Benefit/risk evaluation : patients now invited to oral explanations @ CHMP

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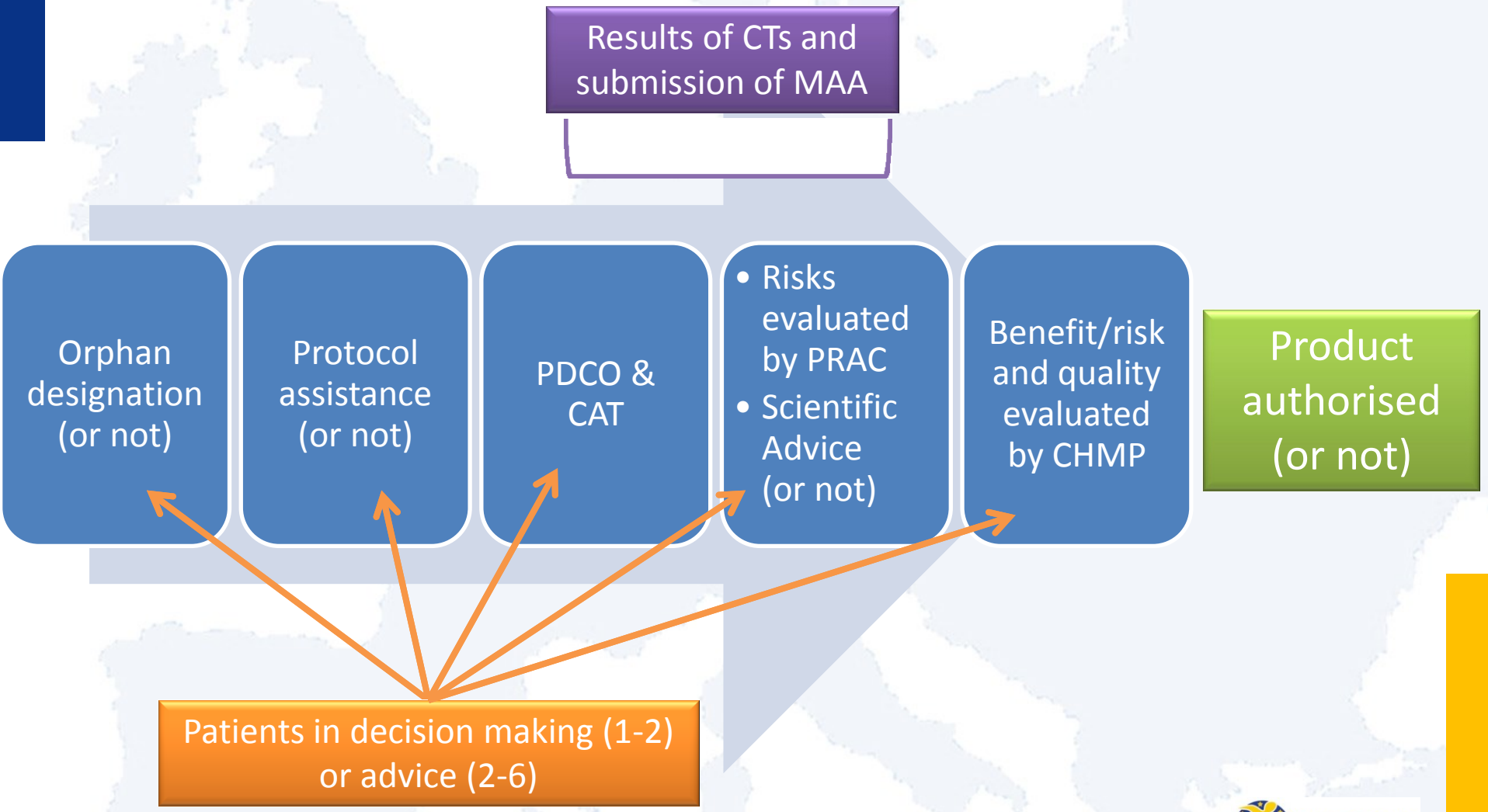
The regulatory process and where we jump in



The CHMP momentum

+/- 2 months to organise it

Day 0
- Submission of marketing authorisation application (MAA)

Day 120
- Rapporteurs' report
- Comments from CHMP members
- List of Questions

Day 121
- Submission of responses by applicant

Day 150
- Rapporteurs' report on responses
- Day 180: CHMP outstanding issues

Day 180-210
- Final opinion +/- hearing of the company (oral explanation)

Early detection that the dossier is a difficult one. Scientific Advice can be envisaged

If concerns or doubts within CHMP members: an oral explanation can be proposed
If relevant : to invite 2 patients and a mentor

EMA announces

Press release

26/09/2014

Patients to discuss benefit-risk evaluation of medicines with the Committee for Medicinal Products for Human Use

EMA launches pilot project to integrate patients' unique and critical views into CHMP discussions

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2014/09/news_detail_002172.jsp&mid=WC0b01ac058004d5c1

(few) consultations with patients in the past (not exhaustive, rare diseases)

- January 2002 - 2007
- August 2003
- 2005 – 2006
- July 2006
- 2009 - present
- April 2011
- May 2011
- Sept. 2012

- Thalidomide, multiple myeloma (RMP, b/r)
- rhGH (Serostim® HIV cachexia, OMP) (b/r, request by rapporteur – in his report)
- Lenalidomide (myelodys. s.) (RMP, b/r)
- Lenalidomide (mult. myeloma) (RMP)
- Cerezyme/Fabrazyme shortages
- Celecoxib (Onsenal® familial adenomatous polyposis) (b/r renewal)
- Vpriv® (velaglucerase alfa long-term ERT type 1 Gaucher) (b/r)
- Pomalidomide (multiple myeloma) (RMP)

As of 2014: Patients' and Consumers' Working Party and CHMP have agreed

- a pilot phase which would explore how this could occur to maximal effect
  - To demonstrate our participation **adds value** to the scientific discussion
- The Rapporteurs and EMA team leaders will decide on a case-by-case basis when this will be needed
  - When the CHMP is likely to recommend the refusal of a MA for a new medicine where there remains an unmet medical need
  - When the PRAC/CHMP are likely to recommend the withdrawal, suspension, revocation or restriction of an indication for a medicine for which a significant impact in patient population is expected

Which patients should be invited?

- Patients (or carers) with personal experience and knowledge of the particular disease/condition under evaluation will be invited to participate
- Assessment of any potential conflicts of interest
- Contact via the EMA network of eligible patient organisations, other European or national organisations or individuals who have expressed an interest to participate in the Agency's work
- Need to be able to understand and communicate in English.

The patients will be accompanied by a ‘mentor’ (likely a PCWP member)

- During pilot phase (1st year)
  - By order of “appearance”
    - September 14 – November 14: François Houÿez (EURORDIS)
    - December 14 – February 15: Hildrun Sundseth (EIWH)
    - March 15 – May 15: Richard West (EURORDIS)
    - June 15 – Sept 15: Erik Briers (Europa Uomo)
- Their role: more to explain the procedure, to remind them some rules, and to make them comfortable than to intervene in the discussion/content

How should patients join the discussion?

- Usually CHMP will send a few questions ahead of the meeting
- Patients will give their views on these questions and may participate actively in the discussions
- Patients can also ask questions to the company
- Patients do not take part in any decision - making process (no voting rights)
- Patients may join the meeting for the briefing by the rapporteurs, followed by the company presentation (20 min) and subsequent Q&A session. They may also remain for the discussion and conclusions, but would leave prior to voting.

How should patients join the discussion?

- Other provisions
  - Patients would be given the option to remain in a separate meeting room and follow the oral explanation by video link if they prefer, joining the CHMP for the subsequent discussion
  - If a patient is not able to travel, there is also the possibility to join via teleconference or to provide comments in writing beforehand
  - 'Rules of participation' and confidentiality undertaking
  - You receive the list of questions to the company, and the rapporteurs' report (80+ pages)
    - But often very shortly before the meeting

Potential conflicts of interest and confidentiality aspects

- Every patient will be required to complete a declaration of interest / confidentiality agreement prior to formal invitation.
- They undergo screening for conflicts of interest in the same manner as all other experts.
- Patients must adhere to the confidentiality of the documentation they receive and the discussions they partake in.
- They participate as individuals and should not discuss the documents received with others.

In practice

- Company can attend the oral explanation with their own experts. These experts can be:
  - Clinical investigators
  - Other scientists
  - Patients (different than the 2 ones invited by CHMP)
- Where you can play a key role: when CHMP members have difficulties understanding what the product can improve/change in your life
  - Evaluation criteria not always clinically meaningful
- You can make a Powerpoint presentation to guide your speech, or not

Concerns

- This is a test, an experiment. Crucial to make it right from the beginning. Can stop at any time
- There are formal rules, need to be fully compliant with them
  - The form is as important as the content
  - As soon as invited by CHMP, and until the EMA announces the opinion on its web site:
    - You can't talk with anyone else except the other invited patient, the EMA staff/rapporteurs and the mentor
    - Refrain from talking with other patients, or clinicians
    - Refrain from talking with the company
- Confidentiality +++, prevention of insider trading +++

Recommendations

- Adapt your practices
  - When meeting with the developer of a medicine, even years before the CHMP momentum, make the agendas of your meetings public
  - When invited by the EMA, provide them with the dates when you met with the company, and the agenda of the meetings
- Sign the “Code of Practices guiding the Relations Between the Healthcare Industry and Patients’ Organisations”
  - And implement it in your organisation

Remember

- All documents and discussions are confidential from the moment you're invited (by a mentor or the EMA)
- Your names are communicated to the company ahead of the meeting
- After the CHMP opinion is public on EMA website:
  - The content of the discussion remains confidential for ever
  - The only thing that becomes public: date of meeting, name of product, name of company, and the fact you were invited
- Oral Explanation can always be cancelled at the last minute – CHMP meeting with patients should be maintained

Also remember

- That EMA doesn't consider pricing and reimbursement of medicines
  - No need to talk about this
- Political statements are useless
  - What matters is your views, or data from your organisation you may wish to share
- CHMP knows when there is an unmet need