SETTING THE SCENE ON RESEARCH AND DEVELOPMENT OF HIV MEDICINES

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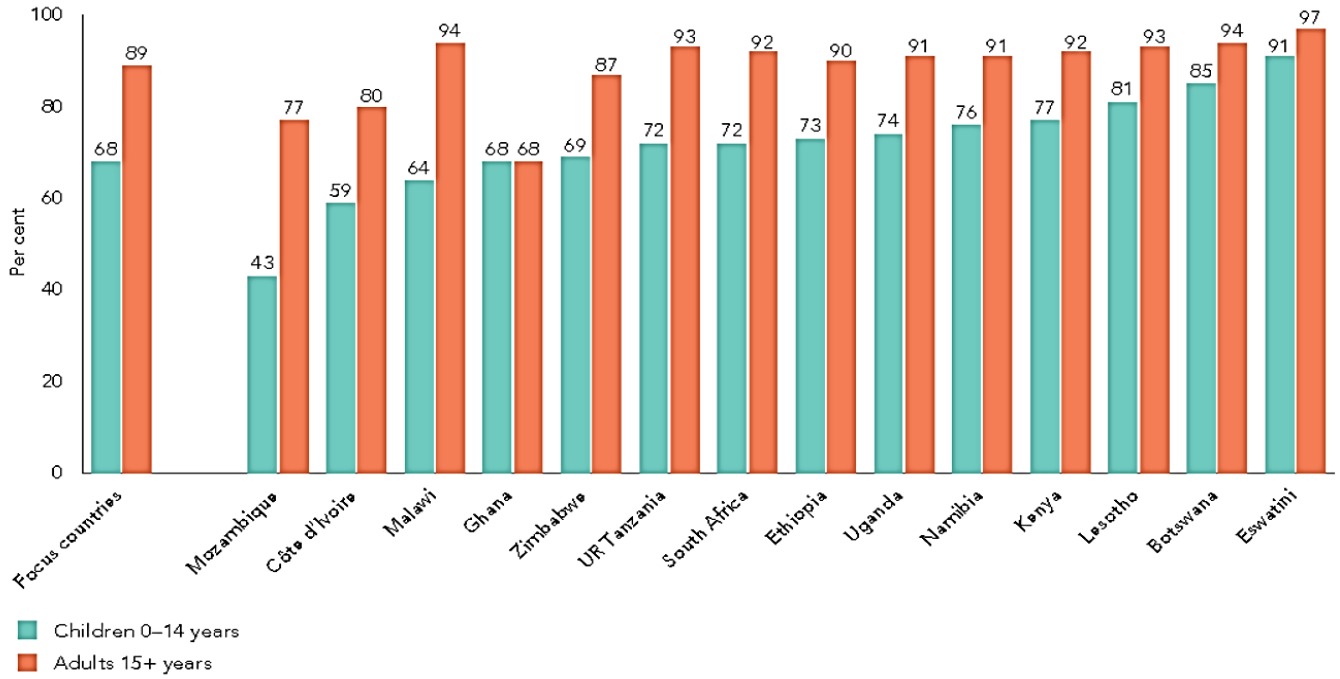
Getting to an AIDS FREE generation requires better medicines for infants and children

For treatment

and

postnatal prophylaxis!

Figure 14. Viral load suppression among people aged 0–14 and 15+ years receiving antiretroviral therapy by age group and country in 15 focus countries, 2019



Source: UNAIDS epidemiological estimates, 2020.

ROME 3 and 4: a critical milestone for getting better medicines to children

- Action is required NOW!
- We need to **FOCUS** our efforts on the most needed formulations
- We must **ACCELERATE** the research, the development and the approval of priority drugs and formulations
- We must **COLLABORATE** and ensure coordination of all stakeholders involved

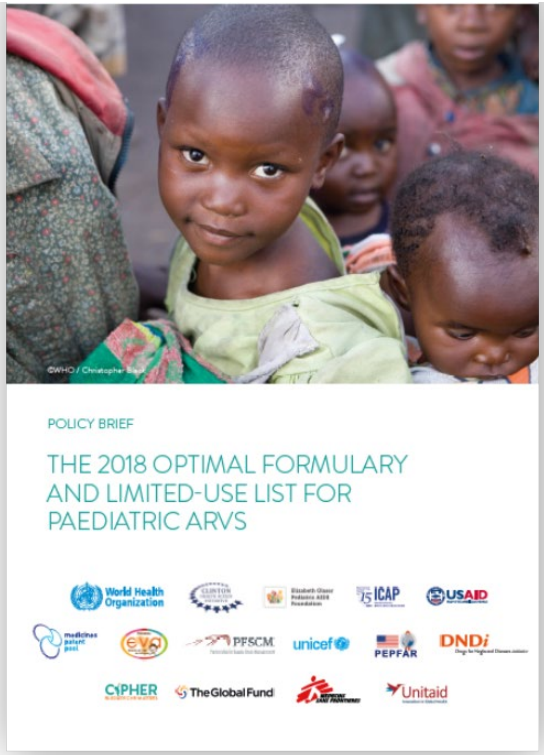
- **WE ALL NEED TO COMMIT** - mutual accountability

Several Challenges Hamper the Rapid Development of Paediatric Formulations

- Natural history, co-morbidities and drug metabolism
- Lack of data for development and use of new drugs
- Delay in completion of clinical studies
- Challenges with taste and administration
- Lack of child friendly formulations
- Complexity and cost of the projects
- Internal prioritization within companies
- Lack of market incentives and small markets
- Difficult and slow market uptake

Over the last 4 years through the support of this platform and dedicated efforts by many stakeholders involved….

We have increased our alignment on the priority formulations



PADO4 list

- DTG 10 mg scored
- DRVr 120/20 mg
- XTC/TAF
- XTC/TAF/DTG

PADO4 watch list

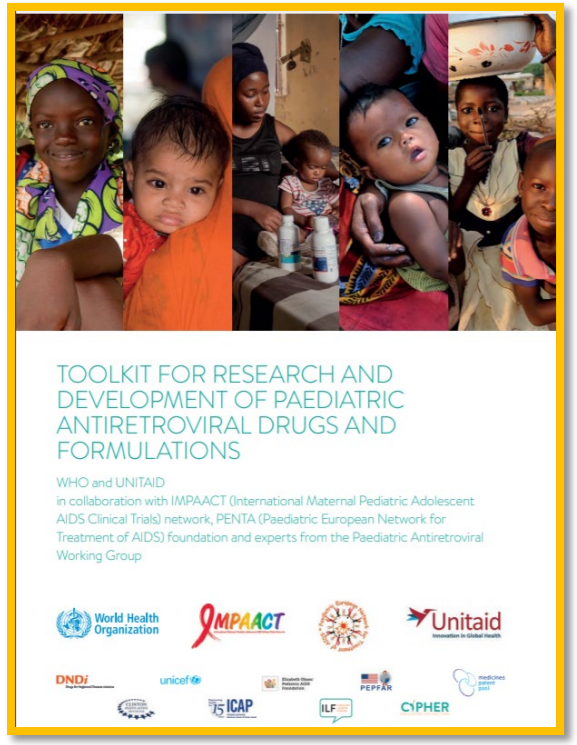
- Islatravir
- Doravirine
- Neutralizing antibodies
- Long-acting agents
- New delivery systems

ARVs to manufacture

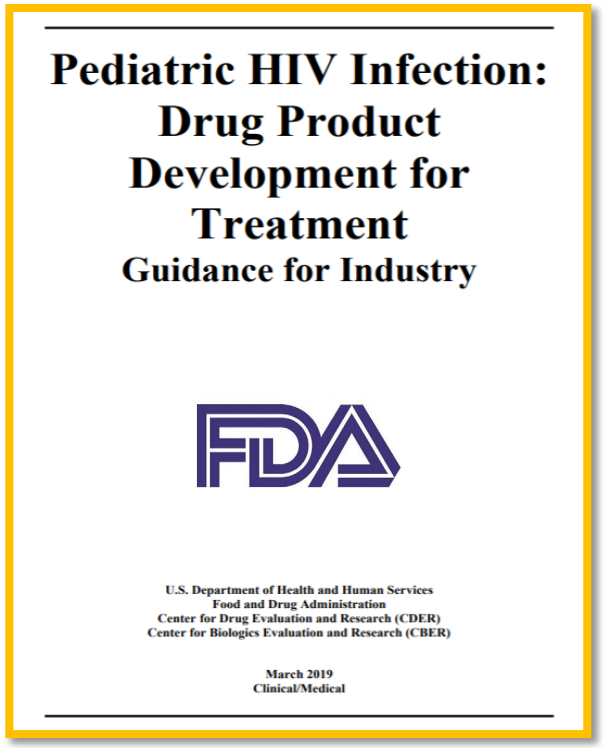
ARVs to develop

ARVs to investigate

As well as on key principles for acceleration



Available on WHO website: http://www.who.int/hiv/pub/research-dev-toolkit-paediatric-arv-drug-formulation/en/



Pediatric HIV Infection: Drug Product Development for Treatment

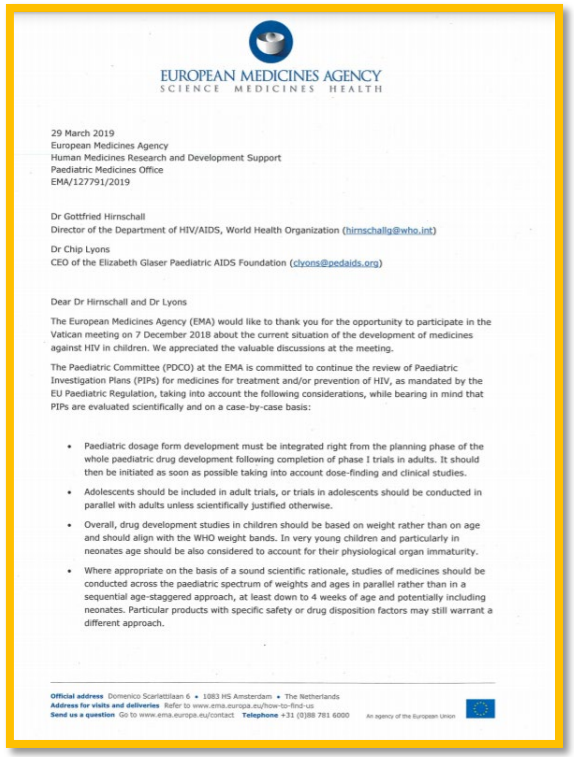
Guidance for Industry

FDA

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

March 2019
Clinical/Medical

Available on FDA website: https://www.fda.gov/media/113319/download



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

29 March 2019
European Medicines Agency
Human Medicines Research and Development Support
Paediatric Medicines Office
EMA/127791/2019

Dr Gottfried Hirnschall
Director of the Department of HIV/AIDS, World Health Organization (hirnschallg@who.int)

Dr Chip Lyons
CEO of the Elizabeth Glaser Paediatric AIDS Foundation (clyons@pedaids.org)

Dear Dr Hirnschall and Dr Lyons

The European Medicines Agency (EMA) would like to thank you for the opportunity to participate in the Vatican meeting on 7 December 2018 about the current situation of the development of medicines against HIV in children. We appreciated the valuable discussions at the meeting.

The Paediatric Committee (PDCO) at the EMA is committed to continue the review of Paediatric Investigation Plans (PIPs) for medicines for treatment and/or prevention of HIV, as mandated by the EU Paediatric Regulation, taking into account the following considerations, while bearing in mind that PIPs are evaluated scientifically and on a case-by-case basis:

- Paediatric dosage form development must be integrated right from the planning phase of the whole paediatric drug development following completion of phase I trials in adults. It should then be initiated as soon as possible taking into account dose-finding and clinical studies.
- Adolescents should be included in adult trials, or trials in adolescents should be conducted in parallel with adults unless scientifically justified otherwise.
- Overall, drug development studies in children should be based on weight rather than on age and should align with the WHO weight bands. In very young children and particularly in neonates age should be also considered to account for their physiological organ immaturity.
- Where appropriate on the basis of a sound scientific rationale, studies of medicines should be conducted across the paediatric spectrum of weights and ages in parallel rather than in a sequential age-staggered approach, at least down to 4 weeks of age and potentially including neonates. Particular products with specific safety or drug disposition factors may still warrant a different approach.

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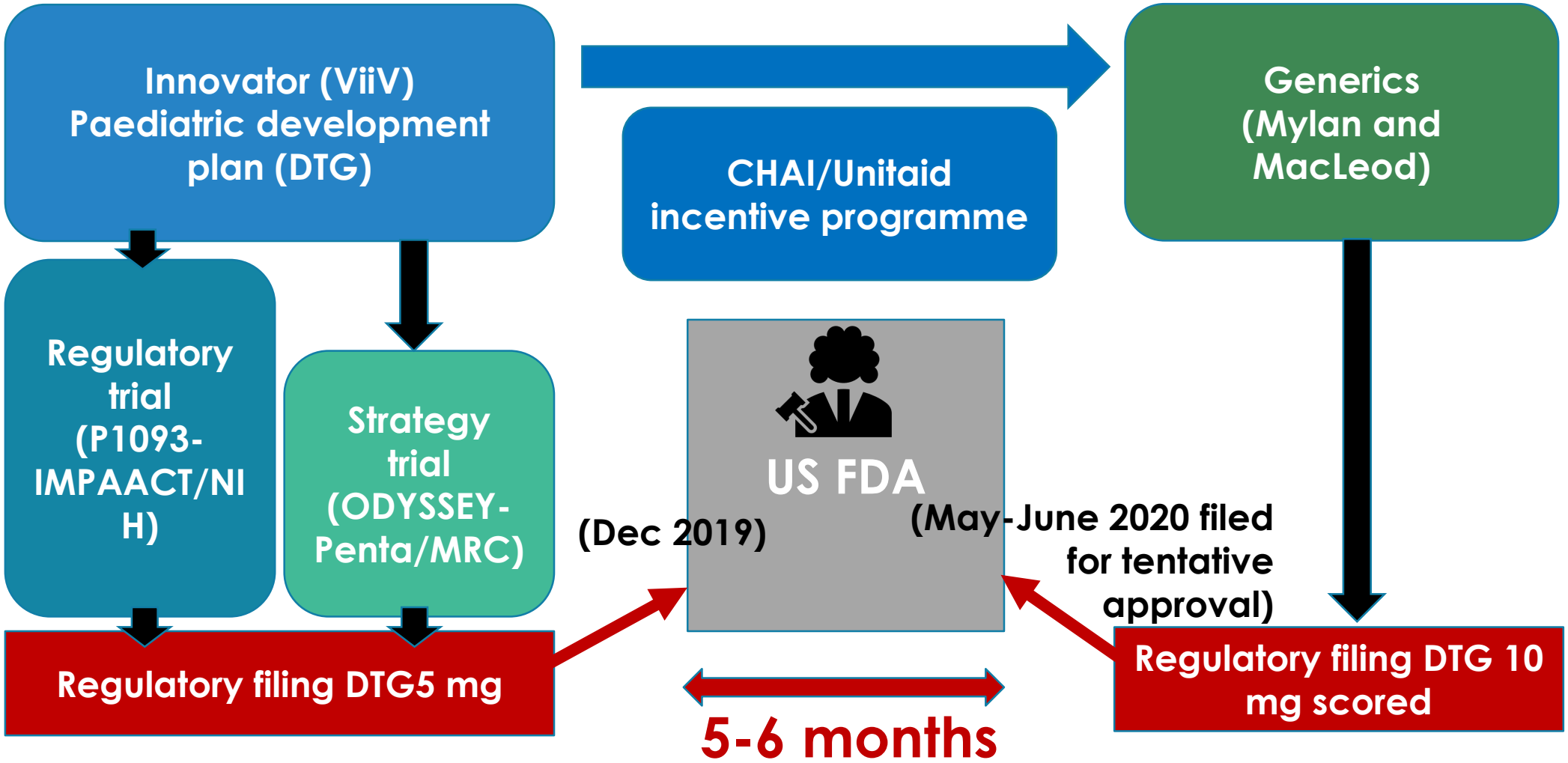
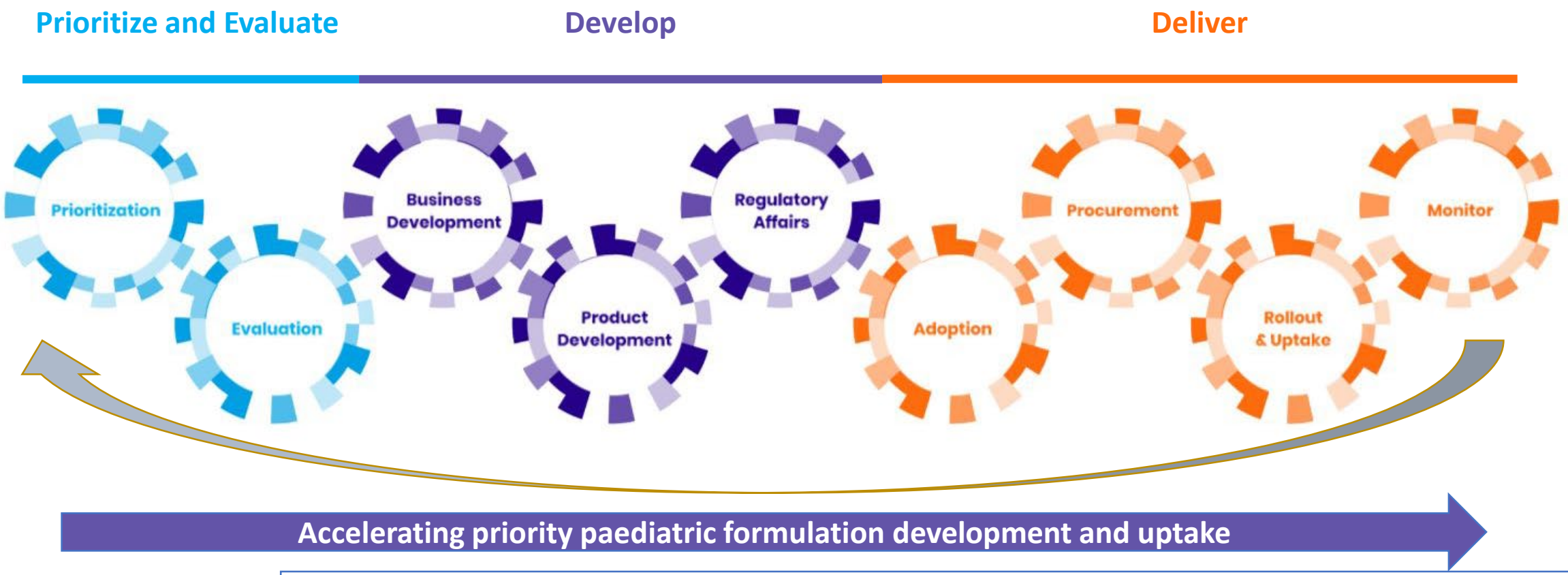
An agency of the European Union

Available on EMA website: https://7516e5c8-88e8-4634-85db-70d27ff1f169.filesusr.com/ugd/38bdff_204c9b65a0bc4aca8cc51e29f09628ee.pdf

We have explored new models of collaboration



And consolidated the good ones for the future



GAP•f
Global Accelerator for Paediatric formulations
WHO Hosted Network

A collaboration platform supported by an innovative financing mechanism that promotes a faster, more efficient and more focused approach to paediatric clinical studies, formulation development and introduction.

So…What do we need to do today?

We need to sustain our efforts on old commitments that have fallen behind

- Challenges with development of paediatric formulations to implement full investigation plans (ie. dispersibility, palatability, bioavailability)
- Delays with securing resources to support development of priority formulations.
- PIP/PSPs not fully designed for accelerated implementation
- Alignment among regulators still needs improvement
- Newer trials designs not fully capturing opportunities for acceleration
- Need to engage with ERBs so that protocols can be approved quickly
- Limited interest in development of low volume products

COVID-19: a challenge and an opportunity

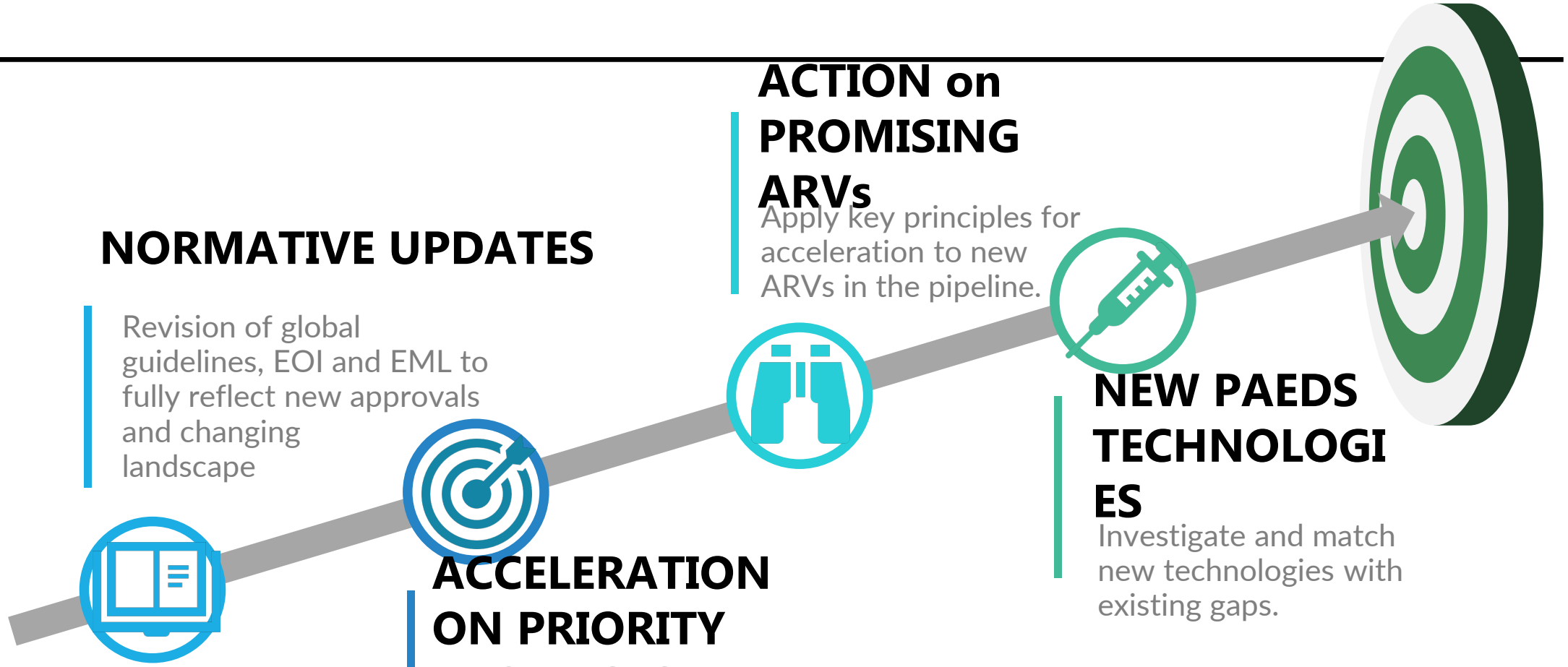
Opportunities

- Accelerated timelines for investigation
- Pilot new study designs
- Models for partnership

Challenges

- Stop enrollments and FU visits
- Challenges with procurement of study drugs
- Competing priorities for industry, researchers an regulators

Focus on what's next



NORMATIVE UPDATES

Revision of global guidelines, EOI and EML to fully reflect new approvals and changing landscape

ACCELERATION ON PRIORITY PRODUCTS

A number of priority products in the PADO list have been falling behind and require acceleration.

ACTION on PROMISING ARVs

Apply key principles for acceleration to new ARVs in the pipeline.

NEW PAEDS TECHNOLOGIES

Investigate and match new technologies with existing gaps.

Engage with a longer term vision

Knowing we all have a role to play

RESEARCH NETWORKS

Proactively support design and implementation of PIPs/PSPs alongside strategy trials while expanding collection and use of observational data
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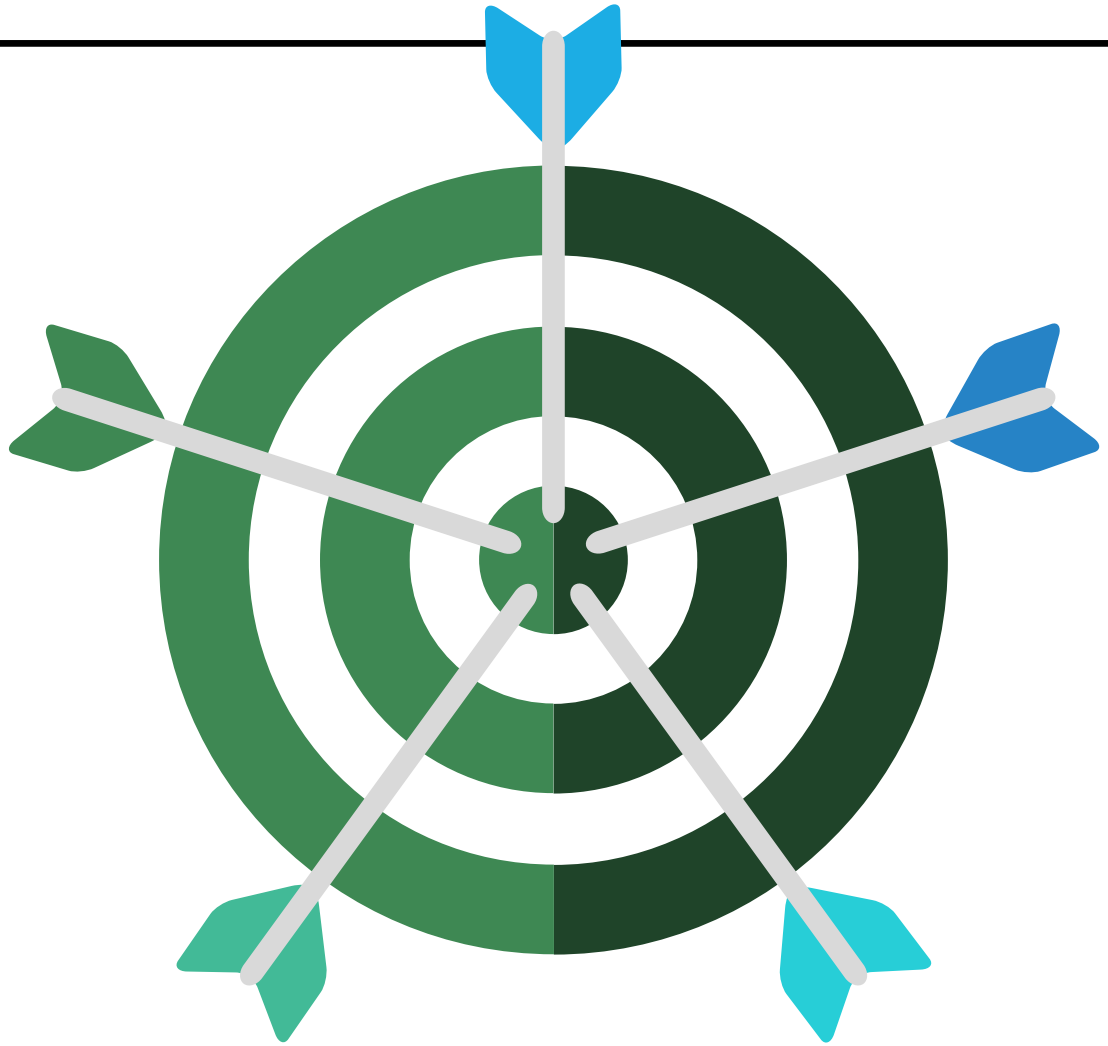
NGOs and KEY DONORS

Identify, support and monitor strategic partnerships to accelerate investigation and development of better Paediatric ARVS



POLICYMAKERS

Timely revision of key guidance documents to maintain alignment and advance drug optimization

INDUSTRY

Proactively reach out to PAWG and work with GAP-f network to explore partnerships to de-risk and accelerate investigation and development of new ARVs for children

REGULATORS

Continue efforts to ensure alignment and streamlining of processes

NOW IS OUR TURN….