Communication on regulatory tools for early access

Presented by Sonia Ribeiro on 20 October 2015 at 3rd STAMP meeting
Head of Regulatory Affairs Office
Human Medicines Research and Development Support Division
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.
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

Reinforcing **understanding** of existing tools.
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.

**Accelerated Assessment**
Major public health interest, unmet medical need.
Reduce assessment time to 150 days.

**Adaptive Pathways**
Scientific concept of development and data generation.
Iterative development with use of real-life data.
Multi-stakeholder engagement.

**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

**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
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

Jordi.Llinares@ema.europa.eu
Zahra.hanaizi@ema.europa.eu

European Medicines Agency
30 Churchill Place • Canary Wharf • London E14 5EU • United Kingdom

Telephone +44 (0)20 3660 6000 Facsimile +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Follow us on @EMA_News