



DMD Data Set Project

Roll Out Call

April 2021

Agenda

1. Introductions
2. TGDOC Survey data & your registry
3. DMD dataset v1.1 for CR Registries & 2013 version
4. Global Registries Platform overview
5. Dataset Specification Tool
6. DMD dataset v1.1 for PR Registries
7. Project roll out

Introductions

1. John McKenna: DMD Dataset Project Manager
2. Janet Wilkins: DMD Dataset Project Coordinator
3. Caroline Ogden: Global Registries Platform (GRP)
Project Manager
4. Susan Cardiff: Global Registries Platform (GRP)
Data Systems Support

Confirmation of TGDOC Survey data 30/11/20:

(Qs1&3) Registry Name & Location:

LUKAM Registry, Lokman Hekim University Neuromuscular Diseases Application and Research Center, Ankara Turkey

Curator/PI

(Primary) Ayse Karaduman ayse.karaduman@lokmanhekim.edu.tr, Azize Reda Tunc reda.tunc@lokmanhekim.edu.tr

PI: Haluk Topaloglu, htopalog@hacettepe.edu.tr

(Q16) Total Patients:

No. of patients 278

(Q21) DMD Patients :

No. of patients 112 (Genetic confirmation 110)

(Q20/21/22/28) Disease Areas:

DMD/CMD/CMT/LGMD/SMA

(Q32) TREAT-NMD DMD Dataset Collected:

All 23 Mandatory & Highly Encouraged items (2013 version)

(Q49) Current Registry Platform:

TBC

(Qs 29/30/31/42) Reporting & Enquiries:

Clinician reported, updated every 6 months, only collect data from patients in the local Ankara region of Turkey

DMD Dataset for CR Registries v1.1

Vs

Current 2013 Version



2013 Dataset

23 Data Items

14 mandatory items

9 Highly Encouraged Items

4 broad categories

Examples but no guidance

V1.1 Dataset

136 unique data items

72 mandatory &

64 non mandatory items

21 defined categories

Examples, data rules & tools

- An Excel version of the dataset is available to download & review now.
- It can be accessed via the TREAT-NMD website and [this link](#)
- We're currently developing web-based dataset tool to support curators

Global Registries Platform overview

Caroline Ogden/Susan Cardiff:
Global Registries Platform
(GRP)
Project Manager

Dataset Specification Tool

- A web-based support portal aimed at Registry Curators
- Is separate to, but accompanies & supports, the new Dataset and your current registry platform OR the GRP
- The tool provides:
 - A list of the data items for the dataset
 - Detailed descriptions, definitions, and supporting information
 - Dataset appendix & example questions
 - A feedback feature to log any queries direct with TREAT-NMD
- Registry feedback will inform regular dataset revisions
- The tool is currently in development, but a prototype is available [here](#) as a guide

- We are currently working with a Focus Group to agree a PR registry view of the dataset
- Once feedback is completed, & changes adopted we will seek approval from the projects Principal Investigator and Chairs of the TGDOC
- Once approved we will ratify the dataset with the full Working Group & formally publish the v1.1 of the DMD dataset for Patient Reported registries
- Work is ongoing & currently expected to complete in April

Next Steps

- Engage with TGDOC Registries to share recent project progress
- Collaborate & understand the likely challenges involved in implementing the new dataset
- Support Registries to implement DMD Dataset into their existing systems
- OR to use the TREAT-NMD Global Registries Platform (GRP) to collect the data

Thoughts/Feedback
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