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...





DNDi R&D Portfolio

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

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	Phase I	Phase IIa/PoC	Phase IIb/III	Registration	Access
Human African Trypano- somiasis		SCYX-7158 * oxaborole	Fexinidazole *		NECT Nifurtimox-Eflornithine Combination Therapy
	Fexi/MF * Combination		New Treatments for HIV/VL		SSG&PM Africa
Leishmaniasis			New Treatments for PKDL		New VL Treatments Asia
			MF/Paromomycin Combo for Africa		Adia
	Anfoleish ★ (CL)	New CL Combination		New VL Treatments Latin America	
Chagas		New Benz ★ Regimens +/- fosravuconazole			Benznidazole Paediatric Dosage Form
		Fexinidazole *			
Filaria	Emodepside *				
Paediatric HIV	Two '4-in-1' LPV/r FDC granules			LPV/r pellets with dual NRTI	Superbooster Therapy Paediatric HIV/TB
Hepatitis C			Ravidasvir/ * Sofosbuvir		Malaria FDC ASAQ
Mycetoma			Fosravuconazole *		Malaria
DINDI Druge for Neglected Diseases initiative				June 2016	FEC ASMQ

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



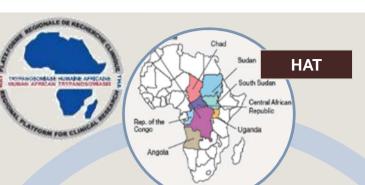
Characteristics of our CTs

- 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





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





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

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Determination of an Optimal Dosing Regimen for Fexinidazole, a Novel Oral Drug for the Treatment of Human African Trypanosomiasis: First-in-Human Studies







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

