THERAPEUTIC GRANT CALL 2024
Launches on June 24, 2024; EOI submissions due 23:59 pm on 4th of August 2024

Overview

Despite the progress in the development of treatments that aim to address the root cause of DMD through the delivery of replacement (micro) dystrophin (i.e. gene therapy), as a research community we are still faced with major challenges preventing us to achieve transformative change in the treatment of DMD. For therapies in the clinic or in development, these include:

- Limited clinical efficacy and benefit profiles of current dystrophin replacement approaches available in the clinic
- DMD genetic profiles associated with immunogenicity and significant concerns on safety, which will require immunomodulation/immunosuppression and more sophisticated screening strategies
- Sub-optimal, viral based, drug-delivery systems used for genetic therapies which:
    - preclude all patients to have access to genetic treatments due to the host pre-existing antibodies to the virus
    - prevent re-dosing, leading to issue of effect durability, due to the host response to viral vectors
    - can only deliver small genetic sequences, leading to limited functional effects associated with the shorter protein (instead of a fully functional, full-length protein)
- Lack of clear understanding of the impact of these treatments on the heart (efficacy and safety)
- Lack of understanding of the long-term clinical benefit (and safety) of dystrophin replacing therapies, which require a better mechanistic understanding of how these drugs work at a cellular and muscular tissue level.

We want to accelerate progress of novel therapeutic programmes that address these challenges to ensure new, safer, and more effective treatments reach the clinic as soon as possible or within up to 3 years from us funding the programme.

Scope

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, including cardiac outcomes, and delivery profile, over current SoC therapies and therapies in the clinical development pipeline.

Examples of therapeutic approaches we would like to support:

- Novel genetic therapeutic approaches that use non-viral delivery models or address the current limitations of viral delivery

- Full length dystrophin gene approaches

- Novel cell-based therapeutic approaches

- Other innovative approaches that can complement the above, and hence can be implemented clinically as combination therapies

- Holistic approaches with potential to target all domains of DMD (muscle, heart, brain)

- All the above will have to address safety using clinically relevant strategies, as relevant, to prevent or manage immune response related or other harmful effects on patients

Eligibility

Our funding is open to universities, research institutes and small companies (micro and medium) worldwide. Projects are expected to last up to 2 years, with total project costs up to £500,000.

Higher project costs may be considered subject to how many projects will be funded. Matched funding from other funders will be considered for projects with costs above £500,000.

What projects we are looking to fund

Within the calls scope described above, we will fund projects that meet the following criteria:

- Ready to enter the clinic or aimed at reaching the clinic within 3 years from funding (e.g. lead selection or lead optimisation stage).
- Based on a strong Target Product Profile, which clearly demonstrates the rationale for superiority over current SoC therapies and novel therapies in clinical development.
- Have reached robust proof of mechanism preclinically (e.g. biological evidence of an effect which is DMD relevant/specific).
- Have preliminary evidence of efficacy or relevance in DMD relevant preclinical models
- Earlier stage proposals may be considered if they are highly clinically relevant, innovative and disruptive, and our funding is uniquely positioned to enable progress.
- Safety considerations will be paramount in the assessment of the proposed research and its potential translation to the clinic, and therefore they may be an important factor in deciding whether to fund a proposal or not

We will NOT fund:

- projects that are very early stage and have no preliminary mechanistic evidence of the proposed drug therapy having an effect in the target tissue in DMD.
- Technology development projects, including the development of preclinical model (*in vivo* and *in vitro*)

Submission process

- Stage 1-Expression of Interest: this will involve a short outline of the project, including approximate costs. Projects accepted for further consideration will progress to Stage 2.
- Stage 2-Full submission: a detailed proposal will be completed at this stage, including a full experimental plan and budget using a bespoke template specifically developed for this call.

Throughout Stage 1 and 2, applicants will be expected to engage closely with the DUK team in the development of the final project proposal for funding.

Review process

- Expression of interest submissions will be reviewed by the DUK team (Research team and CEO) and projects will be selected based on whether they are in scope, meet the eligibility criteria and the project criteria.
- Full submissions will be reviewed by a peer review panel, specially convened for the call. This will involve members of DUK Scientific Advisory Board and other international experts selected based on relevant expertise and experience.
- Applicants will be advised of the progress of their submission at all stages.

Key dates

Call launch-Expression of Interest (EOI) applications invited	24th of June 2024 (open for 6 weeks)
EOI applications due	23:59 pm on 4th of August 2024
Full applications invited	23rd of August 2024 (open for 8 weeks)
Full applications due	23:59 pm, 20th of October 2024
Successful applicants notified (subject to contracting)	w/c 9th of December

If you are interested in applying, please download your expression of interest form here:

If you would like to discuss this funding call with a member of the team or have any questions, please email us on research@duchenneuk.com