Call for nominations: ERDERA Multistakeholder Advisory Group

What is ERDERA?

The European Rare Diseases Research Alliance (ERDERA) is a new partnership championed by the European Union and member states that aims to improve the health and well-being of over 30 million people living with a rare disease in Europe by advancing prevention, diagnosis and treatment research. This new partnership brings together over 170 organisations from 37 countries with a shared mission of making Europe a world leader in Rare Disease (RD) research and innovation and contribute to the provision of concrete health benefits to rare disease patients in Europe and beyond.

This Partnership will deliver an RD ecosystem that builds on the successes of previous programmes by supporting robust patient need-led research, developing new diagnostic methods and pathways, spearheading the digital transformational change towards connecting the dots between care, patient data and research, while ensuring strong alignment of strategies in RD research across countries and regions. Structuring goal-oriented public-private collaborations that are targeted at interventions all along the R&D value chain will ensure the route from knowledge to patient impact is expedited, thereby optimising EU innovation potential in RD. To support its ambition and missions ERDERA has been designed as a comprehensive and integrated ecosystem of which its structure can be compared to an institute encompassing three main parts: (i) funding, (ii) internal (in house) Clinical Research Network that implements research activities targeting clinical trial readiness of RDs and accelerating diagnosis and translation of research discovery into improved patient care, and (iii) related supporting services (Data, Expertise, Education and Training) as well as an acceleration hub that serves external and internal RD community, supported by effective coordination, strategic input and foundational (inter)national alignment.

The permanent Multistakeholder Advisory Board (MAB)

The permanent Multistakeholder Advisory Board (MAB) will be established to provide an objective overview of the progress in ERDERA and allow informed advice on the future directions. The MAB will support the Consortium, the Governing Board, and the Board of Funders by providing key advice that will feed into the strategy of the research and support activities foreseen in ERDERA. This may include suggestions on the future orientations of the Clinical Research Network, Joint Transnational Calls and Clinical Trial Calls, the exploitation strategies of research results, encompassing, for example, targeting of under-represented diseases, exploring meaningful yet under-researched topics, and guaranteeing high-quality, open, and re-usable results that translate into clinical impact. In addition, the MAB will advise

on how to better address patient needs as key drivers for funded research and support activities, with a focus on priorities that maximise the impact of the partnership.

MAB members and required expertise

To guarantee the agility and responsiveness, it is expected that the MAB, being the main advisory body of the ERDERA Partnership, will be composed of a maximum of 20-25 external (independent – see below potential conflict of interest section) experts representing different types of stakeholders and horizons. It is envisaged that all experts will have a broad and extensive understanding of the European and/or international rare diseases landscape. More specifically, ERDERA aims to engage advisory board members with the following expertise:

1. **Rare Disease Researchers:**
    - **Clinical Researchers and Geneticists**: Scientists with experience in rare disease research, including those with specific knowledge of clinical trials, genetic testing, and the molecular biology of rare diseases.
    - **Translational Scientists**: Researchers focused on translating basic scientific discoveries into clinical development.

2. Healthcare Professionals

- **Clinicians and Specialists in Rare Diseases**: Physicians, particularly those specialising in rare conditions that will provide insights into current clinical challenges and needs.
- **Patient Advocates**: Representatives from patient advocacy groups who can bring to the board the patient's perspective, ensuring that research is aligned with patient needs.

3. Public-Private Partnership and Industry Professionals

- **Pharmaceutical and Biotech Industry Representatives**: To foster public-private collaborations and provide acumen on drug development, commercialisation, and regulatory aspects.
- **Health Economists**: Experts to evaluate cost-effectiveness and value assessments of rare disease interventions.

4. Digital Health and Data Scientists

- **Bioinformaticians and Data Scientists**: With extensive experience in patient data processing, including, but not limited to, the analysis of large datasets, and expertise in integrating digital health solutions (into practice).
- **Digital Health Transformation Specialists**: Proficient at implementing digital technologies and platforms that connect patient care, data, and research.
- **AI and Machine Learning Engineers**: For developing advanced diagnostic tools and predictive models for rare diseases.

5. Regulatory, HTA and Policy Groups

- **Regulatory and HTA Affairs Specialists**: To navigate the complex regulatory landscape for rare disease research, clinical trials, and approval processes.
- **Health Policy Experts**: To ensure alignment with national and EU-level health policies, and to advocate for the integration of research outcomes into healthcare systems.

6. Funding and Grant Management Bodies

- **Investment and Financial Professionals**: For managing funding streams and advising on sustainable financing models.

7. Education and Training Personnel

- **Medical Education Professionals**: To develop training programs for clinicians, researchers, and other stakeholders in the RD ecosystem.
- **Training and Capacity Building Experts**: Focused on upskilling the workforce in clinical research, digital tools, and patient-centred care.

8. Strategic Coordinators and Program Managers

- **Program Managers**: Experienced in coordinating large, multi-stakeholder initiatives across different countries and sectors.
- **Strategic Planners and Alignment Specialists**: To ensure RD research aligns with the strategies across various regions and stakeholders.

9. Ethics and Patient Consent Groups

- **Bioethicists**: To address ethical considerations in rare disease research, particularly around data privacy, patient consent, and the use of genetic information.

10. Legal Experts in Healthcare and Researchers

- **Legal Advisors**: Expert officials in healthcare law, particularly in the areas of intellectual property, data protection, and clinical trial regulations.

MAB duties and planning

MAB members will be nominated for an initial mandate of 3 years that may be potentially renewed, depending on the evolving needs of ERDERA and on a necessary continuity within MAB proceedings.

The MAB will meet every four months, and at least once in-person during the first year of the project. Some topics of interest have been already pre-identified as they are considered of primary importance to advance on some of the activities of ERDERA, namely clinical trials and therapy development. However, when relevant, the consortium may require the assistance of MAB that lies beyond its core expertise. In such cases, it is possible for the MAB to initiate

Thematic Groups and invite additional experts to provide advice on the requisite topic(s). These Thematic Groups will always include some MAB members and will be chaired by one of the MAB permanent experts.

Potential Conflict of Interest:

According to the ERDERA Grant Agreement, beneficiaries must take all necessary measures to prevent any situation where the impartial and objective execution of the Agreement could be compromised due to family ties, personal relationships, political or national affinity, economic interests, or any other direct or indirect influence (referred to as a 'conflict of interest' - COI). This extends to COI identified for the composition and functioning of the MAB. Beneficiaries are required to promptly notify the coordinator of any situation that constitutes or could lead to a conflict of interest and take immediate action to redress the issue.

Rules for MAB Members:

MAB members must adhere to the following guidelines to avoid COI:

1. **Must not have any professional, financial, or personal connections** to ERDERA beneficiaries (a list of ERDERA partners is available here: https://erdera.org/participants/) **that could result in personal or professional gain** (e.g., the project involves immediate family members or partners).
2. Are not eligible to apply if they have **co-authored publications with ERDERA partners or their colleagues in the last three years, are currently collaborating with them or, if professional dependencies exist**.
3. MAB members **will be required to sign the "Conflict of Interest Disclosure Agreement."**

Applications:

To apply, please **send your CV and a Letter of Motivation** to mab-erdera@ejprarediseases.org **by October 31st, 2024, 6 PM CET**. If deemed useful, you might add other relevant documents to support your application, within the same message.

MAB selection process:

The selection process for MAB members will be based on a combination of several key criteria:

- Uniqueness and breadth of expertise
- Geographical diversity
- Gender balance
- Absence of conflicts of interest

The ERDERA Coordination Office will compile the list of applications and propose an initial pool of candidates to the ERDERA Executive Committee. If any specific expertise is found to be lacking, the ERDERA Executive Committee will be consulted for recommendations to address these gaps. The final list of pre-selected MAB members will then be submitted to the ERDERA Governing Board for validation.

30 September 2024