EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PRI*ority* ***ME****dicines (****PRIME****)*

Supporting patient access to innovative medicines

3rd STAMP meeting, 20 October 2015

Presented by Zaide Frias
Head of Human Medicines Research & Development Support Division

EU Medicines Agencies Network Strategy to 2020 (Draft)

- **Ensure timely access to new beneficial and safe medicines for patients**
  - Ensuring that existing flexibilities to get appropriate medicines to patients more quickly are used to their maximum potential

- **Support for patient focused innovation and contribute to a vibrant life science sector in Europe**
  - Opportunities for greater collaboration and integration across the network and with academia will be explored to translate innovation into medicinal products
  - Consider further regulatory incentives for innovation, particularly in certain areas of public health need

Development support and early access tools

Reinforcing understanding of existing tools

Legislative tools

- Conditional MA
- Accelerated assessment
- CHMP opinion on compassionate use
- Scientific advice
- Orphan designation
- ATMP classification, certification
- SME office

Development support tools

to optimise use of existing legislative tools

- Adaptive Pathways
- PRIME

Objectives

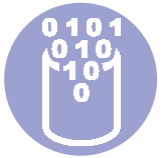
- Improve planning and optimise resource allocation
- Raise awareness of regulatory-scientific requirements earlier in development
- Promote generation of high quality data
- Improve sharing of knowledge gained during development

PRIME scheme: Goal & Scope

To foster the development of ***medicines with high public health potential.***

Reinforce scientific and regulatory advice

- Foster and facilitate early interaction
- Raise awareness of requirements earlier in development



Optimise development for robust data generation

- Focus efficient development
- Promote robust data generation

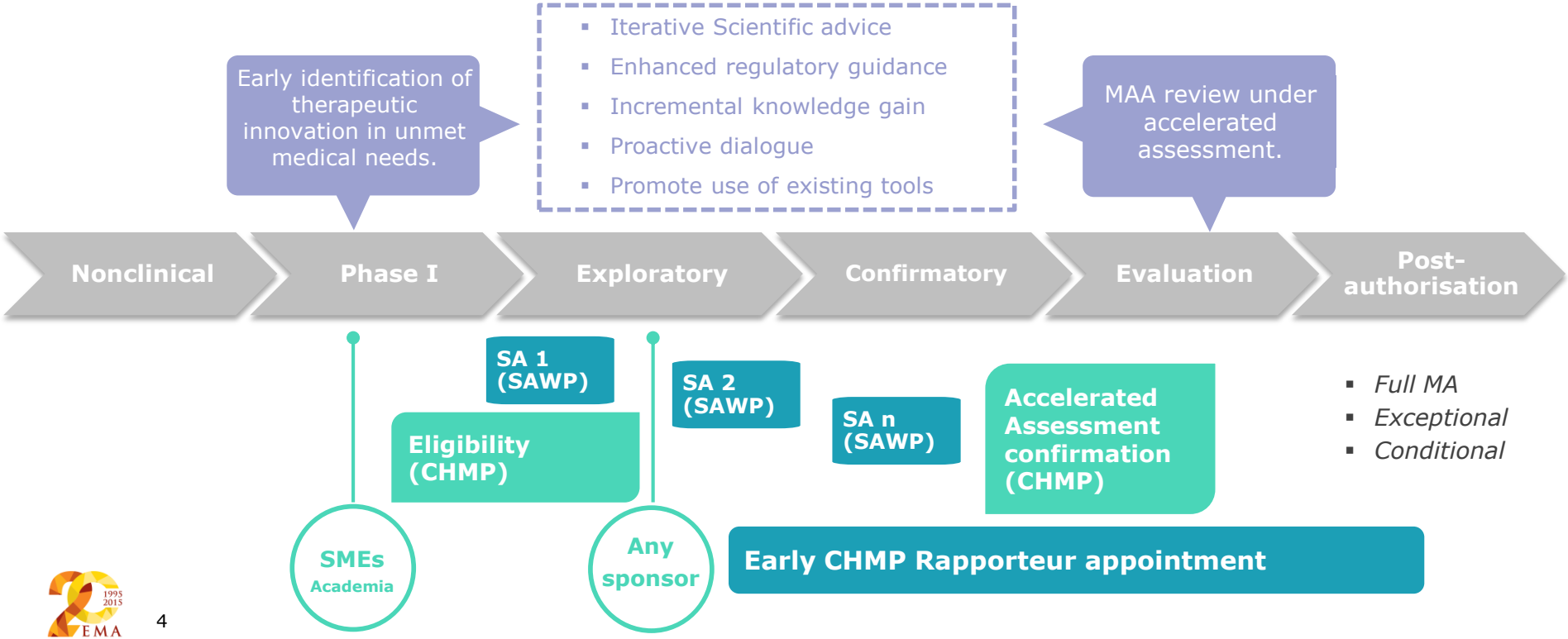
Enable accelerated assessment

- Promote generation of high quality data
- Facilitated by knowledge gained throughout development

Building on existing framework;

Eligibility according to existing Accelerated Assessment criteria.

Overview of PRIME scheme



Features of the PRIME scheme

Early access tool, supporting patient access to innovative medicines.

- Early confirmation of potential for accelerated assessment;
- Written confirmation of PRIME eligibility;
- Timely CHMP Rapporteur appointment;
- Scientific advice at key development milestones/decision points;
- Early, proactive, continuous and strengthened regulatory support;
- Promote awareness and better use of existing development and authorisation tools;
- EMA dedicated entry point;
- Complementarity and collaboration with National innovation schemes;
- Fee incentives for SMEs on Scientific Advice requests.

Eligibility to PRIME scheme

Based on Accelerated Assessment criteria

Medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation.

- Potential to address to a significant extent **an unmet medical need**
- Scientific justification, based on data and evidence available from nonclinical and clinical development

No satisfactory method or if method exists, bring a major therapeutic advantage

Introducing new methods or improving existing ones

Meaningful improvement of efficacy (impact on onset, duration, improving morbidity, mortality)

Eligibility to PRIME scheme

For products under development which are yet to be placed on the EU market.

- Entry to scheme at two different stages in development:
  - at **proof of concept** (prior to phase III/confirmatory clinical studies).
  - at the earlier stage of **proof of principle** (prior to phase II/exploratory clinical studies) focusing on SMEs and applicants from academic sector.
- Must be based on adequate data to justify a potential major public health interest.

Applicants not eligible to PRIME can still request accelerated assessment.
Guideline of the procedure for Accelerated Assessment (Article 14(9) of Reg (EC) No 726/2004).

Timing of PRIME eligibility requests: Expectations

SMEs
Academia

Confirmation

Any sponsor

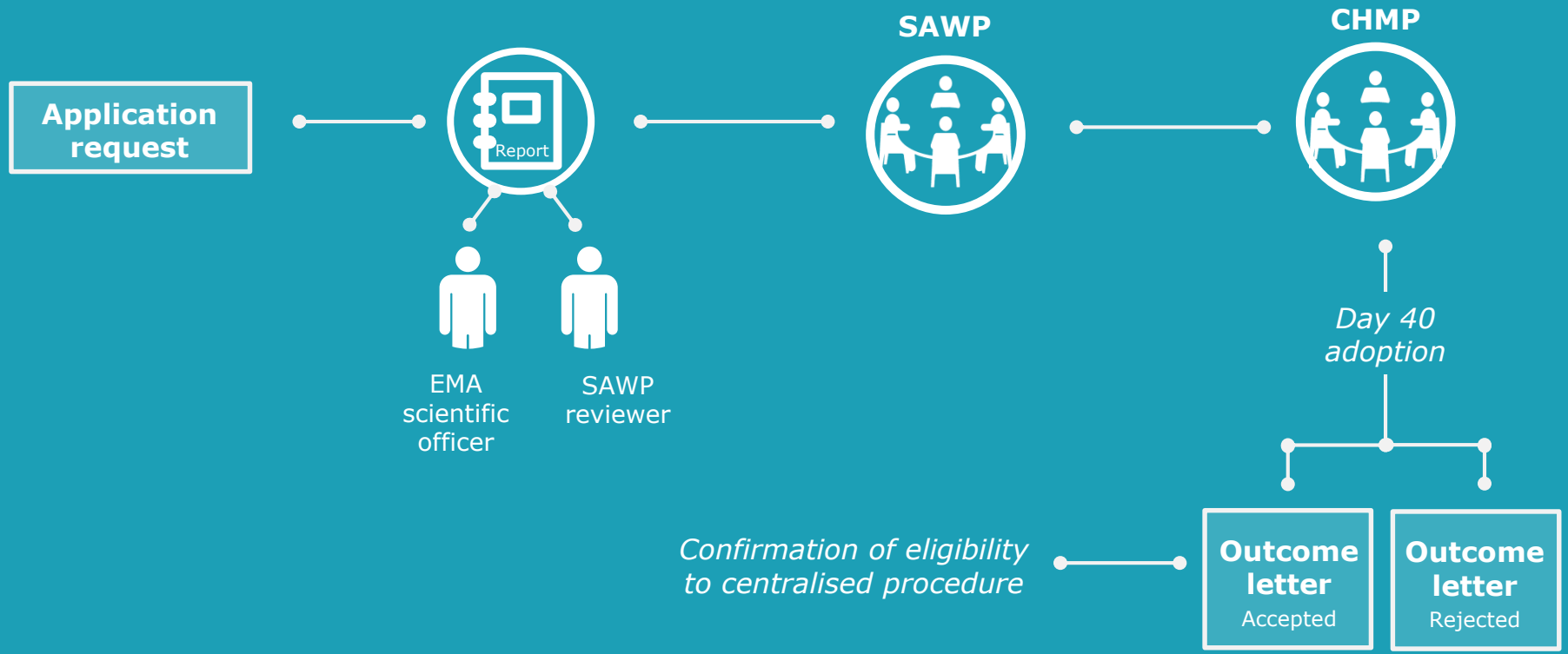
Proof of principle

- Exceptionally, for SMEs and academia
- Sound pharmacological rationale, convincing scientific concept
- Relevant nonclinical effects of sufficiently large magnitude and duration
- Tolerability in first in man trials

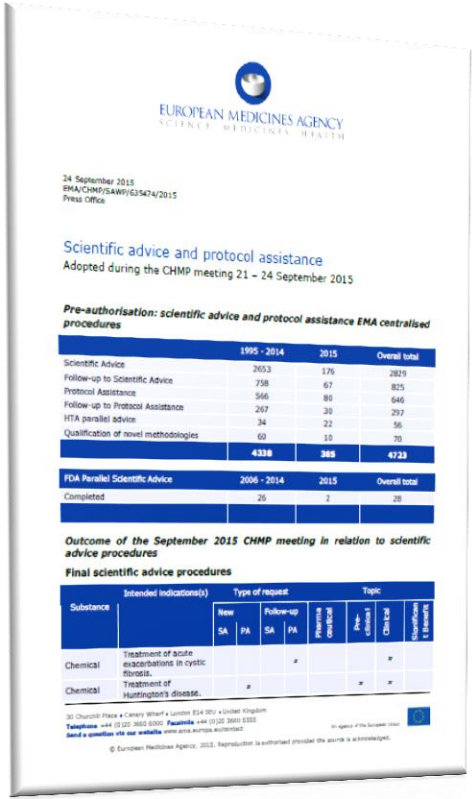
Proof of concept

- Sound pharmacological rationale
- Clinical response efficacy and safety data in patients (exploratory trials)
- Substantial improvement
- Magnitude, duration, relevance of outcomes to be judged on a case by case basis

Assessment of Eligibility: SAWP/CHMP 40-day procedure



PRIME: Transparency of outcomes



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24 September 2015
EMA/CHMP/SAWP/635474/2015
Press Office

Scientific advice and protocol assistance
Adopted during the CHMP meeting 21 – 24 September 2015

Pre-authorisation: scientific advice and protocol assistance EMA centralised procedures

	1995 - 2014	2015	Overall total
Scientific Advice	2653	176	2829
Follow-up to Scientific Advice	758	67	825
Protocol Assistance	566	80	646
Follow-up to Protocol Assistance	267	30	297
HTA parallel advice	34	22	56
Qualification of novel methodologies	60	10	70
	4338	**385**	**4723**

FDA Parallel Scientific Advice	2006 - 2014	2015	Overall total
Completed	26	2	28

Outcome of the September 2015 CHMP meeting in relation to scientific advice procedures

Final scientific advice procedures

Substance	Intended indications(s)	Type of request: New SA	New PA	Follow-up SA	Follow-up PA	Topic: Pharma quality	Pre-clinical	Clinical	Pharmacovigilance & Risk Mgmt
Chemical	Treatment of acute exacerbations in cystic fibrosis.			*				*	
Chemical	Treatment of Huntington's disease.		*				*	*	

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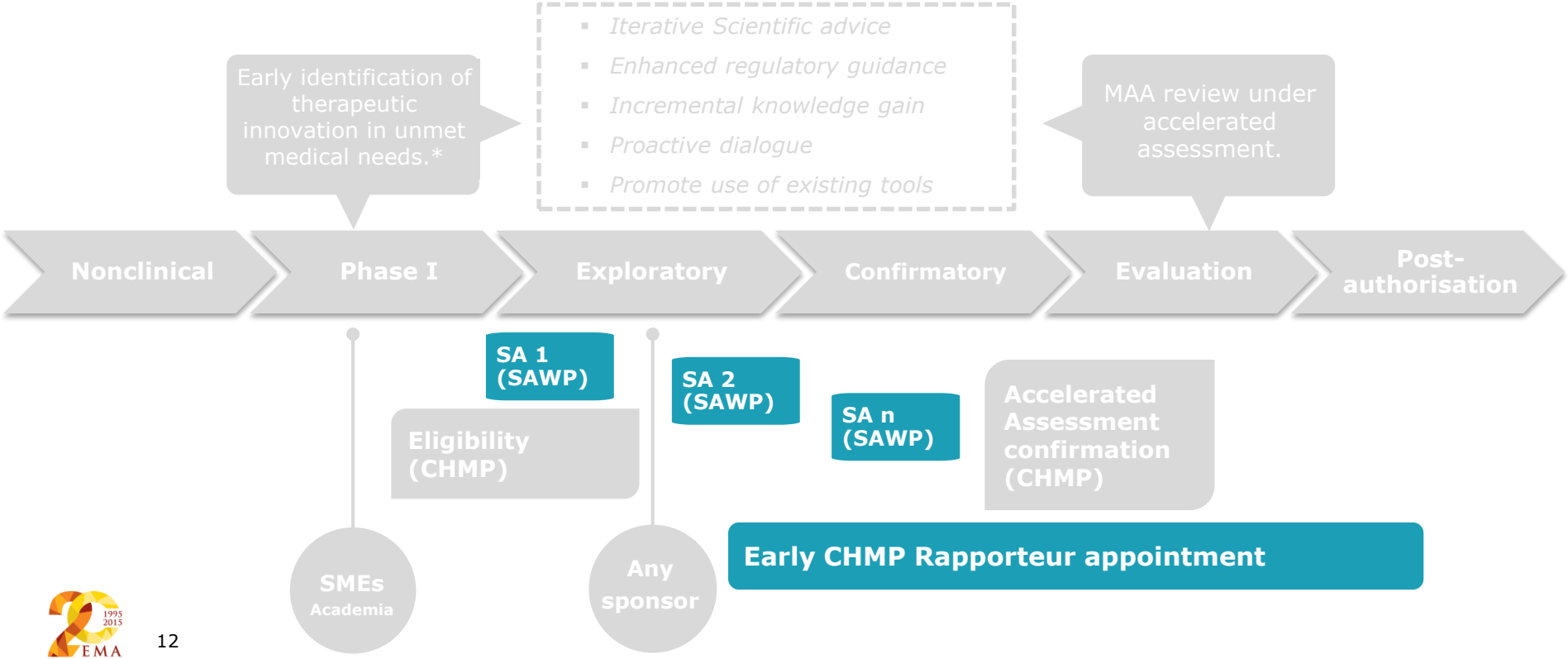
- Not on a per product basis
- CHMP monthly report, broad characteristics
- High-level statistics regularly updated on EMA public website
- Sharing information with relevant partners and stakeholders
- Guidance on eligibility criteria will be refined as experience is gained

Monitoring of development

- Monitoring within iterative Scientific Advice
- Regular updates from applicants expected
- Possibility to withdraw products from scheme if criteria are no longer met
- Accelerated assessment to be re-confirmed prior to MAA submission

PRIME: Support through the development stages



Support to be channelled through Scientific Advice by SAWP/CHMP

- Discuss detailed development plan, design of pivotal studies
- Discuss key issues for MAA, at major milestones
- Expectation of iterative advice, higher frequency of interactions
- Continuity will facilitate sharing of knowledge from development to life-cycle
- Fee waivers for SMEs

Role of SAWP coordinator

- One SAWP coordinators to follow all iterative Scientific Advices
- Provide guidance on overall development plan
- Facilitate preparation of SAWP/CHMP advice
- Recommend next milestones for SA requests
- Monitor development based on information provided in SA requests

Timely CHMP Rapporteur appointment

Key feature enabling continuity with life-cycle approach

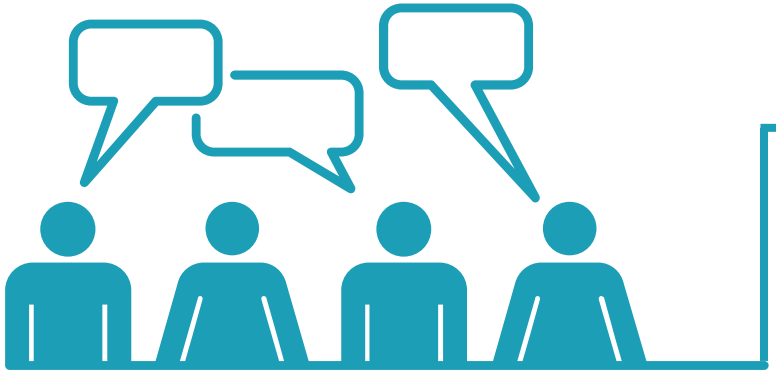
- Provide dialogue and assistance on regulatory pathway/MAA requirements
- Promote use of tools/initiatives (e.g. parallel EMA/HTA advice, adaptive pathways)
- Highlight the need to involve specific expertise from other committees
- Knowledge gained throughout the development will facilitate accelerated assessment of MAA

Timely Rapporteur appointment

Objectivity/independence of assessment: Mitigation of risk perception

- Advice on major issues/development milestones though scientific advice:
  - ✓ 2 SAWP coordinators for each advice
  - ✓ Peer review of advice letters, adopted by CHMP
- CHMP Co-Rapporteur, CHMP peer-reviewer and PRAC Rapporteur not involved during development
- Two assessment reports, peer review system, critical discussion at Committee level
- Adoption of the Opinion by the Committee

EU system has rigorous procedures and safeguards in place to ensure the independence of the assessment

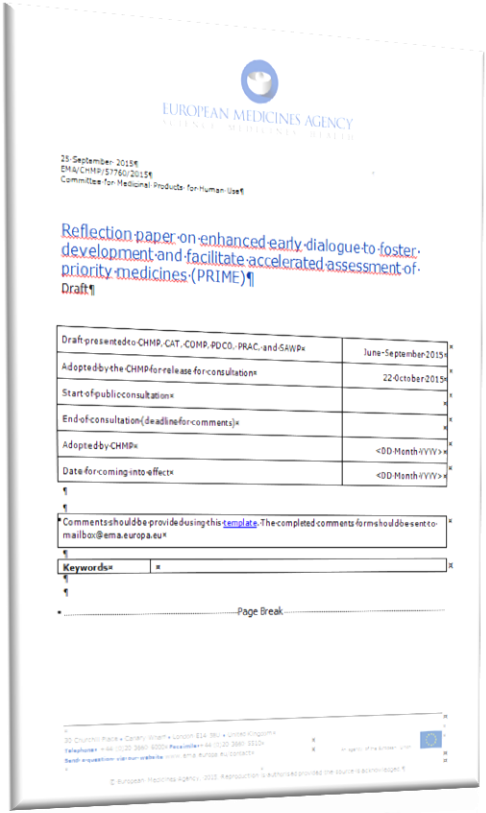


Consultation on proposal

- ✓ CHMP, CAT, COMP, PDCO, PRAC & SAWP
- ✓ HMA, EMA Management Board, EU Innovation offices network
- ✓ PCWP, HCPWP
- ✓ Industry Stakeholders

- *Overall support from committees and WP, with recognition of the need to support development of promising products in areas of unmet need*
- *Need clear eligibility criteria*
- *Impact of early Rapporteur appointment on independence of assessment*
- *Patient access and HTA involvement*

Next steps



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25 September 2015
EMA/CHMP/57760/2015
Committee for Medicinal Products for Human Use

Reflection paper on enhanced early dialogue to foster development and facilitate accelerated assessment of priority medicines (PRIME)
Draft

Draft presented to CHMP, CAT, COMP, PDCO, PRAC, and SAWP	June-September 2015
Adopted by the CHMP for release for consultation	22 October 2015
Start of public consultation	
End of consultation (deadline for comments)	
Adopted by CHMP	<DD Month YYYY>
Date for coming into effect	<DD Month YYYY>

Comments should be provided using this template. The completed comments form should be sent to mailbox@ema.europa.eu

Keywords	

Page Break

Today: Discussion at EC STAMP

26 Oct: Start of 2-month public consultation after CHMP adoption of reflection paper

Target launch in March 2016

Thank you for your attention

Further information

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