



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

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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 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 (Dx) of DM1.

METHODS

Figure 1. Study Design

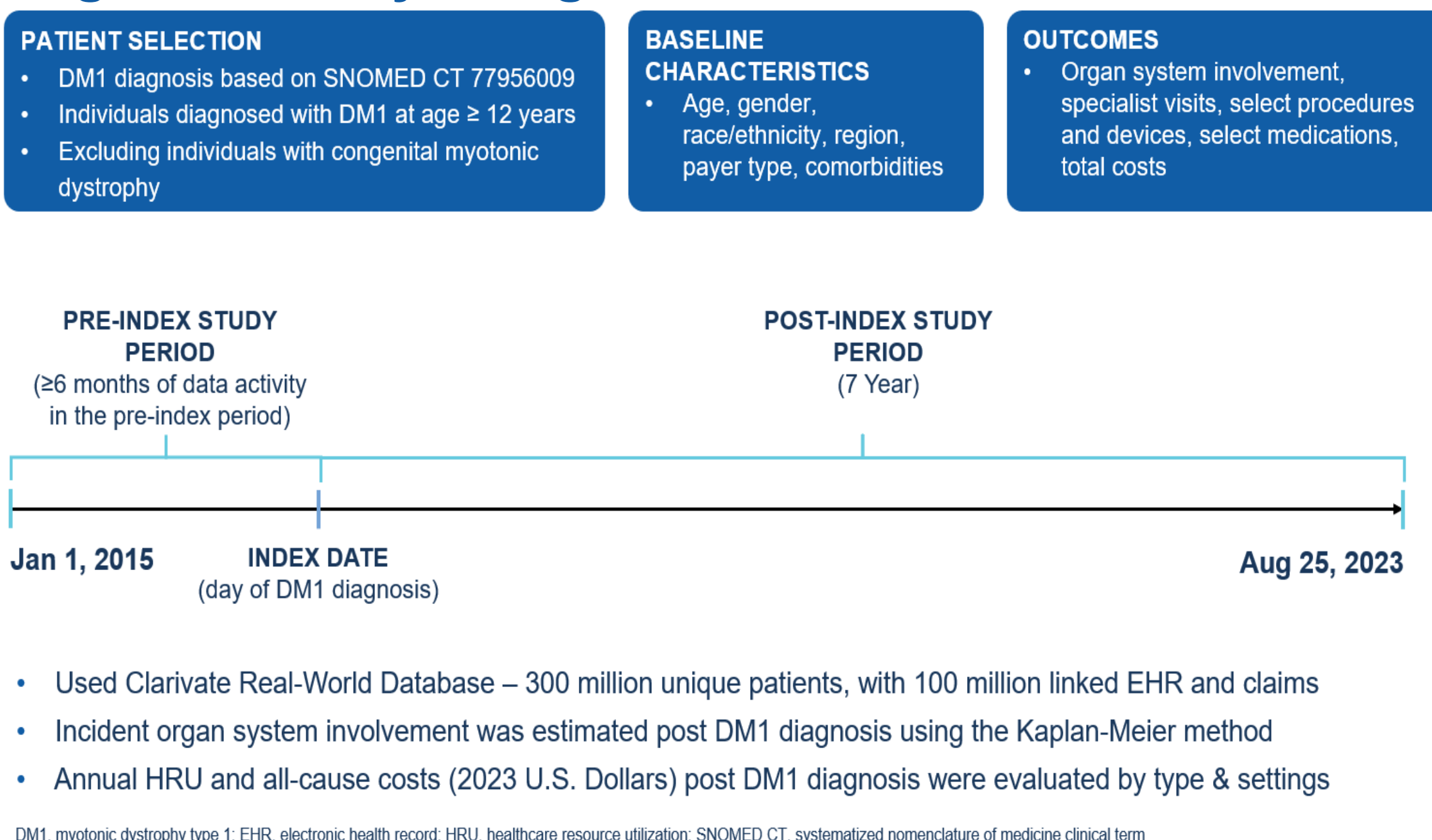
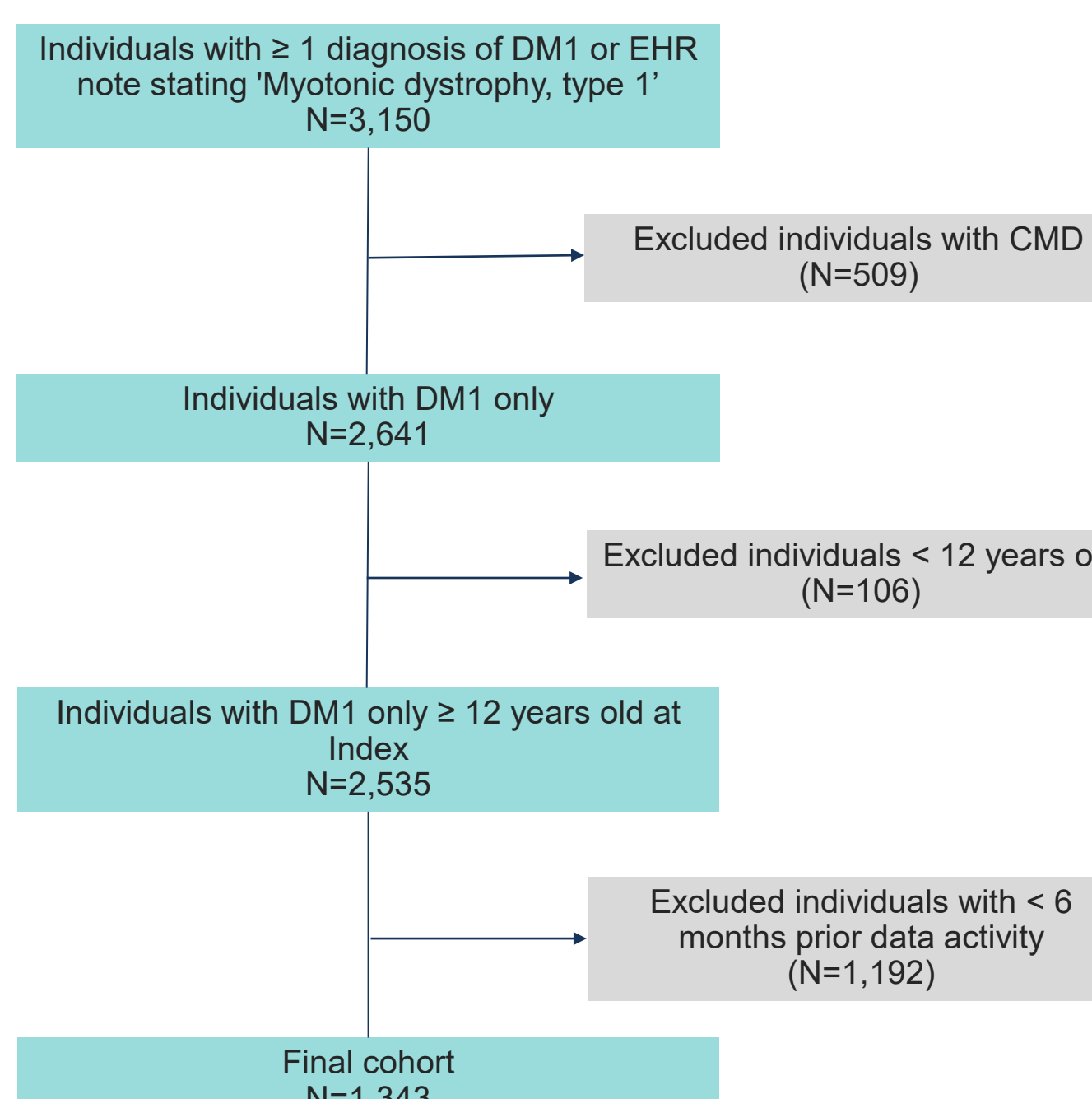


Figure 2. Patient Flow



CONCLUSIONS

- 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.

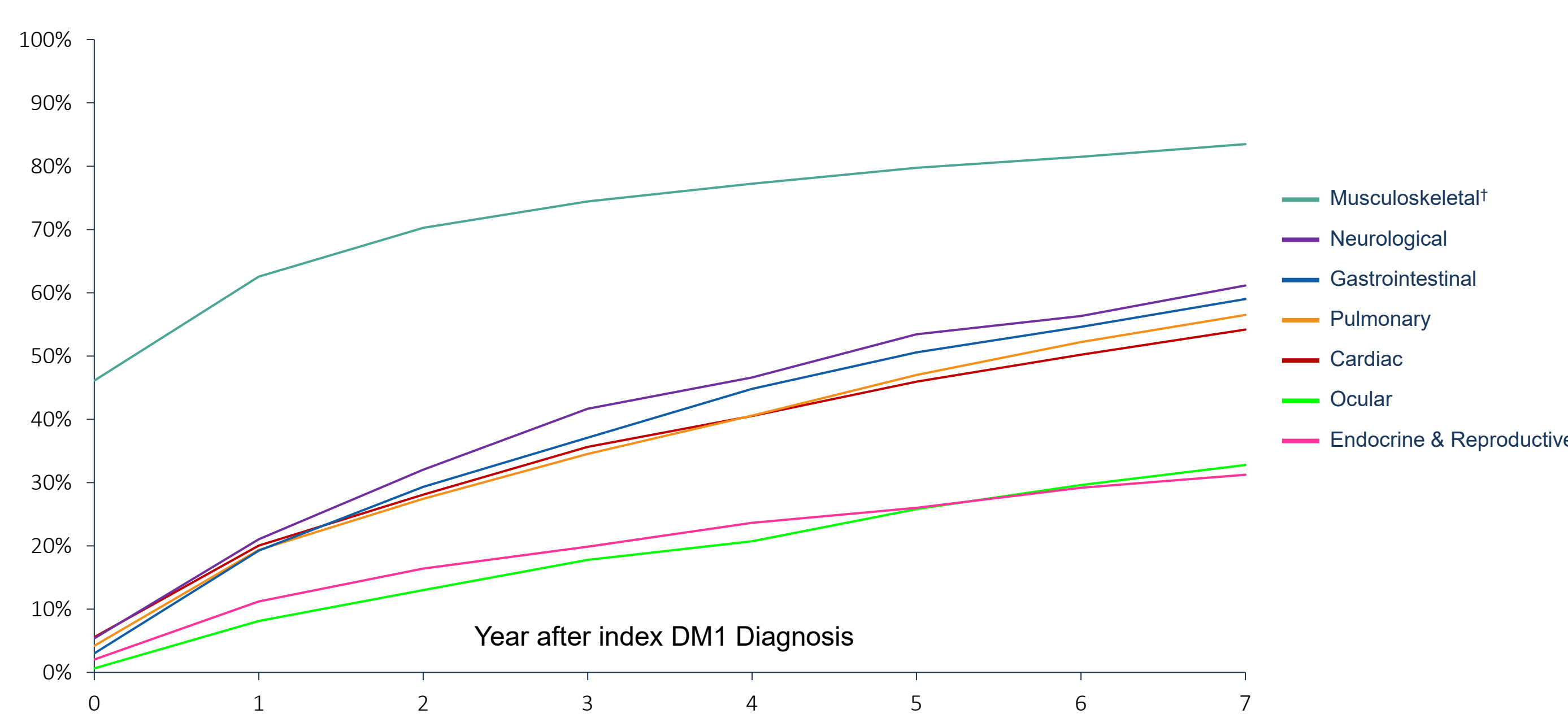
RESULTS

Table 1. Demographics & Clinical Characteristics

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, 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)

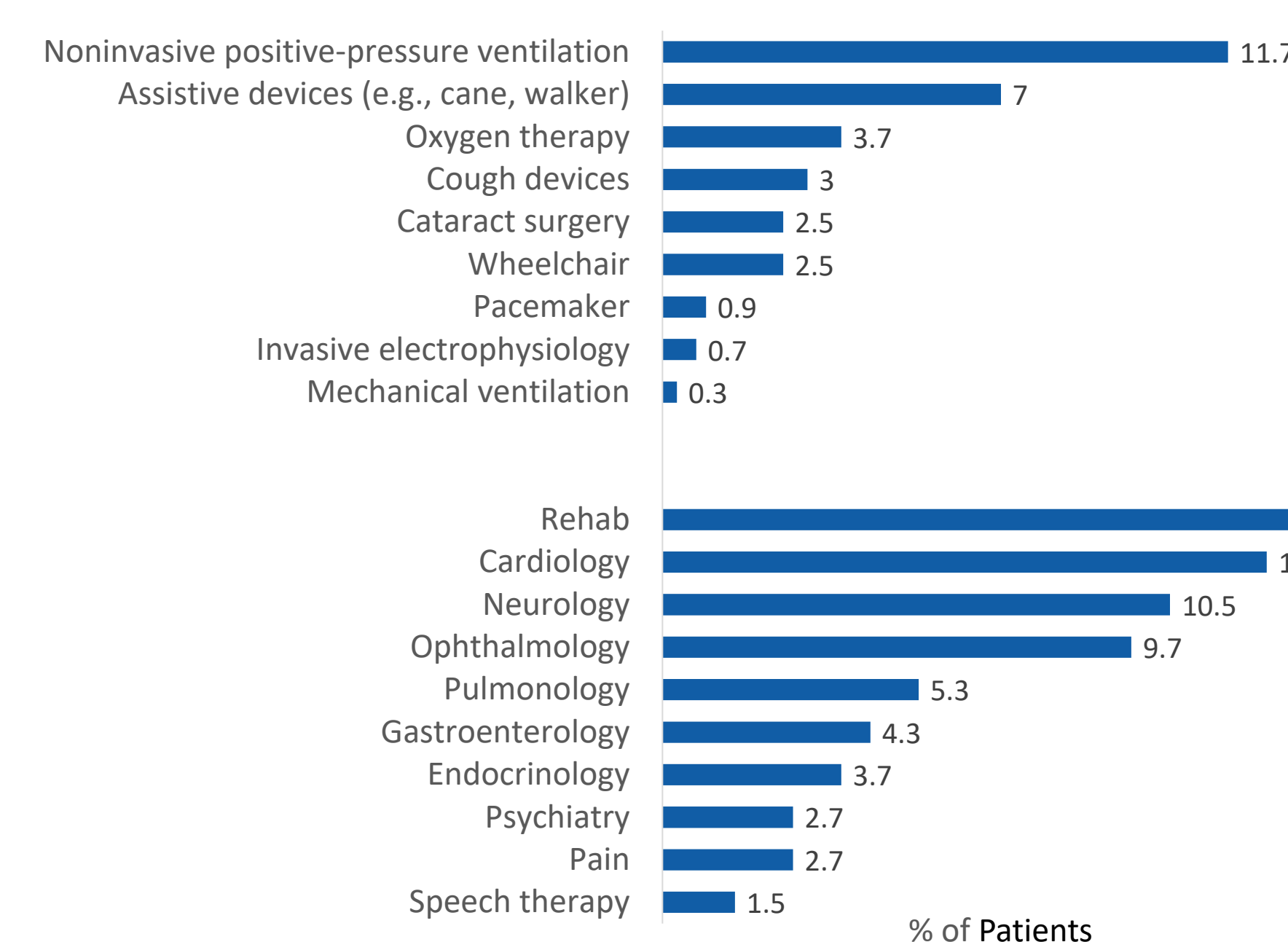
Figure 3. Cumulative Incidence of Organ System Involvement After DM1 Dx



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

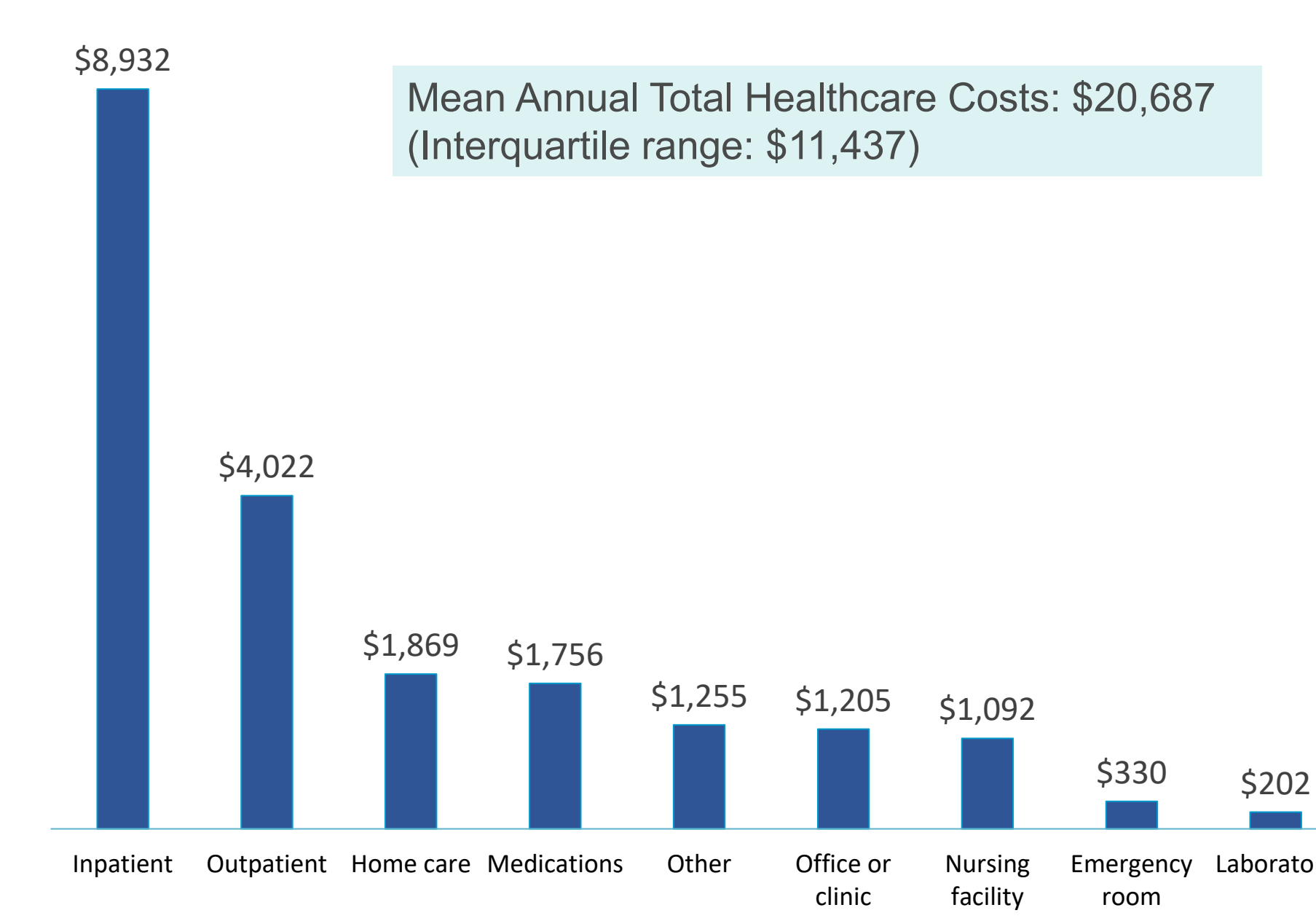
❖ Most individuals with DM1 have organ system involvement that increases over time, pointing to significant clinical burden

Figure 4. Annual HRU After DM1 Dx



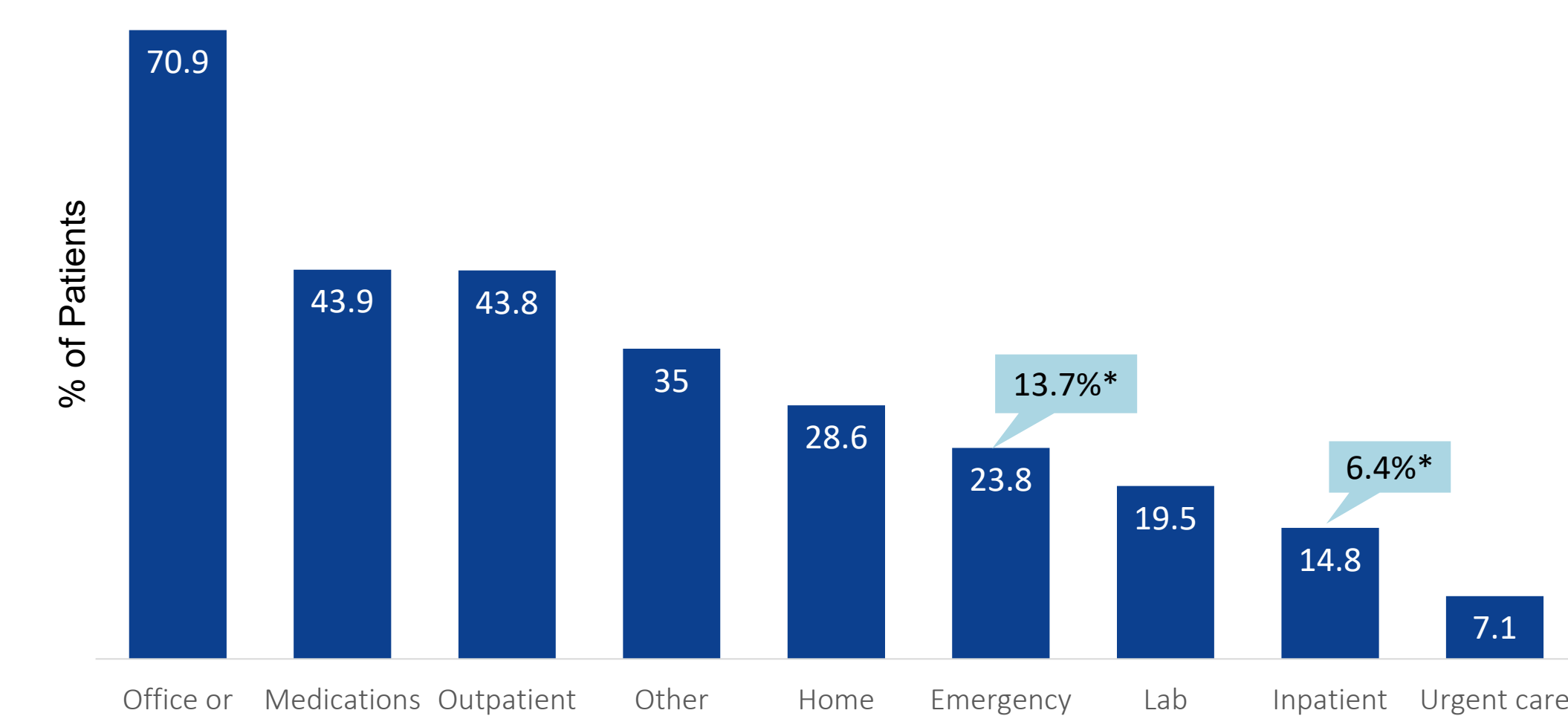
❖ DM1 is linked to high utilization across various types of specialty visits, devices, and procedures, highlighting the disease's medical complexity and heterogeneity

Figure 5. Annual Costs (2023 USD) After DM1 Dx



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

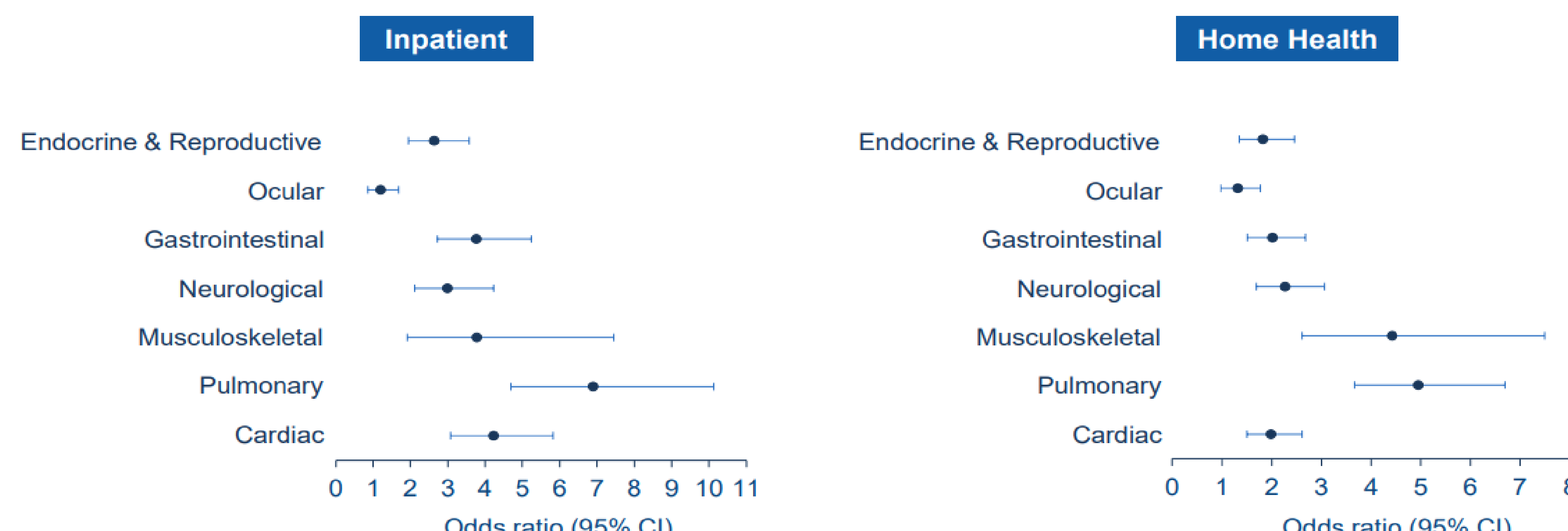
Figure 6. Annual HRU by Setting After DM1 Dx



*Annual rates observed in the general US population based on 2017 data from the Medical Expenditure Panel Survey⁵

❖ DM1 is associated with high rates of HRU across settings, exceeding the ones in the general US population

Figure 7. Regression-adjusted Utilization Associated with Organ System Involvement After DM1 Dx



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

❖ Organ system involvement in DM1 is associated with substantially elevated resource utilization across settings, most notably with inpatient and home health, which are the top cost drivers in this population

STUDY 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.

REFERENCES

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DISCLOSURE INFORMATION

A. Novack, E. Delage, and A. Dugar are employees of Dyne Therapeutics Inc. and may hold Dyne Therapeutics stock and/or stock options. M. Samnaliev and D. Ito are employees of Stratevi, a research consulting firm that received research funding from Dyne to conduct this analysis. J. Hamel provided consulting services to Vertex Therapeutics and PepGen.

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