EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Cancer Medicines Forum treatment optimisation framework

Cancer Medicines Forum workshop 5th April 2024

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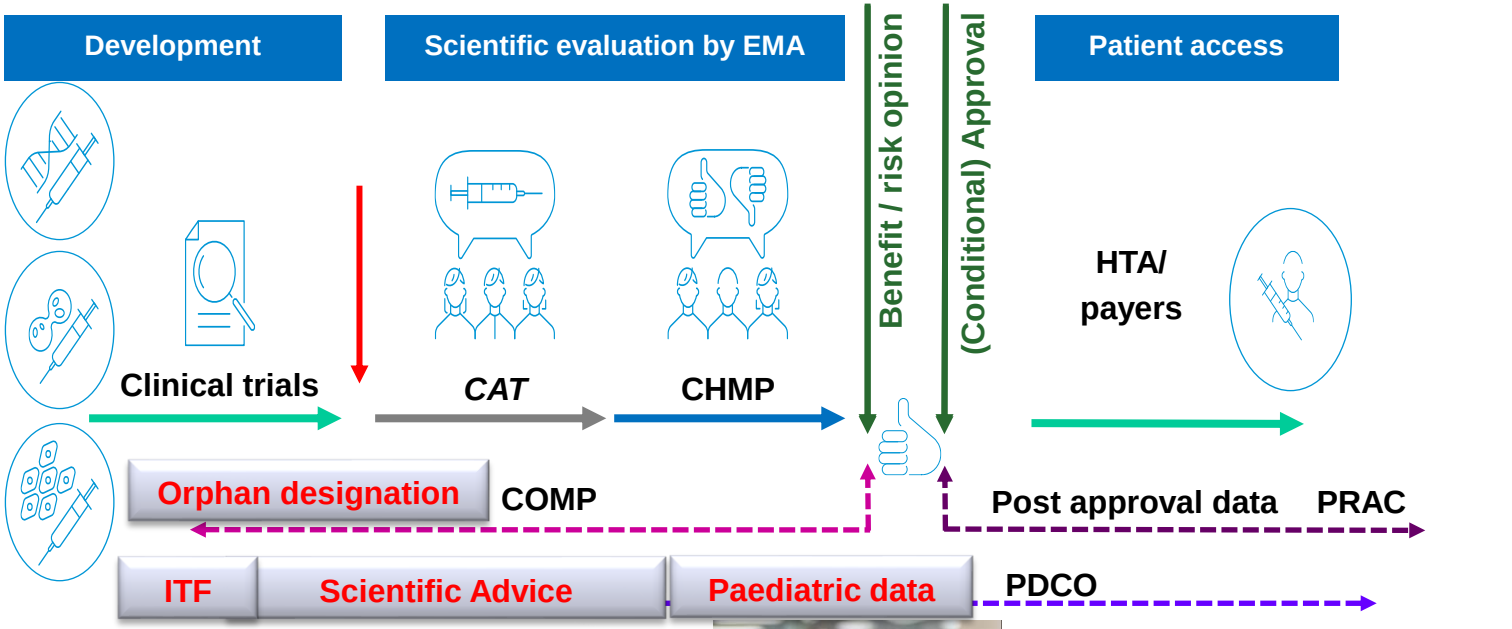
The views expressed in this presentation are the personal views of the speaker and may not be understood or quoted as being made on behalf of or reflecting the position of the EMA committees or working parties

Setting the scene

- EMA **has limited responsibilities** with regards to treatment optimisation
- EMA is responsible for confirming positive Benefit/Risk balance of a medicine
- In this presentation **personal views are expressed based on current thinking and might evolve and be subject to change**
- **Expected to be developed** into guideline following standard processes and communication

Evaluation of medicines today



CAT — Committee for Advanced Therapies
CHMP — Committee for Medicinal Products for Human Use
COMP — Committee for Orphan Medicinal Products
PRAC — Pharmacovigilance Risk Assessment Committee
PDCO — Paediatric Committee

EMA Cancer pathfinder initiatives

Build **on collaborations with stakeholders**

- Optimal application of regulatory tools and science
- Address the complexity of approval based on limited data

Support the network

- Support building capacity scientific quality in the assessment

Support relevant **evidence generation**

- Gain experience with new approaches (pragmatic trials);
- Develop guidance on **patient-focussed drug development**
- Establish a **Cancer Medicines Forum with academia** to develop treatment optimisation priorities and studies

Cancer pathfinder

Where to start?

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

clinical evidence generation

Initiate disease specific workshops/webinars

- Understand current challenges with existing treatments, clinical practice
- Unmet medical need, gaps in existing treatments

Facilitate companies' seeking advice systematically and involve relevant decision makers

- Based on existing experience
- Collaboration with health technology bodies, FDA, insurers as well as patients and learned societies
- Linked with ACT EU initiatives

- Systematically include treatment optimisation questions and planning in drug development and scientific advice

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

How to integrate treatment optimisation in existing EMA procedures

post-marketing

- **Scientific assessment reports** should systematically **describe treatment optimisation questions**; updates to be made publicly available (e.g. submission of study results)

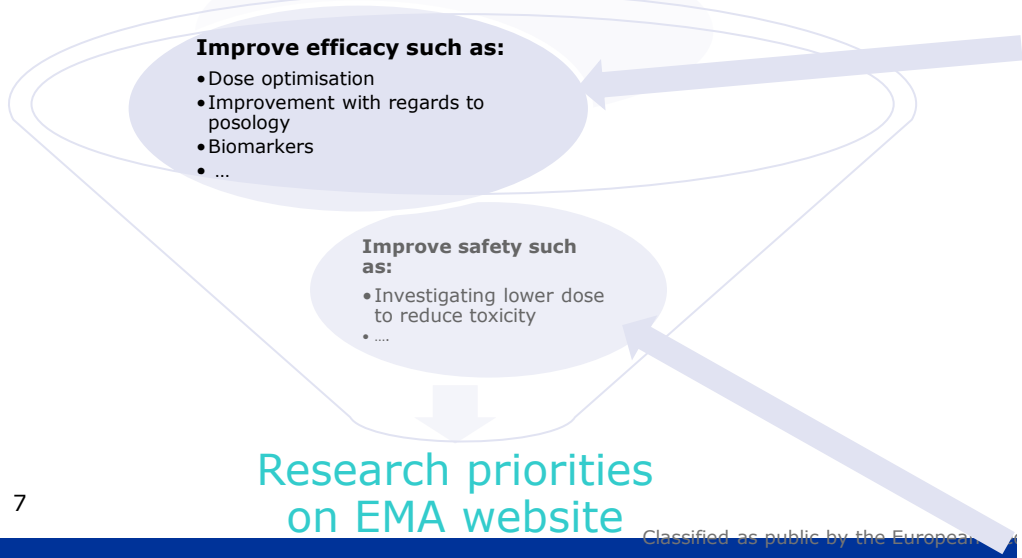
- **Monitor use**:

Establish a system **for monitoring use in real life**. This should be systematically investigated, reported as part of PSURs, and published to inform the research community

Treatment optimisation questions post-marketing

Highlighting gaps in order to improve the efficacy and safety profile of the medicine:

- Generally aspects not precluding a MA
- Gaps identified endorsed by EMA as area for further research
- Aim to improve treatment for patients (societal questions)



5.1. Therapeutic Context

5.1.1. Disease or condition

5.1.2. Available therapies and unmet medical need

5.1.3. Main clinical studies

5.2. Favourable effects

COMMENTS

- Avoid interpretation and value judgements (e.g., it was convincingly shown that overall survival was greatly improved for treatment X).
- This section should be consistent with the favourable effects described in 5.6. Effects Table and with the SmPC section 5.1. No new results should be introduced here that have not been described in detail in the previous sections
- This section does not need to be updated during the procedure unless new key results are submitted

For more guidance on definitions of favourable effects, how to select "key" effects, and examples, see the *D80 assessment report Overview template/guidance+D120 LOQ*.

5.3. Uncertainties and limitations about favourable effects

5.4. Unfavourable effects

COMMENTS

- Avoid interpretation and value judgements (e.g., low-grade toxicity for treatment X was significant);
- Try to avoid long lists of individual side-effects. If meaningful, try to group them (e.g., in terms of their consequences such as life-threatening reactions or by System Organ Classes).
- This section should be consistent with the unfavourable effects described in 5.6. Effects Table, the important identified risks described in section 3.4 Risk Management Plan, and the SmPC Section 4.8. No new results should be introduced here that have not been described in detail in the previous sections (typically under Clinical Aspects).
- This section does not need to be updated during the procedure unless new key results are submitted

For more guidance on how to describe unfavourable effects, see the *D80 assessment report - Overview & D120 LOQ template with guidance* .

5.5. Uncertainties and limitations about unfavourable effects

Cancer is a priority

Cancer Medicines forum (CMF)

- Launched together with EORTC
- Multi-stakeholder platform for exchange of information
- Successfully being piloted since 2022
- Future to be defined

Possible actions and next steps

- Number of actions identified in development and post-marketing
- Consider development of guideline and update of CHMP AR template

Thank you for your attention

Acknowledgements:

Francesco Pignatti
Fran Day
Pierre Demolis
Harald Enzmann

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