

# Submission guidelines



The PTC Innovative Research Funding program, **PRIORITY**, is a global program designed to provide funding, through duly eligible research entities, to biomedical researchers or clinicians interested in developing unique and collaborative research programs that will improve clinical practice for patients with rare genetic disorders. The 2019 PRIORITY program will focus on Duchenne muscular dystrophy (DMD), and invites funding proposals for innovative research projects that will help improve rates of DMD screening in infants and facilitate earlier diagnosis of this progressive muscle-wasting disease.

## Submission process

Eligible institutions are invited to submit research proposals by **March 31, 2019**. Applications can be submitted via email to [PRIORITY\\_submissions@ptcbio.com](mailto:PRIORITY_submissions@ptcbio.com). Please include the completed funding application form as an attachment and ensure that you have included clear and appropriate contact information. Within five business days, you will receive confirmation that your application has been accepted for review. *If you do not receive this confirmation, please contact [PRIORITY@pharmagenesis.com](mailto:PRIORITY@pharmagenesis.com).*

A written agreement, defining the basic conditions for the financing of the project, will be entered into by PTC and funding recipients.

## Submission guidelines

- Applicants must complete the [funding application form](#) found at the end of this document.
- Applications must be concise and no longer than 4 pages in length. Font size should be at least 10 point.
- The proposal must be written in English. For those unable to submit a proposal in English, translation will be provided at no cost. For further information on translation, contact [PRIORITY\\_help@ptcbio.com](mailto:PRIORITY_help@ptcbio.com). However, the funding recipient will be responsible for the translation of additional funding-related documents at their own expense. These include, but are not limited to, a written agreement with PTC defining the basic conditions for the financing of the project; an interim report/status update at the midpoint of the funding year; and a final report at the end of the funding year.
- Applications will only be accepted during the submission window.
  - The submission window will open on January 31, 2019.
  - The submission window will close on March 31, 2019.
  - Applicants will be notified of the outcome of their submission in late April 2019.
  - Recipients of the funding will be announced via a press release in late April 2019.

- Each research group is only permitted to submit one (1) application per funding year.
- Countries that are eligible to apply for 2019 funding are Argentina, Austria, Brazil, Canada, Chile, Colombia, Cyprus, Czech Republic, Denmark, Finland, France, Germany, Greece, Israel, Italy, Kuwait, Latvia, Lebanon, Norway, Peru, Poland, Portugal, Romania, Russia, Saudi Arabia, Singapore, Slovakia, Slovenia, Spain, Sweden, Switzerland, Turkey, the UK and the USA.
- Review Committee members will not be eligible to submit an application for this program.
- PTC's commitment to successful applicants is subject to all applicable rules and regulations and, where required, the approval of the relevant ethics committee review.

## Funding application form: components

### 1. Applicant details

Please provide the details of the investigator-sponsor as well as of other potential investigators. All communication will be through the investigator-sponsor, who will be in charge of passing on the information to the other investigators and their institution.

### 2. Project proposal

Applicants must provide information relevant to their proposed project under each of the subheadings included in the funding application form. You may wish to cite research or statistics, or provide a brief story or case study if relevant to the proposed project.

Additional guidance on what should be included under each subheading is provided below. Please note: the character limit for each section of the funding application form includes spaces.

- **Project title (514 characters):** a concise description of the project.
- **Rationale for the project (642 characters):** an explanation of why the proposed research is needed.
- **Project objective(s) (770 characters):** a brief statement that describes what idea(s), unmet need(s) or challenge(s) your project seeks to address.
- **Proposed method (1140 characters):** a description of how the proposed research will be performed and how you will evaluate the success of the project.
- **Team (642 characters):** a brief description of your research group and/or institution and why you believe you are suited to develop and execute this project.
- **Materials (642 characters):** an outline of what materials/facilities will be used for the project.
- **Participants (642 characters):** a short description of the proposed study population.
- **Sample size and rationale (642 characters):** the approximate number of study participants and your reason for selecting this sample size.
- **Expected outcome of the research project (1540 characters):** a clear description of the expected outcome(s) and impact on the DMD community.

- **Geographical reach (642 characters):** a description of the potential of your research to benefit the international Duchenne community.
- **Potential to replicate the program in other regions (642 characters):** an explanation of how the project could be expanded to other regions (consider the availability of materials/facilities and ease of recruitment of participants).
- **Summary (1925 characters):** a short overview of the project that reiterates the objectives, participants, expected outcomes and reasons why your team are suited to perform the research.
- **Estimated duration of the research (642 characters):** an estimation of how long the project will take to complete (consider the timeframe for the recruitment of study participants).

### 3. Project category

Proposals submitted in 2019 must fulfill one of the categories outlined below. Applicants must select the appropriate category for their proposal on the funding application form.

- **Collaborative proposals** involve two or more research groups working together towards a common goal.
- **Innovative proposals** reflect a unique and novel approach that will benefit the neuromuscular disease community, either in a specific geographic location or the overall global community.
- **Sustaining proposals** are designed for a worthy project that is already underway and requires funding to continue.

The **supporting information** section (642 characters) can be used to add any additional comments to support the chosen project category.

### 4. Support requested (900 characters)

For each of the categories above, proposals will be reviewed based upon the financial request. The funding budget for 2019 is up to US\$500,000, which will be divided between successful research projects according to the requirements outlined in the applications. Eligible individuals and teams who meet the criteria are invited to apply for funding support of up to US\$200,000.

Applicants must specify the amount of funding they are requesting on the funding application form and provide their rationale for the request. If the project will continue beyond the funding year, applicants must explain how the project will be supported in the future.

### 5. Additional comments (1925 characters)

Please add any additional comments or information that you deem relevant to your proposal and that does not fit in the above categories.

## Review criteria

All submitted proposals will be handled by an independent Review Committee comprised of individuals from around the world who are familiar with evaluating requests for funding programs. Committee members have been selected based on their significant interest and expertise in the diagnosis of DMD. Each proposal will be blind reviewed, scored, and ranked using the following standard criteria.

**Innovation:** How unique is the project? Is the project one that will inspire others and demonstrate leadership in the Duchenne community?

**Vision:** How far-reaching is the project? Does the project have achievable goals, a reasonable budget and a realistic action plan to meet the vision of the project? How will the project be sustained after the funding period? What are the plans for growing the initiative?

**Empowerment:** Will the project offer hope to those living with DMD as well as to their family members? How will it empower patients and their families to advocate for better health outcomes?

## Resources

Research groups seeking guidance in preparing their proposals may wish to visit the websites of the following non-profit and governmental organizations.

- Foundation Center: [www.foundationcenter.org](http://www.foundationcenter.org).
- The Grantsmanship Center: [www.tgci.com](http://www.tgci.com).
- The Guardian: [www.theguardian.com/higher-education-network/blog/2013/apr/19/tips-successful-research-grant-funding](http://www.theguardian.com/higher-education-network/blog/2013/apr/19/tips-successful-research-grant-funding).
- GuideStar: [www.guidestar.org/rxa/news/articles/2006/grant-writing-101-resources-for-grant-writers.aspx](http://www.guidestar.org/rxa/news/articles/2006/grant-writing-101-resources-for-grant-writers.aspx).
- National Institutes of Health: [http://grants.nih.gov/grants/grant\\_tips.htm](http://grants.nih.gov/grants/grant_tips.htm).
- Alfred P. Sloan Foundation: [www.sloan.org/apply-for-grants/tips-for-writing-a-successful-grant-proposal/](http://www.sloan.org/apply-for-grants/tips-for-writing-a-successful-grant-proposal/)

# Funding application form

Please complete all fields with as much detail as possible.



## 1. Applicant details

<b>Investigator-sponsor</b>	<b>Additional address information, if applicable</b>
<b>Institution</b>	
<b>Address line 1</b>	<b>Telephone</b>
<b>Address line 2</b>	<b>Email</b>
<b>City</b>	<b>Other investigators</b>
<b>State</b>	
<b>Postal code</b>	<b>PTC Therapeutics contact, if applicable</b>
<b>Country</b>	

## 2. Project proposal

<b>Submission date</b>
<b>Project title</b> (514 characters)
Section continues on next page.

**Rationale for the project** (642 characters)

**Project objective(s)** (770 characters)

**Proposed method** (1140 characters)

**Team** (642 characters)

**Materials** (642 characters)

**Participants** (642 characters)

**Sample size and rationale** (642 characters)

**Expected outcome of the research project** (1540 characters)

**Geographical reach** (642 characters)

**Potential to replicate the program in other regions** (642 characters)

**Summary** (1925 characters)

**Estimated duration of the research** (642 characters)

### 3. Project category

Collaborative

Innovative

Sustaining

**Supporting information, if applicable** (642 characters)

### 4. Support requested

**Amount of funding requested and rationale** (900 characters)

### 5. Additional comments (1925 characters)