



Request for Proposal

Introduction and Purpose

BioMarin is a world leader in developing and commercializing first-in-class and best-in-class innovative biopharmaceuticals for rare diseases driven by genetic causes. BioMarin's mission is to bring new treatments to market that will make a big impact on small patient populations. We are inviting scientists to submit short proposals to join our efforts in advancing treatments for rare diseases.

Focus Area

BioMarin is looking for scientists who are developing innovative therapeutic approaches and enabling technologies that correct the underlying molecular defect in genetic-based rare diseases. Our standard boundary conditions are:

- Rare mono genetic disease with a prevalence of at least 1:50,000
- Broad therapeutic areas, but excluding oncology, infectious disease
 - Focus on CNS, muscular skeletal and cardiac diseases
- Significant unmet clinical need with discernable clinical endpoints
- Treatment addressing the underlying molecular pathology
- Transformative impact - where treatment is expected to stop progression or provide reversal

Enabling Technologies of interest are platform technologies for genome editing, vector engineering, new or improved modes of delivery and enhanced lead identification.

Technology Scope:

We are interested in a broad range of modalities and relevant enabling technologies including:

- Small Molecule and Biologic therapeutic approaches
- Gene Therapy and Gene Editing
- Oligonucleotide and mRNA directed therapies
- Cell-based therapies

Format and Criteria

Proposals should be brief and **not exceed 500 words** total and should include the following:

- Abstract on your proposal highlighting the potential impact in advancing treatments for rare diseases

- High-level timeline to achievable goals (ideally the proposal would be no longer than 18 months, can include a possibility to extend based on the results)
- High-level budget summary
- Background on you and your team
- Please don't add any unpublished or confidential information

Key Dates and Notes

- Proposal submissions are due by August 31, 2021, but teams are encouraged to submit ASAP
- Selected proposals will be reviewed and contacted in approximately 30 days post submission

If you are interested in submitting a proposal please email me the following information so that we can work on the proposal submission:

First and Last Name	
Institution or Company Name	
E-Mail Address	
Phone Number	

Project Title	
Therapeutic Area	
Indication	
Prevalence of Disease (Underline your choice)	Less than 1:50,000 patients More than 1:50,000 patients, NA
Technology Scope (Underline your choice)	Small Molecule, Biologic, Gene Therapy / Gene Editing, Oligonucleotide / mRNA therapies, Cell-Based Therapies
Relevant Links (optional)	

Submission Proposal (500 word maximum)

Your proposal submission....

Please include Rationale for project and evidence to support rationale, high-level overview of project approach/aims and expected outcomes/deliverables within the timeframe.

For any questions, please contact Gayathri Srinivasan (gayathris@emory.edu).