# Costs and Healthcare Resource Utilization Evaluation in Myotonic Dystrophy Type 1: Results from the Real-world CARE-DM1 Study

Johanna I. Hamel[1], Aaron Novack[2], Erwan Delage[2], Mihail Samnaliev[3], Diane Ito[3], Ashish Dugar[2]

[1]Department of Neurology, University of Rochester Medical Center, Rochester, NY, USA; [2] Dyne Therapeutics Inc., Waltham, MA, USA; [3] Stratevi LLC, Santa Monica, CA, USA.

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# Disclosures

- I am an employee of Dyne Therapeutics, Inc. and own stock in the company

# Background

- Myotonic dystrophy type 1 (DM1) is a multi-systemic disease affecting multiple tissue types including skeletal and smooth muscle, eye, brain, and heart.[1,2]
- No disease-modifying therapies are currently available, highlighting an unmet care gap.
- Previous studies assessing the economic burden in myotonic dystrophy have not differentiated between DM type 1 and type 2.[3,4]
- This retrospective real-world study aimed to:
  - Characterize the demographic and clinical profiles of DM1 patients in the U.S.
  - Evaluate their healthcare resource utilization (HRU) and associated costs, both overall and by organ system involvement following a diagnosis of DM1

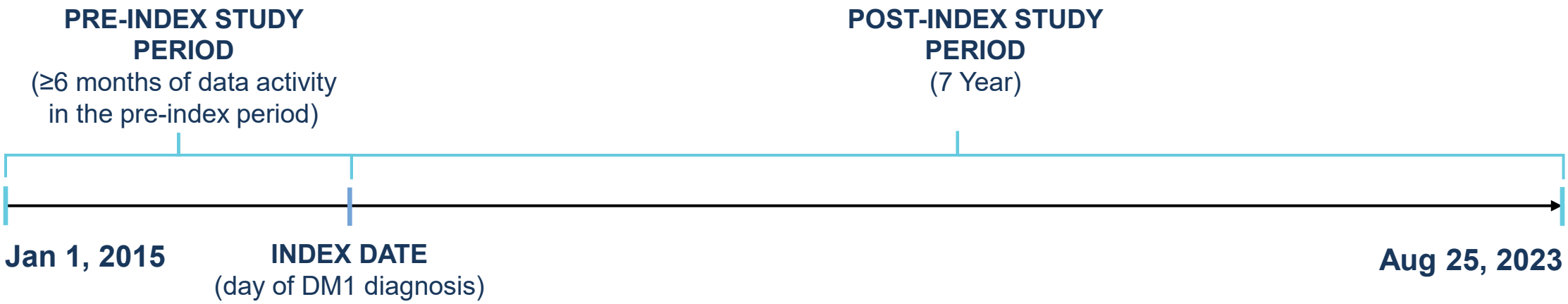
1. Ashizawa T, et al. *Neurology: Clinical Practice* 2018;8:507-520. 2. Orphanet. Steinert myotonic dystrophy. Available online: https://www.orpha.net/en/disease/detail/273?name=myotonic%20dystrophy%20type%201&mode=name (Accessed on 3/6/2024). 3. Howe SJ, et al. *Orphanet J Rare Dis*. 2022;23:17:79. 4. Larkindale J, et al. *Muscle Nerve*. 2014;49:431-438.

# Methods

**PATIENT SELECTION**
- DM1 diagnosis based on SNOMED CT 77956009
- Individuals diagnosed with DM1 at age ≥ 12 years
- Excluding individuals with congenital myotonic dystrophy

**BASELINE CHARACTERISTICS**
- Age, gender, race/ethnicity, region, payer type, comorbidities

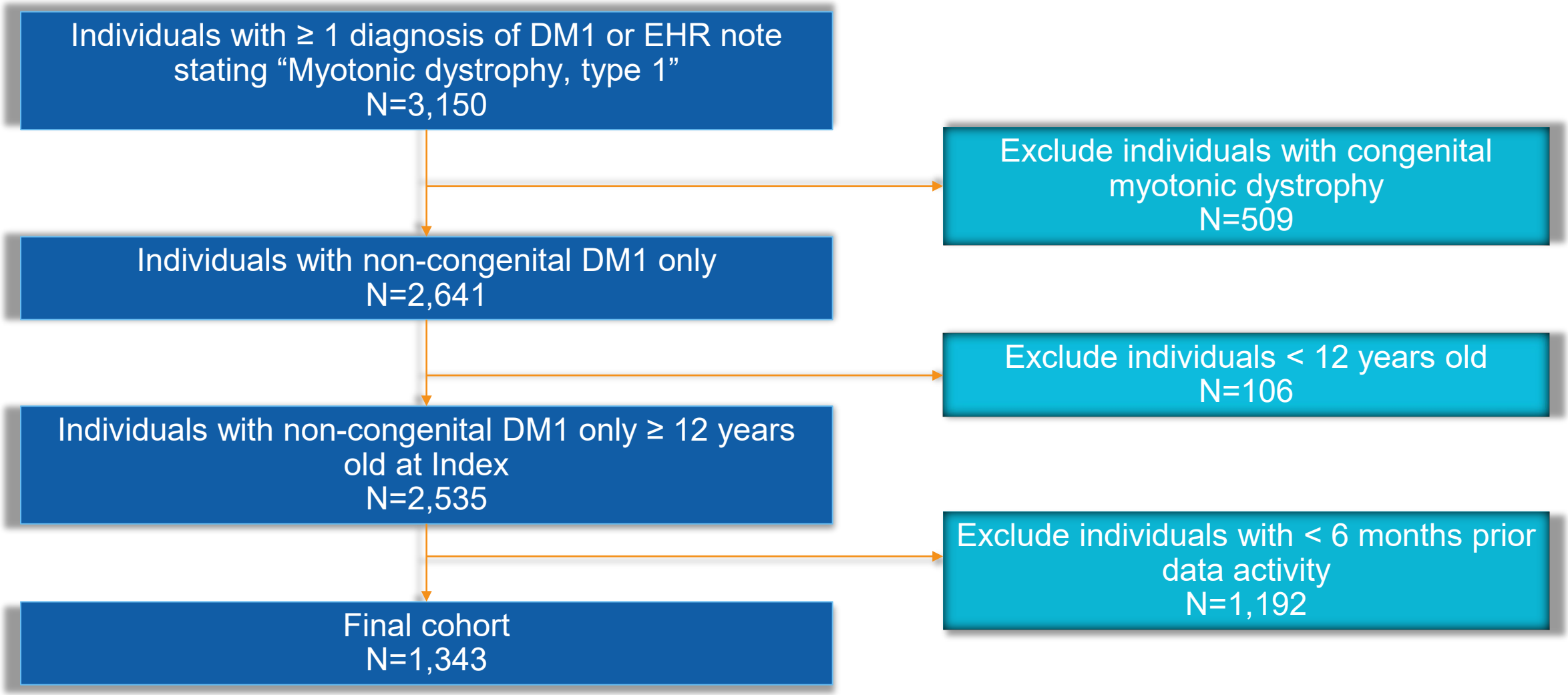
**OUTCOMES**
- Organ system involvement, specialist visits, select procedures and devices, select medications, total costs




- Used Clarivate Real-World Database – 300 million unique patients, with 100 million linked EHR and claims
- Incident organ system involvement was estimated post DM1 diagnosis using the Kaplan-Meier method
- Annual HRU and all-cause costs (2023 U.S. Dollars) post DM1 diagnosis were evaluated by type & settings

DM1, myotonic dystrophy type 1; EHR, electronic health record; HRU, healthcare resource utilization; SNOMED CT, systematized nomenclature of medicine clinical term

# Patient Flow



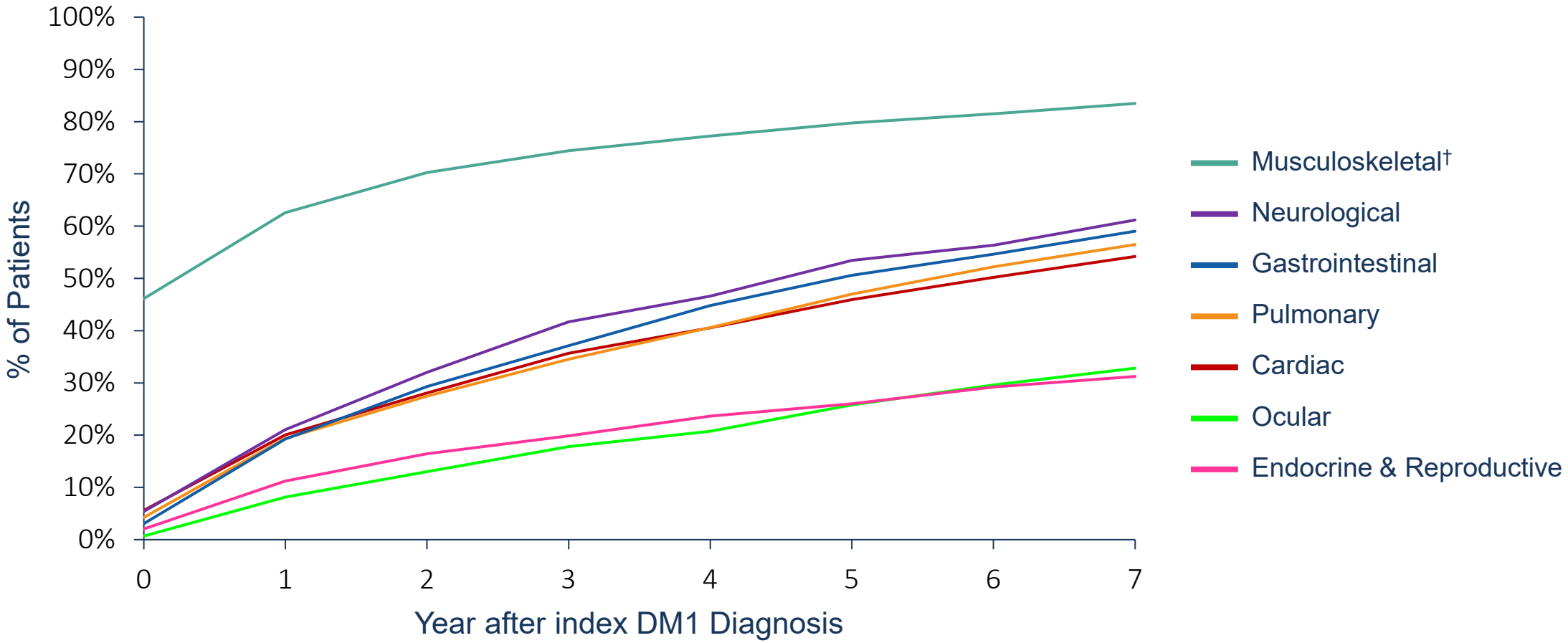

DM1, myotonic dystrophy type 1; EHR, electronic health record

# Demographic & Clinical Characteristics of Individuals with DM1

| Characteristics | N=1,343 |
|---|---|
| **Female**, n (%) | 735 (54.7%) |
| **Age** | |
| Mean (SD) | 47.6 (16.1) |
| Median (Min-Max) | 48 (12-86) |
| **Race/Ethnicity**, n (%) | |
| African American | 21 (1.6%) |
| Asian | 8 (0.6%) |
| Hispanic | 76 (5.7%) |
| White (Non-Hispanic) | 588 (43.8%) |
| Other/Unknown | 650 (48.4%) |
| **Payer**, n (%) | |
| Private | 711 (52.9%) |
| Public* | 632 (47.1%) |
| **Charlson Comorbidity Index (CCI)**, n (%) | |
| 0 | 824 (61.4%) |
| 1 to 2 | 346 (25.8%) |
| 3+ | 173 (12.9%) |
| Mean (SD) | 1.0 (1.8) |

* Including Medicaid (n=121), Medicare (n=225) and VA/Other (n=286)

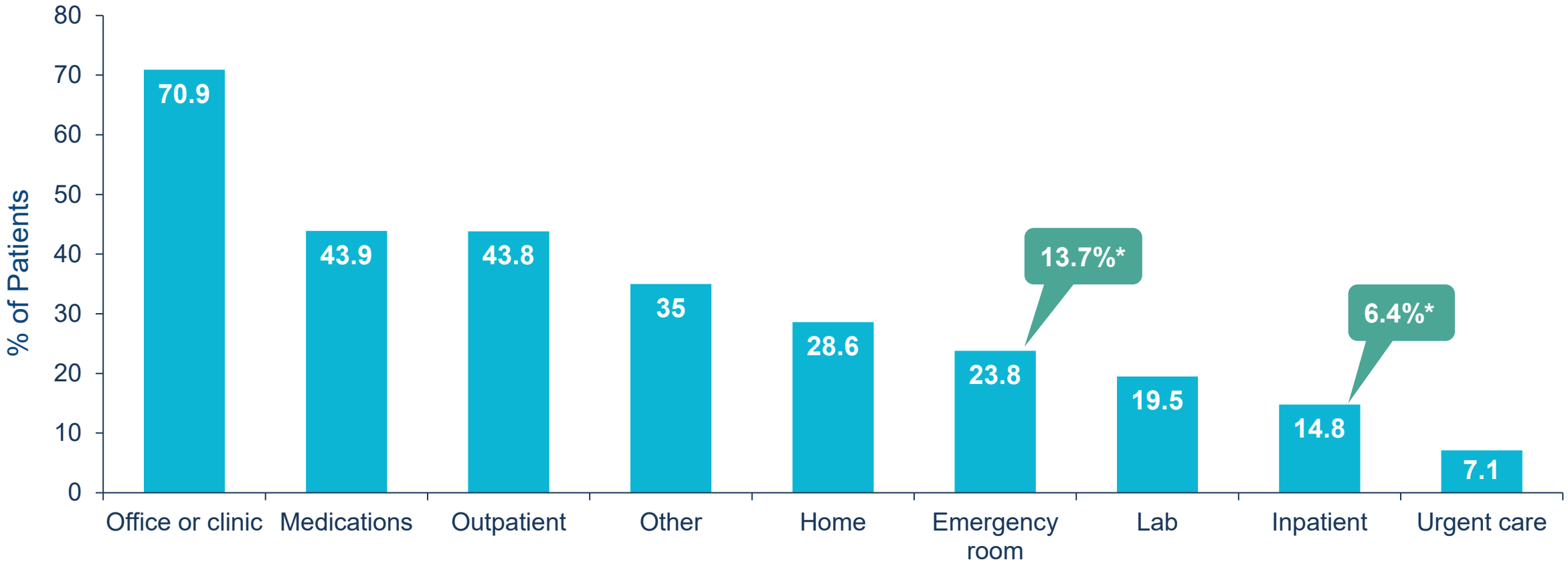
# Cumulative Incidence of Organ System Involvement After DM1 Diagnosis




**Most individuals with DM1 have organ system involvement that increases over time, pointing to significant clinical burden**

Patients with the respective organ system involvement prior to index were excluded to capture incident organ system involvement after DM1 diagnosis
† Including DM ICD codes in the definition of musculoskeletal organ system involvement
DM1, myotonic dystrophy type 1; ICD, international classification of disease

# Annual Utilization of Healthcare Services by Setting After DM1 Diagnosis



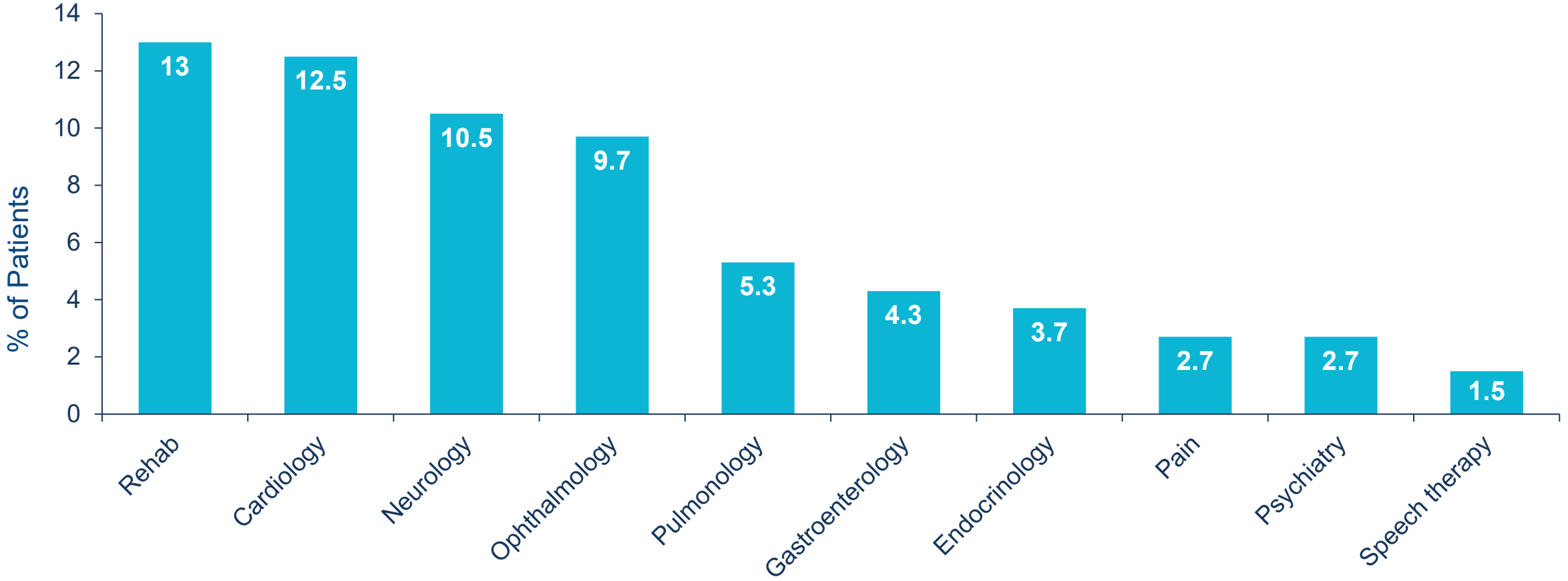

**DM1 is associated with high rates of healthcare resource utilization across settings, exceeding the ones in the general US population**

* Annual rates observed in the general US population based on 2017 data from the Medical Expenditure Panel Survey[1]

Mortensen K, et al. *Med Care.* 2021;59:704-710

DM1, myotonic dystrophy type 1

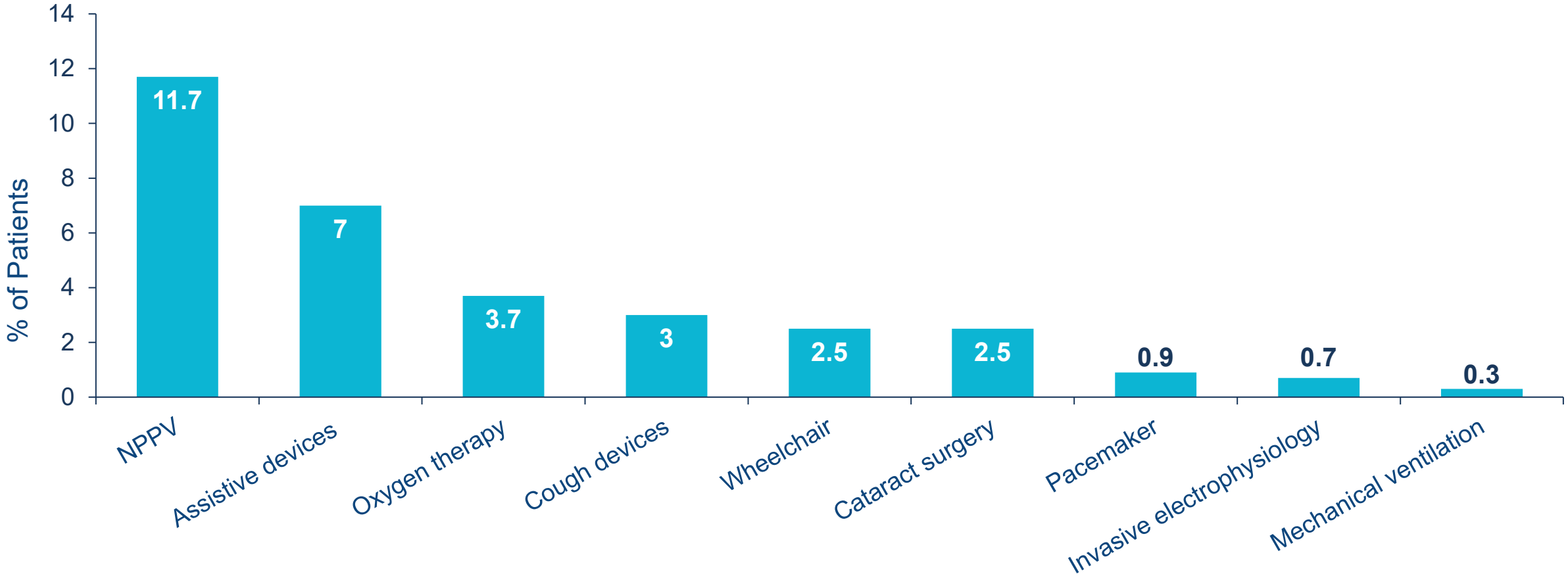
# Annual Utilization of Healthcare Services by Specialty After DM1 Diagnosis




**DM1 is linked to high rates of various types of specialty visits, highlighting the disease's medical complexity and heterogeneity**

DM1, myotonic dystrophy type 1

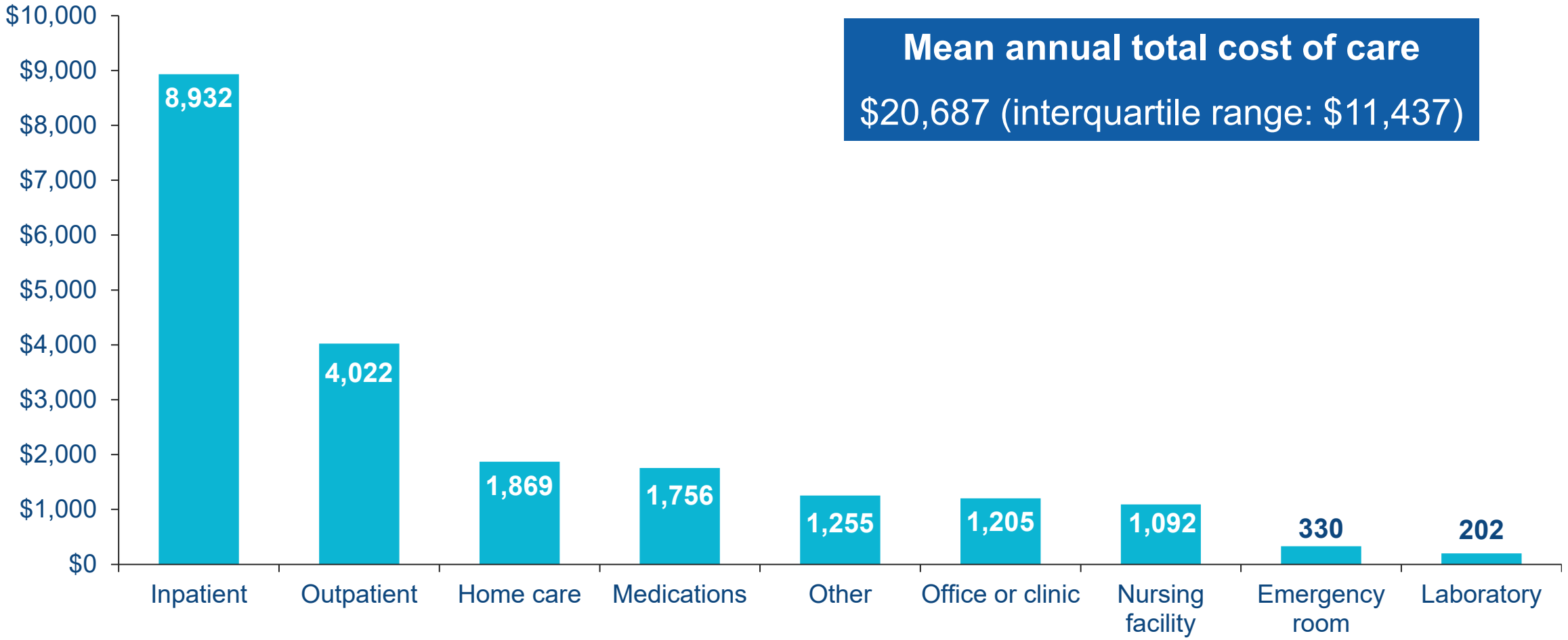
# Annual Utilization of Select Devices and Procedures After DM1 Diagnosis




**DM1 is linked to high utilization across various types of devices and procedures, highlighting the disease's medical complexity and heterogeneity**

Assistive devices include cane, walker, orthoses, etc
NPPV, non-invasive positive pressure ventilation; DM1, myotonic dystrophy type 1

# Annual Healthcare Costs (2023 USD) After DM1 Diagnosis



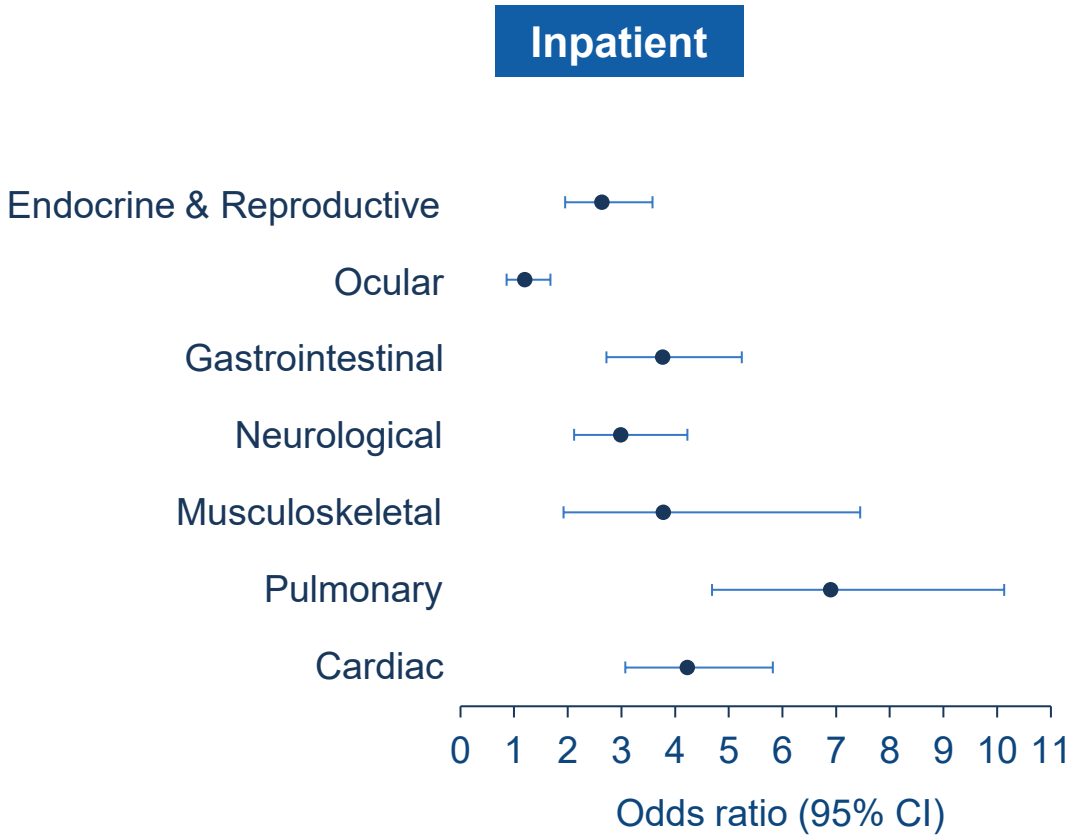

**Mean annual total cost of care**

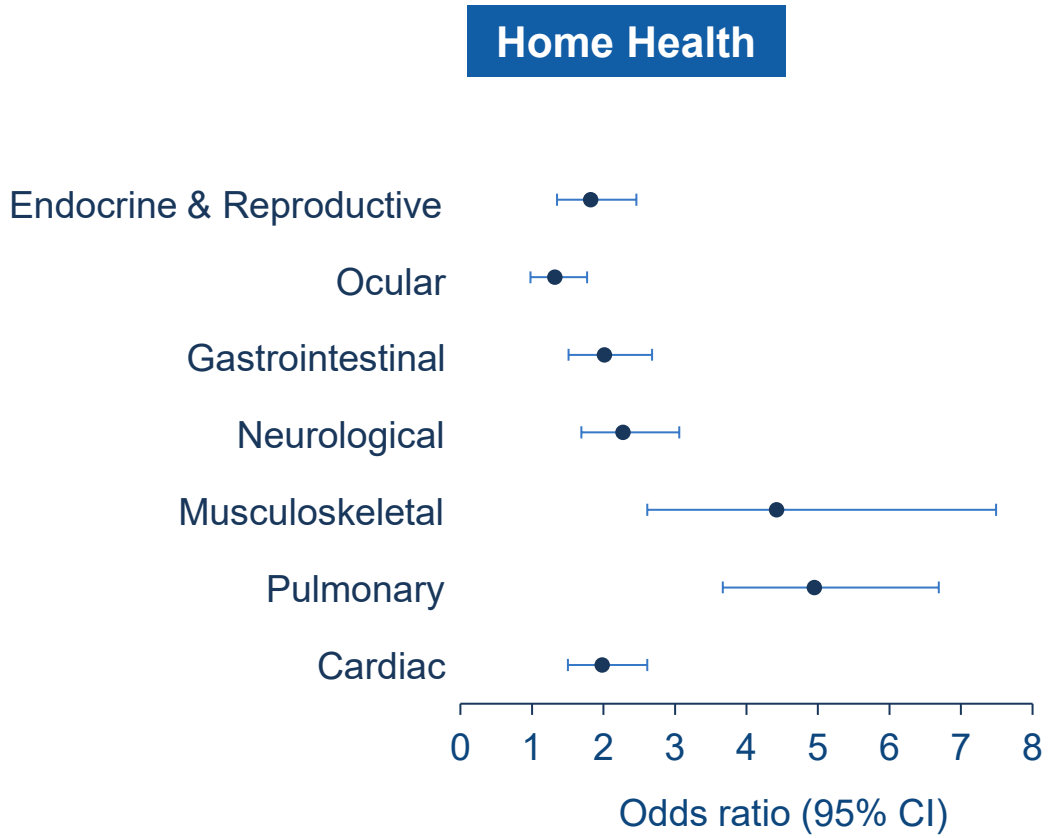
$20,687 (interquartile range: $11,437)

DM1 is associated with high costs of care across settings, underscoring the economic burden of disease

USD, United States dollar

# Regression-adjusted Utilization and Costs Associated with Organ System Involvement After DM1 Diagnosis




**Organ system involvement in DM1 is associated with substantially elevated resource utilization across settings, most notably with inpatient and outpatient hospital care, which are the top cost drivers in this population**

Comparing annual utilization between individuals with to those without each organ system involvement post DM1 diagnosis, using multivariable logistic regression models adjusting for age, gender, race/ethnicity, baseline Charlson Comorbidity Index, payer type, and geographic region

# Study Strengths and Limitations

## Strengths

- Study was based on a large dataset from all U.S. geographic regions
- Longitudinal dataset with median follow-up of 5 years
- Data from a diverse range of public and private payers
- Findings likely generalizable to the broader population of individuals with DM1 in the U.S.

## Limitations

- Open claims in which patients' follow-up was based on data activity rather than insurance eligibility
- Cost to payers were not directly observable and were estimated using averaged cost-to-charge ratio for individuals with DM1
- As in all observational studies using EHR or claims data, there is a potential for bias due to unobserved confounding, missing data, and inaccurate data coding

DM1, myotonic dystrophy type 1; EHR, electronic health record

# Conclusion

- First study to describe the characteristics  and resource utilization of patients with DM1 who present in routine clinical practice in real-world settings in the US
- Individuals with DM1 experienced a cumulative increase in incident rates of multiple organ system involvement over a 7-year period following the diagnosis of DM1, resulting in substantial resource utilization and costs across settings, type of care, and specialties
- Findings underscore a significant clinical and economic burden emphasizing the need for safe and effective treatments
- Early detection and treatment of DM1 is likely to lead to significant clinical and economic benefits

DM1, myotonic dystrophy type 1