PHARMACEUTICAL STRATEGY FOR EUROPE

Update on public consultation on review pharmaceutical legislation

CEF meeting 10 November 2022

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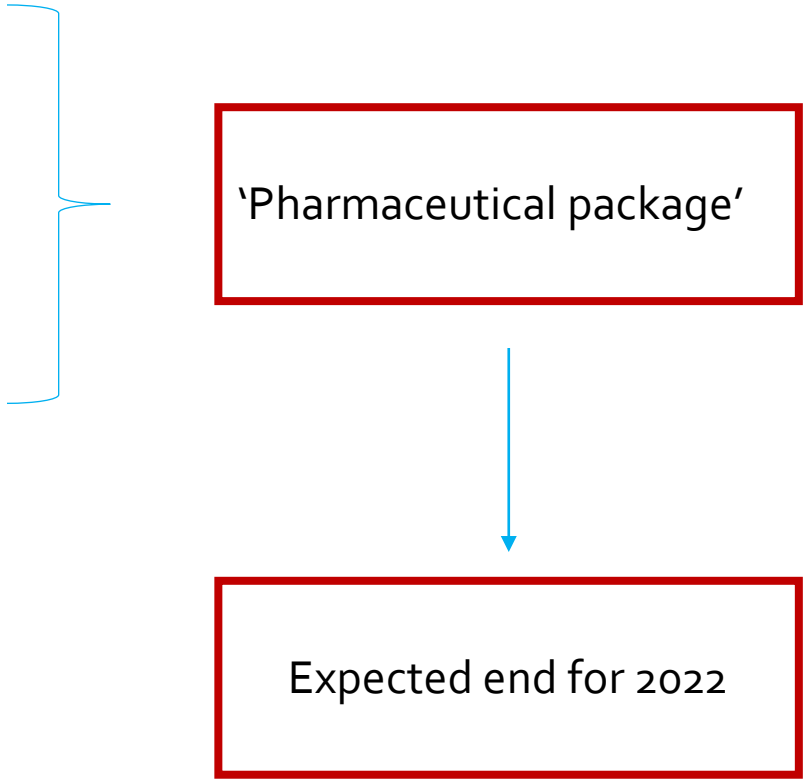
In the next 45 minutes

- Where are we coming from – a quick reminder
- The 'Pharmaceutical Package' – what & when
- Public consultation – your input needed

Contained in the EC work programme 2022

- Revision of the pharmaceutical legislation
- Revision of the legislation on supplementary protection certificates
- Revision of the EU legislation on medicines for children and rare diseases



2021 Legislative measures

- Adoption of Regulation on **Health Technology Assessment**
- Legislative proposal on **European Health Data Space**
- **Review of variation framework for medicines** – to be completed by 2023
- Full implementation of **regulatory framework for clinical trials**
- Legislative proposal for **EU Health Emergency Preparedness and Response Authority (HERA)**
- Review of the **EMA fee legislation**

- Public consultation on **OMP / Paediatric Medicines (CLOSED)**
- Public consultation on **Cross Border Healthcare (CLOSED)**
- Public consultation on **European Health Data Space (CLOSED)**
- Public consultation on **Pharmaceutical legislation (OPEN 28/09)**

2021 Non-legislative measures

- Develop **cooperation** among regulatory authorities on **pricing, payment and procurement policies** – to be completed in 2024
- Launch pilot with industry and academia to **test framework for repurposing off-patent medicines** and inform **possible regulatory action**
- Initiate **pilot** with EMA, Member States and MAHs to **explore causes of deferred market launches**
- Consider **voluntary process to increase transparency on the supply chains**
- Launch a dialogue with actors in the **pharmaceutical manufacturing value chain** and **public authorities** to identify **vulnerabilities in the global supply** of critical medicines, raw materials and APIs
- Dialogue with MS on **guidelines on principles and costing methods for establishing the R&D costs** of medicines – to be completed in 2024
- Enhance dialogue among authorities to increase **cooperation on evidence generation**
- Pilot approaches to EU **R&D and public procurement for antimicrobials** and alternatives to provide pull **incentives for novel antimicrobials**
- Upgrade register of centrally authorised products to include a statistical dashboard and **make data fully available for secondary use**
- Initiate pilot with EMA and Member States on **cross-border data analysis of health data** – to be completed in 2025
- Support public-private partnerships through **Innovative Health Initiative**

Relevance of incentives

APPROACHES FOR BETTER ADDRESSING THE NEEDS OF RARE DISEASE PATIENTS

	Very adequate	Moderately adequate	Not at all adequate	Total
Q3.1. When considering whether a particular medicine is eligible for support, the rarity of the disease – the total number of cases of a disease at a specific time, currently less than 5 in 10 000 people – forms the main element of the EU rules on medicines for patients suffering from rare diseases.	146 (50%)	112 (38%)	33 (11%)	291 (100%)
Q3.2: Some diseases occur frequently, but last for a relatively short period of time (for example, some rare cancers). These are covered by the EU rules on medicines for rare diseases and the principle of rarity. However, because many patients acquire such diseases during a specified, limited period of time, those diseases should not be considered as rare in the EU anymore.	53 (18%)	59 (20%)	178 (61%)	290 (100%)
Q3.3: Amongst all medicines for rare diseases which become available to the EU patients, only those bringing a clear benefit to patients should be rewarded. Clear rules should apply to decide if one medicine brings a clear benefit to patients when compared to any other available treatment in the EU for a specific rare disease.	161 (56%)	99 (34%)	29 (10%)	289 (100%)
Q3.4: Additional incentives and rewards should exist for medicines that have the potential to address the unmet needs of patients with rare diseases, for example in areas where no treatments exist.	233 (81%)	35 (12%)	19 (7%)	287 (100%)

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Source: compiled by the study team based on the OPC Q3: In your opinion, how adequate are the approaches listed below for better addressing the needs of rare disease patients?

Unmet needs

Study supporting the Impact Assessment of the revision of the EU legislation on medicines for children and rare diseases

DEFINITIONS OF UNMET THERAPEUTIC NEEDS OF RARE DISEASE PATIENTS AND CHILDREN

	NUMBER OF RESPONSES	SHARE OF RESPONSES
Authorised medicines for a particular rare disease or a disease affecting children are not available, and no other medical treatments are available (e.g. surgery).	273	90%
Treatments are already available, but their efficacy and/or safety is not optimal. For example, it addresses only symptoms.	235	77%
Treatments are available, but impose an elevated burden for patients. For example, frequent visits to the hospital to have the medicine administered.	206	68%
Treatments are available, but not adapted to all subpopulations. For example, no adapted doses and/or formulations, like syrups or drops exist for children.	233	77%
Other *(please specify)*	176	58%
TOTAL	**297**	**98%***

Note: There were 7 (2.3%) respondents who provided no answer to this question.*

Source: compiled by the study team based on the responses to the OPC Q5: What do you consider to be an unmet therapeutic need of rare disease patients and children? (N=298)

Support for proposed measures

MEASURES FOR BOOSTING THE DEVELOPMENT OF MEDICINES ADDRESSING UNMET THERAPEUTIC NEED OF PATIENTS SUFFERING FROM A RARE DISEASE AND/OR FOR CHILDREN

	NUMBER OF RESPONSES	SHARE OF RESPONSES	WEIGHTED SCORE*
Assistance with Research & Development (R&D), where medicines under the development can benefit from national and/or EU funding.	292	96%	2561
Assistance with authorisation procedures, such as priority review of the application from the European Medicines Agency and/or expedited approval from the European Commission.	289	95%	2320
Additional scientific support for the development of medicines from the European Medicines Agency.	289	95%	2281
Additional post-authorisation incentives that complement or replace the current incentives and rewards.	288	95%	2259
Do you have other suggestions that would allow the EU to boost the development of specific medicinal products? *(please specify)*	212	70%	N/A
Do you see any drawbacks with the approaches above? (*please describe*)	179	59%	N/A

PUBLIC CONSULTATION ON PHARMACEUTICAL LEGISLATION

'Pharmaceutical Package'

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2022 Legislative measures

Review of basic pharmaceutical legislation (Directive 2001/83/EC and Regulation (EC) No 726/2004) to:

- **Simplify and streamline approval procedures**
- **Review system of incentives and obligations** taking into account the relationship with IPRs and innovation
- **Adapt to cutting-edge products and scientific developments**
- **Enhance security of supplies and address shortages**
- Give authorities more power to **adapt terms of marketing authorisations based on scientific evidence**
- Revise **manufacturing and supply provisions**
- Address barriers to **competition** and **market effects on affordability**
- Improve **access to generics and biosimilars**
- Strengthen **environmental requirements** and conditions of use for medicines
- Introduce **measures and incentives to optimise antimicrobials use**

Simplify and upscale SPC framework

Review of **OMPs and Paediatrics Regulations**

Incorporate **EMA PRIME scheme** in regulatory framework

Adapt **regulatory requirements for medicines that contain GMOs**

Review **framework on good manufacturing practice** and encourage inspections to improve compliance

2022 Non-legislative measures

Launch of EMA & EU Commission pilots to **test fitness of pharma framework to cutting-edge products development**

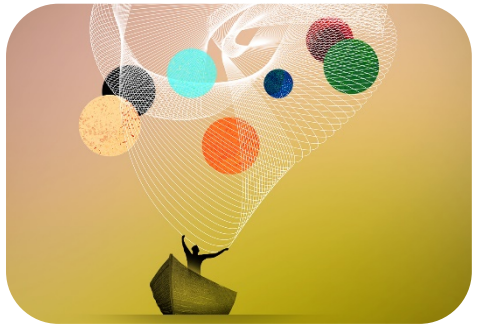
Develop and implement **Electronic Product Information for all EU medicines**

Initiate regulatory pilots of EMA & EU Commission to test **adaptability of pharma framework for new cutting-edge product developments**

What's in the public consultation

Unmet medical needs and market failures for medicines other than medicines for rare diseases andchildren;

Unequal access to available and affordable medicines for patients across the EU;



The current legislative framework may not be fully equipped to respond quickly to innovation;

Inefficiency and administrative burden of regulatory procedures;

Vulnerability of supply of medicines, shortages of medicines;

Environmental challenges and sustainability;

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RARE DISEASES EUROPE

FOCUS ON UNMET NEEDS, ACCESS TO TREATMENT, TABLE OF NEGOTIATION AND EU FUND

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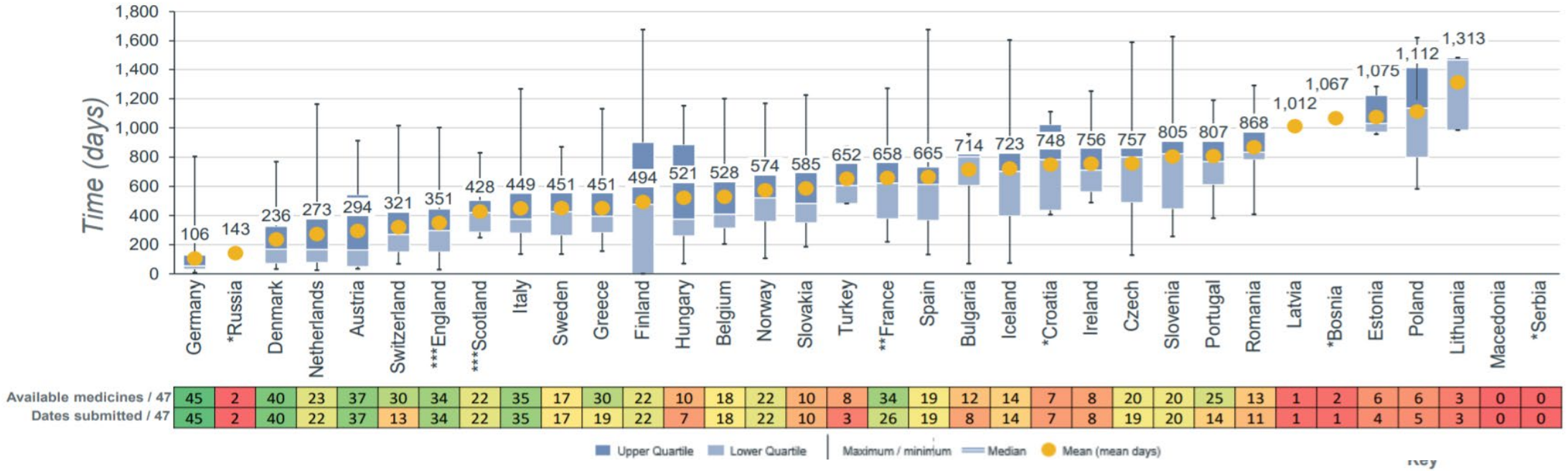
Important time to availability differences between EU countries



Measure	EU average for all products	Oncology	Orphan	Non-oncology orphan	Combination therapy
Rate of availability	49%	58% ↑	41% ↓	34% ↓	64% ↑
Average time to availability	504 days	561 days ↑	653 days ↑	667 days ↑	411 days ↓

Arrows indicate direction from the EU average for all products

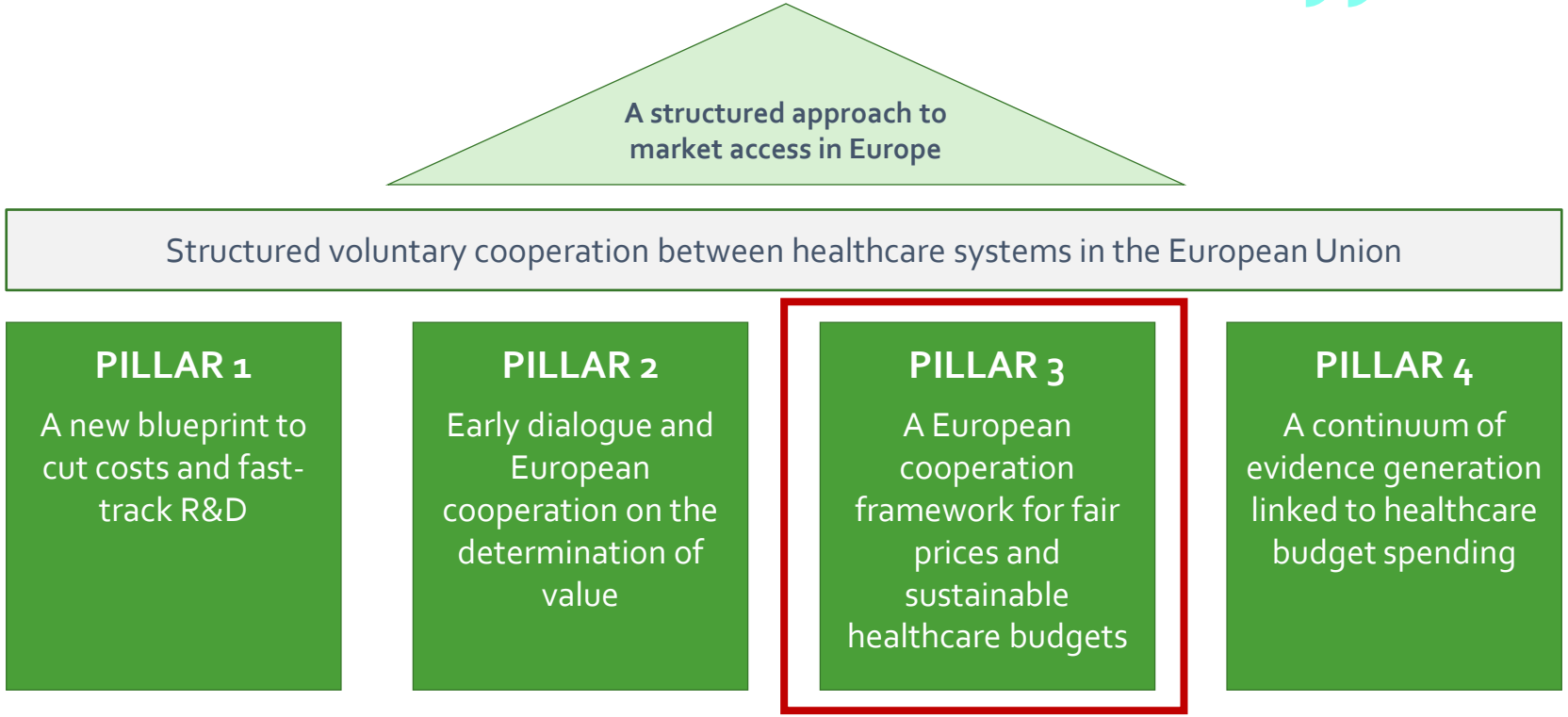
↑ *Higher availability* ↓ *Lower availability*

↓ *Shorter* ↑ *Longer*

SOURCE: IQVIA (2021)

EURORDIS' key proposals on a more equitable and efficient patient access across Europe

"The ambition of EURORDIS is to have 3 to 5 times more new rare disease therapies approved per year, 3 to 5 times cheaper than today by 2025"



SOURCE: EURORDIS, 'BREAKING THE ACCESS DEADLOCK' (2018)

Key changes needed

III

PILLAR 3

A European cooperation framework for fair prices and sustainable healthcare budgets

European Table of Negotiation

- **Who:** CAPRs from EU Member States
- **What**: pooling based on population (and GDP)
- **Why:** (1) to increase collaboration between MS; (2) to provide a space for well-informed dialogue; (3) to enact the commitment to approach P&R based on the balance of value, volume and evidence generation
- **How**: by consolidating existing initiatives (MoCA)

European Transactional Price and Differential Pricing

1. Establish a European Transactional Price: 'fair' price negotiated at the EU Table of Negotiations, serving as a **benchmark**
2. Adjust the transactional price to match MS' **ability to pay (equity tiered based pricing)**
3. Exempt OMPs from **external reference pricing** and **parallel trade**

Establishing a 'fair' price

Option 1: based on "*justification of the price*"

Option 2: based on "a dynamic, mutually constructed approach to incentivize value and healthcare priorities"

Cost-based price	×	*Agreed product value*	×	*Investment bonus/malus*
All structural costs 20% profit margin		10-100% based on product value		Premiums of 0-30%

New approaches to funding

- Use **outcome-based payments and other types of financing arrangements** for specific RDs with established RWE collection
- Manufacturers to grant a temporary **discount on uncertainty** proportional to the level of uncertainty
- Explore widespread used of **joint purchasing**

Towards a 'Table of Negotiations'

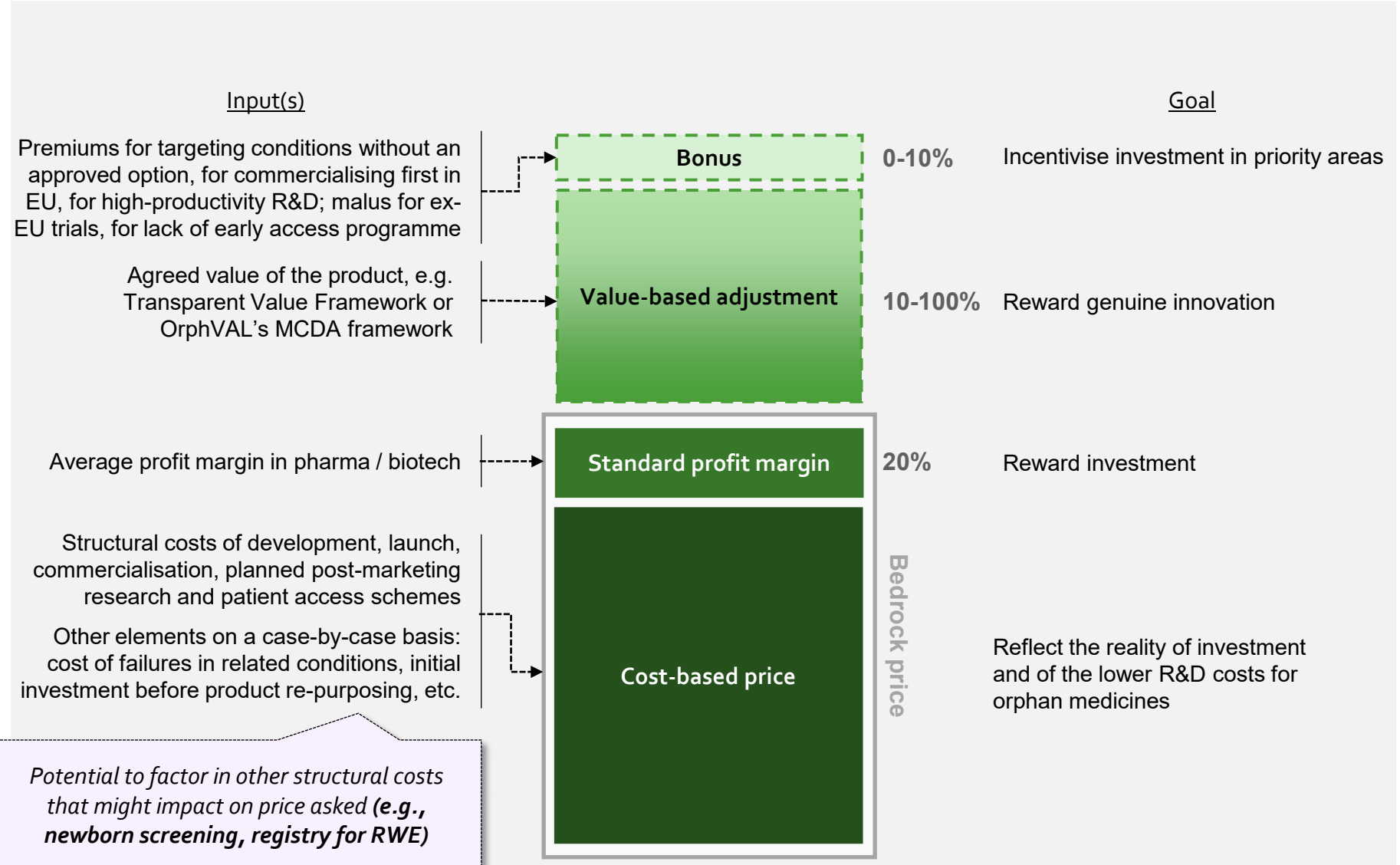
1. A **stronger European collaboration** between the national competent authorities of several EU Member States (MS),
2. A **trusted space for a well-informed dialogue,** helping participating authorities to engage with the industry
3. A commitment to approaching P&R decisions based a **balance of three factors: value, volume, and evidence generation**

- Grouping of voluntary MS based on demographic and/or economic aspects
- Joint negotiations to be conducted within an EU comprehensive framework
- Achieved by consolidating existing initiatives, e.g., MoCA and regional initiatives (BENELUXAI, FINOSA, etc.)
- Contributing to allowing for earlier patient access (e.g. initiation of price negotiations around time of CHMP decision)

New models can be explored and start being implemented, for example:

- *Apply **consistent approach** on value principles, value determinants for assessment and European HTA clinical assessment*
- ***Flexible agreements** based on outcomes or other financial aspects*
- ***Discounts on uncertainties**, and allowing price to fluctuate over time based on additional generated evidence*
- *Registries and post-marketing authorisations activities coordinated cross-border (ERN) co-funded by a **European fund to reduce uncertainties***
- ***Joint purchasing** based on European legislation (and examples from COVID19, not only vaccine-related)*
- ***Focus on smaller population** first then progressively expands negotiations to more prevalent diseases*
- *Transactional Price + **Differential pricing (or equity tier based pricing)** + control over parallel trade*

A conceptual proposal for pricing products based on costs and value



What are we asking for in a 'European Fund'?

To finance the ***generation of evidence for high uncertainty orphan medicines*** from the time point of marketing authorisation up to the first reassessment of their value.

Member States (which would be subject to less financial pressure in the early days of the commercialisation of a new orphan medicine);

Pharmaceutical manufacturers (lesser short-term unpredictability about access, plus better chances of generating valuable real-world evidence);

and above all of rare disease patients, who would be able to receive rapid and full access to the medicines they need.

A new life cycle perspective on access to rare disease therapies

NOW

Horizon scanning: "*passive and reactive*" process, whereby MS do not directly influence innovation

Price set based on limited evidence, setting up for tense negotiations and commercial deals

***Ex ante* assessment** of value based on a level of evidence often insufficient to perform a stringent effectiveness assessment

FUTURE

Horizon shaping: governments to drive which new treatments are needed and should be developed

Formative assessment at the moment of implementation in clinical practice

Real-world data generation through registries, with support from ERNs

Price evolution (upwards or downwards) linked to evidence generation on real-life use and effectiveness

Summative assessment offering a 360° view of the full impacts of the innovation under study

European Fund to co-fund RWE generation from marketing authorization for ~3 years and/or pay for a share of the cost of treatment in a scheme with MSs