

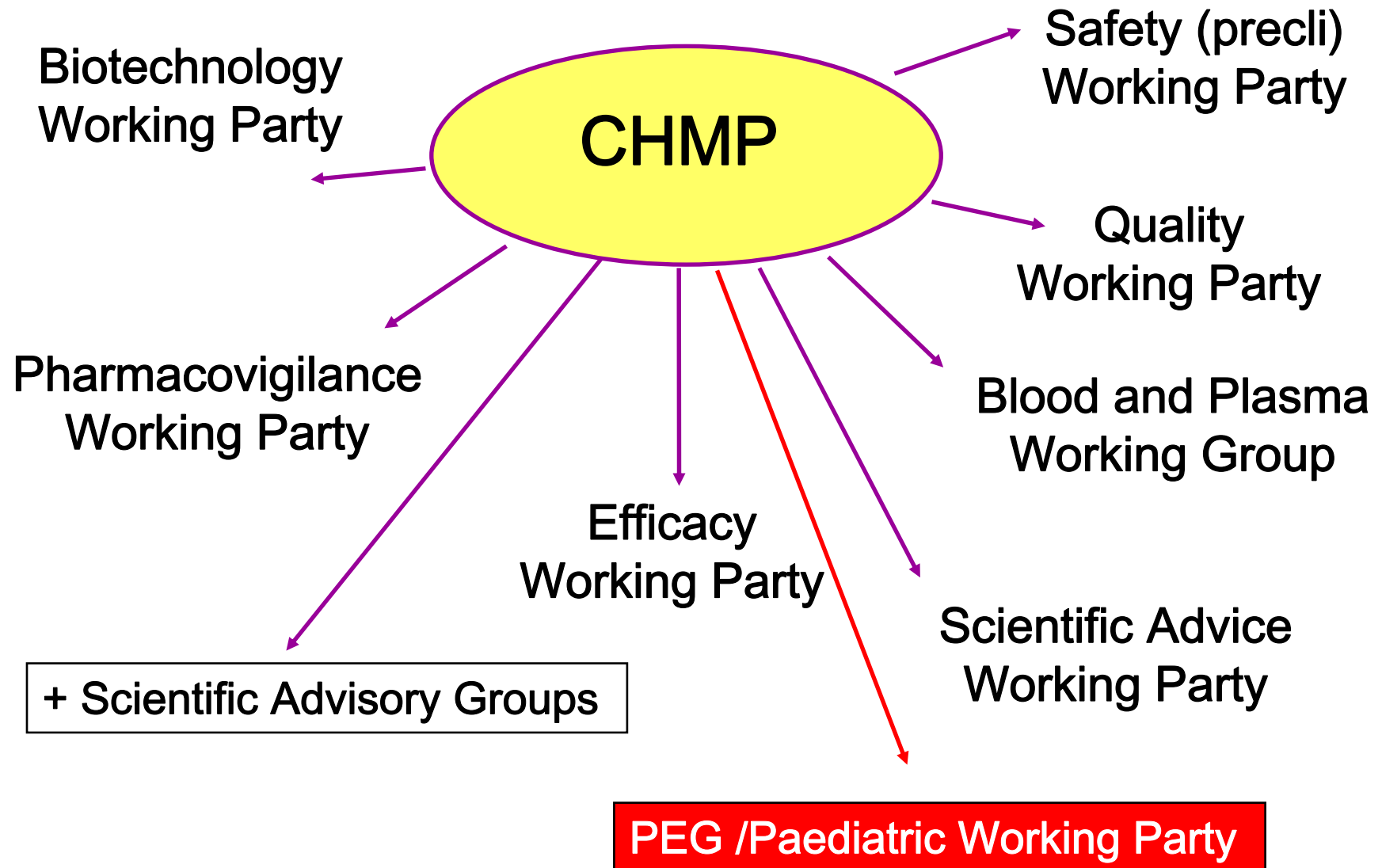
# **The Off patent Priority List**

## **Paediatric Working Party (PEG)**

**EMEA**

# The Paediatric Expert Group

- Expert Group for the Committee for Human Medicines (CHMP) created in 2001
- 13 experts in various domains, + observers
  - Met 4 times a year
  - Worked on guidelines
  - Worked on Paediatric Needs at EU level
  - Worked on Priorities for studies into off patent medicines



# Why work on priorities?

- Previous attempts to draw up a list had not achieved the objective (US, UK)
- Main reasons were lack of expert consensus
- Difficulty to agree priority criteria

Request from European Commission:

- Preparatory work for future Regulation
- Budget for clinical trials publicly funded
- Refer to existing lists (UK, FR, Ge, US)

# The process

*Request July 2003- Completion January 2004*

- Prepare strategy and criteria with PEG
- Draft list of conditions and products (use existing lists)
- All potential off-patent products from review articles
- Consult national authorities, Learned Societies at European and National level
- Compile comments and finalise...

# Strategy

- Source information on medicines from published literature (general review of therapeutics in the various domains) and textbooks
- List potential paediatric conditions
- List products per conditions
- Set priority criteria for conditions AND priority criteria for products

# Prioritisation

- About 300 conditions in various therapeutic areas: priority for
  - Seriousness (lethal or debilitating)
  - High prevalence (relative to children)
  - Disease affects all age ranges
  - Disease affects newborns
  - Disease has
    - no therapeutic options available (authorised),
    - Some options but not fully satisfactory
    - Full therapeutic options

# Paediatric medicines

- List as exhaustive as possible (no info on patent, but long existence?)
- Evidence-based medicine
  - ‘Win-Win’ situation
- Priority:
  - Availability of data on the product (evidence vs. expectations)
  - Positive points for evidence of efficacy
  - Negative points for evidence of safety concern in children

# Adding up the points!

- Maximum of 9 for condition
- Maximum of 3 for evidence (i.e. when randomised controlled trials)
- Cut-off value for priority was set at 10
- Outcome: 65 products with 10 or more priority points

# Experts consultation

- Initial disbelief in the procedure
- No outcry in view of outcome then large support of the exercise
- Most comments were included in the tables
- Reasonable agreement with priority list produced by NIH/FDA in 2003 (12 then 8 products)

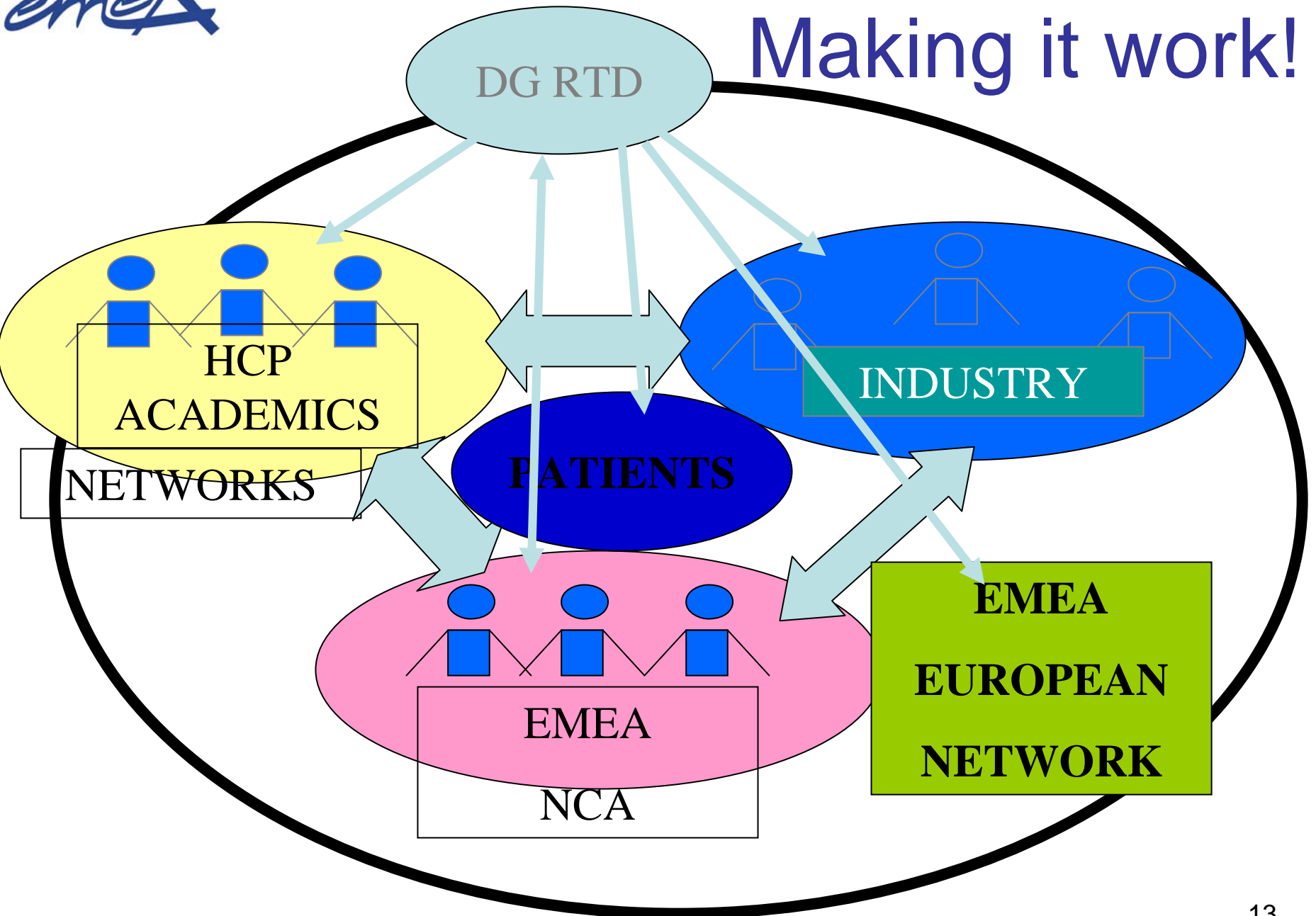
# Critical analysis

- No information on patent or authorisation
- Cut off value
- Favours frequent diseases
- Evidence for available treatment (no assessment)
- Products of interest may not be the old ones
- No analysis of need for formulations
- Method not appropriate for cancer products
- Areas where no therapeutic agents have been assessed are completely left out (e.g. rheumatology)

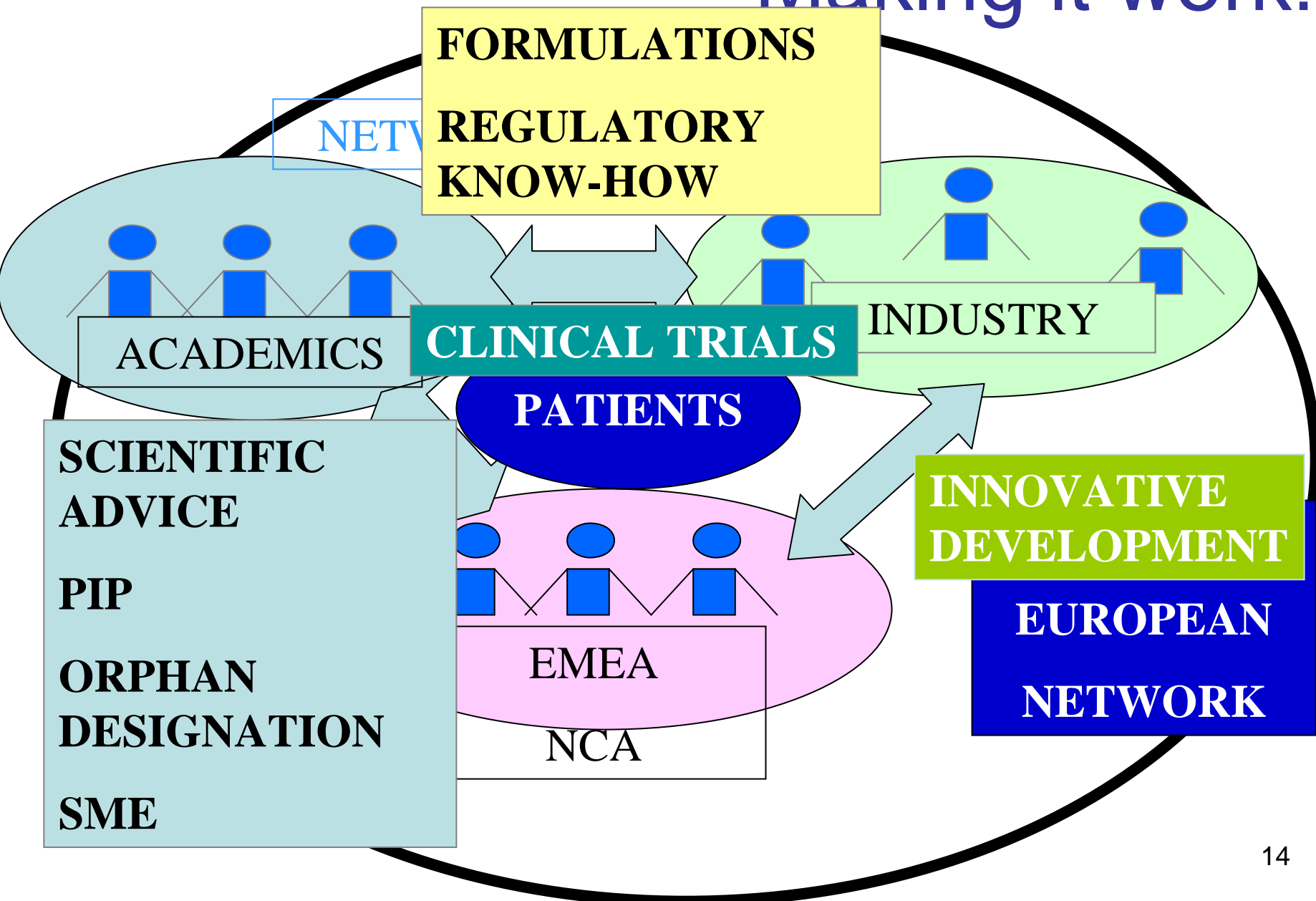
# First Conclusions

- Method not validated...
  - Expert agreement
  - Reasonable amount of products to be studied in the next years
- 
- New request once Regulation adopted
  - Update through PEG end of 2006
  - Publication for brief consultation

# Making it work!



# Making it work!



# Who does what?

- **European Commission:**
  - DG Enterprise: legislation and guidelines, delivers administrative approvals of drugs (marketing authorisation)
  - DG Research: funding of research on off patent medicines (and other health issues)
- **EMA; prepares assessment of PIPs and manages implementation of paediatric activities**
  - Co-ordinates Member States activities
  - EMA hosts the Paediatric Committee: scientific evaluation of PIPs
  - EMA hosts Committee for Human medicines, CHMP: scientific opinions on drug approval
- **Member States:** provide expertise, appoint members of the Committee, fund research at national level. Authorise at national level.