

**Shaping the global innovation
and access landscape for
better paediatric medicines:**

**Global Accelerator for
Paediatric Formulations
2022-2024 business plan**



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Abbreviations

CHEETA	Chasing Expedited and Equitable Treatment Access for Children
DCV	daclatasvir
GAP-f	Global Accelerator for Paediatric Formulations
PADO	PAediatric Drug Optimization
SOF	sofosbuvir
TB	tuberculosis
PK	pharmacokinetic
PPC	Preferred Product Characteristics
R&D	Research and Development
TB	tuberculosis
TPP	Target Product Profile
WHO	World Health Organization

Executive summary

Launched in 2020, the Global Accelerator for Paediatric Formulations (GAP-f) has catalysed the response to the 2016 World Health Assembly Resolution 69.20 on promoting innovation and access to quality, safe, efficacious and affordable medicines for children. During its phase 1 strategy (2020–2021), HIV, tuberculosis and hepatitis C had been identified as priority therapeutic areas, and GAP-f's work leveraged historical and ongoing investments.

By the close of phase 1, 30 partner organizations were engaged and collaborating across four working groups. Three new disease areas had been identified, and the groundwork was laid to enable lessons learned and technology advances from HIV, TB and hepatitis C to benefit new areas, including childhood cancer, neglected tropical diseases and antimicrobial resistance. A comprehensive assessment of the WHO Model List of Essential Medicines for Children was in progress.

In early 2022, GAP-f launched its (phase 2) 2022–2024 Strategy. This business plan further details how this Strategy will be operationalized. For each of its main strategic pillars (Fig. 1) – prioritize and align (see p. 5), accelerate (see p. 13) and intervene (see p. 19) – a dedicated section offers a bird's-eye view of how the work will be carried out, the top-level targets for specific workstreams and the timelines for each and how impact will be measured.¹

The GAP-f governance and operating model section (see Fig. 9 and p. 24) shows how, through the process of developing its phase 2 Strategy, GAP-f has further clarified its unique role in coordinating a broad range of stakeholders and in orchestrating cross-cutting work that can improve and accelerate the development and delivery of medicines for children.

With a refined GAP-f governance and operating model designed to match phase 2 objectives, this section also explains how the various stakeholders will work together, with links to the new terms of reference for the GAP-f Strategic and Coordination Committee and the GAP-f engagement forums. Finally, GAP-f's functions, in turn, also aim to leverage greater funding for prioritized medicines for its growing network of partners and expanded disease focus. To do this, GAP-f provides the budget required to realize the goals of its phase 2 Strategy and this current business plan. An overview of the budget secured and required is provided in addition to the financing needs for GAP-f partners to carry out a shared vision and mission.

GAP-f Vision: All children have equitable access to the medicines they need.

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.



Introduction

Officially launched in 2020, the Global Accelerator for Paediatric Formulations (GAP-f) has catalysed the response to the 2016 World Health Assembly Resolution 69.20 on promoting innovation and access to quality, safe, efficacious and affordable medicines for children (7). It aims to remove a specific bottleneck to the achievement of Sustainable Development Goal 3 across several targets, including universal health coverage. During its phase 1 strategy (2020–2021), HIV, tuberculosis (TB) and hepatitis C had been identified as priority therapeutic areas, and GAP-f's work leveraged historical and ongoing investments. This also provided the foundations to test similarities and explore synergy across disease areas among the start-up phase partners.

By the close of phase 1, 30 partner organizations were engaged in four working groups. Collaborations had been initiated across the three priority disease areas (HIV, TB and hepatitis C), and key cross-cutting activities were supporting product prioritization through PADO; regulatory guidance; more efficient, high-quality clinical research; and increased coordination of product development (with targeted product support provided for formulations through weight bands, pharmacokinetics and taste improvements) and introduction efforts. Three new disease areas had been identified and the groundwork was laid to enable lessons

learned and technology advances from HIV, TB and hepatitis C to benefit other disease areas, notably childhood cancer, neglected tropical diseases and antimicrobial resistance. In addition, a comprehensive assessment of WHO Model List of Essential Medicines for Children was called for and initiated.

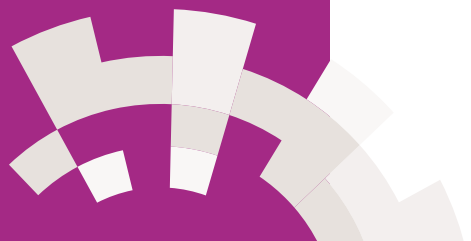
The phase 2 GAP-f Strategy, launched in March 2022 (2), solidifies the foundations laid during phase 1 (2020–2021) while bringing a more refined approach to enhance GAP-f's added value through a transparent priority-setting process and gap analysis to help to reduce siloed approaches and improve funding streams. It also paves the way for more cross-learning across products and disease areas to accelerate drug development and delivery and for identifying specific gaps that require intervention to remove innovation roadblocks.

The strategy also provides for a broadened disease scope, partner base and stakeholder engagement for GAP-f to deliver impact through a three-tiered framework: prioritize and align; accelerate; and intervene (Fig. 1). Phase 1 comprised the GAP-f start-up and activities, and the phase 2 GAP-f Strategy and this accompanying business plan aim to efficiently expand the GAP-f disease scope and reinforce the cross-cutting work, ultimately paving the way for consolidating an optimized operating model and functioning of GAP-f over the longer term.



Abstract geometric shapes in the top left corner, including a large light beige 'X' shape and several overlapping squares and rectangles in shades of beige and white.

Operationalizing the GAP-f 2022- 2024 Strategy



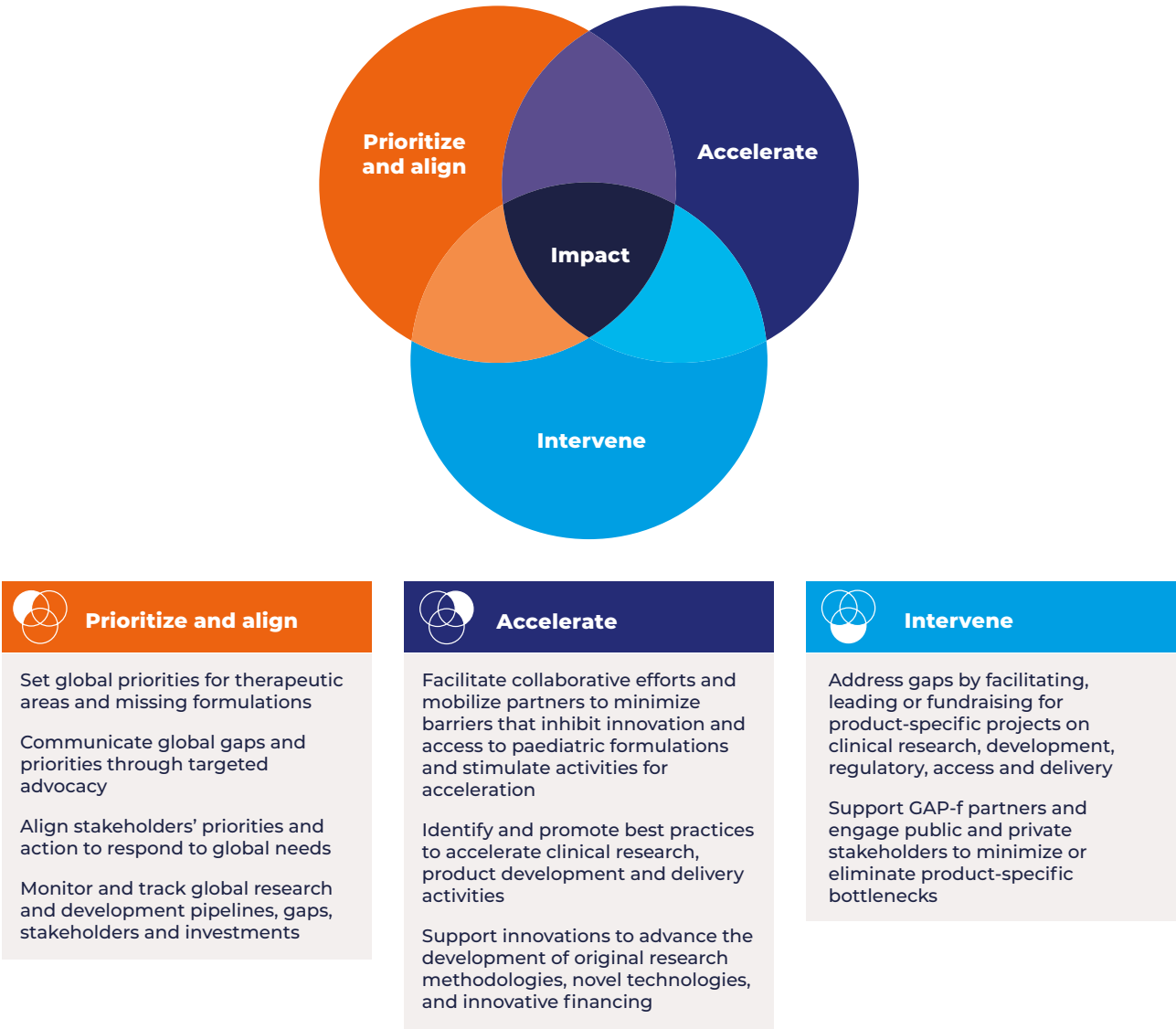
This business plan outlines the operationalization of the GAP-f phase 2 Strategy. To achieve impact, GAP-f will require ramped-up resources both for the GAP-f Secretariat and for the work undertaken by GAP-f partners to achieve its strategic objectives (see Annex 1, p. 38). Ultimately, over the longer term, success will be measured by the reduction of mortality and morbidity among children through access to safe, effective, high-quality, affordable and available essential medicines for children. This business plan paves the way to achieving such impact through intermediate goals outlined in each section below that contribute to overarching impact indicators (see measuring impact, p. 22).

Through a stronger collaborative, multisectoral approach based on shared principles, GAP-f is evolving towards a more fit-for-purpose governance and operating model as a critical

component of this business plan. As a WHO network and in accordance with the structure put forward in the GAP-f phase 2 Strategy, GAP-f is hosted and thus rooted within WHO, but its operational model is designed to ensure that coordinated action is shaped and effectively implemented in a way that capitalizes on the breadth and depth of expertise that its partners bring. Flagship projects will test the collaboration among its partners and offer concrete examples of areas in which GAP-f partners lead and contribute to a range of activities.

Finally, the ability to achieve the strategic objectives laid out in the strategy is tied to harnessing political commitment, adequate financial resources and appropriate and meaningful engagement of public and private partners over the coming three years.

Fig.1. GAP-f strategic framework





Operationalizing pillar 1: prioritize and align

The work attached to the first pillar stems from the paramount importance of a transparent and robust method for global priority-setting and needs identification across paediatric therapeutic areas. Identifying missing paediatric formulations is a first step to mobilization, to be matched by mapping the pipeline of specific projects that each stakeholder conducts at each stage of research, development and delivery: in other words, who is doing what and when for each stage. WHO's role in this strategic pillar is key to convening global expertise and leveraging the work led by its technical departments, existing systems and processes to further GAP-f specific goals:

- to develop a global priority-setting approach, informed by global disease burden and anticipated public health needs, that will result in timely engagement across therapeutic areas and a list of prioritized missing formulations and indications;
- prioritize products needed within at least three new therapeutic areas, including antimicrobial resistance, childhood cancer and neglected tropical diseases;
- to facilitate a system to monitor and track global children-focused research and development, including gaps, actors and financing;
- design and implement targeted advocacy to align and mobilize key stakeholder groups: WHO Member States, affected communities and patient groups, civil society, research networks, product development partnerships, international organizations, private sector entities, regulatory agencies and donors; and
- elevate and promote partner and stakeholder contributions to paediatric medicines innovation and access as related to the overall GAP-f vision and mission.

Areas of focus and operationalization

Global priority-setting and product prioritization

The process by which GAP-f determines priorities for its work is embedded in existing global processes. The global burden of disease will be analysed through the lens of the global burden of disease among children as a starting-point. Within the disease burden of children, the research and development needs and gaps for paediatric medicines will be analysed, building on the critical input of end-users such as health-care providers and caregivers. These aspects will be gathered and analysed through a prioritization framework to guide timely and targeted engagement across disease areas. Disease-specific activities will first leverage ongoing analysis on the appropriateness of existing products included in the WHO Model List of Essential Medicines for Children. The goal is to identify suboptimal formulations that may require removal from the list and to identify formulations used in countries that may be optimal and valuable additions to the WHO Model List of Essential Medicines for Children.

GAP-f's own priority-setting work will begin where products or formulations are missing or require optimization: for example, identifying gaps in clinical evidence, prioritizing dispersible tablets over bulky syrups or enabling formulary consolidation with flexible dosage forms to limit market fragmentation. The prioritization framework will guide timely initiation of the PAediatric Drug Optimization (PADO) standard procedure (3) for specific disease areas and will support GAP-f partners in enhancing their focus and targeting their work directly at the products given priority to address the most pressing gaps and needs. The PADO process previously developed for HIV and adapted for TB and hepatitis C will be leveraged to identify research and development priorities in the areas of neglected tropical diseases, childhood cancer and antibiotics (Fig. 2). Additional targeted PADO processes will also be considered for COVID-19 and hepatitis B. Dissemination of the PADO priority products and their inclusion in

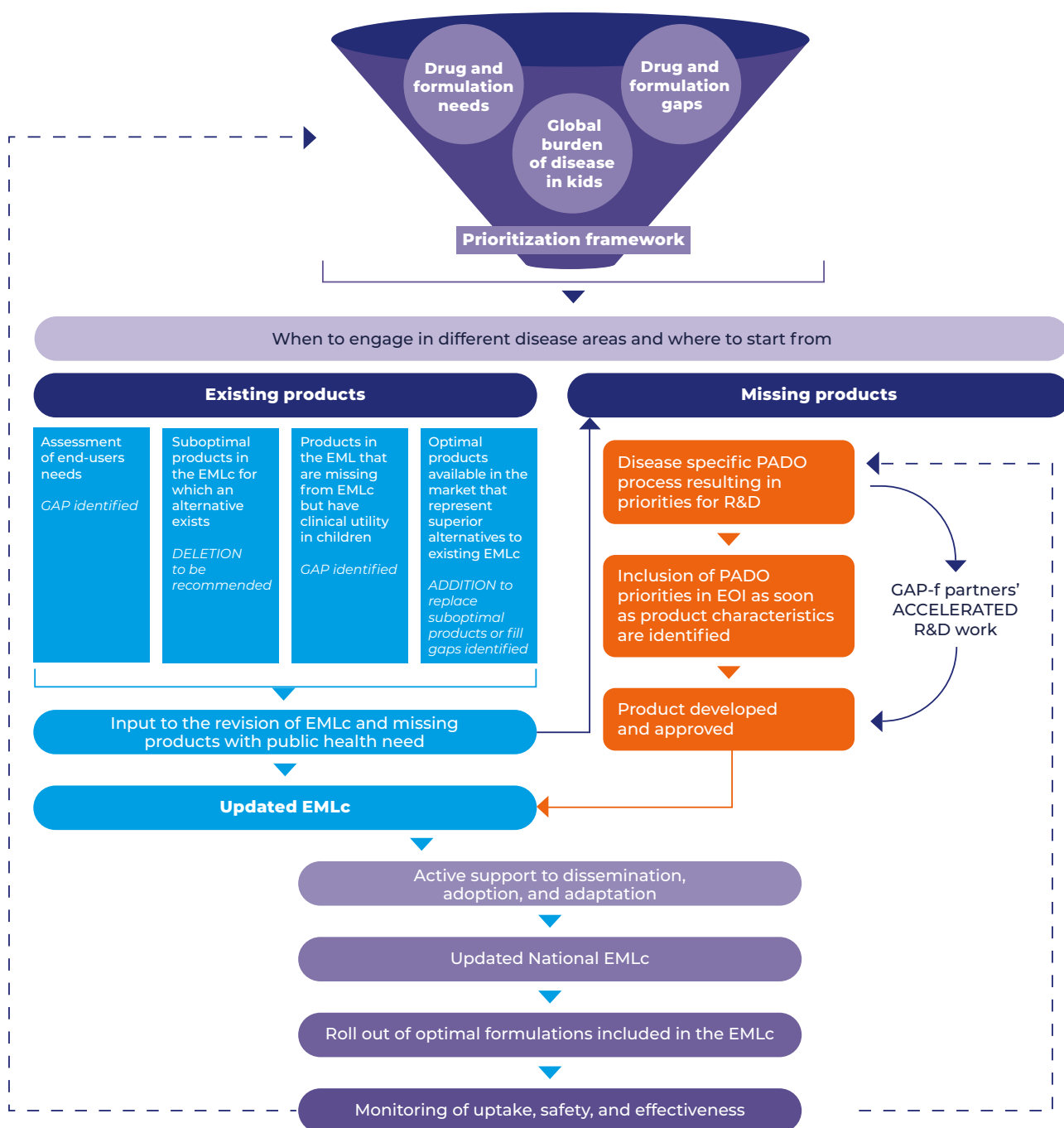


the WHO Expression of Interest List, as soon as preferred product characteristics are identified, will increase global alignment and contribute to targeted research and development work among several stakeholders. It will ultimately enable more rapid and wider access to new paediatric formulations by better targeting WHO prequalification assessments.

Once products become available, an update will be provided through the global WHO Model List of Essential Medicines for Children revision process so that the best available formulations can be effectively given priority globally and

at the country level. The updated version of the WHO Model List of Essential Medicines for Children will be actively disseminated to promote the updating and revision of national essential medicine lists with guidance to be developed in support of roll-out. Finally, monitoring the adoption and adaptation of the WHO Model List of Essential Medicines for Children and the use, safety and effectiveness of newly introduced products will enable systematic feedback to the global prioritization process across and within therapeutic areas.

Fig.2. GAP-f prioritization process



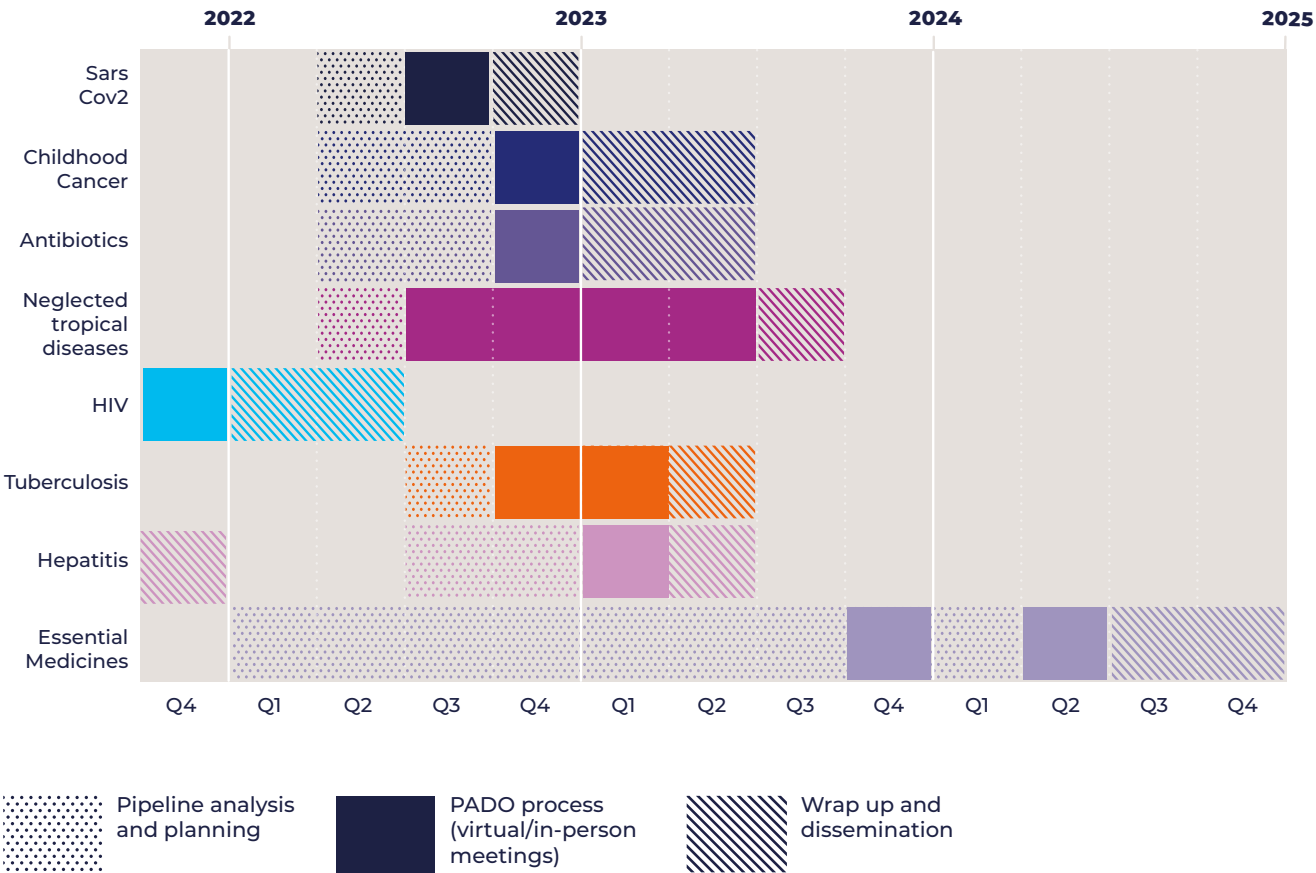
This work will be facilitated by the GAP-f Prioritization Working Group (see page 26), and disease-specific prioritization (PADO processes) will be led by WHO technical departments with contributions from relevant GAP-f partners. This work aims at more timely engagement across therapeutic areas and the development of a list of prioritized missing formulations. In turn, the list of prioritized products will enable acceleration, transparency, advocacy and resource mobilization.

The critical PADO processes conducted for paediatric HIV, followed by hepatitis C and TB, and the lessons from this work indicate a crucial need to continue applying and adapting PADO process to each new disease area taken on by GAP-f, both in communicable and

noncommunicable disease fields. The process brings together a wide range of stakeholders and follows a sequence of various assessments of gaps and opportunities, including focused clinical research, technology landscapes, available formulations, drug pipelines and access and market scanning, to name a few.

The process is robust and the outputs concrete: a priority list of formulations for the short term (3–5 years); a watch list for the medium term (5–10 years); and finally, a targeted research agenda to fill gaps. Key targets over the next three years include COVID-19 (ongoing), selected neglected tropical diseases, childhood cancer and antibiotics. Existing PADO lists for HIV, TB and hepatitis will be reviewed and revised according to needs.

Fig.3. PADO process plan



Mapping drug pipelines, actors and funding

To map and track work being done on paediatric innovation, access and delivery, GAP-f is working with the WHO Global Observatory on Health Research and Development (R&D) (4) to develop a coherent and end-to-end approach to monitoring research and development on children's health. This will highlight progress and expose the gaps to be filled, including gaps in funding. Also, the Observatory (4) and WHO technical departments will work with GAP-f to develop a global dashboard for paediatrics, which currently does not exist, as well as more specific dashboards reflecting the various stages of the product life cycle for GAP-f priority disease area products. If and where a specific dashboard does exist (such as for TB), the global dashboard will direct the user to appropriate web pages.

The images below reflect the current dashboards, from which new dashboards will be developed for specific disease areas for children.

In accordance with the current practice of the Observatory, this system of monitoring for the paediatric medicines pipelines will draw from any existing data sources available. When needed, the GAP-f Network will conduct additional data collection. Further, for tracking the funding pipeline with maximum precision, partnerships will be explored, to avoid duplication and capitalize on tried and tested methods for collecting and analysing data for research and development funding and gaps.

Fig.4. Illustrative examples of WHO Global Observatory on Health R&D dashboards



Align and mobilize key stakeholder groups

The GAP-f mapping work enables transparency around needs and gaps – whether that be data, products, actors, or funding. It serves to align funders and implementers and supports decisions for new investments in research and development and capacity strengthening. To catalyse this and determine more focused future action, GAP-f's coordination role will extend beyond its close partner base through more robust stakeholder engagement, communication, and advocacy. Therefore, in this strategic period, a stakeholder engagement platform will be created as a mechanism to reach out and consult with various key stakeholder groups. The GAP-f Network facilitates partnerships and alliances across the paediatric medicines landscape to align all groups, overcome barriers and/or fill gaps, share lessons and practices and identify opportunities for GAP-f to complement their work. The new Stakeholders Engagement Forum will allow for outreach and periodic interaction with regulators, funders and investors, civil society and community organizations, and private sector entities. Through this platform convened by WHO according to the existing framework of engagement with non-state actors (FENSA), the GAP-f Network will engage in dialogue sessions and in this way, further contribute towards shaping the paediatric medicines landscape in an inclusive way.

These dialogue sessions will occur separately within each stakeholder group and connect as deemed appropriate with the goal of fostering mutual understanding on the scope of work, priorities, challenges and acceleration of work on innovation of and access to paediatric medicines. The outcomes of these dialogues will feed into the deliberations of the Strategy and Coordination Committee (SCC) as deemed appropriate and offer opportunity for enhanced collaborations (see page 25).

Four stakeholder groups will be convened in dialogue sessions under the broader Stakeholders Engagement Forum as described below:

1. Dialogue sessions with Regulators:

The GAP-f dialogue sessions with regulators will occur through the existing WHO Paediatric Regulatory Network (PRN) to build synergies with an already well functioning WHO mechanism.

PRN is a global network of regulators and other interested stakeholders serving as a forum for exchange of regulatory information on medical products for children. It aims to support the availability of quality-assured medical products for children by facilitating communication, collaboration, training and regulatory harmonization across the development, registration, marketing authorization and pharmacovigilance of medical products for children. The goal is to streamline regulatory processes for smooth progress of priority formulations and to promote the WHO principle of good reliance practice (5) for a more efficient approach to regulatory oversight, thereby improving access to quality-assured, effective, and safe medical products over the entire life cycle of drug development.

2. Dialogue sessions with Funders and Investors:

The dialogue sessions with the funders and investors will support the implementation of the GAP-f Strategy and will improve access to medicines for children by contributing to and promoting stronger, more diverse funding and investment following the shared principles of the GAP-f Network. The intent of the sessions with this stakeholder group is to contribute to the development of a shared vision for the evolving GAP-f Network funding models, including the development of innovative funding mechanisms that can result in better medicines for children.



These dialogues will provide a platform for exchange on high-level progress updates from GAP-f Network partners to support the evolution of the GAP-f Network, its priorities and progress made. Through dialogue and discussion with other funders, a space will be created to promote coordination and donor alignment in addressing the global priorities of the GAP-f Network and to advocate on the financial needs and gaps in medicines for children. The ultimate goal will be to generate interest, foster and enlarge support by funders and investors to diversify and increase the overall resourcing of the GAP-f Network.

3. Dialogue sessions with NGOs, including Civil Society and Community Actors:

Dialogues sessions with NGOs, including civil society and community actors, will support the implementation of the GAP-f Strategy and contribute to improving access to medicines for children. These sessions will allow:

- to undertake political advocacy and awareness-raising activities with NGOs, including civil society and community actors;
- to contribute to synchronized action to leverage and elevate the work of the GAP-f Network, and to create awareness and momentum that helps address needs and gaps in paediatric medicines and optimal formulations;
- to leverage interest and to build relationships with NGOs, including civil society and community actors, that work on GAP-f priority disease areas;
- to engage with GAP-f working groups, to support their activities and to promote the outcomes of their work;

- to actively contribute to measuring the impact of GAP-f work and to influence its future shaping by spurring and enabling community-led monitoring efforts on the ground; and
- to link with other GAP-f mechanisms and to contribute to creating a movement that fosters the development of and better access and delivery of paediatric medicines, especially in low- and middle-income countries.

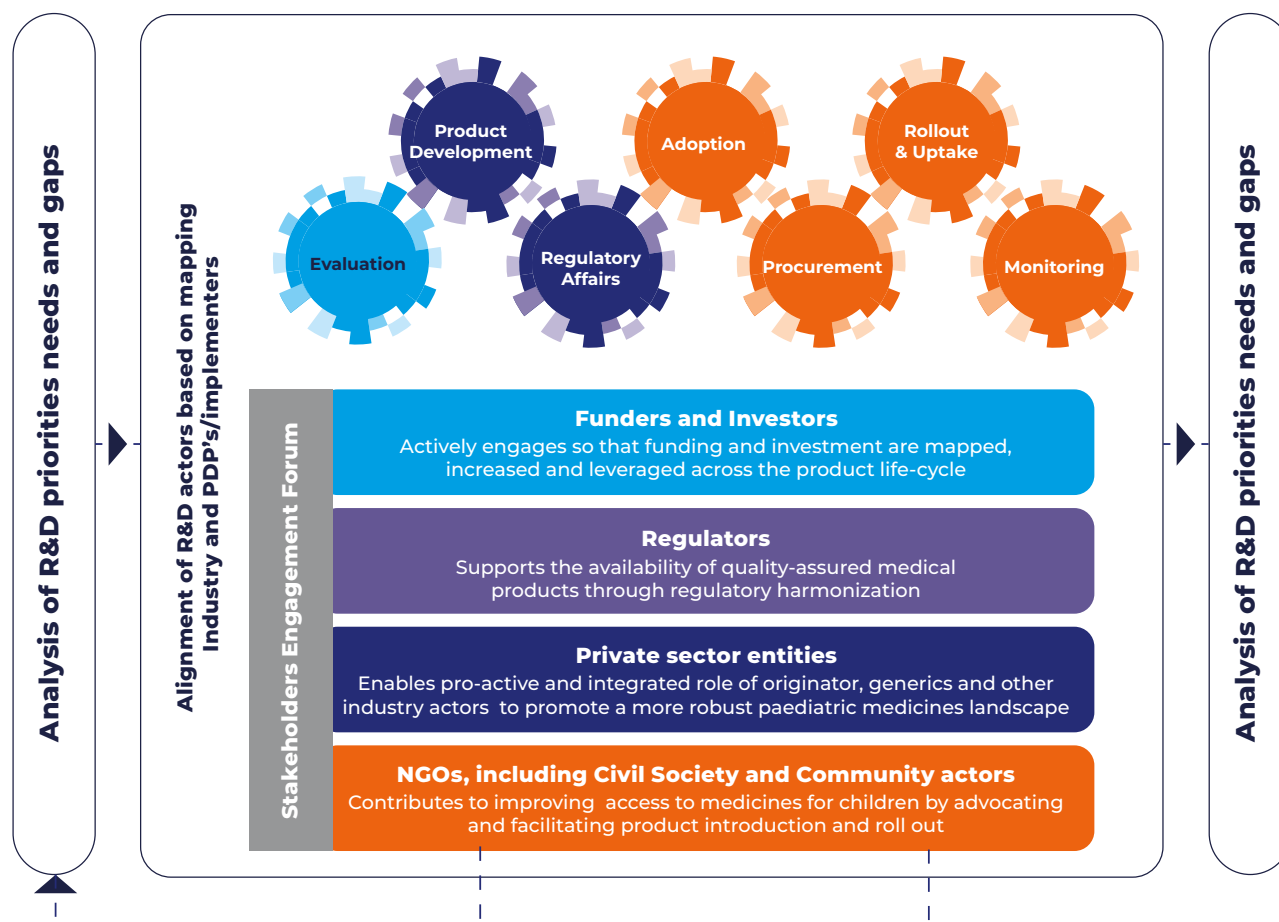
4. Dialogue sessions with Private Sector Entities:

Dialogue sessions with private sector entities in particular originator and generic pharmaceutical companies, will focus on supporting the GAP-f Network to better articulate and integrate the role of originator, generics and other industry actors, in the current paediatric medicines landscape. Through these regular sessions with private sector entities, GAP-f will attain a better grasp on their challenges and capitalize on the wealth of experience and technical capabilities across the value chain, with a focus on priority medicines for children. Accelerating product development by reducing the time it takes from prioritizing products to market introduction is a key strategic pillar for GAP-f, and this engagement with private sector entities will support this goal by:

- exploring, identifying and advocating for targeted interventions across the value chain and agreeing on partnering for launching programmes that address unmet public health needs;
- contributing to designing and shaping sustainable and innovative incentive mechanisms to accelerate paediatric products' progress across the value chain; and
- partnering to minimize or eliminate process bottlenecks.



Fig.5. GAP-f stakeholder alignment and mobilization

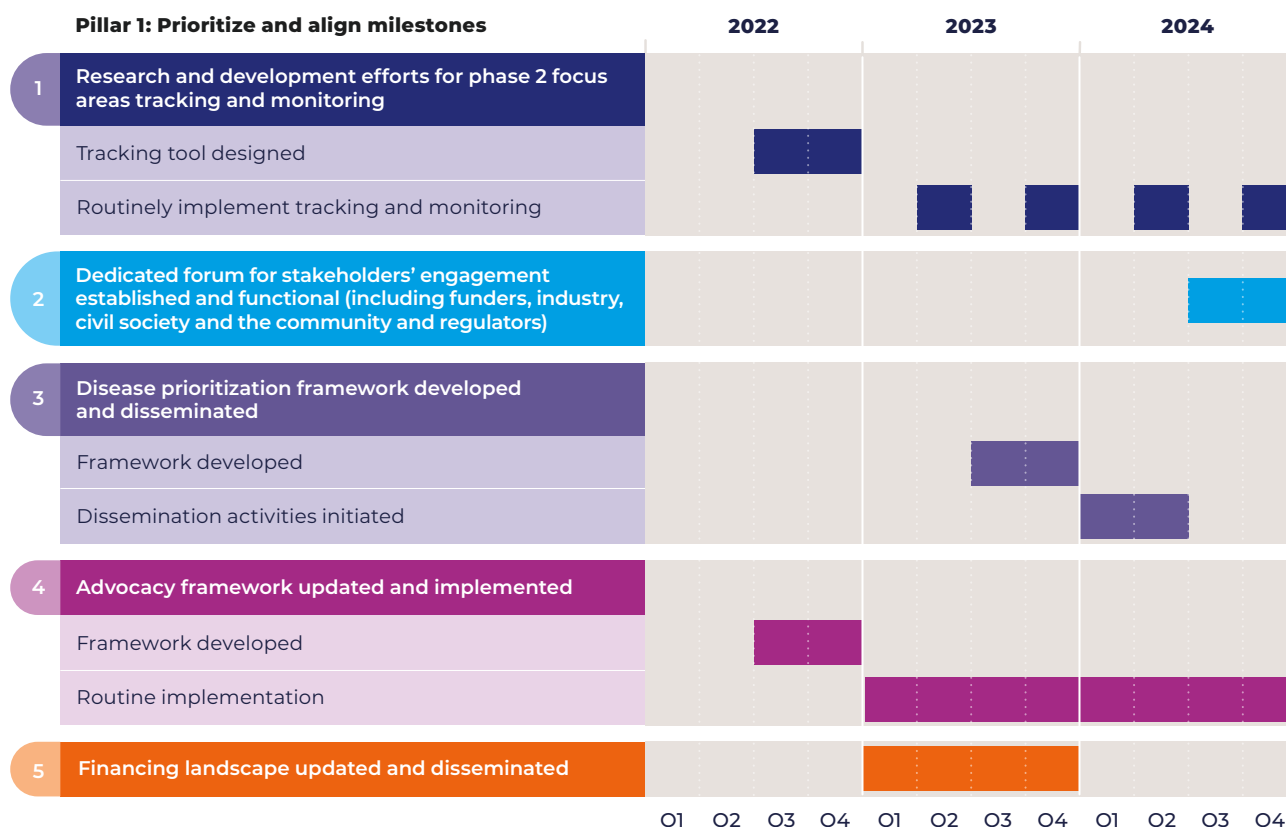


The GAP-f Secretariat will coordinate engagement with various stakeholders and relevant partners within the Strategy and Coordination Committee (see page 25). This includes targeted outreach to key stakeholder groups, hosting events and publishing on the needs, gaps, successes and failures. To further communicate gaps (and opportunities) and encourage collaboration, GAP-f will regularly communicate with and convene its full membership and four dedicated stakeholder forums listed above (Fig. 5).

A tailored strategy for this alignment and mobilization will determine the key targets

to ensure that GAP-f's work is enhanced, ultimately through robust policies or political declarations and funding. All stakeholder groups will be mobilized, including WHO Member States, affected communities and patient groups, civil society, research networks, product development partnerships, international organizations, industry, regulatory agencies, donors and investors. One approach to promoting partner and stakeholder contributions is through a coordinated communication strategy that will help to promote partner and stakeholder contributions to ensure better alignment and impact.

Key milestones



Impact indicators

The global needs, prioritization framework and GAP-f focus areas being well defined widely communicated and broadly accepted

A system for global progress of transparent and public monitoring and tracking should be in place to enable stakeholder alignment and targeted action to accelerate research on, development of and introduction of medicines for children. This will be measured by periodically updating progress indicators. Alignment will be measured based on new investments and initiatives matching the priority products identified through PADO processes. All of this will be tracked through the Observatory.

Measurable increase in the funding dedicated to medicines for children and broader diversity of funding sources: GAP-f partners better funded to deliver against GAP-f priorities

This will be measured by a methodology to attain the global need of funding required for priority products, assessing the current investments and measuring the increase in resources allocated to filling priority gaps. In addition, a donor and investor landscape will serve as a baseline, and new donors and investors will be measured over the three-year period based on established criteria for inclusion.

Number of countries reporting on inclusion of the optimal formulations of the WHO Model List of Essential Medicines for Children in their national essential medicines lists

This will be measured through periodic surveys led by WHO with facilitation by GAP-f partners across WHO Member States to monitor and evaluate the adoption and adaptation of the WHO Model List of Essential Medicines for Children for children to national essential medicines lists.

Key people and partners

The GAP-f Secretariat will coordinate this work with relevant partners within the Strategy and Coordination Committee in conjunction with focal points in WHO technical departments. This stakeholder engagement function will require the contribution of the entire Strategy and Coordination Committee and additional stakeholders that will contribute to the running of various dialogues under the remit of the stakeholders engagement forum. Over the three-year strategy period, additional support will be required for fundraising and monitoring functions in anticipation of the increased needs these components will present.

Resource needs

The overall budget for this pillar for 2022–2024 is US\$ 6 million, which will support the core function and activities of the GAP-f Secretariat (about US\$ 2 million and US\$ 1.5 million, respectively), the contribution of relevant WHO staff members and the GAP-f Strategy and Coordination Committee member organizations (about US\$ 2.5 million).



Operationalizing pillar 2: accelerate

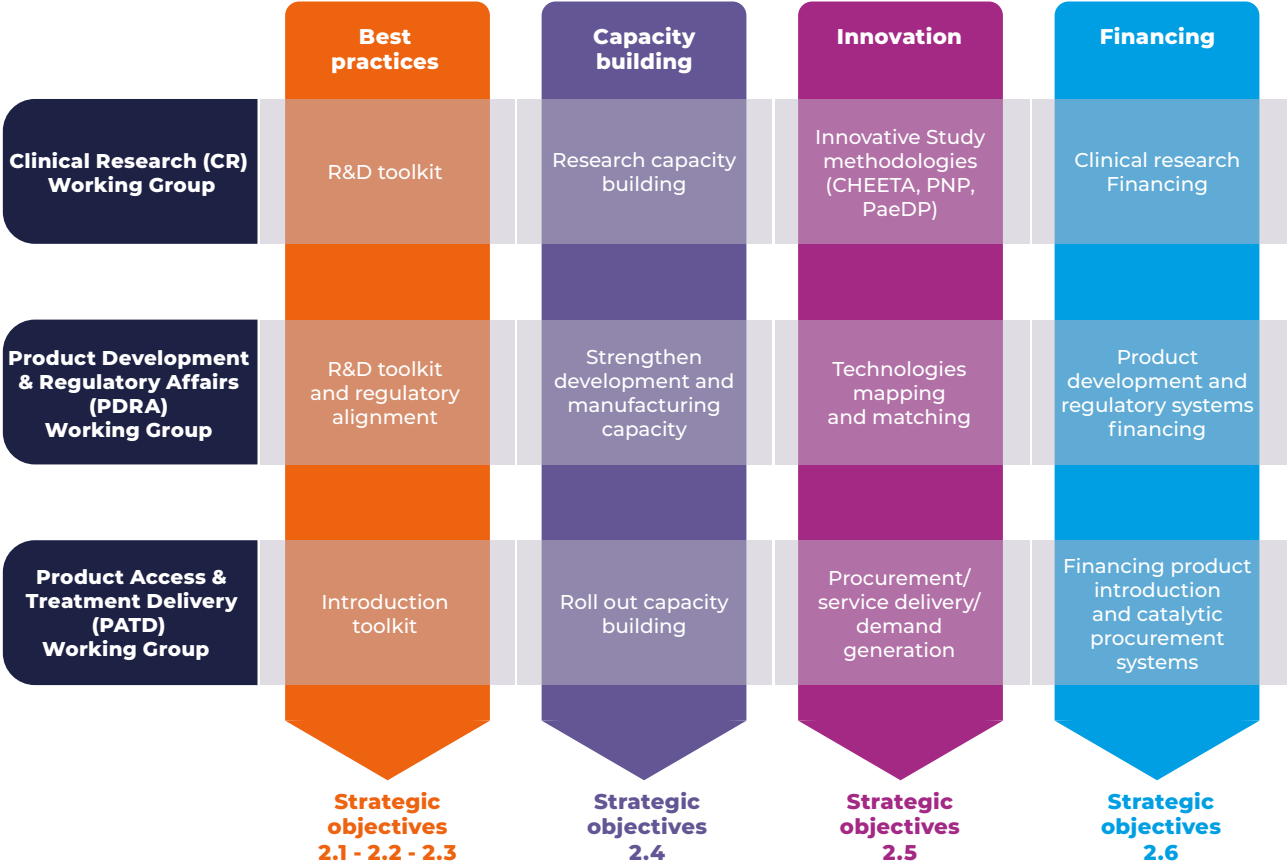
The second pillar of the Strategy addresses areas for acceleration to focus the collaborative efforts of the GAP-f Network. It brings together cross-cutting and cross-learning approaches to research, development and delivery efforts for medicines for children that can be applied across therapeutic areas and products. The work will involve applying lessons learned to build more enabling policies and guidelines and, where needed, developing specific technical tool kits for acceleration. GAP-f, by leveraging its experience in HIV and TB, will work with regulators and partners to open new,

critical regulatory pathways, applying them to new disease areas. In addition, it will publish regulatory alignment guidance. GAP-f will invest in innovations to save time and resources and improve the appropriateness and acceptability of medicines for children. It will also invest in the capacity strengthening needed to implement high-quality clinical research and introduce and roll out innovations as required. Finally, by scoping, analysing and matching innovative financing mechanisms to specific funding gaps, GAP-f will address one of the most fundamental barriers to developing medicines for children by identifying a better and more sustainable approach through a collaborative process.



Areas of focus and operationalization

Fig.6. Overview of GAP-f working group activities within the accelerate pillar, in alignment with strategic objectives outlined in the GAP-f Strategy



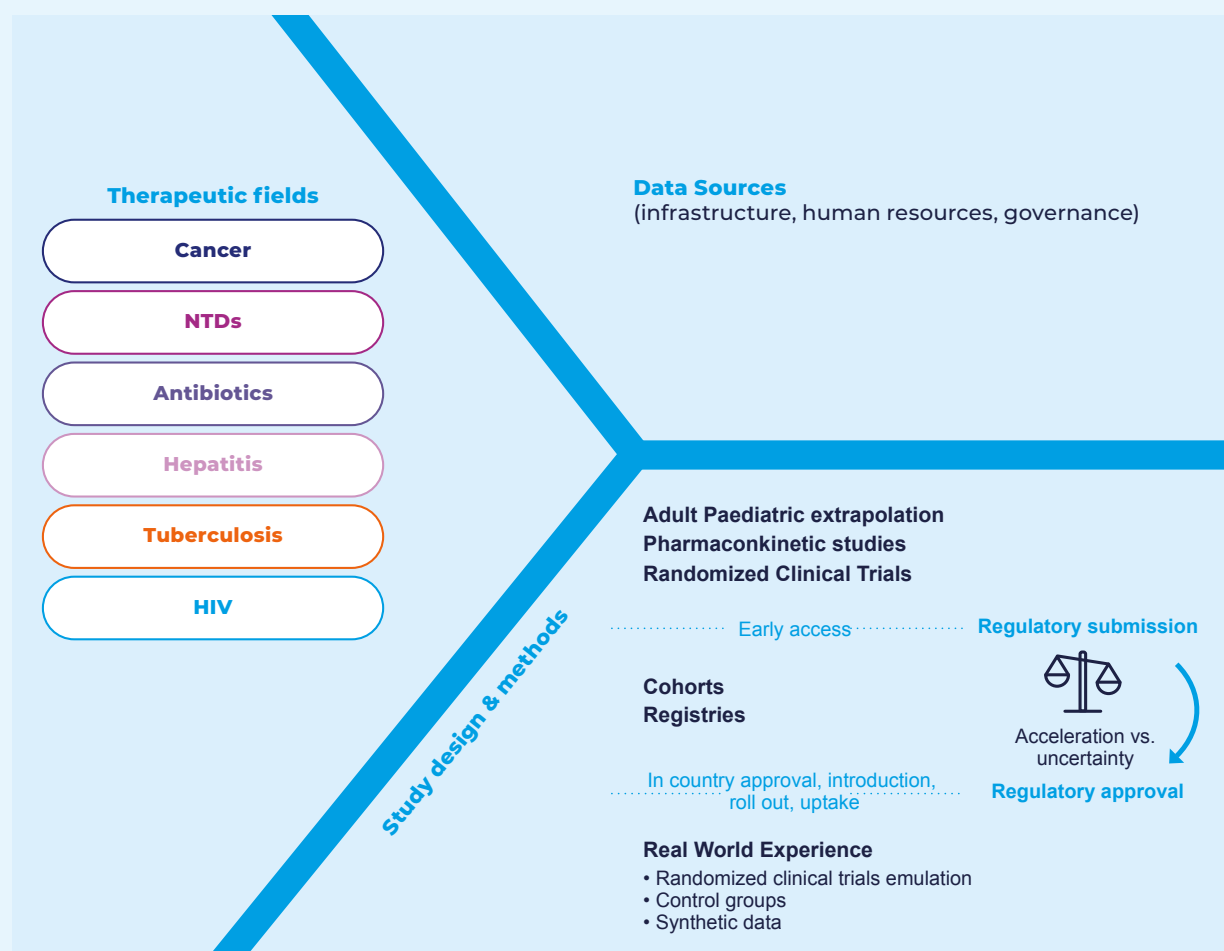
Accelerate – flagship 1: Paediatric Data Hub

Accelerated introduction of better medicines often means doing so despite limited clinical data specifically collected in a paediatric population. As a result, a safety net needs to be put in place to rapidly capture any adverse events that may only be detected when new medications are rolled out. Leveraging work in HIV as a proof of concept, GAP-f aims to foster and enhance the use and interpretation of safety and effectiveness data for new medicines. The Paediatric Data Hub will be designed and implemented to improve the availability, quality and use of real-world data to monitor and optimize treatments. This project will leverage electronic medical records systems at various levels of maturity in countries and complement other more traditional sources of effectiveness and safety data such as following up of randomized control trials and observational cohorts.

In the GAP-f spirit of non-duplication and synergizing efforts and data, the Paediatric Data Hub aims at building on existing data sources across disease areas, connecting and harmonizing these to reach a more integrated data pool that accounts for data governance and regulations while more rapidly monitoring the effectiveness and safety of new drugs and formulations introduced in countries. This will also have a strong capacity development component, as well as bringing innovation, for example, with the introduction of federated data analysis approaches and use of artificial intelligence. At the end of this three-year period, GAP-f anticipates that the structure will be fully designed and a governance and operating model clarified. We also expect to pilot the platform across three disease areas so that a system is eventually in place to ensure that future targeted projects can be effectively implemented.

Fig.7. Assessing short, mid, long term Safety & Effectiveness of paediatric products

The Paediatric Data Platform



Accelerate – flagship 2: innovative technologies

Health-care providers, caregivers and all stakeholders involved in developing medicines for children acknowledge the challenges of poor adherence resulting from pill size, pill burden, administration complexity and product palatability. Building on ongoing work to identify universal bitter blockers, GAP-f will expand efforts to identify and then act on opportunities to innovate drug delivery and explore and then match technologies with the potential to substantially improve the friendliness of essential medicines.

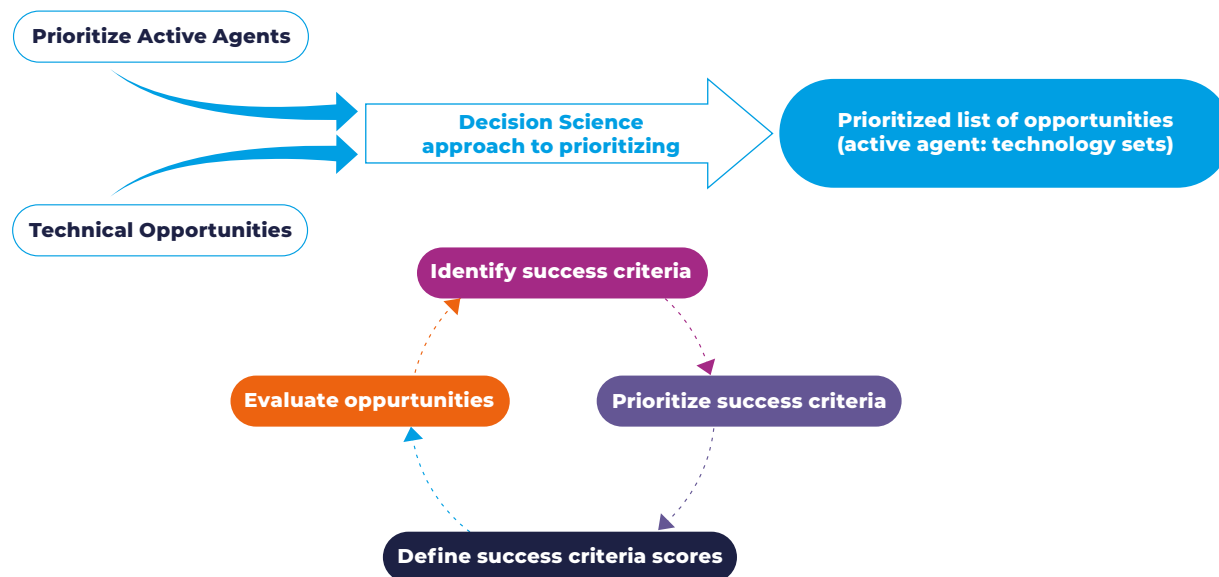
To achieve this, GAP-f partners convened within the GAP-f Product Development and Regulatory Affairs (PDRA) Working Group (see page 27) will use decision science methods that have been effectively used for several different purposes including

setting priorities for product development and commercialization portfolios in HIV, TB, hepatitis C, family planning and diagnostic platforms across disease areas. This approach has helped scientists with diverse backgrounds to find a common language on which quality decisions can be made. GAP-f will therefore use these methods to identify and weigh parameters that will give priority to relevant technologies that may offer opportunities for substantially improving priority products identified with the PADO processes undertaken during phase 2 (Fig. 8).

This workstream will ultimately lead to:

- a broadly supported, prioritized list of active agent and technology pairs (or sets);
- advice on the next steps necessary to create better child-friendly products; and
- a long-lasting method to be deployed once additional drugs are prioritized by GAP-f.

Fig.8. Mapping and prioritizing essential active agents for children with innovative technologies



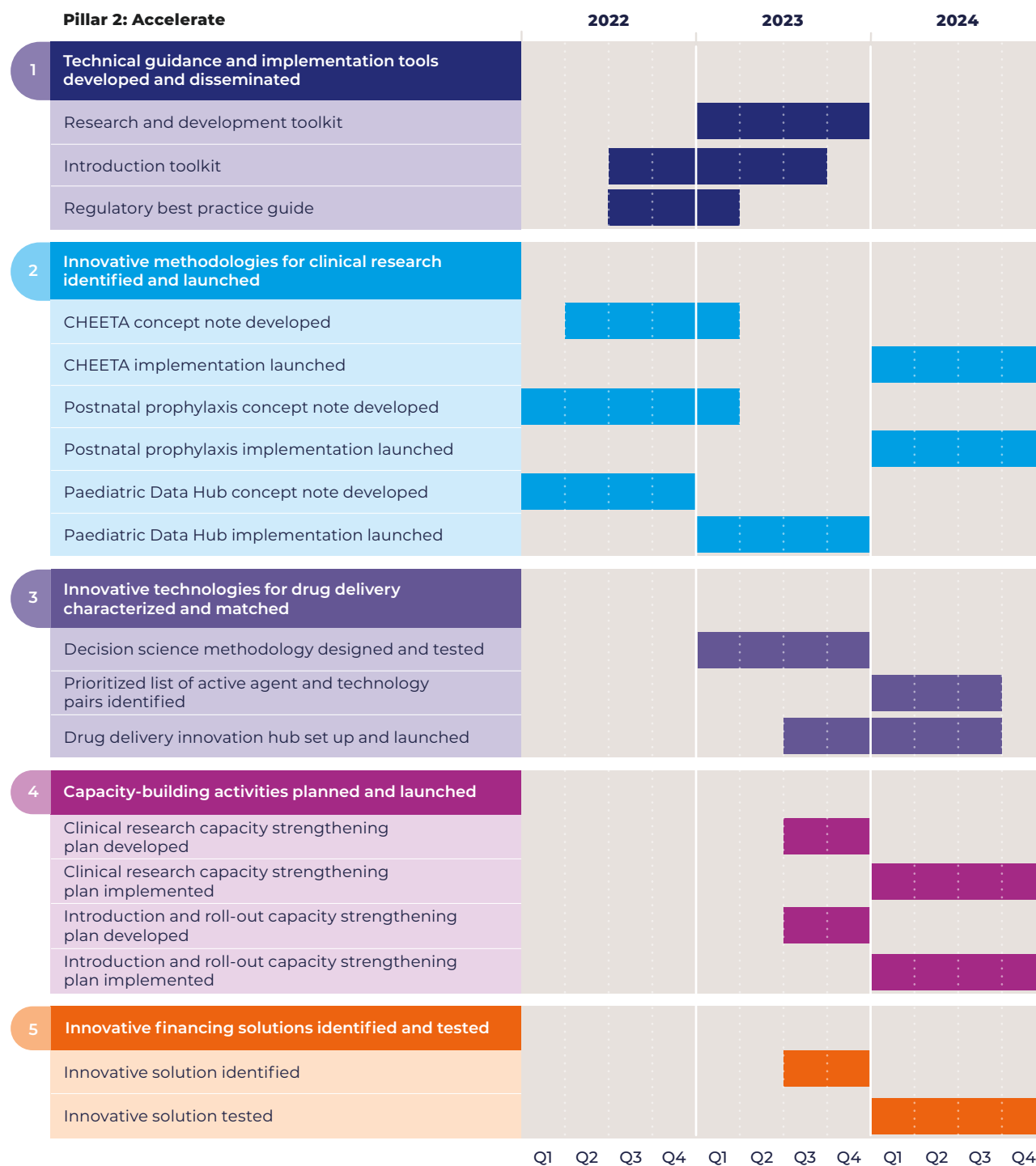
Accelerate – flagship 3: regulatory pathways

Efficiently navigating the regulatory landscape and using the best regulatory pathways are key aspects for the successful acceleration of access to essential formulations for children. Through its cumulative and collaborative experience from its partners, GAP-f can play a key role in helping to identify the most appropriate and effective regulatory tools in the whole product life cycle and to promote best practices based on reliance and work-sharing. GAP-f partners convened under the PDRA Working Group will share their collective experience to develop a technical briefing highlighting best regulatory practices to accelerate regulatory pathways for medicines for children.

In addition, since real-world evidence takes on an increasing role in providing data for evidence-informed regulatory decisions, especially in a field such as paediatrics, with

limited enrolment capacity and substantial need for data, the PDRA Working Group will be used as a platform for discussion and exchange on the best available models to rely on real-world evidence for developing drugs for children. Lastly, the rapidly evolving science and innovations may present challenges to fit the current and slower evolving regulatory framework. Innovative approaches may trigger some regulatory gaps or challenges the PDRA Working Group could anticipate or prepare for. The work of the PDRA Working Group is strongly linked with the work of the WHO Paediatric Regulatory Network, where connections may be made when needed in terms of product-specific issues or cross-cutting discussions. Although the impact of this effort may not be realized rapidly, this work holds tremendous potential for long-lasting acceleration of regulatory pathways for the future development of drugs for children.

Key milestones





Impact indicators

Amount of time saved at each stage of research, development and introduction

This will be measured by analysing the counterfactual scenario (without GAP-f intervention) and estimating the time saved compared with historical timelines for product investigation, development and introduction – estimated at 10 years or more from the time of approval for adults to time of introduction of a paediatric formulation.

New technologies and methods matched to priority medicines for children across multiple stages of priority-setting, research, development, introduction and monitoring

This will be measured by the number of products in the paediatric drug pipeline that are matched with an appropriate child-friendly formulation or benefit from innovative methodologies for study design and monitoring (Fig. 8).

Key actors and partners

Apart from the innovative funding work, cross-cutting activities are implemented through GAP-f working groups (see page 26) comprising partners' expertise targeting a specific segment

of the product development life cycle (stages). Working group leads and specific members of the working groups will lead and or contribute to the cross-cutting activities belonging to pillar 2, leveraging existing skill sets, expertise and ongoing work. Efforts by the working groups will foster closer collaboration and ensure that individual experiences result in shared lessons and this, in turn, shapes and aligns actions. Where of value, these activities will leverage existing or ongoing WHO work and global processes that may offer opportunities for increased efficiency, greater reach, better coordination and a more systematic measure of impact.

The Strategy and Coordination Committee will coordinate the innovative funding work with expertise from external consultants and advice from the Funders and Investors dialogue under the remit of the stakeholders engagement forum.

Resource needs

The overall budget for this pillar for 2022–2024 has been estimated to be about US\$ 25 million, which will be critical to address set-up costs and establish systems, effective collaboration platforms and capacity strengthening. As these initiatives move into routine operations, the resources for these activities are anticipated to be leveraged from product-specific programmes.



Operationalizing pillar 3: intervene

Building on a clear view on priorities obtained from pillar 1 as well as opportunities for systemic acceleration and innovation identified by pillar 2, this pillar 3 aims to address the product-specific gaps requiring direct intervention. GAP-f will define and take up projects that are not undertaken by other actors yet and that pose a threat to acceleration, whether by stopgap

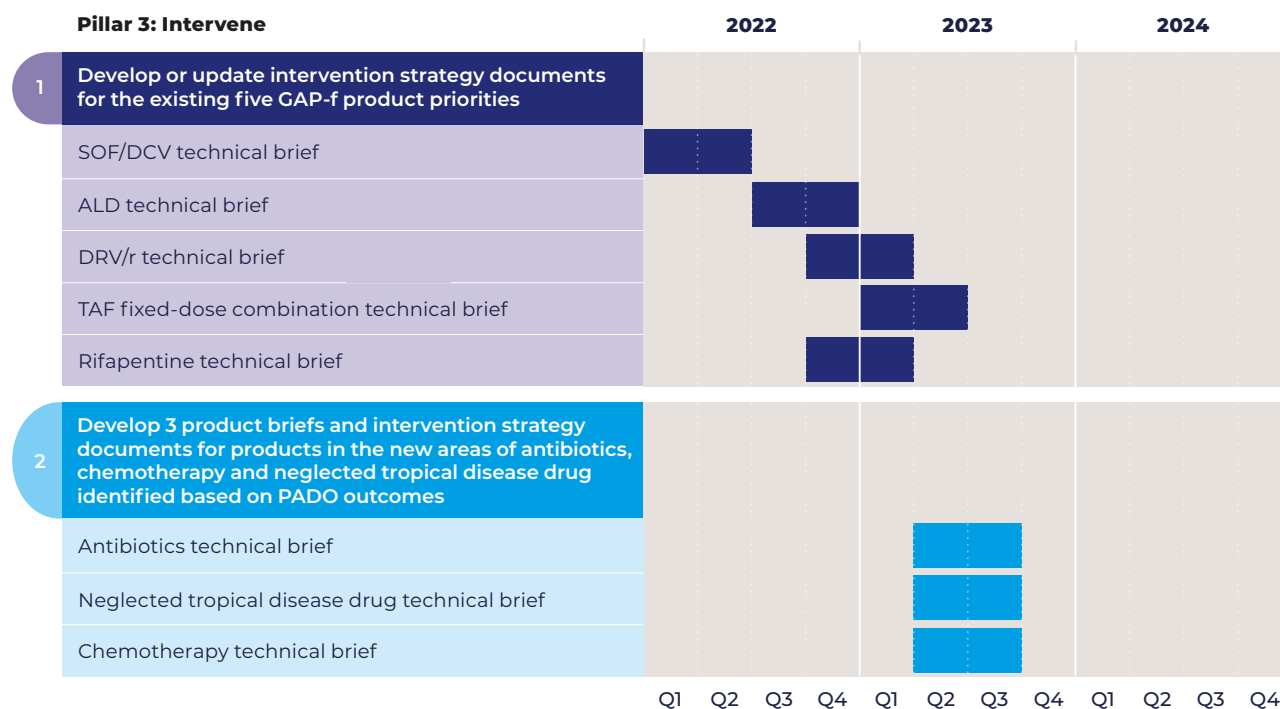
funding, providing ad hoc support or directly stepping in through a partner. During the 2022–2024 strategy period, GAP-f will continue to follow product priorities from its phase 1 strategy and to define product-specific priorities for antimicrobial resistance, childhood cancer and selected neglected tropical diseases. In each case, it will define an intervention strategy after examining gaps, potential solutions and actors, as illustrated in the example of hepatitis C treatment below.

Intervene – flagship 4: hepatitis C among children

The GAP-f Network has facilitated the alignment of partners around the optimal strategy to deliver a low-cost, generic, pan-genotypic treatment regimen for eliminating hepatitis C among children. In reviewing the evidence available, anticipated need, product development, regulatory and market landscape, GAP-f partners have identified that the most pragmatic route to enable broad access to hepatitis C treatment is providing sofosbuvir (SOF) + daclatasvir (DCV), which requires developing a SOF 100-mg product. GAP-f is developing a work plan to accelerate the development of this product to enable introduction of this regimen. This plan includes clinical validation by designing and conducting a clinical pharmacokinetics study, confirming the market size, designing a product development plan that would engage with the innovator and specific

generic manufacturers and a fundraising plan. GAP-f anticipates that a development and product launch incentive programme will be needed, and advocacy by GAP-f and its partners will be critical in mobilizing the resources required. To lay the foundations for introduction, GAP-f will establish a GAP-f SOF Task Team under the Product Access and Treatment Delivery Working Group (see page 28) to define specific activities to support Network members' efforts at introducing SOF + DCV to ensure that work across countries is coordinated and that best practices are shared. As an interim solution to address the acute need for care, the GAP-f SOF Task Team will catalyse the introduction of the adult formulation of SOF for children weighing more than 25 kg. When product development of the 100-mg SOF is one year away from commercialization, the Task Team will then turn its attention to introducing the SOF + DCV regimen.

Key milestones





Impact indicators

Over the next three years, the facilitation of GAP-f will enable partners to include four new products in clinical trials, develop five new formulations and gain approval by stringent drug regulatory authority approval or prequalification assessment for four of these. GAP-f anticipates that as a result, five new products will be introduced and monitored with its facilitation.

This will be ultimately measured by the following indicator.

Number of additional children accessing better and appropriate prioritized medicines per disease area

Depending on the disease area and availability of data, this will be measured by using global disease-specific monitoring mechanisms in triangulation with procurement agencies' key process indicators, with national burden of disease against reported treatment coverage estimates as a first step. When possible, specific analysis of regimens will be used to estimate the increase of treatment with optimal formulations. This will be triangulated with procurement data.

Key actors and partners

GAP-f partners will largely lead this pillar, based on expertise and interest that the product-specific work may require. However, GAP-f working groups will assist implementation by offering input for acceleration and innovation as well as a more joined-up approach to anticipate action and promote greater efficiency and complementarity across the product life cycle. The GAP-f Secretariat and members of the Strategy and Coordination Committee will remain actively engaged to advocate for resources and facilitate strategic partnerships when required.

Resource needs

The overall budget for this pillar for 2022–2024 is estimated as follows. Activities related to the advocacy and facilitating role of GAP-f will be covered by leveraging the coordination and cross-cutting work undertaken by the GAP-f Secretariat and Strategy and Coordination Committee (see above: resource needs for pillar 2). However, substantial investment will be required to support product-specific work that partners will lead, to ensure the accelerated investigation, development and introduction of priority products. This funding, which will not be channelled through GAP-f but rather be given directly to its relevant partners, has been estimated to be close to US\$ 140 million, which will enable critical programmes to be launched and implemented across the product life cycle.



Abstract geometric shapes in the top left corner, consisting of several overlapping, curved, light beige shapes on a dark blue background.

Measuring impact

The ultimate impact GAP-f seeks is to significantly decrease mortality and morbidity of children by accelerating access to safe, effective, quality, affordable, and available paediatric medicines in optimal formulations.

During the next three-year strategic period, GAP-f and partners will measure their impact in six key areas as stated above for each of the strategic pillars. The strategic framework, together with specific milestones for this strategy period serve to realize the impact indicators stated below.



Prioritize and align

The global needs, prioritization framework and GAP-f focus areas being well defined, widely communicated and broadly accepted

A system for global progress monitoring and tracking is in place to enable stakeholder alignment and targeted work to be undertaken to accelerate research on, development of and introduction of medicines for children. This will be measured by periodically updating progress indicators. Alignment will be measured from new investments and initiatives matching the priority products identified through PADO processes. All of this will be tracked through the Observatory.

Measurable increase in the funding dedicated to medicines for children and broader diversity of funding sources: GAP-f partners better funded to deliver against GAP-f priorities

This will be measured by establishing a methodology to attain the global need of funding required for prioritized products, assessing the current investments and measuring the increase in resources allocated to filling priority gaps. In addition, a donor and investor landscape will serve as a baseline, and new donors and investors will be measured over the three-year period based on established criteria for inclusion.

Number of countries reporting on inclusion of the optimal formulations of the WHO Model List of Essential Medicines for Children in their national essential medicines lists

This will be measured through periodic surveys led by WHO with facilitation by GAP-f across WHO Member States to monitor and evaluate the adoption and adaptation of the WHO Model List of Essential Medicines for Children for children to national essential medicines lists.



Accelerate

Amount of time saved at each stage of development and introduction

This will be measured by analysing the counterfactual scenario (without GAP-f intervention), estimated according to baseline information and historical timelines for product investigation, development and introduction – estimated to be 10 years or more from the time of approval for adults to the introduction of a formulation for children.

New technologies and methods matched to priority medicines for children across multiple stages of priority-setting, research, development, introduction and monitoring

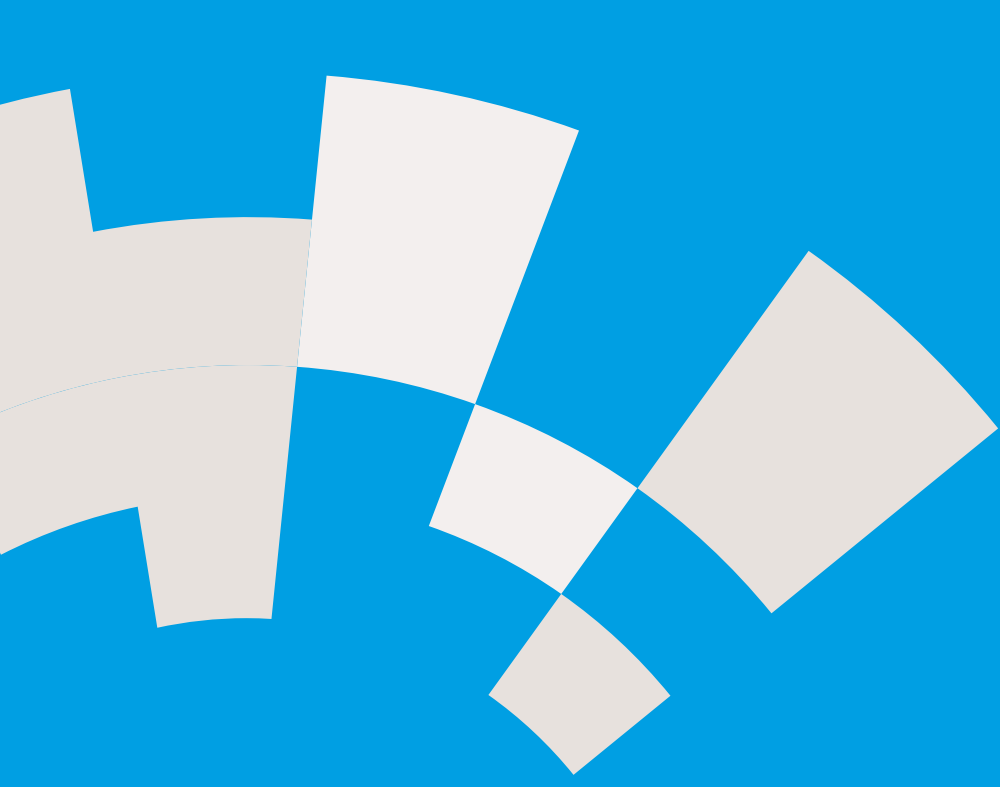
This will be measured by the number of products in the drug pipeline for children that are matched with an appropriate child-friendly formulation or benefit from innovative methods for study design and monitoring (Fig. 8).



Intervene

Number of additional children accessing better and appropriate prioritized medicines per disease area

Depending on the disease area and availability of data, this will be measured by analysing the national burden of disease against reported treatment coverage estimates as a first step. When possible, specific analysis of regimens will be used to estimate the increase of treatment with optimal formulations. This will be triangulated with procurement data.



GAP-f governance and operating model

The GAP-f Network is based on a collaborative and adaptive governance model that enables the active participation of partners and stakeholders and is based on a set of shared principles as outlined in the GAP-f Strategy 2022–2024.

Transparent information sharing and accountable processes characterize the decision-making processes. The GAP-f model contributes to, and ideally strengthens, the individual organizational missions of its partners, while contributing to the collective vision, mission and strategic objective of GAP-f.

In accordance with the Strategy, the governance of the GAP-f Network has been refined.

WHO Steering Group and Secretariat

Within the GAP-f Network, WHO has a twofold role. It has the legal responsibility for the GAP-f Network as its host organization. In addition, WHO directors from technical departments² comprise the GAP-f Steering Group. This Group, convened and represented by the Director of the Research for Health Department, is formed to review strategic decisions to ensure alignment with the WHO Programme of Work and compliance with WHO's legal and ethical framework. The GAP-f Secretariat oversees the day-to-day management of GAP-f's work and engages across several departments and divisions, through an agile team of paediatric focal points to provide critical contributions to GAP-f, convenes disease-specific collaborations and ensure engagement with appropriate bodies within WHO. The Secretariat thus provides an important liaison function across the Strategy and Coordination Committee and its working groups and the stakeholders engagement forum.

External Advisory Committee

The GAP-f External Advisory Committee is an external body of advisers who provide overall guidance and strategic advice to the GAP-f Steering Group and the GAP-f Secretariat on request. It has an important function to leverage the GAP-f Network publicly, thus contributing to advocacy efforts in pillar 1.

Strategy and Coordination Committee

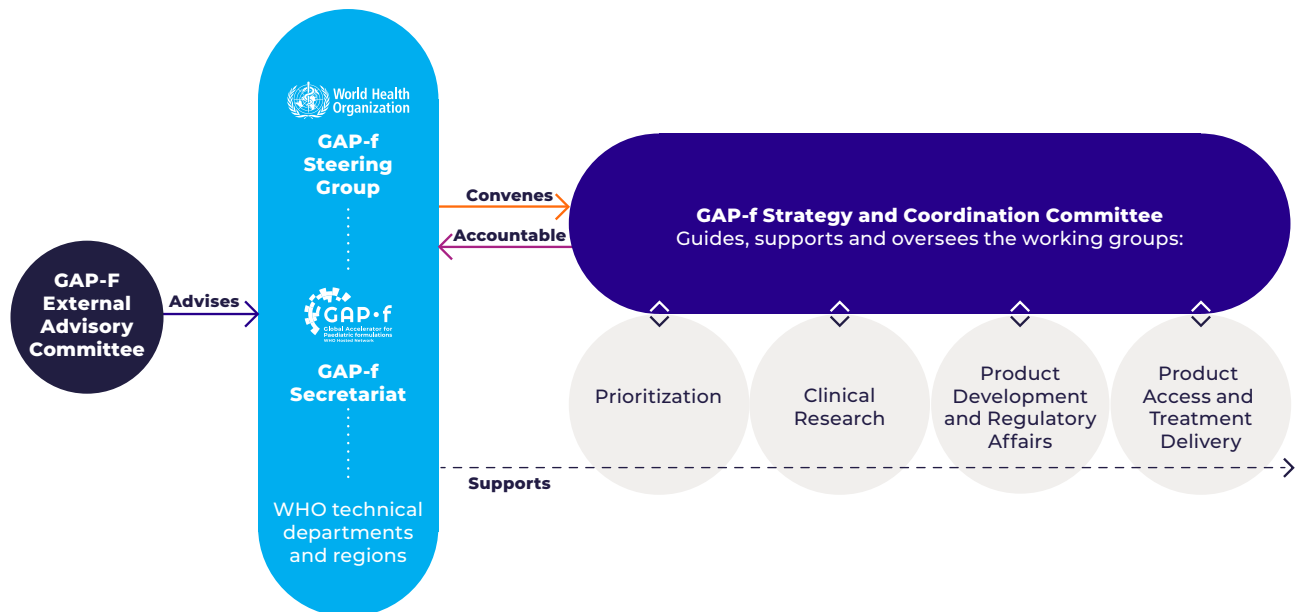
The Strategy and Coordination Committee leads the strategic and operational work of the GAP-f Network and supports the Network to achieve equitable access to medicines for children. It provides oversight and drives decision-making regarding the implementation of the strategy. The Strategy and Coordination Committee supports the Network's efforts in giving priority to the most urgent needs of children and considers opportunities for the GAP-f Network to grow and evolve, including its fundraising efforts. The members of the Strategy and Coordination Committee include a maximum of 10 GAP-f member organizations invited by the GAP-f Steering Group. Senior representatives of these organizations provide GAP-f with strategic input and global health advice. The technical representatives coordinate the activities of the working groups, foster collaboration among members of the GAP-f Network and solicit input from forum representatives.

The Strategy and Coordination Committee is accountable through the Secretariat to the WHO GAP-f Steering Group.



² HIV, hepatitis and sexually transmitted infections programmes; Global Tuberculosis Programme; neglected tropical diseases; noncommunicable diseases; and antimicrobial resistance that lead work on the therapeutic focus areas taken up by the GAP-f Network and those from cross-cutting departments, such as Essential Medicines List; Regulatory; and Maternal and Child Health.

Fig.9. GAP-f governance structure



Working groups

The working groups are structured to mirror the product life cycle (stages). Their members comprise subject matter experts from WHO and GAP-f partner organizations. All working groups contribute to the three strategic pillars (prioritize and align, accelerate and intervene). Each working group, co-led by technical representatives of two Strategy and Coordination Committee member organizations, produces an annual work plan with short- to medium-term goals to achieve the targets of the GAP-f strategy for 2022–2024. The Working Groups meet quarterly to:

- contribute to overall prioritization, tracking and advocacy activities led by the GAP-f Secretariat and designated WHO technical staff members;
- assess cross-cutting opportunities and identify approaches that will accelerate multiple steps of the product life cycle across disease areas; and

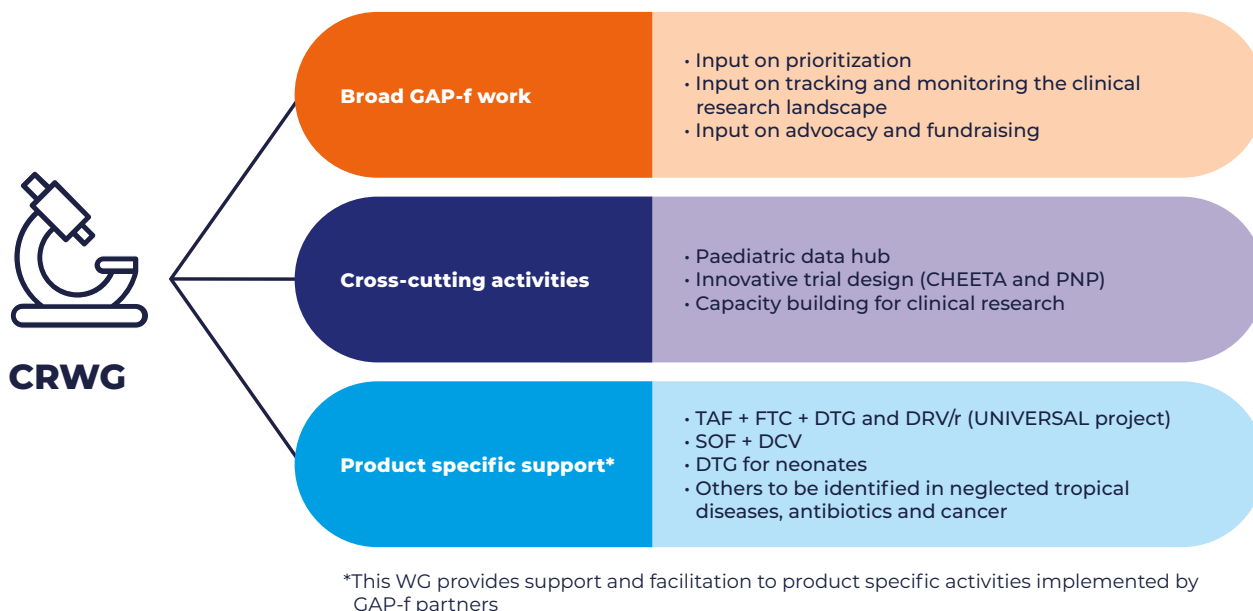
- actively review the status of drugs in the paediatric drug portfolio to identify intervention that would help accelerate product research, development and introduction.

More specifically, the four working groups together fulfil the following roles and intermediate goals.

The Prioritization Working Group has an overarching role in ensuring a transparent process in determining the global needs, gaps and priorities for medicines and formulations for children. Flowing from this, it monitors results from ongoing research, in conjunction with anticipated policy changes and guideline implementation on the ground. Notably, the group supports and facilitates paediatric drug optimization processes to identify priority products and associated target product profiles (Fig. 2).

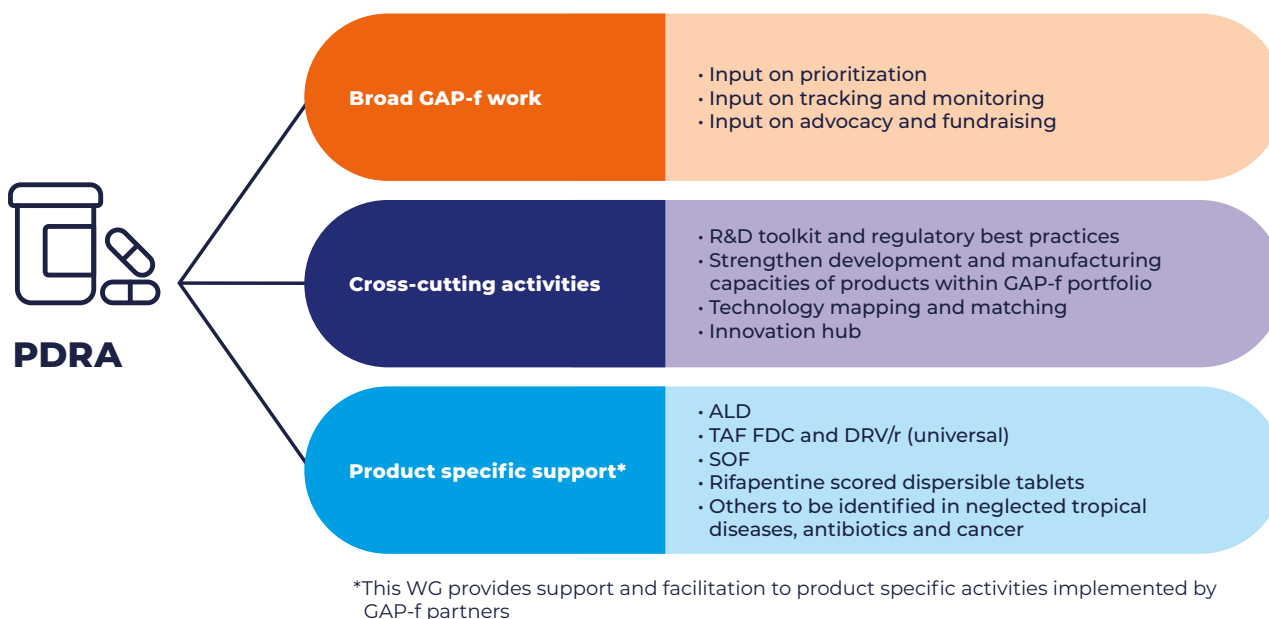
The **Clinical Research Working Group** surveys the landscape of studies in disease areas of the Strategy, leverages lessons from study design across disease areas and implements clinical studies needed to fill gaps: for example, to accelerate work on dosing, safety, efficacy or acceptability of new medicines.

Fig.10. Clinical Research Working Group



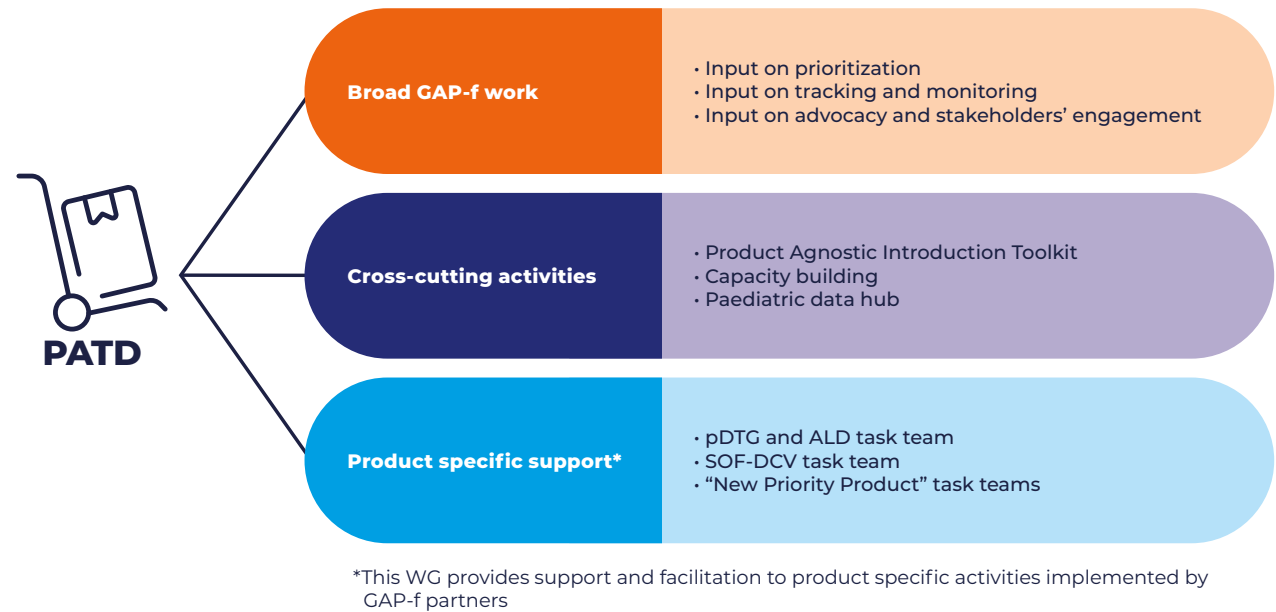
The **Product Development and Regulatory Affairs (PDRA) Working Group** focuses on acceleration of product development, regulatory filings and sustainable product access and acts as an innovation hub for partners to bring in new formulation technologies and to optimize reliance and collaborative regulatory pathways.

Fig.11. PDRA Working Group



The **Product Access and Treatment Delivery Working Group** focuses on ensuring that product availability is accelerated by demand generation from communities, defining and implementing scale-up plans for prioritized products and monitoring safety and efficacy, always with specific attention to leave no one behind. Community-led monitoring will play a key role to ensuring that the needs and demands of communities are well understood and documented and are met.

Fig.12. PATD Working Group





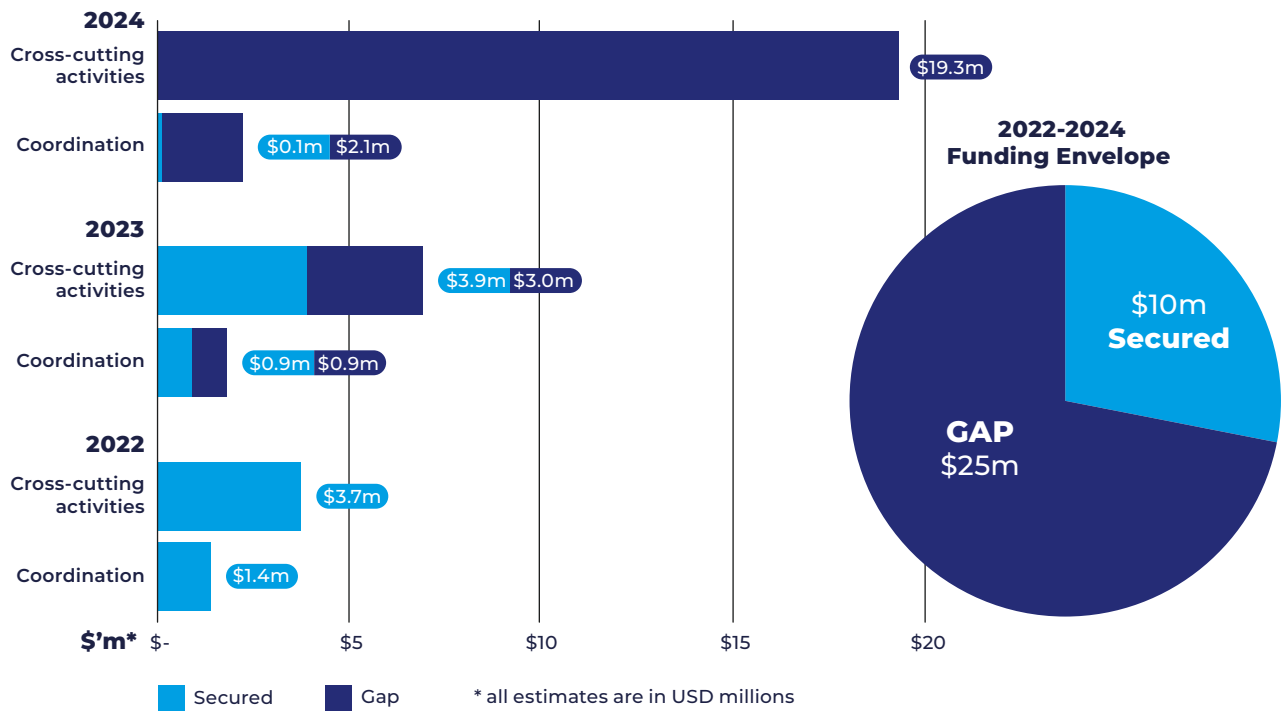
GAP-f human resource planning, financial evolution and resource mobilization strategy

Carrying out this current strategic period will depend on GAP-f's ability to mobilize, coordinate and leverage resources across multiple partners.

Much of the human resources for GAP-f lie within its partner organizations either as in-kind contributions or as resourced work to be carried out through the Strategy and Coordination Committee and the working groups. However, in addition to these contributions and to ensure sustainability, coordination and cross-fertilization of the work, the GAP-f Secretariat will need an increase in human resources to ensure that core coordination is efficient. Aligning across multiple actors for resource mobilization for all partners and communication around the collective needs for medicines for children, for example, will require dedicated human resources. To remain agile and purposefully lean, WHO staff members will be invited to contribute as relevant to their skill set and overall duties. In addition, consultancies or secondments will be considered, and this model will be assessed periodically to define a valuable and sustainable human resources strategy.

The GAP-f Secretariat currently functions with 2.5 full-time equivalent staff members and short-term consultancy contracts. By early 2024, the aim is to increase this to 4.5 full-time equivalent staff members (leadership, management, assistance, regulatory, monitoring and evaluation), and expand the support from technical departments as relevant and appropriate. In addition to this, short-term consultancies (resource mobilization, advocacy and stakeholder coordination) will help to maintain an agile team that can be shaped and redirected according to needs. GAP-f Strategy and Coordination Committee members and working group leads are currently supported with a lump sum that is anticipated to double by the end of 2024 with the growing needs of GAP-f coordination function and cross-cutting activities. The human resource needs ramp up over these three years and will stabilize by the end of this strategy period. Since the cross-cutting budget will mainly serve to set up projects under the GAP-f strategy, these costs will also level off over time and serve to launch new initiatives and mitigate funding gaps and any other resource gaps associated with this work.

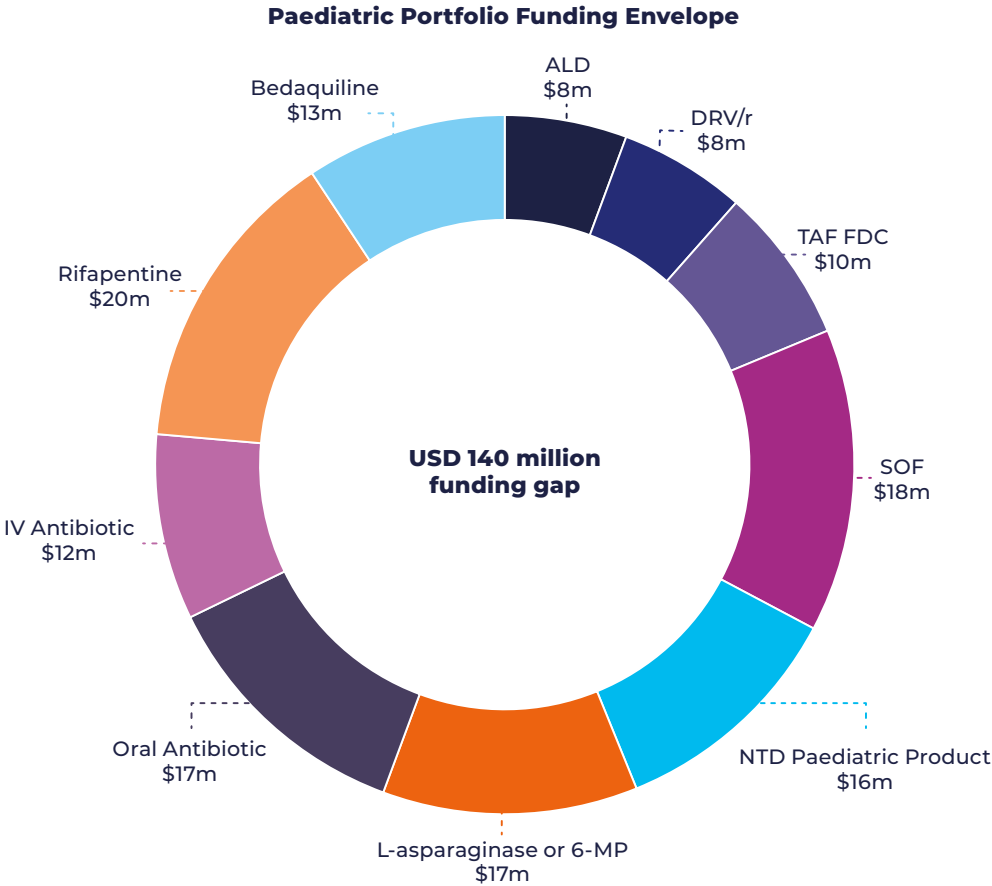
Fig.13. Overall GAP-f budget and resources overview



GAP-f will only play a facilitating and supporting role to its partners that will implement the core activities within the product life cycle of GAP-f priority products. Nevertheless, the availability of the appropriate resources will be essential for GAP-f to fully realize its value and the acceleration required. Anticipated resources in support of core activities that are specific to products that partners will investigate, develop or introduce have been estimated based on drug costing guidelines (Annex 4) developed

after wide consultation within and outside the GAP-f Network. Following detailed mapping of anticipated timelines and activities required to accelerate investigation, development and specific product introduction work of priority products (Annex 3), these estimates provide the basis to outline funding envelopes for each GAP-f priority product and delineate specific funding gaps to be addressed during the next three years of implementation.

Fig.14. Resources to be mobilized for product-specific activities led by partners to launch various activities across the product life cycle which will be implemented over the next 5–8 years



Although GAP-f funding requirements for coordination and cross-cutting work increase steadily to stabilize by the end of this strategy period, this investment lays the foundations for the capacity to leverage funding for GAP-f partners to increasingly and more efficiently respond to the priorities established through its work. As new disease areas are taken on, the processes and knowledge are there to accommodate this.

The GAP-f resource mobilization strategy is threefold and interlinked, aiming to:

- ensure that resources for the GAP-f Secretariat are raised so that its coordination function and specific cross-cutting GAP-f activities are sufficient to carry out its strategy;
- increase overall investment and mobilize resources for GAP-f partners through global advocacy on the needs and gaps, spotlighting partner work that must be funded to accelerate priority medicines for children and
- support the identification, piloting and implementation of innovative funding solutions.

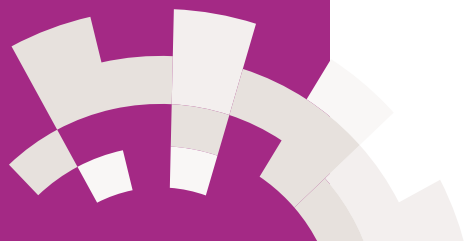
GAP-f will explore new funding models to help to achieve its goal of providing better medicines to children in need. To this end, GAP-f will work in the following three areas: (1) operational support: direct support for the GAP-f Secretariat and Strategy and Coordination Committee to fund its coordination activities; (2) flexible support for GAP-f cross-cutting activities: direct funding that could be deployed as GAP-f sees fit to meet unexpected needs to cross-cutting initiatives to one-off, time-bound, proof-of-concept projects; and (3) project life cycle-based funding (clinical research, development or introduction): indirect funding (to GAP-f partners) dedicated to a specific stage along the product life cycle, such as clinical research or product introduction.

As GAP-f continues to evaluate new funding models for its own operations and to benefit the paediatric drug pipeline, GAP-f will seek specialized expertise to engineer innovative funding instruments that account for the complexity of the GAP-f operational model.





Conclusion and call to action



As GAP-f enters this second phase of its strategy, with a broader disease remit, a clearer role in accelerating cross-cutting efforts, an expanded stakeholder base, a mechanism to engage other critical partners through specific forums and a fit-for-purpose governance and operating model, the potential for the collective action of all actors in innovation in and access to medicines for children has reached a new level.

The phase 2 Strategy and this business plan are part of a stepwise approach to sustainable accept change. Ensuring that the GAP-f Network at large can deliver on its promise to children, caregivers and families will require greater coordination, stronger learning processes, highly children-focused efforts, candid dialogue among partners and staunch support from donors. GAP-f is dedicated to ensuring that partners can continue to work ever more efficiently and work together to be stronger in our response to children's needs worldwide. But to do this, sustainable funding is needed to ensure that momentum is not lost but rather gained.

The GAP-f model and approaches will continue to evolve to more effectively accelerate research on, development of and delivery of better and more appropriate medicines for children. Better coordination, reducing silos, identifying priorities and gaps, capitalizing on new technologies, incentivizing partnerships, listening to communities, aligning funding, and ensuring that innovation in funding will all play a key role – a role that GAP-f is uniquely positioned to play. The years 2022–2024 are crucial to laying the foundations for sustainable change over the long term, ensuring that no child is left behind.

Contact

For further details, including detailed workplans and budgets, please contact us at gap-f@who.int.





References

1. Promoting innovation and access to quality, safe, efficacious and affordable medicines for children. Geneva: World Health Organization; 2016 (<https://apps.who.int/iris/handle/10665/252800>, accessed 11 July 2022).
2. Global Accelerator for Paediatric Formulations 2022–2024 Strategy: shaping the global innovation and access landscape for better paediatric medicines. Geneva: World Health Organization; 2022 (<https://www.who.int/publications/i/item/9789240044647>, accessed 11 July 2022).
3. Paediatric drug optimization standard procedure. Geneva: World Health Organization; 2021 (<https://apps.who.int/iris/handle/10665/349315>, accessed 11 July 2022).
4. Global Observatory on Health Research and Development [website]. Geneva: World Health Organization; 2021 (<https://www.who.int/observatories/global-observatory-on-health-research-and-development>, accessed 11 July 2022).
5. WHO Expert Committee on Specifications for Pharmaceutical Preparations: fifty-fifth report. Geneva: World Health Organization; 2021 (<https://apps.who.int/iris/handle/10665/340323>, accessed 11 July 2022).





Annexes

Annex 1. Strategic framework

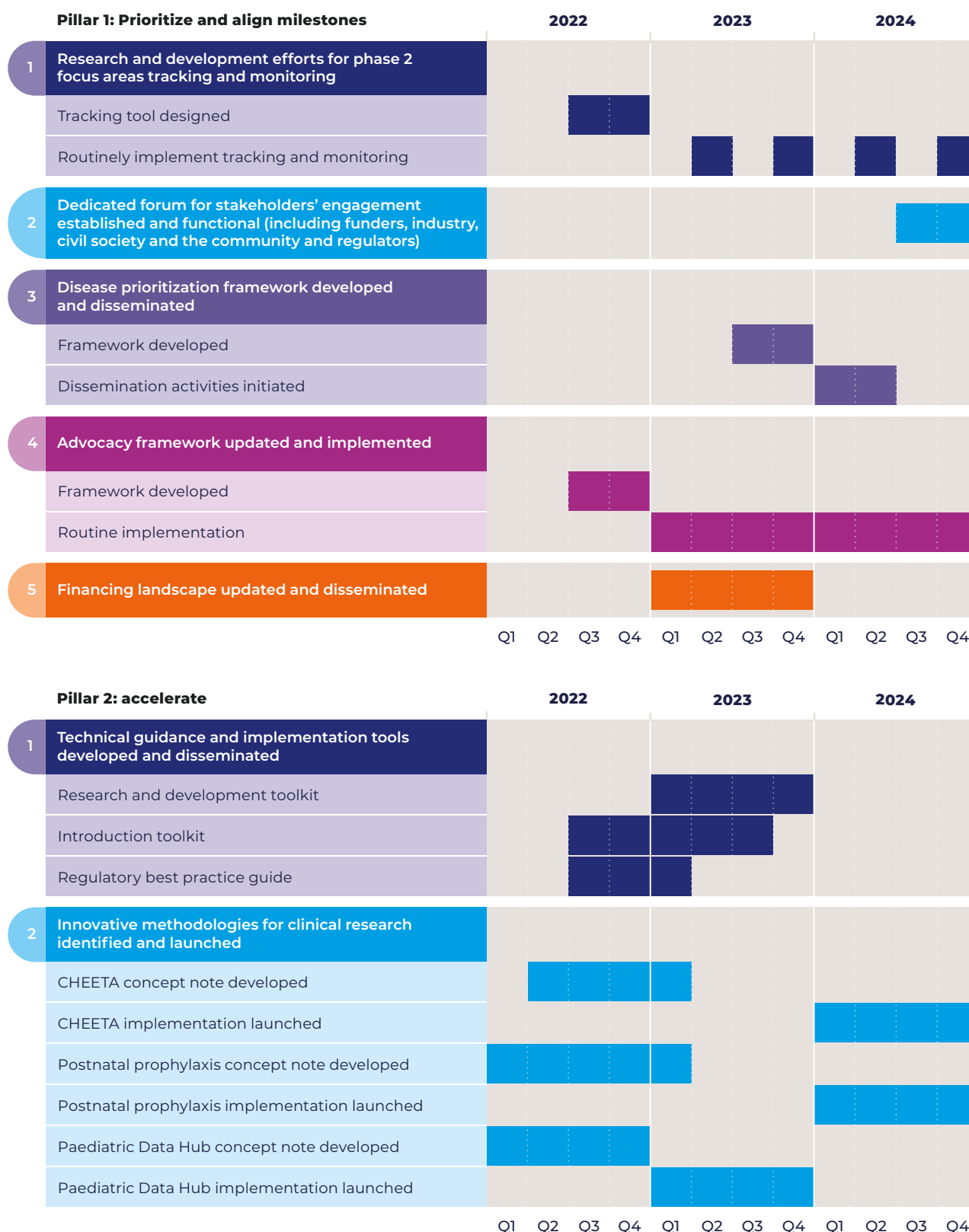
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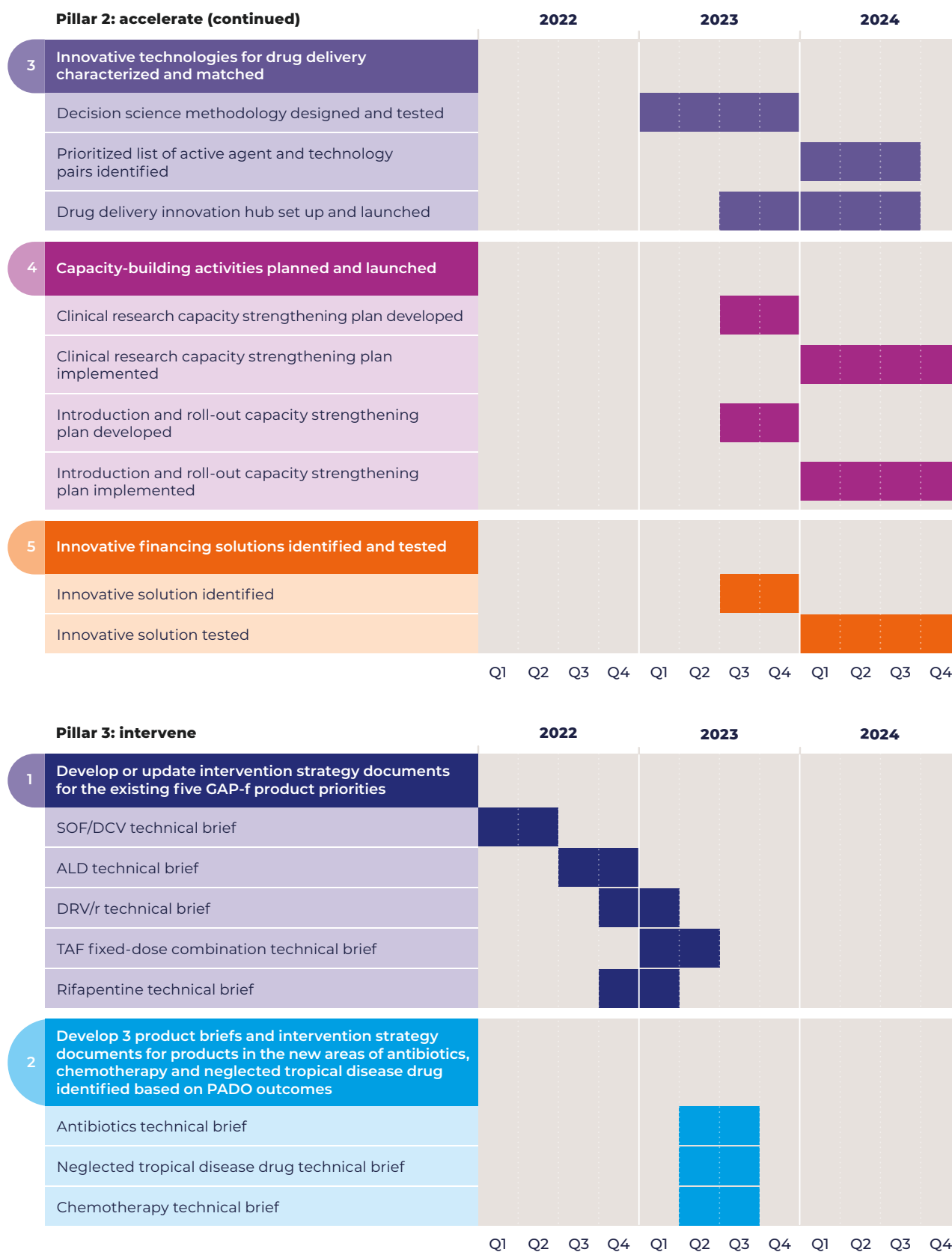
Vision				
All children have equitable access to the medicines they need.				
Mission				
Remove barriers to developing and delivering appropriate, quality, affordable and accessible medicines for children and contribute to universal health coverage by spurring collaboration across stakeholders to identify gaps, set priorities for needs and accelerate product investigation, development and delivery to improve and save the lives of children.				
Shared principles				
Action anchored in global goals and frameworks – Sustainable Development Goal 3, universal health coverage, United Nations Convention on the Rights of the Child and WHO resolution WHA69.20	Agile and efficient mechanism supporting the rapid development of high-quality, effective medicines	Knowledge sharing and dialogue to rapidly respond purposefully and with urgency to change	Good governance that is inclusive, transparent, and accountable	
Effective coordination, collaboration and synergy among multisectoral stakeholders	Innovative thinking for solutions to shared problems	Leverage existing efforts and minimize overlap and duplication	Equitable access to therapeutics that leaves no child behind	
Strategic enablers				
Political commitment to ensure that improving paediatric medicines is high on global, regional and national agendas	Good governance, strong leadership, management and technical knowledge are fit for purpose	Active and transparent engagement of GAP-f partners and stakeholders	Financial resources	Effective strategic partnerships and alliances with public and private partners
Value statement				
<p>GAP-f identifies gaps and sets priorities for the needs in paediatric medicines and formulations worldwide while both leveraging and elevating the important work undertaken by public and private stakeholders in this field. As a unique interface between WHO and its technical departments and paediatric medicines product developers, implementers and funders and civil society, GAP-f:</p> <ul style="list-style-type: none">• leverages the expertise and resources needed to target and optimize investment of time, personnel, effort and funds;• accelerates the time needed to investigate, develop and deliver priority products through synchronized action; and• directly intervenes when needs remain unaddressed.				
Overarching goals				
Prioritize & Align <ul style="list-style-type: none">• Set global priorities for therapeutic areas and missing formulations• Communicate global gaps and priorities through targeted advocacy• Align stakeholders' priorities and action to respond to global needs• Monitor and track global research and development pipelines, gaps, stakeholders & investments	Accelerate <ul style="list-style-type: none">• Facilitate collaborative efforts and mobilize partners to minimize barriers that inhibit innovation and access to paediatric formulations and stimulate activities for acceleration• Identify and promote best practices to accelerate clinical research, product development and delivery activities• Support innovations to advance the development of original research methodologies, novel technologies and innovative financing		Intervene <ul style="list-style-type: none">• Address gaps by facilitating, leading or fundraising for product-specific projects on clinical research, development, regulatory, access and delivery• Support GAP-f partners and engage public and private stakeholders to minimize or eliminate product-specific bottlenecks	

2022–2024 strategic objectives		
Prioritize & Align <ol style="list-style-type: none"> 1. Develop a global priority-setting approach, informed by global disease burden and anticipated public health needs, that will result in timely engagement across therapeutic areas and a list of prioritized missing formulations and indications 2. Prioritize products needed within at least three new therapeutic areas 3. Facilitate a system to monitor and track global paediatric-focused research and development, including gaps, actors and financing 4. Design and implement targeted advocacy to align and mobilize key stakeholder groups: WHO Member States, affected communities and patient groups, civil society, research networks, product development partnerships, international organizations, industry, regulatory agencies and donors 5. Elevate and promote partner and stakeholder contributions, illustrating their roles in the GAP-f work 	Accelerate <p>Convene and support acceleration efforts via stand-alone projects or through GAP-f's working groups (Clinical Research; Product Development and Regulatory Affairs; and Product Access and Treatment Delivery) to collaborate on issues that cut across multiple therapeutic areas. Depending on the priorities defined, objectives will include:</p> <ol style="list-style-type: none"> 1. Document and support the implementation of good practices that enable acceleration 2. Facilitate the development of enabling norms, standards, regulations and policies 3. Streamline regulatory pathways 4. Support targeted capacity strengthening 5. Introduce innovations to improve and accelerate investigation, development, introduction and access 6. Explore innovative funding mechanisms to accelerate paediatric innovation and access 	Intervene <ol style="list-style-type: none"> 1. Define product-specific priorities for at least three additional therapeutic areas and intervene when necessary. For the product-specific priorities identified, GAP-f will facilitate, implement or seek funding to support several targeted interventions to accelerate these products through clinical research, regulatory processes, uptake and access. This will likely involve targeted interventions in: <ol style="list-style-type: none"> 1a. Designing and implementing at least three additional clinical trials 2a. Developing at least four new formulations 3a. Introducing at least four new products 4a. Monitoring the safety of five drugs among those given priority. 2. Identify and engage industry partners to support and accelerate development of priority products. For development of priority products, relationships with industry partners will be key to ensuring that adequate manufacturing and supply of these products are available. GAP-f will identify innovator companies for specific products and engage with them to support paediatric development activities early enough to impact decisions such as clinical trial and formulation design. Additionally, focused, strategic business relationships with generic pharmaceutical companies will be fostered to enable accelerated product development of paediatric formulations of priority drugs.
Impact of the GAP-f 2022–2024 Strategy		
<p>The ultimate outcome GAP-f seeks is to reduce mortality and morbidity among children by providing access to safe, effective, high-quality, affordable paediatric essential medicines.</p> <p>During this three-year strategic period, GAP-f will measure its impact by GAP-f's and its partners' contributions to the following:</p> <ol style="list-style-type: none"> 1. The global needs, framework for setting priorities, and GAP-f focus areas are well-defined, widely communicated, and broadly accepted. A system for global progress monitoring and tracking is in place to enable stakeholder alignment and targeted work to be undertaken to accelerate research on, development of and introduction of paediatric medicines 2. Amount of time saved at each stage of prioritization, research, development, introduction and monitoring 3. New technologies and methods matched and applied to priority paediatric medicines across multiple stages of priority-setting, research, development, introduction and monitoring 4. Measurable increase in the funding dedicated to paediatric medicines and the number of new investors in this area: GAP-f partners are better funded to deliver against GAP-f priorities 5. Number of countries reporting on inclusion of formulations on the WHO Essential Medicines List for children in their national Essential Medicines Lists 6. Number of additional children accessing better and appropriate medicines per disease area 		

Annex 2. Overview of key GAP-f activities in the strategy period 2022-2024

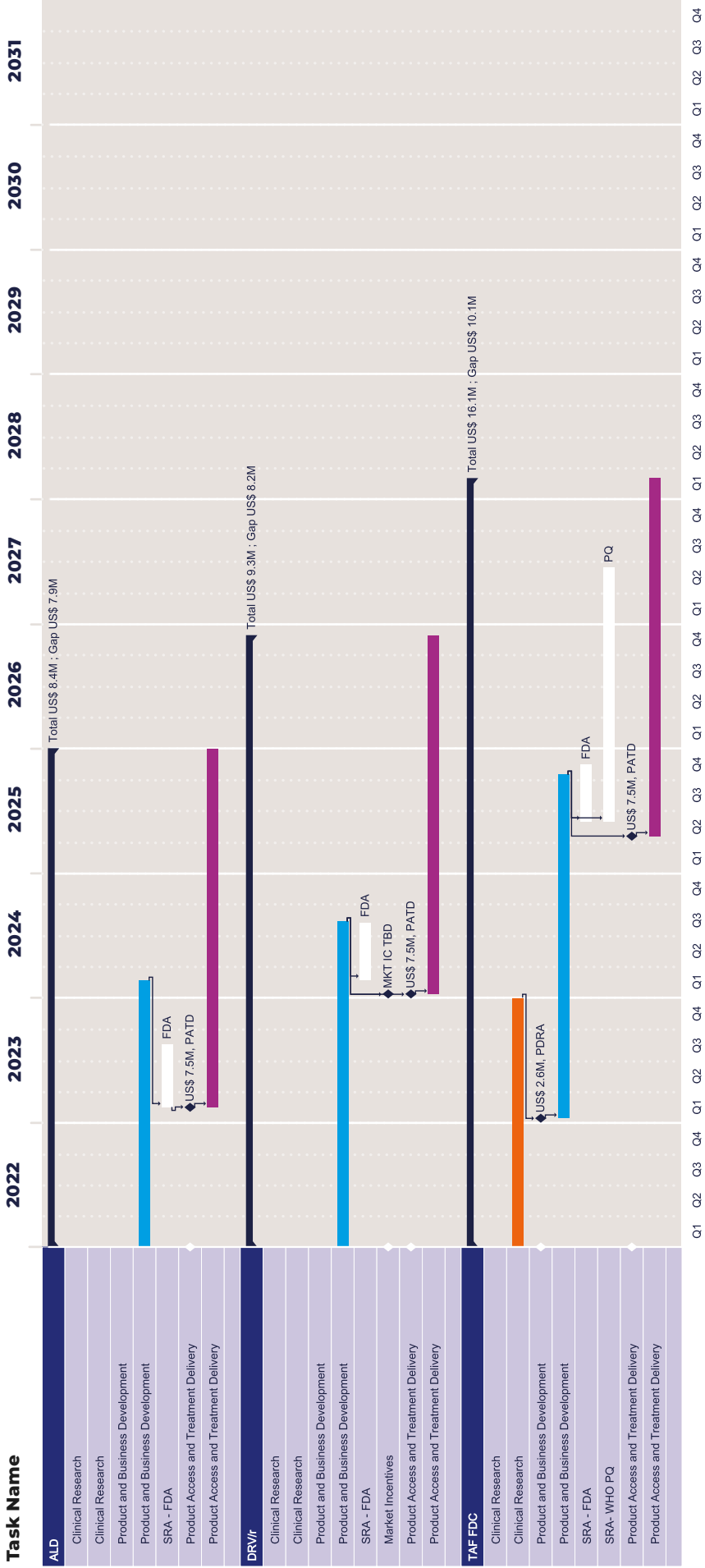
Fig.A2.1

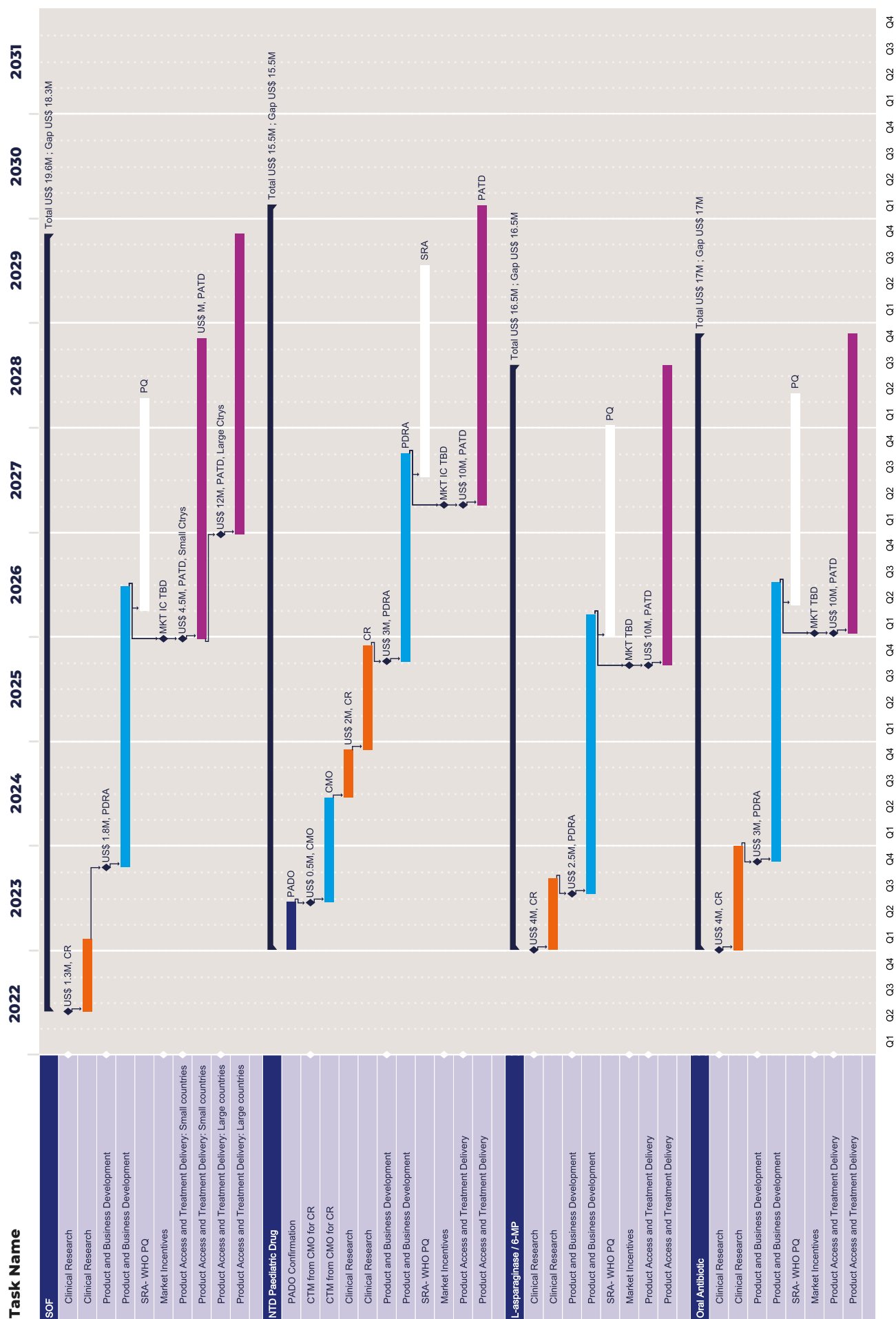


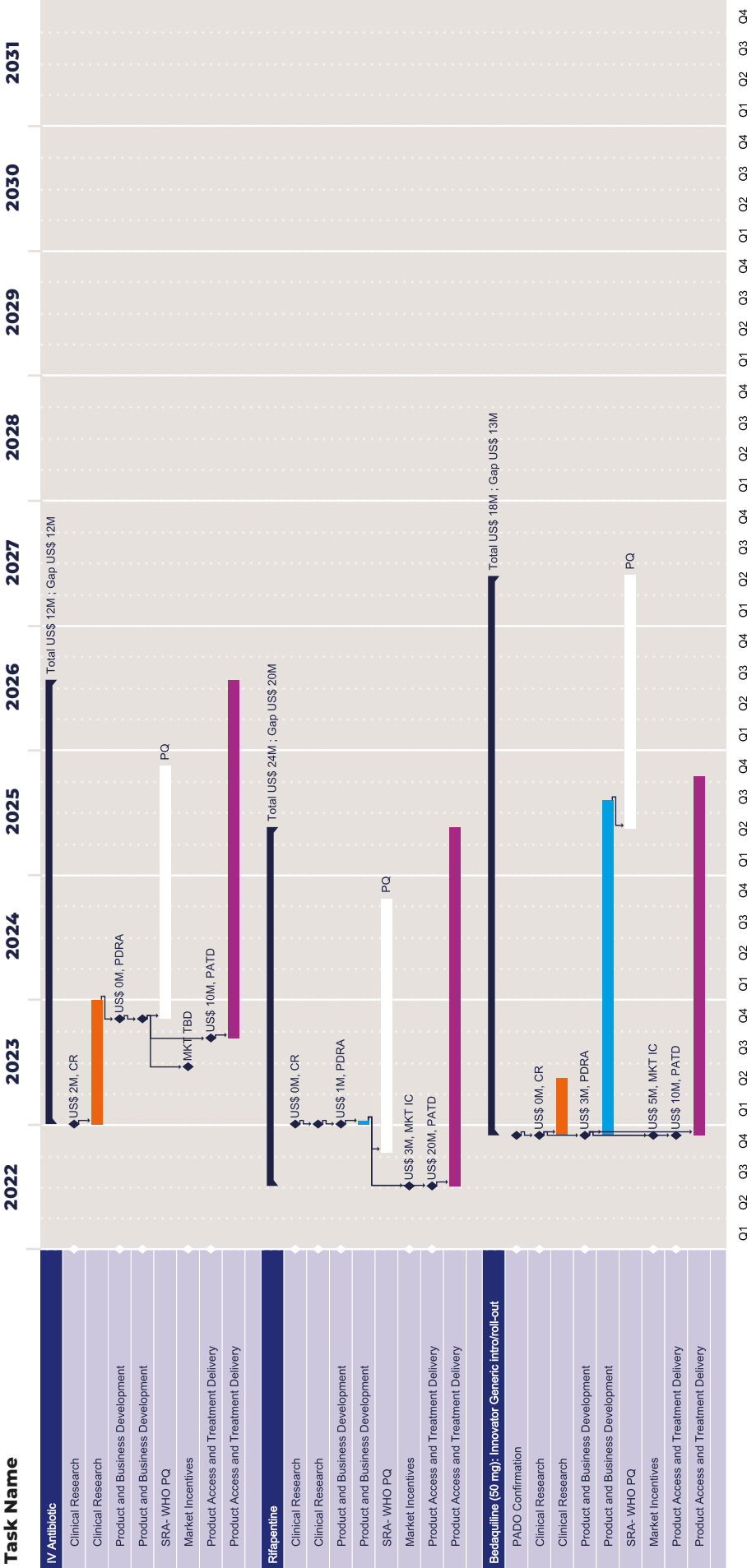


Annex 3. Timelines and Funding envelope to support investigation, development and introduction of priority products

Fig.A3.1







Annex 4. Costing guidelines

Table.A4.1

Category	Definition	Cost drivers	Example or comment	Estimated range
Clinical research	<p>Consider only clinical research when paediatric dosing can be extrapolated from adult dosing</p> <p>For some products, this is strictly modelling; for others, it includes a small clinical trial</p>	<p>Degree of extrapolation</p> <p>Number of months, number of children, number of sites and site readiness</p> <p>Varies by disease area rather than product type</p>	<p>CV-like studies were estimated to be at the lower end of the range (US\$ 1 million) given the short treatment duration</p> <p>TB or cancer were postulated to be at the higher end of the range (US\$ 5 million) due to longer time needed for enrolment and the duration of the study</p> <p>Some costing exercises combine clinical research with development costs. In these situations, stand-alone estimates were not possible</p>	US\$ 1 million to US\$ 5 million
Contract manufacturing organization	Includes only manufacturing costs and not the upfront development costs for the batch even if the contract manufacturing organization is used to support clinical work and early development	<p>Amount of time needed (more than the amount of material)</p> <p>Disease area</p> <p>Product type (oral, solid etc.)</p>	<p>Where clinical trial material must be manufactured by a contract manufacturing organization, these costs mostly depend on the type of product</p> <p>Oral solids for drug areas like HIV and hepatitis C are likely to be at the lower end of the range (US\$ 500 000)</p> <p>Sterile injectables that may be used in cancer or antibiotics would tend to be at the high end of the range (US\$ 2 million)</p>	US\$ 0.5 million to US\$ 2 million
Development or supplier	<p>Business development, product development, and regulatory affairs</p> <p>Excludes the costs of active pharmaceutical ingredient development</p> <p>Regulatory affairs costs do not include actual regulatory fees</p> <p>Excludes development incentives</p>	<p>Type of formulation</p> <p>Number of suppliers, which is a function of market size, size of the adult market and the need for supply security</p>	<p>Oral solid tablets are far simpler to develop and manufacture and will likely be at the lower end of the range (US\$ 1 million)</p> <p>Sterile injectables would tend to be at the high end of the range (US\$ 3 million)</p> <p>Various disease areas target development incentives between 50% and 100% of the total development cost</p> <p>Higher development incentives are associated with less economically attractive markets and where the formulation for children substantially differs from and is more complicated than the adult formulation</p>	<p>Cost per manufacturer = US\$ 1 million to US\$ 3 million</p> <p>Number of manufacturers is based on market dynamics</p>

Category	Definition	Cost drivers	Example or comment	Estimated range
NGO development support	<p>Costs incurred by an NGO or a product development partnership that provides technical assistance to a supplier developing a formulation for children</p> <p>Includes technology transfer, support along the development path, support for development and filings; for product development partnerships, this may also include clinical support</p>	<p>Definition of development support (whether it includes clinical support)</p> <p>Limited leverage across drugs or suppliers</p>	<p>A modest two-year engagement by an NGO or a product development partnership with each manufacturer will likely fall at the lower end of the range (US\$ 0.3 million)</p> <p>A broad and involved relationship will tend towards the higher end of the range (US\$ 1 million)</p> <p>For an organization providing support, little leverage was reported across suppliers for the same product (to support two suppliers on the same product, the costs doubled)</p>	<p>Cost per manufacturer = US\$ 0.3 million to US\$ 1 million</p> <p>Number of manufacturers is based on market dynamics</p>
Market incentives	<p>Incentives used to jump start the transition to a new product for children such as pooled procurement or catalytic procurement</p> <p>May be used in conjunction with development incentives such as with risk sharing or guaranteed batch buying</p>	<p>May or may not be necessary depending on the market</p>	<p>Accelerating formulation development may require a different tool than supplier market entry or risk reduction</p> <p>Unlikely other cost categories, there is less of a formulaic approach to where costs fall and more of a nuanced understanding of the market and experience with incentives to determine the size and type of incentive, but these approaches typically range from US\$ 1 million up to US\$ 5 million per product</p>	<p>US\$ 1 million to US\$ 5 million</p>
Introduction cost per country per introduction period	<p>Cost for technical assistance to a global supplier associated with product introduction</p> <p>Cost for one-time, in-country costs associated with bringing a new product to market and driving uptake</p> <p>Country costs include any global team or organizational global support costs</p> <p>Two- to three-year introduction period</p>	<p>Maturity of the market</p> <p>Experience of countries with introducing new products</p> <p>Cost structure of introduction partners</p>	<p>For markets that have already introduced a number of products, such as HIV, the costs tend to be at the lower end of the range (US\$ 0.75 million)</p> <p>For countries with more nascent markets, such as cancer or hepatitis, costs may be at the higher end of the range (US\$ 2 million)</p>	<p>Cost per country per period = US\$ 0.75 million to US\$ 2 million</p> <p>The number of countries is based on disease strategy and country readiness</p>

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