Strengthening Capacity for Research and Development in Developing Countries: The DNDi Experience

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At the ReAct Annual Conference, Machakos, Kenya
19th September 2017
Drugs for Neglected Diseases initiative (DNDi) 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) *

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

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How we work

With Public and Private Funding

Conducts research with:
- Biotechnology & Pharmaceutical Industries
- Universities
- Public Research Institutions
- Ministries of Health

For underprivileged patients

To develop and deliver treatments
7 new treatments delivered, recommended, implemented

- 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

✓ Easy to use
✓ Affordable
✓ Field-adapted
✓ Non-patented
DNDi’s success is only possible through innovative partnerships

CRITERIA FOR SUCCESS
✓ Share the same vision
✓ Mutual understanding
✓ Involvement throughout the whole process

Over 160 partnerships worldwide
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
Partnering and Building Capacity for Clinical Trials in Endemic Regions

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

**UGANDA:**
- Makerere Univ.
- Ministry of Health

**KENYA:**
- KEMRI
- Ministry of Health
LEAP Activities

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

- 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:
  - 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.