Q/A: JTC 2025 - Extended Information Webinar

Date: January 14, 2025
Time: 2:00 PM –4:00 PM

General Proposal Preparation Questions (Ralph Schuster, Christine Kinnon):

- **Q: How should we integrate a project from a larger consortium?**
  A: Clearly separate the proposed project from the larger consortium's scope. Ensure there is no double funding and explain how the proposed work complements the larger initiative while remaining independent.
- **Q: What defines "scientific excellence"?**
  A: It's a combination of feasibility, innovation, and relevance to patient needs. The narrative coherence of the proposal matters, and track record plays a role but is not the sole deciding factor.
- **Q: How should early-career researchers (ECRs) be included in the consortium?**
  A: At least one ECR-led group is mandatory. However, projects should not include ECRs or subprojects solely to meet criteria; they must contribute meaningfully to the overall aims.
- **Q: Should we include patient organizations in the proposal?**
  A: Yes, patient organizations should be engaged from the planning phase to ensure relevance and alignment with patient needs. This is closely evaluated during proposal review. It is strongly encouraged, and roposals without a PAO must justify their exclusion, and patient involvement is assessed during evaluation.
- **Q: How do we address midterm evaluation challenges?**
  A: View midterm evaluations as an opportunity for feedback and problem-solving. Be transparent about issues (e.g., recruitment delays) and engage evaluators and peers identify solutions.

Innovation Management Toolbox (IMT) & Mentoring Services Questions (Agustin Arasanz Duque):

- **Q: Can the IMT toolbox help with writing pre-proposals?**
  A: The IMT provides templates, guidance, and resources to structure your proposal but doesn't directly assist in writing.
- **Q: When can mentoring services be accessed?**
  A: Mentoring services are available only during the full proposal stage (after shortlisting).
- **Q: Can we contact the IMT team directly for specific questions?**
  A: Yes, contact details will be shared after the webinar.

Regulatory and Ethical Requirements Questions (Viviana Giannuzzi):

- **Q: Is a Paediatric Investigation Plan (PIP) required at the pre-proposal stage?**
  A: No, but it's important to include a timeline for engaging with regulators on paediatric development if relevant.
- **Q: When are ethical approvals required?**
  A: Ethical approvals are not required at the pre-proposal or full proposal submission stages. However, they must be obtained before initiating relevant activities like animal experiments or clinical sample collection.

- **Q: Does ERDERA provide support for obtaining orphan drug designation or scientific advice?**
  A: Yes, ERDERA provides guidance on preparing applications for orphan drug designation, scientific advice, and regulatory engagement.
- **Q: How can discrepancies between regulatory timelines and project timelines be addressed?**
  A: While regulatory procedures can take time, including a plan to engage with regulatory agencies during the project can mitigate delays. ERDERA offers support to navigate these processes.

Methodological Design Questions (Ralf-Dieter Hilgers):

- **Q: How can the three Rs principle (Reduce, Refine, Replace) be applied to animal studies?**
  A: Design experiments to minimize animal use while ensuring scientific validity. Use sound biostatistical methodologies to ensure robust and reliable results with fewer experiments.
- **Q: Is statistical support mandatory for proposal submission?**
  A: While not mandatory, biostatistical input is strongly recommended to ensure robust experimental design, sample size justification, and reproducibility.

Data Management & FAIR Compliance Questions (Marco Roos):

- **Q: Can we mention that ERDERA's data services will help us manage our data?**
  A: Yes, but proposals must demonstrate your consortium's commitment to FAIR data practices and allocate resources for data stewardship.

  Additional Note:Yes, you can mention that ERDERA will provide support for data management. However, the proposal must demonstrate a commitment from your consortium, including budgeting for data stewardship.

- **Q: Should we allocate budget for data stewardship?**
  A: Yes, data stewardship requires time and resources, which should be reflected in your budget and project planning.
- **Q: How can animal data be made shareable and reusable?**
  A: Work with ERDERA's data services early to align data collection and sharing practices with FAIR principles. Contribute data to the ERDERA ecosystem for future reuse.
- **Q: Does "do it together" mean within the consortium or with ERDERA?**
  A: Primarily with ERDERA, though collaboration within your consortium on data management is also encouraged.

Additional Questions Across Speakers:

- **Q: How should we address administrative delays (e.g., recruitment, regulatory approvals)?**
  A: Include a realistic project management plan that accounts for potential delays. Engage with regulatory agencies early, and communicate regularly within the consortium to mitigate risks.

- **Q: Can IMT resources be used to assist in pre-proposal writing?**
  A: IMT resources (e.g., templates, guides) can help structure proposals but do not provide direct writing assistance.
- **Q: Are midterm evaluations mandatory, and how should we prepare?**
  A: Yes, they are mandatory and provide an opportunity for feedback. Be honest about challenges and prepared to adapt based on evaluator input.

Written Q/A:

General Questions About Eligibility & Proposal Scope:

- **Q: Can enterprises (SMEs or larger companies) participate in more than one initiative as non-funded partners?**
  A: Yes, there are no restrictions, but their involvement must be justified.
- **Q: Can the same patient organization participate in two different projects for this call?**
  A: Yes, but if central funding is involved, consult the funder (e.g., ZonMW) to confirm eligibility (see guidelines, p. 105).
- **Q: Should proposals address diseases with no EU-approved therapeutic options?**
  A: As 90–95% of rare diseases lack therapeutic options, these diseases should be in the focus of proposed work. However, it is still possible to apply if there is a high unmet medical need (e.g., current options have significant side effects or limited accessibility).
- **Q: What if a proposed drug has applications for non-rare diseases?**
  A: The focus must remain on rare diseases. Proposals can mention broader applications but cannot allocate project resources to common disease development.

Regulatory & Ethical Requirements:

- **Q: Can regulatory tasks (e.g., orphan drug designation) be managed by a pharmaceutical partner who is not seeking funding?**
  A: Yes, this is possible, but only funded partners count towards the required "4 partners from 4 countries" criterion. Be mindful of intellectual property (IPR) and conflict of interest.
- **Q: Is obtaining EMA scientific advice or orphan drug designation (ODD) mandatory for pre-proposals?**
  A: No, these are not mandatory. However, projects should outline plans to engage with regulatory authorities during the project. Note that EMA scientific advice is free for academic organizations.
- **Q: When are ethical approvals needed?**
  A: Ethical approvals must be obtained before starting activities like animal studies or patient data collection but are not required at the pre-proposal stage.

Methodology & Models:

- **Q: Are animal models mandatory, or can in vitro/human 3D models be used?**
  A: Animal models are not mandatory. Proposals can use alternative models (e.g., human organoids) if they are scientifically justified and ethically approved.

- **Q: Are large animal models acceptable?**
  A: Yes, large animal models can be used if scientifically justified and aligned with the project's objectives.

Data Management & FAIR Principles:

- **Q: Are ERN (European Reference Network) registries connected to the ecosystem?**
  A: Yes, several ERN registries are connected or are in the process of connecting to the ecosystem.
- **Q: Is there guidance on patient data collection without navigating every ethics committee in Europe?**
  A: Patient data protection and ethics regulations differ by country and institution. The proposal should consider these complexities and plan accordingly.

Consortium & Project Management:

- **Q: Can the project manager also be the coordinator?**
  A: Yes, in smaller projects, the roles can overlap. For larger, complex projects, it is better to separate these roles for efficiency.
- **Q: Would you encourage early-career researchers (ECRs) to act as project coordinators?**
  A: ECRs can coordinate if they have adequate support, but this may be challenging for first-time coordinators.
- **Q: Should a patient advocacy organization (PAO) be included in the consortium?**
  A: Yes, involvement of PAOs is strongly encouraged. Proposals without a PAO must justify their exclusion, and patient involvement is assessed during evaluation.

Innovation Management Toolbox (IMT) & Support Services:

- **Q: Can the IMT toolbox assist with pre-proposals?**
  A: Yes, the IMT is a helpful resource at any stage, offering templates, videos, and guides for project development.
- **Q: Can we collaborate directly with ERDERA experts (e.g., data services or regulatory group) as partners in our consortium?**
  A: ERDERA experts typically provide support services rather than act as partners. Specific collaborations should be discussed directly with ERDERA

Miscellaneous Questions:

- **Q: Are slides from the webinar available?**
  A: Yes, they will be uploaded to the ERDERA website.
- **Q: How important is preliminary data for pre-proposals?**
  A: Preliminary data is not mandatory but can strengthen your proposal by supporting your hypothesis and demonstrating feasibility.