EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Early dialogue with regulators

Improving Multi-Stakeholder Early Dialogues to Optimise Determination of Value

3rd EURORDIS Multi-Stakeholder Symposium on Improving Patients' Access to Rare Disease Therapies

Presented by Kristina Larsson on 13 Feb 2019
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Benefits (with early regulatory interaction)

- Several early interaction possibilities (at repeated time points, and multiple stakeholders):
  - Scientific advice / Protocol assistance
  - Qualification of novel methodologies
  - PRIME, orphan designation, Innovation Task Force (ITF), Paediatric Investigation Plan (PIP)
- Patients are asked to participate in all protocol assistances.
- Planning: study design, relevant endpoints, best use of resources and patients, what is most relevant for patients
- Global development

Challenges

- Few patients:
  - Not always possible to find participants for advices
  - Short time lines
- Natural history of the diseases:
  - Endpoints: clinically relevant or pharmacodynamic / biomarker
  - Duration
  - Methodology
- Best time point to discuss the development plan with regulators (including post approval development)