CPMP / CHMP consultations with patients on b/r

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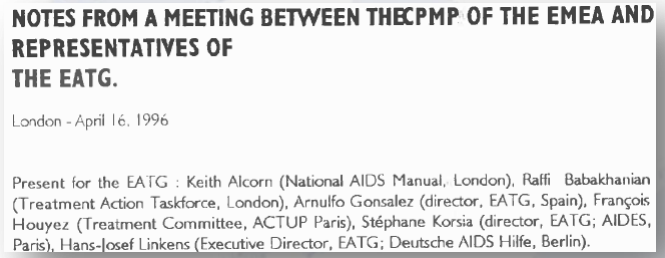
Workshop on the patient's voice in the evaluation of medicines. 26/09/2013 @ EMA

Consultations triggered by CHMP (not exhaustive)

- January 2002 - 2007 — Thalidomide, multiple myeloma (RMP, b/r)
- August 2003 — rhGH (Serostim® HIV cachexia, OMP) (b/r, request by rapporteur – in his report)
- 2005 – 2006 — Lenalidomide (myelodys. s.) (RMP, b/r)
- July 2006 — Lenalidomide (mult. myeloma) (RMP)
- 2009 - present — Cerezyme/Fabrazyme shortages
- April 2011 — Celecoxib (Onsenal® familial adenomatous polyposis) (b/r renewal)
- May 2011 — Vpriv® (velaglucerase alfa long-term ERT type 1 Gaucher) (b/r)
- Sept. 2012 — Pomalidomide (multiple myeloma) (RMP)

Interactions triggered by POs (not exhaustive)

- 16 April 1996



NOTES FROM A MEETING BETWEEN THECPMP OF THE EMEA AND REPRESENTATIVES OF THE EATG.

London - April 16, 1996

Present for the EATG : Keith Alcorn (National AIDS Manual, London), Raffi Babakhanian (Treatment Action Taskforce, London), Arnulfo Gonsalez (director, EATG, Spain), François Houyez (Treatment Committee, ACTUP Paris), Stéphane Korsia (director, EATG; AIDES, Paris), Hans-Josef Linkens (Executive Director, EATG; Deutsche AIDS Hilfe, Berlin).

- May 2002
- Nov. 2009
- July 2009
- May 2008
- Sept. 2010
- June 2013

- HIV products evaluation criteria
  - To replace clinical endpoints by surrogate markers HIV RNA and CD4 cells
  - Workshop Sept. 96
  - Duration of trials shortened (48 to 6 months)
- Ganciclovir implant (withdrawal)
- Bendamustine (MM, with EMP Greetje Goossens and Doris Mayerböck) (b/r)
- Dextropropoxyphene (pain-killer) (b/r)
- Icatibant (Firazir® hereditary angiodema) (b/r)
- Modafinil (Provigil® narcolepsy pts idiopathic hypersomnia)
- Scenesse® (erythropoietic protoporphyria – online patient community) (b/r)

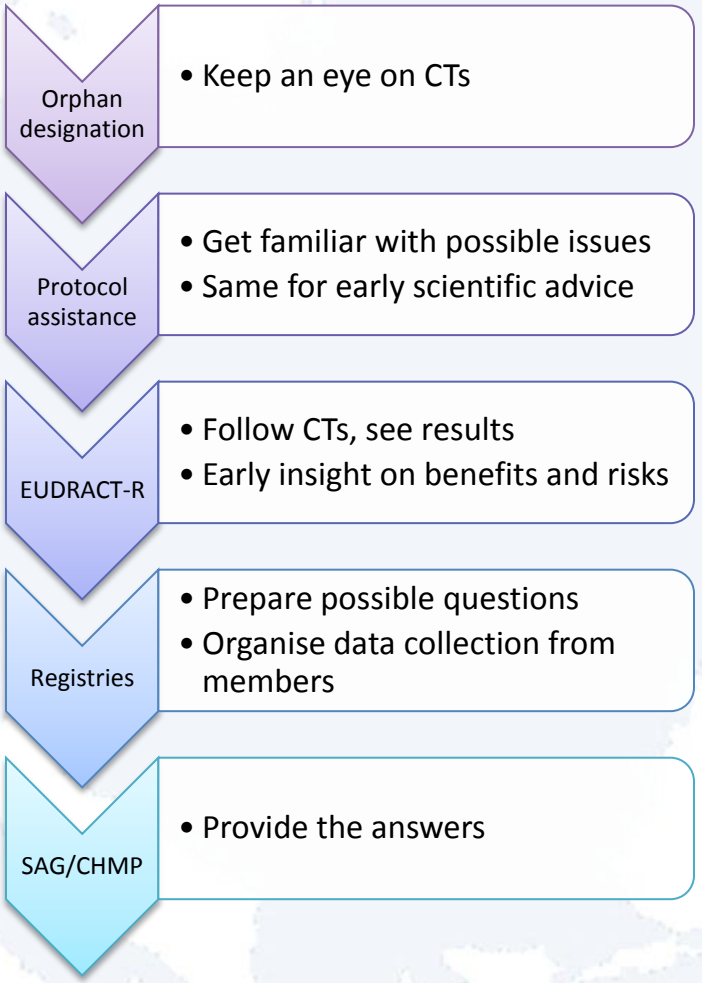
Some questions are challenging

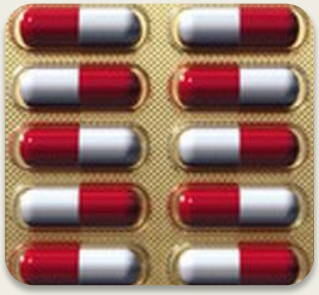
Onsenal® (celecoxib) for familial adenomatous polyposis

1. How does the PO capture the use of celecoxib in patients with FAP in Europe? What differences can the PO report in the management of this disease in patients living in EU countries where Onsenal is not marketed as compared to those where Onsenal is marketed and available to them?
2. What kind of general feedback does the PO have from patients with FAP who are currently using Onsenal?
3. Are patient organisations aware that Onsenal belongs to a class of medicines for which public health concerns had been identified? Is the information on severe cardiovascular risk included in the Package Leaflet (PL) of Onsenal sufficiently clear for the patients who are currently taking them?
4. Can patients report any (long-term) impact due to the treatment with Onsenal (both in terms of benefits and tolerability)?

Get ready, plan ahead

- Consultation process is rapid: tight timelines
- Little time to prepare responses, even less time to collect data
- CHMP agendas now public, but when product comes on the table, often too late to prepare yourself (as an organisation)
- Advice from “scientific committee” not what is expected
- Facts, data on b/r, no political statement, no statement on price or reimbursement





Constrains and ease of use

Frequency of side effects

Efficacy including quality of life

Severity of side effects

Uncertainty