



No.: 72/01/2021-RD/BMS

Date: 12/12/2022

## Call for Proposals on “Therapeutics for Inherited Rare Diseases”

### Background:

Inherited rare diseases are infrequently occurring disorders and have been defined by countries in terms of prevalence in its own population. WHO defines rare diseases as often debilitating lifelong disorder with a prevalence of 1 or less, per 1000 population. Only 5% of inherited rare disorders have treatment among which most of the treatments are highly priced and often not available worldwide. Availability and access to medicines are important to reduce morbidity and mortality associated with rare diseases. Therefore, there is a need for research and development in intervention, technology transfer and indigenously manufactured therapeutics for rare diseases which could be achieved by collaborative efforts.

### Purpose of the call:

The goal of this call is to support research and development research proposals towards developing intervention strategies on the following aspects of inherited rare diseases<sup>a</sup>-

- Discovery of newer therapeutic agents and approaches<sup>b</sup>
- Development of therapeutics including-
  - Repurpose Drugs
  - Biologics
  - Genetic error corrective measures
  - Management to improve quality of life of patients with rare diseases such as orthopedic/ surgical approaches, innovative management protocols etc.

<sup>a</sup> Inherited rare diseases under the following groups will **ONLY** be considered -

1. Storage disorders
2. Small Molecule Inborn Errors of Metabolism
3. Primary Immunodeficiency Disorders (PID)
4. Neuromuscular Disorders (NMD)
5. Hematological disorders (excluding Sickle cell diseases and Thalassemia)
6. Skeletal dysplasia

<sup>b</sup> Approaches may include development of resources such as cellular and animal models, vector for genetic error corrective measures, mechanistic aspects that are required for drug development *as a part of the project*.

### Scope:

Research Groups working or having expertise in discovery and development of drugs/therapies are encouraged to apply. *Collaborations are encouraged. \*An outstanding project requiring funding more*



*than 1.5 Crore/study, maybe converted to a Task force by ICMR, wherein the proposed PI will be the PI (single centric study) or will be one of the PIs (multicentre study).*

### **Deliverables:**

These projects are time-bound with a goal-oriented approach and clearly defined targets, specific time frames. The proposed research and development should be a potential therapeutic molecule (novel/ repurpose drug) or strategies to improve quality of life of patients with rare diseases. Proposals in multidisciplinary collaborative mode will be encouraged.

### **Exclusions:**

This call excludes proposals focused on:

- Descriptive studies
- Other systems of medicines
- Rare cancers
- Early stage of development i.e., *in-silico* and *in-vitro* **ONLY**
- Biomarker
- Registry/ Patient Database

### **Who Can Submit Project Proposal:**

The Project Proposal should be submitted for financial support through **ONLINE MODE ONLY (application format available on Annexure -1 )** by Scientists/ professionals who have a permanent employment in Medical Institutes/ Research Institutes/ Universities/ Colleges/ recognized Research & Development laboratories/ Government and semi-government organizations and NGOs (documentary evidence of their recognition including DSIR certificate should be enclosed with the proposal). He/ She should have relevant experience in the field as evident from publications of past three years. The proposal must have at least one Co-PI from the host institute.

### **Specific points for submission of project proposals:**

- The proposal should cover achievable targets, specific time frames, justifiable budget requirement and engagement with prospective technology transfer partners or technology transfer facilitating bodies/Technology Business Incubators. Investigators should mention if they are likely to have an intellectual property from their study.
- Proposal should be focused on outcomes that can be translatable.
- The host institutions should be well-equipped and have the essential equipment and infrastructure for the proposed development. Only minor equipment may be supported based on requirement of the proposed objectives.
- Incomplete applications will be rejected.



- The projects should be time-bound normally for duration of 2-3 years.
- Evaluation will be based on the following:
  - significance of the proposal,
  - innovation/ novelty,
  - project description,
  - research team's profile, and
  - infrastructure
- Monitoring of the Project: Implementation of the projects is monitored annually through review of progress reports based on the achieved targets within proposed timeline by expert Committee.

**Timeline:**

- Launch of call for full proposals on ICMR website: **12th December 2022**
- Full project submission ends: **25th January, 2023**

*Interested PIs can join the webinars that will be organized by ICMR in a week after the release of call (tentatively 20<sup>th</sup> December 2022). If required, another webinar will be scheduled one week before the last date of submission of projects, (tentatively 20<sup>th</sup> January 2023) to discuss related queries.*



## How to apply:

1. Before proceeding to submit the proposal, it is suggested to read user manual and ICMR Extramural Research Program guidelines; and make ready all relevant information, documents and research plan (<https://epms.icmr.org.in/adhoc>).
2. Open the ICMR Electronics Project Management System (e-PMS) portal <http://epms.icmr.org.in>. The user manual of e-PMS (under Guidelines → e-PMS manual) is available at the portal.
3. Project proposal submission is three steps process in e-PMS:
  - Step 1: PI registration/ Login (<https://epms.icmr.org.in/userLogin>)
  - Step 2: Verify email ID and complete/ update PI profile
  - Step 3: Proposal submission
4. Click on “LOGIN” and select “Register” for new registration OR if already registered, provide details to login and enter into e-PMS portal.
5. Verify your registered email and complete the PI profile.
6. After completing mandatory section of PI profile, click on “Proposal submission → Click on Submit new proposal → Select the program from drop-down box as "Adhoc" → Fill the form step by step (Please select Broad Area: BMS, Advertise/Call: Call for Proposals on Therapeutics for Inherited Rare Diseases)
7. There is no separate budget head for consumable. Expense for consumable can be submitted under contingency budget head only along with item wise proper justification. It is advised to read carefully the budget provision available at Guideline for Extramural Research Programme of ICMR.
8. Overhead charge will be as per ICMR order F. No. Sr.ACO/Misc.4(86226) dated 22/09/2020

## For any queries related to the call, please contact:

Proposal Submission related query	Program related query
<b>Dr. Lokesh Sharma</b> Scientist E and Program Officer Division of BMI, ICMR Headquarters, V Ramalingaswami Bhawan, Ansari Nagar, New Delhi-110029 <i>Email: <a href="mailto:po.epms@icmr.gov.in">po.epms@icmr.gov.in</a></i>	<b>Dr. Monika Pahuja</b> Scientist D and Program Officer Division of BMS, ICMR Headquarters, V Ramalingaswami Bhawan, Ansari Nagar, New Delhi-110029 <i>Email: <a href="mailto:consortiumrarediseases@gmail.com">consortiumrarediseases@gmail.com</a></i>

**NOTE: Calls for queries related to online submission will be taken till 25-01-2022, 17:00 hrs IST ONLY**

**Last date of Submission: 25<sup>th</sup> January 2023, 23:59 hrs, IST**

**FORMAT OF RESEARCH PLAN UNDER CALL FOR PROPOSALS ON  
THERAPEUTICS FOR INHERITED RARE DISEASES****(All fields are mandatory)**

1. **Title of the proposed research project (up to 25 words):** should be **specific, concise** and yet sufficiently descriptive and informative. Title may include study design such as randomized controlled trial; a case-control study etc.
2. **Summary (up to 250 words):** A structured summary should contain the following subheadings: Rationale/ gaps in existing knowledge, Novelty, Objectives, Methods, and Expected outcome.
3. **Rare disease group area of research (Please tick one):**
  - Storage disorders
  - Small Molecule Inborn Errors of Metabolism
  - Primary Immunodeficiency Disorders (PID)
  - Neuromuscular Disorders (NMD)
  - Hematological disorders (excluding Sickle cell diseases and Thalassemia)
  - Skeletal dysplasia
4. **Keywords:** Six keywords separated by comma which best describe your project may be provided.
5. **Abbreviations:** Only standard abbreviations should be used in the text. List of abbreviations maximum of ten may be given as a list.
6. **Current status (up to 500 words):** State the currently available information to adequately present the problem.
7. **Rationale of the study (up to 250 words):** Mention how the research question addresses the critical barrier(s) in scientific knowledge, technical capability, and/or programmatic/clinical/lab practice and its relevance to local, national and international context with relevant bibliography.
8. **Hypothesis/ Research question (up to 100 words):** Please provide details
9. **Preliminary work done by the PI (up to 250 words):** Proof of concept (if any)
10. **Study Objectives (up to 25 words/ objective):** Define the objectives clearly and in measurable terms; mention as primary and secondary objectives if necessary. Do not include more than 3-4 objectives.
11. **Methodology:** Include objective-wise work plan under the following sub-headings:
  - a. Rationale (up to 100 words)
  - b. Experimental design (study design, sample size, etc) (up to 500 words)
  - c. Expected outcomes (up to 100 words)
  - d. Statistical methods (up to 50 words)

e. Pitfalls and alternate strategies (up to 100 words)

12. **Limitations of this study (up to 100 words):**

13. **Expected outcome/ Deliverables aligned with research question (up to 100 words):**

14. **Future plan based on expected outcomes (up to 100 words):**

15. **Whether the study is going to generate new intellectual property or will be in conflict with the existing one?:** Please provide details

16. **Timelines with achievable targets:** GANTT/ PERT chart to be included

17. **Execution strategy (up to 500 words):** Explain how the study will be conducted, e.g- Study site(s), where the requisite number of patients be recruited, data management, available technical expertise, etc.

18. **Institutional Support/ Facilities:** Mention the efforts made to achieve inter-departmental or interinstitutional collaboration needed for study implementation, details of coordination between clinical, laboratory and data management procedures, mention:

- laboratory facilities (*in-vitro/ in-silico*)
- institutional resources such as instruments/ equipment and other physical resources available for use in the project proposed
- animal house etc.

19. **Budget:** Budget should not exceed 1.5 Crores\*/ study and as per ICMR guidelines available on the website. Justifications for all sub-headings under budget (as per ICMR format) is to be provided in detail.

*\*An outstanding project requiring funding more than 1.5 Crore/study, maybe converted to a Task force by ICMR, wherein the proposed PI will be the PI (single centric study) or will be one of the PIs (multicentre study).*

20. **Suggested reviewers:** PI should suggest 2-3 reviewers well versed with the area. Suggested reviewer should not have any conflict of interest-

S. No.	Name of the reviewer	Position and Affiliation	Email ID
1			
2			
3			

21. **Conflict of Interest Declaration (if any):** PI should include a statement for conflict of declaration (if any).

*Note: Additional supplementary information including figures, tables, flow diagrams, etc can be shared as PDF (20-30 KB).*