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

- Exceptional Circumstances
- Compassionate Use
- Conditional Approval
- Accelerated Assessment
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.

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

EMA/CHMP/697051/2014 Rev. 1
EMA/CHMP/509951/2006 Rev. 1
Revision of guidelines on Early Access tools

**News**

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Draft

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

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

Keywords

Draft
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 concluded from the analysis:

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

We faced the problem statement:

Limited uptake of AA reviews

Few AA requests / low acceptance rate
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

**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

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

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)

Overview of CMAs granted by year and current status

- **Switch decision pending**
- **MA withdrawn due to commercial reasons**
- **Switched to full MA**
- **CMA**
Positive and negative CHMP outcomes by time of consideration of CMA

* 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