Fondazione Regionale per la Ricerca Biomedica

EUROPEAN JOINT PROGRAMME RARE DISEASES

21 Gennaio 2022
Zoom Meeting

Regione Lombardia

European Joint Programme on Rare Diseases Joint Transitional Call (JTC 2022)

- Scopo del Bando
- Valutazione delle proposte
- Regole amministrative
- Regole di FRRB
- Pre-proposal template
- Calendario
- *www.ejprarediseases.org*

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Scopo del bando

Focus: Gruppo di malattie rare o singola malattia rara

(secondo la definizione Europea: patologia che affligge non più di 5 ogni 10.000 persone)

Scopo Specifico:
"Development of new analytic tools and pathways to accelerate diagnosis and facilitate diagnostic monitoring of rare diseases"

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Topic list

Diagnosi basata sul fenotipo: integrazione tra diverse ontologie, integrazione di pathway condivisi, fenotipizzazione digitale, sviluppo di approcci/applicazioni di intelligenza artificiale per estrarre dati relativi alla salute a supporto alla diagnosi.

Indagini di **marcatori prognostici/biomarcatori per la diagnosi precoce e il monitoraggio**

Metodologie per risolvere casi attualmente difficili da analizzare, a causa di diversi meccanismi sottostanti (ad es. mosaicismo, alterazioni genomiche (non codificanti), regolazione genica, ereditarietà complessa), comprese nuove tecnologie di genomica, multi-omica, matematica, biostatistica, bioinformatica e approcci di intelligenza artificiale.

Strategie per stratificare globalmente varianti di significato sconosciuto (VUS) per uso clinico; messa a punto di sistemi (in vitro) per distinguere tra VUS e varianti patogene.

Sviluppo di modelli di pathway per consentire la diagnosi, in particolare per malattie scoperte di recente che possono condividere i meccanismi molecolari sottostanti con malattie già note.

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Elementi da considerare nella stesura della proposta

- È possibile utilizzare **modelli cellulari e animali** per la validazione dei nuovi approcci diagnostici previsti nella topic list.
- **Il disegno dello studio** (raccolta di campioni, potenza statistica, etc.) deve essere ben giustificato e deve essere parte integrante della proposta;
- Per **natural history studies e registri dei pazienti**: devono essere incluse strategie e tempistiche per il reclutamento, la valutazione e l'analisi dei pazienti. I dati a supporto dei numeri di reclutamento proposti sono obbligatori. Il disegno e gli obiettivi dello studio dovrebbero prendere in considerazione quali informazioni sulla popolazione sono necessarie. (Considerazione su elementi di dati comuni sono delineate nella recente pubblicazione "Set of Common Data Elements for RD Registration");
- **L'integrazione di adeguate competenze bioinformatiche e statistiche** dovrebbe costituire, se richiesta, parte integrante della proposta e il personale dedicato dovrebbe essere chiaramente specificato;
- Le proposte dovranno considerare **l'impatto di sesso e/o genere** sull'attività di ricerca. I candidati sono incoraggiati a visitare la pagina CIHR's Sex, Gender and Health Research resource page per considerazioni sull'adeguata integrazione di elementi di sesso e genere nella proposta;
- Data management plan che rispetti i **principi FAIR** (Findable, Accessible, Interoperable, Reusable)
- **Open Access**

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Approcci ed argomenti esclusi dalla JTC2022

- **Sperimentazioni cliniche interventistiche per dimostrare l'efficacia** di farmaci, trattamenti, procedure chirurgiche, procedure di tecnologia medica (esclusi anche studi che confrontano l'efficacia e gli studi clinici di farmacovigilanza di fase IV).

- Studi per testare esclusivamente la **sicurezza dei dispositivi medici.**

- **Sviluppo di nuove terapie**, come già trattato nella JTC 2020.

- **Progetti incentrati solo su malattie neurodegenerative rare** che rientrano negli obiettivi Joint Programming Initiative on Neurodegenerative Disease Research (JPND):
  - Alzheimer e altre demenze;
  - Morbo di Parkinson e disturbi correlati;
  - malattie da prioni;
  - malattie dei motoneuroni;
  - Malattia di Huntington;
  - Atrofia muscolare spinale e forme dominanti di atassia spinocerebellare.

  IMPORTANTE: le demenze/malattie neurodegenerative infantili non sono escluse.

- **Malattie infettive rare, tumori rari ed eventi avversi rari da farmaci impiegati nel trattamento di malattie comuni.**

  IMPORTANTE: Non sono escluse le malattie rare con predisposizione al cancro.

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Management e revisione

La valutazione coinvolge 3 organi distinti:

Joint Call Secretariat (JCS)

Carlos III Health Institute
ISCIII, Spain
Ignacio Baanante
ibaanante@isciii.es
Maria Druet
mdruet@isciii.es

Call Steering Committee (CSC)

(1 rappresentante per ogni Funding Agency che partecipa alla Call)

- Definisce le procedure della Call
- Dà la raccomandazione finale sui progetti da finanziare sulla base della graduatoria

Scientific Evaluation Committee (SEC)

(panel di scienziati indipendenti riconosciuti a livello internazionale)

Valutazione dei progetti

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Evaluation and Scientific revision

Criteri di valutazione:

- **Eccellenza** (0-5)
- **Impatto (**0-5)
- **Qualità ed efficienza dell'implementazione** (0-5)

Threshold per criterio=3
Threshold complessiva=12
Max score totale=15

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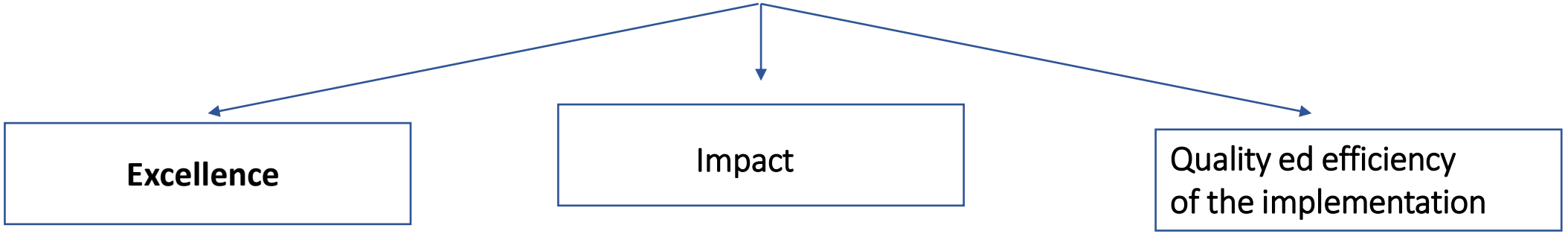
Evaluation and Scientific revision

Criteri di valutazione



- Clarity and pertinence of the objectives
- Credibility of the proposed approach and methodology
- Soundness of the concept
- Innovative potential
- Feasibility of the project
- Competence of partners in the field(s) of the proposal
- Active and meaningful participation of PAOs and patient representatives in the project

- Potential of the expected results for exploitation and for future clinical, public health and/or other socio-economic health relevant applications, including patient's needs,
- Added value of transnational collaboration: gathering a critical mass of patients/ material, sharing of expertise and resources, harmonization of data, sharing of specific know-how and/or innovative solutions,
- Effectiveness of the proposed measures to exploit and disseminate the project results (including management of IPR), to communicate the project, and to manage research data. A data management strategy in the full proposal is mandatory,
- Inclusion of Early Career Researchers as full partners,
- Benefit to patients, their families, and carers with an active and meaningful involvement of patient organizations and patient representatives,
- Involvement of industry (when appropriate/applicable/available).

- Coherence and effectiveness of the work plan, including appropriateness of the allocation of tasks, resources and timeframe,
- Complementarity of the participants within the consortium, including the integration of PAOs where possible
- Appropriateness of the management structures and procedures, including risk management, contingency plans and innovation management
- Plan for sustainability of infrastructures or resources initiated by the project
- Budget and cost-effectiveness of the project (rational distribution of resources in relation to project's activities, partners' responsibilities and time frame)

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Evaluation and Scientific revision

Criteri di valutazione

Inclusion of Early Career Researchers as full partners

La definizione di ECRs segue le regole dell' European Research Council criteria for starting grants:

-Il ricercatore deve avere conseguito il dottorato (PhD) da 2 a 7 anni prima della deadline per l'invio della proposta.
(Estensioni permesse per interruzioni giustificate e documentate, quali maternità, paternità, malattie, servizio militare obbligatorio).

-La specialità medica (MD) non è equivalente al PhD.
Gli applicants con MD dovranno avere anche il PhD o di incarichi che richiedano esperienza equivalente al dottorato (es. borsa di studio post-dottorato o assegnazione di cattedre).

-I candidati con **MD** (ed esperienza equivalente al dottorato) devono aver ottenuto la specialità **da 4 a 9 anni prima della scadenza per la presentazione della proposta.**

-Per i medici che hanno conseguito sia la specialità medica (+ esperienza equivalente al dottorato) che il dottorato, verrà considerato il titolo conseguito prima.

- Sono previste estensioni di tale periodo (con documentazione) per periodi di attività clinica fino a un massimo di quattro anni.

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Evaluation and Scientific revision

Il processo di valutazione si articola in 2 fasi:

STAGE 1 Pre-proposal → **STAGE 2 Full-proposal**

STAGE 1 Pre-proposal

Le proposte che hanno passato l'eligibility check sono valutate da 2 membri del **SEC**

↓

Consensus meeting del **SEC** e graduatoria delle pre-proposal

↓

Meeting del **CSC** e decisione finale sulle pre-proposal

↓

Invio delle valutazione/raccomandazioni del SEC agli **applicants**

In questa fase gli underrepresented or undersuscribed countries possono unirsi alle proposal

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Evaluation and Scientific revision

STAGE 2
Full-proposal

Presentazione del Full proposal

Invio ad almeno **2 revisori specializzati** (esterni al SEC)

Invio delle valutazioni al **coordinatore** per il REBUTTAL ← OPZIONALE

Valutazione+rebuttal al **SEC** per definire la graduatoria finale in un consensus meeting
(Patient reviewer and statistical evaluation)

Valutazione etica di esperti indipendenti

DECISIONE FINALE: Il CSC prende atto della graduatoria del SEC e si incontra per stabilire quali progetti verranno finanziati
(in base alla graduatoria, ai fondi disponibili, alle malattie trattate,
al coinvolgimento degli underrepresented countries e dei PAOs)

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Regole amministrative

The maximum duration of the project is three years.

Attenzione alle regole nazionali e regionali!

Eligible partners:

- academia
- clinical/public health sector
- enterprise (all sizes of private companies). Participation of small and medium size enterprises (SMEs) is encouraged when allowed by national/regional regulations
- patient advocacy organisations (PAO)

The inclusion of a non-eligible research partner (principle investigator) in a proposal leads **to the rejection of the entire proposal** without further review.

Consortia:

Each consortium submitting a proposal must involve **four to six eligible principal investigator partners** (referred to as partners below) from at least **four different participating countries**. In specific cases this can be increased to eight partners.

No more than two eligible partners from the same country.
PAOs requesting funding do not count toward the total.

The number of partners can be increased to 8 in two cases:

1. The inclusion of partners from participating countries usually underrepresented in projects (Slovakia, Hungary, Lithuania, Poland, and Turkey).
2. The inclusion of Early Career Researchers as full partners

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Regole amministrative

Each partner (with their respective research groups) must contribute substantially to at least one of the project work packages. Consortia may include collaborators that secure their own funding. **Collaborators** do not count toward the limit of 8 partners requesting research funding.
Each transnational proposal must nominate a **project consortium coordinator** among the project partner principal investigators. The coordinator must be an eligible project partner from an EJP RD JTC 2022 funding country/region.

Double funding of research projects is not permitted.

Consortia are strongly advised to include patient representatives and patient advocacy organizations (PAOs), which are eligible to receive funding for their activities – see list of organizations suggested.
FRRB does not fund PAO

Verificate le risorse suggerite

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Regole amministrative

Research consortia who intend to submit a transnational project proposal should register via the electronic proposal system:
https://ptoutline.eu/app/ejprd22
Please fill in the data sheet in the system.

Two-stage submission procedure for joint applications:

- pre-proposal
- full proposal.

One joint proposal document (in English) shall be prepared by the partners of a joint transnational proposal, and must be submitted by the coordinator **only** to the JCS via the electronic submission system. The proposals must strictly follow the instructions in the proposal form.

In general, no fundamental changes between the pre- and full proposals concerning the composition of the consortia, objectives of the project or requested budget will be accepted. The CSC, however, may allow such changes if detailed justification is provided to the JCS.

Verificate con le Funding Agencies di riferimento

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Regole amministrative

Eligibility check

The JCS will check all pre-proposals to ensure that they meet the call's formal criteria. The JCS will forward the proposals to the CSC members who will perform a check for compliance to country/regional/PAO eligibility rules. **Please note that proposals not meeting the formal criteria or the national/regional eligibility criteria and requirements will be declined without further review.**

EJP RD Mentoring programme

Applicants that are invited to submit a second stage proposal are strongly encouraged to make use of the EJP Rare Diseases Mentoring Programme. This completely free service offered by EJP RD matches your project with mentors that have expertise in applied research and translational development

Applicants should indicate in the pre-proposal form if they wish to make use of the Mentoring service. If they are invited to full proposal submission, the Mentoring team will soon after contact the applicants to schedule a planning call, after which mentors will be assigned to the project based on the individual needs.

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Regole amministrative

Widening for the inclusion of under-represented or under-subscribed countries

For proposals invited to the full proposal stage, there will be a widening step to provide the opportunity to add partners to the consortium (up to a maximum total of 8, see section 5.4 Consortium Makeup of the Call Text). This step will allow for the addition of partners from participating countries that are usually underrepresented in the call, as well as those undersubscribed (countries without any selected applicants for the 2nd stage)

This inclusion will not be considered as a fundamental change between pre- and full proposal. Inclusion of new research teams is not mandatory. The new teams included should bring an added value and expertise to the projects.

A list of countries eligible for this widening procedure will be published on the EJP RD website after completion of the 1st stage of evaluation and sent to the coordinators that are invited to write a full proposal.

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Regole di FRRB

Country	Italy
Funding organization	Fondazione Regionale per la Ricerca Biomedica - Regional Foundation for Biomedical Research (FRRB)
National contact person	Paola Bello, Marcello De Amico Via Taramelli 12, 20124 – Milano Tel: +39 02 67650174 bandi@frrb.it
Funding commitment	€ 1.500.000,00
Overheads	Up to 20% flat rate calculated on direct costs – Subcontracting costs excluded.
Anticipated number of fundable research partners	3-4
Maximum funding per grant awarded to a partner	Maximum € 500,000 **per project. MAXIMUM TWO PARTNERS FROM LOMBARDY PER PROJECT.** (If there are two Lombardy partners in the same consortium, the amount of 500,000 will be shared)
Eligibility of a partner as a beneficiary institution	**Eligible applicants:** • Public or Private Italian IRCCS (Scientific Institutes for Health Research, Hospitalization and Health Care) • Public Health Care Providers (ASST) • Universities (only in in partnership with one IRCCS, public or private, or ASST located in Lombardy and requesting funding to FRRB) • Research Institutes (only in in partnership with one IRCCS, public or private, or ASST located in Lombardy and requesting funding to FRRB) All applicants must be located in Lombardy and their activities should take place in Lombardy. Enterprises and for-profit Organisation are **NOT** eligible. **A Principal Investigator (PI) cannot simultaneously hold more than one FRRB active grant.** PIs who are currently FRRB grant holders cannot apply to the EJP RD JTC 2022 unless their project is closed before the deadline for EJP RD JTC 2022 pre-proposals. A project is considered closed when the final financial and scientific reports have been sent to FRRB. This rule applies only to PIs (grant holders), not to their team members.

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Eligibility of costs, types and their caps	Direct costs: •Personnel (for **public** IRCCS and ASST, **ONLY** staff recruited specifically on the project) •Consumables, animals purchase, maintenance and breeding. •Equipment (on hire or eligible amortization rate). •Travel: max 10% of the total direct costs (*overheads and subcontracting costs excluded*) •Publications (only open access): max 5% of the total direct costs (overheads and subcontracting costs excluded). •Overheads: 20% flat rate calculated on direct costs (Subcontracting costs excluded from this calculation). •Subcontracting: max 20% of the total direct costs (overheads costs excluded) •Other direct costs: please include here other costs, including those related to patient involvement (insurance, reimbursement, etc.). FRRB will require the submission of a financial audit certificate together with the final financial report. This cost, to be included under the "Subcontracting" category will be eligible up to a maximum of € 8.000. Only costs generated over the lifetime of the project will be considered eligible.
Conditions for PAO funding	PAO are not eligible for FRRB funding
Submission of the proposal at the regional level	It is not necessary to send the proposal to FRRB. However, FRRB requires a **Pre-eligibility form**. According to internal procedures, Regional Foundation for Biomedical Research (FRRB) will carry out an **eligibility check** to potential applicants prior to the submission of the pre-proposals. The eligibility check will be based on the verification of a dedicated form ("***Pre-eligibility form***"), also available on the FRRB institutional website, to be returned, by email, to FRRB (bandi@frrb.it), duly completed and signed by the Principal Investigator at least 10 working days before the pre-proposal submission deadline. FRRB will provide feedback on the "*Pre-eligibility form*", **ONLY** in case of major non-eligibility issues. Principal Investigators (PIs) who submit a proposal without sending the "*Pre-eligibility form*" to FRRB beforehand will be automatically excluded. In addition, FRRB provides an excel sheet to help applicants abide by FRRB funding rules. This form is meant to support the PIs in the elaboration of the proposal budget, but it does not need to be sent to FRRB. Information and instructions on how to fill the Pre-Eligibility check form will be published on the dedicated webpage (https://www.frrb.it/it/ejp-jtc-2022) Following the award, Lombardy beneficiaries will be requested to submit annual scientific and financial reports.
Further guidance	Administrative and financial guidelines will be provided by FRRB in due time to the contact persons of the funded organisations.

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Regole di FRRB

- **Leggere attentamente le guidelines:** importanti link, riferimenti e indicazioni sugli allegati.
- PRIMA DI INIZIARE, CONTATTATE SEMPRE IL VOSTRO GRANT OFFICE O PERSONALE AMMINISTRATIVO DEPUTATO
- Ciascun ente interessato a richiedere un contributo a FRRB nell'ambito di una Call dovrà obbligatoriamente inviare, all'indirizzo bandi@frrb.it il form di «**Pre-eligibility check form»** scaricabile dal sito internet di FRRB e dalla pagina ufficiale del singolo programma.
- Tale modulo dovrà pervenire agli uffici di FRRB **almeno 10 giorni lavorativi (2 febbraio 2022)** prima dalla deadline di presentazione del pre-proposal così da permettere alla Fondazione di verificare l'ammissibilità a partecipare alla Call degli enti proponenti.
- Soltanto nel caso in cui il controllo dovesse avere **esito negativo o dovessero essere necessarie maggiori informazioni** sarà cura di FRRB contattare l'ente interessato.
- Domande e quesiti dovranno essere inviati a bandi@frrb.it, specificando «EJP RD JTC 2022» nell'oggetto. Le domande, preferibilmente senza elementi identificabili, saranno pubblicate con la relativa risposta nelle **FAQ** pubblicate nella pagina web di FRRB dedicata alla call.

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Pre-proposal template

PRE-PROPOSAL APPLICATION FORM

EUROPEAN JOINT PROGRAMME RARE DISEASES

Call for Proposals 2022

"Development of new analytic tools and pathways to accelerate diagnosis and facilitate diagnostic monitoring of rare diseases"

Submission deadline for pre-proposals:
February 16th, 2022; 2 p.m. (CET)

Pre-proposal application form

Please note:

- Proposals that do not meet national/regional eligibility criteria and requirements will be declined without further review.
- Format is Century Gothic font size 11, single-spaced, with margins of 1.27 cm. Incomplete proposals, proposals using a different format or exceeding length limitations of any sections will be rejected without further review.
- Once completed, the pre-proposal must be converted in a single PDF document before being uploaded to the submission website.
- In case of inconsistency between the information registered in the submission tool and the information included in the PDF of this application form, the information in the application form shall prevail.
- The information given in the pre-proposal is binding. Thus, any fundamental change between the pre- and full proposals, e.g., composition of the consortia, objectives of the project, or the budget must be communicated to the JCS with detailed justification and will only be allowed under exceptional circumstances[1].
- *Text marked in Italics and highlighted in yellow can be deleted for proposal submission.*

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Project awarded!

FRRB: Successivamente all'approvazione della graduatoria, il Cda di FRRB riceve l'informativa dal Direttore generale e approva formalmente la graduatoria finale dei progetti ammessi e finanziati e non finanziati. Gli Enti interessati sono contattati da FRRB per le pratiche amministrative (accettazione del contributo, conferma da parte dell'ente finanziato che nell'ambito del progetto non si svolgano attività economiche, convenzione, modalità di erogazione...)

JCS: The project coordinator is the point of contact for consortia during the application procedure, and is responsible for forwarding relevant information from the JCS to their consortium members.

The **coordinators** of all funded projects must submit an **annual scientific project report** (due on the 28th of February 2024 and subsequent years) **and a final scientific project report** (due within six months of the end of the project). All reports must be in English and must use the reporting templates provided. The research partners are jointly responsible for delivery of the reports. Only reports delivered on behalf of the consortium, via the project coordinator, will be accepted.

Consortium members of projects selected for funding **must fix a common project start date**, which will be the reference date for yearly and final reports and extensions. This common project start date must also appear in the **Consortium Agreement (CA).**

The coordinators and national/regional group leaders will be asked to present the results of their projects at an **intermediate status symposium** organized by EJP RD. The presence of at least one representative (coordinator and/or partner) per project will be mandatory. Therefore, **the coordinator and respective partners must budget a sufficient amount for the expenses related to these events**.

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Timeline

16th December 2021	Information webinar for potential applicants
16th February 2022	Pre-proposal submission deadline
End of April 2022	Invitation to full proposal
15th June 2022	Full proposal submission deadline
28th July 2022	Deadline for rebuttals
December 2022	Notification of funding decision

Pre-eligibility check form: FRRB richiede a tutti i PI lombardi di presentare questo modulo, debitamente compilato e firmato almeno 10 giorni lavorativi prima della presentazione della pre-proposal (al più tardi il 2 febbraio)

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Domande?

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per la
Ricerca
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