GLOBAL ACCELERATOR
FOR PAEDIATRIC FORMULATIONS

Accountability towards accelerated access to better medicines for children

17 January 2024 / 13:00-14:30 CET

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World Health Organization

Agenda for today's public briefing

Time (CET)	**Agenda item**	**Presenter**
14:00-14:10	Welcome: Framing accountability towards access to medicines for children	David Ruiz Villafranca (EGPAF)
14:10-14:20	GAP-f as a mechanism to respond to WHA Resolution 69.20 on access to medicines for children	Farihah Malik (GAP-f)
14:20-14:30	Advocating for accelerated access to better medicines for children: Key elements of advocacy	Sébastien Morin (MPP)
14:30-14:40	Advocating together: Upcoming opportunities around the WHO EB and the WHA in 2024	Michelle Childs (DNDi)
14:40-15:25	Facilitated discussion: Coordinating possible advocacy actions from now until the WHA in May	Michelle Childs (DNDi)
15:25-15:30	Summary and next steps	David Ruiz Villafranca (EGPAF)

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Welcome: Framing accountability towards access to medicines for children

David Ruiz Villafranca (EGPAF)

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URGENCY

- 5.2 million children under 5 die each year mostly from preventable and treatable causes.
- Paediatric medicines lag behind that of adults by nearly a decade.

Political attention

Specific WHA Res: WHA 60.20 and WHA 69.20

Related WHA Res: WHA 69.25, WHA 67.20, WHA 74.6, WHA 75.8

- Access to medicines
- Clinical trials
- Access to diagnostics

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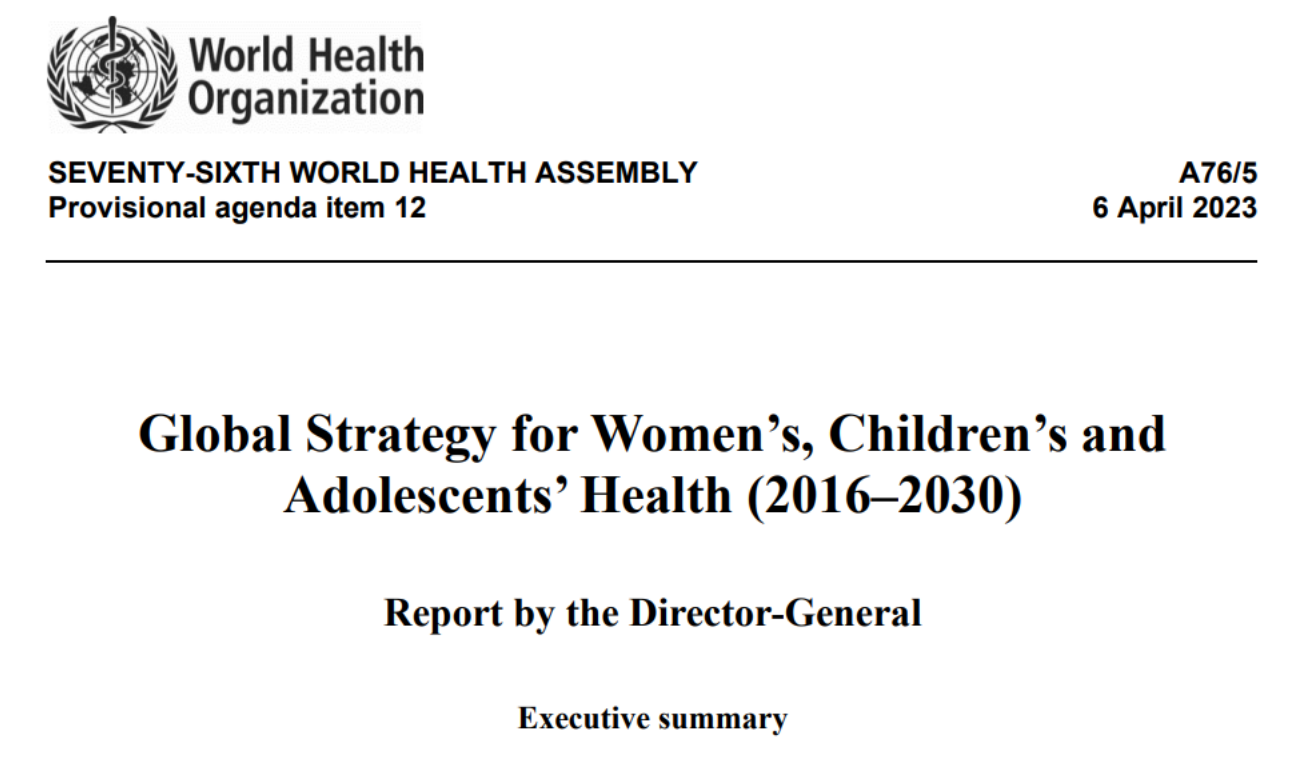
BUT... Still, many countries face important challenges to access to optimal medicines for children. What actions did those Resolutions trigger in country?



World Health Organization

SEVENTY-SIXTH WORLD HEALTH ASSEMBLY
Provisional agenda item 12

A76/5
6 April 2023

Global Strategy for Women's, Children's and Adolescents' Health (2016–2030)

Report by the Director-General

Executive summary

Appropriate medicines to save and improve the lives of infants and children often do not exist, especially in low-resource settings. The Global Accelerator for Paediatric Formulations, a WHO-coordinated network hosted within the Science Division, was established to respond to these gaps in paediatric treatment. It works across three main strategic pillars (to prioritize and align, accelerate and intervene) across the product life cycle and in different disease areas.

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Some considerations

- Limited advocacy and mobilization on this topic
- Limited social accountability around Access to Medicines for Children
- Limited investments on pediatric R&D
- "Technical complexities"

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GAP-f as a mechanism to respond to WHA Resolution 69.20 on access to medicines for children

Farihah Malik (GAP-f)

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The problem

Problem #1

Siloed approaches lead to efforts falling through the cracks, especially in a void of global priority-setting of needs, and a lack of coordinated and sustainable funding for paediatric health priorities.

Problem #2

Too much time is lost in translation.

Problem #3

In the fragile product development landscape, one gap unfilled can halt an entire life-saving solution.

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Our Vision

All children have equitable access to the medicines they need

What Makes Us Unique

We work across the entire life cycle of paediatric drug development

GAP-f

Our Mission

Remove barriers to developing and delivering appropriate, quality, affordable and accessible medicines for children and contribute to universal health coverage by spurring collaboration across stakeholders to identify gaps, set priorities for needs and accelerate product investigation, development and delivery to improve and save the lives of children

Our Network

GAP-f is a WHO network, created to respond to the paediatric treatment gap

A concerted response across the product life-cycle

Prioritizing and Evaluating:

Developing a prioritized drug portfolio of the most needed formulations and assisting in the design and implementation of efficient, and high-quality clinical trials.

Developing:

Establishing and maintaining relationships to launch effective products and supporting regulatory submission activities to facilitate paediatric medicine approvals.

Delivering:

Supporting efforts to introduce new, adapted formulations in an equitable, accelerated, safe and coordinated manner.

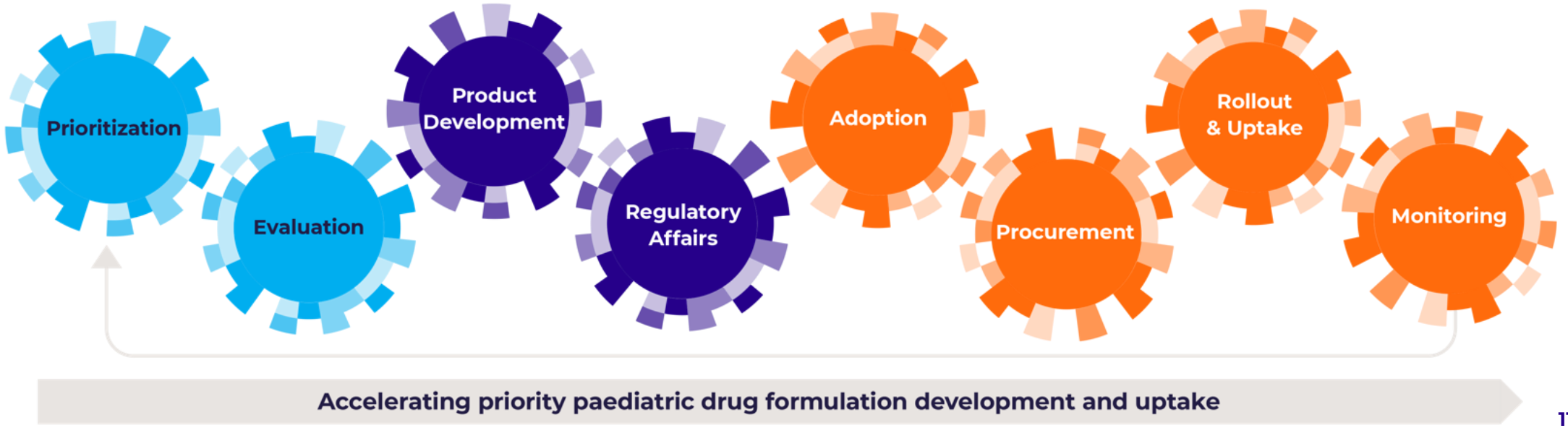
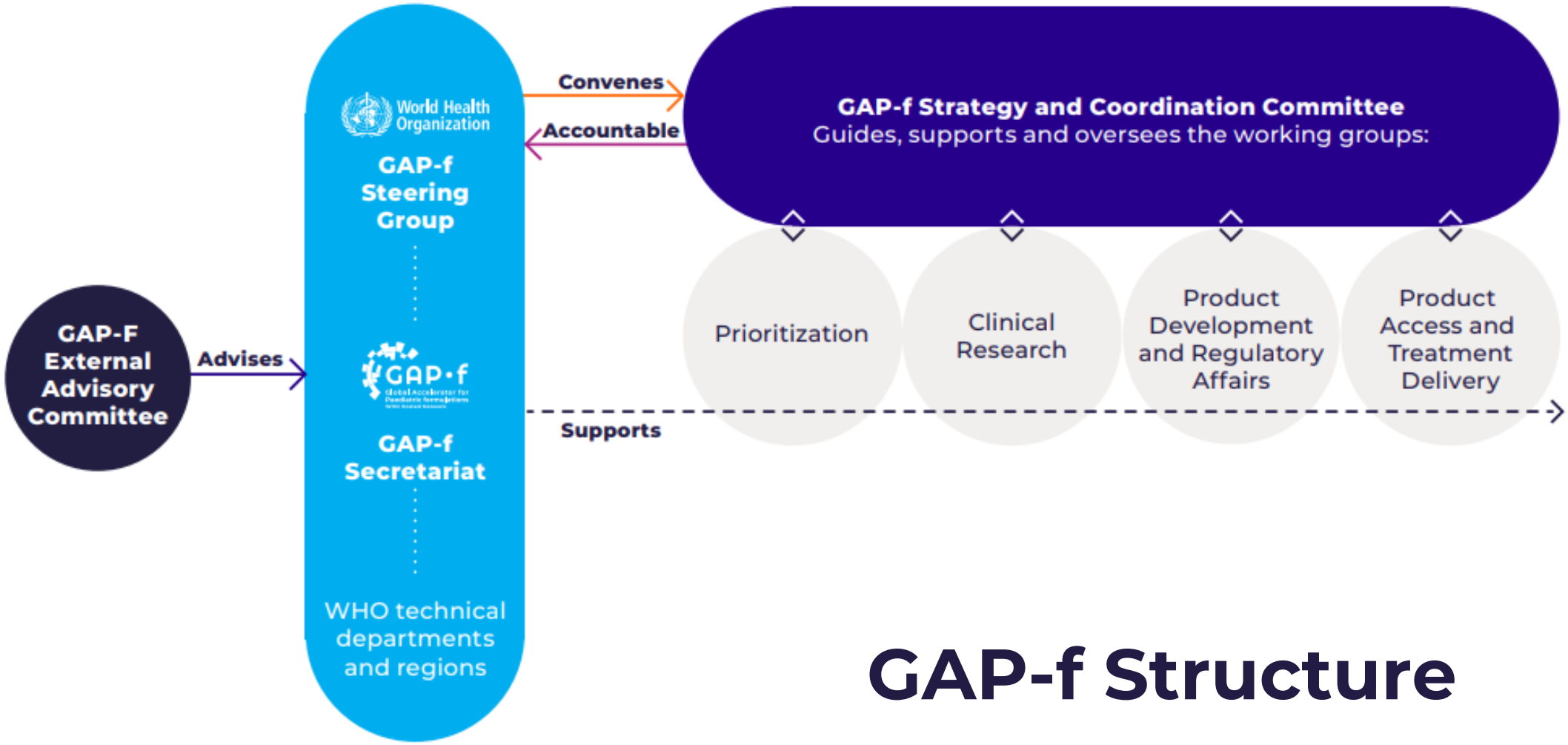


Fig.9. GAP-f governance structure



GAP-f Structure

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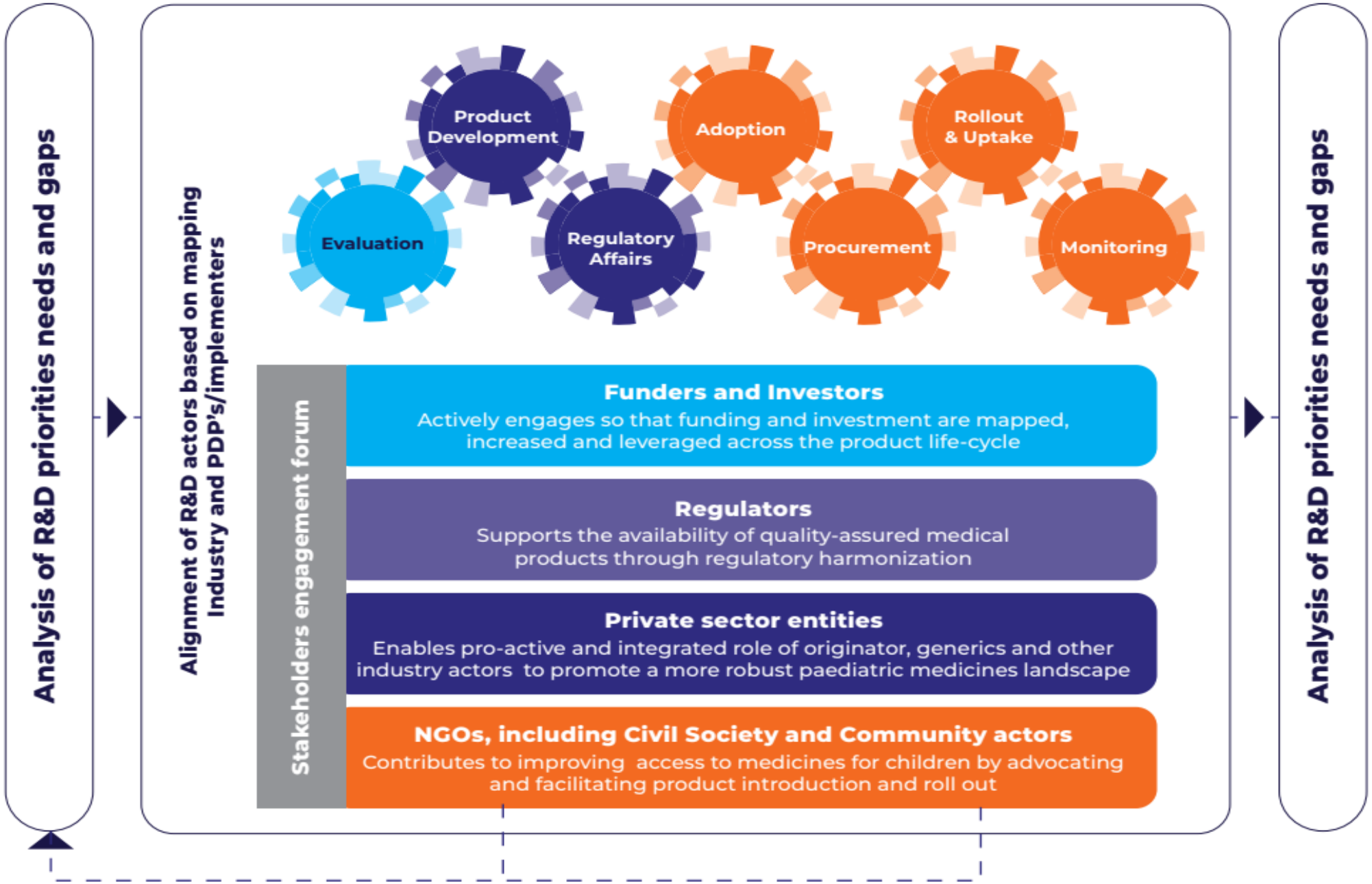
Fig.5. GAP-f stakeholder alignment and mobilization



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GAP-f Phase 1 (2020–2021)

30 institutional partners

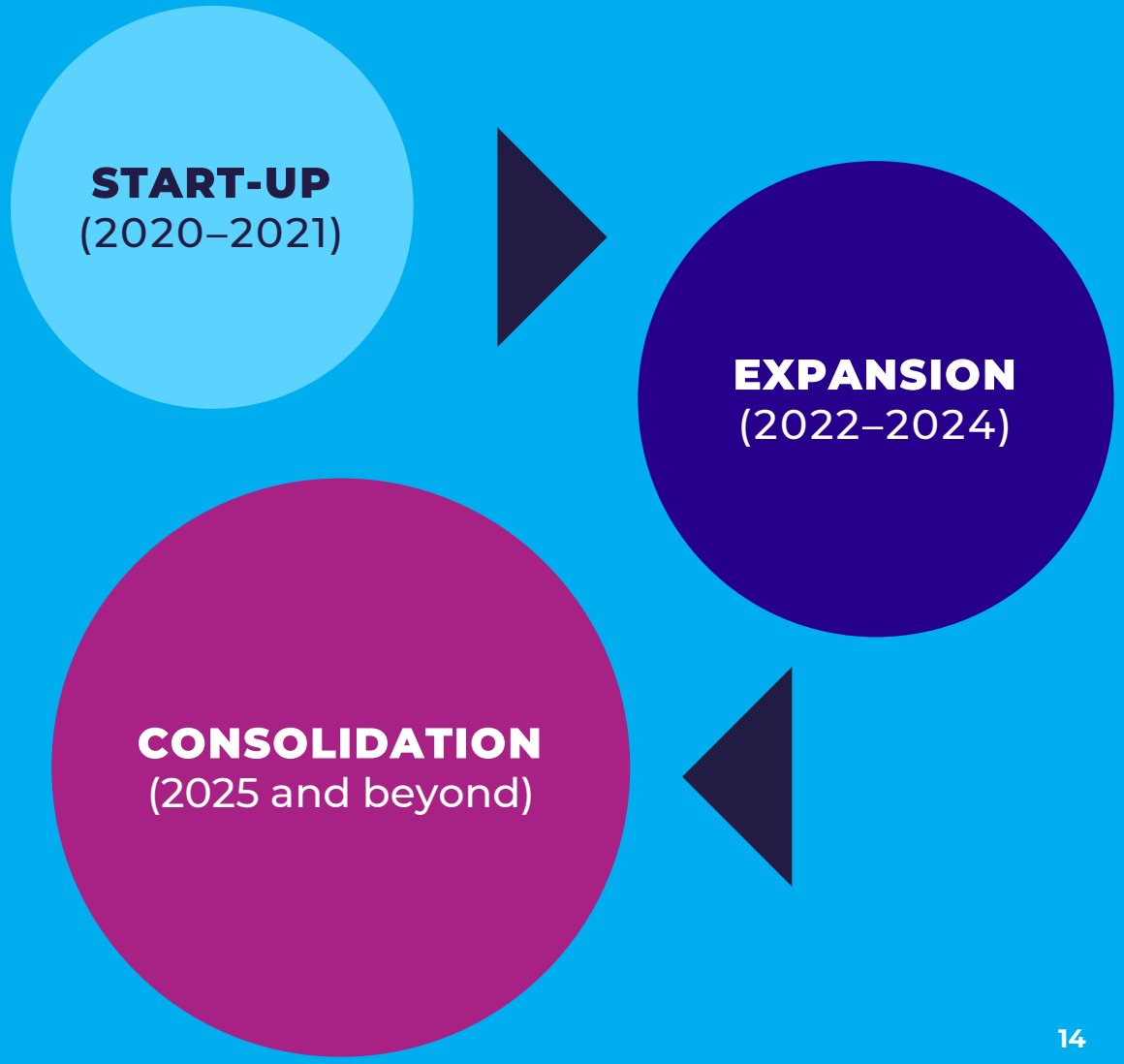
04 product life cycle–targeted working groups

03 disease areas (HIV, TB and hepatitis C) supporting prioritization efforts (PADO guidance) and product-specific activities (weight bands dosing, taste improvements and product introduction)

03 additional exploratory disease areas (childhood cancer, neglected tropical diseases and antimicrobial resistance)

Comprehensive assessment of WHO Essential Medicines List for children (EMLc) initiated

Shaping GAP-f Evolution



GAP-f's diverse group of 33 institutional members

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GAP-f disease focus

Maintain and enhance focus on **HIV, TB, Hepatitis**

Extend catalytic efforts to **Antibiotics, Childhood cancer and selected neglected tropical diseases.**

Actively engage and respond to **urgent needs** (ie. SARSCoV-2) as well as scoping opportunities for support to **malaria and non-communicable diseases** (ie. diabetes, sickle cell and epilepsy).

A STEPPING-STONE TO ADDRESS THE FULL SPECTRUM OF ESSENTIAL MEDICINES IN PHASE 3 (FROM 2025)

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Priority products identified by PADO processes

	Priority products	Watchlist products
Antibiotics	Amoxicillin-clavulanic acid Nitrofurantoin Azithromycin Cefiderocol IV	Cefepime-taniborbactam Sulbactam-durlobactam
Childhood cancer	PADO process underway	
Hepatitis C	Sofosbuvir 100mg (to be used with daclatasvir)	Sofosbuvir / Velpatasvir Glecaprevir / Pibrentasvir
HIV	Dolutegravir 10 mg Abacavir/lamivudine/dolutegravir 60/30/5 mg Darunavir/ritonavir 120/20 mg TAF-XTC ± DTG Long-acting cabotegravir	Islatravir Lenacapavir Broadly neutralizing antibodies Micro array patches
Neglected Tropical Diseases* <u>Focus on 4 diseases</u> 1. Schistosomiasis 2. Human African trypanosomiasis 3. Scabies and onchocerciasis 4. Visceral leishmaniasis	L-praziquantel 150mg and 300mg Acoziborole 320mg Ivermectin 1 or 1.5mg Moxidectin Miltefosine 20mg Amphotericin B	Emodepside Oxfendazole LXE408
Tuberculosis	Rifapentine 150 mg scored dt Rifampicin 100 mg scored dt* Pretomanid Moxifloxacin 100 mg dt	All compounds in Phase IIa/b as of October 2023 Long-acting formulations Rifapentine oral film

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Advocating for accelerated access to better medicines for children: Key elements of advocacy

Sébastien Morin (MPP)

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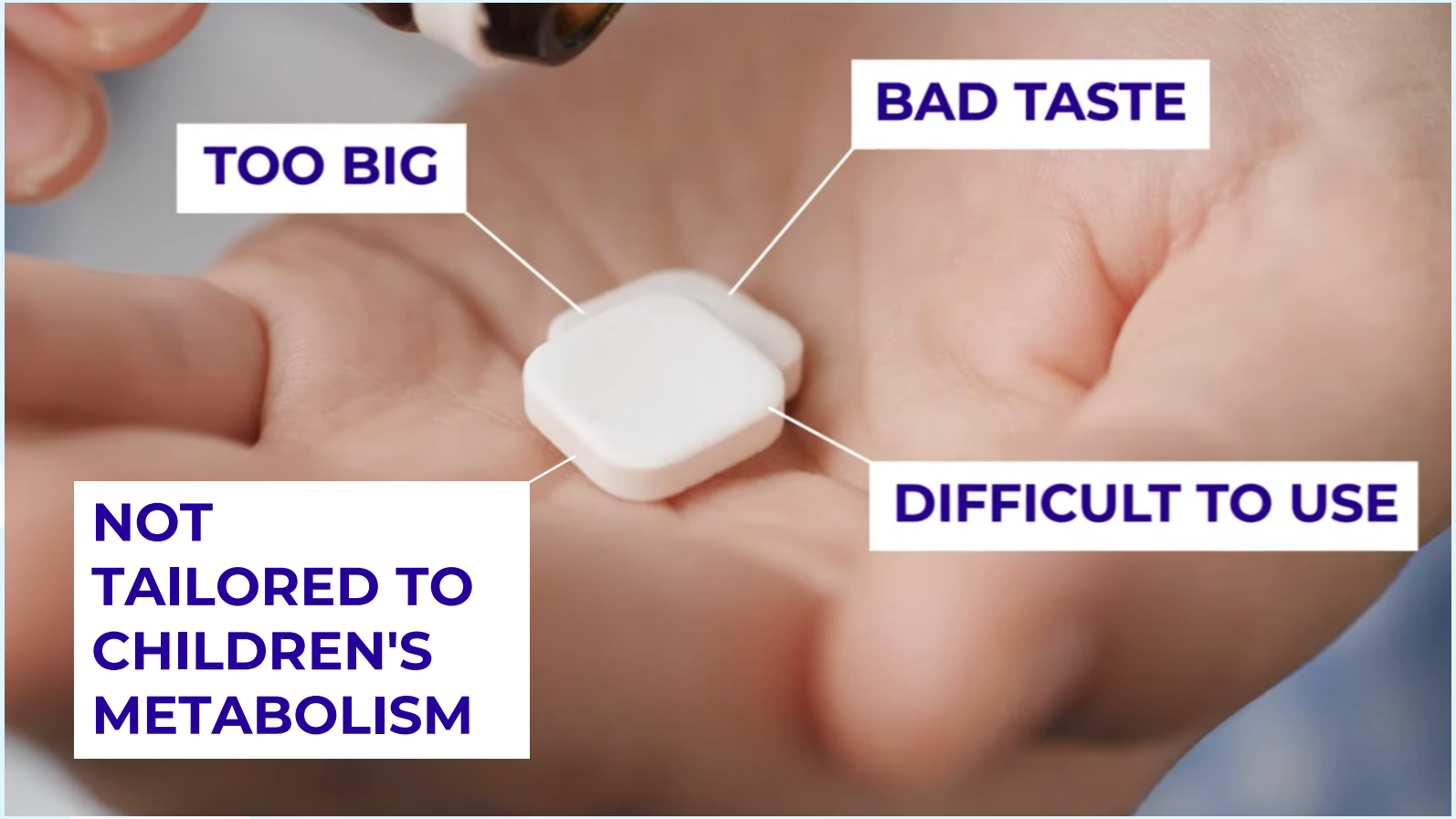
Children need medicines that are adapted to their needs...

But the lack of appropriate medicines is putting their lives at risk...



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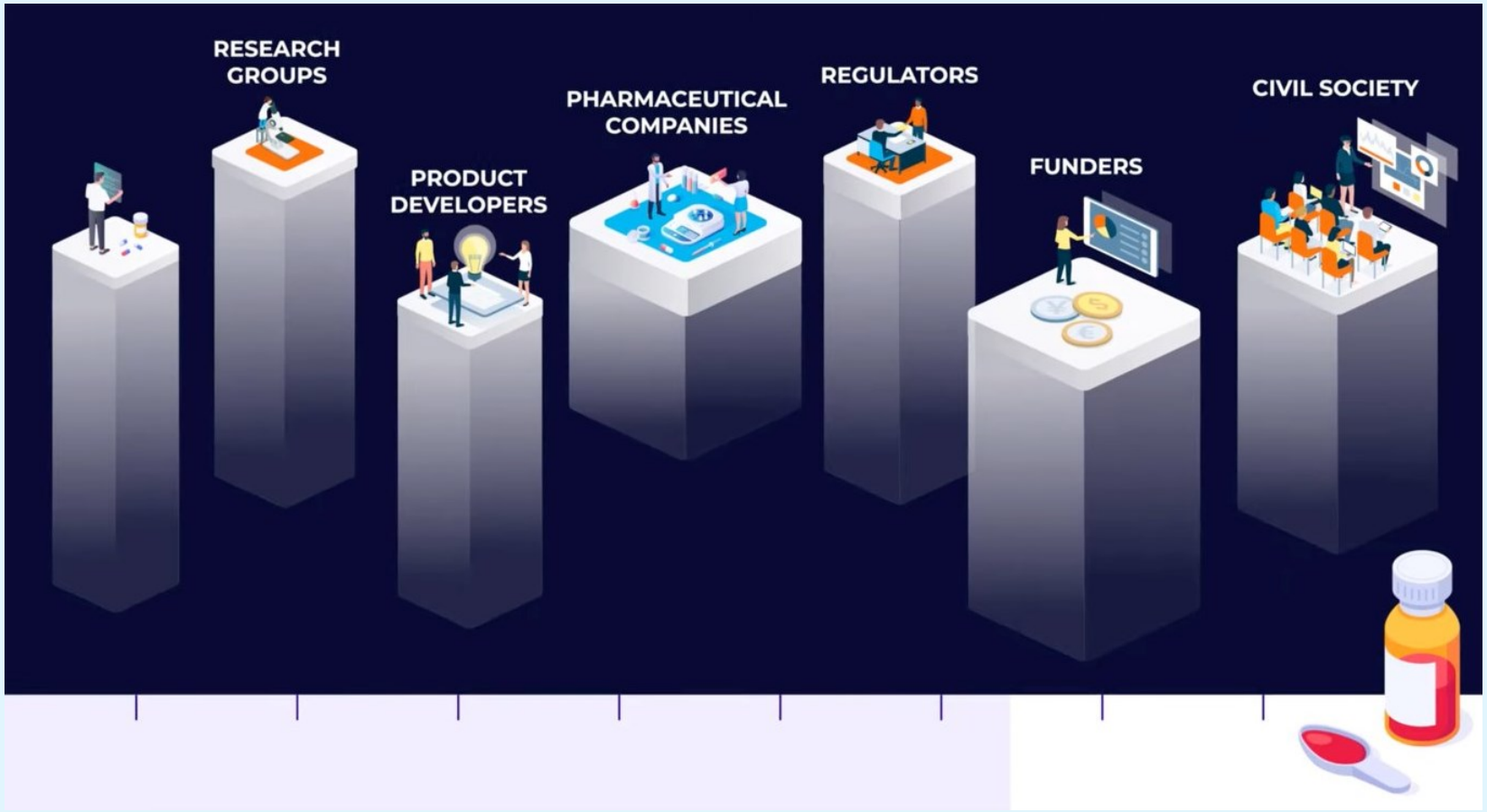
Despite progress in paediatric drug development and access...

Siloed approaches lead to efforts falling through the cracks, especially in a void of global priority-setting of needs, and a lack of coordinated and sustainable funding for paediatric health priorities

Too much **time is lost** in translation

In the fragile product development landscape, one **gap unfilled** can halt an entire life-saving solution



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Despite progress in paediatric drug development and access...

- Paediatric medicines represent low volume markets that disincentivizes companies to invest
- Medicines are generally first developed for adults, and developed only later for children
- In many cases, appropriate paediatric formulations and doses remain inexistant
- There is a lack of global priority-setting and fragmented landscape of innovation and access
- The current approach is siloed, with insufficient attention to upstream or downstream work
- Paediatric-focused clinical research is challenging
- There is a lack of harmonized regulatory guidance for paediatric medicines
- Paediatric drug research, development, and delivery have no dedicated funding mechanisms
- When funding is not aligned across the life cycle, a single gap can bring progress to a halt
- Supply cannot meet demand when demand is small, fragmented, and neither forecasted nor pooled
- Country uptake systems are not always ready for the introduction of new medicines or formulations
- There is limited community mobilization and advocacy to accelerate access to better medicines

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But some solutions exist...

1. **Prioritize efforts!** Focus on priority drugs and formulations that are in the pipeline
2. **Incentivize stakeholders!** Address the problem of a fragmented market leading to the lack of R&D investments and initiatives
3. **Coordinate actions!** Strengthen coordination to accelerate access to prioritized medicines and formulations for children
4. **Invest better!** Mobilize resources to accelerate research and development of better formulations for children
5. **Ensure accessibility!** Accelerate introduction of and sustain access to better and affordable medicines for children in country

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Developing a GAP-f advocacy brief (1 of 2)

Objectives

- **Articulate solutions** to accelerate access to better medicines
- **Contribute** to civil society, community, and other partners' advocacy work at different levels
- **Coordinate, align, and synergize** advocacy actions
- **Help mobilize** leadership and political will
- **Support advocacy to accelerate innovation and access to #BetterMeds4Kids**

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Developing a GAP-f advocacy brief (2 of 2)

Consultative engagement to date

- The idea for a GAP-f advocacy brief to support alignment, messaging, and mobilization of stakeholders at global, regional, and national levels originated in the context of convening the **GAP-f Civil Society and Community Engagement Forum**
- The position paper can help **support** debates, consultations, and meetings, be used and adapted to specific advocacy **efforts by partners**, and support **visibility of potential solutions**
- It **builds on global efforts** (such as WHA and HRC resolutions, platforms and initiatives, progress made in some disease areas, WHO PADO prioritization processes, etc.)
- **Today's discussion can further inform its development and future use**

Please provide any feedback you may have, or express your interest to contribute, during this webinar or after by contacting us at: gap-f@who.int

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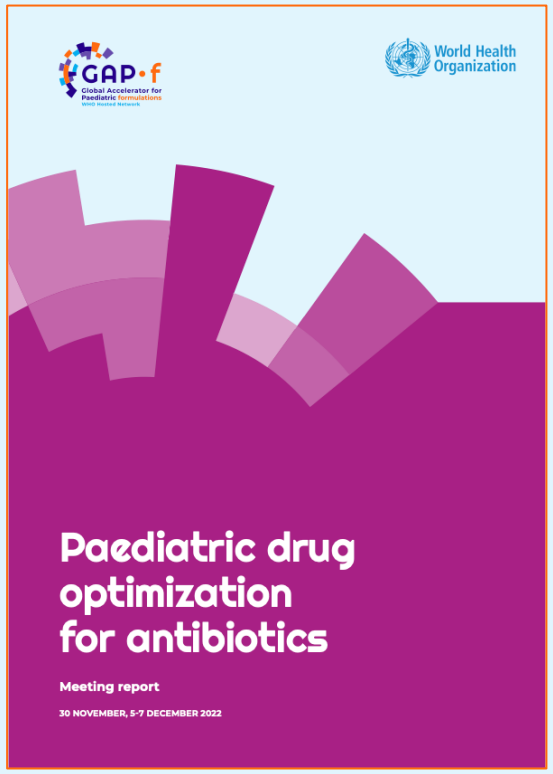
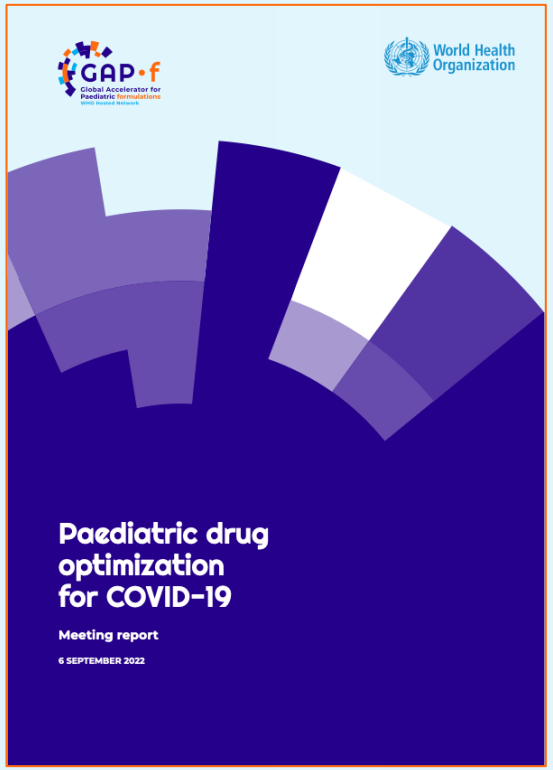
Solution 1: Prioritize efforts!

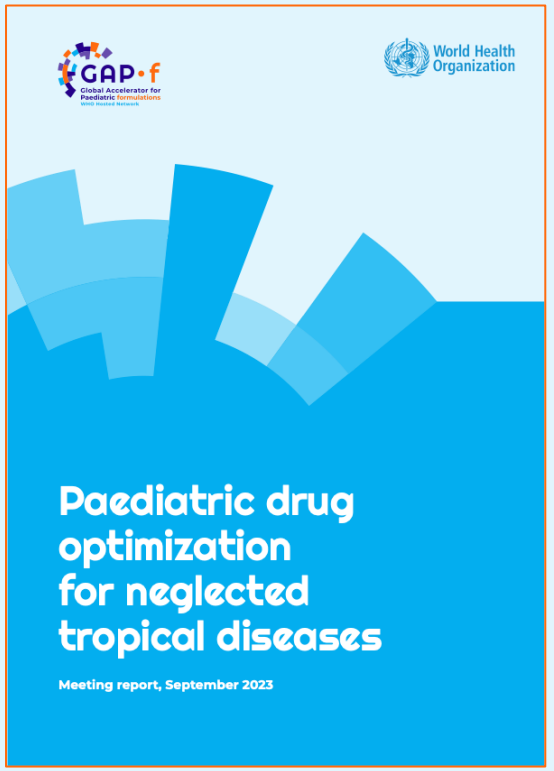
Focus on priority drugs and formulations that are in the pipeline

The Paediatric Drug Optimization (PADO) process is a critical mechanism to **identify priority drugs and formulations** that need to be developed and delivered to fill important access gaps

This can be complemented by **systematically assessing the age-appropriateness of paediatric formulations** included in national essential medicines lists







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Examples of recent Paediatric Frug Optimization (PADO) reports across disease areas
More information at: https://www.who.int/initiatives/gap-f

Solution 2: Incentivize stakeholders!

Address the problem of a fragmented market leading to the lack of R&D investments and initiatives

- **Develop clinical research capacity** and **accelerate the development of guidelines, norms, and standards** for clinical research in children and **ensure feasibility and implementation of results** in the real world
- **Incentivise research and manufacturing** and support companies that commit to paediatric drugs development
- **Simplify and incentivize the paediatric R&D process** by, for example, identifying and **overcoming inefficiencies in regulatory procedures**, and establishing targeted **incentives** such as market entry rewards
- **Streamline regulatory procedures for priority paediatric medicines** by helping to identify the most efficient regulatory route, including through **collaborative registration procedures**
- **Become drivers of access** through access planning by fully integrating plans to **ensure availability, supply, and affordability** into the late stages of the paediatric medicine development process
- **Leverage pooled procurement (global and regional) to address small market challenges** through coordination across procurement agencies, including joint price negotiations and efforts to reduce transaction and operational costs, using **long-term multi-year agreements**, **accurate forecasting**, **demand creation**, and **monitoring of stock outs and supply disruptions**

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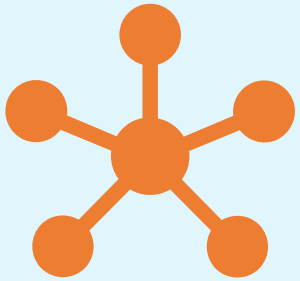
Solution 3: Coordinate actions!

Strengthen coordination to accelerate access to prioritized medicines and formulations for children

- With a fragmented landscape of paediatric drug innovation and delivery, **efforts often focus on tackling just one part of the product life cycle**, with insufficient attention paid to activities upstream or downstream

- The current **lack of a regular and consistent tracking and of needs, gaps, actors, and funding flows** reduces the possibility to align actors and efforts, target interventions, benefit from cross-cutting technologies (e.g., for administration and taste masking) and achieve more coherent and seamless funding streams that allow translation across phases from early R&D to access

- We can contribute to ensuring:
  - **Alignment on priorities and coordinated actions** across the R&D to uptake continuum, and consciously making links between different parts of it
  - **Tracking and accountability** at all levels through the development and follow up of action items at the product/project level, but also from specific resolutions, agreements, debates, and policies



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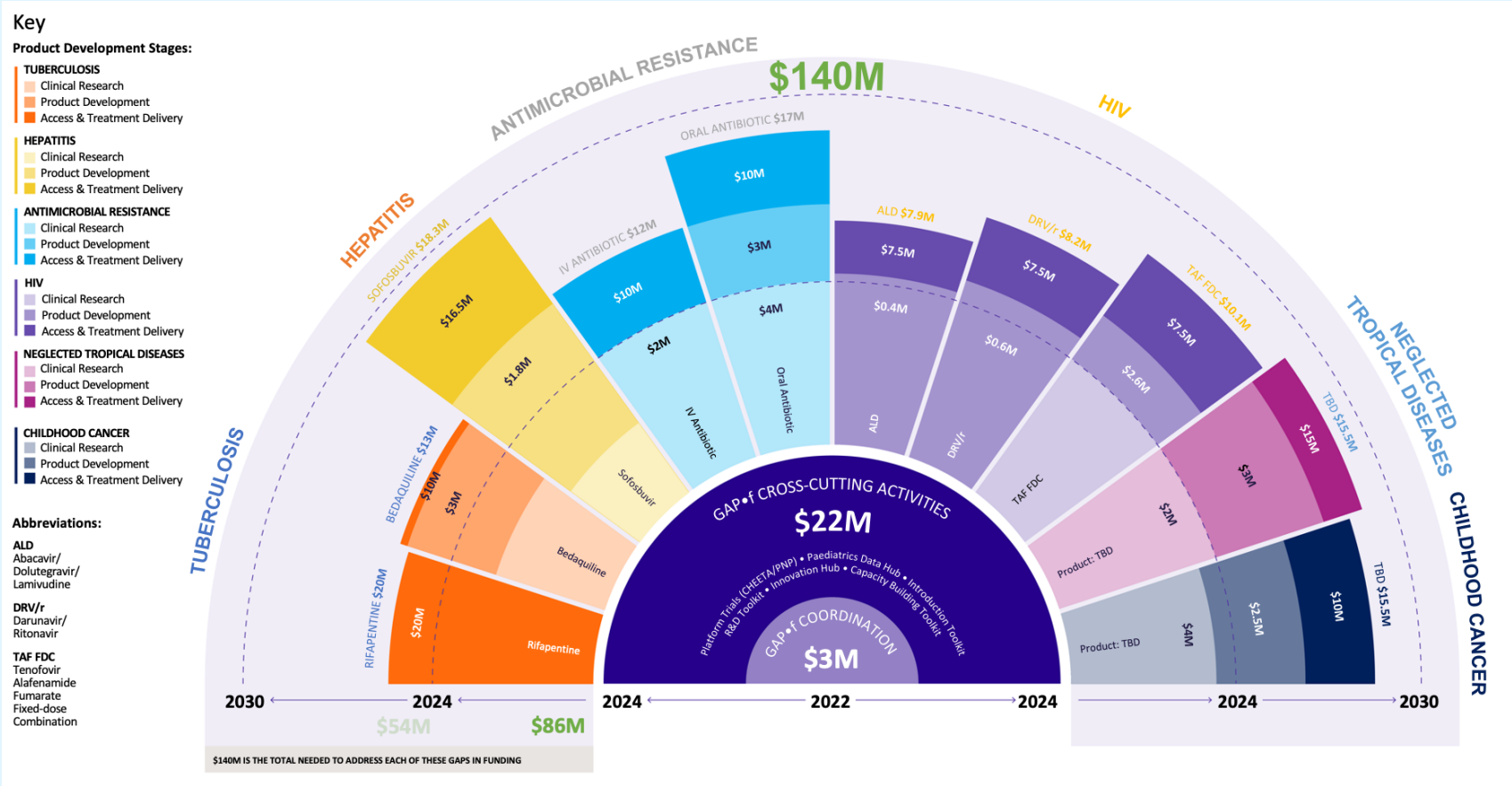
Solution 4: Invest better!

Mobilize resources to accelerate research and development of better formulations for children

- **Funding** for R&D and introduction of optimal formulations for children **is very limited**
- The GAP-f **investment case** sets the funding needs to address a product portfolio of 10 formulations that, if fully funded and realized, could **positively impact >16 million children's lives**



GAP-f estimated funding needs across disease areas

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Solution 5: Ensure accessibility!

Accelerate introduction of and sustain access to better and affordable medicines for children in country

- Introduction of, transition to and sustainability of optimal treatment for children at the country level comes with different challenges that national and other partners need to **plan in advance to ensure system readiness** to introduce and sustain the use of optimal therapies for children:
  - **Policy updates**
  - **Capacity building**
  - **Demand creation**
  - **Affordability**
  - **Procurement**
  - **Distribution**
  - **Monitoring**

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Advocating together: Upcoming opportunities around the WHO EB and the WHA in 2024

Michelle Childs (DNDi)

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Agenda item 12: Acceleration towards the Sustainable Development Goal targets for maternal health and child mortality

- Report from WHO. Main points:
  - Current trends, progress towards coverage of key interventions
    - World is far from achieving universal coverage for these interventions, with the largest gaps involving family planning services, breastfeeding and **treatment of childhood illnesses**.
  - Barriers in reaching 2030 SDG targets
    - Lack of adequate access to quality medicines, equipment and commodities
  - EB discussion will focus on
    - What actions do Member States recommend for accelerating progress towards achieving:
      - Sustainable Development Goal target 3.1 (on reducing maternal mortality)?
      - Sustainable Development Goal 3.2 (on ending preventable deaths of newborns and children and reducing neonatal mortality)?
    - What do Member States propose should be the role of the WHO Secretariat in supporting these actions?

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Draft resolution '*Accelerate progress towards reducing maternal, newborn and child mortality in order to achieve SDG targets 3.1 and 3.2*'

Draft resolution for discussion at EB:

- Proposed by the Federal Republic of Somalia; Co-sponsored by Ethiopia, Paraguay and Tanzania
- Not yet available on WHO EB site, but draft was last discussed between MS on the 11th of Jan
- Broader than R&D and access to medicines for children (access to sexual and reproductive health and rights, nutrition, immunisation, addressing social determinants of health, etc.).
- Current draft contains references to previous resolutions focused on improving access to better medicines for children (WHA 69.20 & WHA 75.8) **plus** mention of GAP-f and R&D needs for pregnant and lactating women
- EB possible outcomes:
  - Agreement to send to WHA with endorsement from EB;
  - Recommendation for further informal discussion with MS prior to WHA to resolve any areas of disagreement;
  - Rejection (unlikely).

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Draft resolution '*Accelerate progress towards reducing maternal, newborn and child mortality in order to achieve SDG targets 3.1 and 3.2*'

Relevant extracts from Chairs proposal as of 4th Jan

Directed to Member States

OP 1.7. Enable access to essential quality medicines for pregnant women, lactating women, mothers, newborns and children through accelerating implementation of the actions laid out in resolution WHA69.20 and WHA75.8 and by promoting, supporting and financing accelerated investigation, development, manufacturing, registration and supply of age-appropriate, quality assured formulations of medicines for diseases that affect mothers, newborns and children;

Directed to the WHO Director General

OP 3.5. Accelerate implementation of the actions laid out in resolution WHA69.20 & WHA75.8, strengthen and expand collaborative efforts such as those promoted by WHO technical departments and the Global Accelerator for Paediatric Formulations (GAP-f) network for securing better access to medicines for children, and report to the 78th World Health Assembly, and subsequently as appropriate, on progress achieved, remaining gaps and specific actions needed to further promote better access to age-appropriate, quality assured, affordable medicines and commodities for pregnant and lactating woman, and for maternal, adolescent, child and newborn health services;

Other opportunities at the EB to highlight R&D and access to medicines for children

- **Agenda item 11: Road map for neglected tropical diseases 2021–2030**
  - Children comprises 34% of the 20 million DALYs resulting from NTDs and face a form of double neglect when it comes to R&D.
  - To address part of this R&D neglect, WHO in November 2023, published a list of priority paediatric formulations for five NTDs that impact children.

Agenda item 13: Antimicrobial resistance: accelerating national and global responses:

- Draft Resolution currently sponsored by Thailand and Mexico. Mentions R&D but not children's needs.
- One out of every five deaths due to AMR are in children under the age of five.
- New treatments for children to address AMR are delayed by at least a decade following approval of new treatments for adults.
- WHO, in March 2023, published list of priority paediatric formulations for AMR

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Potential advocacy options

- Prior to and at EB and WHA
  - Ask Member States to support and retain current language in the resolution on *'Accelerate progress towards reducing maternal, newborn and child mortality in order to achieve SDG targets 3.1 and 3.2*'
  - Review resolution to see whether additional actions are needed and propose to MS.
  - Ask Member states to include children's needs in NTD and AMR discussions/resolutions
  - Make an intervention in support; include in briefings.
  - Others?
- Policy fora outside of EB and WHA
  - UN High Level Meeting on AMR in 2024
  - Pandemic Accord
  - G7/G20
  - National and regional policy forums

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Facilitated discussion: Coordinating possible advocacy actions from now until the WHA in May

Michelle Childs (DNDi)

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Summary and next steps

David Ruiz Villafranca (EGPAF)

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Thank you for participating!
We look forward to continued engagement with you!

#BetterMeds4Kids | @GAP_f_Network

together, stronger, for kids

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