZeneca Takeda Eisai Celgene MERCK abbvie DNDi Institut Pasteur Korea



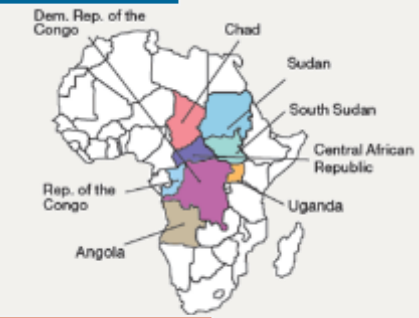
GHIT Fund
Global Health Innovative Technology Fund

Partnering and Building Capacity for Clinical Trials in Endemic Regions

VL

LEAP
LEISHMANIASIS
EAST AFRICA PLATFORM

HAT



CHAGAS

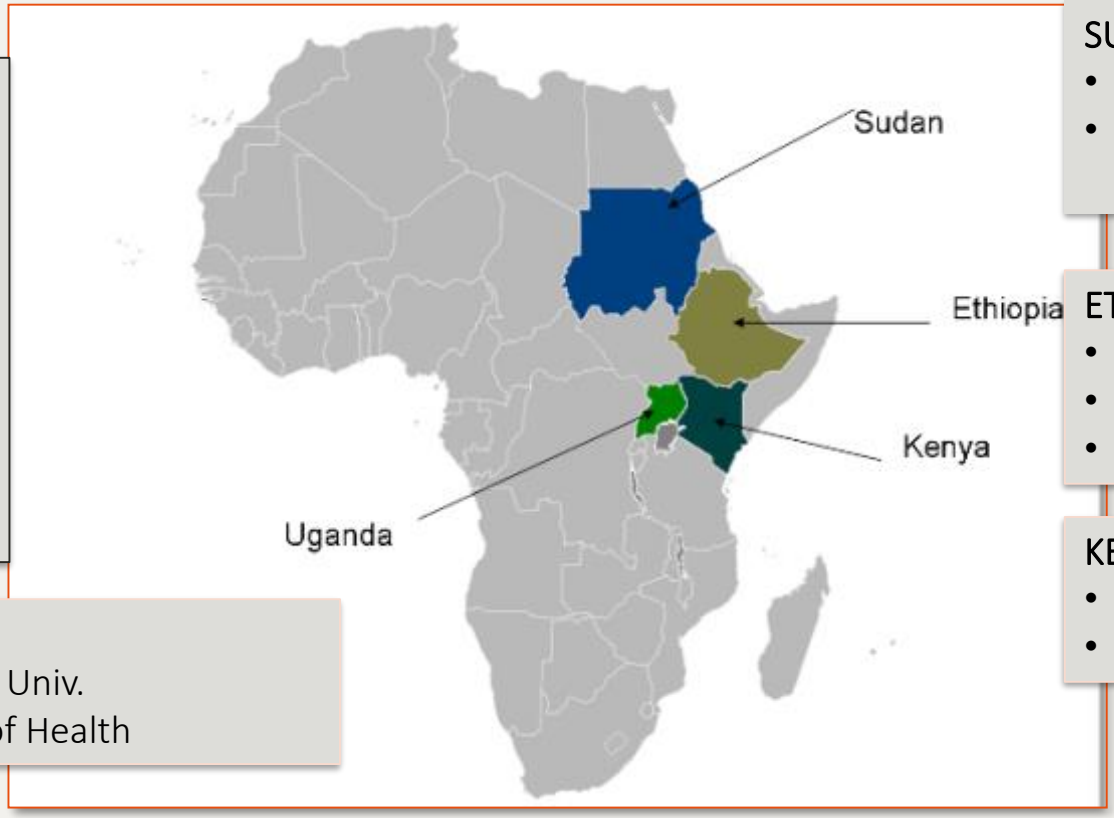
Major Role of Regional Disease Platforms:

- Strengthening local capacities
- Conducting clinical trials (Phase II/III studies)
- Facilitating registration
- Accelerating implementation of new treatments (Phase IV & pharmacovigilance studies)
- **Defining patients' needs and target product profile (TPP)**

Leishmaniasis East Africa Platform (LEAP)

LEAP - is a clinical research network that brings together experts from leishmaniasis endemic eastern African countries to facilitate clinical testing and improved access to better treatments for leishmaniasis in the region.

Study sites:
Gondar (Eth)
Arbaminch (Eth)
Abdurafi (Eth)
Kassab (Sudan)
Dooka (Sudan)
Um el kher (Sudan)
Amudat (Uganda)
Kimalel (Kenya)
Kacheliba (Kenya)



SUDAN:
- Univ. of Khartoum
- Federal Ministry of Health

ETHIOPIA:
- Addis Ababa Univ.
- Gondar Univ.
- Ministry of Health

KENYA:
- KEMRI
- Ministry of Health

UGANDA:
- Makerere Univ.
- Ministry of Health

LEAP Activities

LEAP
LEISHMANIASIS
EAST AFRICA PLATFORM

Capacity Building

Lab Upgrading

Training

Infrastructure

Research

High standard of research in endemic areas

Combination treatment delivered
More in the pipeline

Clinical research in difficult field conditions

LEAP Activities – Access/Advocacy

LEAP
LEISHMANIASIS
EAST AFRICA PLATFORM

Working with community leaders & governments

Media coverage and advocacy

LEAP meetings

Supporting treatment

GARDP:A virtual not-for-profit R&D organization

Focus:

Drug-resistant bacterial infections for which adequate treatment is not available

Global scope:

Low-, middle- and high-income countries

Joint initiative:

- World Health Organization
- Drugs for Neglected Diseases *initiative*

2023 Objectives

Develop 4 new treatments through:

- Improvement of existing antibiotics
- Development of new chemical entities

Build a robust pipeline of pre-clinical and clinical candidates

Actively support appropriate use of and access to new antibiotic treatments

GARDP Business Model

Funds raised from public and private sources and will be directed in two ways:

- Active R&D programmes driven, sponsored and directly executed by GARDP
- Equal partnerships on agreed principles to which GARDP brings appropriate funding, direction, and support

Operational models:

- Product development and management
  - Key role in R&D strategy, target product profiles (TPPs)
  - Point of entry (ideally post-IND) to patient delivery
- Sustainable access (affordable, equitable, stewardship)
- In house scientific and R&D capacity
  - clinical trials/networks, CMC, regional capacity
- Sponsor role

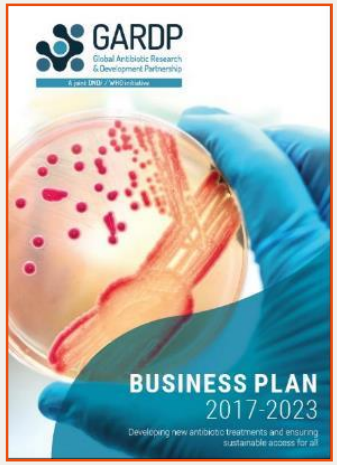
Programmes

Neonatal Sepsis: develop treatments for highly drug-resistant infections

Sexually-Transmitted Infections: develop a new treatment for drug-resistant gonorrhea and other STIs

Paediatric Antibiotics: optimize current and develop new antibiotics for children

Memory Recovery & Exploratory: revive old knowledge and abandoned projects; support early research



Funding boost to advance R&D programmes

- **GARDP launched in May 2016**, with seed funding that enabled:
  - **Governance** structure established, scientific and support teams hired
  - **Business Plan** sets out priorities and strategy
  - Scientific meetings held to develop **target product profiles for key diseases** and pathogens, with WHO guidance
  - **Partnerships** with industry, academia & government research agencies being built
- **Today, new boost of funding allows us to progress on R&D programmes**:
  - **Sexually-transmitted infections:** Partnership with US biotech (first deal for GARDP) announced July 2017, for development of a first-in-class drug candidate. Phase III trials in USA, South Africa, Thailand, Europe, possibly Latin America.
  - **Neonatal sepsis:** Launching observational, pre-clinical & clinical pharmacokinetic studies in Europe, Africa and Asia.
  - **Memory Recovery:** Over 70 'recovered' drug candidates reviewed, "REVIVE" hub for knowledge sharing to be unveiled tomorrow.

Lessons Learnt from DNDi's R&D Model(s)

- Partnerships are crucial for R&D; more can be done with less resources.
- Partnerships lead to ownership of R&D process and results at all levels; (pharma companies, Ministries of Health, communities etc)
- Patients needs in endemic countries must be put upfront, at the start of the innovation process to ensure seamless R&D process & delivery of new treatments
- Health R&D monitoring, coordination, and financing must be integrated for successful delivery of new treatments
- Regional platforms are crucial for the development of regional clinical trials capacities i.e. human resource and infrastructure targeting neglected diseases
- The gaps between R&D, Registration and access of much needed new treatments is reduced through the virtual models

Challenges

- Overcoming regulatory barriers - Different regulatory processes in the countries make it difficult to conduct studies
- Access to treatments - Transforming regulatory approval to country adoption and implementation;
- Ensuring sustainable production of treatments for neglected diseases;
- Policy environment - Securing an enabling policy environment including clear global norms on IP management;
- Ensuring sustainable financing for R&D towards NTDs
- Research Capacity - Lack of/different levels of capacity
- Tough terrain - Endemic regions are **difficult to work in**


Give neglected patients a voice. They exist and must be heard.
Thank you.
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