

REQUEST FOR APPLICATIONS: CASE REPORTS OF EARLY EXON SKIPPING THERAPY INITIATION

Solicitation Name: Early Exon Skipping – Case Reports 2023

Applications Due: 4/15/23

Notification of Selection: Within 30 days of proposal submission

Period of Award: 6 months

Award amount: \$2000

The purpose of this RFA is to solicit case reports demonstrating the benefit of early diagnosis and treatment of Duchenne muscular dystrophy. Case reports should meet the following criteria:

1. Discuss a child with Duchenne who initiated exon skipping therapy before age 3.
2. Duration of exon skipping therapy of at least one year is required.
3. Outcome measures such as Bayley or NSAA must be available.

Eligibility

Applications are welcome from academic/clinical institutions and/or non-profit research institutes.

Those eligible to apply for a PPMD Grant Award must:

1. Be a professional or faculty member at an appropriate educational, medical or research institution and be qualified to conduct and supervise a program of original research;
2. Have both administrative and financial responsibility for the grant;
3. Have access to institutional resources necessary to conduct the proposed research project; and
4. Hold a Doctor of Medicine, Doctor of Philosophy, Doctor of Science, Master of Science, or equivalent degree.

Award Requirements

1. Successful completion of case report
2. Timely publication of results, which could include conference abstract
3. Ability to comply with PPMD policy manual, which can be viewed in the WebGrants application

Submission Process and Requirements

PPMD uses an online grant submission and management system to reduce the burden of paper and to standardize our procedures.

To apply for this RFA, visit the PPMD Application site, WebGrants: <http://ppmd.dullestech.net/> or scan the QR code below.

Apply to **Early Exon Skipping-Case Reports 2023** under the Opportunity tab.



Proposals cannot exceed 2 pages in length, excluding bibliography, and must be submitted in 11-point font.

**Technical questions should be sent to PPMD:
Niki Armstrong, niki@parentprojectmd.org.**

The proposal must include the following information for the case provided (within the 2-page limit):

- Age of diagnosis
- Reason for early diagnosis (family history, symptomatic, incidental finding)
- Specific exon skipping therapy
- Age of initiation of exon skipping therapy
- Duration of exon skipping therapy
- Functional outcomes available
- Developmental history, including age of attainment of ambulation
- Specific mutation
- Corticosteroid status (if using, age of initiation)
- Current age
- Current functional status
- Any lapses in exon skipping therapy, duration and why

Review and Selection

This is a rolling application. Proposals will be reviewed as received, with notification occurring within 30 days of receipt. Funding will be distributed once approved, so funds may be depleted prior to the **4/15/23** deadline.

Individuals may submit multiple cases.

Decision for awarding will be made by PPMD Care and Research staff, with potential review by experts in newborn screening from PPMD's Duchenne Newborn Screening workgroups. All reviewers will sign confidentiality agreements.

Review criteria

The proposal will be evaluated on:

- Overall demonstration of the impact of early therapy.
- Preference will be given to cases with longer duration of therapy or more available outcome data.

Other Award Conditions

Awards will be managed as cooperative agreements, with an initial 50% payment at the time of award and a final payment at the time of publication.

Indirect costs or overhead will not be covered by this grant.

PPMD can assist in identifying potential journals and/or conferences for publication of the case report and may act as the coordinator for compiling cases for publication or abstract submission.