European Commission

EU Pharmaceutical reform: **Steering innovation to address unmet medical needs**

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WHY THE REFORM?

Many serious diseases still lack appropriate treatment. Current investments in developing medicines do not always prioritise the greatest unmet medical needs. This is particularly true for diseases that face scientific challenges (e.g. limited understanding of the disease) or limited commercial interest (e.g. rare diseases). As a result, there are serious diseases, such as certain cancers or neurodegenerative diseases, where satisfactory treatments are still lacking. In addition, there are over 6000 known rare diseases, with 95% currently having no treatment option.

WHAT DOES THE REFORM ADDRESS?

Our proposal aims to steer pharmaceutical investment into medicines for unmet medical needs. The proposal defines **specific criteria for unmet medical needs (UMN)** to ensure that the products concerned bring an added therapeutic value to patients that suffer from serious diseases that are not yet sufficiently addressed by existing treatments.

All medicines for rare diseases are considered to address a UMN. In addition, to promote further development of orphan medicines for underserved rare disease areas and to reward exceptional therapeutic advancements, we are proposing an **additional category of high unmet medical need (HUMN)**.

Targeted regulatory protection incentives and regulatory support for medicines addressing UMN and HUMN will help steer innovation from the current supply-driven model towards a **more need-driven innovation model**, that will better serve **patients and health systems**.

The EMA will develop detailed guidelines for the application of these criteria with input from health technology assessment (HTA) bodies, pricing & reimbursement (P&R) authorities, patient organisations and other relevant groups and will establish a forum for regular exchanges of information and pooling of knowledge on general scientific and technical issues.

This new approach will create a more seamless process along the lifecycle of a medicine, ultimately accelerating patient access to medicines.

The criteria for UMN and HUMN are likely to have important additional positive outcomes, much like when the orphan designation was introduced in the EU Orphan Regulation in 2000. The concepts of UMN and HUMN, thanks to their strict criteria, would make it easier to identify medicinal products with particularly high value for patients and help **channel public resources – either through research funding or through favourable P&R conditions – towards them**.

OUR OBJECTIVE

To use EU regulation to incentivise pharmaceutical companies to develop and launch products that address unmet medical needs and to bring them more quickly to patients.

The reform of the EU's pharmaceutical legislation offers:

1 Regulatory protection incentives

Companies can benefit from an additional period (+6 months) of regulatory data protection (RDP), if they develop a medicine that meets the UMN criteria.

All orphan medicines are considered to address an UMN, and therefore benefit from the additional 6 months RDP. In addition, orphan medicines that meet the criteria for HUMN, will benefit from an additional period (+1 year) of **market exclusivity**.

2 Increased regulatory support for developers

The EMA offers scientific support to medicine developers on how to generate robust evidence for authorisation e.g., early scientific advice on the design of clinical trials.

Under the pharma reform, developers of medicines for HUMN or promising medicines for UMN will receive **enhanced scientific support and accelerated assessment (PRIME)**.