Linking Innovation and Access for Neglected Patients

Eric Stobbaerts, DNDi
ASTMH Nairobi 9th February 2016
Fatal imbalance still exists, an adapted R&D response is required

Among 756 products developed, only 4 NCE’s for NTD’s (excluding vaccines & biologicals) (2000-2011)*

=> Failure of the current biomedical research and development (R&D) system

What has changed?
Where are the gaps?
What must we do?
The R&D landscape for neglected patients has changed but large gaps still remain

1. R&D priorities do not sufficiently originate from low- and middle-income countries

2. Patients’ needs are not prioritized (e.g. Ebola, Zika, Mycetoma, etc.)

3. Innovation is not linked to equitable access even when there is commercial incentive to drive innovation (e.g. HCV)

4. Market incentives aligned with IP/exclusivity do not adequately address health needs in LMICs (e.g. AMR)

These are the fundamental challenges for the future of biomedical innovation.
DNDi’s New Business Plan: to deliver 16 to 18 treatments by 2023

- Influence the R&D landscape for neglected patients
  - Political leadership for needs-driven R&D
  - Creation of a global fund and mechanism
  - Evidence on alternative R&D models

- Develop treatments for people suffering from neglected diseases
  - Deliver 16-18 treatments
  - 3 new chemical entities (NCEs)
  - ~10 disease areas
  - Focus on access and measure impact

- Strengthen research capacity, led by Regional Offices
  - R&D platforms in disease-endemic countries
  - Regionally-driven initiatives
  - Patient access to treatments
  - Transfer of technology
DNDi’s success is only possible through innovative partnerships

CRITERIA FOR SUCCESS
• Share the same vision
• Mutual understanding
• Involvement throughout the whole process
DNDi Portfolio December 2015
6 new Treatments since 2003

New Chemical Entity (NCE); Fexinidazole (for HAT, VL, and Chagas disease) = 1 NCE
10 Years achievements: 6 New Treatments Developed

**Malaria**
- **ASAQ** (2007): Fixed-dose combination of artesunate + amodiaquine
  - Innovative partnership with Sanofi
  - Simple regimen: 1 or 2 tablets once a day for 3 days
  - Registered in 35 countries, of which 31 in Africa
  - WHO prequalified
  - WHO Essential Medicines List (adults and children)
  - 320 million treated in 31 African countries

- **ASMQ** (2008): Fixed-dose combination of artesunate + mefloquine
  - Developed by DNDi and Farmanguinhos/Fiocruz, Brazil
  - Simple and adapted regimen for children and adults
  - South-South technology transfer from Farmanguinhos to Cipla, India
  - WHO prequalified (Cipla)
  - WHO Essential Medicines List (adults and children)
  - 1.2 million treated in Latin America and Asia

- **NECT** (2009): Nifurtimox-eflornithine combination therapy
  - Partnership between DNDi, MSF, governments, pharmaceutical companies, and WHO
  - Approximately 96% of all stage 2 sleeping sickness patients in endemic countries treated with NECT (2013)
  - WHO Essential Medicines List (adults and children)
  - On essential medicines lists of 12 African countries (covering 98% of reported cases)
  - 13,000 treatments in Africa

**Visceral Leishmaniasis**
- **SSG&PM** (2010): Sodium stibogluconate & paromomycin combination therapy
  - Partnership between DNDi, the Leishmaniasis East Africa Platform (LEAP), national control programmes of Kenya, Sudan, Ethiopia, and Uganda, MSF, and WHO
  - Recommended by the WHO Expert Committee on the Control of Leishmaniasis for East Africa (2010)
  - National VL, guidelines of Sudan, South Sudan, Kenya, and Ethiopia
  - Paromomycin registered in Uganda (2011), in Kenya (2013), and underway in other East African countries
  - 25,000 treated in East Africa

- **NEW VL treatments in India** (2011): (SD AmBisome® / PM+M / M+A®)
  - Large-scale implementation programme with health authorities at state, national, and regional levels
  - High efficacy and good safety profiles
  - Field-adapted
  - Recommended by the WHO Expert Committee on the Control of Leishmaniasis (2010)
  - SD AmBisome® and PM+M recommended in revised Indian VL elimination roadmap

**Chagas Disease**
- **Benznidazole 12.5 mg** (2011): (Paediatric dosage form of benznidazole)
  - Partnership with LAFEPE, Brazil
  - Age-adapted, easy-to-use, and affordable treatment
  - Easily dispersible tablet for children under 2 years of age
  - Registered in Brazil in 2011
  - WHO Essential Medicines List
  - Agreement with Mundo Sano Foundation for second source (2013)
  - Only child-adapted dosage form
A Key Role for Regional Disease Platforms

- Defining patient needs and Target Product Profile (TPP)
- Strengthening local capacities
- Conducting clinical trials (Phase II/III studies)
- Facilitating Registration of new therapies
- Accelerating implementation of new therapies, ensure therapies reach patients
People behind the work… in proximity to patients

Partnerships are critical to access – DNDi continues to play a facilitating role

DNDi staff

Partner staff

2010

2014

2023

HQ

RO

~125

Regional Offices

X 4

315

276

447

~85

287

255

232
Teams in Asia
Teams in Africa
Our concern for Access: Keeping Patients at the Core of Innovation

Serafino Moreno
Colombian miner
Access at DNDi has taken many shapes and formats over the years...

Access Objectives

- Facilitate maximum impact via appropriate use of treatments
- Assure effective transition of treatments to relevant access partners and implementers, including national control programs, WHO and NGOs
- Further demonstrate success to support the DNDi model
- Target disease control strategies
Linking Innovation with Access is difficult and requires many stakeholders working on collective activities.

Some activities have been completed for certain disease areas. DNDi will create a more systematic approach to ensure Access at multiple levels across the organization.
Access Framework*

**Availability**
- Regulatory
- Manufacturing
- Forecasting
- Procurement
- Distribution
- Delivery

**Adoption**
- Global
- National
- Provider
- Patient

**Affordability**
- Government
- NGO
- Patient

The example of Chagas Disease in Latin America

**Its impact**

- Approximately **5.7 million** infected
- **10,000** deaths per year
- **528,000** DALYs

In Brazil alone, losses of over US$ **1.3 billion** in wages and industrial productivity were due to workers with Chagas disease.

Endemic in **21 countries in Latin America**, Chagas kills more people in the region than any other parasite-born disease, including malaria. Patient numbers are growing in developed countries.

Two old treatments available - Few Health Technologies

Only **1% are currently under treatment** - No access
Amazonas, Brazil
João Roberto Ripper, 2010
The example of Chagas Disease in Latin America
Access Barriers: What we have achieved so far…

- Increasing medical consensus
- More evidences and data
- Two sources of Production of Benz
- PAHO DNDi+MSF Demand-Forecasting
- Patients Federation + Global Coalition
- Piloting deploy. Projects (Col+Me +US)
Connecting the dots: break the cycle of neglect

Neglected cycle faced by Chagas patients
Collaboration for Access to Health Technologies

WHO

Public institutions in endemic countries

Pharma Industry and Biotechs

Patients Groups and Campaigns

Philantropy

New Funding Mechanisms

Governments

PPPs & PDPs

Public institutions in endemic countries
Linking Innovation to Access: Partnering in Access

- Re-Introduce the notion of **emergency response**: patients are dying!
- **Leadership and coordination** from the endemic countries
- Break the silence: Visibility; **Voice of the patients** and Campaigning
- Build **Collaborative models**: develop road maps (drug access strategies per disease)
- New **Organizational structures** established with the purpose of coordinating the availability, affordability, and adoption activities (define roles and responsibilities+ joint KPI’s)
- Moving beyond: **advocating to change the R&D Landscape**
Without political commitment and strong collaborations, we are bound to fail!
Amazonas, Brasil
João Roberto Ripper, 2010

ASANTE- OBRIGADO - GRACIAS

THANK YOU