Task Force on Gene Therapy Research
Funding Opportunities for Gene Therapy Research Projects targeting Human Disease

Focus areas:
- Gene therapy for diseases (both inherited and acquired including chronic diseases)
- Novel gene therapy vector development
- Gene editing based therapeutic approaches
- Development of new gene transfer techniques and combinations
- Immunotherapy based on genetic modifications: Understanding immune response to gene therapy

Background: In the current era of genomics and genetic testing, it is evident that a large swath of the Indian population harbours causative mutations leading to various disorders. Conversely, for many inherited and complex disorders, the underlying genetic causes are known. However, most of such inherited disorders are currently not treatable by small molecule drugs or traditional therapies. Similarly, there are major developments in the area of gene therapy for cancer and other diseases including chronic disease. Over that past 3 decades, significant strides have been made in the field of Gene Therapy across the world. This has culminated in the recent FDA approvals for LUXTURNA (Gene Therapy for Retinitis Pigmentosa), YESCARTA (CAR-T cell therapy for Lymphoma), etc. Many such approaches are currently under investigation or in early clinical trials.

However, for the vast majority of inherited diseases, appropriate targeted therapies are yet unavailable despite the large load of genetic disease in our population. To address this particular gap in treatment options for the Indian population, the Indian Council of Medical Research (ICMR) invites proposals for funding Gene Therapy research projects. This call aims to fill the gaps in research thrust in India by providing emphasis on the above focus areas which require more attention to address the needs for the large existing patient base.

Proposals: Research proposals are invited in the following disease areas focused on the theme of gene therapy:

A. Inherited genetic diseases affecting any organ such as neuro-muscular disease (including DMD, BMD, SMA, myopathies, etc), retinal or corneal disorders (including Retinitis Pigmentosa, Stargardt disease, LCA, Fuch’s dystrophy, etc), heart diseases (familial cardiomyopathy, channelopathies, etc) blood disorders (Thalassemia, Sickle Cell Disease, haemophilia etc), etc, including syndromes.

B. Treatment of multifactorial diseases such as cancers, diabetes, lung diseases, neurologic diseases, etc.

The proposals are expected to clearly outline and test a path towards development of functional treatment options. The strategies proposed should have the possibility of translation into future human trials.
Proposals must address both efficacy and toxicity aspects of the Gene Therapy approach. Gene discovery studies will not be considered for this proposal, but in vivo/in vitro model development for the aforesaid disease areas are allowed if they are meant to be applied for testing gene delivery modalities. However, gene function studies should be part of the study design to evaluate the therapeutic approach. Where possible, the host response, vector production and immune response to the proposed gene therapy modalities should be addressed.

Criteria for application: The proposals should adhere to the focus areas and disease areas described. The scientific team should have demonstrable expertise in the areas of disease focus and gene therapy. Collaborations between institutes and between research and clinical teams are encouraged. Proposals must be written in the English language and clearly titled in the ICMR format. Project descriptions and style should conform to ICMR guidelines and have all requisite approvals and permissions required.

HOW TO APPLY Duration of the research proposals should be up to three years. All projects involving research on human beings/animals must be cleared by the Human ethics committee/Animal ethics committee of the respective institute. All Gene therapy projects require approval of Institutional Bio safety Committee (IBSC). A proposal envisages gene therapy product development and clinical trials must work in compliance with National Guidelines for Gene Therapy Product Development and Clinical Trials-2019.

Interested applicants are required to email the concept proposal to icmr.genetherapy@gmail.com. The format for preparing the concept proposal is given below and is also available on ICMR website www.icmr.nic.in. Our advisory panel will review the submitted proposals and shortlisted applicants will be asked to submit the detailed proposal.

Timeline:

- Launch of call for concept proposal on ICMR website: 19th November ends on 18th December, 2019 at 5:00 PM
- Scrutiny of concept and results by 30th January 2020
- Full project submission ends: 15th March, 2020
- Announcement of Successful Projects: 15th April, 2020

Eligibility: All scientific institutions, including Universities, DSIR certified institutes and laboratories are eligible.

Review process: The applications will be screened for technical correctness. Thereafter, each proposal will be reviewed for feasibility of the gene therapy approach, scientific applicability of the approach for disease, novelty of gene therapy design or delivery, clarity in experimental design, demonstration of prior experience/preliminary data, achievable milestones and timelines and potential for clinical translation.
FURTHER INFORMATION CONTACTS:

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CONCEPT PROPOSAL SUBMISSION FORMAT

Applicants are required to Email the concept proposal to icmr.genetherapy@gmail.com in PDF format. The applicants should also mention the name of the thematic area in the subject of the Email. Concept Proposal must to be prepared under the following sub-headings (mandatory) and exceeding the word limit will lead to disqualification.

1. **Thematic Area**: The applicants must mention the thematic area under which they wish to apply.

2. **Title of the proposed research project**: Should be concise and informative.

3. **Rationale** (up to 250 words): Describe the current knowledge available on the subject, critical gaps in knowledge and the national relevance of the research question which this project aims to address. Also, mention the preliminary work done by the applicant, if any.

4. **Novelty/Innovation** (up to 100 words): Describe how the proposal challenges and seeks to shift the current research/knowledge/clinical practice paradigms by utilizing novel theoretical concepts, methodologies, instrumentation or interventions.

5. **Project description** (up to 600 words): Describe the study setting, study design, sampling strategy, sample size, research methodology and outcomes measures. Also, mention expected timelines, total budget, name and designation of co-investigators and intra and inter institutional collaborations, if any.

6. **Strength of PI**: Describe academic qualifications, employment details, previous experience of handling research projects (past and ongoing) and the scientific contributions made from these projects. Enumerate 10 relevant publications (in Vancouver style).

7. **Institutional support** (up to 200 words): Mention the institutional support in terms of basic infrastructure, departments and laboratories with equipment required for the proposed research work.