EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Early access tools: accelerated assessment and conditional marketing authorisation

EMA-EuropaBio Information Day, London, 15 October 2015

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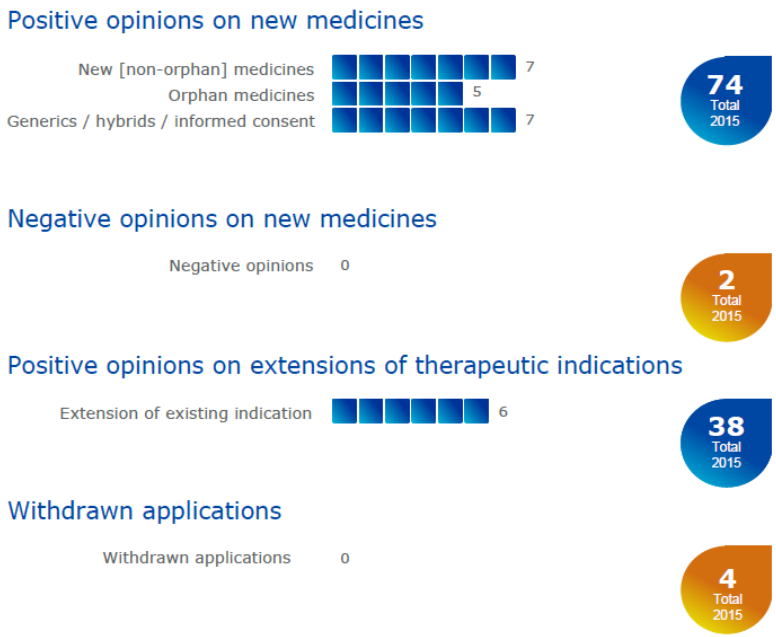
Medicines evaluation highlights so far in 2015



- PCSK9 inhibitors for treatment of hypercholesterolemia
- New treatment option for ADHD
- Treatments for orphan metabolic diseases
- First treatments for rare sleep-wake disorder
- Several new cancer treatments
- GLP-1 agonist for weight management
- First malaria vaccine
- Neprilysin inhibitor (in combination with an ARB) for the treatment of heart failure
- Antidote to the anticoagulant dabigatran

Until September 2015, 4 recommendations for marketing authorisation have been adopted following accelerated assessment and 2 positive Opinions concerned conditional marketing authorisations.

Revision of guidelines on Early Access tools

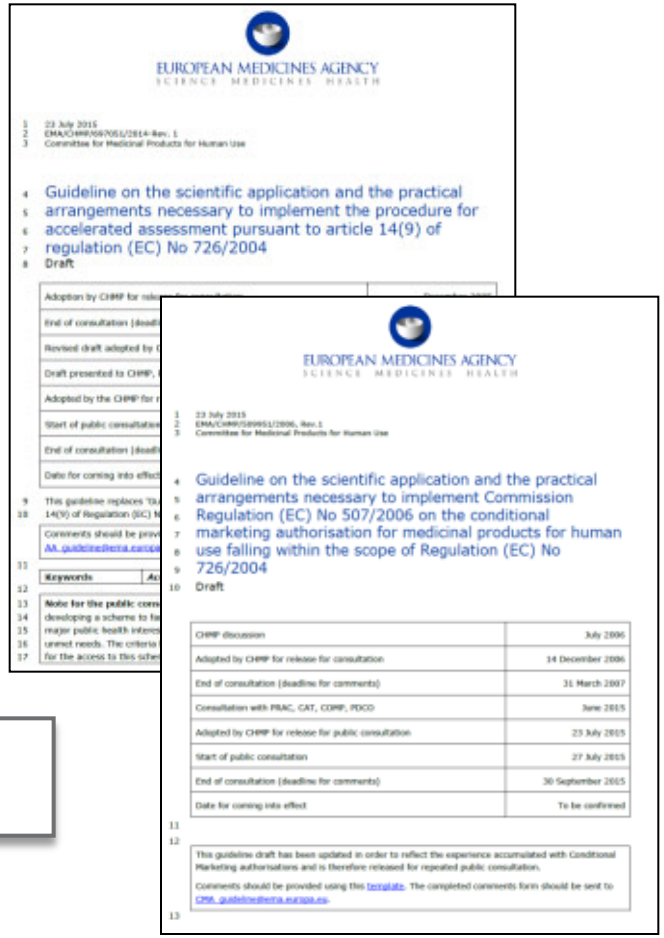
News

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

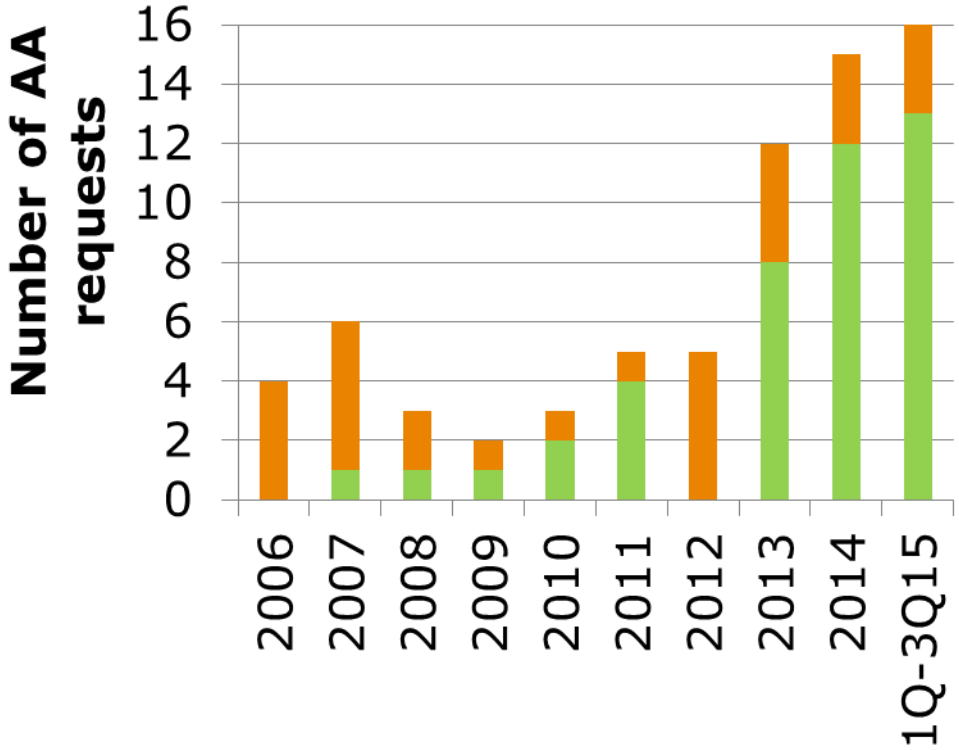
Draft revisions of these guidelines were published for public consultation until 30 September 2015

Commission Expert Group on Safe and Timely Access to Medicines for Patients ("STAMP")

- "Exchange views and information about the experience of Member States, examine national initiatives and identify ways to use more effectively the existing EU regulatory tools with the aim to further improve safe and timely access and availability of medicines for patients"
- Meetings held in January 2015 and May 2015
- Meeting materials (e.g. presentations) and report publicly available

http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm

Experience with Accelerated Assessment: Number of requests and acceptance rates



Acceptance rate in 2015 (1Q-3Q):
81%

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

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

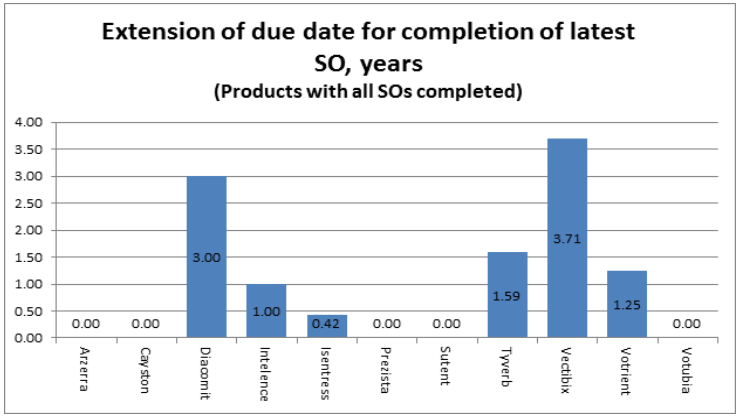
Process

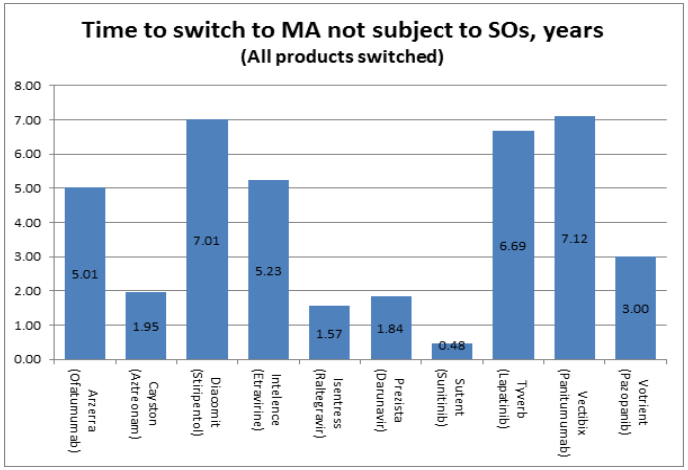
- Intent to request accelerated assessment to be indicated 6-7 months in advance and submission of accelerated assessment request to take place 2-3 months ahead of marketing authorisation application
- Importance of early dialogue / pre-submission discussions.
- Optimisation of the assessment timetable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

Conditional Marketing Authorisation granted in the centralised procedure (until 09/2015)

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 11 products with Specific Obligations (SOs) completed, on average the due date for completion of the **last SO was extended by 1 year**
- For 10 products that currently have a full MA (i.e. no longer subject to SOs), the switch was granted **on average after 4 years**





Note: data lock September 2015

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

Next steps for the finalisation of the revision of the guidelines

- The comments received during the public consultation are currently being reviewed: 17 different stakeholders have provided input on the accelerated assessment guideline, and 19 on the conditional marketing authorisation guideline.
- The finalisation of the revisions will involve the relevant scientific committees and the European Commission. An update report will be given at the forthcoming STAMP meeting.
- Coming into force of the revised guidelines is expected for 1st Quarter 2016.

Thank you for your attention

Further information

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