EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA presentation on proposals to optimise treatment within current procedures

Cancer Medicines Forum meeting 4th December 2023

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The views expressed in this presentation are the personal views of the speaker and may not be understood or quoted as being made on behalf of or reflecting the position of the EMA committees or working parties

How to integrate treatment optimisation in existing EMA procedures?

In Scientific Advice/Protocol Assistance

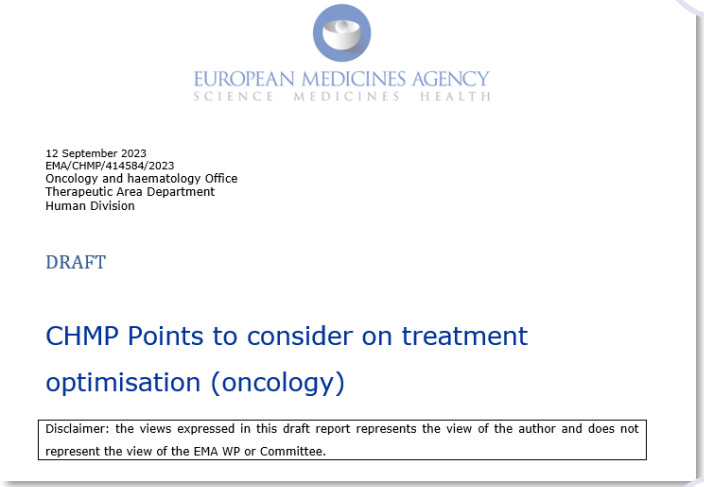
- Prompt applicant to ask specific questions around treatment optimisation (e.g. dose)?

Project Optimus and Pragmatica by FDA

- Some questions might only arise during evaluation of the MAA
- CHMP identifies these gaps which could be:
  - Either addressed as imposed post-authorisation studies
  - Or clearly described in the European Public Assessment Reports

As part of Marketing Authorisation Application

How to integrate treatment optimisation in existing EMA procedures?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Oncology and haematology Office
Therapeutic Area Department
Human Division

DRAFT

CHMP Points to consider on treatment optimisation (oncology)

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- **Scientific Advice should be sought systematically** and involve relevant decision makers (parallel joint SA):

  Based on existing extensive experience with collaboration with health technology assessors and insurers as well as patient and learned societies

- **Proactive guidelines** to be developed in active fields

- **Advice should systematically include treatment optimisation** questions and planning

- **Scientific assessment reports** should systematically **describe treatment optimisation questions**; updates to be made publicly available

- **Monitor use and opportunities for further development**:

  Establish a system for monitoring of use in real life and opportunities for further development, e.g., in new populations. This should be systematically investigated, reported as part of PSURs, and published to inform the research community

Treatment optimisation questions and recommendations

TO question	Actions
Posology, special patient populations, predictors of benefits and harms	• Describe assessment of different dosage groups (dose, treatment schedule and duration) and criteria for dosage selection and further optimisation. • Describe any questions addressed during exploratory and confirmatory trials, and subsequent trials. • Describe explanatory trials in selected institutions and populations, surrogate endpoints. • Describe expected number of patients for PK/PD studies and relevant subgroups and special populations. • Describe any newer technologies such as virtual control arms, total personalisation through AI. • Describe differences in patient characteristics between studied and expected target population.
Informing treatment decisions	• Comparative effectiveness to allow for informed clinical decision making. • Develop comprehensive evidence on true clinical endpoints if gaps identified (quality of life, overall survival).
Off-label use; therapeutic context	• Monitor off-label use and opportunities for further development and optimisation, e.g., new combinations, development in further patient populations, minimising toxicity and adverse interactions. Monitor target population in real life and any opportunities for further optimisation (e.g., subgroups). • Systematically seek treatment optimisation identified by the patient/academic community which arise when the medicine is put on the market or when other products are authorised in the same indication.

Which broader activities should be thought about?

Discussion

Thank you for your attention

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