ERDERA Clinical Trial Call 2026

Information Webinar – July 6th, 2026

FAQ

1. Disease scope, rare cancers, rare infectious diseases and eligible interventions

1.1 Rare cancers, oncological diseases, tumour predisposition syndromes and borderline tumour conditions

Related attendee questions (verbatim):

- Is Myelodysplastic syndrome in children (MDS) considered out of scope since it is a clonal bone marrow stem cell disorder that can be considered a malignancy? Or can it be in scope because, if untreated, it can lead to AML, so you could also consider it as pre-malignant/ low-grade?
- Would a glioblastoma drug development be an eligible case for this programme?
- Did I understand correctly that rare cancers are excluded?
- If I understand correctly, oncological diseases, unless part of a genetic condition, are excluded?
- Dear ERDERA team, could you please elaborate on why rare cancers are excluded? (If I recall well, they were also excluded from the first ERDERA call)
- Could you please clarify whether tumors occurring in the context of rare diseases are eligible under this call? Specifically, would tumors arising in patients with rare genetic disorders or cancer predisposition syndromes be considered within scope?
- Just to clarify, rare cancers are excluded, despite having an Orphanet designation (i.e., classical Hodgkin lymphoma, listed as a rare disease in Orphanet registry, under number 391)?
- Dear ERDERA team, Thank you very much for this informative webinar. Could you clarify whether myeloproliferative neoplasms, particularly myelofibrosis and essential thrombocythaemia, are considered out of scope under the “rare cancers” exclusion, or whether they may be eligible as rare chronic haematological/myeloid diseases with high unmet medical need?

Response:

Rare cancers are explicitly excluded from the scope of the call, even where the disease may be rare and have an ORPHA code. The Call Text also states that tumour-predisposition syndromes and non-malignant or low-grade tumour manifestations within a rare genetic disease are not excluded and remain eligible.

For borderline situations, the applicant should carefully check the Orphanet classification and explain why the proposal should be considered within scope. Call Secretariat will not provide definitive pre-assessment of borderline disease eligibility; such cases will be assessed by the independent Clinical Trial Scientific Committee during evaluation.

Therefore, proposals primarily targeting a rare cancer should be considered out of scope. Proposals focused on a rare genetic disease with tumour manifestations may remain eligible if the proposal rationale is clearly positioned within the eligible rare disease scope.

1.2 Rare infectious diseases

Related attendee questions (verbatim):

- Is there another ERDERA call available for research in rare infectious diseases?

Response:

Rare infectious diseases are explicitly excluded from this Clinical Trial Call. There is no specific alternative ERDERA call for rare infectious diseases.

1.3 Repurposed drugs, repurposed biologics, ATMPs, combined ATMPs and antisense oligonucleotides

Related attendee questions (verbatim):

- Are repurposed drugs eligible?
- Can you please indicate whether repurposed drugs can be eligible?
- Are combined ATMPs for rare diseases eligible?
- Are antisense oligonucleotides in scope?

Response:

Repurposed drugs and repurposed biologics are eligible therapeutic interventions under the call. The Call Text includes specific requirements for repurposed drugs and repurposed biologics, including the need to address IMP supply, supply disruption risks, post-trial access and translation or implementation strategy.

ATMPs are also eligible, provided that the manufacturing process has been developed and validated under GMP conditions appropriate for Phase I/II use before Full Proposal submission. However, manufacturing costs up to and including GMP-compliant batch production for Phase I/II trials are eligible, including for ATMPs.

Combined ATMPs are not specifically excluded in the call text; eligibility would therefore depend on whether the proposed intervention and trial meet the full call requirements.

Antisense oligonucleotides are eligible and in the context of this call ASOs will be classified as “Antisense Oligonucleotide (ASO)” in the plateform.

1.4 Phase I, Phase I/II, Phase II, Phase II/III, First-in-Human and N-of-1 trial designs

Related attendee questions (verbatim):

- Dear all, Thank you for this very clear presentation. Are phase 2/3 clinical trials eligible?
- Phase I clinical trials are generally conducted with healthy subjects and generally are not multicentric/multi-country. This basically excludes Phase I trials... Some thoughts?
- Is it mandatory to include patients in the clinical trial or can ERDERA be applied for a clinical trial in healthy volunteers?
- Are N-of-1 trial designs eligible?

Response:

The call funds multinational, sponsor-led, GCP-compliant Phase I, Phase I/II and Phase II interventional clinical trials involving investigational medicinal products. **Phase III and Phase IV trials are explicitly out of scope**. Therefore, Phase II/III trials are not eligible if they include a Phase III component as part of the funded clinical trial.

First-in-Human trials are defined in the Call Text and are not excluded. Phase I studies are often conducted with healthy volunteers and may not always be multicentric. However, in rare diseases, Phase I studies may require multiple countries or sites to be feasible and to support capacity building. Applicants should justify the target population, eligibility criteria and development pathway, including how the trial remains coherent with the multinational objectives of the call.

N-of-1 designs are not specifically excluded. Their suitability will depend on scientific quality, methodology, feasibility and relevance to the proposed development pathway.

1.5 Preclinical, mechanistic, biomarker and laboratory activities within a clinical trial proposal

Related attendee questions (verbatim):

- Is it possible to have a pre-clinical phase in the project: for example, development of organoid models for the targeted disease?
- Is a preclinical phase, such as the development of an antisense oligonucleotide for a clinical trial, eligible as part of the project?
- Can the project include biomarker measurement during the trial and using some in vitro laboratory models to verify the source (cell types) of the biomarkers?
- Could a clinical trial of new drugs for a rare disease be accompanied, within the same proposal, by experimental evaluations aimed at generating mechanistic insights into the proposed drugs and the targeted disorder? Or would this risk being considered outside the scope of the call?
- It is clear that preclinical studies are not eligible. However, would studies using digital twins or blood–brain barrier models to evaluate whether a molecule can effectively cross the blood–brain barrier and reach the brain be considered eligible?
- Could a WP for the qualification of a diagnostic/patient stratification tool be financed as a means for inclusion criteria?

Response:

Non-clinical studies, including in vitro, in silico, animal studies and preclinical work, are explicitly out of scope. A proposal whose primary objective is preclinical development would therefore be ineligible.

Focus must remain the interventional clinical trial. Activities that are directly embedded in and necessary for the conduct of the clinical trial may be considered by reviewers, but additional experimental or mechanistic studies outside the clinical trial focus may risk being considered outside scope. The scientific committee will assess whether such activities are proportionate, justified and sufficiently linked to the trial objectives.

Applicants should therefore avoid presenting preclinical development as a funded work package unless it is clearly and directly linked to the clinical trial and compatible with the call scope.

2. Multinational requirement, sites, countries and consortium composition

2.1 Meaning of multinational clinical trial and minimum multinational requirement

Related attendee questions (verbatim):

- Could a single-country study be funded on an exceptional basis, or are only multinational studies eligible?
- What do you mean by multinational clinical trials? Do we need several sites in several countries?
- Thank you for this webinar. Is a preclinical phase, such as the development of antisense oligonucleotide for a clinical trial, eligible as part of the project? Can it be a single center in one country, or must it be multiple centers in several countries? Alternatively, is it possible to have a single center in Spain, for example, that admits patients from Europe? Would it be eligible?
- Can you clarify if the clinical trial must include clinical sites in at least 3 different countries? Can it be conducted at 1 clinical site in one country but include partners in different countries for the translational work etc?
- Does multinational clinical trials necessarily mean multicentric clinical trial?
- Can we recruit patients from one center only, but have different nationalities?

Response:

Only multinational/multicentric clinical trials will be funded. Each consortium must involve at least three independent eligible-for-funding partner institutions from at least three different ERDERA member countries. The multinational requirement is therefore a core eligibility condition, not only a recommendation.

ERDERA will fund only multinational, multicentric clinical trials. Certain operational scenarios may be acceptable when they are clearly justified, including cases involving ultra-rare diseases with a prevalence of 1:50,000, and/or advanced technologies that require treatment in a highly specialised centre. In such cases, applicants should explain the rationale for the proposed site configuration.

Applicants should therefore ensure that the proposal demonstrates genuine multinational added value, including in recruitment, expertise, trial delivery and impact. Site configuration should be justified scientifically and operationally.

2.2 Minimum and maximum number of countries, partners and centers

Related attendee questions (verbatim):

- Is there a maximum number of participating centers allowed?
- Which is the maximum number of Partners?
- Is there an optimal or preferred number of participating countries?
- Is there a minimum sample sizes for rare disease trials?

Response:

The minimum requirement is three eligible partners/sites/centers from three eligible ERDERA member countries. The call does not specify a maximum number of partners, centres, or countries, nor does it define a preferred number of participating countries.

Applicants are advised that the consortium and trial must remain manageable and justified. A larger consortium is not automatically advantageous: each partner or centre should have a clear role and added value.

No minimum sample size is specified. Applicants must justify sample size, recruitment feasibility and methodological appropriateness. These elements will be part of the scientific and feasibility evaluation.

2.3 Participation in multiple applications

Related attendee questions (verbatim):

- Is it possible to be the coordinator of two different ERDERA projects?
- Can a partner be involved in more than one application? E.g., ECRIN could be asked to be part of multiple applications.
- Can an organisation be part of more than one consortium/application in this call?
- Is it possible to apply as sponsor for one project and as a contributing partner for another?
- Are there restrictions for PIs based on specific funding institutions? Eg, already funded for other proposals?

Response:

There is no specific limitation preventing a partner, organisation, CTMO or coordinator from being involved in more than one application. Participation in multiple applications is possible.

However, ERDERA may cross-check involvement across applications. If an organisation or individual is involved in several applications, evaluators may consider whether the proposed workload and operational capacity are realistic. Applicants should therefore ensure that multiple participations are credible and adequately justified.

2.4 Eligible countries: Slovenia, Switzerland and non-ERDERA countries

Related attendee questions (verbatim):

- Please confirm if Slovenia is eligible- it was mentioned in the slides presented but not in the call-info online.
- Do I understand correctly that Switzerland-based universities and/or SMEs are excluded from the call, as Switzerland is not in the list of eligible countries?
- Can a trial be eligible if it fulfills the 'sites in at least 3 EU member countries' criteria and also has additional sites outside (e.g. US, China...)?
- Can we also have a clinic as part of the project in the US?

Response:

Slovenia is listed as an eligible country in the Call Text and was confirmed as eligible.

Switzerland is not listed among the eligible countries in the Call Text and therefore, Switzerland-based institutions are not eligible to receive ERDERA funding under this call.

Institutions from non-listed countries may participate only as self-funded collaborators. They must demonstrate clear scientific added value, secured funding for their participation and must not receive ECTC funding. They also cannot act as Sponsor, Coordinating Investigator, work package leader or beneficiary responsible for deliverables or milestones.

3. Sponsor, Coordinating Investigator, CTMO, CRO, collaborators and subcontractors

3.1 Sponsor eligibility and location

Related attendee questions (verbatim):

- Can a sponsor be based in the UK - can a UK SME act as the Clinical Trial Sponsor?
- Can the sponsor be from Israel? Does the sponsor have to be from a participating country? Can he be localized in the US? Can the sponsor also be the coordinator of the trial?
- Can an ineligible-country organisation remain Sponsor while another entity receives funding?
- What is the position with UK?
- Can UK be a partner?

Response:

The legal Sponsor must be established in an EU Member State or Norway. Consequently, an organisation established only in the United Kingdom, Israel, the United States, Canada or another non-EU/non-Norway jurisdiction cannot act as the legal Sponsor under this call.

UK organisations may participate as eligible partners because the United Kingdom is listed as an eligible country for participation and funding; however, UK organisations cannot act as the Sponsor because the Sponsor must be established in an EU Member State or Norway.

The Sponsor may also be the Coordinating Investigator when the eligibility rules are fulfilled.

3.2 Roles of Sponsor, Coordinating Investigator, company and CRO

Related attendee questions (verbatim):

- Can the Sponsor also be the Coordinating Investigator?

- Can a company be the Coordinating Investigator?
- Can a CRO be the Coordinating Investigator?

Response:

The Sponsor and the Coordinating Investigator may belong to the same institution or to different consortium partners. The Coordinating Investigator must come from EU countries, Norway, the United Kingdom or Canada. If the Sponsor and Coordinating Investigator are the same institution, that institution must be established in an EU Member State or Norway.

A company may act as Coordinating Investigator if it is eligible and fulfils the relevant requirements. A CRO acting in this role is considered possible if it fits within the eligible applicant categories and role requirements.

In all cases, the proposal must describe the division of responsibilities and demonstrate adequate scientific, operational and regulatory capacity.

3.3 CTMO requirement, CRO use and procurement model

Related attendee questions (verbatim):

- Do you have a list of CTMO?
- Is it recommended to include a CRO in the consortium or can it be subcontracted?
- Can we use a commercial clinical research organization (e.g, IQVIA)?
- Can you clarify how a commercial clinical research organisation *could* be included in a proposal, and what roles they can/cannot have?
- A CTMO is mandatory. However, many CTMOs are private, for-profit companies that are not SMEs. How can these large CTMOs be included as part of the minimum consortium?
- Does ERDERA maintain a roster of pre-vetted CTMOs consortia can use, or do we procure one independently?
- Will ERDERA provide a matchmaking platform, partner-search tool, or service-provider list to help applicants identify eligible partners, CTMOs/CROs, patient organisations, or clinical sites?
- Can CROs be a partner as well?
- Can commercial CRO be used? Should CRO be a consortium partner or subcontractor?

Response:

A qualified multinational CTMO is mandatory. Each consortium must either include such an organisation or demonstrate a formal access arrangement with one. The CTMO must be identified in the Full Proposal; applicants are strongly encouraged to engage the CTMO at the earliest possible stage and no later than Stage 2.

ERDERA does not provide a pre-approved CTMO list and does not want to prescribe the use of a specific organisation. Examples mentioned in the Call Text, such as ECRIN, c4c or CVBF, are non-exhaustive examples only.

Commercial CROs/CTMOs can be used. Depending on the role, they may be included as full consortium partners or subcontractors/service providers. A CTMO providing trial management support does not count toward the minimum three-country consortium composition unless it participates as a full consortium partner contributing directly to the scientific conduct and implementation of the trial.

Large private for-profit companies other than SMEs are not eligible to receive ERDERA funding as beneficiaries or funded partners. They may nonetheless provide commercial services through procurement/subcontracting arrangements where allowed and justified. Core Sponsor and Coordinating Investigator responsibilities cannot be subcontracted.

3.4 Same entity as Sponsor and CTMO; co-sponsorship and sponsor changes

Related attendee questions (verbatim):

- Can the same non-profit academic research organisation act as both the Clinical Trial Sponsor and the qualified multinational CTMO, provided it has the appropriate infrastructure and expertise, or are these expected to be separate entities?
- Is co-sponsorship allowed, as defined under Regulation EU 2014/536?
- Can a sponsor be changed during the grant or application process? E.g. right now, the sponsor of our trial is non-profit, but if we need to attract investors to get more funding, we might need to open a company, small SME, founded by non-profit. Will that be possible to change during the call or grant?

Response:

The Call Text states that the CTMO does not assume the legal responsibilities of the Sponsor unless separately designated as Sponsor. At Short Proposal stage the organization will be asked to provide a track record of showing the ability to manage clinical trials.

Co-sponsorship under Regulation (EU) No 536/2014, is permitted, provided that the main legal Sponsor for the call is based in EU or Norway. The co-sponsorship must be regulated by an agreement between the two parties that will be evaluated by the CTSC in Stage 1.

Regarding sponsor changes, ERDERA indicated that the proposal should be well structured at submission. A change of Sponsor during the application or grant process could be complicated and would need careful assessment, particularly because Sponsor capacity and eligibility are central to evaluation and contracting.

3.5 Collaborators, subcontractors, clinical sites and pharma in-kind participation

Related attendee questions (verbatim):

- Can you give an example of what could cover a collaborator? It is clear from the call text what it cannot be, but not so clear what it can be/do.
- Can a pharma industry partner be part of the consortium?
- Can pharmaceutical industry (not SME) contribute in-kind for trials that would not be feasible otherwise for financial reasons?
- The current budget allocation may not fully account for the cost of the drugs being administered in this trial, which are quite expensive. Could you speak to how in-kind contributions - such as drug supply directly from manufacturers or partner institutions -might be factored in to offset costs? And separately, are there additional funding avenues being explored, whether through supplemental grants, sponsor top-ups, or partnerships with pharmaceutical companies, to ensure the full amount required is actually secured?

Response:

Collaborators are self-funded participants. They may contribute to the project if they add clear scientific value, have secured their own funding and submit a signed letter of commitment. They cannot act as Sponsor, Coordinating Investigator, work package leader or beneficiary responsible for deliverables or milestones.

Private for-profit companies other than SMEs are not eligible to receive ERDERA funding as beneficiaries or funded partners. However, they may participate as self-funded collaborators or provide in-kind contributions where justified and compatible with the project. Pharmaceutical industry in-kind contribution is confirmed as possible.

Applicants should describe and justify any in-kind contribution, including drug supply, and ensure that the proposed project remains feasible within the requested ERDERA budget and any confirmed additional resources.

4. GMP, manufacturing, IMP supply and production location

4.1 GMP readiness and timing

Related attendee questions (verbatim):

- Is it mandatory to have the bioproduction process validated to initiate a Phase I/II clinical trial?
- Regarding the Advanced Therapy Medicinal Products, at what time the applicant need to prove they have the GMP-validated manufacture of the product? Should it be at the time of the full application or before? Will be eligible a project that in the Letter of intent and/or in the brief proposal phase is in process of developing the GMP manufacturing protocol to be concluded at the time of full application? What "validated" stands for in the "GMP-validated" requirement?
- Considering a start in 2028 - At what stage of the application process must GMP product development be completed?
- Is it possible to apply with a therapy that does not count with GMP production right now, but it will do in 6 months?

Response:

The Call Text states that ATMPs are eligible provided the manufacturing process has been developed and validated under GMP conditions appropriate for Phase I/II clinical use. Applicants must also demonstrate access to the investigational medicinal product (IMP) and appropriate manufacturing and supply arrangements to support the proposed clinical trial.

It is not expected that all manufacturing-related activities will be fully completed at the Expression of Interest (EOI) or Short Proposal stage. However, applicants should demonstrate a clear, credible and realistic development plan showing progressive readiness throughout the application process.

Specifically:

- **EOI:** applicants should demonstrate the feasibility of the proposed manufacturing strategy and provide an indicative development roadmap.
- **Short Proposal:** applicants should provide evidence of progress towards GMP manufacture, including the planned manufacturing process, timeline and regulatory readiness.
- **Full Proposal:** applicants should demonstrate that the manufacturing process has been established under GMP conditions appropriate for a Phase I/II clinical trial and that robust manufacturing and supply arrangements will be in place to support trial initiation. This should include evidence that GMP manufacture is expected to be available before the planned start of the clinical trial and that the product can be released in accordance with applicable EU GMP requirements.

Therefore, projects that are still developing their GMP manufacturing process at the EOI or Short Proposal stage remain eligible, provided they present a realistic and credible plan demonstrating that GMP-compliant manufacture will be achieved before the clinical trial starts.

4.2 Manufacturing location, US manufacturing, PPQ and commercial-use manufacturing costs

Related attendee questions (verbatim):

- Does manufacturing of the drug / IP have to be in one of the funded countries or could this be outside, e.g. US?
- Would the call cover PPQ and manufacturing costs for commercial use of the IP?

- For cGMP manufacture, we practice dual assessment and validation to ensure that geographic location does not influence manufacture, and then we test in duplicate in different centres. The second location for manufacturing is in the USA. The amount produced is often small: Would the small scale duplicate manufacture in the USA for QA/QC purposes be considered an eligible cost?
- Can the project include the setup of a production center to distribute the medicinal product to different countries?
- Can the ATMP production be centralized? In the case of centralized production, the hematopoietic harvest can be shipped to the site of ATMP production without moving the patient.

Response:

The Call Text states that eligible investigational medicinal product (IMP) costs include manufacturing activities necessary to support the proposed Phase I/II clinical trial, including GMP-compliant manufacturing of the clinical trial batches. Costs related exclusively to commercial manufacturing, commercial supply or Phase III/commercial-scale production are not eligible.

Manufacturing does **not** necessarily need to take place in one of the participating countries, provided applicants can demonstrate that the proposed manufacturing arrangements comply with all applicable regulatory requirements, are compatible with the national eligibility rules of the participating funding organisations, and ensure timely and reliable supply of the IMP for the clinical trial.

Activities such as Process Performance Qualification (PPQ), additional quality control or duplicate manufacturing may be considered eligible **only where they are necessary and adequately justified for the manufacture and release of the clinical trial IMP**. Activities undertaken solely to establish or validate commercial manufacturing processes, support future commercial production or supply commercial products would not be eligible.

Similarly, projects may include centralised manufacturing and distribution of ATMPs where this represents the most appropriate manufacturing strategy. Applicants should demonstrate the feasibility of the proposed approach, including manufacturing capacity, logistics, product transport, regulatory compliance and the ability to administer the investigational product safely across all participating clinical sites.

Applicants should clearly distinguish activities required to manufacture and supply the IMP for the proposed Phase I/II clinical trial from activities intended to support future commercial manufacture or market supply.

4.3 Repurposed-product supply and post-trial access

Related attendee questions (verbatim):

- What standard of evidence must we obtain or verify regarding the efficacy of a treatment on a key mechanism of the disease before submitting a proposal for a therapeutic trial?
- What if the applicant already has a trial approved? Can the funding of ERDERA be added to already approved trial?
- A clinical trial that is already funded by a national organisation in a monocentric way can be eligible to extend the investigation centers and become multinational?

Response:

The Call Text requires the Short Proposal to demonstrate scientific merit, feasibility and development potential, including the quality of preliminary evidence supporting the study rationale and objectives. Full Proposals must demonstrate robustness of the scientific evidence supporting the medical need, rationale, IMP and sample size justification.

For repurposed drugs/biologics, applicants must address uninterrupted IMP supply, contingency planning, post-trial access and implementation strategy. These are important because third-party suppliers or marketing authorisation holders may affect trial feasibility and later patient access.

Regarding already approved or funded trials, expansion of an existing trial into a multinational clinical trial may be possible if the proposal submitted is genuinely a multinational clinical trial and is fairly evaluated through the submission process. Simply adding ERDERA funding to an already approved trial without a distinct eligible proposal will be evaluated.

5. Budget, funding model, co-financing, regulatory costs and data service costs

5.1 Co-financing and funding rates

Related attendee questions (verbatim):

- Is co-financing required, or is this 100% funding?
- If the CTMO is a SME and is eligible to have 60-70% of their budget covered by ERDERA, as these are still for-profit companies, how does the Sponsor offer them a motivation to run their core commercial business where they have to cover 30-40% of their own costs?
- What role is expected for SMEs in these types of consortia? Is there a range of the potential budget that could be allocated to SMEs within the overall consortium?

Response:

There is no national or regional co-funding requirement for eligible non-SME partners funded through the ERDERA central model. The Call Text states that the full requested budget, within eligible limits, will be provided centrally through FTELE for partners from ERDERA full member countries.

SMEs are treated differently because State aid rules apply. Small enterprises may receive up to 70% of eligible costs and medium-sized enterprises up to 60%; the remaining costs must be covered by the SME's own resources or compatible funding sources.

If a CTMO is an SME and participates as a funded partner, SME funding rates apply. If the CTMO is procured as a subcontractor/service provider under an eligible partner's budget, the arrangement is different and should follow procurement and value-for-money principles. Using the CTMO as a subcontractor may avoid the SME co-financing issue in some cases.

There is no predefined budget share for SMEs. SMEs may participate as eligible-for-funding consortium partners or Sponsors if eligible and able to fulfil their role.

5.2 Additional funding, in-kind contribution and expensive IMP costs

Related attendee questions (verbatim):

- What about completing the CT with additional funding not from ERDERA?
- The current budget allocation may not fully account for the cost of the drugs being administered in this trial, which are quite expensive. Could you speak to how in-kind contributions - such as drug supply directly from manufacturers or partner institutions -might be factored in to offset costs? And separately, are there additional funding avenues being explored, whether through supplemental grants, sponsor top-ups, or partnerships with pharmaceutical companies, to ensure the full amount required is actually secured? Can pharmaceutical industry contribute in-kind? For a rare disease trial, drug costs alone could be a major barrier to completion if this isn't addressed early

- Can pharmaceutical industry (not SME) contribute in-kind for trials that would not be feasible otherwise for financial reasons?

Response:

Additional funding and in-kind contributions may be included where they are transparent, secured and compatible with the project. Applicants must avoid double funding of the same eligible costs and must declare other public funding, grants, subsidies or aid requested or received for the same activities.

In-kind contribution by pharmaceutical industry is possible. However, the project must still be financially credible. Applicants should show that the full trial can be delivered with the ERDERA budget plus any confirmed external or in-kind contributions.

The expected ERDERA budget per project is generally between €1 million and €5 million, although projects outside this range may be eligible with adequate justification. Applicants should not assume that the full €30 million call envelope can be allocated to one trial.

5.3 Regulatory costs, EMA advice costs and Horizon Europe cost principles

Related attendee questions (verbatim):

- Does the funded budget cover regulatory-affairs activities, or only trial conduct costs?
- Can you kindly confirm whether the eligibility criteria applying to costs are aligned with the rules applicable under Horizon Europe (including the 25% flat rate for indirect costs)?

Response:

Regulatory costs are eligible where they are part of the funded project. The Call Text explicitly lists ethics committee submissions and Clinical Trial Authorisation fees as eligible costs. EMA scientific advice fees may also be eligible for applicants who do not qualify for an EMA fee waiver; applicants who qualify for a waiver are expected to apply for it, and the corresponding costs will not be eligible for ECTC funding.

The Call Text states that financial rules derive from the ERDERA Grant Agreement with the European Commission and Horizon Europe rules are operationalised by FTELE. During the webinar, alignment with Horizon Europe principles was confirmed, including in relation to indirect costs, although the applicable rate will be defined in the Award Agreement with FTELE.

Applicants are encouraged to engage early with relevant regulatory authorities, including ODD, EMA Scientific Advice, Protocol Assistance, ITF briefing meetings and national competent authority advice.

5.4 Data collection and management service

Related attendee questions (verbatim):

- Could you provide information regarding data ownership (using ERDERA's central data collection and management service), e.g. with regard to future use of data in market authorisation processes
- Could you please clarify which services are included in the ERDERA Data Services Hub? The information currently available on the website is not sufficiently detailed, and we would appreciate a clearer overview of the services provided.

Response:

During the webinar, it was clarified that the ERDERA Data Services Hub is not the same as the central data collection and management service for the Clinical Trial Call. The latter refers to a specific clinical trial data-management provider or service for the data-management part of the clinical trial.

Regarding data ownership, data is normally owned by the project: the use of a central service provider does not mean that ERDERA owns the clinical trial data. Applicants may use trial data for further purposes such as regulatory pathways, subject to the project's governance, legal, data protection and regulatory arrangements.

5.5 Payments, milestones and timing of funds

Related attendee questions (verbatim):

- Funding decisions February 2028: when is expected for the participants to receive the funds?
- What are the timelines appr. start/end date of the trail that will be accepted?

Response:

The indicative timeline provides notification of funding decisions in February 2028. Funding will be provided through an Award Agreement between FTELE and the Sponsor institution, and disbursed in milestone-linked instalments.

The Call Text lists typical milestones: regulatory and ethics approvals, First Participant First Visit, Last Participant First Visit, Last Participant Last Visit, clinical study report and entry of study results in the clinical trial registry. The exact start date after the funding decision will depend on ethics approval and CTIS submission (first milestone), and therefore, no fixed month can be guaranteed.

Stage 4 is expected to last approximately 2–3 years. Stage 5, if approved after Go/No-Go review, is also expected to last approximately 2–3 years, with Stages 4 and 5 combined not exceeding seven years.

6. Regulatory interactions, ODD, EMA advice, Stage 2 support and evaluation

6.1 ODD, EMA and national scientific advice

Related attendee questions (verbatim):

- May we apply for ODD and EMA protocol assistance during the project development phase?
- During stage 2, a scientific advice should be completed including feedback from the national agency/EMA?
- Do proposed IMPs need to have ODD status to proceed through the application process?

Response:

ODD is not mandatory at the start of the application process. However, applicants are strongly encouraged to have obtained, applied for or initiated preparations for ODD where relevant.

Stage 2 provides structured regulatory support, including support for the preparation of an EMA Scientific Advice briefing package. The full EMA procedure remains external to the PSS and follows EMA timelines. EMA or national competent authority advice may not always be fully completed within the Stage 2 timeline, but feedback obtained, including draft or partial feedback, should be used to strengthen the Full Proposal where possible.

For Phase I studies, Mark Turner, coordinator of the PSS, noted that advice from national competent authorities is also important because Phase I trials are approved at the national level. Applicants are encouraged to seek advice in multiple directions where appropriate.

6.2 EOI, Short Proposal, templates, page limits and eligibility of all EOIs

Related attendee questions (verbatim):

- Will all eligible EOI be invited to proceed to short proposal submission?

- Will templates be available for the EOI and short and full proposals? Any limits for the number of pages?
- What is the portal/tool used to submit EOI in Stage 0? And should the EOI be submitted by the Coordinating Investigator on behalf of the consortium?

Response:

The EOI is a mandatory gating step but not a selection stage. Submission of an EOI is required to proceed to the Short Proposal stage (i.e. all the eligible EOI are invited to proceed to a Short Proposal submission), but submitting an EOI does not oblige the applicant to submit a Short Proposal and does not itself constitute a funding application.

The EOI must be submitted through the Fondazione Telethon electronic submission system (link) by the Coordinating Investigator, who is identified as the Lead Applicant in the system. The EOI Guidelines specify the sections required in the EOI form, including project data, project description, Coordinating Investigator/Sponsor, partners, estimated budget, notes/supporting documents and declarations.

Templates and page limits for Short and Full Proposals will be made available. The EOI form is already available among the Call Documents on the ERDERA website (https://erdera.org/call/ctc2026/).

6.3 Interview, rebuttal and number of applications selected for Stage 2

Related attendee questions (verbatim):

- Regarding the interview phase, will the interview be done only with the PI of the project [Ed. Note: the Coordinating Investigator] or the whole team (PI research team) would be invited?
- How many applications are you envisioning to select for the Stage 2? How many applications can the support team handle? There must be a limit based on the quality of the service provided and the involvement of several experts.

Response:

The Call Text states that the Coordinating Investigator and a designated representative of the Sponsor institution will participate in the interview. The interview is conducted by a panel of three CTSC experts, with the Scientific Secretariat attending as observer. If the Coordinating Investigator and Sponsor are represented by the same person/entity, the composition may be adjusted accordingly.

The interview assesses the consortium's readiness to implement the trial, including experience, Sponsor oversight, CTMO arrangements, regulatory, pharmacovigilance, monitoring, quality management, risk mitigation and governance structures.

During the webinar, it was indicated that approximately 20 applications are expected to enter Stage 2, depending on Short Proposal evaluation.

6.4 Stage 4 / Stage 5 model and Go/No-Go criteria

Related attendee questions (verbatim):

- I don't fully understand how the Stage 4 / Stage 5 project design works. What kind of go/no-go are expected here? Do the proposal and the budget need to stop at stage 4 or include stage 5 activities? If Stage 5 activities are included in the proposal, do we need to identify and separate Stage 5 activities from the other activities in the budget construction?

Proposed response:

Stage 4 covers the initial trial execution phase and aims to generate early evidence of safety, feasibility and preliminary clinical activity as appropriate for the development stage. The implementation plan will include predefined milestones and Go/No-Go criteria agreed upon during grant preparation.

Stage 5 is conditional continuation funding. At the conclusion of Stage 4, the CTSC will review results against the pre-agreed Go/No-Go criteria. These may include safety, feasibility, recruitment performance, pharmacodynamic or biomarker evidence, preliminary efficacy signals or other study-specific endpoints appropriate to the development stage and modality.

The Call Text states that Stage 5, if approved, will be implemented through an amendment to the original Award Agreement, defining scope, budget, implementation plan and milestones. More detailed guidance would be provided separately.

6.5 Redress and scientific judgement

Related attendee questions (verbatim):

- No direct attendee question in the Q&A list, but relevant for FAQ completeness.

Response:

Applicants may request redress only for procedural irregularity or factual error affecting an eligibility check or evaluation outcome. Disagreement with scientific, technical, methodological, ethical, regulatory or strategic evaluation by independent experts is not a ground for redress.

Redress must be submitted by the Coordinating Investigator within seven calendar days of notification of the relevant outcome and must identify the call, proposal acronym, proposal title and alleged procedural irregularity or factual error.

7. Patient involvement, PAOs, PPIE and patient participation

7.1 Mandatory PAO / patient partner role and funding

Related attendee questions (verbatim):

- Could you please provide specific examples of qualified involvement of patient organization in the call?
- A PAO is mandatory and there is a very strong emphasis on PPIE. However, PAOs work on PPIE is not funded by call. What are the PAOs expected to provide in that case?
- Could you clarify the role of funded patient partner in the consortium?
- Are patient organisations considered partners (does that count as one of the 3 minimum requirements for the application)?
- "Importantly, PAOs participating in Patient and Public Involvement and Engagement (PPIE) activities do not count towards the minimum consortium composition requirement of three partners from three different countries." So, currently we have in mind the Sponsor and CI from the same country, a mandatory CTMO from a second country and a PAO from a 3rd country. But if the PAO is involved in the highly-desired PPIE work, it is excluded from the eligibility list and the consortium fails for only having 2 different countries. Do we need to have 2 PAOs then? One to do the PPIE work, and another simply to count for the 3rd country?

Response:

Meaningful Patient and Public Involvement and Engagement is mandatory. Each consortium must include at least one Patient Advocacy Organisation or, where no disease-specific PAO exists, another organised patient group representing the target patient population.

The PAO must participate as a funded consortium partner and, therefore, receive appropriate financial support. Eligible PAO costs include staff time, travel and subsistence, translation and interpretation, reimbursement of out-of-pocket expenses and costs of developing patient-facing materials. The call therefore does fund PAO/PPIE activities when properly budgeted.

PAOs participating in PPIE activities do not count towards the minimum consortium composition requirement of three partners from three different countries. The only exception is a PAO acting as the Clinical Trial Sponsor, where it may count if it fulfils Sponsor eligibility and capacity requirements. Applicants should therefore ensure that the minimum three eligible-funded-partner requirement is met independently from a PAO when not acting as a Sponsor.

7.2 Patient organisations from non-eligible countries and patient recruitment from abroad

Related attendee questions (verbatim):

- If the patient advocacy is in the USA, is that acceptable?
- Could patients from non-site countries (USA) participate in a trial funded by ERDERA in case they are treated at one of the trial site which are funded by EREDRA?
- Can we recruit patients from one center only, but have different nationalities?

Response:

The Call Text allows collaborators from non-ERDERA countries if their participation is scientifically justified and they do not directly receive ECTC funding. Participation of patient organisations from non-eligible countries is considered potentially possible, but they would not be eligible for ERDERA funding.

Patients enrolled in the clinical trial can have different nationalities. Only patients from eligible Countries are allowed to receive ERDERA-funding. The Call Text does include strong requirements for cross-border financial and logistical support where multinational trials require participants to attend sites abroad. Applicants should therefore clearly describe patient mobility, support, ethics, consent, insurance, local care and reimbursement arrangements where relevant.

7.3 Partner search and patient-organisation identification

Related attendee questions (verbatim):

- Is there a place where we can find partners? We are a PAO in ALS and we will be interested to meet with people considering submitting on ALS (Charcot disease).
- Will ERDERA provide a matchmaking platform, partner-search tool, or service-provider list to help applicants identify eligible partners, CTMOs/CROs, patient organisations, or clinical sites?

Status: Answered with available resources.

Response:

No general partner-search or matchmaking platform will be provided by ERDERA.

Applicants seeking patient organisations are encouraged in the Call Text to consult sources such as Orphanet, EURORDIS, European Reference Networks and the European Patients' Forum. For Canadian networking, CIHR provides a partner linkage tool and MICYRN/RareKids-CAN resources.

8. Canada-specific questions

8.1 Canadian partner eligibility, roles and SMEs

Related attendee questions (verbatim):

- Is it possible to include a Canadian SME as a non-paid partner?
- Can Canadian SMEs participate as non-funded partners?
- Can Canadian investigators act as Coordinating Investigator?
- Can Canada be the Sponsor?

Response:

Canadian partners may participate as consortium partners and may also act as Coordinating Investigator. Canadian PAOs or patient partners may participate as partners or collaborators.

Canadian SMEs are not eligible for CIHR funding and are also not eligible to receive ERDERA funding. Participation as a non-funded partner/collaborator was considered possible, provided the role is justified and no ERDERA/CIHR funding is requested.

Canadian institutions cannot be the legal Sponsor (see also the next FAQ section). All consortia, including those with a Canadian Coordinating Investigator, must include a legal Sponsor established in an EU Member State or Norway.

8.2 Canadian sub-sponsor, Canadian CTMO and funds management

Related attendee questions (verbatim):

- How does the Canadian funding mechanism interact with the consortium?
- What additional requirements apply to Canadian partners?
- Can a Canadian component be funded through CIHR or ERDERA central funds?

Response:

For consortia involving Canadian partners, a Canadian sub-sponsor or equivalent institutional structure must be identified for the Canadian component. A Canadian CTMO must also be identified, or access to such an organisation must be demonstrated, in addition to the overall multinational CTMO requirement.

CIHR funds will be awarded to top-ranked consortia with a Canadian component until the available CIHR funds are exhausted. For CIHR-funded Canadian partners, funds are transferred directly to the NPA's Canadian institution and managed in Canada. Once CIHR funds are exhausted, additional eligible Canadian partners may be funded through the ERDERA central fund, with FTELE rules applying and funds transferred via the EU legal Sponsor.

Canadian applicants follow the same ERDERA/FTELE submission process for Stages 0–2. At Stage 3, Canadian applicants invited to submit a Full Proposal must also submit a short proposal and budget in Canadian dollars to CIHR via ResearchNet.

9. Additional Q&A

- Are rare diseases or rare disease forms missing an ORPHAcode eligible?

Rare diseases or disease subtypes with rare or ultra-rare incidence that lack an assigned ORPHA code remain eligible for the ECTC, provided the application clearly describes the disease (including prevalence), the unmet

medical need, and the rationale for the study. The classification as a distinct rare disease will be evaluated by the Clinical Trial Scientific Committee.

10. Final remarks

Slides, recording, and written Q&A/FAQ would be shared and made available on the ERDERA website. Applicants should consult the Call Text and Guidelines first, as many questions are answered in those documents.

EOI submission is available at the following link.

For questions relating to the ERDERA Clinical Trial Call, applicants should contact the Scientific Secretariat: ctc.secretariat@erdera.org

For financial and Award Agreement enquiries after funding decisions, applicants should contact the FTELE grants management team: telethonscience@fondazionetelethon.it.

For Canadian partner requirements, applicants should contact CIHR: PDD-IGStrategic-CEP-IGStrategique@cihr-irsc.gc.ca