



Call for Proposals for "European research projects on rare diseases"

Submission deadlines:

Electronic version: May 11th 2007 at 10 am (GMT)

Paper copies: May 18th 2007 (certified post with recorded delivery only)

Remember that a complete proposal must include the **Application Form, the Financial Plan Form and a letter of intent declaring the project partners willingness to collaborate.**

Please, consult the annex at the end of this document to obtain information concerning the specific national regulations that may apply to this call.

For further information, please visit us on the web (www.e-rare.eu) or contact Dr Beitia or Dr Borensztein at:

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1. Motivation

There are at least 6000 to 7000 distinct rare diseases, the great majority being of genetic origin. Although individually rare, taken together rare diseases affect at least 20 million people in Europe. Moreover, they represent a major issue in health care: a large number of these diseases lead to a significant decrease of life expectancy and most of them cause chronic illnesses with a large impact on quality of life and the health care system.

Therefore, further research on rare diseases is badly needed to provide knowledge for prevention, diagnosis and better care of patients. But research is hampered by lack of resources at several levels. (1) Few scientists work on one specific disease. (2) There are few patients scattered over a large geographic area, causing difficulties to gather the necessary cohorts. (3) Existing databases and material collections are usually local, small, and not accessible or standardised. (4) Diseases often have complex clinical phenotypes and require interdisciplinary approaches to treatment and interdisciplinary cooperation for research.

Due to the small number of patients affected by single specific disease and to fragmentation of research, rare diseases are a prime example of a research area that could strongly benefit from coordination on a transnational scale.

In this context, the ERA-Net for research programmes on rare diseases (E-Rare) has been established to coordinate the research efforts of European countries in the field of rare diseases. The Era-Net is coordinated by GIS-Institute for rare disease (France) with the participation of The National Research Agency (ANR, France), The Federal Ministry of Education and Research (BMBF, Germany), The PT-DLR project management agency (Germany), The National Institute of Health (ISS, Italy), The Chief Scientist Office of the Ministry of Health (CSMOH, Israel), The Institute of Health Carlos III (ISCIII) – Fund for Health Research (FIS) / Institute for Research on Rare Diseases (IIER) (Spain), The Scientific and Technological Research Council of Turkey (TÜBİTAK, Turkey).

The following partners,

- The National Research Agency (ANR), France
- The Federal Ministry of Education and Research (BMBF), Germany
- The Chief Scientist Office of the Ministry of Health (CSMOH), Israel
- The National Institute of Health (ISS), Italy
- The Institute of Health Carlos III (ISCIII), Spain
- The Scientific and Technological Research Council of Turkey (TÜBİTAK), Turkey

have decided to open the first joint call for funding multilateral research projects on rare diseases. The call is being opened simultaneously by the PARTNERS in their respective countries.

2. Aim of the call

The aim of the call is to enable scientists in different countries to build an effective collaboration on a common research project based on complementarities and sharing of expertise. Projects shall involve a group of rare diseases or a single rare disease following the European definition i.e. a disease affecting not more than five in 10.000 persons in the Community. Rare infectious diseases, rare cancers, rare adverse drug events are excluded from the topics of this call.

Transnational research proposals must cover at least one of the following areas:

a) Collaborative research using or constituting cohorts/case series of patients/families and corresponding collections of biological material that would generally not be possible at a national scale. This research must have well defined scientific objectives e.g.:

- Definition of new nosological entities, epidemiological studies, genotype/phenotype correlations.
- Characterization of the genetic/molecular basis of specific diseases.

b) Basic research on rare diseases including genetic and pathophysiological studies, using innovative and shared resources, technologies and expertises. The clinical relevance of this research must be clearly demonstrated.

c) Research on diagnosis and therapies for rare diseases. This may include identification and characterisation of biological targets, development of innovative screening systems, generation of relevant cellular and/or animal models and gene or cell therapies.

d) Patient oriented research in the area of social and human sciences - e.g. psychological, psychosocial and behavioural research – as well as health services research and health economy research in the field of rare disorders.

Clinical trials are excluded from the scope of the call.

Project proposals must clearly demonstrate the potential health impact and the added-value of transnational collaboration: gathering a critical mass of patients/biological material, sharing of resources (models, registries, diagnosis etc.), harmonization of data, sharing of specific know-how and/or innovative technologies.

3. Application

3.1 Funding recipients

Joint research proposals may be submitted by higher education institutions, non-university public research establishments, hospitals, patient organizations as well as commercial companies, in particular small and medium-size enterprises (SMEs), according to relevant national funding agencies' regulations for research funding.

Only transnational projects will be funded. Each proposal must involve a minimum of two research groups from two different participating PARTNER countries. As the designated amount of funding for research groups from Israel is very limited and only sufficient for one or two research projects, consortia who would like to include research groups from Israel in their project are advised to contact the representative of their national research funding agency for further advice. Additionally, the research groups from Israel should contact their national administration for further information.

The number of participants should be appropriate for the aims of the research project and reasonably balanced in terms of national participation. Each collaborative project should represent the critical mass to achieve ambitious scientific goals and should clearly demonstrate an added value from working together.

Special attention will be given to the quality of patient's recruitment, biometric/statistical methods, phenotyping and collection of biological resources with regards to the objectives of the project. For linkage analysis, sufficient genealogical information should be given in the application.

Each project must nominate a project coordinator, who represents the consortium externally and is responsible for its internal management (such as controlling, reporting, IPR issues). Within a joint proposal, each group leader will be the contact person for the relevant national funding agency/organization.

Research groups from non-PARTNER countries may participate in projects if they secure their own funding. They have to state clearly in the proposal if these funds are already secured or how they plan to secure these funds until the project start. If the contribution of the third party is considered essential for the success of the entire proposal, the third party will be required to document the availability of their funds before the final funding decision.

3.2 Financial and legal modalities

Funding is granted for a maximum of three years according to national regulations. Eligible costs may vary according to the corresponding national funding agency regulations. Each group is subject to the rules and regulations of the respective funding agency.

3.3 Submission of joint proposals

Joint proposals (in English) must be submitted to the Joint Call Secretariat in an electronic version **no later than 11.05.2007 at 10 am GMT (12 pm Paris summer time)** and in a signed paper version **(1 original + 2 copies) no later than May 18th 2007**. Paper copies must be sent by **certified post with recorded delivery**. Further information on how to submit proposals electronically will be made available through the E-Rare website. The forms that have to be used for submission of proposal are available on the E-Rare website. For proposals including Spanish research groups, in addition to this forms the Spanish part has to be submitted concurrently to the Spanish funding agency (for details, see the Spanish national call text). **Complete proposals** must comprise the **Application Form, the Financial Plan Form and a letter of intent declaring the project partners willingness to collaborate if the project is approved**. Proposals must include the following information:

- Names and full affiliations of the group leaders and other personnel participating in the joint project. Indication of the project coordinator designated by the consortium to act as its representative.
- Project summary (max. ½ page)
- Background and present state of the art in the research field (max. 2 pages)
- Work plan (aims, methodology, involvement of participants, time plan, project coordination and management; max. 8 pages)

- Added value of the proposed collaboration (max. 1 page)
- Possible exploitation of expected project results (max. ½ page)
- Present and anticipated future position with regard to IPR (e.g. any barriers to sharing materials or results), both within and outside the consortium, if applicable (max. ½ page)
- Description of patents and ongoing projects of each participating group related to the present topic, indicating funding sources and possible overlaps with proposal (max. ½ page per group)
- Justification of requested budget (if applicable, also specifying co-funding from other sources necessary for the project; max. 1 page)
- Ethical and legal issues (e.g. informed consent, data protection, use of animals), if applicable, according to national regulations
- Brief CVs for each participating group leader with a list of up to five relevant publications within the last five years demonstrating the competence to carry out the project (max. 1 page each)
- Financial plan for each consortium member (on form provided on the E-Rare website).

Applicants are invited to name potential experts suited for the evaluation of their proposal as well as experts not suited due to conflict of interest.

3.4 Further information

If you need additional information, please contact the Joint Call Secretariat, or your national funding agency representative (see annex or www.e-rare.eu).

4. Evaluation

The evaluation of the joint project proposals will be organised as follows:

Formal check of proposals: The Joint Call Secretariat, together with the national PARTNERS, will assess all proposals to ensure that they meet the call's formal criteria (date of submission; number of participating countries; inclusion of all necessary information in English; appropriate limits on length) and that applicants are eligible to submit applications according to national rules. Proposals not meeting the formal criteria will be declined.

Peer-review of proposals: The Scientific Evaluation Committee will carry out a scientific evaluation based on international scientific expertises using specific evaluation criteria (see below) and a common *Evaluation Form*. A scoring system from 0 to 5 will be used to evaluate the proposal's performance with respect to the different evaluation criteria: Evaluation will be carried out in two steps: 1) selection of proposals for external review by the Scientific Evaluation Committee and 2) discussion of external reviews and final funding recommendations

0: fails or missing/incomplete information; 1: poor; 2: fair; 3: good; 4: very good; 5: excellent

The point scores of the criteria have different weights, reflecting the fact that some criteria are more important for the overall assessment than others. The weights are given below in brackets.

1. Relevance to the aim(s) of the call (weight:1)
2. Scientific quality of the proposal (innovative potential, adequate methodology) (weight:2)
3. Feasibility of the project (adequate budget, resources, time schedule) (weight:1)
4. International competitiveness of participating research groups in the field(s) of the proposal (previous work in the field, expertise of the research groups) (weight:1)
5. Level of collaborative interaction between the groups and added value of the transnational consortium (weight:2)
6. Potential of the expected results for future clinical and other health relevant applications (weight:1)

The Scientific Evaluation Committee will establish a ranking list of the proposals.

Decision: Based on the ranking list, the Transnational Call Steering Committee will suggest the projects to be funded. Based on these recommendations, final decisions will be made by the national funding agencies.

Before a project may start, it is recommended that a Cooperation Agreement should be signed by all project members (addressing the issues given in *Information for Applicants on cooperation agreements*, see E-Rare's website). On request, the Cooperation Agreement must be made available to the national funding agency, together with any other information required by national regulations.


5. Reporting requirements

Each project coordinator, on behalf of all participating teams, should submit to the Parties a brief annual scientific progress report on the project (in English). In addition, each team will have to report to its funding Party, in accordance with the respective national funding agency's regulations.

Annex

Please, notice that country specific requirements might apply to this call. For further information follow the links below or contact your national representative:

Country	Contact Person	Country specific requirements
France	Dr. Igor Beitia Ortiz de Zárate GIS-Institut des Maladies Rares Phone: +33 1 58 14 22 85 E-mail: secretariat@e-rare.eu	Please, contact us to get further information on ANR's specific requirements for this call or visit the ANR web site: (www.agence-nationale-recherche.fr)
Germany	Dr. Ralph Schuster PT-DLR Phone: +49 228 382 12 33 E-mail: ralph.schuster@e-rare.eu	http://www.gesundheitsforschung-bmbf.de/de/1556.php
Israel	Dr. Zelina Ben-Gershon CSO/MOH Phone: +972 2 568 12 09 E-mail: zelina.ben-gershon@e-rare.eu	Please, contact us to get further information on CSO/MOH's specific requirements for this call.
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