# Medicines for Europe Factsheets on
# **Boosting affordable innovations**

Medicines for Europe is committed to improving access to medicines for all Europeans. Yet, many patients across Europe face restricted access to medicines which undermines public health. The 2019-2024 EU legislature should reshape pharmaceutical policy by prioritising **equitable access to essential medicines** for all Europeans. Equitable access is an achievable goal as the majority of essential medicines are already generic or biosimilar medicines.

There is significant untapped potential in Europe to **optimise existing therapies**. Whereas the strategy covers the full cycle of medicines, by fostering continuous off-patent patient-centric innovation, a molecule can be repurposed, reformulated and combined with new digital technologies (complex combination) to address unmet patient needs and contribute to the sustainability of the healthcare system. These so-called "value added medicines" are increasing the quality of life for patients with chronic diseases and offer significant benefits to the healthcare community.

**This document develops Medicines for Europe key priorities that should be reflected in the Pharmaceutical Strategy for Europe.**

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# 1. Value Added Medicines

The Pharmaceutical strategy for Europe aims to address unmet health needs and the accessibility and affordability of medicines. Value Added Medicines are defined as an accessible, affordable innovation to address health needs that are especially important to larger patient populations in both, communicable and non-communicable disease management. We recommend the establishment of a new, simplified regulatory pathway for VAMs. By recognising VAMs as a category of innovation with a dedicated pathway and tailoring the system of incentives provided by the EU pharmaceuticals framework to support innovation throughout a molecule's lifecycle, we can achieve a complete and resource-efficient EU pharmaceutical industry while delivering medicines to satisfy the unmet need and improve the lives of patients in Europe.

## How can Value added medicines make a difference to patients and healthcare systems during a pandemic and beyond?

**REPOSITIONING - FINDING NEW INDICATIONS TO ADDRESS UNMET MEDICAL NEED**

Dexamethasone, an affordable steroid, repurposed for Covid-19 treatment, reduced deaths by 1/3 in hospitalised Covid-19 patients receiving mechanical ventilation.

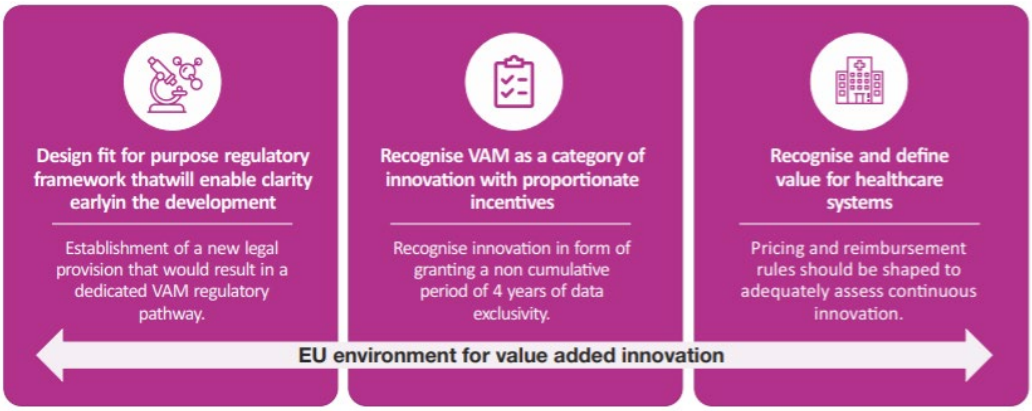
**REFORMULATION - FACILITATING PATIENT TREATMENT IN A HOME CARE SETTING**

Covid-19 dramatically reduced accessibility of care and changed patients' needs in a number of ways. VAMs can support patient-centred reform of care with medicine reformulation and offer patients new ways to administer their own treatments at home and avoid in-person hospital visits.

**COMPLEX COMBINATIONS - UTILISING DIFFERENT RESOURCES TO DELIVER THERAPY**

Digital Value Added Medicines, combine medicines with innovative technological solutions and can support the patient-HCP relationship and improve treatment adherence in a remote care setting.

## Policy recommendations:




## Relevant documents:

Medicines for Europe White Paper: Creating a European Ecosystem for safe, timely and affordable patient-centric innovation

# 2. Orphan & paediatric medicines incentives

## Background

EU Pharmaceutical legislation provides for 10-yr of market exclusivity for medicines for rare diseases (orphan) with free regulatory advice and fee reductions and a 6-month supplementary protection certificate (SPC) for paediatric medicines. For off-patent paediatric medicine developments (PUMA), there is a 10-yr market exclusivity. For orphan/paediatric medicines, there is a choice between a 6-month SPC or an additional 2-yr orphan exclusivity.

**The scope of the review**

As urged in the Health Council Conclusions of 2016, in the context of the EU Pharmaceutical Strategy, the EC will propose amendments to the orphan & paediatric legislation in 2022 since there is:

- *Insufficient development in areas of greatest unmet needs à 95% of rare disease are still without treatment*
- *Limited availability & accessibility across Member States à incl. delayed generic & biosimilar competition*
- *Multiplication of rare diseases out of common diseases*

The Health Council Conclusions of 2016 also called for timely access to generic & biosimilar medicines, which are fundamental to ensure budgetary sustainability.

To this end, the Commission proposed several options in its Inception Impact Assessment (IIA) on this legislation.

## The recommendations

While incentives have generated some success, there is a need to ensure that incentives achieve the objectives originally intended whilst avoiding abuses/misuses of the system that delay generic/biosimilar competition

**Orphan medicines**

- The overlapping exclusivities blocking generic/ biosimilar competition could be tackled by explicitly stating in the legislation that generics/biosimilars can enter the market for any orphan medicine that has already benefitted from 10yrs of orphan exclusivity
- A specific framework for value added medicines could be developed with proportionate incentives & rewards for the effort invested so to address market failures related to repurposed products and continuous innovation (e.g. off-label prescribing).
- The EU should address the risk of multiplication of exclusivity periods ("salami-slicing" of indications)
- Novel incentives via transferrable exclusivity vouchers should be excluded, also for paediatric medicines, as they would extend monopolies on more profitable products, increasing costs for HC budgets, legal uncertainty incl. on market formation dates & unduly delaying access to generics/biosimilars

**Paediatric medicines**

- While the proposals include the need to restrict the use of the 6-month SPC extension where no unmet need exists, it is key for legal certainty to ensure early clarity on future paediatric SPC extensions
- There is a recognised market failure of the off-patent incentive (PUMA). As stressed in the pharma strategy communication, there is need to "*stimulate innovation in particular in areas of unmet needs*", incl. off-patent paediatric developments, where there is "*absence of commercial interest*". As is proposed for novel antimicrobials, the reform should include pull incentives based on new P&R models to incentivise development of off-patent paediatric medicines, but also free pre-submission scientific advice (as for orphans) & clear framework for repurposed off-patent products for new indications for children only

**Use in combination of orphan/paediatric incentives**

- A holder of a product with orphan exclusivity should be prevented from withdrawing the orphan designation in order to obtain an SPC extension later as this delays effective competition & patient access.

To stimulate faster competition from follow-on orphan developments (ie. on day-1 of exclusivity expiry) & investments in off-patent paediatric products, the reform should:

**Incentives for follow-on orphans & off-patent paediatric products**

- Tackle barriers to development by tailoring clinical requirements for biosimilars based on science & allow single development for multiple jurisdictions (comparable to international harmonisation of paediatric & orphan development of originator products).
- Facilitate access to reference product for clinical trials
- Remove barriers to day-1 launch after protections expire by banning patent linkage, harmonising the Bolar exemption & introducing uptake measures to stimulate competition
- Reduce timelines and obstructions to P&R decisions in line with Bolar
- P&R uptake measures to encourage investments in follow-on orphan development