



**Recommendation of the EUCERD to the
European Commission and Member States on**

**Improving Informed Decisions Based on the
Clinical Added Value of Orphan Medicinal
Products (CAVOMP) Information Flow**

Adopted on 12 October 2012

Orphan Medicinal Products Specificities

- Unmet medical need for debilitating life-threatening diseases
- Lack of patients, data & expertise
- Regulatory approval despite overall lack of evidence
- Difficult for national authorities to understand the value of what they have to assess
- Cooperation & gathering of existing knowledge and expertise can help throughout the RD field

CAVOMP in its policy environment

The CAVOMP stems from the EU Regulation on OMPs - already based on EU cooperation - and from 5 years of policy work with the EU MSs.

- 2008 - The conclusion of the EU Pharma Forum “Improving Access to OMPs for all affected EU citizens” mentioned the *exchange of knowledge amongst MSs and EU authorities on the clinical added value of OMPs*;
- 2008 - The Commission Communication on Rare Diseases: Europe’s Challenges called for the establishment of a *Working Party to exchange knowledge between MSs and EU authorities*.

CAVOMP in its policy environment

- 2009 – The EU Council Recommendation on a European Action in the Field of Rare Diseases mentioned the *sharing of MSs assessment reports at Community level, where the relevant knowledge and expertise is gathered.*
- 2010 - The Commission mandated Ernst & Young to conduct a study and produce a report on the “creation of a mechanism for the exchange of knowledge between MSs and EU authorities on the scientific assessment of the clinical added value for OMPs”.

EUCERD

EUCERD Members:

- 27 EU MSs representatives and EEA/candidate countries
- Patients representatives
- Industry representatives
- Academia / representatives of European-funded projects
- European Commission, ECDC
- EMA, COMP – Observers

The EUCERD role is to advise the European Commission with the preparation and implementation of Community activities in the field of rare diseases.

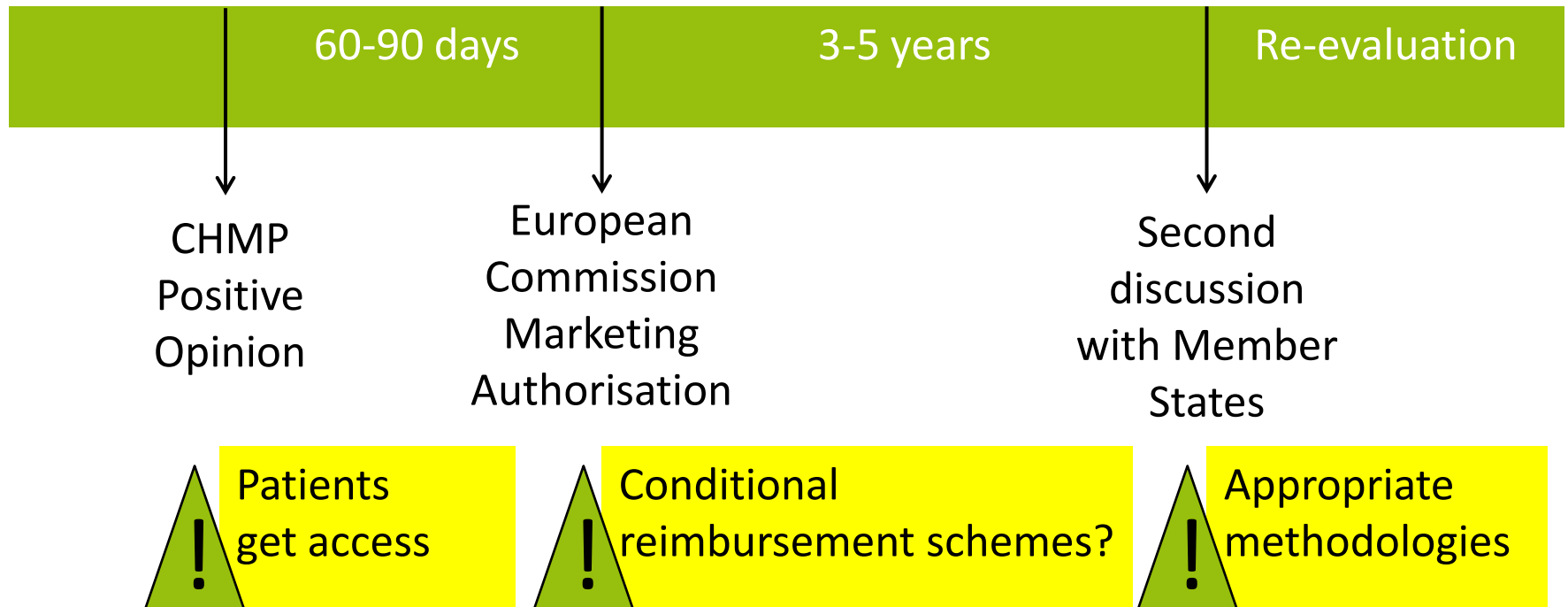
EUCERD Recommendation

“Improving informed decisions based on the clinical added value of OMPs” in order to enhance access for patients through optimisation of processes via EU collaboration.

- ◆ CAVOMP is a process for the exchange of knowledge between MSs
- ◆ CAVOMP respects the roles and responsibilities of all actors at all levels of the process
- ◆ CAVOMP respects the existing steps of the regulatory process for OMPs authorisation
- ◆ CAVOMP includes a series of actions/interactions to facilitate the exchange of knowledge
- ◆ CAVOMP adds “oil in the machine” – not a new machine

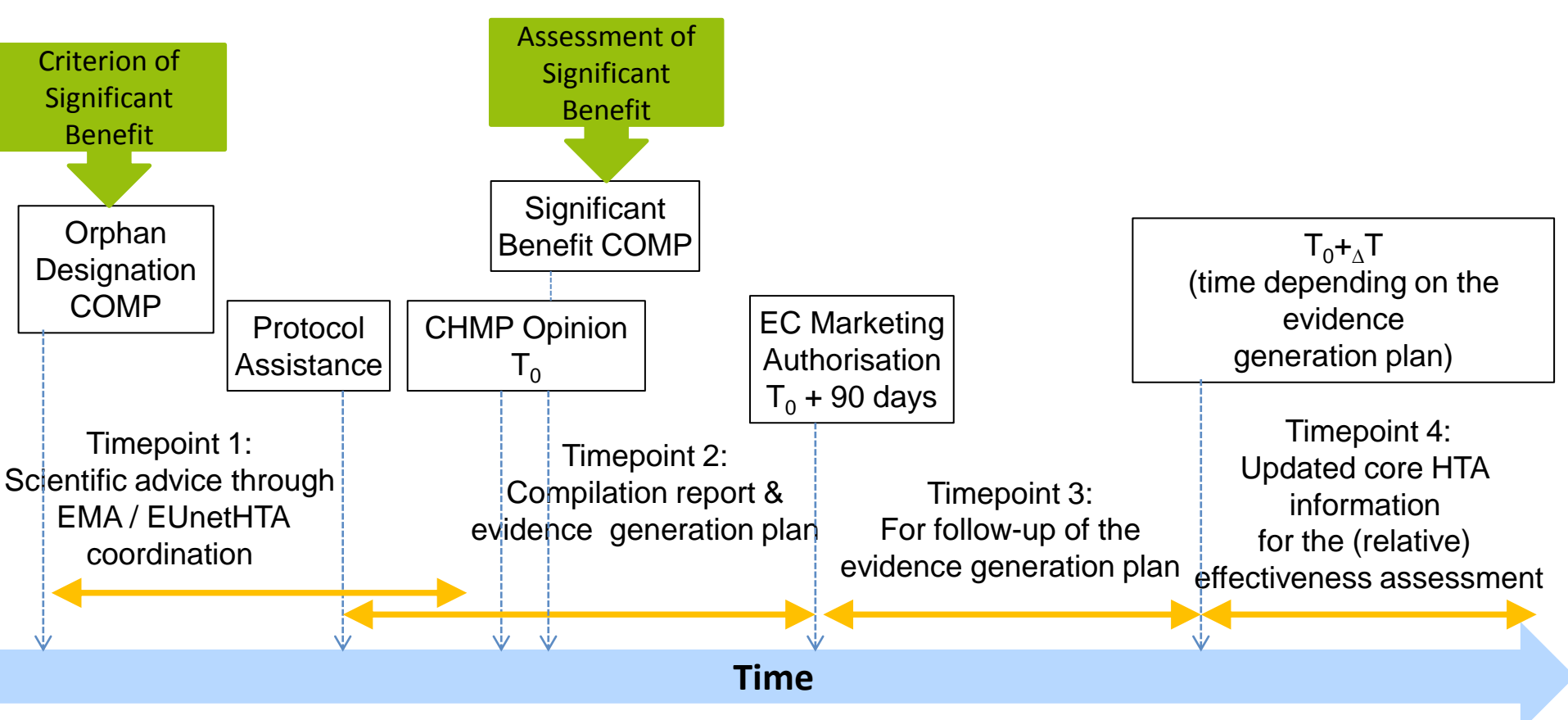
“To facilitate Member States’ informed decisions”

- Consolidated Common Report
- Data – MA & COMP revision of criteria
- Agree on “Post-MA research activities”
- Compilation of post-MA data – registries, etc.
- Updated consolidated Common Report
- Data – In-use



Four key time-points in the process

1. Early dialogue
2. Compilation Report & evidence definition / Evidence Generation Plan
3. Follow-up of the evidence generation plan
4. Assessment of Relative Effectiveness



Early Dialogue	Information exchange and defining the evidence generation plan	Evidence generation	Assessment
<ul style="list-style-type: none"> EMA EUnetHTA / payers Sponsor Patients Experts 	<ul style="list-style-type: none"> EMA EUnetHTA / payers Sponsor Patients & treating physicians 	<ul style="list-style-type: none"> EMA EUnetHTA / payers MAH Centres of Expertise (CE) & European Reference Networks (ERNs) 	<ul style="list-style-type: none"> EUnetHTA / payers EMA MAH Patients & CEs/ERNs
<ul style="list-style-type: none"> Could be implemented already 	<ul style="list-style-type: none"> Could be implemented already 	<ul style="list-style-type: none"> Could be implemented already 	<ul style="list-style-type: none"> Adapted methodological tools for OMPs to be developed

Building on EMA + HTA collaboration

- The European network of HTA agencies (EUnetHTA) already collaborate with the EMA
- The Cross-Border Healthcare Directive provides for a permanent network of HTA bodies
- EMA & HTA already cooperate on key elements:
 - Beyond cooperation on improvement of the EPARs;
 - Early dialogue and scientific advice – including multi-stakeholder pilot meetings;
 - Post-launch collaborative data collection;
 - Cooperation on guideline development, including assessments and Clinical Trial design.

The CAVOMP Report

- A single report based on existing assessments by experts from Member States to be made available at time of Marketing Authorisation (MA)
- Compiled information from:
 - European Public Assessment Reports (Committee on Human Medicinal Products)
 - Orphan Designation Reports (Committee on Orphan Medicinal Products - COMP)
 - Confirmation of Significant Benefit at time of MA (COMP)
 - Paediatric Investigation Plan (EMA Paediatric Committee)

Aim of the CAVOMP Reports

The aim of these Reports on the scientific assessment of the relative effectiveness of OMPs is to provide a well-informed opinion on the place of the authorised products in the therapeutic strategy of the rare condition, to the best knowledge at time of MA and few years later based on the agreed post-marketing evidence generation plan.

→ This mechanism does not imply any additional burden, no new review, no new data to be provided while respecting the roles and responsibilities of all involved parties.

Thank you for your attention!

The text of the recommendation can be found on the EUCERD website

<http://www.eucerd.eu/?p=1699>

Home page: www.eucerd.eu