ERDERA

European Rare Diseases
Research Alliance

Information Webinar
ERDERA Clinical Trial Call 2026

Carmen Fotino, FTELE
July 6th, 2026

Co-funded by
the European Union

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595.
Views and opinions expressed are those of the author(s) only and do not necessarily reflect those of the European Union or any other granting authority, who cannot be held responsible for them.

AGENDA

15:00 – 15:10
Welcome and introduction to ERDERA
Daria Julkowska

15:10 – 15:50
ERDERA Clinical Trial Call 2026
Carmen Fotino

15:50 – 16:00
Information for Canadians
Anne-Cécile Desfaits, Ilana Gombos (CIHR)

16:00 – 16:10
Support stage – Stage 2
Mark Turner (C4C)

16:10 – 17:00
Q&A

Improving the health and well-being of 30 million people living with a rare disease by making Europe a world-leader in RD research and innovation.

Diagnosis established or enrolment in systematic research in average **within 6 months** after coming to medical attention

New **effective therapies approved** in Europe and beyond, the majority of which addressing diseases without approved options

Better understanding of the impact of rare diseases on patients, families and society to improve quality of life

ERDERA: Our Global Scale & Investment

Diverse Partnership Network

Our mission is powered by a broad network of nearly **180 organizations from 37 countries**, including:

- Funders
- Research Institutions
- Hospitals
- Research Infrastructures
- Patient Organizations
- Industry Partners

Extensive Global Reach

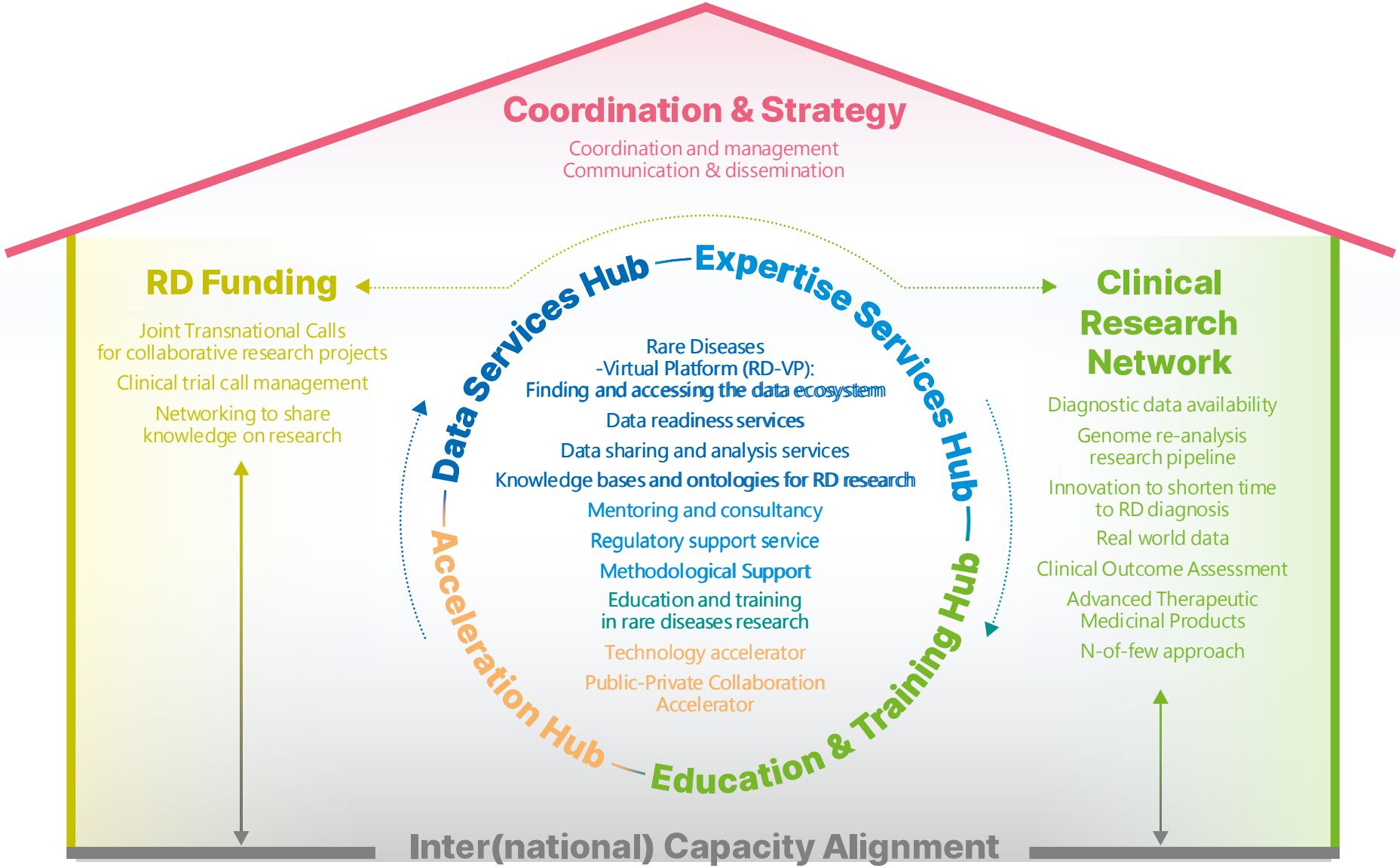
ERDERA spans **25 European Union countries** (except Croatia and Malta) and **numerous associated and non-EU countries**, fostering international cooperation (Australia, Canada, Iceland, Israel, Georgia, Morocco, New Zealand, Norway, Serbia, Switzerland, Türkiye, United Kingdom)

Substantial Financial Commitment

A significant financial commitment underpins our ambitious goals, with a total budget of approximately **€380 million**:

- **€150 million** investment from the European Commission
- Over €230 million contributed by participating countries and institutions
- **Every partner contributes** to our collective actions

ERDERA brings together diverse expertise and significant resources to advance its mission, demonstrating a strong commitment to global collaboration.



ERDERA European Rare Diseases Research Alliance

Welcome to

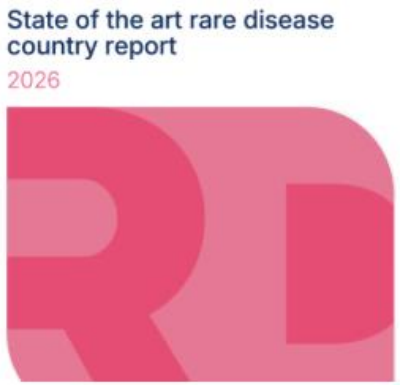
The European partnership for rare disea public and private organisations

LEARN MORE ABOU

LATEST

News & Updates

VIEW ALL



European **Rare Diseases** Research Alliance

Thank you!

Follow us

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AIM OF THE CALL

The aim of the **ERDERA Clinical Trial Call (ECTC)** is to fund multi-national, sponsor-led, GCP-compliant Phase I/II interventional clinical trials on rare diseases, generating data of sufficient quality and regulatory relevance to support future EMA regulatory filings, where appropriate. The call specifically targets areas of high unmet medical need where centrally coordinated, multinational funding offers a decisive advantage over individual national efforts.

IN SCOPE

Study proposals must meet all of the following criteria:

- Address a rare disease that qualifies as a rare disease under Regulation (EC) No 141/2000 on orphan medicinal products, **defined as a condition affecting not more than five in 10,000 persons in the European Community, as classified by Orphanet**.
- Address a clearly defined, high unmet medical need in a severe, debilitating, or life-threatening condition in the relevant patient population. **"Unmet medical need"** is understood in accordance with the definition adopted by EMA/CHMP (Committee for Medicinal Products for Human Use): a disease or condition where no satisfactory method of diagnosis, prevention, or treatment is authorised, or where, even if such a method exists, the proposed intervention provides substantial clinical benefit compared to existing therapies for patients with an unmet need.
- Target diseases with no satisfactory or sufficiently effective therapeutic option currently available, or with substantial residual unmet need despite existing treatment.
- Consists of **multi-national Phase I, Phase I/II, or Phase II interventional clinical trials** conducted in accordance with Good Clinical Practice (GCP) as per ICH E6(R3) guidelines and applicable EU regulations (EU Clinical Trial Regulation No. 536/2014).

PRIORITY AREAS

Priority areas for this call

While proposals on all eligible rare diseases are welcome, the call particularly encourages applications addressing the following disease categories, where the need for coordinated multinational funding is most acute:

- **Paediatric rare diseases -** conditions in which the relevant patient population is predominantly paediatric, as classified by Orphanet.

- **Rapidly progressive rare diseases -** conditions characterised by swift clinical deterioration over a short timeframe, creating heightened urgency for access to investigational therapies.

- **Conditions lacking approved therapeutic options -** rare diseases for which no medicinal product has received marketing authorisation in the EU, or for which existing authorised treatments provide only partial or insufficient clinical benefit.

These priority areas are not eligibility criteria. However, where two or more proposals are of otherwise equivalent scientific quality, alignment with the above priority areas will be taken into account in the final strategic prioritisation.

OUT OF SCOPE

The following are explicitly excluded from the scope of this call:

- Non-clinical studies (in vitro, in silico, animal studies, preclinical work).
- Studies of medical devices, food supplements, radiological or surgical procedures, or behavioural/rehabilitation interventions without any investigational medicinal product.
- Phase III and Phase IV clinical trials.
- Pharmacovigilance studies (Phase IV).
- Rare infectious diseases and rare cancers. (Tumour-predisposition syndromes and non-malignant or low-grade tumour manifestations within a rare genetic disease are not excluded and remain eligible).
- Rare adverse drug events or secondary outcomes in the treatment of non-rare diseases.
- Projects where the primary objective is a health economic assessment without an interventional component.

ELIGIBLE THERAPEUTIC INTERVENTION

- **Small molecules** - both new chemical entities (NCEs) and repurposed drugs (i.e., existing authorised substances proposed for a new indication).
- **Advanced Therapy Medicinal Products (ATMPs)** - provided that the manufacturing process has been developed and validated under GMP conditions appropriate for Phase I/II use.
- **Biologics and New Biological Entities (NBEs)** - i.e., biological substances not previously authorised as a medicinal product in the EU.
- **Repurposed biologics**. Proposals involving repurposed drugs or repurposed biologics must demonstrate, at Stage 2, that both (i) uninterrupted IMP supply for the full duration of the trial and (ii) a credible pathway towards patient access following successful trial completion are secured or realistically achievable

ELIGIBLE APPLICANTS AND ORGANIZATIONS

Research proposals must be **multinational** and may be submitted by applicants belonging to one or more of the following categories:

- Academia-research teams working in universities, higher education institutions, or research institutes.
- Clinical centres-research teams working in hospitals, clinical research organisations, or other healthcare settings, regardless of their legal status or profit nature.
- Patient Advocacy Organisations (PAOs).
- Small and Medium Enterprises (SMEs), eligible as funded partners and may act as Sponsor.
- **SMEs from Canada are not eligible to receive funding from CIHR or ERDERA.**
- Non-profit research organisations or foundations.

Private for-profit companies other than SMEs are not eligible to receive ERDERA funding as beneficiaries or funded partners within the consortium.

ELIGIBLE COUNTRIES

Austria	Italy
Belgium	Latvia
Bulgaria	Lithuania
Canada (Canadian Institutes of Health Research, CIHR)	Luxembourg
Cyprus	Morocco
Czech Republic	Norway
Denmark	Poland
Estonia	Portugal
Finland	Romania
France	Serbia
Germany	Slovakia
Georgia	Slovenia
Greece	Spain
Hungary	Sweden
Ireland	Türkiye
Israel	The Netherlands
	United Kingdom

CONSORTIUM COMPOSITION REQUIREMENTS

- Only **multinational** clinical trials will be funded.
- Each consortium must involve a minimum of three independent eligible-for-funding partner institutions from at least three different ERDERA member countries. Partners from the same country may participate, provided that the multinational requirement is fulfilled and the participation of each partner is scientifically justified.
- The consortium must designate a **Clinical Trial Sponsor and a Coordinating Investigator** and include at least one funded patient partner represented by a **Patient Advocacy Organisation (PAO)** or another organised patient group.
- The consortium must include, or demonstrate a formal access arrangement with, a qualified multinational **Clinical Trial Management Organisation (CTMO)**

THE COORDINATING INVESTIGATOR

The Coordinating Investigator is responsible for the scientific leadership and coordination of the project.

The Coordinating Investigator will:

- Be responsible for the scientific leadership and internal coordination of the project, including scientific reporting and communication with evaluation bodies.
- Ensure timely submission of all scientific deliverables, milestone reports, and other documents required by the Scientific Secretariat and FTELE.
- Coordinate scientific activities and serve as the primary interface with the Scientific Secretariat, the CTSC, and FTELE on scientific and project implementation matters.

THE CLINICAL TRIAL SPONSOR

- **The Sponsor** is the legal entity that assumes full responsibility for the initiation, management, conduct, and financial accountability of the clinical trial in accordance with Regulation (EU) No. 536/2014 and applicable national legislation. **The Sponsor must be established in an EU Member State or Norway.**
- Any eligible legal entity participating in the consortium may act as Sponsor, including academic institutions, clinical centres, non-profit foundations, Patient Advocacy Organisations (PAOs), and SMEs.
- **Private for-profit companies** other than SMEs are not eligible to act as a sponsor.
- The Sponsor and the Coordinating Investigator may belong to the same institution or to different consortium partners.

THE CLINICAL TRIAL SPONSOR

The Sponsor assumes all legal and regulatory responsibilities associated with the clinical trial, including:

- submission and maintenance of the Clinical Trial Application through CTIS,
- pharmacovigilance reporting,
- safety oversight
- trial monitoring,
- quality management,
- fulfilment of Good Clinical Practice (GCP) obligations across all participating clinical sites.

The Sponsor remains ultimately responsible for ensuring compliance with all applicable regulatory and GCP requirements, irrespective of any delegation of activities or support provided by consortium partners or external service providers.

CTMO AND PAO

- **Clinical Trial Management Organisation (CTMO).** Each consortium must include, or demonstrate a formal access arrangement with, a qualified organisation with proven experience in coordinating multinational clinical trials. For consortia including a Canadian partner, a Canadian CTMO must be identified in addition to the European CTMO

- **Patient and Public Involvement and Engagement (PPIE).** Patient engagement must be integrated throughout the design, conduct, oversight, and dissemination of the proposed study and must go beyond a purely advisory or consultative role. Patient involvement should be substantive, meaningful, and documented throughout the clinical trial lifecycle.

COLLABORATORS

- Collaborators (self-funded participants) are entities that participate in the project but do not receive funding. They cover their own costs of participation from their own resources or other funding sources.
- Collaborators must demonstrate clear scientific added value to the clinical study, provide evidence that funding for their participation is secured, and submit a signed letter of commitment.
- Collaborators may contribute to project activities but may not act as Sponsor, Coordinating Investigator, work package leader, or beneficiary responsible for project deliverables or milestones.
- Collaborators from non-ERDERA countries are permitted, provided their participation is scientifically justified and they do not receive ECTC funding.

SUBCONTRACTORS

- Subcontractors are third parties contracted by a funded consortium partner to carry out specific, clearly defined tasks or services.
- Subcontractors are remunerated from the ECTC budget of the contracting partner and must be selected in accordance with the applicable procurement and value-for-money principles.
- Subcontracting must be clearly described and justified in the proposal. Core scientific, regulatory, Sponsor, and Coordinating Investigator may not be subcontracted.

ADDITIONAL RECOMMENDATIONS

To strengthen the scientific, operational, and regulatory readiness of the proposed clinical trial, applicants are encouraged to address the following elements:

- Demonstrate a robust understanding of the natural history of the targeted rare disease(s);
- Provide evidence of access to appropriate patient identification and recruitment resources, such as patient registries, databases, natural history studies, ERNs, expert centres, or equivalent mechanisms;
- Describe the anticipated recruitment capacity of participating sites and the basis for recruitment projections;
- Describe the anticipated regulatory, clinical development, and patient access pathway following completion of the proposed study;
- Describe plans for communicating study results to participants, patient organisations, and the broader patient community;
- Demonstrate access to the investigational medicinal product and the manufacturing and supply arrangements necessary to support the proposed trial and subsequent development activities, where applicable.

ADDITIONAL RECOMMENDATIONS

Applicants are furthermore encouraged to engage early with relevant regulatory authorities and development pathways, including, where appropriate:

- **Orphan Drug Designation (ODD)**. Applicants are strongly encouraged to have obtained, applied for, or initiated preparations for obtaining the ODD. ODD is a regulatory status granted by the EC, following a positive opinion from EMA, for medicinal products intended for the diagnosis, prevention, or treatment of rare diseases.

- **Paediatric Investigation Plans (PIPs).** For proposals targeting paediatric rare diseases, applicants are encouraged to describe the status of any PIP or provide justification where a PIP is not applicable.

- **EMA Scientific Advice**, Protocol Assistance, or Innovation Task Force (ITF) briefing Meetings, as well as Scientific advice procedures offered by national competent authorities.

CALL TIMELINES

PRE-ANNOUNCEMENT	June 3rd 2026
Stage 0 – EOI open	July 1st – September 10th 2026
Stage 1 – Short Proposal	September 15th – 29th October 2026
Short Proposal peer review	*By December 2026*
Stage 2 – Support Stage	January – July 2027
Stage 3 – Full Proposal	July – September 2027
Full Proposal peer review	*By October 2027*
Stage 4 – Funding	February 2028

THE APPLICATION PROCESS: STAGE 0 – EOI

Proposals for which no EOI has been submitted by the applicable deadline will not be eligible to proceed to Stage 1.

Requested information:

- Disease area and disease name
- Orphanet/ORPHA code
- Description of the unmet medical need
- Type of intervention
- Target population
- Intended development phase for the trial (Phase I, Phase I/II, or Phase II)
- Whether an ODD has already been requested or obtained
- Confirmation of preliminary readiness
- Whether applicants have already applied for EMA scientific advice (or any other national regulatory body)
- Preliminary information on consortium partners, including countries in which the trial will be conducted and the European Reference Network (ERN) covering the disease, where applicable

THE EVALUATION PROCESS

The evaluation process will be managed by the Scientific Secretariat and conducted by the Clinical Trial Scientific Committee (CTSC). The evaluation comprises two scientific assessment stages:

- **Short Proposal evaluation** (Stage 1), open only to applicants who submitted an Expression of Interest (EOI) in Stage 0;
- **Full Proposal evaluation** (Stage 3), open only to consortia that completed Stage 2 and were invited to submit a Full Proposal.

THE EVALUATION PROCESS

Each application will be reviewed by at least 3 reviewers for the Short Proposal and 3-5 reviewers for the Full Proposal. The reviewers will be appointed by the Call Secretariat, and will include:

- **Scientific experts** with relevant expertise in the disease area, therapeutic modality, or clinical development pathway;
- **Patient expert** with relevant lived experience or patient advocacy expertise;
- Expert in clinical trial **methodology and/or biostatistics**;
- **Regulatory** expert with experience relevant to rare disease medicinal product development.

Potential conflicts of interest will be assessed and managed before proposal allocation.

THE EVALUATION PROCESS: ASSESSMENT CRITERIA

Short Proposal

1. **Scientific Quality**
2. **Impact**
3. **Quality and Feasibility of Implementation**

Full Proposal

1. **Scientific Excellence**
2. **Impact**
3. **Quality and Feasibility of Implementation**
4. **Competence of the Consortium and Environment**
5. **Clinical Trial Design and Statistical Methodology**

THE EVALUATION PROCESS: SCORING SYSTEM

Score	Description
0- Failure	The proposal fails to address the criterion or cannot be assessed due to missing or incomplete information.
1- Poor	The criterion is inadequately addressed, or there are serious inherent weaknesses.
2- Fair	The proposal broadly addresses the criterion, but there are significant weaknesses.
3- Good	The criterion is addressed well, but several shortcomings are present.
4- Very Good	The criterion is addressed very well, but a small number of shortcomings are present.
5- Excellent	All relevant aspects of the criterion are successfully addressed; any shortcomings are minor.

The maximum score for Short Proposals is 15 points (3 criteria × 5).

The maximum score for Full Proposals is 25 points (5 criteria × 5). Full Proposals will not be considered fundable if any individual criterion receives a score below 3, or if the total score is below 18 points.

THE EVALUATION PROCESS: REBUTTAL PHASE

Before the final CTSC funding meeting, each consortium submitting a Full Proposal will receive the complete set of reviewer assessments.

The rebuttal phase allows applicants to respond to reviewer comments and questions. It is not intended to permit modifications to the work plan, study design, consortium composition, or budget.

Applicants will have 7 calendar days to submit an optional written response. Responses submitted after the deadline or addressing matters unrelated to reviewer comments may be disregarded.

Rebuttal responses will be considered by the CTSC during final deliberations.

THE EVALUATION PROCESS: INTERVIEWS

The interview will be conducted by a panel of a minimum 3 CTSC experts. The interview serves as a qualitative assessment of the consortium's readiness to implement the proposed clinical trial and may cover:

- Previous experience of the Coordinating Investigator, Sponsor, and key consortium partners in conducting interventional clinical trials;
- Sponsor oversight arrangements and delegation of responsibilities;
- Clinical trial management arrangements, including the role of the CTMO where applicable;
- Regulatory, pharmacovigilance, monitoring, and quality management arrangements;
- Risk identification and mitigation strategies;
- Governance structures and communication flows across the consortium.

GOVERNANCE

FTELE

- FTELE acts as the **central funding administrator** on behalf of ERDERA. FTELE is responsible for the financial management of awarded grants, including signing Award Agreements with successful consortia, disbursing funds, preparing financial reports, and liaising with the EC on audits.
- FTELE will ensure a smooth **implementation of the funded trials** and follow the **achievement of project-specific milestones** for each funded clinical trial
- Hosts the **Scientific Secretariat (S-CTC),** responsible for the day-to-day management of the call, including applicant communication, formal eligibility checks, organisation of the evaluation process, and coordination of the interview procedure.

BoF

It is the final decision-making body for the ECTC.

Clinical Trial Scientific Committee (CTSC)

Is the independent body responsible for the scientific evaluation of all proposals.

ERDERA Coordination Office

It provides administrative support to the Call Secretariat and contributes to the organisation and oversight of the call process.

BUDGET

Up to €30 million of the ERDERA budget is available to fund projects under this call. Budgets are expected to be in the range of **€1-5 million** per project. Projects outside this range are eligible but must provide adequate justification.

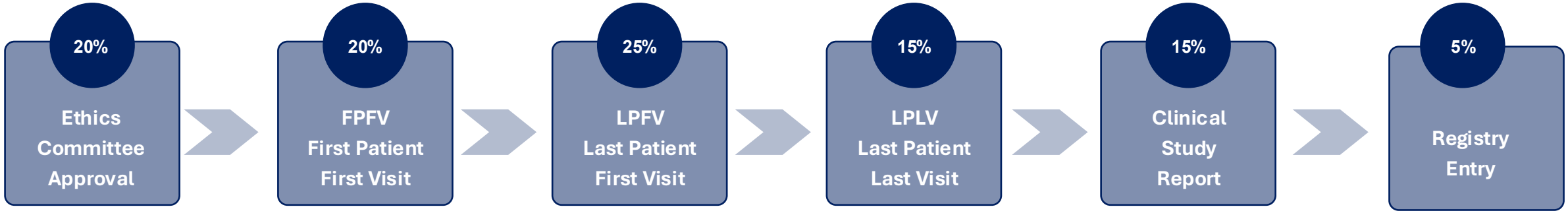
Mandatory budget requirements:

Multinational clinical trial management partner: All funded consortia are required to include, or demonstrate a formal access arrangement with, an organisation with proven experience in coordinating multinational clinical trials.

Central data collection and management service: Funded projects will be required to use the central data collection and management service designated by the ERDERA programme to promote harmonised, interoperable and regulatory-quality data collection across the portfolio.

MILESTONES

Funding will be provided through an Award Agreement between FTELE and the Sponsor institution. The implementation plan will include predefined milestones and Go/No-Go criteria agreed upon during the grant preparation process. For each funded project, key milestones will typically include:



European Rare Diseases Research Alliance

THANK YOU!!!

Scientific Secretariat-ERDERA Clinical Trial Call

Email: ctc.secretariat@erdera.org

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ERDERA CLINICAL TRIAL CALL 2026 – CANADIAN APPLICANTS

- Anne-Cécile Desfaits, CIHR Institute of Genetics
- Ilana Gombos, Program Design and Delivery, CIHR

CIHR IRSC
Canadian Institutes of Health Research | Instituts de recherche en santé du Canada

- ❑ Eligibility Criteria
- ❑ Sponsor Requirements (Legal Sponsor and Canadian Sub-Sponsor)
- ❑ Funds Management (CIHR and ERDERA)
- ❑ Application Process for Canadian Partners
- ❑ Resources: Support for Applicants, Linkage Tool, Information Webinar, CIHR Contacts

ECTC 2026 – ELIGIBILITY CRITERIA

Canada-specific

Eligibility criteria for Canadian partners

- The Nominated Principal Applicant (NPA) leading the Canadian component must be an **independent researcher** affiliated with a Canadian post-secondary institution and/or its affiliated institutions, including hospitals, research institutes, or non-profit organisations with a mandate in health research and/or knowledge translation.
- The NPA must have their substantive role in Canada for the duration of the requested grant term.
- The Institution Paid must be authorised to administer CIHR funds by the application deadline.

Eligible roles

- Canadian partners may participate as consortium partners.
- Canadian partners may also act as Coordinating Investigator.
- Canadian patient advocacy organisations (PAOs) or patient partners are eligible to participate as co-applicants or collaborators.

Restriction

Canadian small and medium-sized enterprises (SMEs) are not eligible for CIHR or ERDERA funding.

At a glance

Canadian participation is allowed, but CIHR-specific applicant and institutional eligibility rules apply.

ECTC 2026 – SPONSOR REQUIREMENTS

Canada-specific

Fondazione Telethon / ERDERA funding

EU / Norway Legal Sponsor (mandatory)

- Signs Award Agreement with Fondazione Telethon
- Receives ERDERA funding
- Redistributes funding to consortium partners

Canadian institutions cannot be the Legal Sponsor

Canadian national structure

- Canadian sub-sponsor / institution — Supports coordination of the Canadian trial component, including full Health Canada Division 5 oversight for regulated trials and other ethics and regulatory approvals.
- Canadian CTMO — Must be identified or formally accessible.
- Participating institutions

Required agreements

- roles and responsibilities
- coordination of trial implementation, including EU–Canada regulatory and operational interfaces

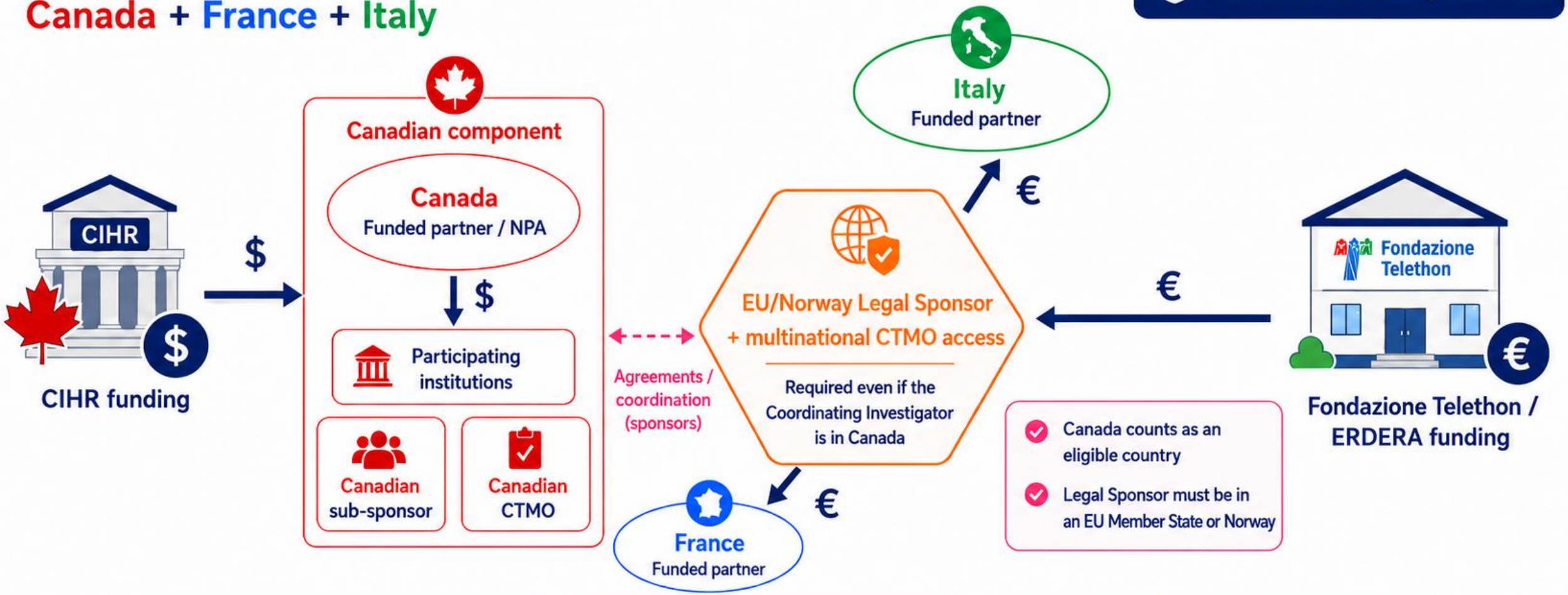
CIHR exception
If CIHR-funded, funds go directly to the Canadian institution.

ERDERA Central Fund requirement
If funded through ERDERA Central Fund, the EU Legal Sponsor must be able to transfer funds to Canadian institutions.

ECTC 2026 – Example of a consortium

Canada + France + Italy

Minimum compliant example
3 funded partners / 3 eligible countries



CIHR funding → direct to Canadian institution

ERDERA Central Fund → transfer to Canada via EU Sponsor

Blue arrow = funding flow

Pink dashed line = agreement / coordination

ECTC 2026 – FUNDS MANAGEMENT

Canada-specific

Budget preparation rule

Canadian partners must prepare budgets compliant with CIHR rules. They do not need to specify whether funds will come from CIHR or ERDERA.

4.1 CIHR Funding (primary source)

- Awarded to the top-ranked consortia that include a Canadian component, until available CIHR funds are exhausted.
- The Institution Paid must be in Canada and authorized to administer CIHR funds by the application deadline.
- Funds are administered under the CIHR Application Administration Guide and the Tri-agency Guide on Financial Administration, then transferred directly to the NPA's institution and managed within Canada.

Primary source

4.2 ERDERA Central Fund (secondary funding)

- Once CIHR funding is exhausted, additional eligible Canadian partners may be funded through the ERDERA Central Fund. In that case, ERDERA — through Fondazione Telethon — becomes the funding source, and Fondazione Telethon's financial rules apply.
- For Canadian partners funded through FTELE, funds are transferred to the consortium's EU legal Sponsor, which redistributes funding to all consortium partners, including Canadian institutions, under the applicable funding arrangements.

Secondary source

At a glance

Prepare one CIHR-compliant budget → CIHR funds top-ranked Canadian components first → If CIHR funds are exhausted, ERDERA Central Fund may support additional eligible Canadian partners through the EU Sponsor

Management route depends on the funding source: direct to the Canadian institution for CIHR, via the EU legal Sponsor for ERDERA Central Fund.

ECTC 2026 – APPLICATION PROCESS

Canada-specific

0 Stage 0 – Expression of Interest (EOI)

- One joint document in English
- Submitted by the Coordinating Investigator
- Via Fondazione Telethon electronic submission system

Deadline:
10 Sept 2026, 1:00 p.m. CET

1 Stage 1 – Short Proposal

- One joint document in English
- Submitted via FTELE platform

Deadline:
29 Oct 2026, 1:00 p.m. CET

2 Stage 2 – Support Stage (PSS)

- Preparation of Study Services (PSS)
- For shortlisted applicants
- Free of charge
- Not a submission or evaluation stage

Support only

3 Stage 3 – Full Proposal

- Full proposal in English
- Submitted via FTELE platform
- Deadline: end of Sept 2027
- Exact deadline announced later

Additional requirement for Canadian partners

1. Submit full proposal through Fondazione Telethon platform (with the consortium)

&

2. Also submit a short proposal + budget in Canadian dollars to CIHR via ResearchNet

More information will be published on the CIHR website in early 2027.

For Canadian partners

- **Stages 0–2:** submission only through Fondazione Telethon platform
- **Stage 3:** FTELE submission + additional CIHR / ResearchNet submission

Key platforms

Fondazione Telethon platform – all stages

CIHR ResearchNet – additional Stage 3 submission for Canadians

Guidelines for short and full proposals will be published later. ERDERA website: erdera.org/call/ctc2026/

ECTC 2026 – SUPPORT AVAILABLE IN CANADA

Canada-specific

***RareKids-CAN** is Canada's CIHR-funded pediatric rare disease clinical trials and treatment network, while **MICYRN** offers national clinical trial management and sponsorship support.*

NATIONAL NETWORK

Work with us

RareKids-CAN Intake Form

RareKids CAN

- Connect sponsors with Canadian sites and investigators
- Support feasibility assessments and site identification
- Facilitate national planning and collaboration
- Subsidy for select clinical trial services

CIHR IRSC
Canadian Institutes of Health Research / Instituts de recherche en santé du Canada

MICYRN

Maternal Infant Child and Youth Research Network

- National network of pediatric research member institutes
- Eligible to receive and administer CIHR funding
- Support grant and proposal development
- Can serve as the Canadian Sub-Sponsor
- Can provide CTMO services

CTMO services include:

- Regulatory and ethics support
- Study start-up and coordination
- Project management
- Monitoring and quality oversight

Together, RareKids-CAN and MICYRN provide a national infrastructure to support Canadian participation, funding administration, and operational delivery of multinational pediatric clinical trials.

MICYRN Maternal Infant Child and Youth Research Network | RareKids CAN

ECTC 2026 – RESOURCES

Canada-specific

CIHR information webinar

- **Purpose:** Information on the requirements of the 'ERDERA Clinical Trial Call 2026 (ECTC 2026)' funding opportunity and Q&A on how to apply.
- **When:** Tuesday, July 7, 2026, at 10:00 AM ET
- **Duration:** 60 minutes
- **Language:** Bilingual presentation followed by a period of questions and answers in both official languages, English and French.
- **Link:** https://cihr-irsc.gc.ca/e/45096.html

Join

Partner linkage tool

To support the formation of multinational consortia and facilitate networking between partners in Canada and other ERDERA-eligible countries, CIHR has created a partner linkage tool.

- More information: https://cihr-irsc.gc.ca/e/54771.html

Networking support

Contact at CIHR

For all enquiries relating to requirements for Canadian partners, please contact the Canadian Institutes of Health Research:

- PDD-IGStrategic-CEP-IGStrategique@cihr-irsc.gc.ca

Questions from Canadian partners

ERDERA
European Rare Diseases
Research Alliance

ERDERA Clinical Trial Call: Preparation of Study Service (Stage 2 of the evaluation process)

Mark Turner

on behalf of:

eatris

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

eYPAGnet

Rationale for the Preparation Study Service (PSS)

- Clinical trials need to lead to impact: marketing authorization, reimbursement.
  - In order to have a high likelihood of contributing to marketing authorization, studies need to be reviewed by the European Medicines Agency: Scientific Advice
- Clinical trials need to use optimized and validated methodology
- Clinical trials need to be centred on people with lived experience

CTC PSS

All shortlisted applications will receive standardized support for three key pillars of a full application:

Pillar 1: Feedback from EMA through Scientific Advice

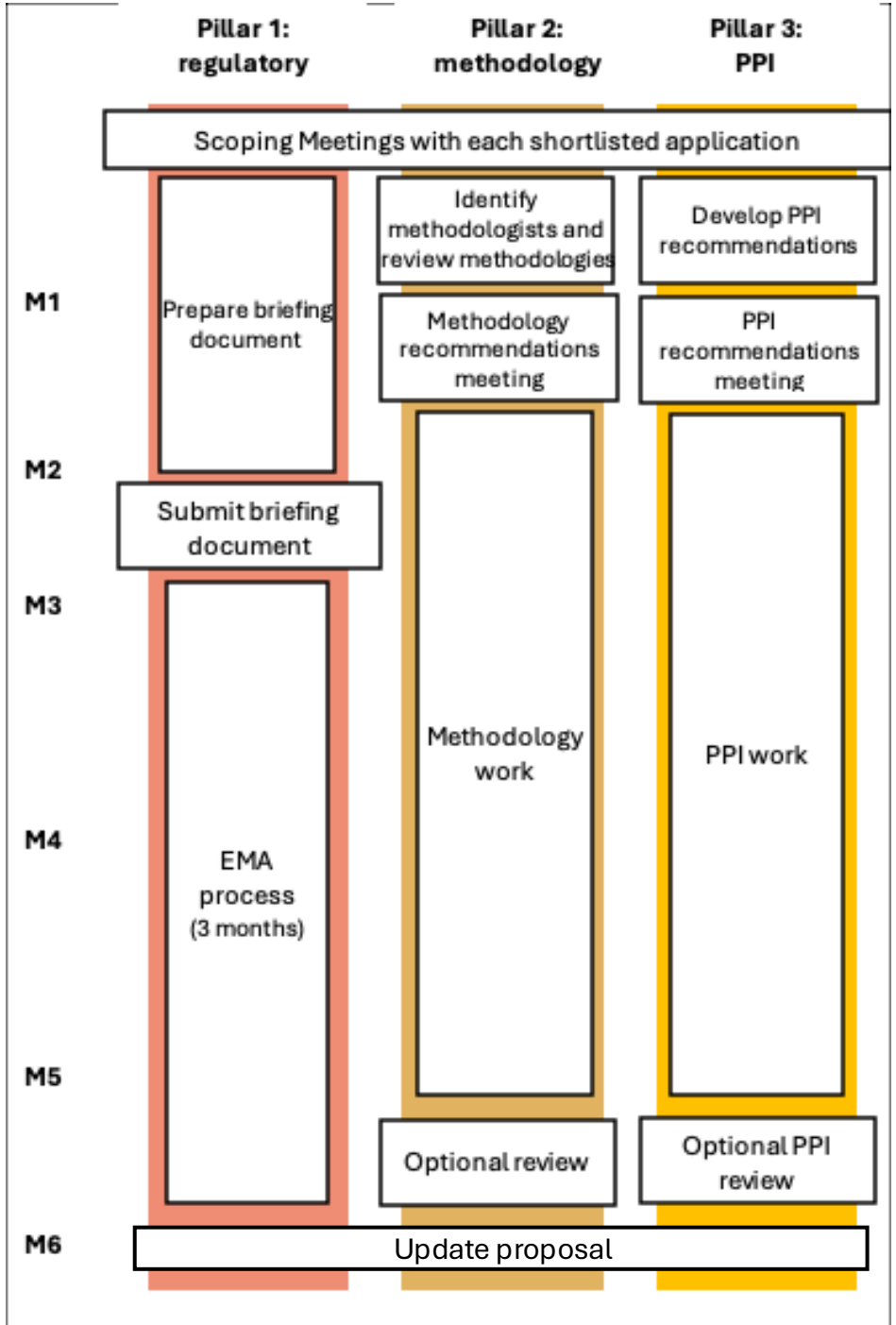
Pillar 2: Methodology

Pillar 3: Patient and Public Involvement

All support will be free from conflicts of interest

Support covers all age groups and conditions and will be provided by members of ERDERA Beneficiaries

ERDERA
European Rare Diseases Research Alliance



ERDERA
European Rare Diseases Research Alliance

Pillar 1: Alignment with Regulators (EMA) – Coordinated by EATRIS

- The aim of Pillar 1 is to support access to the EMA's Scientific Advice procedure so that each application is well-placed to contribute to regulatory decision-making. This could include using the trial results in subsequent steps towards a Marketing Authorisation or meeting requirements such as a Paediatric Investigation Plan.
  Pillar 1 focuses specifically on supporting applicants in the preparation of the briefing package required to request Scientific Advice at the EMA. It does not encompass the full EMA procedure itself.
- As the EMA's standard rules and procedures will apply, applicants are required to meet the appropriate timelines and requirements for requesting Scientific Advice, including the preparation of the briefing package. The applicants are encouraged to submit their application for Scientific Advice and accompanying Briefing Book by the end of Month 2, as EMA process takes approximately 6 months. Pillar 1 secretariat will explain the process and requirements and review drafts.

Pillar 1 secretariat will support applicants throughout this process by:

- explaining the EMA requirements and key procedural steps,
- providing guidance on the preparation of the briefing package,
- reviewing draft materials prior to submission,
- facilitating interactions with experts and supporting follow-up discussions.

- Each application will have access to 2 or 3 independent, conflict-free experts, selected to support the preparation of the documentation required for requesting scientific advice from the European Medicines Agency (EMA). These experts may overlap with experts in Pillar 2.

Pillar 1 secretariat interaction with EMA before the start of PSS

The EMA Scientific Advice process follows standard procedures and timelines, which are external to ERDERA. This process typically takes several months, and it cannot be guaranteed that feedback will be fully received within the duration of the PSS.

To facilitate efficient support an early coordination step with EMA will be organised before the start of the PSS with the Pillar 1 secretariat.

- This coordination aims to i) identify the most appropriate regulatory pathway for each shortlisted project and ii) support efficient sequencing of interactions.
- This step is intended to guide and accelerate the support provided under Pillar 1, but it does not replace or anticipate the formal EMA procedures.

Applicants will be encouraged to request Scientific Advice as early as possible during the PSS. However:

- EMA feedback may be received after the PSS,
- and therefore, may not always be fully available at the time of full proposal submission.

At full proposal stage, applicants may be requested to provide information such as:

- whether a request for Scientific Advice has been submitted,
- whether a briefing package has been prepared and submitted,
- and, if available, how feedback has been received and taken into account.

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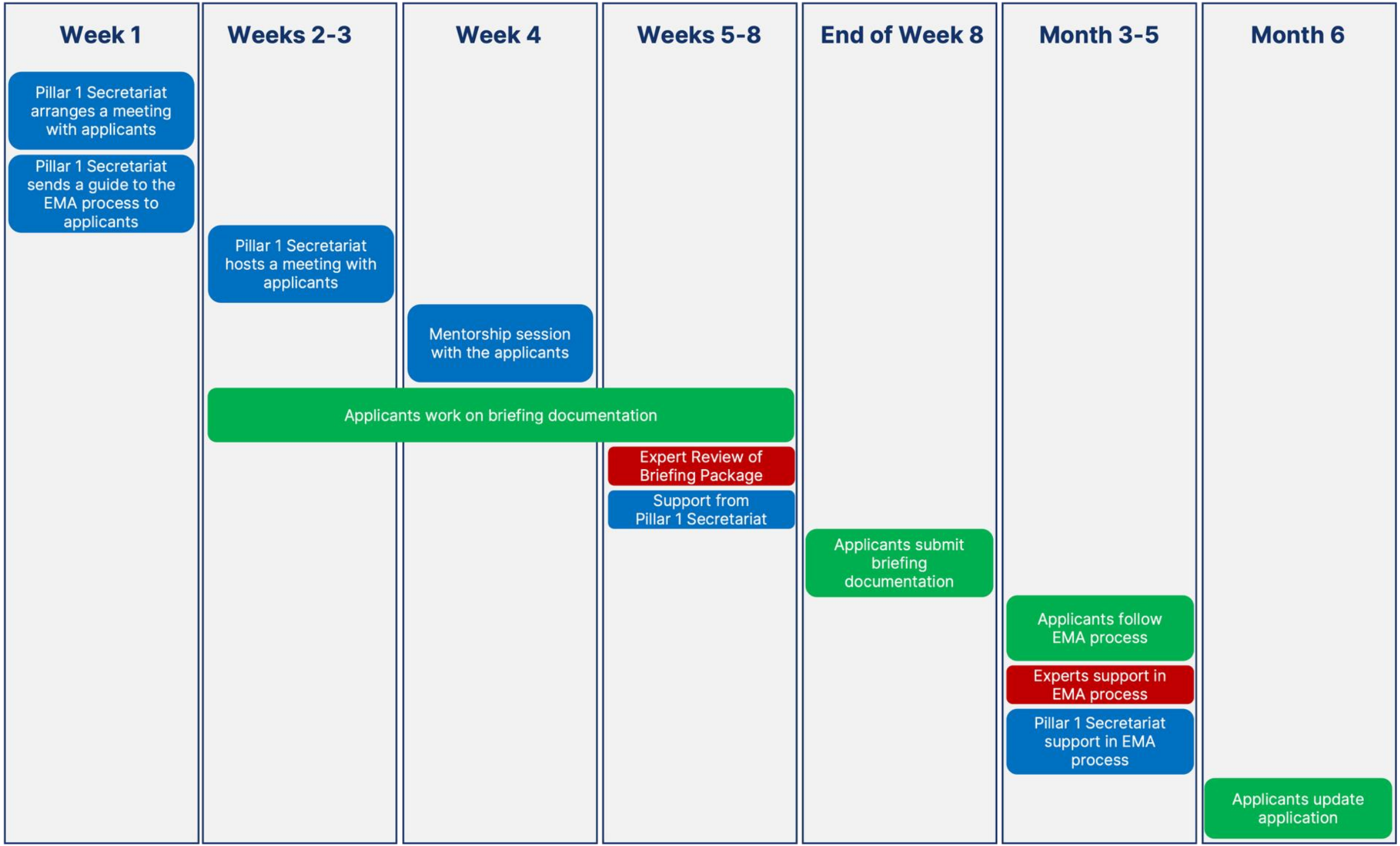
Steps of Pillar 1

1. **Preparation and onboarding of applicants by the Pillar 1 Secretariat** (*Week 1*) - The Secretariat organises the scoping meeting (next step) with applicants and shares a guidance document on the EMA process to support early understanding and preparation.
2. **Scoping Meeting between the Pillar 1 Secretariat with applicants** *(Weeks 2–3)* - An initial meeting to define the scope of engagement, align on expectations, and identify the specific needs of each applicant team.
3. **Mentorship session with the applicants** *(Month 1)* - A dedicated meeting to discuss regulatory considerations, development pathways (therapeutics, diagnostics, biomarkers), innovation management, and other aspects that can strengthen the proposal.
4. **Expert review of the briefing package, supported by the Pillar 1 Secretariat** *(Month 2)* - Appointed experts review and provide feedback on the drafted briefing package, with operational support from the PSS team.
5. **Submission of the briefing document to EMA** *(by end of Month 2)* - Formal submission of the completed briefing document to EMA within the second month deadline.
6. **Support throughout the EMA process, with experts and Pillar 1 secretariat involvement** *(Months 3–5)* - Ongoing assistance to applicant teams during interactions with EMA, with active contributions from both the appointed experts and the PSS team.
7. **Applicants update their proposal considering EMA feedback** (*Month 6)* **-** Applicants revise and strengthen their application by incorporating the scientific advice and recommendations received from EMA, if they were received.

Advice will also be available during Months 3, 4, and 5 through e-mail discussion or video/teleconferences as appropriate to each application.

NB: Pillar 1 Secretariat cannot draft the package but can provide experts to review it.


Week 1
Pillar 1 Secretariat arranges a meeting with applicants
Pillar 1 Secretariat sends a guide to the EMA process to applicants
Weeks 2-3
Pillar 1 Secretariat hosts a meeting with applicants
Week 4
Mentorship session with the applicants
Weeks 5-8
Applicants work on briefing documentation
Expert Review of Briefing Package
Support from Pillar 1 Secretariat
End of Week 8
Applicants submit briefing documentation
Month 3-5
Applicants follow EMA process
Experts support in EMA process
Pillar 1 Secretariat support in EMA process
Month 6
Applicants update application

Pillar 2: Methodological Support – Coordinated by c4c-S.

Pillar 2 aims to provide access to state-of-the-art methodological expertise, enabling each application to develop the most appropriate research question, study design, and analytical strategy.

Each application will be supported by independent, conflict-free methodology experts, identified and appointed by the Pillar 2 Secretariat, and carefully matched to the specific methodological needs of each application.

These experts may overlap with experts in Pillar 1.

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Steps of Pillar 2 after Short Proposal selection (informed by Clinical Trial Call Secretariat)

1. **Preparation and onboarding of applicants by the Pillar 2 Secretariat** (*Week 1*) - The Secretariat organises an the scoping meeting with applicants.
2. **Scoping Meeting between the Pillar 2 Secretariat and applicants** *(Weeks 2–3)* - An initial meeting to conduct a needs assessment, define the scope of methodological support, and identify the specific requirements of each applicant team.
3. **Expert methodological review of applications** *(Weeks 5–8)* - Assigned methodology experts conduct an in-depth review of each application, assessing the soundness and appropriateness of the proposed methodology.
4. **Recommendations Meeting between the Pillar 2 Secretariat, assigned experts, and applicants** *(Week 8)* - A structured meeting in which experts present their methodological findings and recommendations, followed by an open discussion with the applicant team to clarify and prioritise next steps.
5. **Applicants work on methodology revisions** *(Months 3–5)* – Applicants implement the recommended methodological improvements to their proposals, drawing on the expert feedback received.
6. **Follow-up meeting between the Pillar 2 team, assigned experts and applicants** *(Month 5)* - A follow-up session to review progress on methodology revisions, address any remaining questions, and provide additional guidance where required.
7. **Applicants finalise and update their application** *(Month 6)* - Applicants incorporate all methodological revisions and submit the updated version of their proposal ahead of the final deadline.

- Advice will also be available during Months 3, 4, 5 through e-mail discussion or video/teleconferences as appropriate to each application.

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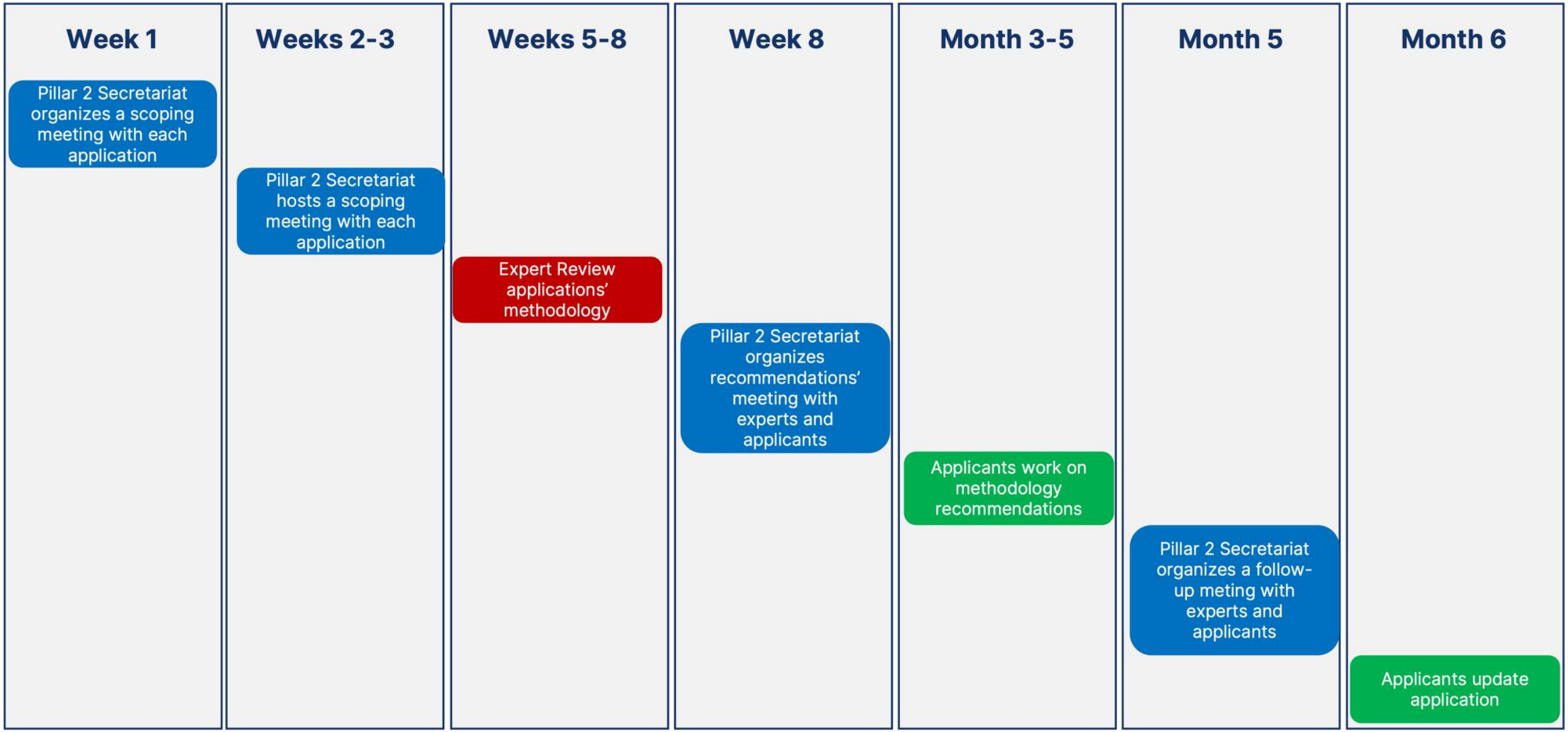
Week 1
Pillar 2 Secretariat organizes a scoping meeting with each application
Weeks 2-3
Pillar 2 Secretariat hosts a scoping meeting with each application
Weeks 5-8
Expert Review applications' methodology
Week 8
Pillar 2 Secretariat organizes recommendations' meeting with experts and applicants
Month 3-5
Applicants work on methodology recommendations
Month 5
Pillar 2 Secretariat organizes a follow-up meting with experts and applicants
Month 6
Applicants update application

Pillar 3: Support for Patient and Public Involvement (PPI) - coordinated by eYPAGnet, hosted by HSJD.

- Applicants are expected to initiate Patient and Public Involvement (PPI) during the application stage and to maintain and further develop these activities throughout the funded project. The Pillar 3 Secretariat provides structured support across both stages to ensure early integration and effective implementation of PPI.
- The objective of this support is to strengthen the relevance, feasibility, and inclusiveness of proposals by embedding meaningful patient involvement from the outset. During the application stage, the focus is placed on assessing existing PPI approaches, identifying gaps, and providing tailored recommendations to improve study design, patient engagement, and overall impact.
- Each application will benefit from the input of independent, conflict-free PPI experts, identified and appointed by the Pillar 3 Secretariat, and selected to match the specific needs and context of the proposal. These experts provide bespoke, practical guidance to support applicants in integrating high-quality and impactful PPI approaches.

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Steps of Pillar 3 after Short Proposal selection (informed by Clinical Trial Call Secretariat)

1. **Preparation and onboarding of applicants by the Pillar 3 Secretariat** (*Week 1*) - The Secretariat organises the scoping meeting with applicants. Applicants are expected to share relevant materials in advance (e.g. PPI plan, draft proposal, shortlisting scores).
2. **Scoping Meeting between applicants and PPI experts** (*Month 1*) – The discussion focuses on the existing PPI strategy, knowledge of the patient population and organisations, target population and recruitment plans, budget allocation, stage of protocol development, and expectations regarding patient involvement.
3. **Recommendations Meeting between PPI experts and applicants** (*Month 2*) – A dedicated meeting is organised for each application during which PPI experts present tailored recommendations. These may cover strategies for engaging individuals with lived experience and patient organisations, approaches for meaningful patient involvement across study phases (protocol development, design, endpoints, documentation, PROMs/PREMs, recruitment, dissemination), as well as methods to improve diversity, inclusion of underserved populations, evaluation of PPI impact, and communication of results to participants.
4. **Applicants work on PPI recommendations** (*Months 3–5*) – Applicants implement the PPI recommendations. Additional PPI-related refinements are undertaken as part of protocol finalisation.
5. **Final Review Meeting between PPI experts and applicants** (*Optional, Month 5*) – An optional follow-up meeting is organised to review how recommendations have been addressed, provide final feedback, and support applicants in refining their PPI approach before submission.
6. **Applicants update application** (*Months 6*) – Applicants implement the PPI recommendations and integrate them into their proposals.

Advice will also be available during Months 3, 4, 5 through e-mail discussion or video/teleconferences as appropriate to each application.

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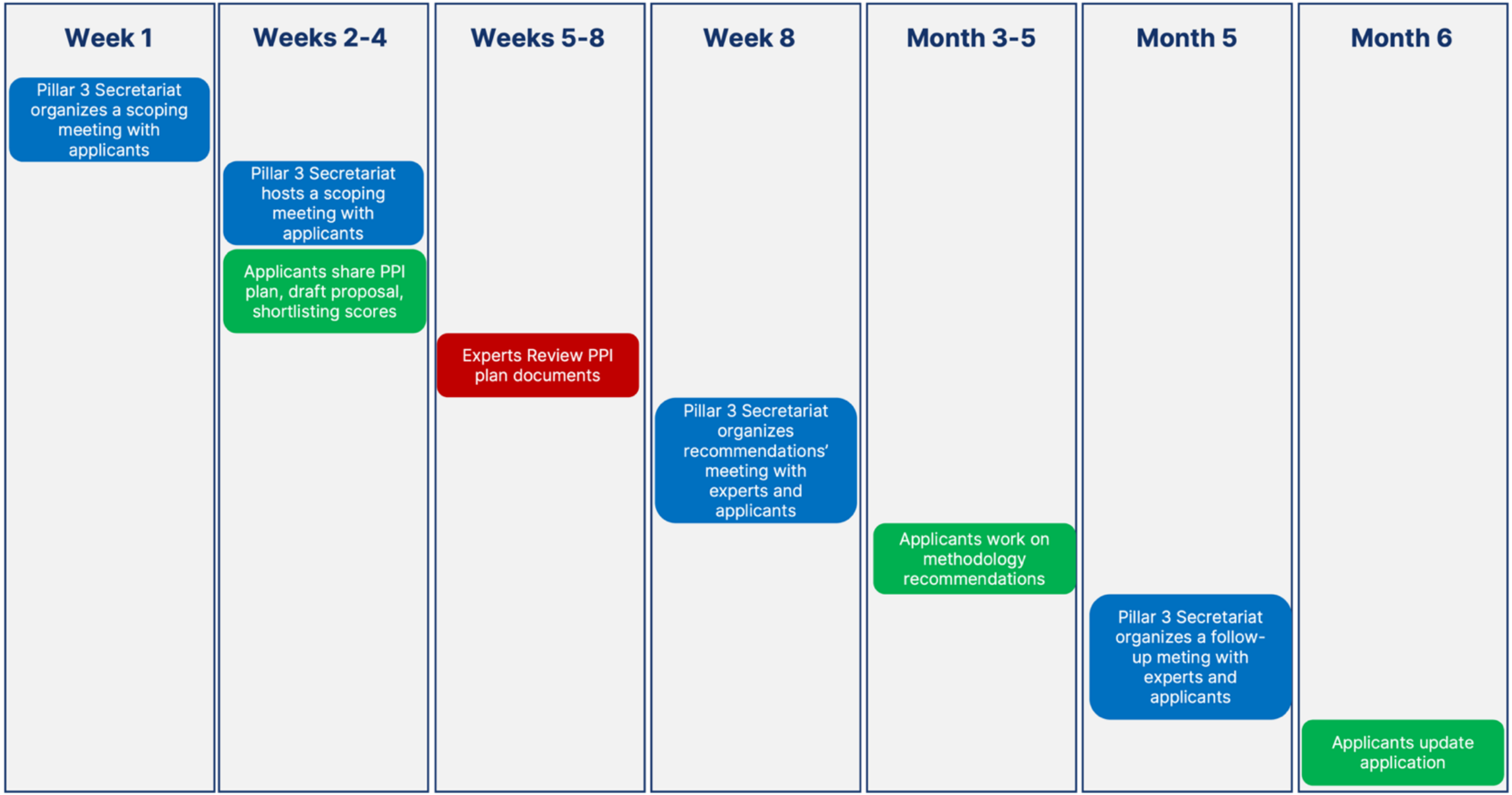
Week 1
Pillar 3 Secretariat organizes a scoping meeting with applicants
Weeks 2-4
Pillar 3 Secretariat hosts a scoping meeting with applicants
Applicants share PPI plan, draft proposal, shortlisting scores
Weeks 5-8
Experts Review PPI plan documents
Week 8
Pillar 3 Secretariat organizes recommendations' meeting with experts and applicants
Month 3-5
Applicants work on methodology recommendations
Month 5
Pillar 3 Secretariat organizes a follow-up meting with experts and applicants
Month 6
Applicants update application

Ground Rules

- The PSS team and experts will not contribute to writing the applications or add missing material but will comment on materials provided by the application teams.
- The PSS team will take a pragmatic approach to arranging meetings. For example, each Pillar will have a specified contact point. Each application will have a single contact person for each pillar. Communication will only flow through these contact points. Meetings with application teams will take place within specified time windows and will not be delayed until all investigators from an application team are available.
- The PSS and expert contributions will be delivered on a “best efforts, as-is” basis with no warranties for accuracy or success of the trial.
- The PSS and expert contributions will be constructive and collegial.
- The shortlisted applications will be under no obligation to follow any of the recommendations made by the PSS.
- PSS and expert contributions will be aligned with the evaluation criteria of the call.

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Additional points

- A detailed recording explaining the support stage and EMA Scientific Advice process will be sent in July to applicants, to make sure the PSS reaches everyone.
- Please review the EATRIS training material prepared together with EMA:

https://www.youtube.com/watch?v=MPlcOXbW3nk

See also:

https://www.ema.europa.eu/en/human-regulatory-overview/research-development/scientific-advice-protocol-assistance

- General clarification questions from applicants about the proceses of study support, including Scientific Advice, can be addressed during the EoI period. No advice will be provided on specific project proposals before evaluation.

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