

Working together to end Duchenne



Duchenne muscular dystrophy (DMD) Joint Call for Therapeutic Projects 2022

Terms and Conditions for Applicants



1. Introduction

- 1.1. Duchenne UK (DUK) and Parent Project Muscular Dystrophy (PPMD) are partnering to deliver a call for academic or company led research projects relating to the development of innovative therapies for the treatment of Duchenne muscular dystrophy (DMD) (*DMD Joint Call for Therapeutic Projects 2022, "Call"*).
- 1.2. We have come together to support and accelerate progress to the clinic of novel therapeutic programmes that a) address the challenges in 'first generation' gene therapies and b) take advantage of opportunities in the cell-based and regenerative therapies field to ensure new, safer and more effective treatments reach the clinic in the next 3 to 5 years ("Projects").
- 1.3. The purpose of this document is to set clear terms and condition for prospective applicants regarding:
 - a) the scope and eligibility of a Project
 - b) the application and Project selection process
 - c) the terms of funding
- 1.4. Projects approved for funding will be subject to a separate funding agreement, as described herein.
- 1.5. Applicants will be asked to acknowledge that they have read and understood these Terms and Conditions before applying for funding.
- 1.6. Submission of an application under these Terms and Conditions does not guarantee that an award of funding will be made, even where such application made is within scope.

2. Scope, eligibility and project selection criteria

- 2.1. Scope-What we will fund
 - a) We are inviting proposals for ambitious, translational projects driving the next generation of transformative DMD therapies, which have the potential to disrupt the current therapeutic landscape, leading to marked improvements in the safety, efficacy, and delivery profile, over current SoC therapies and therapies currently in clinical development.
 - b) These will involve novel genetic therapeutic approaches, including: gene therapy and gene editing, that use delivery models and strategies that address the current challenges of using viral delivery in the clinic; novel cell-based therapeutic approaches; other regenerative approaches such as those targeting the host muscle stem cells.
- 2.2. Eligibility-Who we will fund
 - a) The call will be open to universities, research institutes and small for-profit companies worldwide.
- 2.3. Project selection criteria
 - a) Project will be selected based on the following criteria:
 - i. Expected to last up to 3 years
 - ii. Translational Projects with a clear path to the clinic
 - iii. Based on a strong Target Product Profile, which clearly demonstrates the rationale for superiority over current SoC therapies and novel therapies in clinical development
 - iv. Evidence of robust proof of mechanism preclinically (e.g. biological evidence of an effect which is DMD relevant/specific)
 - v. Preliminary evidence of efficacy in DMD relevant preclinical models
 - vi. Potential to generate a data package which would be compelling for the next round of funding
- 2.4. In addition to the criteria above, the following will apply:



- a) where consensus cannot be reached, DUK and PPMD will use other ways to objectively assess the proposed Projects and reach consensus on funding in a transparent and timely fashion. These may include using an agreed scoring system or clear decision tree.
- b) Proposals must provide a reasonable therapeutic hypothesis and a suggested plan for further developing or validating the evidence mentioned in the criteria above.
- c) At application stage, applicants are encouraged to evidence expertise, research tools and capabilities they have access to which will strengthen the project e.g. unique biological assays, research tools, disease and in vivo models etc.

3. Application and Project selection process

- 3.1. The Call will involve two stages: expression of interest (EOI) and full application (FA).

EOI Stage

- 3.2. The EOI stage will involve the submission of a project outline, including an executive summary, proposal background (including key data and references), therapeutic hypothesis and rationale, scope of work, key deliverables and milestones, expected impact and high-level costs.
- 3.3. EOI submissions will be reviewed by DUK and PPMD and successful proposals will be selected based on whether they are in scope and meet the eligibility and Project criteria.

FA Stage

- 3.4. EOI proposals accepted for further consideration will progress to FA stage. A detailed proposal will be completed at this stage, including a full experimental plan and budget.
- 3.5. Throughout the FA stage, DUK and PPMD will engage closely with the applicants to ensure the proposals selected at EOI stage are developed in robust funding propositions.
- 3.6. When finalised, full submissions will be reviewed by a peer review panel, specifically convened for the call. This will involve experts selected based on relevant expertise and experience.
- 3.7. DUK and PPMD will use their scientific advisory boards and networks to identify the most relevant external experts to invite to join the peer review panel. The choice of panel members will be informed by the proposals received at EOI and progressed to FA stage.
- 3.8. More than one iteration on the Project plan and budget may be required before a Project is approved for funding, and the applicants will be expected to support these as relevant to enable timely reviews and decisions.
- 3.9. Applicants acknowledge that final approval for funding may not be granted if any issues or concerns should arise on the Project feasibility and its overall viability during the project development stage.
- 3.10. Applicants will be advised of the progress of their submission at all stages.

Unsuccessful Applications

- 3.11. If any applications are deemed to be unsuccessful at any stage, basic feedback will be provided upon request as to the reasons. However, DUK and PPMD are unable to provide detailed feedback and enter protracted communication on the merits of the application.



4. Application submission

- 4.1. All applications must be submitted using the application forms provided to the following email address: research@duchenneuk.org.
- 4.2. In the forms, applicants must acknowledge that they have read and understood these Terms and Conditions and the other relevant policies.
- 4.3. Following the submission of an application, applicants will receive an email confirming receipt of their application.
- 4.4. Applications submitted not using the approved procedure will not be considered.
- 4.5. Late submissions will not be considered. DUK and PPMD will not engage in any communication on late submissions.

5. Confidentiality

- 5.1. Subject to these Terms and Conditions, all information shared under the Call will be treated as confidential and shall be used only for the purpose of evaluating the relevant application.
- 5.2. The information included in the application at the EOI stage form will be used by DUK and PPMD to assess whether the application meets the funding criteria and to decide whether to progress it to the next stage.
- 5.3. The information provided in the application at the FA stage will be shared with the external peer review panel for review. The members of the peer review panel will be bound to confidentiality obligations.
- 5.4. The following exceptions to confidentiality will be applied to information disclosed in any applications, if the information disclosed is:
 - a) already in the public domain prior to receipt by DUK and PPMD, the members of the peer review panel or in their possession and at their free disposal; or
 - b) subsequently lawfully disclosed to them without any obligations of confidence by a third party having the right to disclose it free from any obligation of confidentiality; or
 - c) becomes generally available to the public through no act or default of DUK and PPMD, the members of the peer review panel under the relevant obligation of confidence or their employees or professional advisors; or
 - d) shown by reasonable documented proof to have been developed independently by DUK and PPMD, the members of the peer review panel; or
 - e) DUK and PPMD, the members of the peer review panel under the relevant obligation of confidence are required to disclose by or to the courts of any competent jurisdiction, or to any government, regulatory agency or financial authority, provided that they shall:
 - i. inform the applicant as soon as is reasonably practicable; and
 - ii. at the applicant's request and cost seek to persuade the court, government, agency or authority to have the information treated in a confidential manner, where this is possible under the court, government, agency or authority's procedures; and
 - iii. maintain that any copies of information so disclosed shall remain otherwise subject to the obligations of confidentiality and non-use set forth herein.

6. Funding

- 6.1. DUK and PPMD funding commitment to the Call will be subject to identifying and ultimately reaching a funding decision on suitable Projects to fund.
- 6.2. Duchenne UK and PPMD will be open to fund both academic and company led Projects.



- 6.3. The total funding available for this Call is ~\$1,000,000 to fund up to 2 Projects.
 - 6.4. DUK and PPMD will be open to co-funding proposal with other organisations (e.g. other charity partners)
 - 6.5. Unsuccessful research proposals may be considered for funding outside of the Call by either DUK or PPMD, or both.
 - 6.6. Once a Project is approved for funding, the lead organisation will be formally notified, and enter into a funding agreement using the template agreement provided by DUK and PPMD.
 - 6.7. Funding will be subject to signature of the funding agreement.
 - 6.8. The applicants acknowledge that DUK and PPMD have limited ability to re-negotiate clauses, and will aim to achieve the timely signature of the funding agreement and ideally within 3 months from notification of Project approval for funding. If the parties are unable to agree terms within this period of time, the application may be deemed unsuccessful.
 - 6.9. The funding agreement will set the obligations of the parties in relation to the funding and the delivery of the Project; the terms of funding and the finance schedule will be in accordance with the project milestones and go/no-go decision points.
 - 6.10. Applicants acknowledge that funding may be terminated if the Project does not successfully meet a key go/no-go milestone.
 - 6.11. Successful applicants and their organisations will be expected to provide the required support and enabling resources to ensure the timely start of the Project.
 - 6.12. The award will cover all eligible direct research costs associated with the Project, including salary support for research staff as appropriate.
 - 6.13. The award will not fund indirect costs, such as non-specific estimated costs, including estates costs, library costs, finance, legal and HR costs.
- 7. Essential information on intellectual property (IP), exploitation of results and publication**
- 7.1. Prior to the commencement of the Project, the applicant's host organisation ("Host") will enter a funding agreement with DUK and PPMD (the "Funders") which will set the provisions on IP, exploitation of the results, publication and sharing of any revenues resulting from IP developed in connection with a Project. The terms of 7.4-7.6 shall apply solely to academic and other non-profit organizations. The terms of 7.7-7.8 shall apply to both academic (and other non-profit organizations) and for-profit companies. The terms of 7.9 shall apply solely to for profit companies.
 - 7.2. All results and arising IP from a funded research Project shall be owned by the Host.
 - 7.3. The Host shall grant the Funders a non-exclusive, world-wide, perpetual, fully paid-up, royalty-free, and sub-licensable right to use the results and to any intellectual property therein for non-commercial purposes.
 - 7.4. The Funders will each be granted an option to negotiate an exclusive, sublicensable commercial license (or co-exclusive if more than one funder) to the IP and results generated during the Project, to be exercised upon completion of the Project the terms of which will be agreed directly between the Host and the relevant funding partner/s.
 - 7.5. If the Funders do not exercise their option rights provided for in Section 7.4, the Host shall use commercially reasonable efforts to license or otherwise transfer (collectively a "Transfer") the arising IP to a third party that has the intention and ability to further develop the arising IP into a commercial product.



- 7.6. In the event of a Transfer, the Host shall pay to the Funders an appropriate portion, to be negotiated with the Funders, of the consideration the Host receives in connection with the Transfer.
- 7.7. The applicant/s must warrant that they have the ability to grant the rights mentioned above and that such grant shall not breach any third-party rights.
- 7.8. Publication or presentation of the results will be encouraged, however, if they contain patentable subject matter or would materially jeopardise any intellectual property rights, the publication or presentation may be delayed for the time required, as agreed by the institution and the funding partners, to take the necessary steps for the protection of such subject matter.
- 7.9. Notwithstanding the foregoing in this Section 7, if the Host is a for profit entity, the funding provided by the Funders may take the form of a purchase of shares of Host, a convertible note or SAFE, or a royalty-based investment, as appropriate, and as set forth in the applicable award agreement.

8. Conflict of Interest

- 8.1. At application stage and then throughout the Project review and development process, applicants should communicate any potential conflict of interest timely to DUK and PPMD, so that this can be managed appropriately.

9. Personal Data

- 9.1. All information, including any personal data, submitted under the Call will only be used for the processing of and communication regarding the application, in accordance with these Terms and Conditions.
- 9.2. All information, including any personal data, will be processed, stored, shared and retained securely and in accordance with DUK and PPMD data privacy notice.

<https://www.duchenneuk.org/privacy-policy/>

<https://www.parentprojectmd.org/about-ppmd/privacy-policy/>

- 9.3. Applicants should refer to the DUK and PPMD data privacy notices for more details on processing, storage, sharing and retention of personal data.