

Therapeutic strategies for prevalent Rare/Orphan Disorders

Rare genetic diseases are a public health concern in India, with an estimated burden of about 80 million cases. This is about one-fifth of the global burden. High-genetic disease prevalence has been noted in affected communities with high genetic relatedness between parents and the majority of rare diseases in India occur at birth due to recessive genetic defects. While many of the commonly occurring rare diseases have viable therapies, most of these therapeutic agents are patent protected and very expensive for the Indian user. Thus, there is a large unmet need for indigenously manufactured therapeutic agents for rare diseases. Currently, a few pharmaceutical companies are manufacturing drugs for rare diseases globally and there are no domestic manufacturers in India except for Food for Special Medical Purposes (FSMP) for small molecule inborn errors of metabolism.

Objectives:

The goal of this Call is to support milestone-driven proposals for the listed rare diseases (though not limited to) focused on:

- To bring generic drugs which are off- patent.
- To develop process chemistry for drugs under patent to make it affordable once patent expires.

Scope:

Research Groups working in process chemistry / formulations are encouraged to develop new process or unique technology establishment for existing drugs and (new) formulations to make it affordable as described in objectives.

Following is the representative list of rare diseases which may be targeted for the proposals.

Lysosomal storage disorders

Gaucher disease

Mucopolysaccharidoses (all types)

Pompe disease

Fabry disease

Niemann Pick disease

Metachromatic Leukodystrophy

Krabbe disease

Small molecule diseases: Phenylketonuria, Maple Syrup Urine Disease (MSUD), Tyrosinemia etc

Neuromuscular Disorders: Spinal Muscular Atrophy (SMA), Dystrophinopathies / Duchenne muscular dystrophy, etc

Neuronal ceroid Lipofuscinosis

Cystic Fibrosis

*Other rare disorders (**Excluding Cancers**)*

Deliverables:

The proposed R&D should deliver a drug compound/new process/formulation at affordable cost.

Exclusions:

This Call excludes basic research, studies of disease mechanisms or epidemiological studies. Gene therapy and stem cell therapy are beyond the scope of this call. Cancers are also excluded from the Call. **Any proposal beyond the scope of the call will not be entertained.**

Who Can Submit Project Proposals:

The Project Proposal should be submitted for financial support through [ONLINE MODE ONLY](https://onlinedst.gov.in/) (<https://onlinedst.gov.in/>) by scientist/ engineers/ technologists working in Universities and other academic institutions; R&D institutions/ laboratories having adequate infrastructure and facilities to carry out R&D work.

Applicants should register as a PI in DST's Online Portal. Registered PIs may get an option of "submit proposal", under which calls are open. PIs may download the Format under the division of "Technology Development and Transfer" followed by the scheme of "Therapeutic Chemicals (Therapeutic strategies for prevalent Rare/Orphan Disorders)".

The investigators/ R&D Group should have adequate experience and expertise in the relevant area of proposal. Desirable / Not Mandatory - Participation of Clinician working for rare diseases from a reputed hospital to be a part of investigating team as Co-PI. The consortium approach involving researchers, clinicians, industry / startups will be preferred.

Support to academia: scientists/ engineers/clinicians specific points for submission of project proposals

- The PI(s) should have relevant experience as evident from publications of past three years.
- Investigators may interact with Centres of Excellence established by Ministry of Health and Family Welfare, which provide facilities for treatment and care of patients suffering from rare diseases.
- The proposal should cover achievable milestones, time lines detailing how the project will move forward, justifiable budget requirement and engagement with

prospective technology transfer partners or technology transfer facilitating bodies/Technology Business Incubators.

- The host institutions should be well-equipped and have the essential equipment and infrastructure for the proposed development. Only minor equipment will be supported based on requirement of the proposed objectives.
- Evaluation will be based on scientific and technical merit.
- The PI can submit only one proposal against the Call.
- The ethical clearances, wherever necessary, may be appended with the proposal.
- No financial support will be provided for Clinical Trials.
- Financial support from DST will not be provided to the industry/startup.
- Incomplete Application formats will be summarily rejected.
- It is envisaged that the end product of development shall be transferred to industry / startup for commercial production. Hence it is desirable that industry / startup may be associated with project right from the beginning with defined participation in technical terms.
- The projects should be time-bound normally for duration of 2-3 years.
- Monitoring of the Project: Implementation of the projects is monitored quarterly through Progress Reports, Financial Statements and Committee of Experts in Group review meetings and onsite as well. Subsequent releases will be subject to achievement of projected milestones.
- The Project Proposal should be submitted through ONLINE MODE ONLY (www.online.dst.gov.in). Hard copies are not required.

For any queries related to Call, please contact:

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