The way forward needs-driven public health research

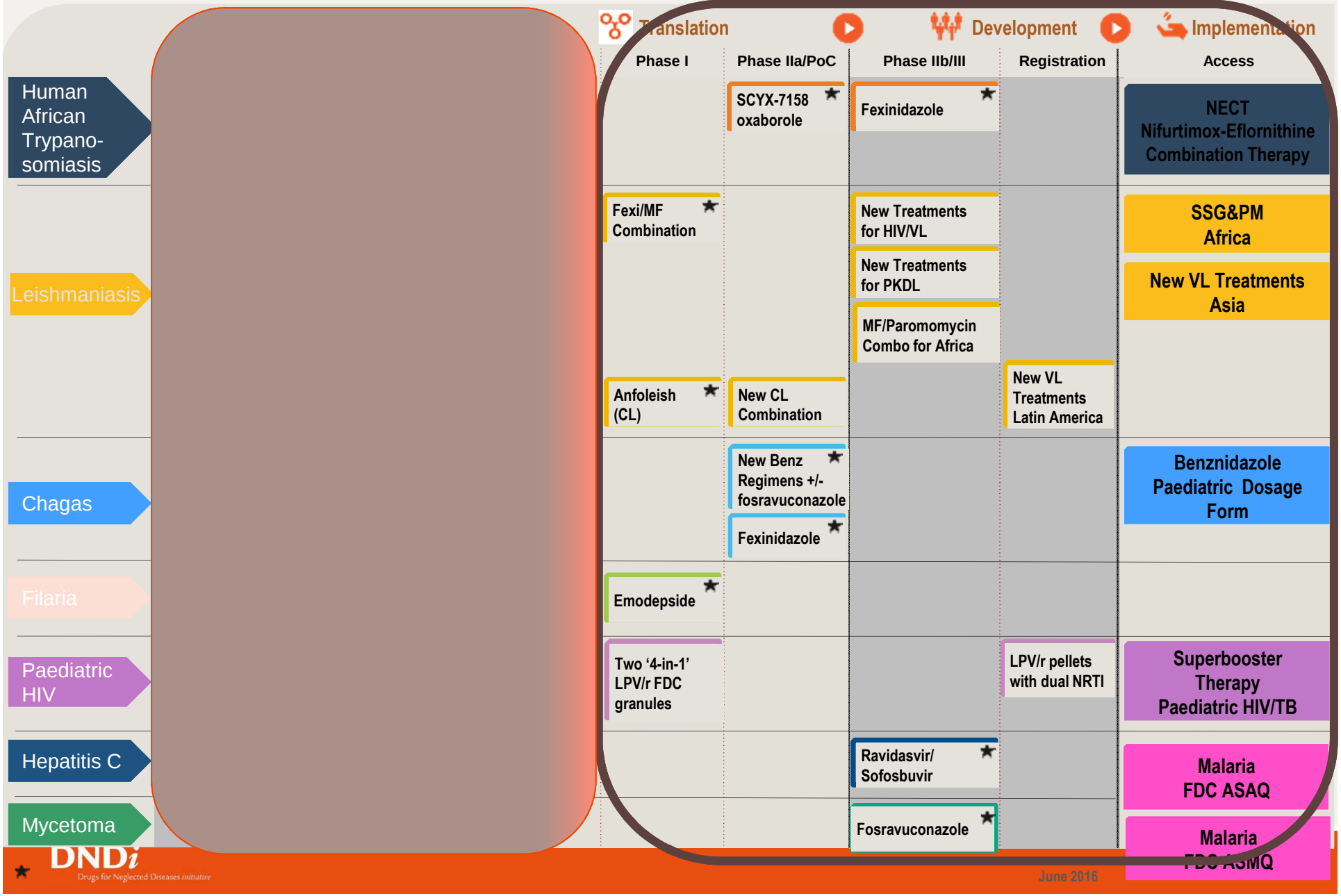
PUBLIC CONFERENCE ON
CLINICAL DRUG TRIALS
30 SEPTEMBER, 2016 | GENEVA, SWITZERLAND

Not for Profit R&D
Responding to the Needs of Patients Suffering from Neglected Diseases…

… from Bench to Bedside

DNDi R&D Portfolio

7 new treatments available and 15 new chemical entities in the pipeline



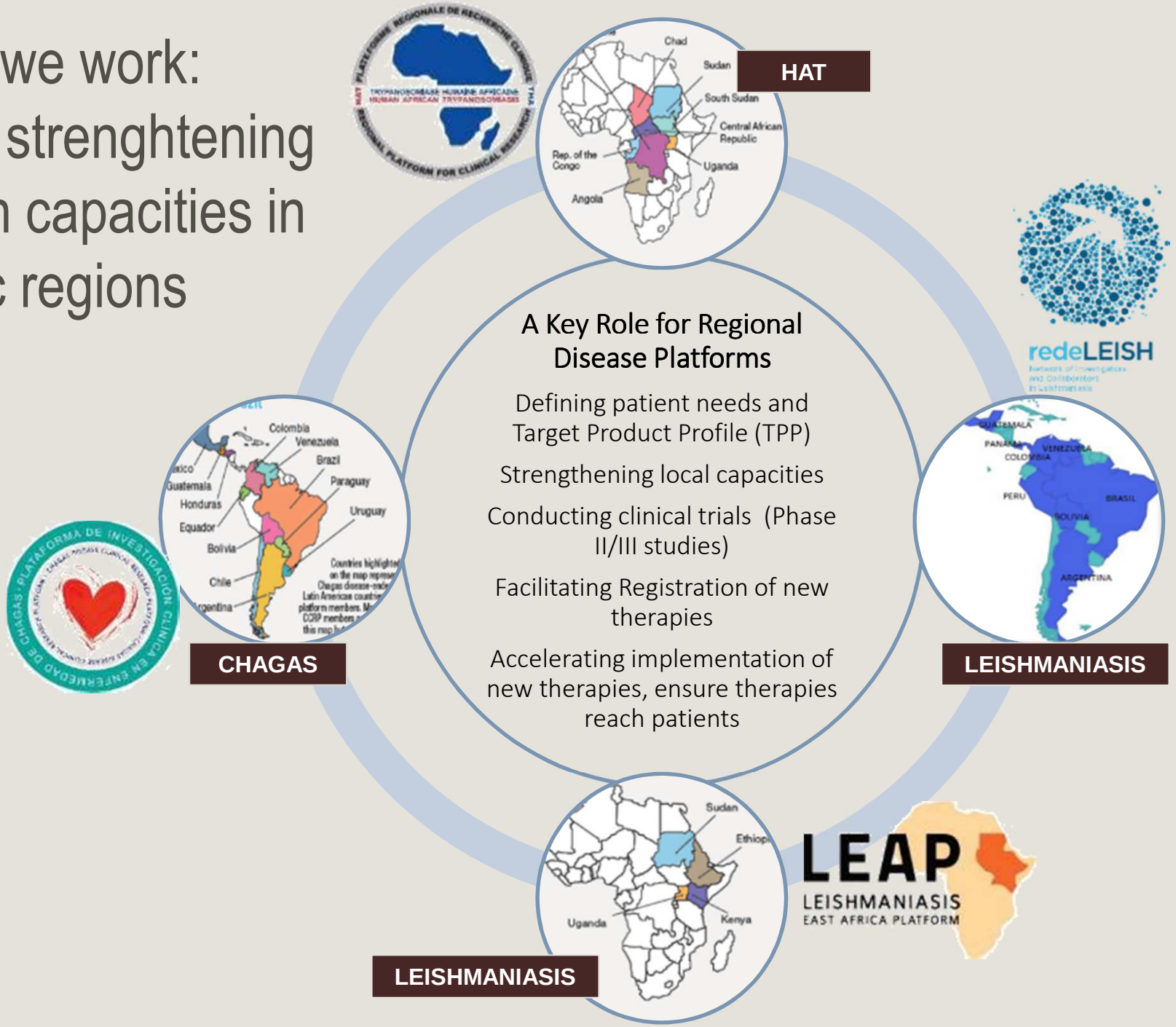
Characteristics of our model

- Not for Profit
- Patients-need driven
- Independence – public/private funding
- Governance (Public Research Founding Institutions)
- Policies
  - IP / public good
  - Delinkage / affordable
  - Sharing data / open source
  - Transparency

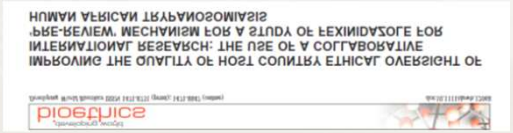
Characteristics of our CTs

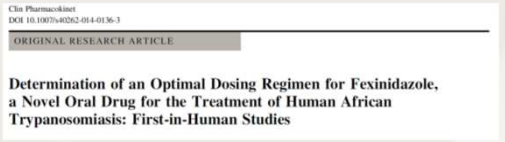
- Pipeline focused on *unmet medical needs*
- Product development has to meet a disease-specific *«Target Product Profile»* developped with public health partners
- Our CTs are conducted in endemic countries with often:
  - Poor, voiceless, non literate, i.e *vulnerable* patients
  - Investigators with little if no experience in CTs
  - Poorly resourced Ethics Committees and / or National Regulatory Authorities

How do we work: Using & strenghtening research capacities in endemic regions



Sleeping sickness





- Lethal disease
- Major endemicity in Democratic Republic of Congo
- Need for better treatments (oral, no lumbar puncture)
- No treatment recently developped for that indication
  - Fexinidazole identified through compound mining
  - Contract with industrial partner Sanofi
  - Tested in Phase I subjects of sub-saharan origin in France
  - Art58 EMA scientific advice to share & obtain advice on clinical devpt strategy
  - Infrastructure and HR (GCP/clinical) capacity strenghthening +++
  - Joint «North/South/South» ethics review
    - Phase 1 results published
  - Working according international standards

    > 500 patients recruited in 10 clinical sites
  - Regular updates made to the WHO-NTD working group on HAT elimination
  - Aiming at Regulatory collaborative review for regulatory approvals
  - Dossier includes Risk management Plan that can address needs for future follow-up

Challenges

- Ethical concerns around vulnerability
- EC/NRAs capacity to reveiw CTs
- Transparency: technical, cost, timing of access to data
- Regulatory marketing autorisation: do these disease deserve a «priority process?»
- Post approval safety detection / Pharmacovigilance

- Future
  - Defining public health priorities and expected value of new drugs
  - Promote inclusive collaborative procedures with public health actors
  - R&D Observatory and Fund …


Thank you
DNDi
Drugs for Neglected Diseases initiative