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Is the Orphan Regulation addressing patient needs?

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Content outline

- Introduction: Yes…but…
- 1- Is the Orphan Regulation Addressing Patients' Needs?
- 2- Facilitating Access to Orphan Drugs
- 3-Addressing Public Health Needs or Unmet Medical Needs of Rare Disease Patients
- Conclusion: Looking beyond the Orphan Regulation

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Is the Regulation Adressing Patients' Needs?

- **Is the implementation of the Regulation only driven by patients' needs?**

- **Can the Regulation do better to address patients' needs?**

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Doing better in the next 5 years

- **A 5 year programme on Orphan Drug at EMA-COMP at the cross-road of:**
  - The Orphan Regulation & COMP mandate
  - The EMA's Road Map to 2015
- **Stronger interactions:**
  - EU Policy & Commission & EU CERD
  - Dialogue with Stakeholders
  - International collaboration & FDA

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Facilitating Access to Orphan Drugs

- **Bridging the gap between the EU centralised procedure for ODD & MA and the national decision making-process to assess the drug value at the time of pricing & reimbursement: the Clinical Added Value of Orphan Drugs – CAVOD – and EMA-COMP**
- **Pay for value of medicines based on knowledge: conditional pricing & other innovative pricing approaches**
- **Better value for money: Benefit Management Plan, Effectiveness & Relative Effectiveness**

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Addressing Unmet Needs of Rare Disease Patients

- **Gaps in orphan drug development / reasons for discontinuation between ODD & MA**
- **Some rare disease therapies are not taking the route of orphan designation**
- **Situation of off-label use of medicines in rare indications**
- **Impact of off-patent for rare diseases**
- **5 000 rare diseases for which there are no treatements, no orphan drug**
- **New paradigme: continuing dialogue with sponsors + working from drivers**

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Looking beyond the current regulation…

- **Do we need to revise the EU Orphan Regulation?**
- **Do we need a new EU regulation to further and better address rare disease patient needs?**
- **Can we work in parallel between the EU and USA?**
- **…. To make it work better now!**

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