

**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 technologiesthat 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, hematology 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 technologiesincluding:

* 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, non-confidential, 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 (Project Dependent)
* Background on you and your team

**Key Dates and Notes**

* Proposal submissions are due by Feb. 20, 2022, but teams are encouraged to submit ASAP
* Email submissions to tido.alliances@childrens.harvard.edu
* Selected proposals will be reviewed and contacted in approximately 30 days post submission
* Please note that based on the feedback received from this call for proposals, BioMarin could consider opening a portal to allow submissions year round to enable scientists to pitch new projects at any time.

We greatly appreciate and thank all applicants for their time and effort. We regrettably do not have the capacity to reach out to proposals that do not meet the criteria listed above.



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| --- | --- |
| 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 frequent than 1:50,000 patients  More frequent 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.*