

## Meeting minutes: JTC 2025 - Extended Information Webinar

**Date:** January 14, 2025

**Time:** 2:00 PM –4:00 PM

### 1. Introduction (Ralph Schuster)

#### Objective:

- Provide guidance on pre-proposal preparation and improve the quality of submissions.
- Focus on lessons learned from previous evaluations and available support services (mentoring, innovation management, regulatory advice).

#### Key Highlights:

- **Project Focus Areas:**
  - Preclinical therapy studies for rare diseases using small molecules and biologics.
  - Proposals must address at least two of the following:
    - Therapy development.
    - Validation of predictive and pharmacodynamic biomarkers.
    - Replication of findings.
    - Proof of concept for therapy readiness.
- **Considerations for Proposals:**
  - Engage experts early for regulatory alignment.
  - Demonstrate the robustness of models and analytical validation.
  - Integrate bioinformatics or statistical expertise into your team.
  - Ensure data complies with FAIR principles for reuse.
- **Deadlines & Workflow:**
  - Pre-proposals due: February 13, 2025.
  - Final decision on funded projects: December 2025.

### 2. Evaluation Insights (Christine Kinnon)

Christine shared her experience as an evaluator for EJPRD calls.

#### Key Takeaways:

- **Scientific Excellence:**
  - Proposals must have realistic expectations (e.g., not curing a disease in 3 years but advancing knowledge or capabilities).
  - Clear hypotheses and feasible methods are essential.
- **Relevance and Narrative:**
  - Ensure coherence; all work packages must contribute to the main objectives.
  - Avoid adding unrelated subprojects just to meet funding criteria.
- **Patient Benefit:**
  - Direct or eventual benefit to patients is critical.

- Engage patient organizations in the project planning phase.
- **Budget Realism:**
  - Budgets should reflect actual project needs. Overinflation is penalized.
- **Midterm Evaluations:**
  - A chance to troubleshoot and adapt with evaluator feedback.
  - Be transparent about challenges (e.g., delays due to recruitment or administrative hurdles).

### **3. Innovation Management Toolbox (IMT) & Mentoring Services (Agustin Arasanz Duque)**

#### **Innovation Management Toolbox (IMT):**

- A library with 550+ open-access resources covering project management, regulatory frameworks, intellectual property, and funding.
- **Recommended Mentoring Packages for Pre-Proposals:**
  - Project Management: Includes templates and manuals for structuring projects.
  - Innovation Management: Guidance on intellectual property strategy and engaging tech transfer offices.
  - Data Management: Templates for ensuring compliance with FAIR principles.

#### **Mentoring Services:**

- Available for full proposals (not pre-proposals).
- Includes tailored support on experimental design, regulatory advice, and project management.

### **4. Regulatory Support Services (Viviana Giannuzzi)**

#### **Key Points on Regulatory Processes:**

- **Orphan Drug Designation (ODD):**
  - Criteria include disease prevalence (<5/10,000 people in Europe) and significant patient benefit.
  - Benefits: Fee reductions, market exclusivity (10 years), and centralised marketing authorization.
- **Scientific Advice & Innovation Task Force:**
  - Offers free guidance for academic and SME projects.
  - Relevant for study design, protocol development, and biomarkers.
- **Paediatric Investigation Plans (PIP):**
  - Mandatory for medicines intended for children (e.g., specific age groups, formulations).
  - Early engagement with regulators is advised.

### **5. Methodological Support (Ralf-Dieter Hilgers)**

#### **Key Focus:**

- Ensuring rigorous design and analysis of preclinical studies.

- Address common pitfalls in animal studies (e.g., underpowered experiments, lack of statistical rigor).

**Recommendations:**

- **Sound Study Design:**
  - Clearly define research questions.
  - Use appropriate statistical hypotheses (e.g., equivalence, superiority).
- **Sample Size Justification:**
  - Ensure studies are adequately powered to yield valid and reproducible results.
- **Statistical Analysis:**
  - Predefine methodologies to prevent bias and ensure reliable conclusions.

## **6. Data Standards & FAIR Compliance (Marco Roos)**

**Key Points:**

- Emphasis on the FAIR principles (Findable, Accessible, Interoperable, Reusable).
- Proposals should briefly address data management, including:
  - Data to be collected/generated.
  - Commitment to contributing to the ERDERA data ecosystem.
  - Compliance with data-sharing standards.

**Recommendations:**

- Assign a data steward role in the project.
- Use ERDERA tools for standardizing data descriptions and access protocols.