The Consumer Voice in Europe

REFLECTION PAPER ON A PROPOSAL TO ENHANCE EARLY DIALOGUE TO FACILITATE ACCELERATED ASSESSMENT OF PRIORITY MEDICINES (PRIME)

BEUC response to the EMA public consultation

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1. General comments

Stakeholder number *(To be completed by the Agency)*	General comment (if any)	Outcome (if applicable) *(To be completed by the Agency)*
	BEUC welcomes the opportunity to comment on the European Medicines Agency *Reflection paper on a proposal to enhance early dialogue to facilitate accelerated assessment of priority medicines (PRIME)*. Consumers welcome improvements to the design and conduct of clinical trials in order to maximize the quality of data collected while minimizing the risk to participants and adhering to good governance standards. Consumers also see the value in developing an expedited process to bring a limited number of medicines with a clearly defined and demonstrated impact on public health to the market. Regardless of which process is followed, consumers trust regulators to ensure that the benefits of medicines available on the market outweigh their risks. However, experiences in the US show that expedited regulatory evaluation programmes have resulted in safety implications for patients, including a higher risk of serious adverse drug reactions (ADRs) and higher	

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	rate of patient information leaflet (PIL) revisions for dose, safety and efficacy issues.[1] Any move to bring unproven medicines to the market sooner raises many questions about patient safety and consumer protection. With these general concerns in mind, we wish to make the following specific recommendations to the *Reflection paper on a proposal to enhance early dialogue to facilitate accelerated assessment of priority medicines (PRIME)*.	

[1] Kesselheim et al. JAMA 2011;305:2320-6 and Berlin. Am J Pub Hlth 2009;99:1693-8

2. Specific comments on text

Line number(s) of the relevant text *(e.g. Lines 20-23)*	Stakeholder number *(To be completed by the Agency)*	Comment and rationale; proposed changes *(If changes to the wording are suggested, they should be highlighted using 'track changes')*	Outcome *(To be completed by the Agency)*
43		Comment: The PRIME scheme focuses on developing new medicines to address major public health needs. A clear definition of a major public health need is lacking. This is necessary to set the scope and boundaries of the PRIME scheme.	
52		Comment: There are many conceptions of what medicines innovation means and it is necessary to specify how the EMA defines 'therapeutic innovation'. BEUC highlights that true therapeutic innovation is the development of medicines that have added value compared to existing alternatives.	
90		Comment: A clear definition of an 'unmet medical need' should be agreed**.** A lack of a definition could enable the excessive use of the PRIME scheme in inappropriate situations, thereby wasting resources and potentially exposing consumers to unnecessary risks associated with expedited assessment.	
Multiple		Comment: There should be a clear link between the unmet medical need and the product considered for PRIME. Three	

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		elements of justification are crucial to ascertain the suitability of potential products for the PRIME scheme: the scope of the unmet medical need, the extent to which the product fulfils that need and is safe for consumers to use, and the strength of the evidence. Proposed changes: (line 89) As such, products eligible for PRIME support **shall** target conditions where there is an unmet medical need,… (line 93) In these conditions, a product eligible for PRIME support **shall** demonstrate a positive benefit/risk ratio **and** the potential to address to a significant extent the unmet medical need for maintaining and improving the health of the Community… (line 203) In general, the justification may be more convincing if based ~~**as much as possible**~~ on epidemiological data about the disease… (line 205) These claims **shall** be substantiated e.g., from published literature or registries or healthcare databases. (line 209) A description of the available treatment	

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		options/standard of care (SOC), including all relevant treatment modalities, …, radiotherapy **shall** be included. The effect of available treatments **shall** also be described together with a description of how the medical need is not fulfilled by the available treatments. (line 218) The justification **shall** include a description of the medicinal product's observed and predicted effects, their clinical relevance, the added value of the medicinal product and its impact on medical practice.	
98		Comment: Only clinically significant impacts are valuable for patients and should be part of the eligibility criteria for PRIME. Proposed change: Data available to support a request for eligibility should support the claim that the product has the potential to bring a major therapeutic advantage to patients, through a meaningful improvement of efficacy, such as having a **clinically significant** impact **for the patient** on the onset and duration of the condition…	
118		Comment: Disclosure of the data used to determine a product's eligibility for the PRIME scheme aids patients' and healthcare professionals' understanding of the	

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		rationale for regulators' decisions and contributes to a restoration of public confidence in regulators following recent scandals which have affected the medical sector. Proposed change: In case of a subsequent centralised marketing authorisation, ~~**reference to**~~ **the data used to show the product's** eligibility to the PRIME scheme granted by the CHMP will be mentioned in the European Public Assessment Report **and the summary**.	
132-149		Comment: It is vital to ensure that regulators' involvement in scientific or regulatory advice does not undermine their independence. The Reflection Paper indicates that the CHMP/CAT Rapporteur will be appointed at an early stage (line 132) to 'enable continuity in a lifecycle approach' (line 143), will participate in meetings with the applicant (line 134-135) and will provide scientific and regulatory advice (lines 146-149). BEUC would have strong reservations about this scheme if the CHMP/CAT Rapporteur is the same individual who will serve as Rapporteur for a future market authorization application for this product. To avoid any potential conflict of interest, those individuals involved in scientific or regulatory advice on behalf of the EMA should not be involved in the evaluation of the marketing authorization application.	

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		To maintain public trust in the EMA's objectivity, there should be a section introduced in the reflection paper to indicate how conflicts of interest will be identified and prevented.	
166		Comment: When monitoring development (chapter 5), the product being tested should always be compared with available alternatives/standard treatment. This is essential if PRIME is to target unmet medical needs and to determine if products are still eligible for PRIME (line 179).	
251-253		Comment: We note that the use of intermediate endpoints is most valuable and certain when their relationship with clinical outcomes is validated. Proposed changes: Established surrogate, other **validated** intermediate endpoint or pharmacodynamics marker ~~**that strongly suggest the potential for a clinically meaningful effect**~~ can be used to justify eligibility for PRIME support.	

END

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