What could the development of medicines in ultra-rare indications look like ?

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Very simple thoughts

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- We are not here to draft the ‘special guideline on exceptional exceptions’

- What can the EMA offer?
  - *Haute couture* help. FLEXIBILITY ADPATABILITY DIALOGUE EXCHANGES
  - Tools. ITF, Scientific advices, PRIME, Orphan Designations
  - A TOOLBOX wide OPEN

- What can physicians and patients offer?
  - Experience, observations, science, real issues

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Wishlist

What do regulators need?

- Uncertainties on benefits and risks limited as much as possible
- Retrospective observations often not reliable for ‘comprehensive’ evidence
- Homogeneous prospective confirmatory data (benefits, risks, selection, markers, follow-up…)
- A common structure to build this ‘as aolis as possible’ evidence.