Heath Technology Assessment Network

Update on the European Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP)

10 November 2016

European Commission

Health and Food Safety

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STAMP - the first two years

- **Early access initiatives**
  conditional marketing authorisation, adaptive pathways and PRIority MEdicines (PRIME)
- **Compassionate use**
- **Repurposing of established medicines**
- **Off-label use** of medicinal products
- **Council conclusions**
- Synergies with **Health Technology Assessment** Network (reflection paper)

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Conditional marketing authorisation (CMA)

- **Scope** - limited to seriously debilitating/life-threatening diseases
- **Criteria –** "unmet medical need"; "major therapeutic advantage". Consider role of improved patient care (major therapeutic advantage).

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CMA: important issues raised

- amendment of an existing marketing authorisation to include a new 'conditional' indication
- reinforce actions in case of non-compliance with specific obligations
- reinforce the prospective planning of CMA application

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Adaptive Pathways

EMA Pilot

- Launched in March 2014 – closed 2016
- Relevant for medicines with the potential to treat serious conditions where there is an unmet medical need
- Cooperation between a wide range of stakeholders

- **Scientific issues - EMA pilot project**
- **Legal-policy issues - 28 Member States in the Pharmaceutical Committee**
- **Report of the pilot published 3 August 2016**
- **Stakeholder workshop planned 8 December 2016**

PRIME - webpage and supporting documents

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- Scientific advice and protocol assistance
- Support for early access
  - PRIME: priority medicines
  - Accelerated assessment
  - Conditional marketing authorisation
  - Compassionate use
- Adaptive pathways
- Scientific guidelines
- Innovation Task Force
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- Orphan designation
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PRIME: priority medicines — Email | Print | Help | Share

PRIME - PRIORITY MEDICINES

PRIME is a scheme launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.

Through PRIME, the Agency offers early and proactive support to medicine developers to optimise the generation of robust data on a medicine's benefits and risks and enable accelerated assessment of medicines applications.

This will help patients to benefit as early as possible from therapies that may significantly improve their quality of life.

Accelerated assessment

PRIME builds on the existing regulatory framework and tools already available such as scientific advice and accelerated assessment. This means that developers of a medicine that benefitted from PRIME can expect to be eligible for accelerated assessment at the time of application for a marketing authorisation.

Fostering early dialogue

By engaging with medicine developers early on, PRIME is aimed at improving clinical trial designs so that the data generated is suitable for evaluating a marketing-authorisation application.

Early dialogue and scientific advice also ensure that patients only participate in trials designed to provide the data necessary for an application, making the best use of

Related content

- Support for early access
- Launch of PRIME – Paving the way for promising medicines for patients (07/03/2016)

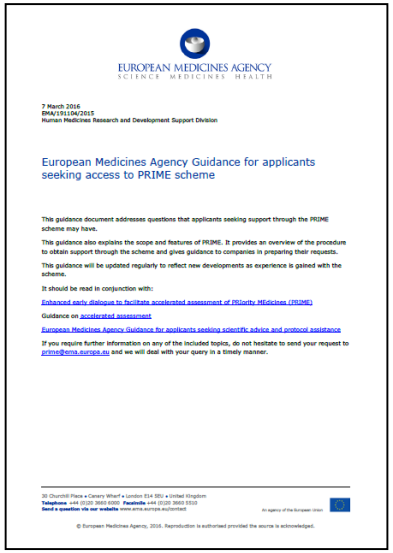
PRIME at a glance - Factsheet

Related documents

- Enhanced early dialogue to facilitate accelerated assessment of PRIority MEdicines (PRIME) (07/03/2016)
- European Medicines Agency guidance for applicants seeking access to PRIME scheme (07/03/2016)
- PRIME eligibility requests:

Factsheet in lay language

Q&A, templates, application form for applicants



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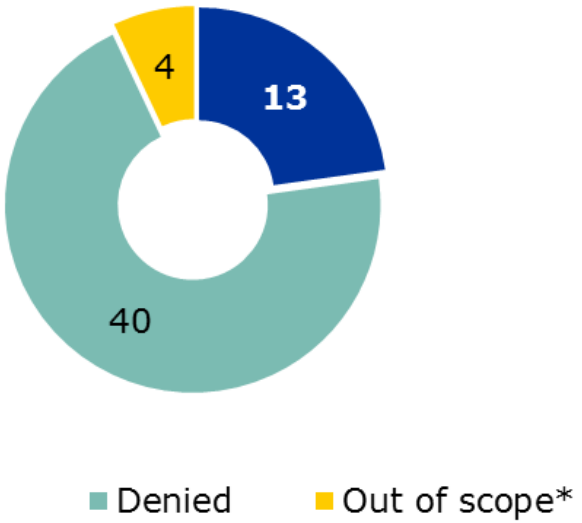
PRIME scheme

- Launched 7 March 2016
- Outcome on 57 applications received until 24 August 2016:



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Compassionate Use

- Member States schemes
- Requests for CHMP opinion on conditions of use of a medicine in compassionate use
  - Exploration of why not widely used

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Repurposing of medicines

- process of identifying a new use for an existing drug in an indication outside the scope of the original indication
- Issues considered:
  - Common usage of medicines that would be suitable for repurposing?
  - Potential barriers and incentives

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Off-label use of medicines

- Belgian Healthcare Knowledge Centre Report "*Towards a better managed off label use of drugs*"

- Presentation by contractor of draft final report on off-label use of medicinal products in the EU
  - Members of STAMP invited to review the draft report and to comment during the meeting or in writing

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Council Conclusions - Actions (Member States)

- Consider further voluntary and Member State driven cooperation on pricing and reimbursement
- Strategic policy reflection and exchange between Member States

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Council Conclusions – Actions

(Member States or Commission)

- Cooperate together and **set clear and enforceable (pre-) conditions regarding the use of early access tools**
- **Further develop cooperation on Health Technology Assessment at EU level**
- **Improve and strengthen dialogue and cooperation (between regulators, HTA bodies)** in existing fora in the field of pharmaceuticals, while also assessing their relevance, functioning and added value
- Analyse effects of pharmaceutical related incentives on the accessibility, availability and affordability of medicines, as well as the price strategies of industry

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Council Conclusions – next steps?

- Ongoing/ foreseen EC studies
- Cooperation- *Consider ways to*:
  - to improve and strengthen dialogue and cooperation between regulators, HTA bodies
  - discuss clear and enforceable (pre-) conditions regarding the use of early access tools

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Thank you for your attention

More information:

http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm

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