EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Communication on regulatory tools for early access

Presented by Sonia Ribeiro on 20 October 2015 at 3rd STAMP meeting
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EU legislation provisions to promote early access

Recital 33 of Regulation (EC) No 726/2004

In order to meet, in particular, the legitimate expectations of patients and to take account of the increasingly rapid progress of science and therapies, accelerated assessment procedures should be set up, reserved for medicinal products of major therapeutic interest, and procedures for obtaining temporary authorisations subject to certain annually reviewable conditions. In the field of medicinal products for human use, a common approach should also be followed, whenever possible, regarding the criteria and conditions for the compassionate use of new medicinal products under Member States' legislation.

*Reinforcing **understanding** of existing tools.*

Early access tools

Legislative tools

- Conditional marketing authorisation
- Accelerated assessment
- CHMP opinion on compassionate use

Development support tools

to optimise use of existing legislative tools

- Adaptive Pathways
- PRIME

Early access tools: Overview

PRIME

Major public health interest, unmet medical need.

Dedicated and reinforced scientific and regulatory support.

Enable accelerated assessment.

Better use of existing tools.

Adaptive Pathways

Scientific concept of development and data generation.

Iterative development with use of real-life data.

Multi-stakeholder engagement.

Accelerated Assessment

Major public health interest, unmet medical need.

Reduce assessment time to 150 days.

Conditional MA

Unmet medical need, seriously debilitating or life-threatening disease, a rare disease or use in emergency situations.

Early approval of a medicine on the basis of less complete clinical data.

Compassionate Use

Unauthorised medicinal products in seriously debilitating or life threatening disease with no satisfactory treatment
CHMP opinion on conditions of use, distribution & target population

Early access tools: Ongoing activities

PRIME

Reflection paper on new scheme to be adopted

Relevant Q&A, scientific guidance and templates under development for launch

Accelerated Assessment

Ongoing revision of the guideline.

More detailed guidance on justification.

Optimisation of the assessment timetable.

Emphasis on the importance of early dialogue.

Possible combination with CMA acknowledged

Adaptive Pathways

Pilot ongoing.

ADAPT-SMART

Conditional MA

Ongoing revision of the guideline.

Emphasis on importance of prospective planning and early dialogue.

Compassionate Use

Review of CHMP experience with CU on-going
Discussion at future STAMP

High level overview document

- Summary table to help applicants identify

  When, how and for which medicines to use them,

  Benefits of each tool

  Possible combination of tools

- Links for easy access to relevant information
- To be published at time of PRIME launch

Future development of wider interface for easy access to other development guidances (eg scientific advice, ITF, orphan)

Thank you for your attention

Further information

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