EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

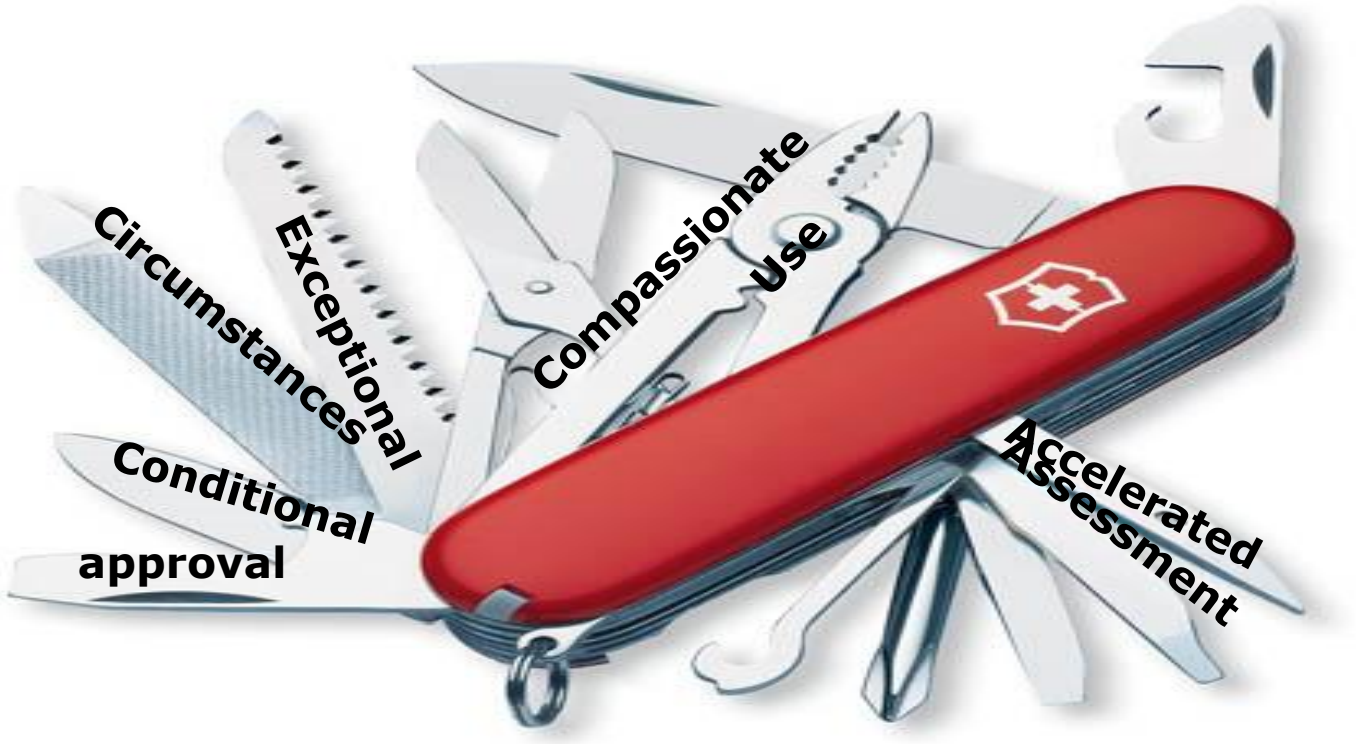
Accelerated (& Early) approvals – current situation in the EU

New Developments in Drug Regulations
Pretoria, September, 2015

Presented by: Tomas Salmonson

Review 2001

" Early Access " Tools



Revision of guidelines on Early Access tools

News

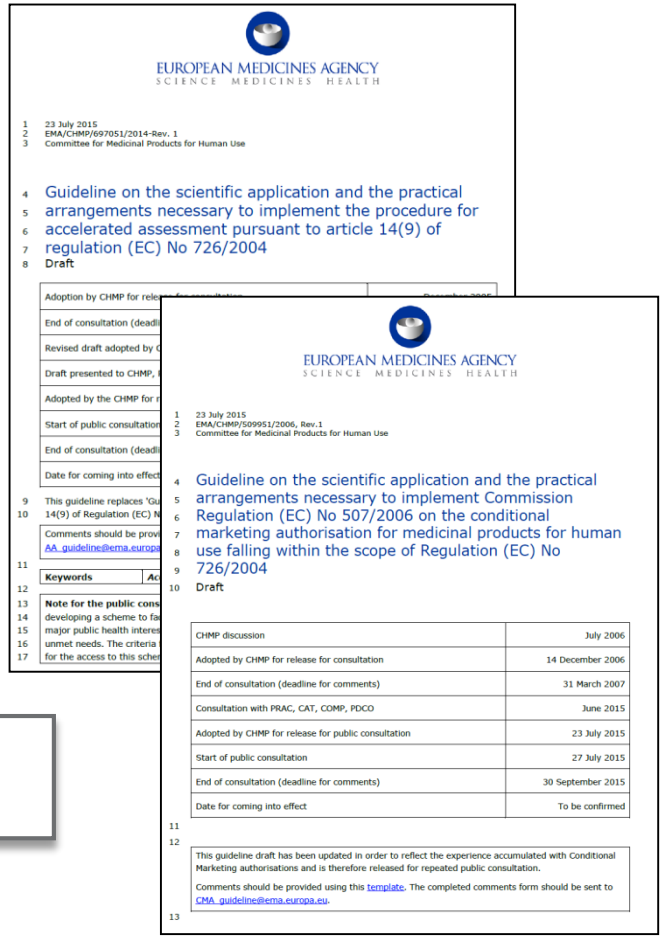
27/07/2015

Fast track routes for medicines that address unmet medical needs

Launch of two-month public consultations on revised guidelines on accelerated assessment and conditional marketing authorisation

The European Medicines Agency (EMA) has revised its guidelines on the implementation of accelerated assessment and conditional marketing authorisation, two key tools in the European legislation to accelerate patients' access to medicines that address unmet medical needs.

EMA/CHMP/697051/2014 Rev. 1
EMA/CHMP/509951/2006 Rev. 1



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 July 2015
EMA/CHMP/697051/2014-Rev. 1
Committee for Medicinal Products for Human Use

Guideline on the scientific application and the practical arrangements necessary to implement the procedure for accelerated assessment pursuant to article 14(9) of regulation (EC) No 726/2004
Draft

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 July 2015
EMA/CHMP/509951/2006, Rev.1
Committee for Medicinal Products for Human Use

Guideline on the scientific application and the practical arrangements necessary to implement Commission Regulation (EC) No 507/2006 on the conditional marketing authorisation for medicinal products for human use falling within the scope of Regulation (EC) No 726/2004
Draft

CHMP discussion	July 2006
Adopted by CHMP for release for consultation	14 December 2006
End of consultation (deadline for comments)	31 March 2007
Consultation with PRAC, CAT, COMP, PDCO	June 2015
Adopted by CHMP for release for public consultation	23 July 2015
Start of public consultation	27 July 2015
End of consultation (deadline for comments)	30 September 2015
Date for coming into effect	To be confirmed

This guideline draft has been updated in order to reflect the experience accumulated with Conditional Marketing authorisations and is therefore released for repeated public consultation.

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

Draft revisions of these guidelines have been published for public consultation until 30 September 2015

Revision of guidelines on Early Access tools

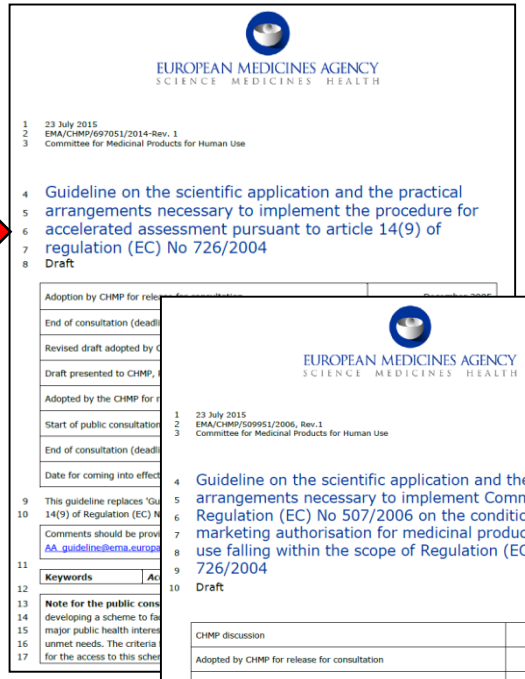
News

27/07/2015

Fast track routes for medicines that address needs

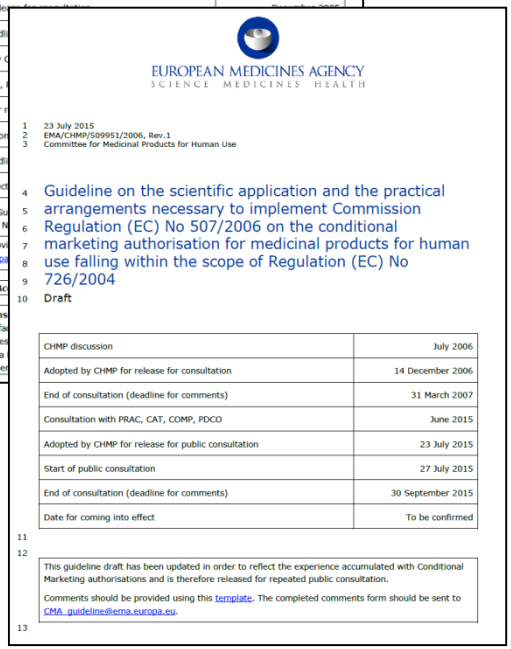
Launch of two-month public consultations on revised guidelines on accelerated assessment and conditional marketing authorisation

The European Medicines Agency (EMA) has revised its guidelines on the implementation of accelerated assessment and conditional marketing authorisation, two key tools in the European legislation to accelerate patients' access to medicines that address unmet medical needs.


EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 July 2015
EMA/CHMP/697051/2014-Rev. 1
Committee for Medicinal Products for Human Use

Guideline on the scientific application and the practical arrangements necessary to implement the procedure for accelerated assessment pursuant to article 14(9) of regulation (EC) No 726/2004
Draft


EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 July 2015
EMA/CHMP/509951/2006, Rev.1
Committee for Medicinal Products for Human Use

Guideline on the scientific application and the practical arrangements necessary to implement Commission Regulation (EC) No 507/2006 on the conditional marketing authorisation for medicinal products for human use falling within the scope of Regulation (EC) No 726/2004
Draft

CHMP discussion	July 2006
Adopted by CHMP for release for consultation	14 December 2006
End of consultation (deadline for comments)	31 March 2007
Consultation with PRAC, CAT, COMP, PDCO	June 2015
Adopted by CHMP for release for public consultation	23 July 2015
Start of public consultation	27 July 2015
End of consultation (deadline for comments)	30 September 2015
Date for coming into effect	To be confirmed

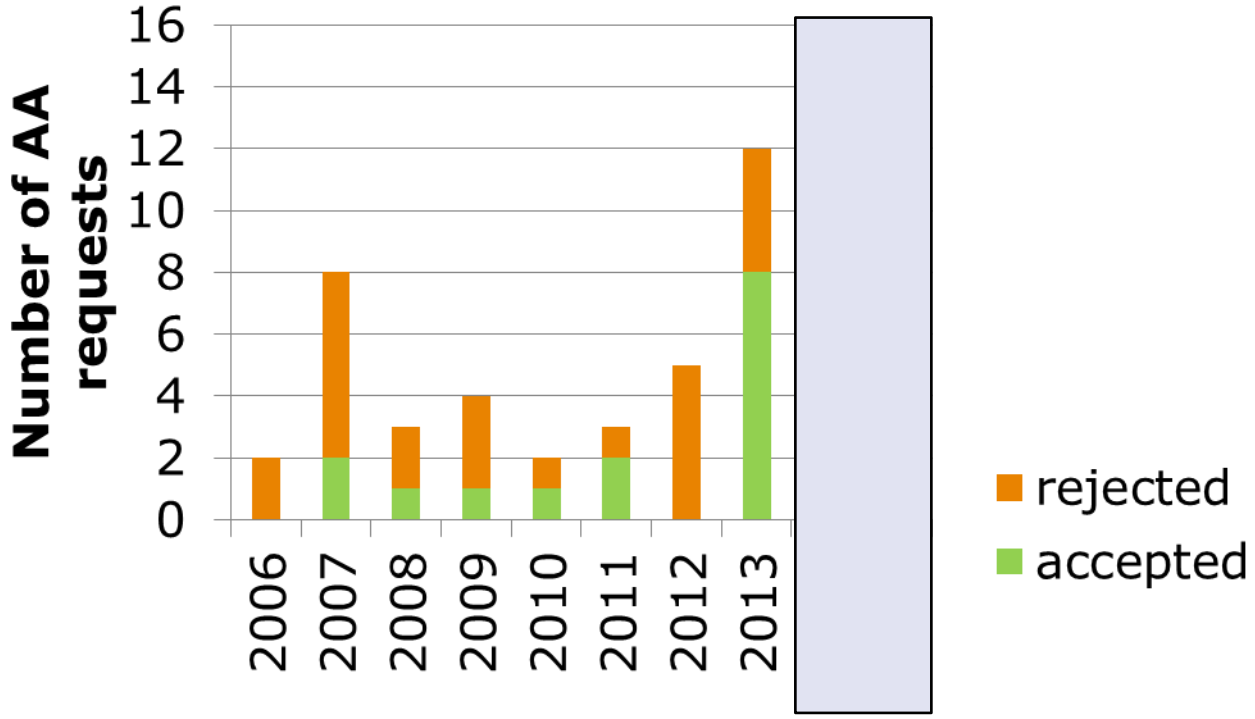
This guideline draft has been updated in order to reflect the experience accumulated with Conditional Marketing authorisations and is therefore released for repeated public consultation.

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

EMA/CHMP/697051/2014 Rev. 1
EMA/CHMP/509951/2006 Rev. 1

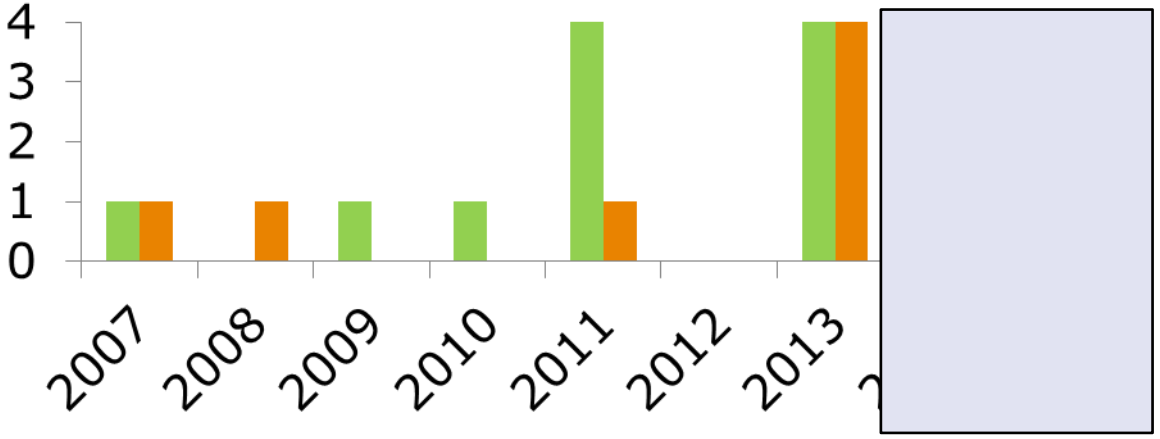
Draft revisions of these guidelines have been published for public consultation until 30 September 2015

Number of requests for Accelerate Assessment an acceptance rates



Experience with evaluations under accelerated assessment

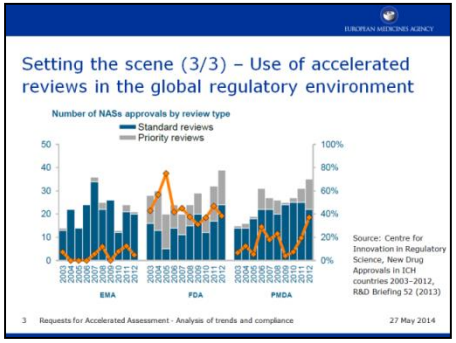
MAA evaluations started under an AA timetable with outcome between 2007 and 2013



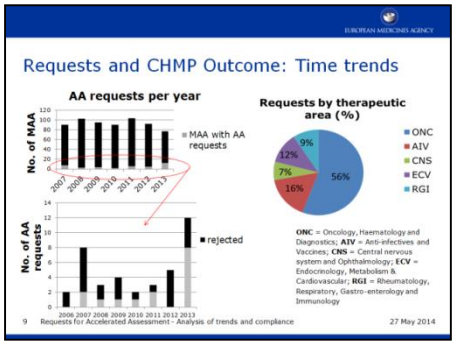
Main reasons for reverting to standard TT were 1/ Major objections identified at Day 120 that cannot be quickly resolved; 2/ Need for inspection.

CHMP discussion at the Presidency meeting May 2014

We faced the **problem statement**:



Limited uptake of AA reviews

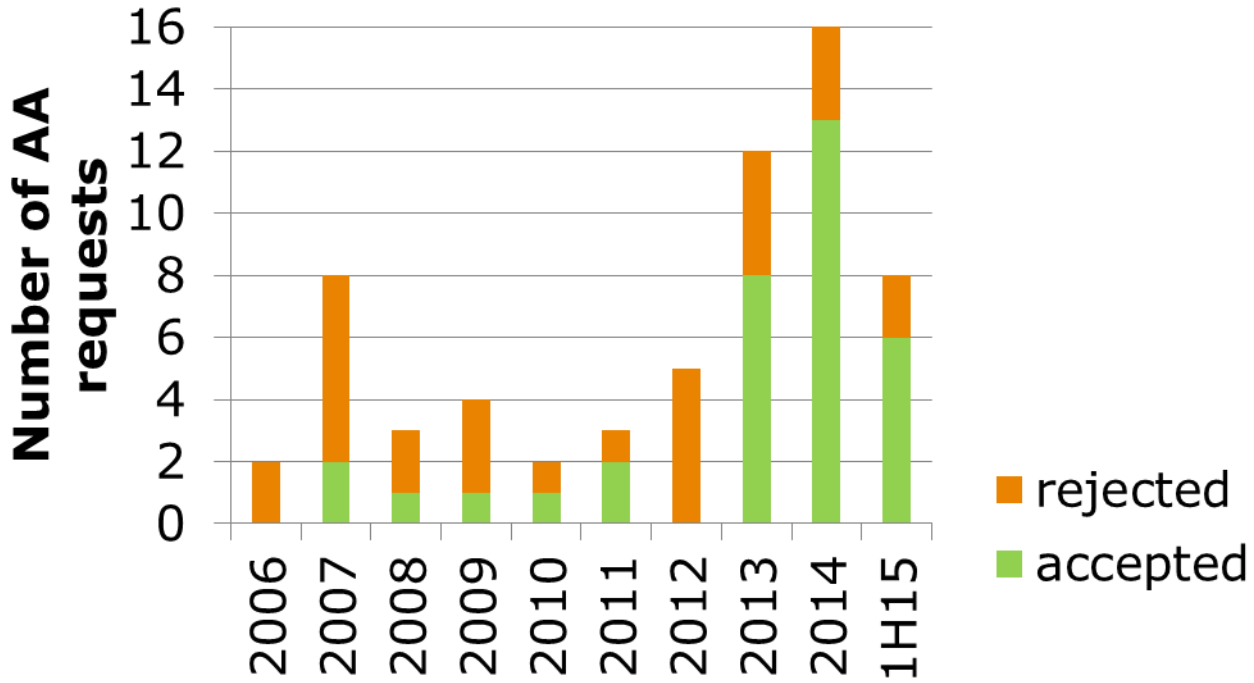


Few AA requests / low acceptance rate

We **concluded** from the analysis:

- Reasonable compliance with template criteria
- Driver of the decision: Unclear ?
- Need to optimise learning from previous assessments

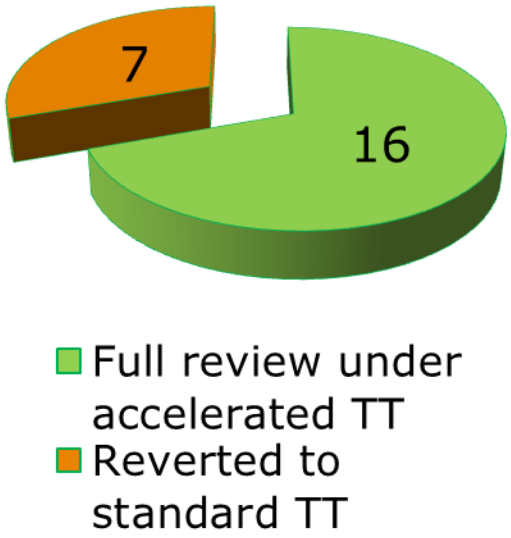
Experience with Accelerated Assessment: Requests and acceptance rates

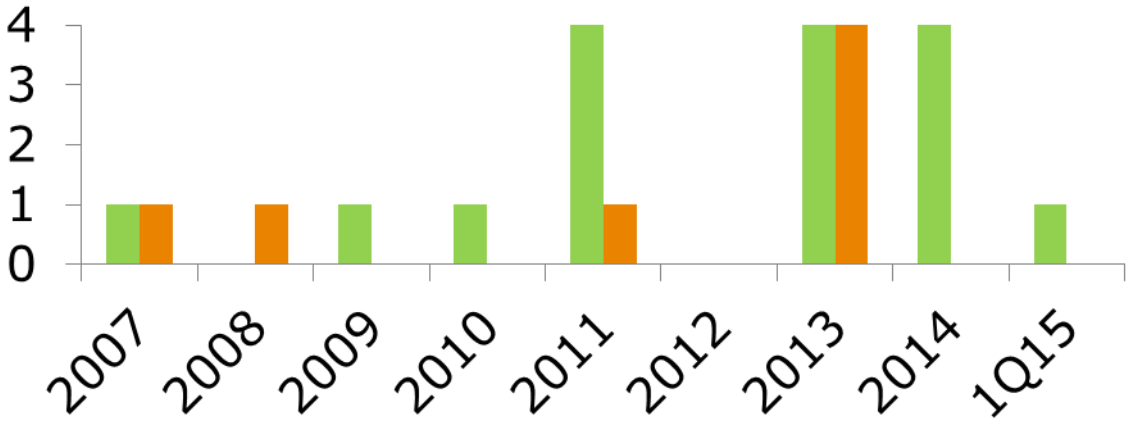


An increase in requests for accelerated assessment was observed over the last years along with a increase of acceptance rate of such requests.

Experience with evaluations under accelerated assessment

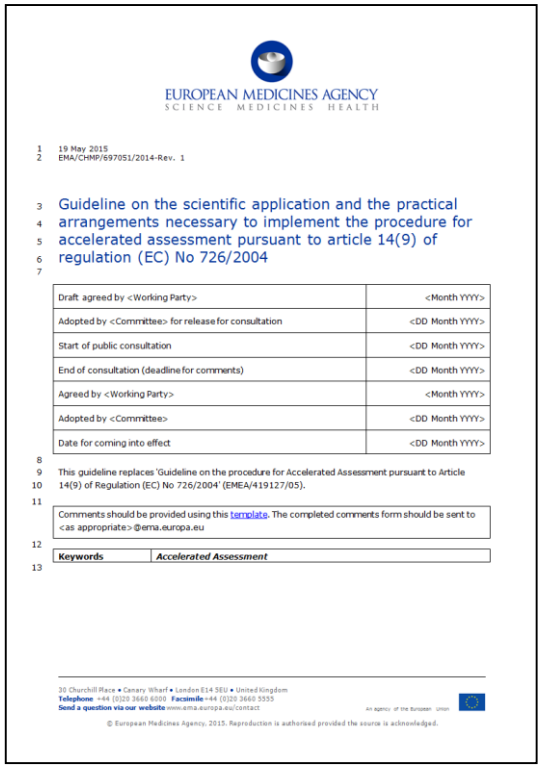
MAA evaluations started under an AA timetable with outcome between 2007 and 1Q15:





Main reasons for reverting to standard TT were 1/ Major objections identified at Day 120 that cannot be quickly resolved; 2/ Need for inspection.

Translation of changes in the guideline into the templates for applicants and rapporteurs



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 May 2015
EMA/CHMP/697051/2014-Rev. 1

Guideline on the scientific application and the practical arrangements necessary to implement the procedure for accelerated assessment pursuant to article 14(9) of regulation (EC) No 726/2004

Draft agreed by <Working Party>	<Month YYYY>
Adopted by <Committee> for release for consultation	<DD Month YYYY>
Start of public consultation	<DD Month YYYY>
End of consultation (deadline for comments)	<DD Month YYYY>
Agreed by <Working Party>	<Month YYYY>
Adopted by <Committee>	<DD Month YYYY>
Date for coming into effect	<DD Month YYYY>

This guideline replaces 'Guideline on the procedure for Accelerated Assessment pursuant to Article 14(9) of Regulation (EC) No 726/2004' (EMEA/419127/05).

Comments should be provided using this template. The completed comments form should be sent to <as appropriate>@ema.europa.eu

Keywords	*Accelerated Assessment*

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

An agency of the European Union

Template for applicant's request

- Follows the structure to provide justifications.
- The strength of evidence was expanded with the guidance.

Briefing note for Rapporteurs

- More detailed guidance on important aspects to be addressed in the assessment .
- More detailed guidance on how to express the conclusions.

High-level summary of proposed revisions

Draft revision of the guideline on the scientific application and the practical arrangements necessary to implement the procedure for accelerated assessment pursuant to Article 14(9) of Regulation (EC) No 726/2004

Content

- More detailed guidance on how to justify major public health interest (unmet medical need, strength of evidence)
- Acknowledgment that comprehensive clinical data may not be available in certain situations, allowing accelerated assessment in the context of a conditional marketing authorisation for example

Process

- Intent to request accelerated assessment to be indicated 6-7 months in advance and submission of accelerated assessment request to take place 2-3 months ahead of marketing authorisation application
- Importance of early dialogue / pre-submission discussions.
- Optimisation of the assessment timetable by better balancing evaluation phases to reach a CHMP opinion within 150 days

Outline of the potential evaluation timelines

Acceleration of the timelines proposal: 90/30/30 days

- 1st evaluation phase: 120 → 90 days (as is extension of indication)
  - Include PRAC evaluation & Peer review
- Clock-stop: 1 month by default
- 2nd phase: 60 → 30 + 30 days:
  - Opportunity to reach an opinion at Day 120 (w/o OE); or
  - LoI with no clock-stop (PI, RMP finalisation) → opinion at Day 150
- Possibility to switch to standard timelines at
  - Day 90 (followed by 90+30 days TT) or
  - Day 120 (followed by 60+30 says TT as usual) TBC

Revision of guidelines on Early Access tools

News

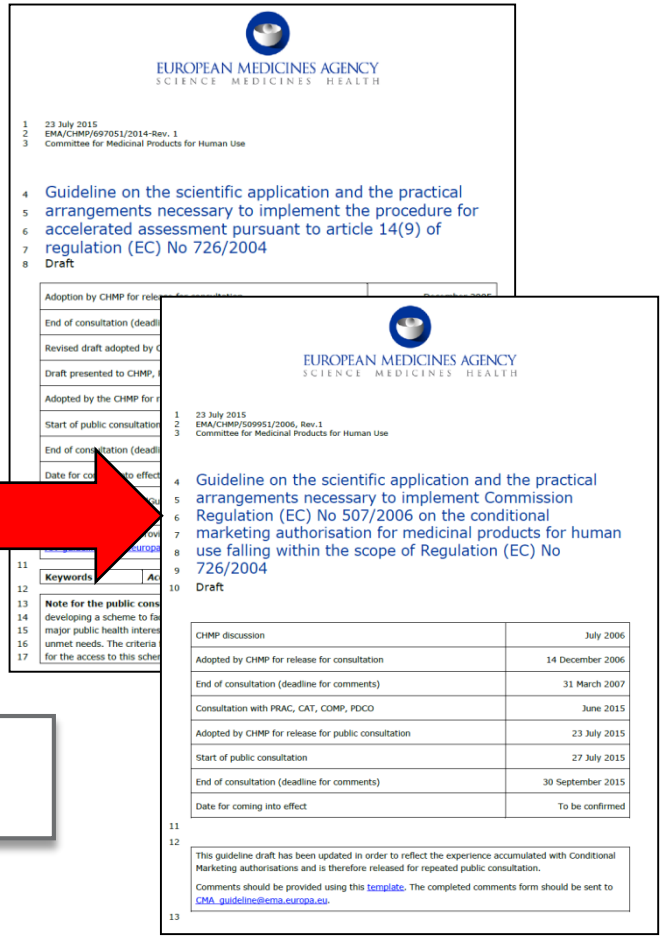
27/07/2015

Fast track routes for medicines that address unmet medical needs

Launch of two-month public consultations on revised guidelines on accelerated assessment and conditional marketing authorisation

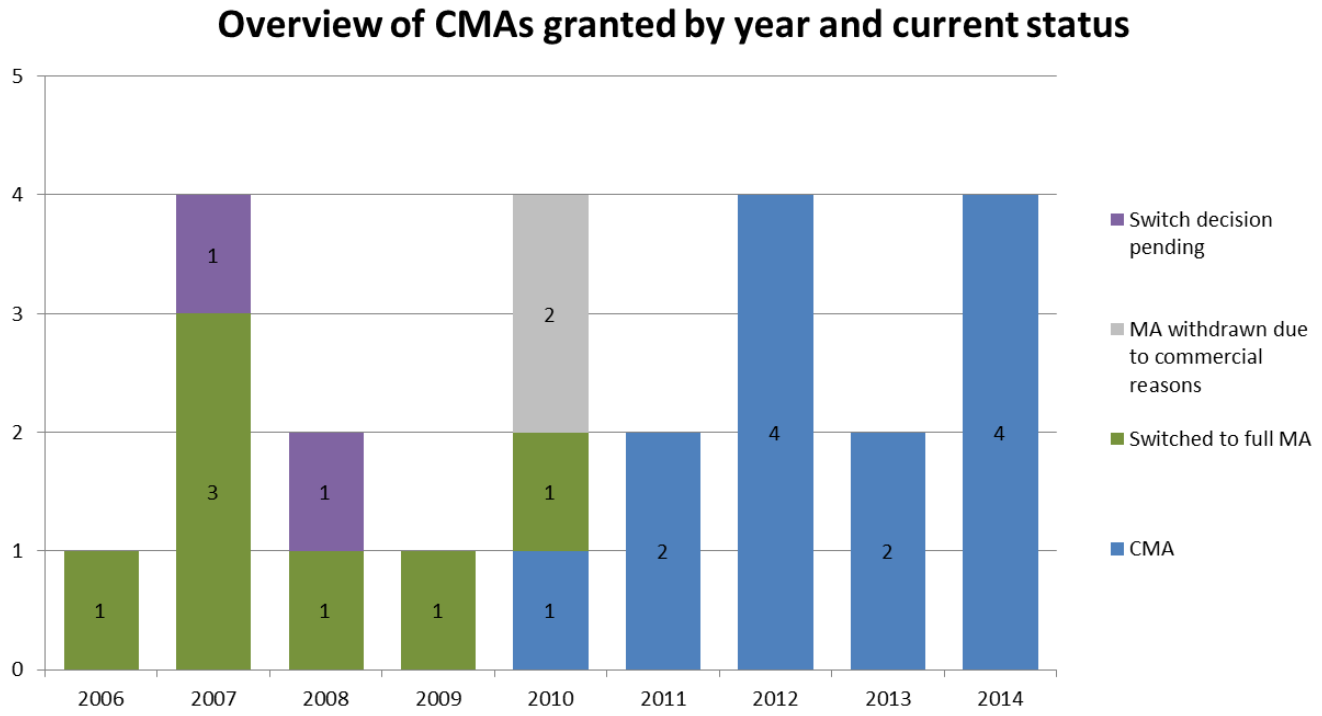
The European Medicines Agency (EMA) has revised its guidelines on the implementation of accelerated assessment and conditional marketing authorisation, two key tools in the European legislation to accelerate patients' access to medicines that address unmet medical needs.

EMA/CHMP/697051/2014 Rev. 1
EMA/CHMP/509951/2006 Rev. 1



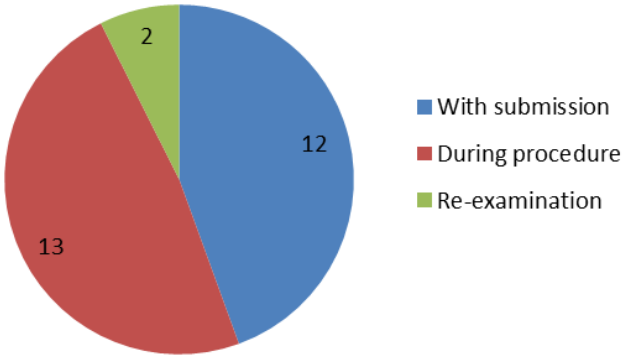
Draft revisions of these guidelines have been published for public consultation until 30 September 2015

Conditional Marketing Authorisation granted in the centralised procedure (until 12/2014)

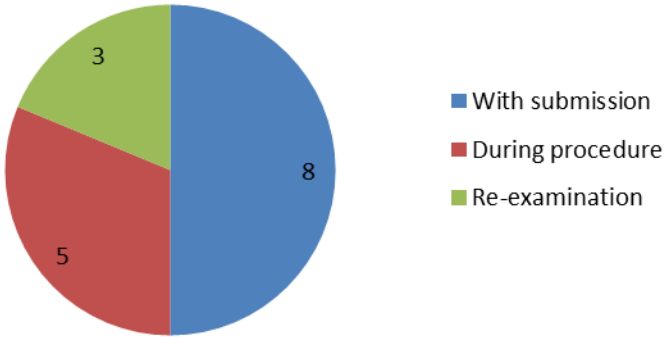


Positive and negative CHMP outcomes by time of consideration of CMA

Step of procedure when CMA is first considered (procedures with positive CHMP outcome, n=27*)



Step of procedure when CMA is first considered (procedures where possiblity of a CMA is discussed in a CHMP document but procedure outome is negative, n=15)**

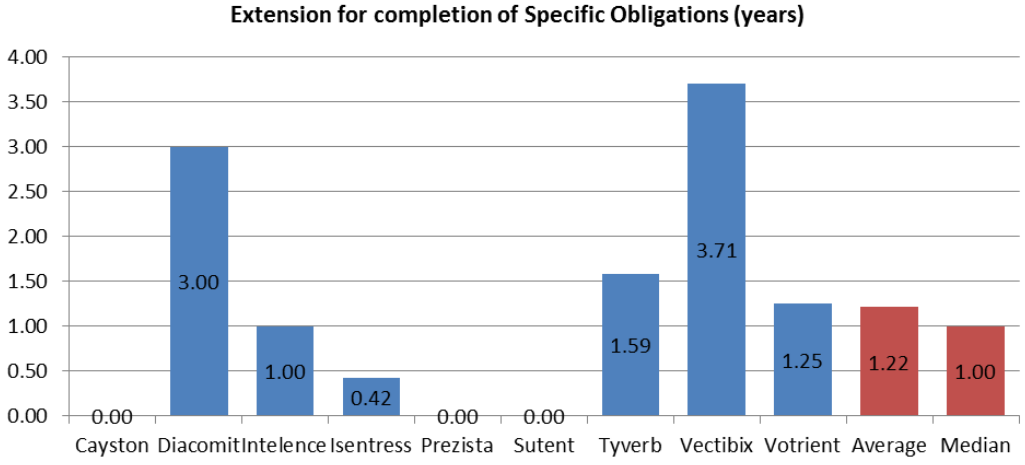


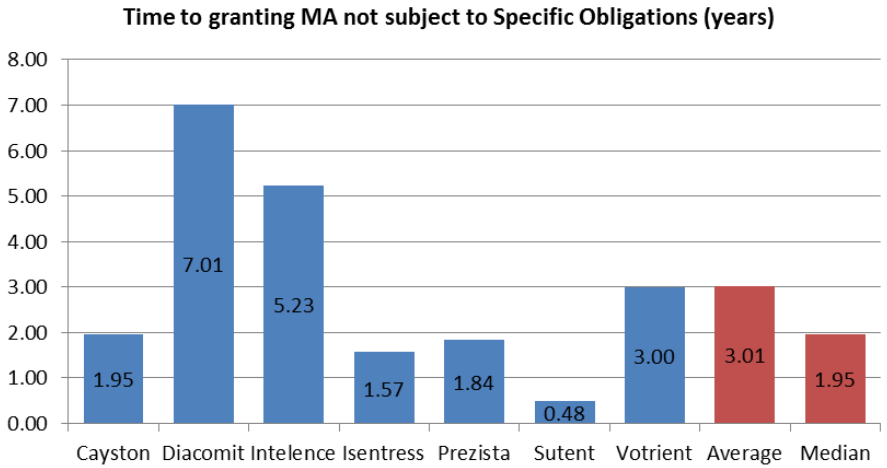
* Positive outcomes include 3 applications that were withdrawn after positive CHMP Opinion

** Negative outcome = Negative final CHMP Opinion or withdrawal of application after D120

Time to 'switch' from conditional to full marketing authorisation

- Approximately half of the products had changes to the scope and/or deadline of at least one of the specific obligations
- For 9 products with SOs completed, on average the due date for completion of last SO was extended by 1.22 years
- For 7 products that have MA not subject to SOs, it was granted on average in 3 years





Note: data lock December 2014

High-level summary of the draft guideline

Draft guideline on the scientific application and the practical arrangements necessary to implement Regulation (EC) No 507/2006 on the conditional marketing authorisation for medicinal products for human use falling within the scope of Regulation (EC) No 726/2004

Content

- Clarification how a positive benefit-risk balance should be substantiated where there are less complete data
- Examples and further guidance on the level of evidence that must be provided at the time of authorisation and data that can be provided post-authorisation
- Guidance on when a condition could be considered life threatening or seriously debilitating if these effects are in the long-term
- Clarification on fulfilment of unmet medical needs, i.e. medicines providing major improvements in patient care over existing therapies can be eligible in certain cases

Planning and submission requirements

- Emphasis on importance of planning conditional marketing authorisation prospectively to ensure swift assessment procedure
- Emphasis on advantages of engaging in early dialogue with EMA on the development programme, in particular in the context of parallel scientific advice with health technology assessment bodies
- Updated guidance on extent and type of data required to be included in annual renewal submissions

What will the future bring?

Thank you!

European Medicines Agency

30 Churchill Place

London E14 5EU