EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Better processes to facilitate earlier authorisation

3rd Industry stakeholder platform on the operation of the centralised procedure for human medicinal products

Presented by Victoria Palmi and Sabrina Spinosa on 21 April 2016

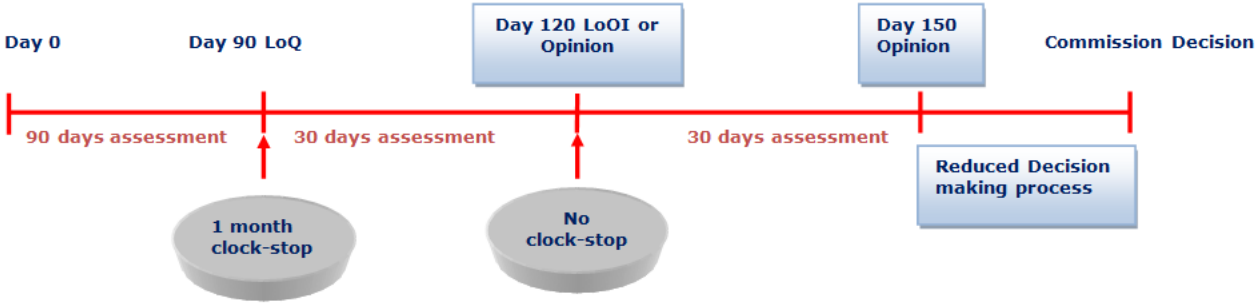
Importance of pre-submission dialogue

- **Early dialogue with EMA, CHMP/CAT and PRAC Rapporteurs :**
  - Notify the intention to request accelerated assessment 6-7 months before submission.
  - Pre-submission meeting with EMA and Rapporteurs as early as possible to present the data package and plans for key dossier elements (e.g. topics in the overviews, draft labeling, RMP outline).
- **Request** accelerated assessment at least **2-3 months** before the actual submission of the marketing authorisation application (MAA).
- Ensure **accuracy of the MAA submission date** for planning purposes.
- Provide **information on GMP and GCP aspects** to integrate routine GCP and pre-approval GMP inspections into the accelerated assessment procedure.

Request ahead of MAA submission

- 30 days assessment process with **clear timelines** will be available on EMA website
- The **Outcome** will be published in the CHMP highlights and minutes.
- The **reasons** that led to the CHMP's decision will be sent to the applicant and later published with the EPAR.
- An **appeal** mechanism is not foreseen but applicants may submit a new request should new evidence be considered.

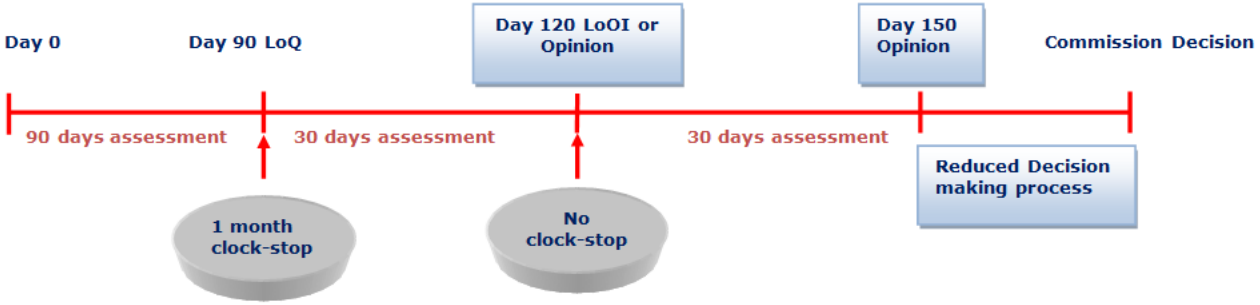
MAA evaluation process (1/2)



For MAA evaluations starting from September 2016

- Assessment split in 90/30/30 days
- The additional list of questions is expected to allow reducing the number of procedures reverted to a standard timetable
- CHMP will **adopt LoQ and loOI on Tuesday** allowing applicants extra time to plan a clarification meeting and to prepare the responses

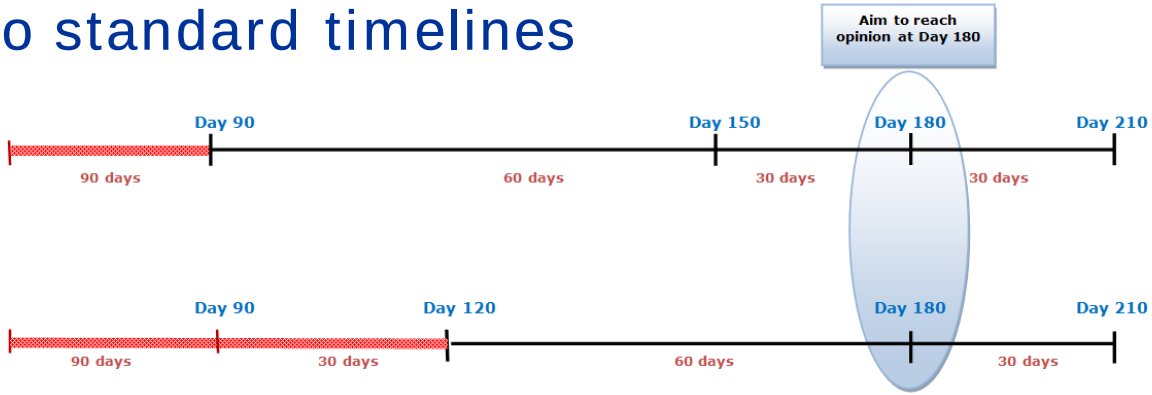
MAA evaluation process (2/2)



For MAA evaluations starting from September 2016

- Provided that all scientific and regulatory issues are resolved, option to reach an opinion at either day 120 or day 150
- Timelines for the translation check of the product information shortened
- European Commission has confirmed to also accelerate the Decision making process
- Reasons for accepting or rejecting a request for accelerated assessment to be published in the EPAR

Switch to standard timelines



- In case of major objections not resolvable under accelerated assessment.
- Request of extension of clock stop by applicants.
- Need for a triggered GMP or GCP inspection.
- Negative trend following the oral explanation.

The reasons for the switch will be described in the CHMP AR and published with the EPAR

Specific aspects for Advanced Therapy Medicinal Products

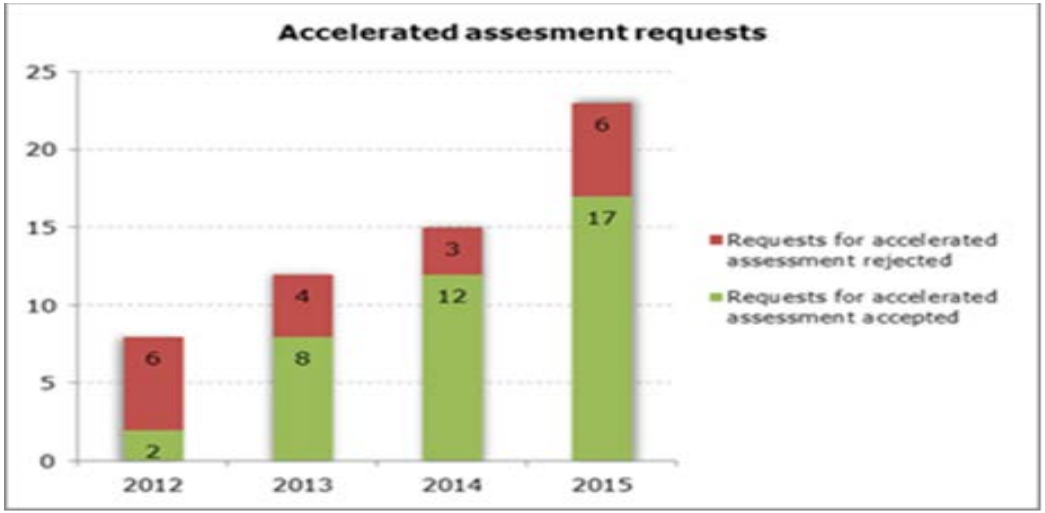
Any request for accelerated assessment will be **reviewed by the CAT** before endorsement by CHMP.

For Advanced Therapy Medicinal Products the timetables remains as **120 + 30 days** due to

- Expected complexity in the scientific and regulatory evaluation of these novel types of products;
- Evaluation complexity by having three Committees involved (CAT, CHMP and PRAC);
- Need for additional consultation in some cases (like with GMO competent authorities or notified bodies) .

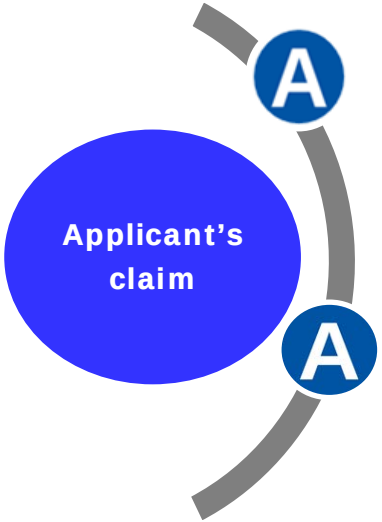
For ATMPs up to **3 months clock-stop** could be allowed.

Recent experience



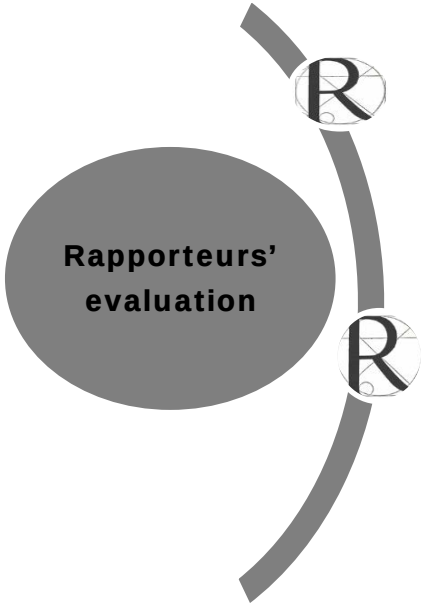
An increase in requests for accelerated assessment has been observed over the last years, along with a increase of acceptance rate by the Committees.

Addressing existing treatments and unmet medical need



Description of all **available treatment** options/Standard of Care and their important effects

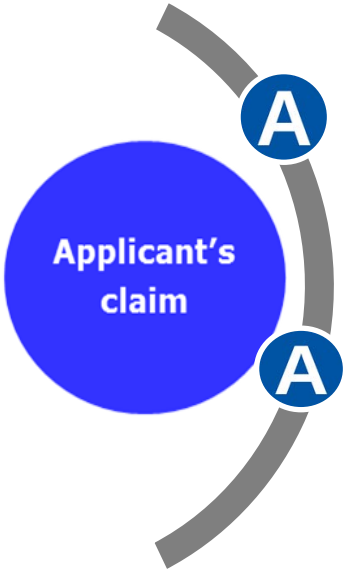
Substantiate **unmet medical need** :
- from literature (different indications or subpopulations)
- based on the observed effects or the added value and impact on medical practice.



Evaluate the applicant's description and its **accuracy** (Does it include all treatment options?)

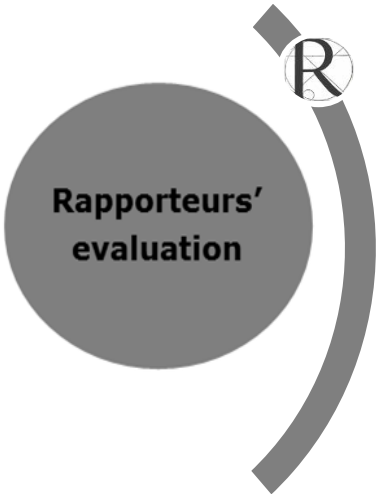
Conclusion on quantification of unmet medical need and identification of population or subpopulation
(How is the product expected or assumed to fulfil the unmet need? Has the unmet need been adequately described in epidemiological terms? Are important differences in the subpopulations well described?)

Addressing strength of evidence to support unmet need



Discuss number and types of **studies**, design and **key** supportive **results**

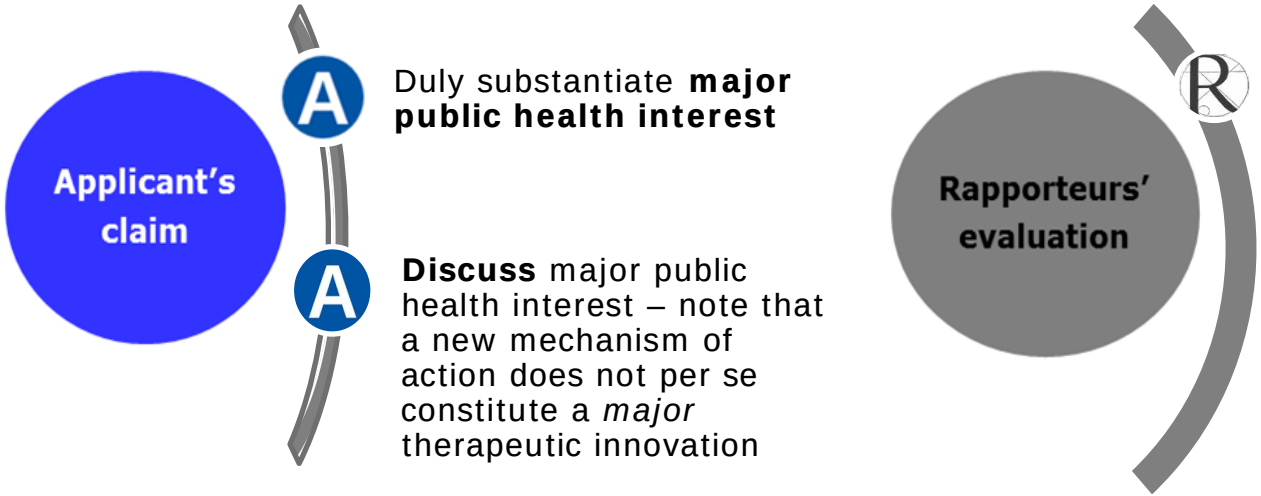
Summary of Scientific Advice and or Protocol Assistance, if development program has been agreed via Scientific Advice/Protocol assistance



Assess the strength of evidence to support the proposed claims.

Are, in principle, the available studies sufficient? Would the studies (e.g. sample size, design) in general allow drawing conclusions regarding the claims made? Do the studies allow for convincing quantification of efficacy and safety?

Addressing major public health interest and major therapeutic innovation



Conclusion on major public health interest and major therapeutic innovation from the point of view of public health

Has the request been duly substantiated?

Why or why not this constitutes a major public health interest ? Useful concepts: expected impact on medical practice, change in the natural history of the disease, therapeutic advantage compared to available treatments, survival benefit, improvement in safety, clinical relevance of the effect…

Better processes to facilitate earlier authorisation

- More detailed guidance how to **justify a request for accelerated assessment**
- **Promote early dialogue** between regulators and the applicant
- Publication of timetables to streamline **evaluation of request** for accelerated assessment
- More **transparency in the decisions** for accepting/rejecting/switch
- **Optimised timetable** for accelerated assessment of MAA with expected reduction of number of procedures reverted to standard timetable

Overall, an optimised procedural framework for the assessment of the marketing authorisation whilst ensuring the robustness of the scientific evaluation.

Further information

Guideline on the scientific application and practical arrangements to implement the procedure for accelerated assessment pursuant to Article 14(9) of Regulation (EC) No 726/2004
http://www.ema.europa.eu/docs/en_GB/document_library/Scientific_guideline/2016/03/WC500202629.pdf

Applicant Request on Accelerated Assessment template
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/document_listing/document_listing_000339.jsp&mid=WC0b01ac05804740c9

EMA Pre Authorisation Guidance Q&A – Question 11. Is my product eligible for an accelerated Assessment?

Support for early access new tab on EMA website.

Thank you for your attention

Further information

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