# Frequently asked questions 1

### Call Topic

- Is disease xyz considered a rare disease?
- Refer to definition "disease affecting not more than five in 10.000 persons in the European Community, EC associated states, and Canada", consult Orphanet or scientific literature for prevalence data
- Do projects need to address a group of diseases or can the also focus on a single disease?
  - Applicants are strongly encouraged to work on groups of rare diseases with commonalities and assemble criteria of meaningful grouping of the
    rare diseases under study, based on state-of-the-art scientific discoveries and clinical practice, to achieve higher global impact, but single rare
    diseases are possible as well with specification why working on a group of rare diseases is not appropriate.
- Is research on rare cancers eligible for funding?
  - No, rare cancers are excluded from the call. Please check TRANSCAN and EC calls (e.g Cancer Mission etc.) for funding opportunities.
- Are gene or cell therapy approaches eligible for funding?
  - No, therapy approaches that fall in the ATMP category are not eligible. These might be subject of a future call.
- Are therapy approaches like ASOs, siRNA, suppressor tRNAs eligible?
  - Yes, if the fall under the definition of "small non-coding chemically synthesized nucleic acid-based therapies".
- Do you fund validation or other forms of research involving intervention in human subjects?
  - No, only pre-clinical research is funded.
- "Projects should focus on rare diseases or disease groups with high unmet medical need, high disease burden, and no currently
  approved therapeutic options in Europe (European marketing authorisation)" can we also apply for therapies in diseases that do
  have approved therapeutic options?
  - · Yes, that is possible, but the high unmet medical need in comparision to already existing therapy options needs to be demonstrated.

**E DERA** European Rare Diseases Research Alliance

# Frequently asked questions 2

#### Call Topic

- Where can I find information on previously funded projects in similar calls?
  - Please visit the EJP RD Website: <a href="https://www.ejprarediseases.org/our-actions-and-services/funding-opportunities/funded-projects/">https://www.ejprarediseases.org/our-actions-and-services/funding-opportunities/funded-projects/</a> and <a href="https://tunded-projects.ejprarediseases.org/">https://tunded-projects/</a>

### Consortium

- Can research groups from countries not participating in the call (e.g. UK, USA, China etc.) receive funding?
  - No, they can only participate as collaborators with their own funding.
- · Is there a partnering platform for this call?
  - Unfortunately, there is no partnering platform available for this call. It may be offered for future calls.
- · Is there a restriction of how many proposals a research group can be part of?
  - There is no general restriction, however some national/regional restrictions may exist → please consult guidelines.
- What are the budget/eligibility criteria for the different national/regional funders/ PAO funding?
  - Please check guidelines and/or contact your local funder.
- · Could an industry/company be eligible as partner/subcontractor?
  - Please check guidelines and/or contact your local funder.
- · What is the maximum budget a consortium can apply for?
  - There is no maximal budget for the whole consortium but national/regional limitations may exist. Please check guidelines and/or contact your local funder.

# Frequently asked questions 3

### Patient Advocay Organisations (PAOs)

- Funding Opportunities for PAOs
  - Funding through the central funding mechanism:
    - The organisation should be formally established and registered as a not-for-profit organisation for more than 1 year in one of the following countries:
    - EU Member States and EU/EEA states: Austria, Belgium, Bulgaria, Croatia, Cyprus, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, Netherlands, Norway, Poland, Portugal, Romania, Slovakia, Slovenia, Spain, Sweden
    - ERDERA partner countries next to EU Member States and EU/EEA states: Georgia, Israel, Morocco, Serbia, Türkiye
  - If applicable: Direct funding through national/regional funding agencies: please contact your national/regional funding agencies
  - If applicable: Funding as a subcontractor: please contact your national/regional funding agencies
  - Please also check the guidelines for ZonMw, Netherlands

## Early Career Researcher (ECR)

- How many months can ECR candidates add to the cut off date of seven years for maternity/paternity leave?
  - As many as they actually took for the leave.
- What is the cut-off date for the ECR seven years qualification?
  - Submission deadline of pre-proposal, 13th February 2025.
- If the ECR is from an underrepresented country, could the number of partners be increased to 7?
  - Yes, as the mandatory ECR and an UR partner are included, it is possible to increase the number to 7.

