

Notice of Funding Opportunity Informational Webinar

Opportunity Name: Surveillance of Muscular Dystrophies

Opportunity Number: CDC-RFA-DD-24-0064

Federal Assistance Listing: 93.073

National Center on Birth Defects and Developmental Disabilities

Division of Birth Defects and Infant Disorders

Centers for Disease Control (CDC)

March 28, 2024

Surveillance of Muscular Dystrophies

- **Purpose:** conduct longitudinal, population-based surveillance of selected muscular dystrophies (MD), analyze data, and disseminate results to the MD community

Component A

Collect and manage data, submit data to CDC, analyze data, and disseminate information

Component B

Train abstractors, implement activities to improve data quality

Component C

Develop novel approaches to data collection, evaluate use of MD specific MD ICD codes

Surveillance of Muscular Dystrophies Goal and Focus

Overall goal: Improve the health and quality of life of people living with MD

Estimate

- Prevalence
- Mortality and survival

Describe

- Health care utilization
- Disease progression and comorbidities

Assess

- Access to care and new treatments
- Factors that lead to better outcomes

Surveillance of Muscular Dystrophy Background / Related Work

- The Muscular Dystrophy Surveillance Tracking and Research Network
- For more information, visit: [Muscular Dystrophy Research and Tracking | CDC](https://www.cdc.gov/muscular-dystrophy/research-and-tracking/)



Surveillance of Muscular Dystrophy

Key Activities – Component A

- Conduct longitudinal, population-based data collection on individuals with muscular dystrophies
- Access clinical and administrative data sources for case identification and data collection
- Use standardized database and applications provided by CDC for data collection and submit individual-level data with limited identifiers
- Routinely monitor data quality and improve quality as needed
- Collaborate with CDC and other grantees in the analysis of surveillance data and dissemination of findings to target audiences

Surveillance of Muscular Dystrophy

Key Activities – Components B and C

Component B:

- Train abstractors in standardized data collection for MDs
- Conduct activities to improve quality of data collection

Component C:

- Develop, implement, and evaluate novel approaches to case-finding and data collection
- Develop and implement a study to evaluate the use of MD-specific ICD-10 codes by source

Organizations applying for Component A are not required to apply for other components. However, organizations applying for Components B and/or C must apply for Component A.

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Expected Awards / Performance Periods

Component A

Award **between 5 and 7 recipients**

for

4-year period of performance

(four 12-month budget periods)

Component B

Award **1 recipient**
for

4-year period of performance

(four 12-month budget periods)

Component C

Award **up to 2 recipients**
for

2-3 year period of performance*

(two to three 12-month budget periods)

*Depending on proposed project, funding may start after year 1

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Expected Awards / Budgets

Component A

Expected total program funding per budget period: \$2,250,000 – 3,500,000

Expected total program funding over the performance period:
\$9,000,000-\$14,000,000

Expected average award amount per budget period:
\$450,000 - \$500,000

Component B

Expected total program funding per budget period: \$100,000

Expected total program funding over the performance period:
\$400,000

Expected average award amount per budget period:
\$100,000

Component C

Expected total program funding per budget period: \$200,000

Expected total program funding over the performance period:
\$800,000

Expected average award amount per budget period:
\$100,000

Surveillance of Muscular Dystrophy

Eligibility: Components B and C



- You must demonstrate that you have applied for Component A by providing a copy of the face page from your Component A application.



Surveillance of Muscular Dystrophies

Focus Population Eligibility

- Population in a state or portion of state with at least 3 million in the surveillance area
- Individuals diagnosed with one of the following MDs:
 - Duchenne muscular dystrophy (DMD)
 - Becker muscular dystrophy (BMD)
 - Myotonic dystrophy (DM)
 - Facioscapulohumeral muscular dystrophy (FSHD)
 - Limb-girdle muscular dystrophy (LGMD)
 - Congenital muscular dystrophy (CMD)
 - Emory-Dreifuss muscular dystrophy (EDMD)
 - Distal muscular dystrophy
- Health encounter on or after January, 1 2015
- Residency in the surveillance area at any time after January 1, 2015

Surveillance of Muscular Dystrophies

Focus Population

- Applicants should describe their surveillance area in the project narrative of the application and include:
 - geographic boundaries (e.g., entire state, a region or regions that border each other within a state, or bordering counties and population size)
 - If proposing an area smaller than the entire state, proportion of the state population covered by this area (e.g., the proposed surveillance area, comprising 10 counties, covers 70 percent of the state population)
 - Distribution of the population by race and ethnicity

Surveillance of Muscular Dystrophy

Strategy 1: Surveillance Capacity (Component A)

- Conduct longitudinal, population-based data collection on individuals with muscular dystrophies
 - Build on MD STARnet surveillance methods to identify eligible individuals with MDs
 - Collect demographic, diagnostic, and key clinical data from medical records
 - Build and maintain existing relationships to conduct and enhance case ascertainment and data collection
 - Engage in data modernization activities including testing novel approaches to data collection
 - Develop a site-specific surveillance manual or standard operating procedure
- Access clinical and administrative data sources for case identification and data collection
 - Access multiple sources to generate an accurate and complete population-based cohort
 - Must have access to both medical records **AND** administrative data sources
 - Use clinic sources to determine other healthcare sources where individuals with MD receive specialty care
 - Collaborate with recipients who share a geographic border to obtain medical record data of eligible individuals
 - Use address tracing services to obtain address histories on eligible cases

Surveillance of Muscular Dystrophy

Sources and Data Elements

Sources

Electronic health records

- **Neuromuscular clinics**
- **Specialty care (e.g., cardiology)**
- Other outpatient clinics
- **Hospitals**

Administrative Data

- **Vital records**
- **Hospital discharge**
- Medicaid/Medicare



Data Elements

Sociodemographics

Diagnosis (signs and symptoms, tests)

Clinical assessments

Clinical interventions

Medications

Procedures related to MD care

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Strategy 2: Data Management and Reporting (Component A)

- Use standardized database and applications provided by CDC for data collection and submit individual-level data with limited identifiers
 - Abstract data from medical and vital records into standardized database
 - Provide data to CDC on community indicators (e.g., socio-economic status) by linking to census data
 - Provide individual-level data to CDC
 - Pooled analytic data sets for each MD that contain limited identifiers will be provided to all recipients
- Routinely monitor data quality and improve quality as needed
 - Participate in initial and ongoing abstractor training and data quality activities
 - Clean local data and perform data quality checks according to protocols

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Strategy 3: Dissemination (Component A)

- Collaborate with CDC and other project sites to analyze and disseminate MD surveillance data
 - Collaborate in identifying knowledge gaps and prioritizing analyses
 - Propose and describe at least 3 project ideas that are expected to be developed into manuscripts
 - Present project results at least 3 times to target audiences.
 - Conduct secondary analysis for at least 1 other research project led by another recipient or CDC
 - Participate in the development of analytic variables
 - Participate in analyses, abstracts, and manuscripts led by other recipient or CDC investigators
 - Build and maintain partnerships with the regional and local MD community to inform community about surveillance results
 - Collaborate with CDC and national MD partners to inform community about surveillance results

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Strategy 1: Abstractor training and data quality improvement (Component B)

- Train, certify, and provide ongoing training to abstractors in the standardized collection of data from medical records
- Conduct data quality activities (e.g., inter-rater reliability exercises) to improve data quality of data collection from medical records
- Develop, modify, and update training materials and manuals
- Communicate regularly with abstractors through leading monthly abstractor calls and providing assistance to recipient sites as needed
- Provide technical assistance as needed through participation in committees, workgroups, and principal investigator meetings

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Strategy 1: Efficiencies in Data Collection (Component C)

- Develop, implement, and evaluate novel approaches to case-finding and data collection
 - Develop data extraction tool (e.g. machine learning, natural language processing) to reduce abstractor effort and time to abstract cases
 - Pilot data extraction using clinic sources within site(s) and compare with current method of data abstraction
 - Develop documentation that clearly describes the functionality of the data extraction tool
- Develop and implement study to evaluate the use of MD-specific ICD-10 codes by source
 - Development a plan to collect MD ICD-10 codes for eligible individuals by source where cases were ascertained
 - Collect data to determine the use of MD-specific ICD-10 codes by source.
 - Analyze data and publish findings

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Outcomes / Measuring Progress

- **Component A**
 - Increased awareness and understanding among providers, patients, caregivers about:
 - Disease prevalence, mortality/survival, care utilization, access to care and new treatments, disease progression and comorbidities, and factors that lead to better outcomes
- **Components A, B, and C**
 - Improved accuracy and efficiency in case finding and data collection
 - Improved data quality and availability of data for action
- **Component C**
 - Improved understanding of the accuracy of ICD codes in identifying individuals with MD

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Organizational Capacity (Component A)

- Describe organization's capacity to perform **project activities**, including:
 - Program and performance management and evaluation
 - Informatics, including experience with data interoperability, systems development and support, data security, data management (including data linkage, cleaning, and quality improvement)
 - Data dissemination, including presenting and publishing data on MD or similar topics
- Describe the **staff responsible** for these activities, the percentage of time they will devote to the project (such as full-time equivalent)
 - Include personnel with expertise in surveillance and epidemiology, MD knowledge and clinical expertise, medical record abstraction, data management, and data analysis
 - Personnel including the principal investigator should have a sufficient level of effort to achieve project outcomes.

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Organizational Capacity (Component A)

- Describe your organization's capacity to **implement population-based MD surveillance**, addressing the following requirements:
 - Demonstrate experience conducting surveillance for MD or rare disease/birth defects
 - Provide documentation such as MOUs/MOAs, DUAs
 - Public health authority to conduct surveillance for MD is strongly encouraged
 - Describe access to clinical and administrative data to identify and collect data on individuals with MD
- Describe ability to submit standardized data with limited identifiers to CDC for inclusion in a pooled dataset access by CDC and other recipients

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Organizational Capacity (Components B and C)

Component B

- Describe experience in conducting abstractor training and data quality improvement activities

Component C

- Describe experience related to the content area of their proposed project (e.g., development of machine learning algorithms/data extraction tools)

Components B and C

- Describe resources and staffing roles and responsibilities

Surveillance of Muscular Dystrophies Collaborations

- Recipients will be expected to collaborate and coordinate with other award recipients and CDC. Examples include:
 - Identifying key public health and clinical questions
 - Coordination of methods for case identification, classification, and data collection
 - Participating in abstractor training and data quality activities
 - Engaging in data modernization activities
 - Evaluating progress
 - Participating on committees and workgroups
 - Analyzing, presenting and publishing multisite data

Surveillance of Muscular Dystrophy Collaborations

- Recipients will be expected to collaborate with relevant local or regional partners to perform strategies and activities
 - Collaborate with state health departments or other state agencies to:
 - Maintain or establish public health authority for MD surveillance
 - Facilitate access to administrative data
 - Communicate information gathered from surveillance that can be useful to states in tracking their patient populations and informing changes in policy and services
 - Collaborate with local and regional patient advocacy groups and clinicians who treat individuals with MD to assist in disseminating results from surveillance data
- Recipients must be able to access multiple sources within their site that enables creation of a population-based cohort of individuals with MD
 - Submit letters of support, MOUs, MOAs, or DUAs that enable recipient to conduct surveillance and access records

Surveillance of Muscular Dystrophies

Evaluation Plan

- The evaluation and performance measurement strategy will assess project performance
- Recipient will track evaluation and performance measures and report progress to CDC using a CDC-defined format and timeline
- With your application, you must include an evaluation plan that:
 - Aligns with strategies and activities from the logic model
 - Includes performance measures (see examples in NOFO)
 - Describes assigned personnel
 - Includes a timeline
 - Describes expected outcomes
 - Describes the use of results to improve your program
- Recipients will be required to submit a more detailed evaluation plan, including a data management plan, if applicable, within the first 6 months of award

Surveillance of Muscular Dystrophies

Data Management Plan (DMP)

- For Component A, applicants must include a draft data management plan that includes:
 - Description of data to be collected
 - Standards to be used for collected data
 - Mechanisms for, or limitations to, providing access to the data, including a description for the provisions for the protection of privacy, confidentiality, security, and intellectual property
 - Statement of the use of data standards that ensure all documentation describes the method of collection and what the data represent
 - Plans for archiving and long-term preservation of the data, or explaining why long-term preservation and access are not justified
- CDC will provide guidance for updating or revising the DMP
- A DMP is not required for Component B. For proposed Component C projects, a DMP will be required if the project involves data collection or generation

Surveillance of Muscular Dystrophies Work Plan

- Provide workplan with high-level overview of entire project period and more detailed description of the first award year
- For each component, the first-year work plan should include:
 - Program strategies
 - Program activities
 - Performance measures
 - Timeframe
 - Persons responsible
 - Activity completion date
- Proposed project period timeline and sample work plan is on page 17

Surveillance of Muscular Dystrophies Applications

- Applications include **5 main elements**
- One application should be submitted even if applying for multiple components
 - 15 pages maximum for base project description
 - 4 additional pages per component for subsections specific to each component
- If you are applying for Components A, B, and/or C, you must include the 5 main elements for **each** component
- **Please see pages 27–31 of the NOFO for application contents and format**

Element

Project Abstract

Project Narrative

Budget Narrative Justification

Attachments

Standard Forms

Submission Form

Use the Project Abstract Summary form

Use the Project Narrative Attachment form

Use the Budget Narrative Attachment form

Insert each in the Other Attachments form

Upload using each required form

Surveillance of Muscular Dystrophies

Key Dates

- **Application deadline:** May 8, 2024 (Electronically submitted applications must be submitted no later than 11:59 pm ET)
- **Optional letter of intent deadline:** April 8, 2024
- **Expected Award Date:** August 1, 2024
- **Expected Start Date:** September 1, 2024
- **Expected Project End date:** August 31, 2028



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Questions:

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The findings and conclusions in this report are those of the authors and do not necessarily represent the official position of the Centers for Disease Control and Prevention.

