EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

# Optimising early access tools: Revision of the guidelines on Accelerated Assessment and Conditional Marketing Authorisation

High-level overview of comments received during the public consultation

Presented by Tomas Salmonson, CHMP Chair
EMA Industry Stakeholder Platform, 9 November 2015

# Background

- Reflections on the experience with AA and CMA
- Drafting of guideline updates
- Consultation with other committees
- Public consultation

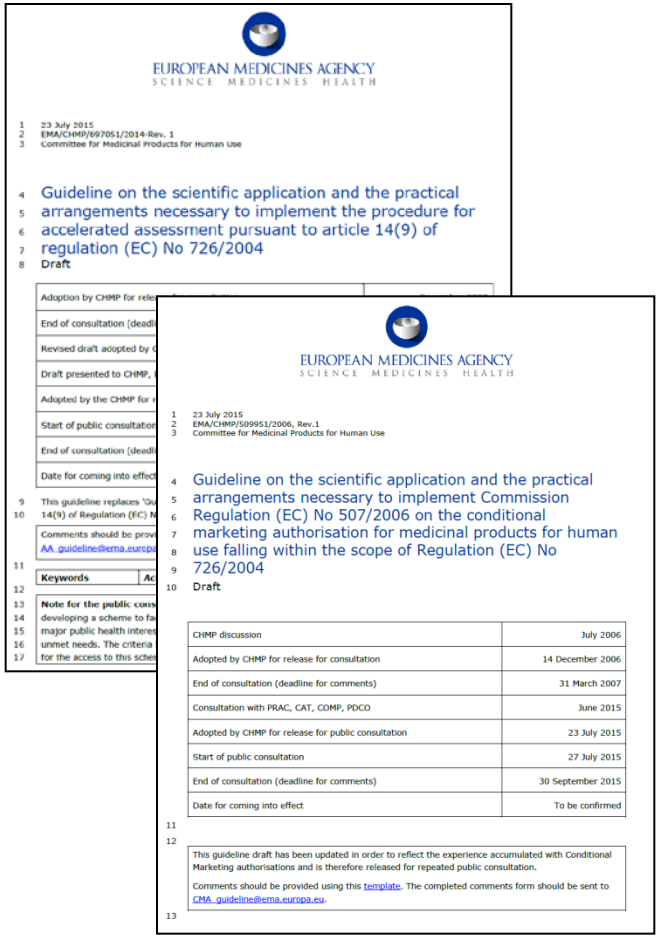
**News**

**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.



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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

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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.

# Reminder: Draft revision of the guideline on accelerated assessment (AA)

| High-level summary of changes | |
|---|---|
| Content<br>• More detailed guidance on how to justify major public health interest (unmet medical need, strength of evidence)<br>• 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<br>• 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<br>• Importance of early dialogue / pre-submission discussions.<br>• Optimisation of the assessment timetable by better balancing evaluation phases to reach a CHMP opinion within 150 days |

# Reminder: Draft revision of the guideline on conditional marketing authorisation (CMA)

**High-level summary of changes**

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

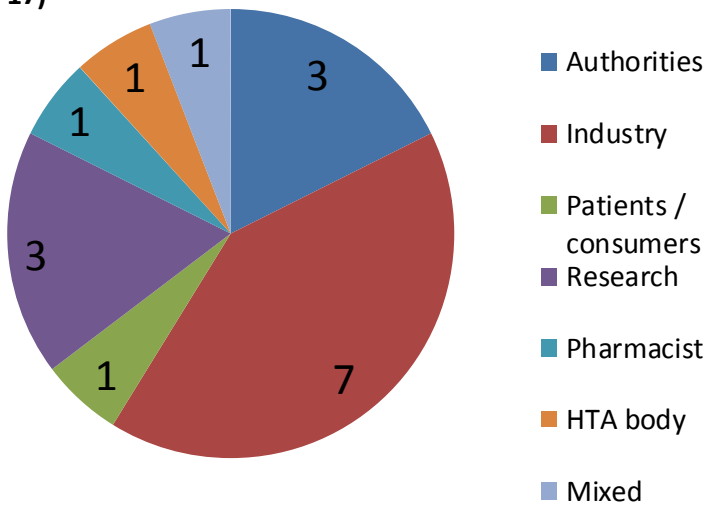
Planning and submission requirements

- 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

# Stakeholders contributing to the public consultation on AA and CMA

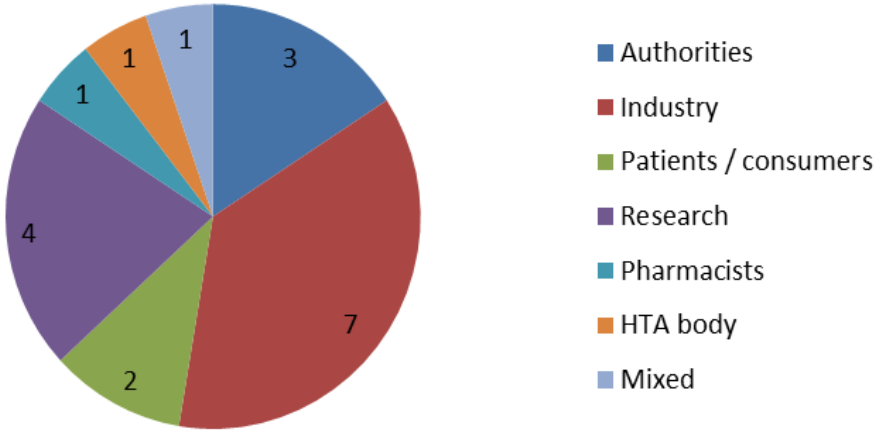
## Accelerated Assessment

**Affiliation of sources of comments (n=17)**



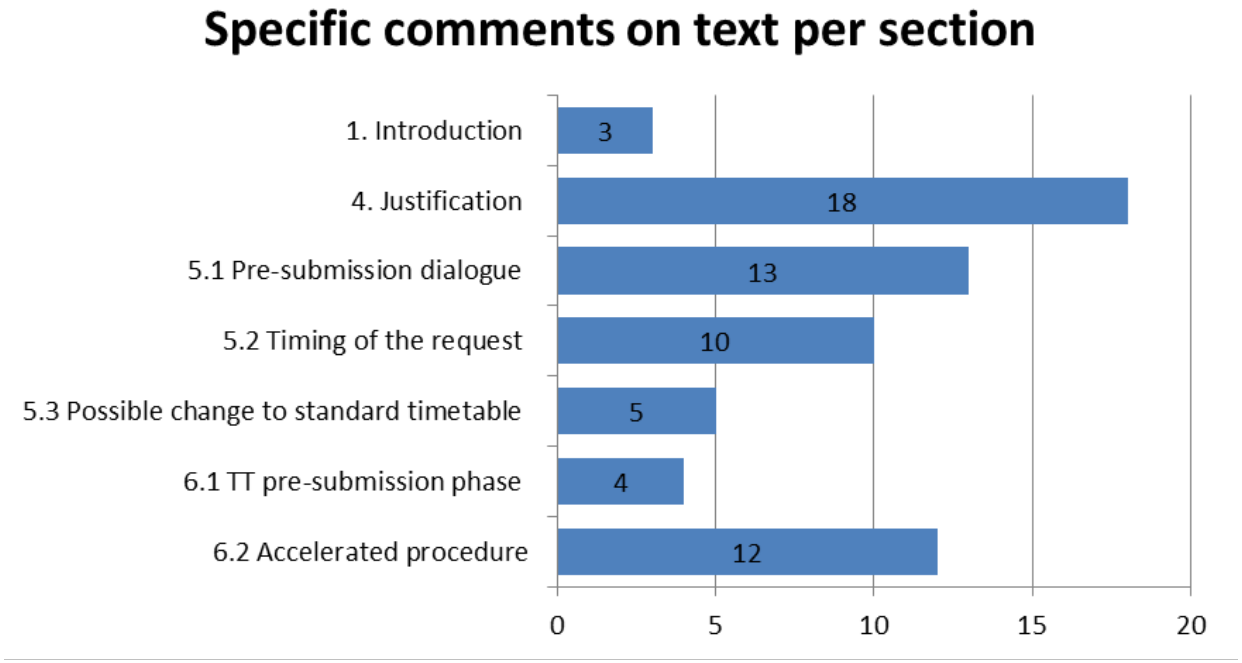

## Conditional Marketing Authorisation

**Affiliation of sources of comments (n=19)**



Comments on 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

# Comments on the text of the guideline



# Aspects raised in stakeholder comments (1/2)

Apply same standards as the regular assessment procedure

Patient safety should not be compromised

Impact of the assessment quality due to reduction of the timing

Detailed description of involvement of assessment teams, committees interactions and timetables

Products not addressing an unmet medical could address major public health interest

Include also proof that product support the use in debilitating or life threatening conditions

Orphan drugs to be automatically eligible for AA

Explanation and justification why the evidence cannot be generated at the time of submission

# Aspects raised in stakeholder comments (2/2)

Request for clarification meeting at any stage of the procedure

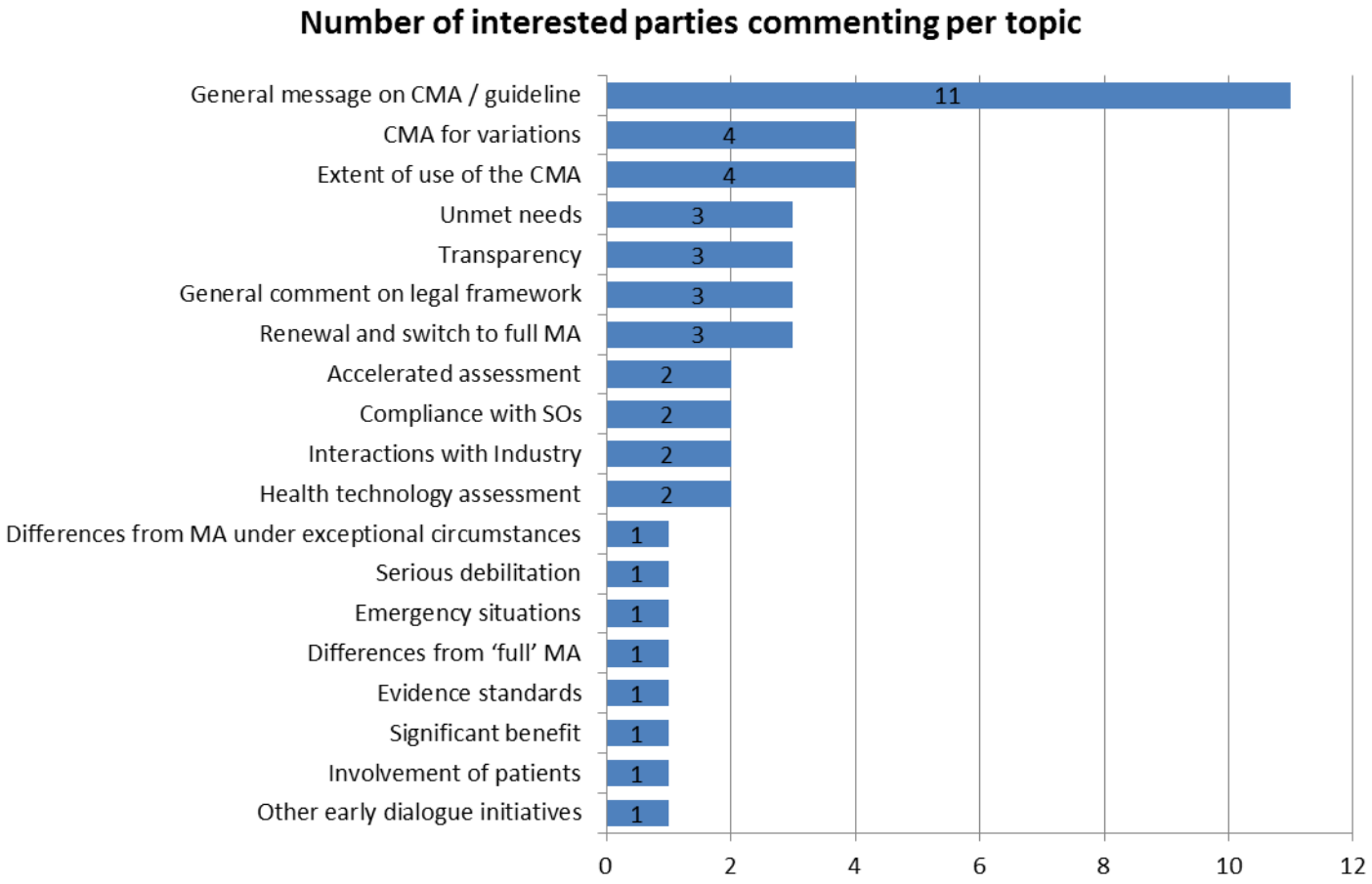
Reinforce mutual understanding of the data package planned for the submission

Useful to have the concept of rolling review

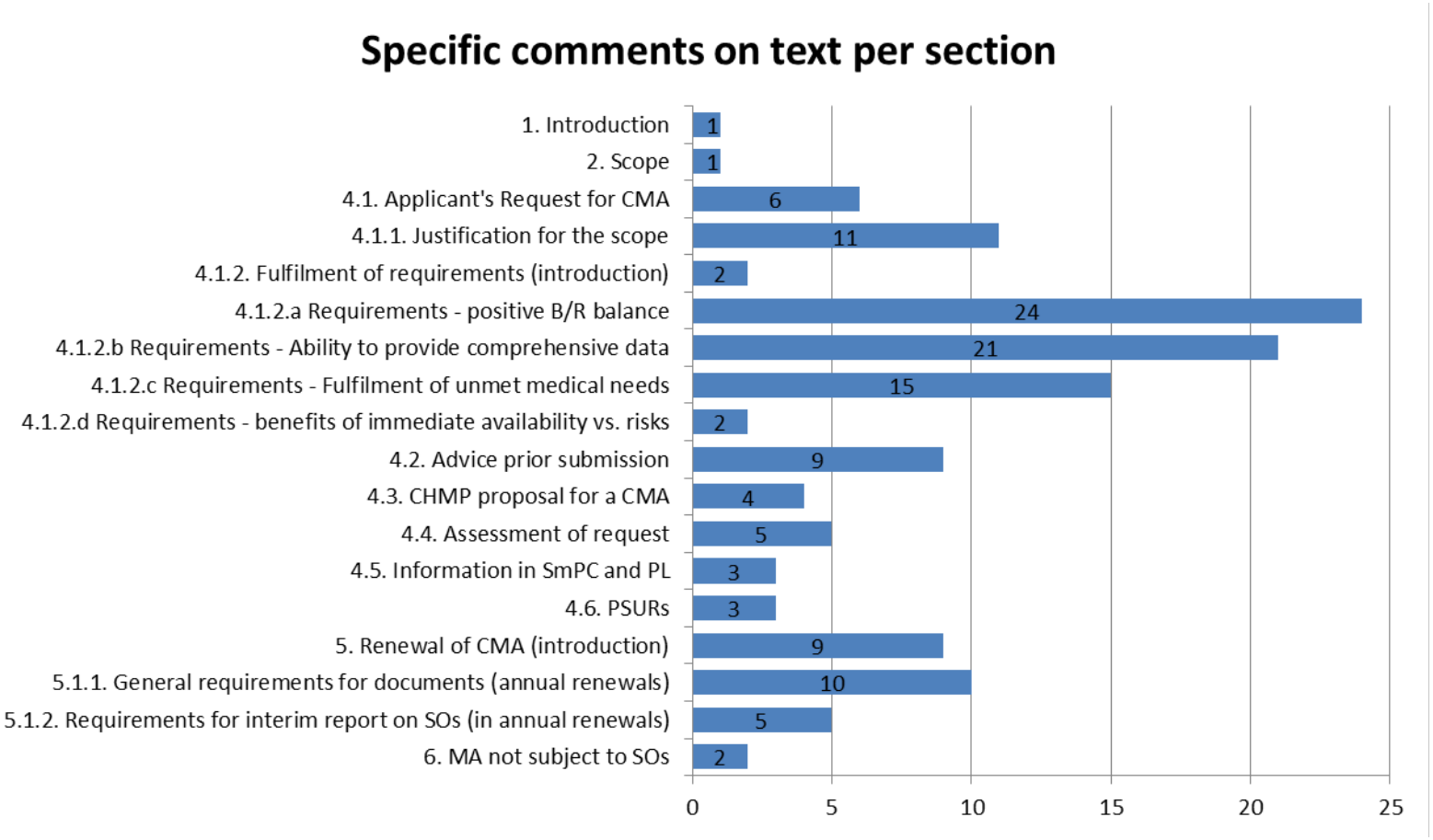
Clarification regarding the concept of mature application

Comments on the 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

# Topics of General comments



# Comments on the text of the guideline



# Aspects raised in stakeholder comments (1/2)

- Importance of early dialogue
- Maintenance of requirement for solid evidence
- Legitimate only in case of real unmet health need
- Cooperation between CHMP and COMP a positive process
- Engagement with those responsible for downstream medicines access policy
- Apply sanctions in case of non-compliance
- Provide more information to public
- CMA for new indications and line extensions

# Aspects raised in stakeholder comments (2/2)

Reduce the requirements for an annual renewal

Comparative-trials against the best available treatment

For all categories should be possible with less preclinical or pharmaceutical data

Support and appreciate the proposed changes

Emergency use requires extremely flexible approaches

Important that the CMA is seen in a positive manner

“unmet medical need” not met as long as the status of the first product is still conditional

Automatic accelerated assessment

# Next steps

Review of comments and revision of the guidelines

Favourable opinion of the European Commission*

Finalisation and adoption of the guidelines

* For the Guideline on conditional marketing authorisation only (as per Art. 11 of Regulation (EC) No 507/2006)

# Thank you for your attention

## Further information

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