



Alternative approach to R&D for emerging infectious diseases

Dr Bernard Pécoul, Executive Director

DNDi

Drugs for Neglected Diseases *initiative*

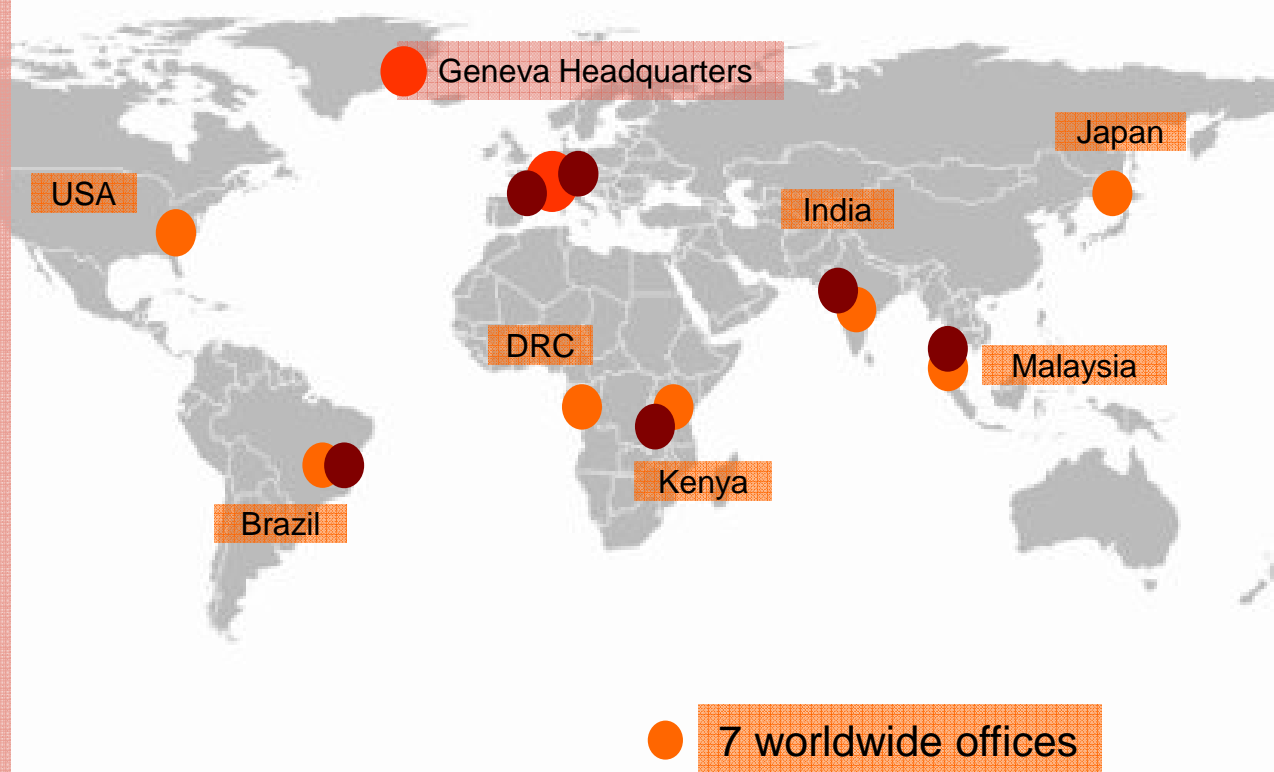
EU-CIS seminar, Annecy, September 2015

Drugs for Neglected Diseases initiative: Patient Needs-Driven & Innovative R&D Model

- ❑ Deliver **16 to 18 new treatments by 2023**
- ❑ Establish a **robust pipeline**
- ❑ Use and strengthen existing **capacity in disease-endemic countries**
- ❑ **Raise awareness** and advocate for increased **public leadership**

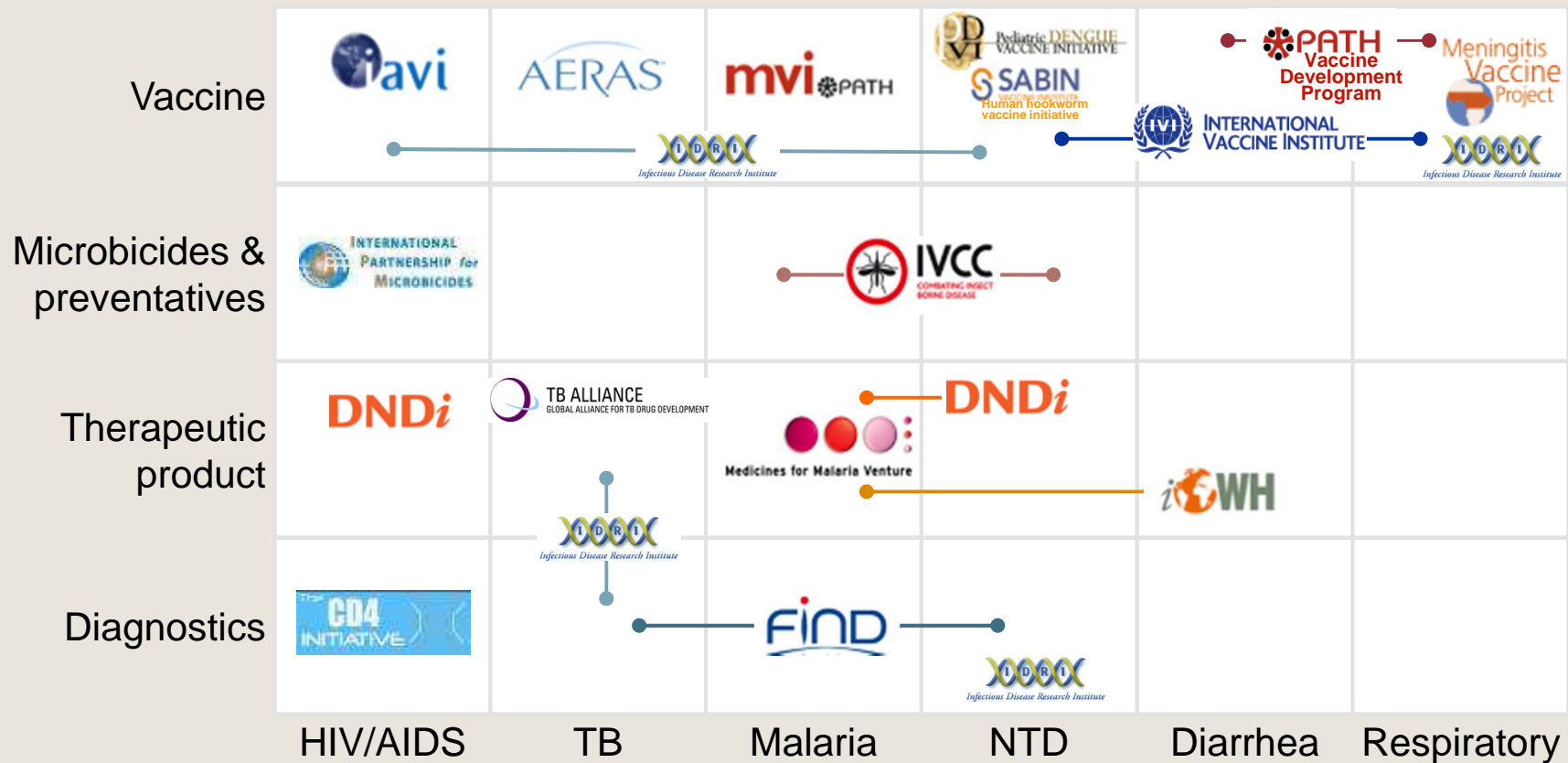
Founding Partners

- Indian Council for Medical Research (ICMR)
- Kenya Medical Research Institute (KEMRI)
- Malaysian MOH
- Oswaldo Cruz Foundation, Brazil
- Médecins Sans Frontières (MSF)
- Institut Pasteur France
- TDR (permanent observer)



Product Development Partnerships (PDPs): Filling the Gaps in Translational Research and Product Development

PDPs work across different diseases and modalities



Source:

BILL & MELINDA
GATES foundation

&

BCG
THE BOSTON CONSULTING GROUP

DNDi
Drugs for Neglected Diseases initiative

Partnerships: No One Can Do It Alone

A Global Network to Leverage Resources

Criteria for Success:

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

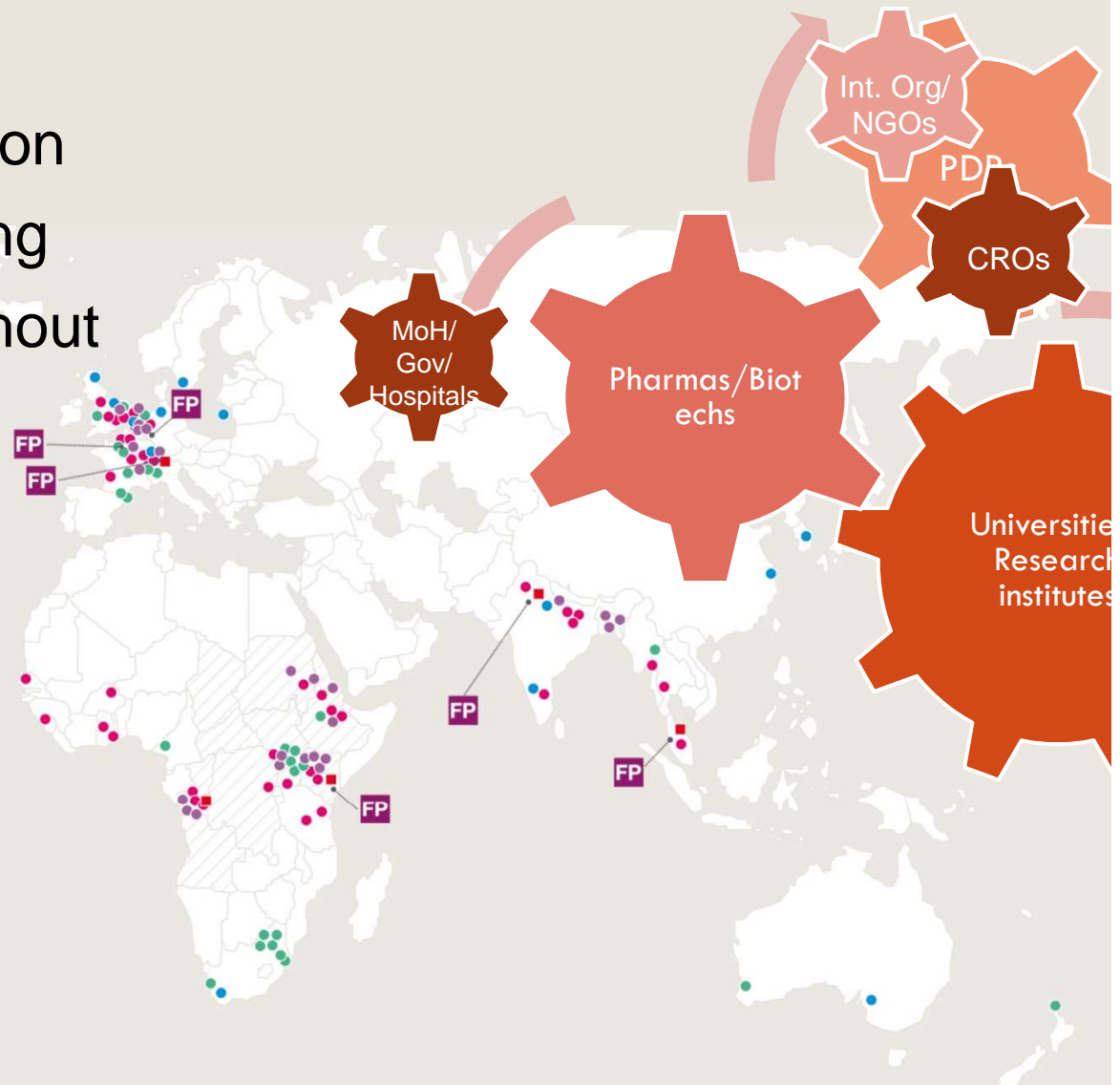
Project Partners

- Research
- Translation
- Development
- Implementation

▨ Platform Member Countries

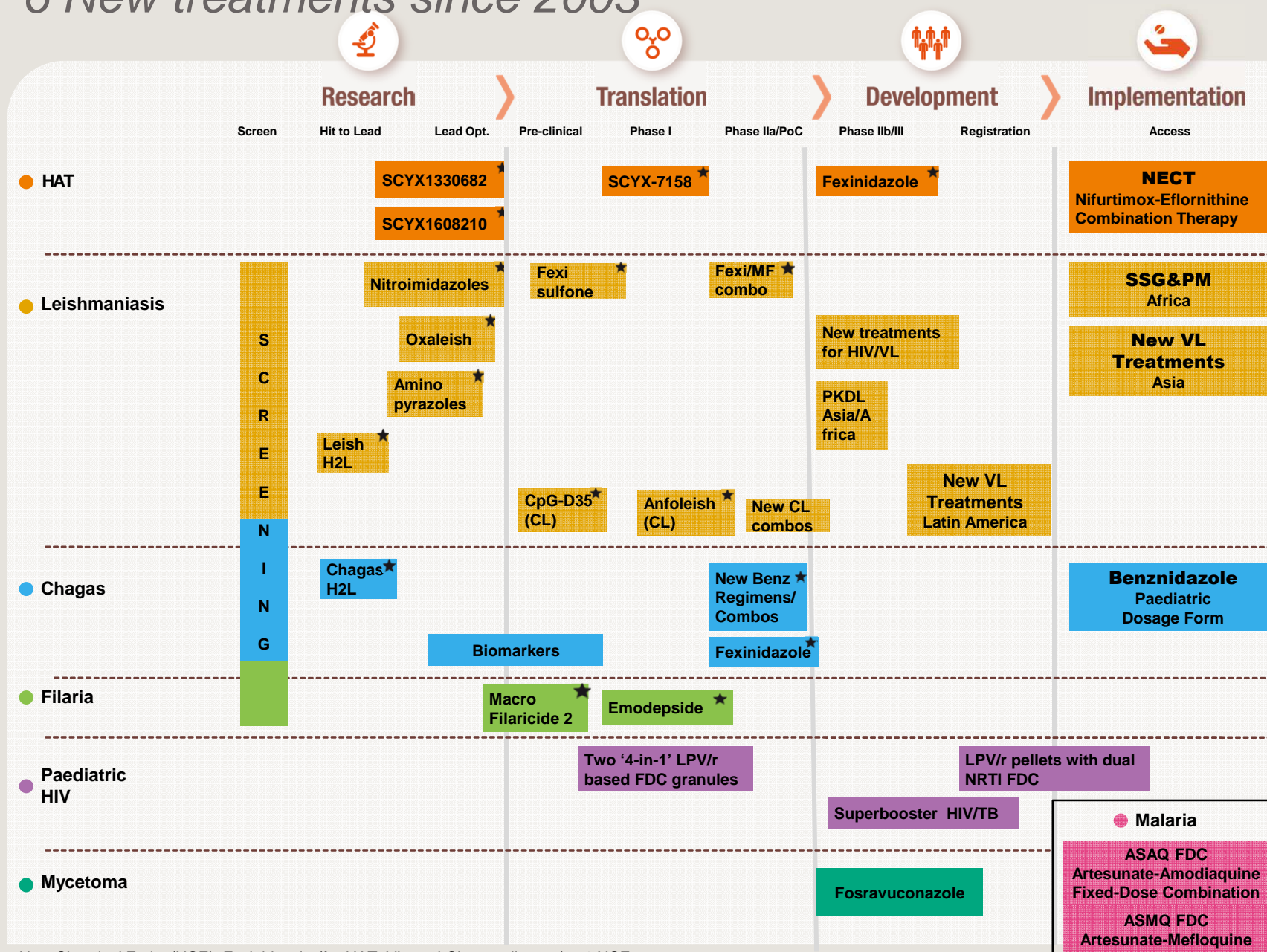
FP Founding Partners

■ DND/Worldwide



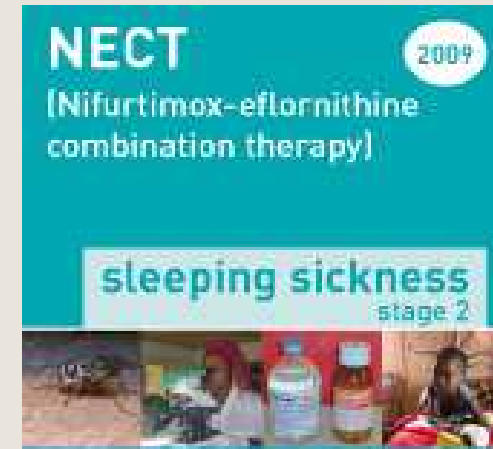
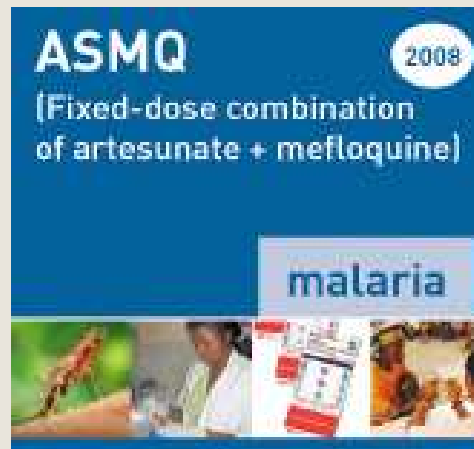
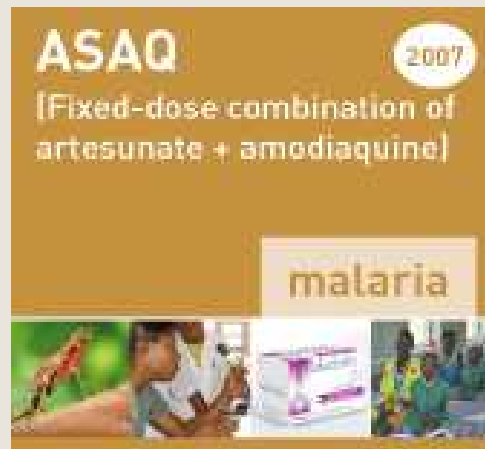
DNDi Portfolio June 2015

6 New treatments since 2003

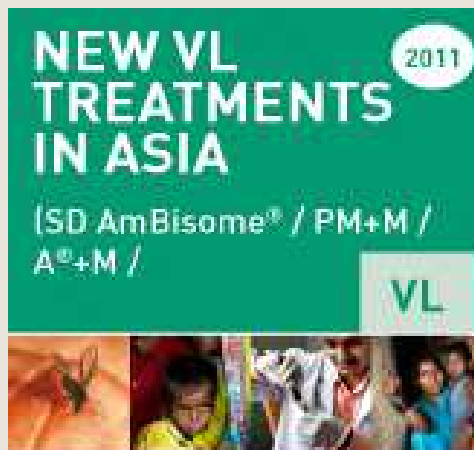


New Chemical Entity (NCE): Fexinidazole (for HAT, VL, and Chagas disease), 4 NCE

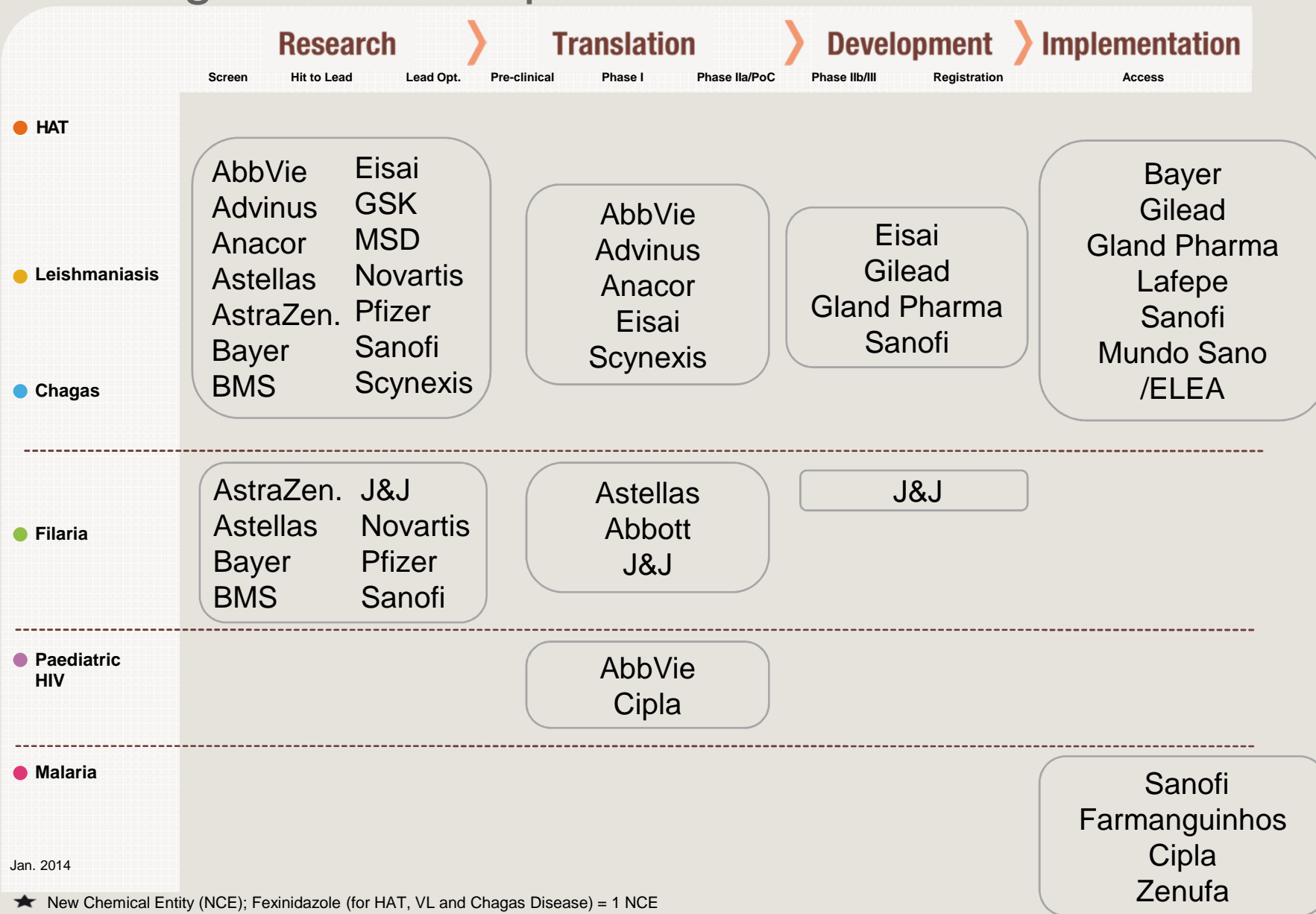
6 New Treatments Developed Since 2007 by DNDI and its partners



✓ Easy to Use ✓ Affordable ✓ Field-Adapted ✓ Non-Patented



Dynamic Industrial Partnerships at All Stages of Development

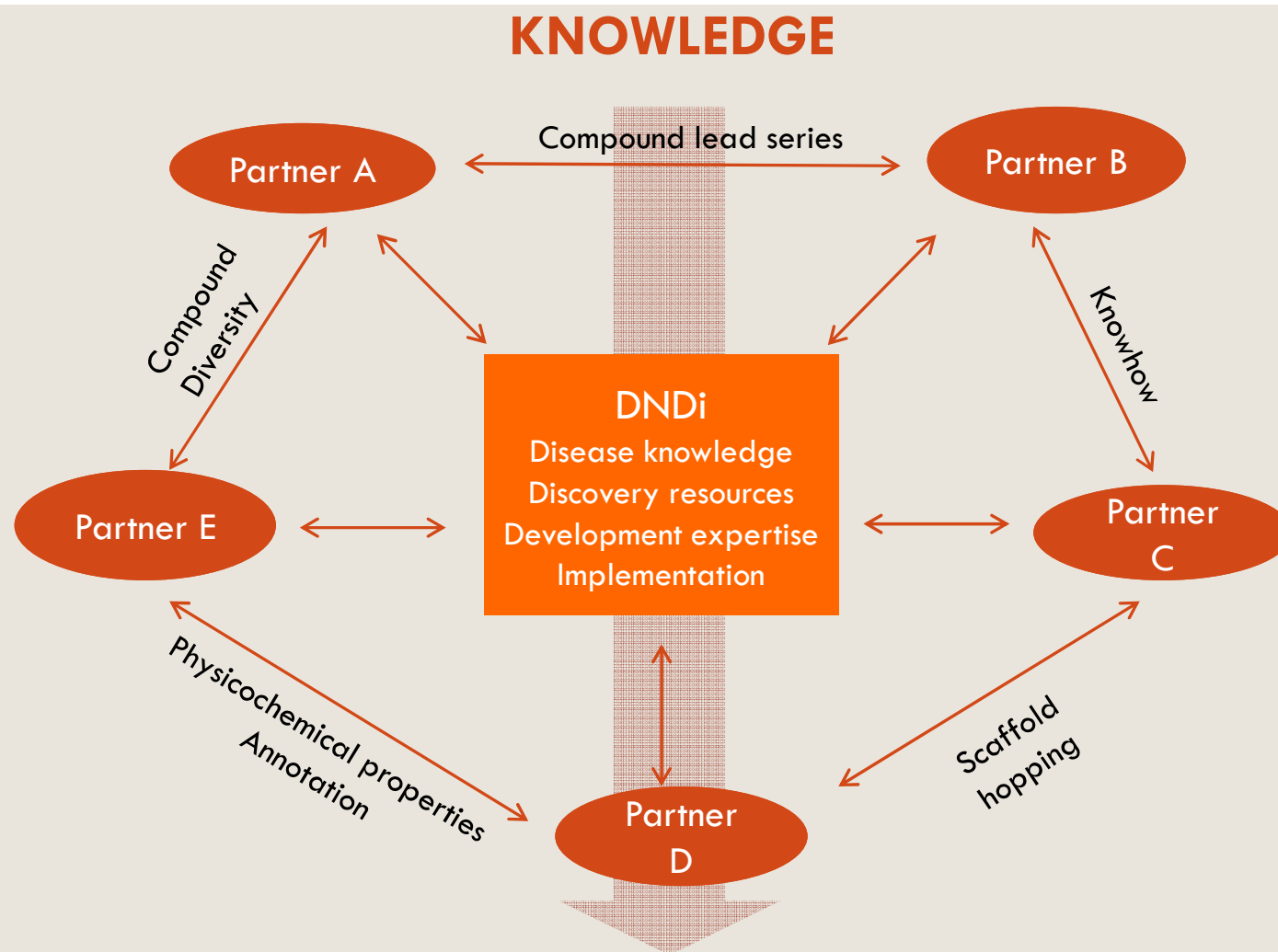


Boosting Discovery

Access to compounds libraries ... Through Multilateral Partnerships

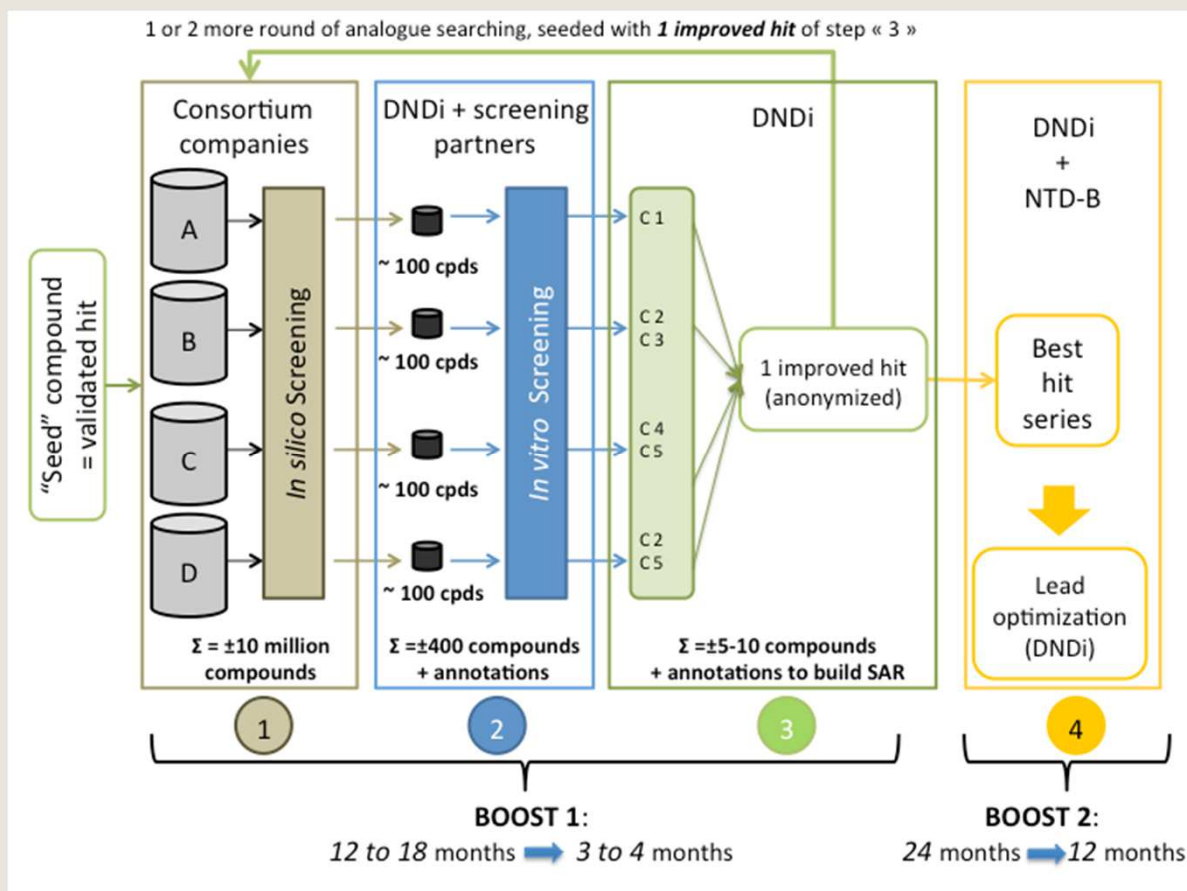


Research



NTD Drug Discovery Booster

An experiment to Accelerate and Expand Discovery



Eisai
Shionogi
Takeda
Astrazeneca

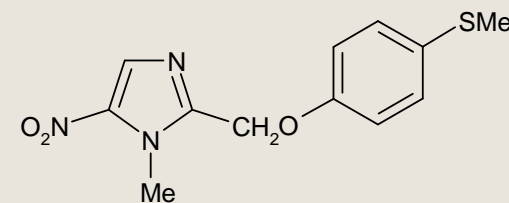
With Support of GHIT
Fund

- Modernize drug development
- Reduce investments
- Avoid research duplication
- Reduce time drug discovery by 2 Years

Sleeping Sickness: Develop New Oral Treatment

Fexinidazole, a Rediscovered New Chemical Entity

- ❑ 'Rediscovered' through compound mining
- ❑ Drug candidate to become an oral, short course treatment for stage 1+ 2 sleeping sickness treatment, caused by either *T.b. gambiense* or *rhodesiense*
- ❑ **In partnership with Sanofi**
- ❑ Phase II/III ongoing in DRC and CAR

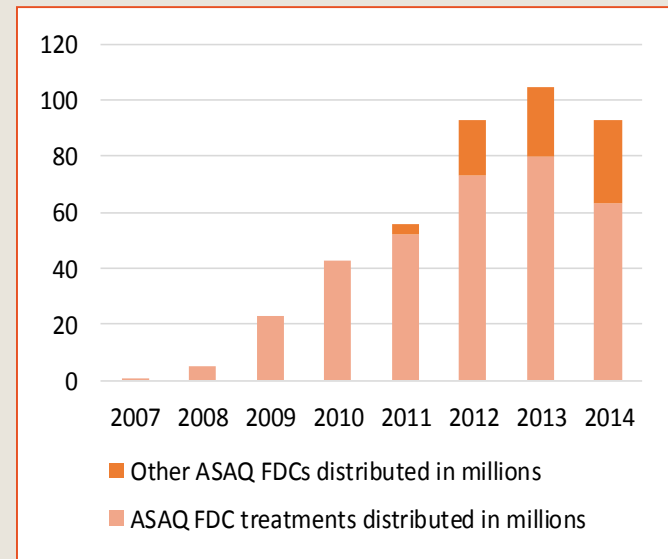


First innovative partnership ASAQ FDC

Over 400 Million Treatments Distributed

- Pre-qualified by WHO in 2008
- Less than 1 USD for adult & less than 0.5 cents for children
- Easy-to-use
- Non-patented product
- Registered in 30 African countries, India, Ecuador & Colombia
- First Risk Management Plan with MMV and Sanofi
- Transfer of technology to Zenufa (Tanzania)

**In partnership
with Sanofi**



Simplified 3-Day Dose Regimen of ASAQ

	NEW Fixed-dose ASAQ Artesunate/amodiaquine 3 dosage strengths available	Co-blistered non-fixed AS+AQ Artesunate-amodiaquine AS: 50 mg; AQ 135 mg
Young children (8-17kg)	AS: 25 mg AQ: 67.5 mg	1 yellow, 1 white
Children (17-35 kg)	AS: 50 mg AQ: 135 mg	2 yellow, 2 white
Adults > 36kg	AS: 100 mg AQ: 270 mg	4 yellow, 4 white

Pediatric HIV: The right dose, the right taste

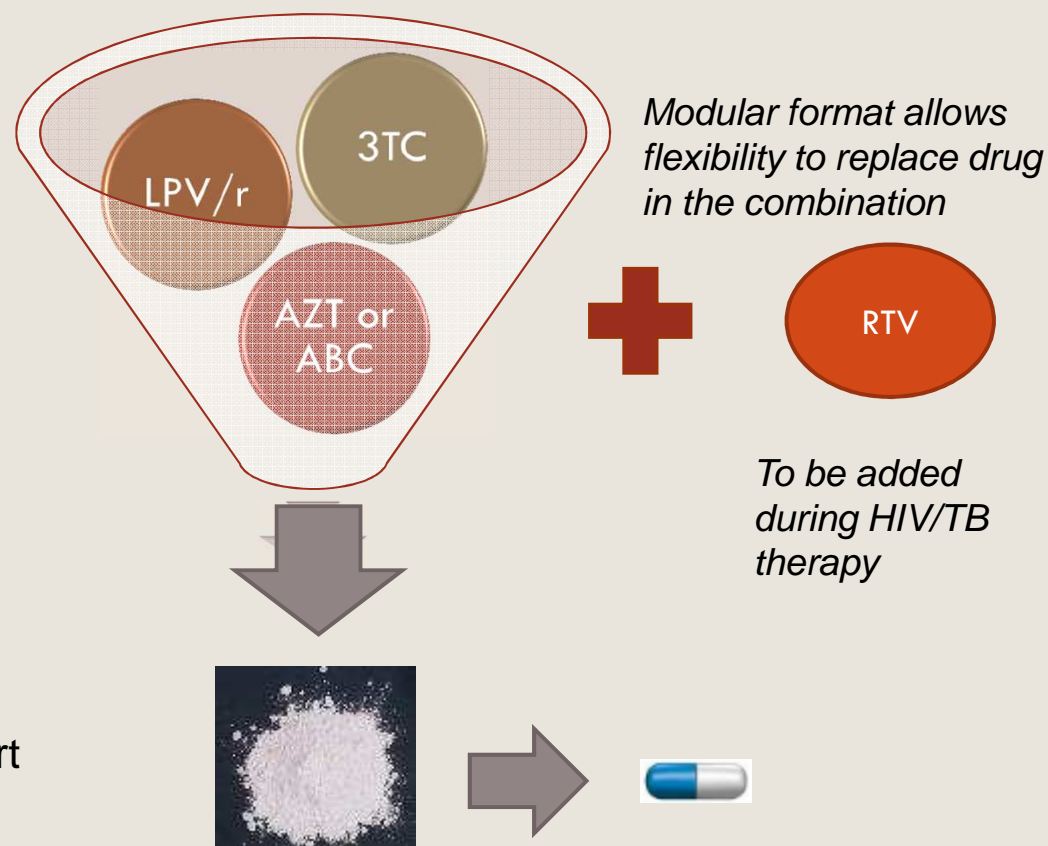
14

Target Product Profile:

- ▣ 4 products in 1: granules (FDC)
- ▣ Simply open and use with water, milk, food
- ▣ No taste
- ▣ No cold chain
- ▣ Suitable for infants (< 2 months-3 yrs)
- ▣ TB-treatment compatible
- ▣ Affordable

Planned with LPV/r pellets & NRTIs dispersible tablet – Phase 3b to start in Kenya Q3 2015 followed by Uganda, Tanzania, South Africa, Malawi and Zimbabwe.

LPV/r pellets received USFDA tentative approval May 2015.



4-in-1 granules in Fixed-Dose Combinations

In partnership with
Cipla

DNDi
Drugs for Neglected Diseases Initiative

Hepatitis C: Pricing Is Exorbitant and Competition Between Companies Prevents Development of Combinations for Public Health Use

13

- ❑ 150-180 million people suffer from HCV
- ❑ New direct-acting antivirals (DAAs) demonstrate cure rates of >95%
 - ❖ Access programs designed by pharma exclude middle income countries – which bear the greatest disease burden
 - ❖ Research prioritizes genotypes predominant in high-income markets
- ❑ Pan-genotypic regimen potentially exists combining SOF (Gilead) and DCV (BMS)
- ❑ *A public health approach is needed combining the optimal drugs from various sources*

Limited Public Health Approaches Using DAAs

DNDi Strategy Addresses Main Drivers of Unmet Need

Drivers of Unmet Need

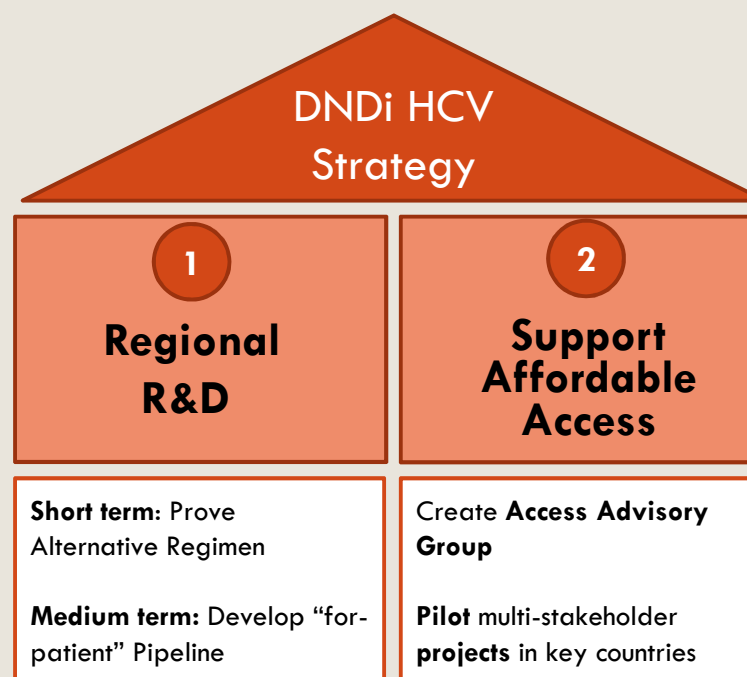
Lack of adequate R&D to support optimal DAA regimen

- Pharmaceutical companies not collaborating, developing regimens in their own pipeline
- Limited information on specific genotypes

Unaffordable treatment

- Prices of HCV drugs are unaffordable (ex. \$84,000 per treatment in US)
- Voluntary Licenses exclude several high-burden countries (ex. Thailand, Malaysia, Brazil...)

DNDi HCV Strategy



Antimicrobial Resistance (AMR): Establish «PDP»-like Initiative

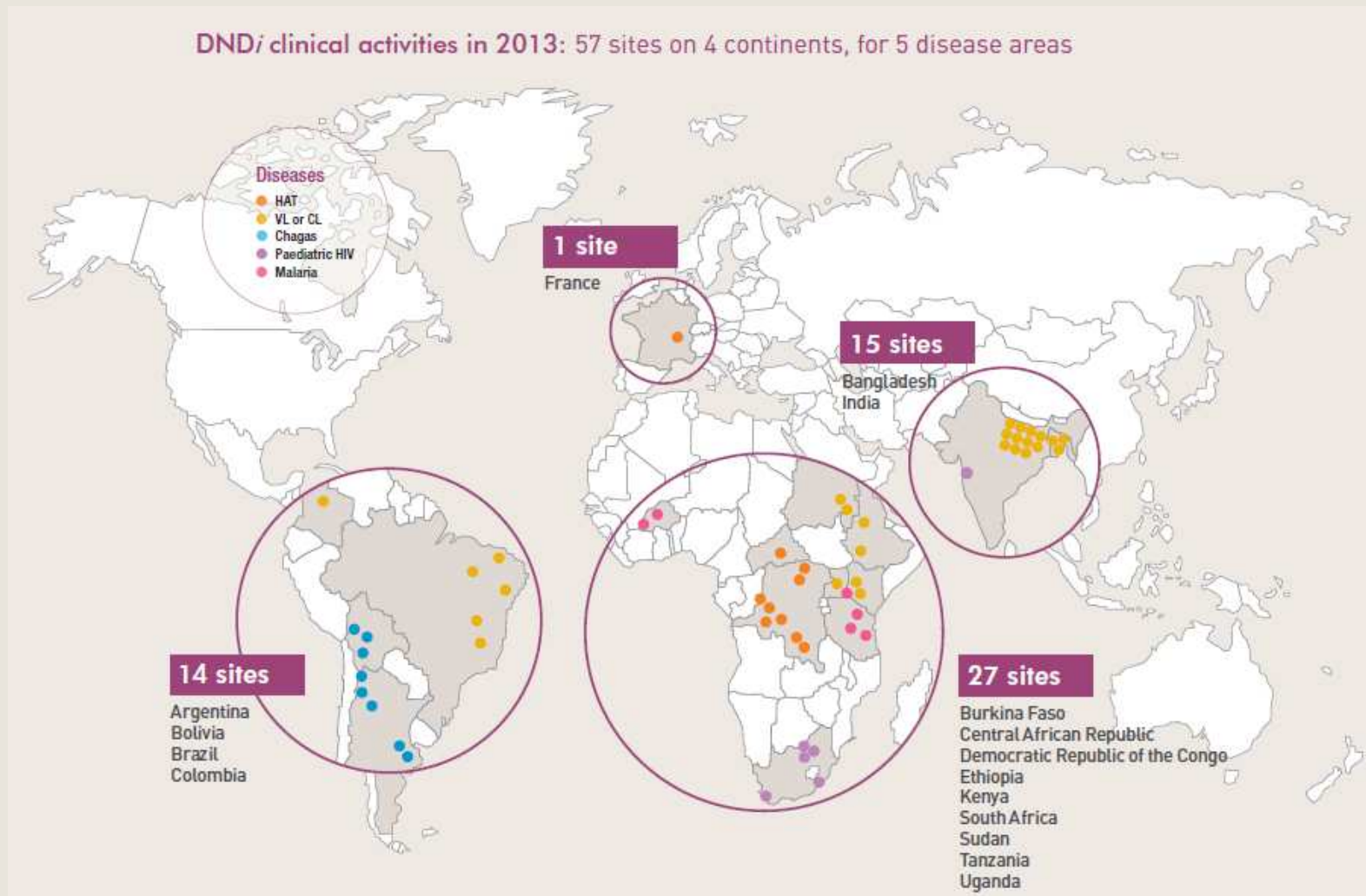
- Goal: Establish a not-for-profit product development partnership (PDP) which, in collaboration with public and private partners in high, middle and low-income countries, sustainably promotes R&D of new antibiotics and ensures their rational use and preservation globally
- Virtual, not-for-profit model:
 - ❖ A “virtual” model is favored because:
 - Provides flexibility, avoids costly overheads, facilitates collaborations with external public and private entities
 - Need assessments, TPPs definitions and scientific strategies will be defined solely by patient’s needs
 - No internal conflict to implement rationale use: no expectations of returns
 - Advocacy, fundraising and access to public funds facilitated, because no perceived or actual conflicts of interest
 - More freedom to design appropriate incentives and rewards, and to negotiate adequate premiums on the antimicrobials developed to ensure patient access

Principles

- ❑ Antibiotics should be considered a global public good.
- ❑ Public investment into development of new antibiotics should come with appropriate obligations with respect to the marketing and rational use of these new products to avoid the rapid build-up of drug resistance.
- ❑ Investment should be coordinated.
- ❑ R&D should focus on the most significant bacterial infections (in particular gram-negative infections) to answer global priority public health needs.
- ❑ New antibiotics have to be affordable to all and should be subjected to a global conservation agenda.

Overcoming Challenges in the Field Thanks to Our Partners in Endemic Countries

In 10 years: >33,000 patients enrolled in >20 clinical studies

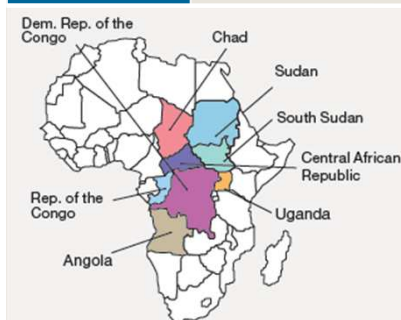


Utilizing and Strengthening Research Capacities in Disease-Endemic Countries

VL



HAT



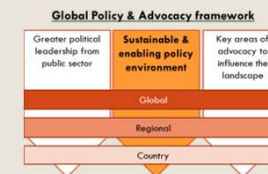
CHAGAS



Major Role of Regional Disease Platforms:

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

A key global objective of DNDi's policy is to support the Global Biomedical R&D Fund and Mechanism



19

Funding for R&D initiatives



BILL & MELINDA GATES foundation



...and others

Global Biomedical R&D Fund and Mechanism

*For innovations of Public Health importance
governed by public leadership*

Global Health R&D Observatory

Priority-Setting, Monitoring, Coordination

AMR
Initiatives

Emerging
Infections
(incl. Ebola)

Poverty
Related /
Neglected
Diseases*

De-linkage

Open Innovation

Licensing for Access

WHO CEWG
Pilot Demonstration
Projects, Voluntary
Pooled Fund (TDR)

* Type II and III diseases, and the specific R&D needs of developing countries in relation to Type I diseases"

To Give a voice to neglected patients



They exist....
They must be heard.

THANK YOU!

*Carmen Rosa,
Bolivia*

DNDi
Drugs for Neglected Diseases Initiative