

**Call for Proposals 2025**

**"Pre-clinical therapy studies for rare diseases using small molecules and biologicals – development and validation”**

**Submission deadline for pre-proposals: February 13th, 2025 at 2 PM (CET)**

**Online pre-proposal submission form preview**

**THIS DOCUMENT ONLY PROVIDES INFORMATION ABOUT THE SUBMISSION PLATFORM AT** [**FUNDING.ERDERA.ORG**](file:///C:\Users\beatr\Downloads\funding.erdera.org)**. IT IS NOT INTENDED TO BE FILLED OUT. ONLY PROPOSALS THAT HAVE BEEN ENTERED ELECTRONICALLY AT** [**FUNDING.ERDERA.ORG**](file:///C:\Users\beatr\Downloads\funding.erdera.org) **WILL BE EVALUATED!**

**Checklist for the Coordinator**

***In order to make sure that your proposal will be eligible for this call, please collect the information required to tick all the sections below before starting to complete the application form in the online system at*** [***funding.erdera.org***](file:///C:\Users\beatr\Downloads\funding.erdera.org)***.***

* **General conditions:**

The project proposal addresses the **AIM/S** of the call

The project proposal meets the **TOPIC/S** included in this call

* **Ethical standards:**

The proposal complies with ethical principles (including the highest standards of research integrity — as set out, for instance, in the European Code of Conduct for Research Integrity — and including, in particular, avoiding fabrication, falsification, plagiarism or other research misconduct).

* **Composition of the consortium:**

The project proposal involves at least 4 eligible research partners from at least 4 different countries participating in the call.

The project proposal does not include more than two eligible research partners from the same partner country participating in the call (check out additional national limits that apply, in “Guidelines for Applicants”).

The consortium coordinator and the partners are eligible to receive funding from their national funding organisation(s) participating in the call.

The project proposal involves a maximum of 6 eligible research partners (including the coordinator) asking for funding, including one mandatory early career researcher (ECR). In case of inclusion of partners from participating underrepresented countries (Czech Republic, Estonia, Latvia, Lithuania, Slovakia, Türkiye) or additional early career researchers, the project involves a maximum of 8 eligible partners asking for funding.

* **Eligibility of consortium partners:**

I have checked that each research partner involved in the project proposal is eligible to receive funding by its funding agency.

I have checked that the applicants have confirmed the eligibility of the pre-proposal with their national/regional Contact Point.

I have checked that the mandatory early career researcher fulfills the eligibility criteria for an ECR.

(If applicable) Italian partners applying for funding at the Ministry of Health involved in the proposal have submitted a pre-submission eligibility check form to their national funding organization at least 10 working days before the submission deadline, through their IRCCS, using WorkFlowResearch System-> ER communication code. The pre-eligibility check form is available here: <https://www.salute.gov.it/imgs/C_17_pagineAree_4441_0_file.pdf>

(If applicable) Italian partners applying for funding at Tuscany Region involved in the proposal have submitted a pre-submission eligibility check form duly filled and signed by the Tuscan Principal Investigator and by the legal representative of the beneficiary to their regional funding organization ([erdera@regione.toscana.it](mailto:erdera@regione.toscana.it)) at least 10 days before the submission deadline.

(If applicable) Italian partners applying for Fondazione Telethon should submit a pre-submission eligibility check form to Fondazione Telethon at least 20 working days before the submission deadline.

(If applicable) Austrian partners have submitted administrative and financial data (in accordance with the FWF guidelines for stand-alone projects) online to the FWF at <https://elane.fwf.ac.at/>.

(If applicable) Hungarian partners have submitted mandatory information to NKFIH, including applicant name and affiliation, as well as an estimation of the requested budget.

(If applicable) Slovak partners have submitted a Letter of Commitment of the partner institute’s in-kind contribution (spoluucast) to SAS.

(If applicable) Swiss partners will submit the pre-proposal within 1 day to www.mySNF.ch together with the submission of the respective proposals to the ERDERA Joint Call Secretariat.

(If applicable) Turkish partners will submit the pre-proposal within 2 weeks, including the electronic signature process, through TUBITAK UIDB application system: <http://uidb-pbs.tubitak.gov.tr/>.

(If applicable) Israeli partners have submitted to CSO-MOH an [abstract and budget table](https://www.gov.il/he/service/era-net-instructions-for-israeli-researchers) and received approval for eligibility prior to the submission of the preproposal to ERDERA (in accordance with [CSO-MOH guidelines)](https://www.gov.il/he/service/era-net-instructions-for-israeli-researchers)

(If applicable) Luxembourgish partners must submit the pre-proposal as well as INTER documents to the FNR up to 7 working days after the submission deadline (see <https://www.fnr.lu/funding-instruments/inter/>).

(If applicable) Portuguese partners need to send the Statement of Commitment to FCT ([erdera@fct.pt](mailto:erdera@fct.pt)) up to 10 working days after the deadline for submission of pre-proposals.

(If applicable) Cypriot partners through the Coordinator of the Cypriot Consortium should also submit a pre-proposal and full proposal to the RIF at (https://iris.research.org.cy) up to 7 calendar days after the submission deadline of pre-proposals and full proposals, respectively.

(If applicable) Czech partners have submitted to AZVCR a Sworn Statement, Sworn Statement of composition consortium, and Application form for national eligibility check no later than the deadline for submission of pre-proposals to ERDERA ([VÝZVA 2025 – AZV ČR).](https://www.azvcr.cz/vyzva-2025/)

(If applicable) Belgian partners applying for funding to F.R.S.-FNRS have checked that they are in accordance with the eligibility rules and criteria, which can be found in the [PINT-Multi regulations](http://www.fnrs.be/docs/Reglement-et-documents/International/FRS-FNRS_PINT-Multi.pdf). It is strongly advised to contact the F.R.S.-FNRS prior to submission regarding the eligibility criteria. Applicants to F.R.S.-FNRS funding must provide basic administrative data by submitting an administrative application on [e-space](https://e-space.frs-fnrs.be/) within 5 working days after the general deadline of ERDERA call to be eligible. Please select the “PINT-MULTI” funding instrument when creating the administrative application.

(If applicable) (If applicable) Belgian partners applying for funding to SPW are requested to contact SPW at least 4 weeks before the submission deadline. Applicants have checked that they are in accordance with the eligibility rules and criteria which can be found in the SPW-Recherche website and the “Guidelines to applicants” edited by the partnership. Applicants to SPW funding must fill in the regional pre-proposal form on the regional application platform ONTIME. Proposals invited to the second stage must also be submitted on the same platform. The submission deadlines are the same as the general deadline of the ERDERA call.

(If applicable) Canadian partners will submit applicant information and the pre-proposal at the pre-proposal stage and the full proposal at the full proposal stage as per CIHR Funding Opportunity (link to follow).

**General Data Protection Regulation**

In the framework of this form, we collect Personal Data freely provided by the user including (but not limited to): name, email address, and any other details specifically asked in the form. ERDERA does not share personally identifiable information with unrelated Third Parties. However, we may disclose, transfer or share your Personal Data - anonymized or in its original format- with certain Third parties without further notice to you, only for reasons related to the purposes of this call.

**I agree with the following conditions:**

Information and Data protection conditions

The information of this form will be used for this purpose only and may be shared with the relevant parties of the ERDERA consortium, Scientific Evaluation Committee (SEC) members, and ethics experts. The title and abstract of this proposal, and names of the consortium members may also be shared with researchers from underrepresented/undersubscribed countries as part of the widening step (see Guidelines for Applicants). The information you should provide includes personal data referring to contact details, such as your name, email address and phone number. Personal data will be collected to allow contact for further details, if needed. No sensitive data will be collected.

All the collected data will be kept confidential and will not circulate beyond the ERDERA consortium, SEC members and ethics experts.

All the information will be made available in an aggregated manner (e.g. cumulative data and statistics).

The Joint Call Secretariat (JCS) will be responsible for the collection of personal data (see Privacy policy). The JCS will be responsible for processing the personal data.

**Declaration**

* **I have read the above information and:**

**I authorise the processing of personal data, in compliance with the European General Data Protection Regulation, Reg (EU) 2016/679 for the specific purpose they are collected (any communication of personal data to private or public subject will be allowed only for the specific purpose they are collected).**

**I authorise the use of my personal data to be contacted for widening activities.**

**I authorise the use of my personal data to be contacted by the ERDERA services program.**

**I authorise to be contacted for involvement in future collaborative initiatives, which might fall within the scope of my research activity.**

**I authorise to be contacted for dissemination and communication activities (e.g. newsletters, invitations to meetings).**

**General project data**

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| **Project title:** |  |

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| **Project acronym:** |  |

The application is:

a new proposal

a resubmission from a previous EJP RD call JTC 2019-2023

a proposal asking for an extension of a previously funded E-Rare or EJP RD project

If so, please state the acronym of the project:

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| **Duration of the project (max. 36 months)** |  | **Months** |

**Consortium Composition**

Proposal includes underrepresented countries yes no how many?

Proposal includes an early career researcher yes no how many?

**Disease area *(drop down menu)***

Cardiology / Vascular diseases

Dermatology

Dysmorphology

Endocrinology

Gastroenterology

Gynecology

Hematology / Immunology

Metabolic diseases

Musculoskeletal diseases

Nephrology / Urology

Neurology

Ophthalmology

Otolaryngology

Psychiatry / Psychology

Pulmonary / Respiratory diseases

Rheumatology

Others – Other Disease Area – please specify

**Diseases or Group of diseases (if applicable) with ORPHAcodes**

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**Prevalence of main diseases covered**

≤ 1:2.000  
≤ 1:10.000  
≤ 1:100.000  
≤ 1:1.000.000

unknown

**Main patient population involved**

Pediatric

Adult

Pediatric and adult

**Type of study**

1. development of novel therapies in a pre-clinical setting through cell, organoid and animal disease model studies, and/or use of in silico or artificial intelligence models to accelerate the success rate of the pre-clinical stage
2. development of predictive and pharmacodynamics biomarkers correlated to the efficiency of the therapy in a preclinical setting that could serve as surrogate endpoints
3. replication of pre-clinical studies in an independent lab to increase validity of exploratory findings
4. pre-clinical proof of concept studies for evidence of pharmacological activity in vitro and in vivo, pharmaco-kinetics and pharmaco-dynamics of the investigational drug (i.e., small molecule(s) and/or biologic) and first toxicology and safety data as well as studies to support readiness for initiating clinical trial authorization conforming to regulatory requirements

**Free Keywords**

*Please identify between three and seven keywords that represent the scientific content, approach (es), tools (animal models, OMICS, etc.), methodology*

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**Fixed Keywords as defined in the taxonomy of Horizon Europe**

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**Scientific abstract *(2.000 characters limit)***

Please note that if your proposal is selected for full proposal submission, this abstract may be communicated to researchers from underrepresented or undersubscribed countries as part of the widening process.

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**Lay summary *(2.000 characters limit)***

Please do mention the main goals. If funded, the summary is to be published in the internet. Please avoid abbreviations.

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**Potential experts suited for the evaluation of your proposal**

*Please provide the names, institutes, e-mail, address and expertise of up to 5 experts suited for the evaluation of your proposal. The proposed experts should not have an obvious conflict of interest, e.g. having collaborated closely or published together with one of the applicants in the last three years, being employed by the same institution or having a direct financial benefit. You can also provide the name of experts NOT SUITED for the evaluation of your proposal.*

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**Description of the project**

**Introduction and background (max. 4.500 characters)**

* Need for research rationale: description of the unmet need that is addressed by the proposed work, rationale of the rare diseases chosen.
* Present state of the art, recent insight from literature.
* Preliminary results obtained by the consortium members.

**Project description (max.13.500 characters)**

**Objectives and hypothesis**

Please highlight the objectives and main hypothesis(es) for the proposed research plan

**Soundness and pertinence**

* Innovative aspects, originality, novelty.

**Workplan and Methodology** *(highlighting feasibility)*

* Research strategy.
* Justification and description of methodology.
* Statistical power (if applicable): appropriate statistical methods description, sample size calculation, name and affiliation of the responsible biostatistics'/bioinformatics' expert.
* Description of the aims/work packages: synopsis and timeframe, including project coordination and management
* Responsibilities and workloads: For each research partner and collaborator: competence and experience in the field(s) of the proposal (previous work in the field, specific expertise); responsibilities in each work package;

**Impact** **(max. 1.500 characters)**

* Results: description of expected results and their implementation.
* Impact: description of the potential impact of the expected results on the addressed unmet need.
* Benefits: description of individual and collective benefits that could be expected.

**Added value of the consortium (max. 1.500 characters)**

* Competence, experience and complementarity of all the participants, benefit of transnational collaboration

**Patient Advocacy Organisations** (**PAOs) engagement/involvement (max. 2.000 characters)**

* Role of PAOs and patient partners within the consortium (active and meaningful participation in all stages of the proposal). (For more details see [Patients in research – EJP RD – European Joint Programme on Rare Diseases](https://www.ejprarediseases.org/wp-content/uploads/2021/03/SHORT-GUIDE-ON-PATIENT-PARTNERSHIPS-IN-RARE-DISEASE-RESEARCH-PROJECTS.pdf))

**Results of previous EJP RD or E-Rare funded project, complete only if applicable (max. 4.500 characters)**

* If the application builds on results obtained in a project or by a consortium funded in previous EJP RD or E-Rare calls, please include a description of the scientific results achieved in that project so far.

**Participant information**

**Consortium coordinator / Project Partners**

Title

Gender (Woman/Man/Non-Binary)

Date of birth

First Name

Last Name

ORCID

E-Mail

Phone

Early Career Researcher (yes/no)

**Early Career Researchers with PhD**

PhD: *indicate date of your PhD certificate*

**Medical doctors**

Medical specialist training:  *indicate date of your medical specialist certificate*

**Reasons for Extensions, if applicable**

Total parental leave: *indicate sum of all months*

Total other career break(s): *indicate sum of all months of other career breaks e.g., long-term sick leave, compulsory military service,* *family care leave*

Please describe your other career break(s):

**Career Stage**

Category A – Top grade researcher: the single highest grade/post at which research is normally conducted. Example: ‘Full professor’ or ‘Director of research’.

Category B – Senior researcher: Researchers working in positions not as senior as top position but more senior than newly qualified doctoral graduates (IsCED level 8). Examples: ‘associate professor’ or ‘senior researcher’ or ‘principal investigator’.

Category C – Recognised researcher: the first grade/post into which a newly qualified doctoral graduate would normally be recruited. Examples: ‘assistant professor’, ‘investigator’ or ‘post-doctoral fellow’.

Category D – First stage researcher: Either doctoral students at the IsCED level 8 who are engaged as researchers, or researchers working in posts that do not normally require a doctorate degree. Examples: ‘PhD students’ or ‘junior researchers’ (without a PhD).

**Partner from underrepresented country (yes/no)**

**Partner is ERN member** (yes/no, 🡪 if yes, please specify ERN (clickboxes with ERN names: Endo-ERN, ERKNet, ERN BOND, ERN CRANIO, ERN EpiCARE, ERN EURACAN, ERN eUROGEN, ERN EURO-NMD, ERN GENTURIS, ERN GUARD-HEART, ERN PaedCan, ERN RARE-LIVER, ERN ReCONNET, ERN RITA, ERN TRANSPLANT-CHILD, ERN-EuroBloodNet, ERN-EYE, ERN-ITHACA, ERN-LUNG, ERN-RND, ERN-Skin, ERNICA, MetabERN, VASCERN)

Institution/Department

Street

Town

Post code

Country

Phone

PIC number of the institution (EC Participant Identification Code)

Type of entity (Academia, Clinical, Public Health, SME, Industry)

Type of entity (public/private-for-profit/private-non-for-profit)

Funding organization to which you apply

**Patient advocacy organisation**

Title

Gender (Woman/Man/Non-Binary)

Date of birth

First Name

Last Name

E-Mail

ePAG member (yes/no, 🡪 if yes, please specify ERN (clickboxes with ERN names: Endo-ERN, ERKNet, ERN BOND, ERN CRANIO, ERN EpiCARE, ERN EURACAN, ERN eUROGEN, ERN EURO-NMD, ERN GENTURIS, ERN GUARD-HEART, ERN PaedCan, ERN RARE-LIVER, ERN ReCONNET, ERN RITA, ERN TRANSPLANT-CHILD, ERN-EuroBloodNet, ERN-EYE, ERN-ITHACA, ERN-LUNG, ERN-RND, ERN-Skin, ERNICA, MetabERN, VASCERN)

Institution/Department

Street

Town

Post code

Country

Phone

Type of entity (public/ private/ private-non-for-profit)

Requested funding (yes/no)

Funding organization to which you apply

**Collaborators (not funded):**

Title

Gender (Woman/Man/Non-Binary)

Date of birth

First Name

Last Name

ORCID

E-Mail

Collaborator is ERN member (yes/no)

specify ERN (Endo-ERN, ERKNet, ERN BOND, ERN CRANIO, ERN EpiCARE, ERN EURACAN, ERN eUROGEN, ERN EURO-NMD, ERN GENTURIS, ERN GUARD-HEART, ERN PaedCan, ERN RARE-LIVER, ERN ReCONNET, ERN RITA, ERN TRANSPLANT-CHILD, ERN-EuroBloodNet, ERN-EYE, ERN-ITHACA, ERN-LUNG, ERN-RND, ERN-Skin, ERNICA, MetabERN, VASCERN)

Institution/Department

Street

Town

Post code

Country

Phone

Type of entity (Academia, Clinical, Public Health, SME, Industry)

Type of entity (public/private-for-profit/private-non-for-profit)

**Narrative CV for each principal investigator and collaborator; for PAOs not required**

**Current position**

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**Personal Statement**

*Provide a personal statement that reflects on your overarching past, present, and future professional goals. This statement should reflect on the motivation for the scientific activities in which you have been involved, including for this project in particular.*

*max. 2.000 characters*

**Personal details - individual narrative profile**

*Describe your professional profile in narrative form, including key qualifications and relevant positions, in order for evaluators to assess your career stage and experiences that have shaped your development as a researcher and fit for this proposal. Your overall active research and professional experience will be considered, so you should mention significant life events, career breaks, secondments, volunteering, part-time work and other relevant events or experience (including time spent in different sectors) that has influenced your progression as a researcher and should be considered. In this way, your complete profile as a researcher can be understood and related to the present proposal. Please note that the focus of this section is not on output indicators, and personal details about life events should be limited to what is necessary for the understanding of how the event impacted your career. Feel free to highlight learnings/reflections you have gained from these experiences and how they have affected your career so far, as well as how they might be applied to the next stage of your career.*

*max. 3.500 characters*

**Key outputs, contributions, and achievements**

*List and describe your key output that demonstrates both your experience relevant to your proposal, as well as relevant to supporting the research community at large. This output should be complementary to what is in your ORCID profile, appropriate to your career stage, and should reflect what you consider most important to provide context for the evaluation of the submitted proposal. Your achievements described here can be from a wide range of sources based upon what you have achieved in your professional life thus far and should be described with enough detail that allows an evaluator to understand its importance, including the impact of what you have achieved. There is no requirement to provide output and achievements for every section; feel free to only add the sections that you feel support your application.*

*Please note that the aim of this section is to highlight outputs and achievements that provide relevant context for panel members, above and beyond your publication record and other achievements listed in your ORCID profile. You should avoid listing and describing solely items from your publication record and provide substantive evidence to back up your claims (if possible).*

*Please provide a description (with evidence if possible) of your output, contributions, and achievements.*

*Please provide a description (with evidence if possible) of your output, contributions, and achievements related to the following areas[[1]](#footnote-2):*

* ***Contributing to the generation and communication of new ideas, hypotheses, tools, or knowledge*** *– explaining how you have contributed to generating new ideas and hypotheses, as well as key skills you have used to develop ideas and test hypotheses. This encompasses how you have communicated your ideas and research results (written and verbally), as well as funding and awards that you have received. Relevant scientific outputs can be highlighted with a description of why they are relevant and considered in this context. Outputs can include datasets, software, publications, commercial/entrepreneurial/industrial products, educational products, clinical practice developments, policy publications, data management practices you have developed and/or implemented, and other similar items. Instead of focusing on journal names for publications, detail what the research findings were and how you contributed. If an output has a DOI/PMID/preprint link, please only include this. Scientific outputs should be examples of rigorous science following high standards, that are reproducible, and others can build upon (or negative results). Please indicate to what extent these outputs have been made openly available to the research community and to potential users of research outputs.*
* ***Contributing to the development and sustainability of research teams and individuals*** *– highlighting expertise critical to the success of other individuals, either within a team, part of a collaboration, or through mentorship. This can include project management, team support, teaching activities, and supervision. It can be used to mention support you provided to the advancement and recognition of colleagues (junior or senior), establishment of local/national/international collaborations (including interdisciplinary). Examples of strategic leadership, directing a team, organization, company, or institution are also relevant, as well as how you have contributed to diverse and inclusive research environments within your team/group.*
* ***Contributing to the wider research and innovation community*** *– engagement to progressing the local and international research community. This can include commitments including editing, reviewing, refereeing, committee/panel work and your contribution to the evaluation of researchers and research projects. It can highlight contributions to increasing research integrity and improving research culture beyond the group level (gender equality, diversity, mobility of researchers, and reward/recognition of researchers’ broad range of activities, open science initiatives). It can be used to mention appointments to positions of responsibility such as committee membership and corporate roles within your department, institution or organisation, and recognition by invitation within your sector.*
* ***Contributing to broader society*** *– emphasizing societal engagement and knowledge exchange. It can include engagement with industry and the private sector, as well as engagement with the public sector, clients, and the broader public. It can be used to highlight positive stakeholder feedback, inclusion of patients in processes and clinical trials, and other impacts across research, policy, practice, and business. It can be used to mention efforts to collaborate with particular societal or patient groups. It can be used to highlight efforts to advise policy-makers at local, national, or international level and provide information through the press and on social media.*

*max. 3.500 characters*

## Financial plan: budget requested by project partner

**Please describe the requested budget for this project partner only.** Eligible costs may vary according to funding agencies´ regulations and national/regional legal framework, and will be processed accordingly. The basis for calculating the grants for universities and research institutions is the eligible project-related expenditure, which can be funded up to a maximum of 100% depending on national/regional regulations. The basis for calculating the grants for commercial companies are the eligible project-related costs, up to 50% of which can usually be covered by government grants depending on national/regional regulations. The coordinator can apply for a specific budget for the management of the project if these are eligible costs according to national/regional legal framework and funding body regulations. These should be listed in the Project Coordinator budget. For a definition of eligible costs, see "Guidelines for Applicants" and contact your national/regional funding agency for more details.

Please use whole numbers only (e.g. 200000).

**Personnel €**

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Description/Justification

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**Consumables €**

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Description/Justification

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**Equipment €**

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Description/Justification

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**Travel €** (Travel expenses should include the participation to intermediate status symposium)

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Description/Justification

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**Other direct costs €** (e.g. subcontracting to PAOs, provisions, licensing fees; may not be eligible costs in all countries and will be handled according to national/regional regulations)

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Description/Justification

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**Overheads €** (Overhead costs and eligible expenses: funding according to national/regional legal framework and funding body regulations)

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Description/Justification

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**Total requested budget € (*automatic calculation*)**

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**Additional documents to upload**

**Diagram** **of the work plan**

Timeline, workflow and interconnections of work packages (Gantt chart, Pert or similar, max. 1 page)

**Diagrams, figures, tables etc. to support the work plan description** (max. 2 pages)

**List of references** (no page limit) *please use Vancouver Style (see: International Committee of Medical Journal Editors. Uniform Requirements for Manuscripts submitted to Biomedical Journals. NEJM 1997; 336:309-15) or Harvard referencing style (see:* [*https://www.mendeley.com/guides/harvard-citation-guide*](https://www.mendeley.com/guides/harvard-citation-guide)*) and include PUBMED, WoS or SCOPUS IDs*. Apply the chosen style consistently throughout the whole proposal.

**Date and signature page of all project partners asking for funding** (electronic signature or a scanned copy of the signature page will be accepted)

**Letter of intent for collaborators**

**Declaration of honor for PAOs**

1. [↑](#footnote-ref-2)