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**TITLE:** Longitudinal End Point Optimization to Provide Assessments of Relevant Drugs

**PRINCIPAL INVESTIGATOR:** Chad R. Heatwole, MD, MS-CI

**CONTRACTING ORGANIZATION:** The University of Rochester

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# REPORT DOCUMENTATION PAGE

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<b>14. ABSTRACT</b> Mitochondrial disease, cardiomyopathy, rheumatoid arthritis, and dystonia are chronic conditions that collectively affect ~1/60 people. These four conditions markedly reduce the well-being and function of military personnel, their families, and civilians while simultaneously limiting U.S. combat readiness and performance. In order to facilitate the development, evaluation, and approval of promising treatments for these four chronic diseases, researchers require valid and sensitive clinical outcome measures that reliably detect relevant changes in all of the symptoms that are most important to patients. The U.S. Food and Drug Administration (FDA) has identified properly validated disease-specific patient reported outcome measures (PROMs) as an acceptable and, at times, preferred mechanism to measure therapeutic change during clinical trials and support drug labeling claims. Clinical trial infrastructure in mitochondrial disease, cardiomyopathy, rheumatoid arthritis, and dystonia is currently limited. The development, validation, and optimization of multifactorial disease-specific PROMs for these populations will significantly bolster this infrastructure. This research will utilize qualitative patient interviews with military personnel and civilians, four large cross-sectional studies, factor analysis, expert review, and longitudinal reliability testing to: 1) determine what symptoms are most important to patients with these diseases; and, 2) develop and validate four disease-specific instruments for these populations. This research will also identify which demographic subgroups are at risk of increased symptomatic burden and identify potential opportunities for early intervention in high-risk populations. The multifaceted state-of-the-art PROMs developed through this proposal are transformative technologies that will expedite relevant therapy development for military personnel, veterans, civilians, and families affected by these four common and undertreated diseases. Upon completion, this research will also provide an efficient mechanism to improve the monitoring and treatment of patients in the clinical setting.		

**15. SUBJECT TERMS**

mitochondrial disease, cardiomyopathy, rheumatoid arthritis, dystonia, chronic disease, patient-reported outcome measures, disease burden, civilian and military population, patient input, patient quality of life, therapeutic trials, treatment effectiveness, drug-labelling claims, clinical monitoring, reduce sample size requirements

**16. SECURITY CLASSIFICATION OF:****a. REPORT**

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**b. ABSTRACT**

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**c. THIS PAGE**

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**17. LIMITATION  
OF ABSTRACT**

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USAMRDC**19b. TELEPHONE NUMBER** *(include area code)*

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## INTRODUCTION:

As therapeutic agents for mitochondrial disease, cardiomyopathy, rheumatoid arthritis, and dystonia are being tested in clinical trials, it is essential to have quality clinical trial tools that are capable of measuring the symptoms and issues that are most important to patients with these diseases and effectively monitor changes in these patient populations. Existing outcome measures for these diseases have limitations. Properly validated, disease-specific instruments will allow patients to provide input into treatment effectiveness, facilitate the efficiency and reliability of clinical trials, provide researchers and clinicians with tools to measure multifactorial disease burden, promote therapeutics research, and help ensure the relevance of future therapies. This research grant will develop, fully validate, and optimize four disease-specific, highly responsive PROMs for use in therapeutic trials utilizing qualitative patient interviews, four large cross-sectional studies, factor analysis, expert review, and longitudinal reliability testing to develop and validate the appropriate instruments.

### 1. KEYWORDS:

mitochondrial disease, cardiomyopathy, rheumatoid arthritis, dystonia, chronic disease, patient-reported outcome measures, disease burden, civilian and military population, patient input, patient quality of life, therapeutic trials, treatment effectiveness, drug-labelling claims, clinical monitoring, reduce sample size requirements

### 2. ACCOMPLISHMENTS:

#### What were the major goals of the project?

As of August 15, 2023, we have now begun recruitment efforts in all four disease categories.

- Major Task 1:** Interview 15-25 patients in each disease category (MITD, CARDIO, RA, DYS) [Months 1-14] (ongoing)
- Major Task 2:** Conduct large cross-sectional studies in each disease category (MITD, CARDIO, RA, DYS) [Months 13-24]
- Major Task 3:** Analyze cross-sectional data [Months 24-34]
- Major Task 4:** Publish and present preliminary research data and results [Months 29-34]
- Major Task 5:** Beta-test first versions of disease-specific instruments by interviewing 15-20 patients from each disease category (MITD, CARDIO, RA, DYS) [Months 29-34]
- Major Task 6:** Evaluate Test-Retest reliability with 15-20 patients and perform final factor analysis in each disease category for each of the four HIs [Months 35-42]
- Major Task 7:** Perform Known Groups testing for each of the four Health Indices [Months 43-46]
- Major Task 8:** Publish and present comprehensive research data and results [Months 47-48]
- Major Task 9:** Include MITD-HI, CARDIO-HI, RA-HI, and DYS-HI in therapeutic trials and clinical settings [Post-Funding]

**What was accomplished under these goals?**

*For this reporting period describe: 1) major activities; 2) specific objectives; 3) significant results or key outcomes, including major findings, developments, or conclusions (both positive and negative); and/or 4) other achievements. Include a discussion of stated goals not met. Description shall include pertinent data and graphs in sufficient detail to explain any significant results achieved. A succinct description of the methodology used shall be provided. As the project progresses to completion, the emphasis in reporting in this section should shift from reporting activities to reporting accomplishments.*

**Specific Aim 1: To identify the symptoms that are most important to patients with mitochondrial disease (MITD), cardiomyopathy (CARDIO), rheumatoid arthritis (RA), and dystonia (DYS) using patient interviews and large cross-sectional studies.**

**Major Task 1:** Interview 15-25 patients in each disease category (MITD, CARDIO, RA, DYS) Months 1-14 (ongoing)

Complete  **Subtask 1:** Obtain internal Institutional Review Board (IRB) approval to recruit, consent, and enroll patients in minimal risk research

Ongoing  **Subtask 2:** Recruit participants from patient foundations/registries/clinics

**Subtask 3:** Conduct audio-recorded, semi-structured, qualitative interviews with patients to ascertain recurring and impactful symptoms that affect patients’ lives (N=15-25 per disease group; total N=60-100) Patients recruited from patient foundations/registries/clinics

**Subtask 4:** Transcribe, code, and analyze de-identified interview data to determine questions to be included in cross-sectional study

Our grant proposal projected 7 months in order to obtain IRB approval from the University of Rochester, Uniformed Services University of the Health Sciences (USUHS), and Walter Reed National Military Center (WRNMC), the three participating recruitment sites. However, due to circumstances beyond our control, the IRB process took a total of 15 months. We submitted our IRB application to the University of Rochester on 3/30/22 and received approval of 5/24/22; our collaborating partners at USUHS submitted the application on 9/7/22 and approval was obtained on 12/5/22; finally, the submission to WRNMC occurred on 1/31/23 and approval was not granted until 6/14/23. We received formal sign off from OHRO on 7/5/23 that we may begin study activities and recruitment activities for Subtask 2 are currently in progress. Participant recruitment (Subtask 2) and the conduct of interviews (Subtask 3) will be occurring concurrently. We have multiple recruitment partners from all three institutions with whom we are actively engaged and anticipate that this process will take four months as originally projected.

**What opportunities for training and professional development has the project provided?**

*If the project was not intended to provide training and professional development opportunities or there is nothing significant to report during this reporting period, state “Nothing to Report.”*

*Describe opportunities for training and professional development provided to anyone who worked on the project or anyone who was involved in the activities supported by the project. “Training” activities are those in which individuals with advanced professional skills and experience assist others in attaining greater proficiency. Training activities may include, for example, courses or one-on-one work with a mentor. “Professional development” activities result in increased knowledge or skill in one’s area of expertise and may include workshops, conferences, seminars, study groups, and individual study. Include participation in conferences, workshops, and seminars not listed under major activities.*

Nothing to Report

*If there is nothing significant to report during this reporting period, state “Nothing to Report.”*

*Describe how the results were disseminated to communities of interest. Include any outreach activities that were undertaken to reach members of communities who are not usually aware of these project activities, for the purpose of enhancing public understanding and increasing interest in learning and careers in science, technology, and the humanities.*

Nothing to Report

**What do you plan to do during the next reporting period to accomplish the goals?**

*If this is the final report, state “Nothing to Report.”*

*Describe briefly what you plan to do during the next reporting period to accomplish the goals and objectives.*

During the next reporting period (September 1, 2023-August 31, 2024), we plan to continue recruiting participants as outlined by Subtask 2 and conduct 60 to 100 qualitative interviews across the four diseases described by Subtask 3. These interviews will be transcribed, coded and analyzed for the creation of four cross-sectional studies as stated in Subtask 4, marking the completion of Major Task 1. Major Task 1 completion was estimated to take 14 months, however, it will likely extend to 16 months. The four cross-sectional surveys will be implemented using an online REDCap link distributed by internal and external recruitment sources during months 17-21. Months 22-23 will be devoted to accomplishing Major Task 3.

**Year 2 Goals & Objectives:**

**Subtask 2:** Recruit participants from patient foundations/registries/clinics

**Subtask 3:** Conduct audio-recorded, semi-structured, qualitative interviews with patients to ascertain recurring and impactful symptoms that affect patients’ lives (N=15-25 per disease group; total N=60-100) Patients recruited from patient foundations/registries/clinics

**Subtask 4:** Transcribe, code, and analyze de-identified interview data to determine questions to be included in cross-sectional study

**Major Task 2: Conduct large cross-sectional studies in each disease category (MITD, CARDIO, RA, DYS) [Months 15-21]**

**Subtask 1:** Recruit patients from internal sources, including University of Rochester clinics, Walter Reed, and the Uniformed Services University, and from external patient foundations and registries. Total N ~ 10,100

**Subtask 2:** Implement cross-sectional studies using online REDCap platform to collect demographics and symptom prevalence and impact data in a de-identified manner

- **Specific Aim 2: To develop patient-centered, disease-specific PROMs for four diseases: the Mitochondrial Disease-Health Index (MITD-HI), the Cardiomyopathy-Health Index (CARDIO-HI), the Rheumatoid Arthritis-Health Index (RA-HI), and the Dystonia-Health Index (DYS-HI), using FDA guidelines.**

**Major Task 3: Analyze cross-sectional study data [Months 22-23]**

**Subtask 1:** Analyze data of participant survey responses to remove questions of low importance to each disease population; execute factor analysis to arrange symptom questions into symptomatic theme subscales; and create first versions of MITD-HI, CARDIO-HI, RA-HI, and DYS-HI instruments.

- 4. IMPACT:** Describe distinctive contributions, major accomplishments, innovations, successes, or any change in practice or behavior that has come about as a result of the project relative to:

**What was the impact on the development of the principal discipline(s) of the project?**

*If there is nothing significant to report during this reporting period, state “Nothing to Report.”*

*Describe how findings, results, techniques that were developed or extended, or other products from the project made an impact or are likely to make an impact on the base of knowledge, theory, and research in the principal disciplinary field(s) of the project. Summarize using language that an intelligent lay audience can understand (Scientific American style).*

Nothing to Report

**What was the impact on other disciplines?**

*If there is nothing significant to report during this reporting period, state “Nothing to Report.”*

*Describe how the findings, results, or techniques that were developed or improved, or other products from the project made an impact or are likely to make an impact on other disciplines.*

Nothing to Report

**What was the impact on technology transfer?**

*If there is nothing significant to report during this reporting period, state “Nothing to Report.”*

*Describe ways in which the project made an impact, or is likely to make an impact, on commercial technology or public use, including:*

- *transfer of results to entities in government or industry;*
- *instances where the research has led to the initiation of a start-up company; or*
- *adoption of new practices.*

Nothing to Report

**What was the impact on society beyond science and technology?**

*If there is nothing significant to report during this reporting period, state “Nothing to Report.”*

*Describe how results from the project made an impact, or are likely to make an impact, beyond the bounds of science, engineering, and the academic world on areas such as:*

- *improving public knowledge, attitudes, skills, and abilities;*
- *changing behavior, practices, decision making, policies (including regulatory policies), or social actions; or*
- *improving social, economic, civic, or environmental conditions.*

5.

Nothing to Report

*the project or its direction. If not previously reported in writing, provide the following additional information or state, “Nothing to Report,” if applicable:*

**Changes in approach and reasons for change**

*Describe any changes in approach during the reporting period and reasons for these changes. Remember that significant changes in objectives and scope require prior approval of the agency.*

Nothing to Report

**Actual or anticipated problems or delays and actions or plans to resolve them**

*Describe problems or delays encountered during the reporting period and actions or plans to resolve them.*

Our study encountered a delay in obtaining the necessary IRB approval from the Uniformed Services University of the Health Sciences, and Walter Reed National Military Center, two of the three participating recruitment sites. Although we are beginning our study work several months later than anticipated, we have a strong network of recruitment partnerships which will allow us to conduct participant recruitment in a highly time efficient manner, enabling us to recover the lost time.

**Changes that had a significant impact on expenditures**

*Describe changes during the reporting period that may have had a significant impact on expenditures, for example, delays in hiring staff or favorable developments that enable meeting objectives at less cost than anticipated.*

Nothing to Report

**Significant changes in use or care of human subjects, vertebrate animals, biohazards, and/or select agents**

*Describe significant deviations, unexpected outcomes, or changes in approved protocols for the use or care of human subjects, vertebrate animals, biohazards, and/or select agents during the reporting period. If required, were these changes approved by the applicable institution committee (or equivalent) and reported to the agency? Also specify the applicable Institutional Review Board/Institutional Animal Care and Use Committee approval dates.*

**Significant changes in use or care of human subjects**

None

**Significant changes in use or care of vertebrate animals**

None

**Significant changes in use of biohazards and/or select agents**

None

6. **PRODUCTS:** *List any products resulting from the project during the reporting period. If there is nothing to report under a particular item, state “Nothing to Report.”*

- **Publications, conference papers, and presentations**

*Report only the major publication(s) resulting from the work under this award.*

**Journal publications.** *List peer-reviewed articles or papers appearing in scientific, technical, or professional journals. Identify for each publication: Author(s); title; journal; volume; year; page numbers; status of publication (published; accepted, awaiting publication; submitted, under review; other); acknowledgement of federal support (yes/no).*

Nothing to Report

**Books or other non-periodical, one-time publications.** *Report any book, monograph, dissertation, abstract, or the like published as or in a separate publication, rather than a periodical or series. Include any significant publication in the proceedings of a one-time conference or in the report of a one-time study, commission, or the like. Identify for each one-time publication: author(s); title; editor; title of collection, if applicable; bibliographic information; year; type of publication (e.g., book, thesis or dissertation); status of publication (published; accepted, awaiting publication; submitted, under review; other); acknowledgement of federal support (yes/no).*

Nothing to Report

**Other publications, conference papers and presentations.** *Identify any other publications, conference papers and/or presentations not reported above. Specify the status of the publication as noted above. List presentations made during the last year (international, national, local societies, military meetings, etc.). Use an asterisk (\*) if presentation produced a manuscript.*

Nothing to Report

- **Website(s) or other Internet site(s)**

*List the URL for any Internet site(s) that disseminates the results of the research activities. A short description of each site should be provided. It is not necessary to include the publications already specified above in this section.*

Nothing to Report

- **Technologies or techniques**

Identify technologies or techniques that resulted from the research activities. Describe the technologies or techniques were shared.

Nothing to Report

• **Inventions, patent applications, and/or licenses**

Identify inventions, patent applications with date, and/or licenses that have resulted from the research. Submission of this information as part of an interim research performance progress report is not a substitute for any other invention reporting required under the terms and conditions of an award.

Nothing to Report

• **Other Products**

Identify any other reportable outcomes that were developed under this project. Reportable outcomes are defined as a research result that is or relates to a product, scientific advance, or research tool that makes a meaningful contribution toward the understanding, prevention, diagnosis, prognosis, treatment and /or rehabilitation of a disease, injury or condition, or to improve the quality of life. Examples include:

- data or databases;
- physical collections;
- audio or video products;
- software;
- models;
- educational aids or curricula;
- instruments or equipment;
- research material (e.g., Germplasm; cell lines, DNA probes, animal models);
- clinical interventions;
- new business creation; and
- other.

Nothing to Report

**7. PARTICIPANTS & OTHER COLLABORATING ORGANIZATIONS**

**What individuals have worked on the project?**

Provide the following information for: (1) PDs/PIs; and (2) each person who has worked at least one person month per year on the project during the reporting period, regardless of the source of compensation (a person month equals approximately 160 hours of effort). If information is unchanged from a previous submission, provide the name only and indicate “no change”.

Example:

Name: Mary Smith

*Project Role:* Graduate Student  
*Researcher Identifier (e.g. ORCID ID):* 1234567  
*Nearest person month worked:* 5  
*Contribution to Project:* Ms. Smith has performed work in the area of combined error-control and constrained coding.  
*Funding Support:* The Ford Foundation (Complete only if the funding support is provided from other than this award.)

**Name:** Jennifer Weinstein  
*Project Role:* Lead Clinical Project Manager  
**ORCID ID:** 0000-0002-3984-2120  
*Nearest person month worked:* 1  
*Contribution to Project:* IRB preparation & submission, coordinator recruitment training  
*Funding Support:* Provided from this award

**Name:** Chad Heatwole, MD, MS-CI  
*Project Role:* PI  
**ORCID ID:**  
*Nearest person month worked:* 4  
*Contribution to Project:* All study oversight  
*Funding Support:* Provided from this award

**Name:** Alicia Brocht  
*Project Role:* Statistician  
**ORCID ID:**  
*Nearest person month worked:* 2  
*Contribution to Project:*  
*Funding Support:* Provided from this award

**Name:** Shaweta Khosa  
*Project Role:* Study Coordinator  
**ORCID ID:**  
*Nearest person month worked:* 6  
*Contribution to Project:* IRB preparation & submission  
*Funding Support:* Provided from this award

Continued

<i>Name:</i>	<b>Spencer Rosero</b>
<i>Project Role:</i>	Clinical Research Coordinator
<i>ORCID ID:</i>	
<i>Nearest person month worked:</i>	4
<i>Contribution to Project:</i>	IRB preparation & submission
<i>Funding Support:</i>	Provided from this award
<i>Name:</i>	<b>Jamison Seabury</b>
<i>Project Role:</i>	Clinical Research Coordinator
<i>ORCID ID:</i>	
<i>Nearest person month worked:</i>	4
<i>Contribution to Project:</i>	IRB preparation & submission
<i>Funding Support:</i>	Provided from this award
<i>Name:</i>	<b>Anika Varma</b>
<i>Project Role:</i>	Clinical Research Coordinator
<i>ORCID ID:</i>	
<i>Nearest person month worked:</i>	7
<i>Contribution to Project:</i>	IRB preparation & submission
<i>Funding Support:</i>	Provided from this award

**Has there been a change in the active other support of the PD/PI(s) or senior/key personnel since the last reporting period?**

*If there is nothing significant to report during this reporting period, state “Nothing to Report.”*

*If the active support has changed for the PD/PI(s) or senior/key personnel, then describe what the change has been. Changes may occur, for example, if a previously active grant has closed and/or if a previously pending grant is now active. Annotate this information so it is clear what has changed from the previous submission. Submission of other support information is not necessary for pending changes or for changes in the level of effort for active support reported previously. The awarding agency may require prior written approval if a change in active other support significantly impacts the effort on the project that is the subject of the project report.*

Please see separate PDF document for updated Other Support documents provided for all key personnel since grant submission

**What other organizations were involved as partners?**

*If there is nothing significant to report during this reporting period, state “Nothing to Report.”*

*Describe partner organizations – academic institutions, other nonprofits, industrial or commercial firms, state or local governments, schools or school systems, or other organizations (foreign or domestic) – that were involved with the project. Partner organizations may have provided financial or in-kind support, supplied facilities or equipment, collaborated in the research, exchanged personnel, or otherwise contributed.*

*Provide the following information for each partnership:*

*Organization Name:*

*Location of Organization: (if foreign location list country)*

*Partner’s contribution to the project (identify one or more)*

- *Financial support;*
- *In-kind support (e.g., partner makes software, computers, equipment, etc., available to project staff);*
- *Facilities (e.g., project staff use the partner’s facilities for project activities);*
- *Collaboration (e.g., partner’s staff work with project staff on the project);*
- *Personnel exchanges (e.g., project staff and/or partner’s staff use each other’s facilities, work at each other’s site); and*
- *Other.*

Our research group has reached out to the following disease-specific organizations to request their in-kind involvement with patient recruitment for our upcoming cross-sectional surveys. These surveys will be made available on REDCap and the public link may be distributed via social media posts, e-newsletters, or email.

**Rheumatoid Arthritis:** The Arthritis Foundation, Arthritis National Research Foundation, The American College of Rheumatology, Rheumatology Research foundation, The National Institute for Arthritis, Musculoskeletal & Skin Diseases, PARTNERS, CorEvitas registry, and the American Joint Replacement Registry.

**Cardiomyopathy:** DCM Foundation, Sarcomeric Human Cardiomyopathy Registry (SHaRe), Heart Failure Society of America, and the American Heart Association.

**Mitochondrial disease:** United Mitochondrial Disease Foundation (meeting next week), Mito Action Foundation, and The North American Mitochondrial Disease Consortium.

**Dystonia:** Dystonia Medical Research Foundation, the Bachmann-Strauss Dystonia & Parkinson Foundation, Dystonia Medical Research Foundation, Dystonia-Coalition – Rare Diseases Clinical Research Network, and the Uniformed Services University of the Health Sciences Dystonia Database.

## 8. SPECIAL REPORTING REQUIREMENTS

**COLLABORATIVE AWARDS:** *N/A*

**QUAD CHARTS:** *N/A*

## 9. APPENDICES: *N/A*