THE FEASIBILITY OF USING US CLAIMS DATA TO ASSESS OUTCOMES IN DUCHENNE MUSCULAR DYSTROPHY

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BACKGROUND

- Numerous types of data exist to assess outcomes among patients with Duchenne muscular dystrophy (DMD)
  - These include measures of clinical status, functional assessments, measures of key clinical milestones, or patient-reported outcomes (PROs)
- However, real-world datasets including these measures to inform the understanding of DMD progression are few
- While administrative claims data are commonly used for health research, their utility for assessing outcomes over time among patients with DMD is unclear

OBJECTIVE

- To assess the suitability of US health insurance claims datasets for assessing outcomes among those with DMD

METHODS

Study Approach

- A literature review was conducted to identify outcomes that have been previously measured to characterize the natural history of DMD
- An assessment of the availability of health insurance claims data by which to assess such outcomes was performed

Outcomes Measures Identified

- Clinical measures: biomarker levels (e.g. dystrophin levels) or key clinical milestones (e.g. age at loss of ambulation, LOA)
- Functional assessments: tests of a patient's physical capabilities (e.g. the six-minute walk test [6MWT])
- PROs: patient assessment on standardized health-related quality of life (HRQoL) scales

METHODS, CONT.

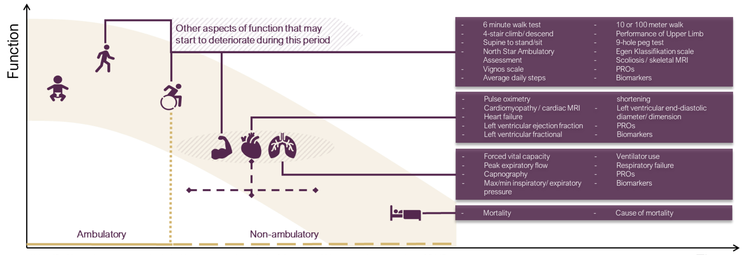
Data Source

- IBM MarketScan Commercial and anonymized Multi-state Medicaid claims data (2013-2018) were used to identify males ≤30 years old with DMD (ICD-9: 359.1, ICD-10: G71.0) from whom the availability of data to inform outcomes would be assessed
- These datasets include individual linked data on:
  - Outpatient visits
  - Hospitalizations
  - Medications

RESULTS

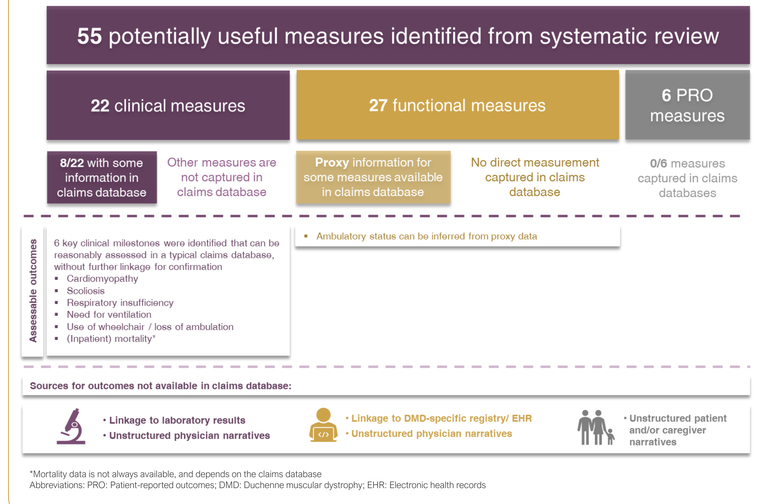
- 55 outcome measures relevant for DMD were identified from the literature review, including (**Figure 1**):
  - 22 clinical measures, 27 functional assessments, and 6 PRO measures
  - 6 of the clinical measures were key clinical milestones (e.g. age at LOA, onset of cardiomyopathy) assessed based on physician report
- Review of target measures against the MarketScan data (**Figure 2**) revealed that:
  - Outcomes that measure clinical or functional status were mostly *not* able to be assessed:
    - Records of clinical assessments, such as spirometry or cardiac MRI, can be identified
    - However, this only provides evidence of a test being performed, and does not yield the actual measurement required to assess severity or even indicate abnormal findings
  - Outcomes related to key clinical milestones could be captured using specific data fields as a proxy measure (**Table 1**)

Figure 1. Commonly-reported clinical, functional, and PRO measures* to track DMD patient functional status and severity over time



*Identified based on measures included in a systematic review of the natural history and progression of DMD.

Figure 2. The availability of commonly-reported clinical, functional, and PRO measure data within US claims datasets



*Mortality data is not always available, and depends on the claims database
Abbreviations: PRO: Patient-reported outcomes; DMD: Duchenne muscular dystrophy; EHR: Electronic health records

Table 1. Data available within claims datasets to inform measures of key clinical milestones*

Milestone	Corresponding measure in claims data
LOA	▪ HCPCS & CPT codes for wheelchairs ▪ ICD diagnosis codes to capture difficulty walking**
Scoliosis	▪ ICD diagnosis codes ▪ HCPCS, CPT, and ICD procedural codes for spinal surgery
Cardiomyopathy	▪ ICD diagnosis codes ▪ NDC medication codes ▪ CPT procedural codes for MRI
Heart failure	▪ ICD diagnosis codes
Respiratory insufficiency	▪ ICD diagnosis codes for respiratory failure ▪ HCPCS, CPT, and ICD procedural codes for tracheostomy, pulmonary management, and assisted ventilation
Mortality	▪ Inpatient death data (limited to before 2016)

*May be directly measured, or using a code for a procedure or diagnosis that is a proxy for the key clinical outcome under study
**not frequently used
HCPCS: Healthcare Common Procedure Coding System; CPT: Current Procedural Terminology; ICD: International Classification of Diseases; NDC: National Drug Codes

CONCLUSIONS

- Limitations to the analyses include that the two datasets used may not be generalizable to all claims datasets; and that no electronic health record data were available to assess their suitability for measuring similar outcomes
- In these standard commercial and Medicaid claims datasets, data to directly track functional outcomes or PROs in DMD are unavailable
- While the occurrence of some key clinical milestones may be inferred, severity cannot
- The reliability and completeness of proxy data is presently unknown and requires further investigation
- Ascertainment may be incomplete for outcomes relying on events that can occur outside of claims (e.g., mortality)

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