



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

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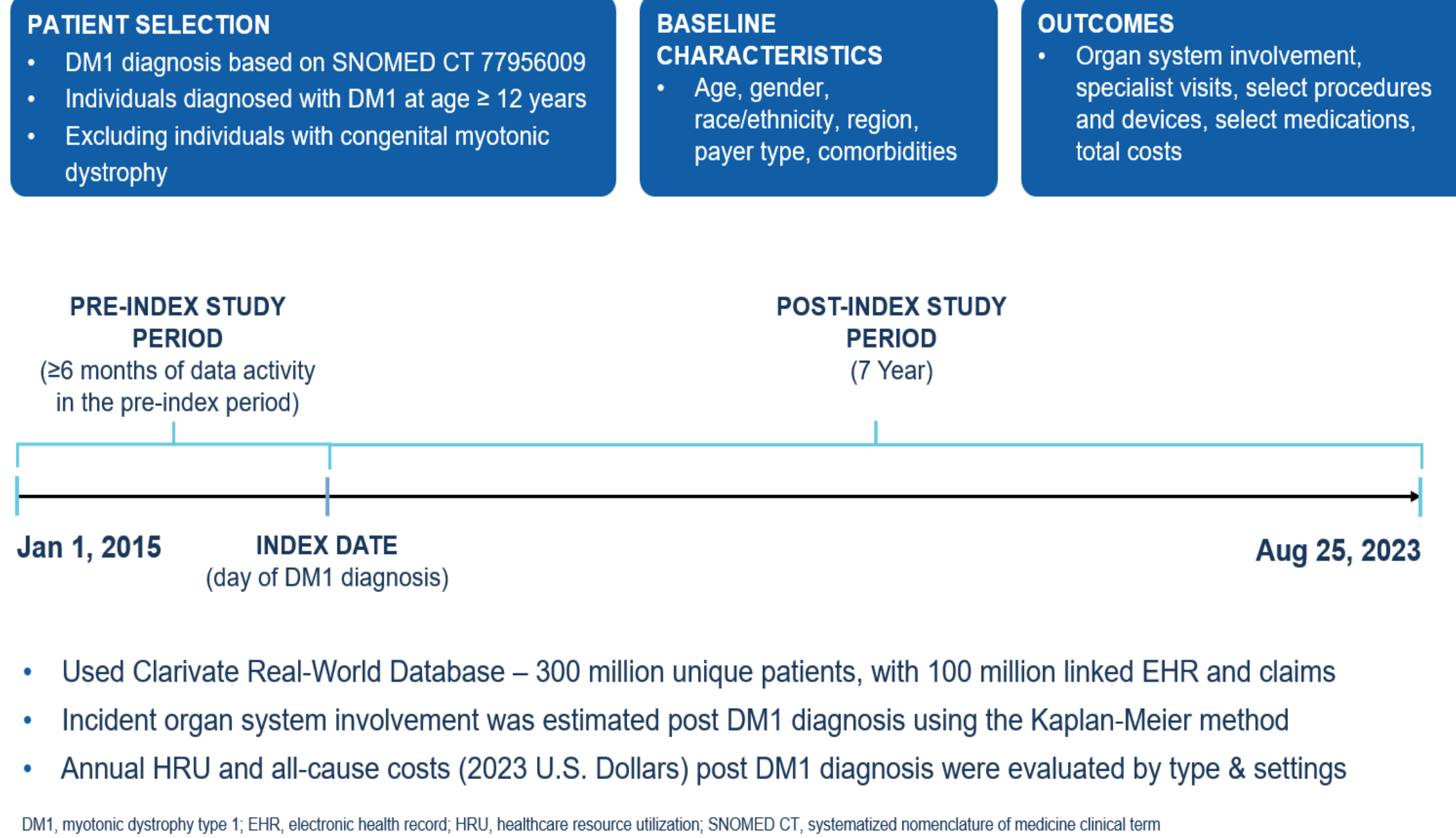
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## BACKGROUND

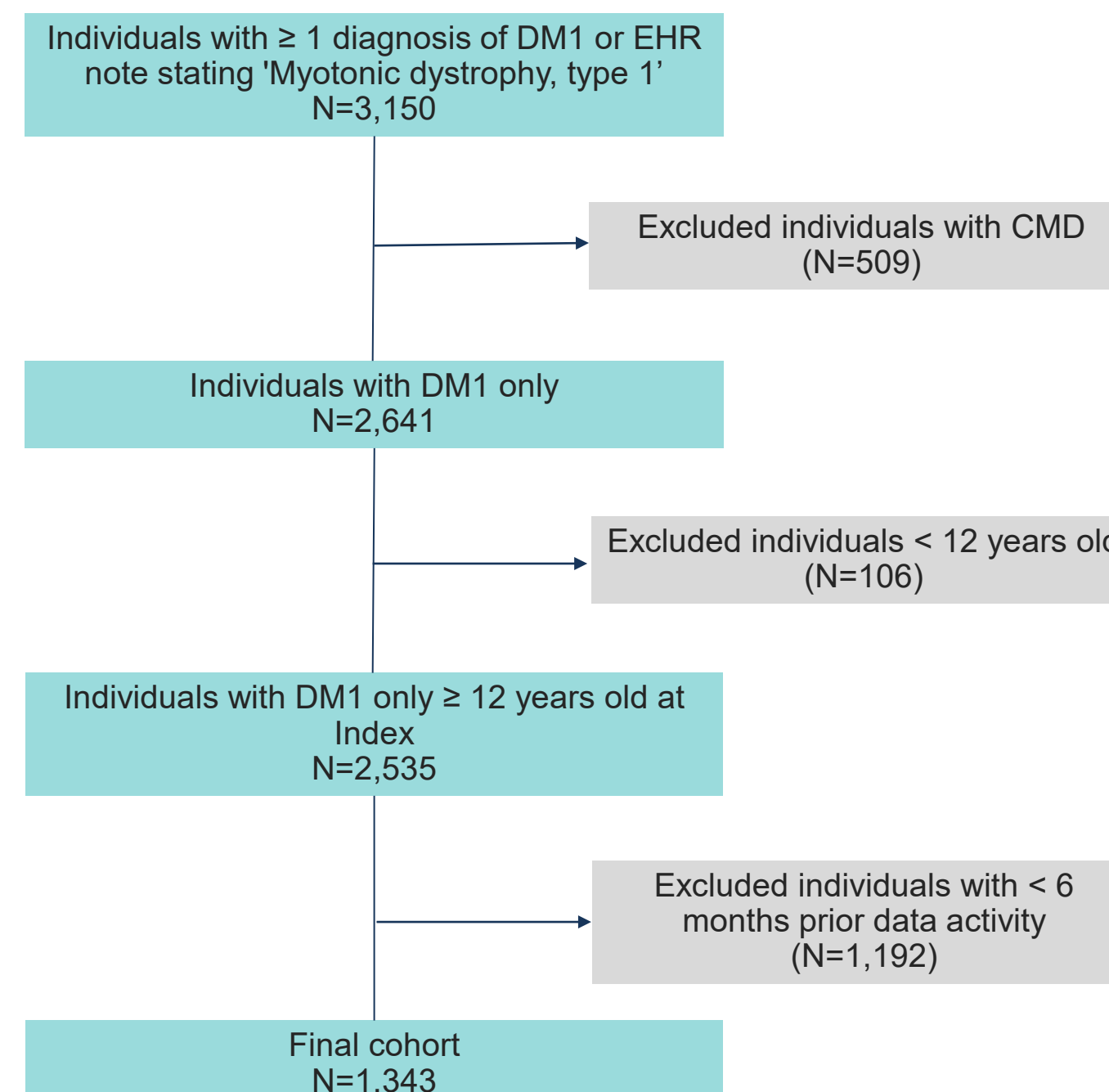
- Myotonic dystrophy type 1 (DM1) is a multi-systemic disease affecting multiple tissue types including skeletal and smooth muscle, eye, brain, and heart.<sup>1,2</sup>
- No disease-modifying therapies are currently available, highlighting an unmet care gap.
- Previous studies assessing the economic burden in myotonic dystrophy have not differentiated between DM type 1 and 2.<sup>3,4</sup>
- 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

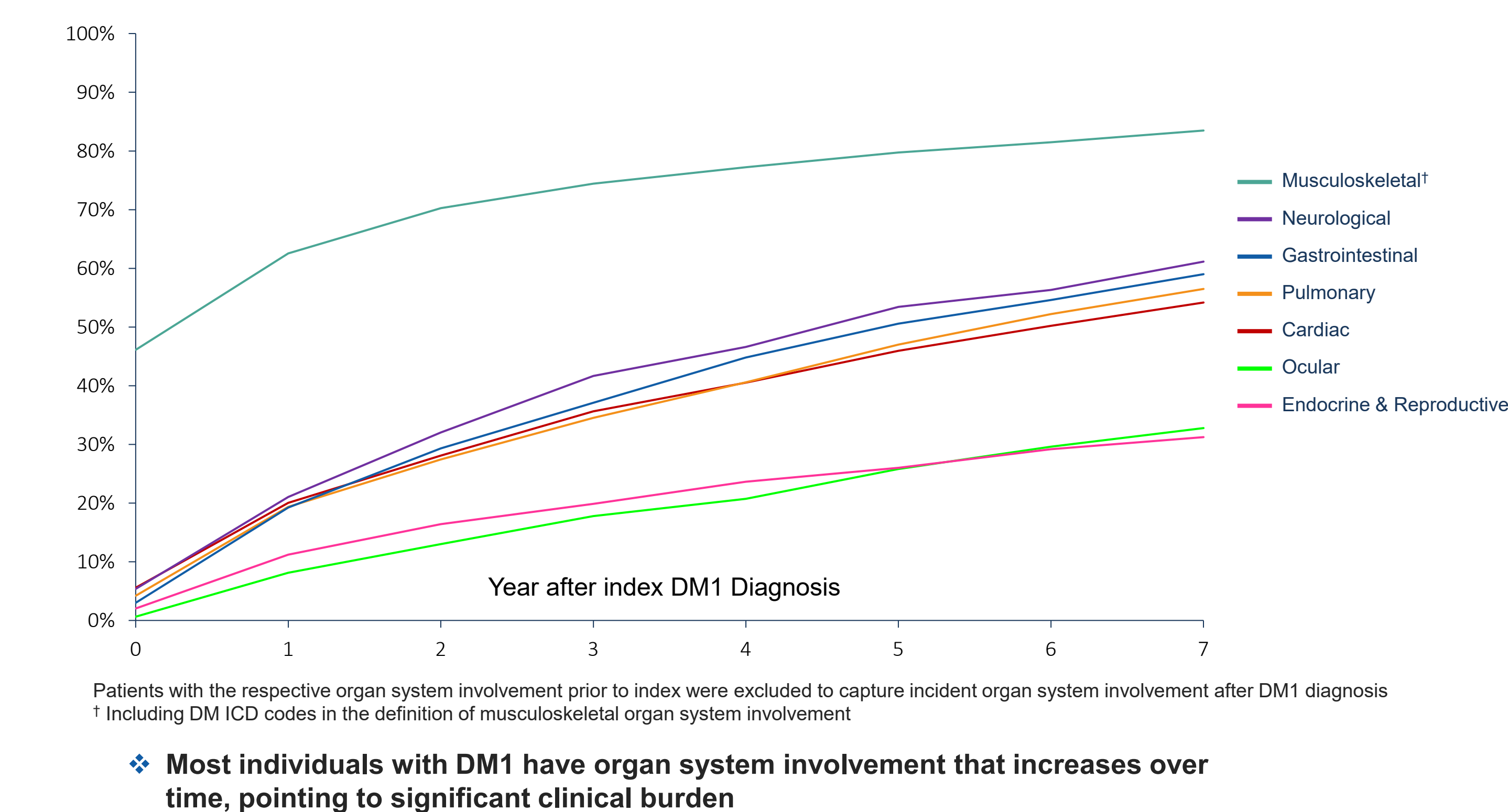


Figure 4. Annual HRU After DM1 Dx

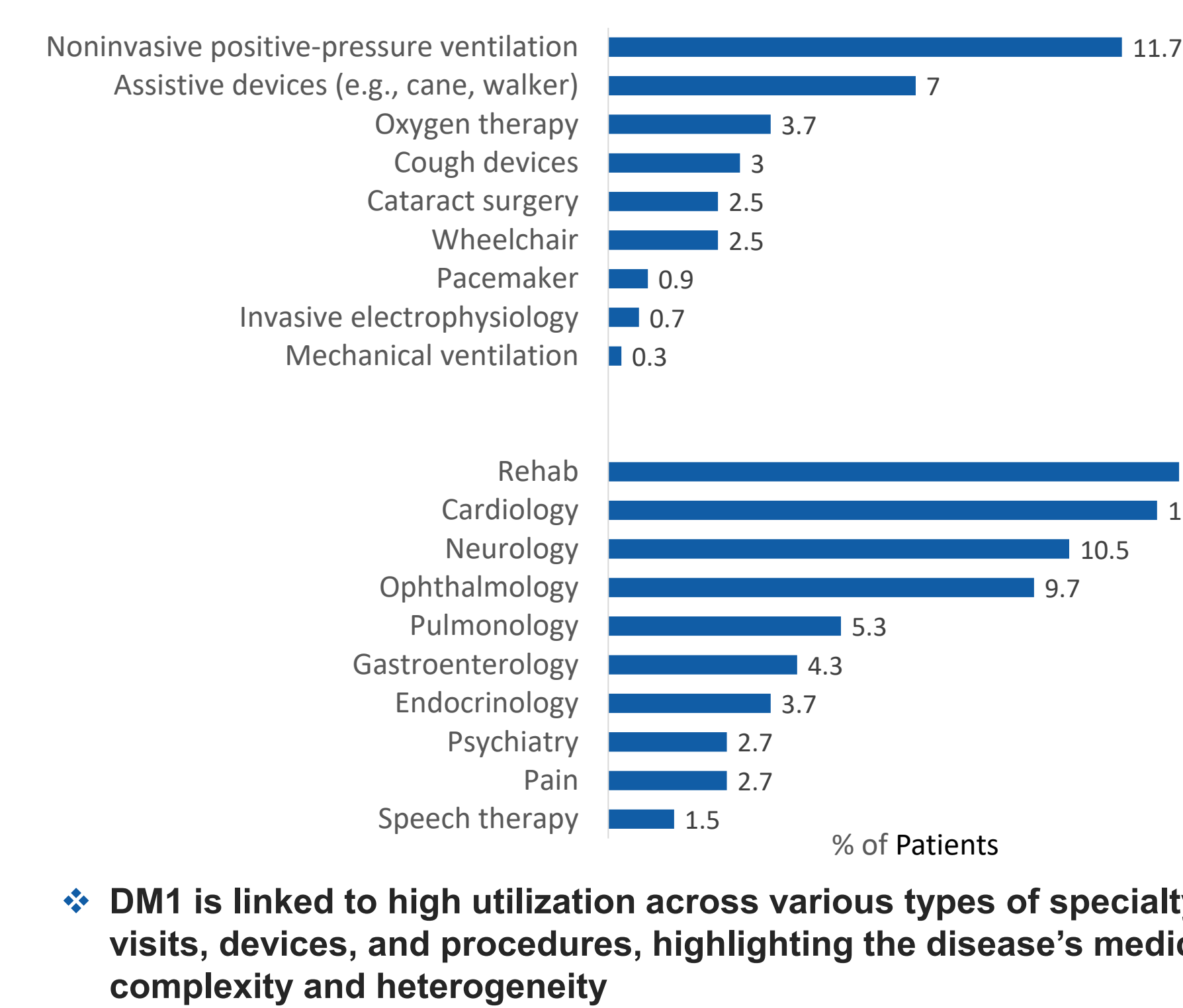


Figure 5. Annual Costs (2023 USD) After DM1 Dx

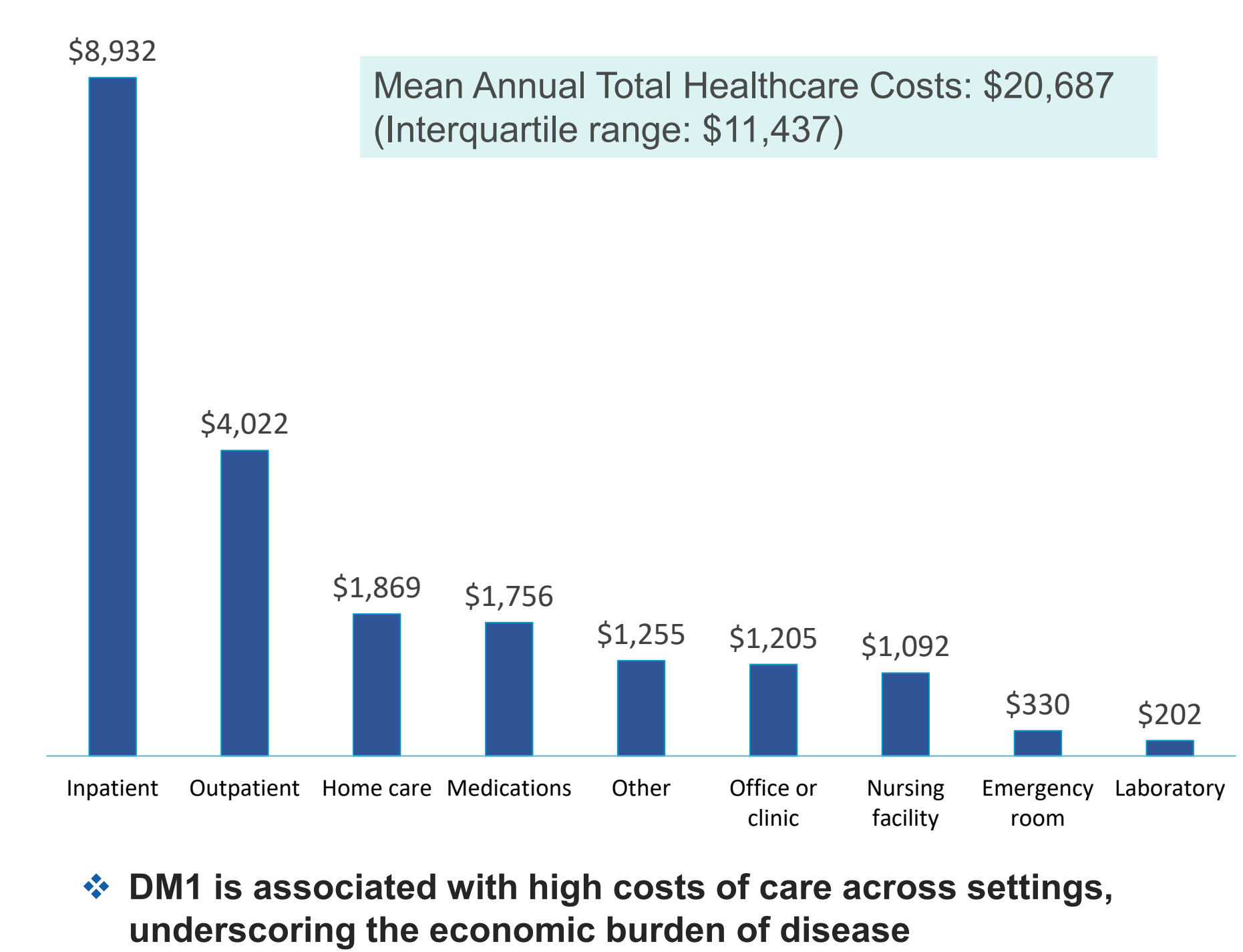


Figure 6. Annual HRU by Setting After DM1 Dx

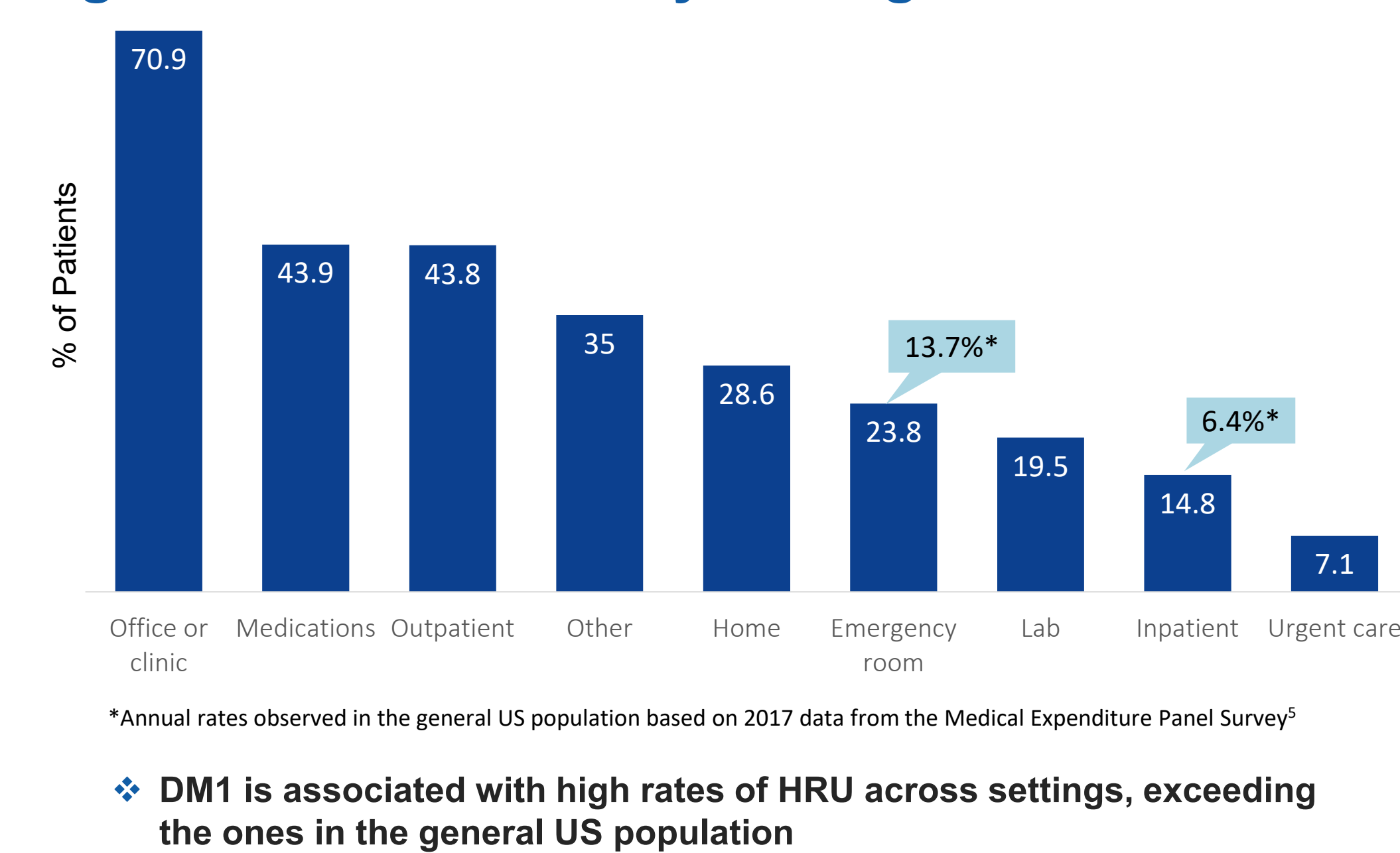
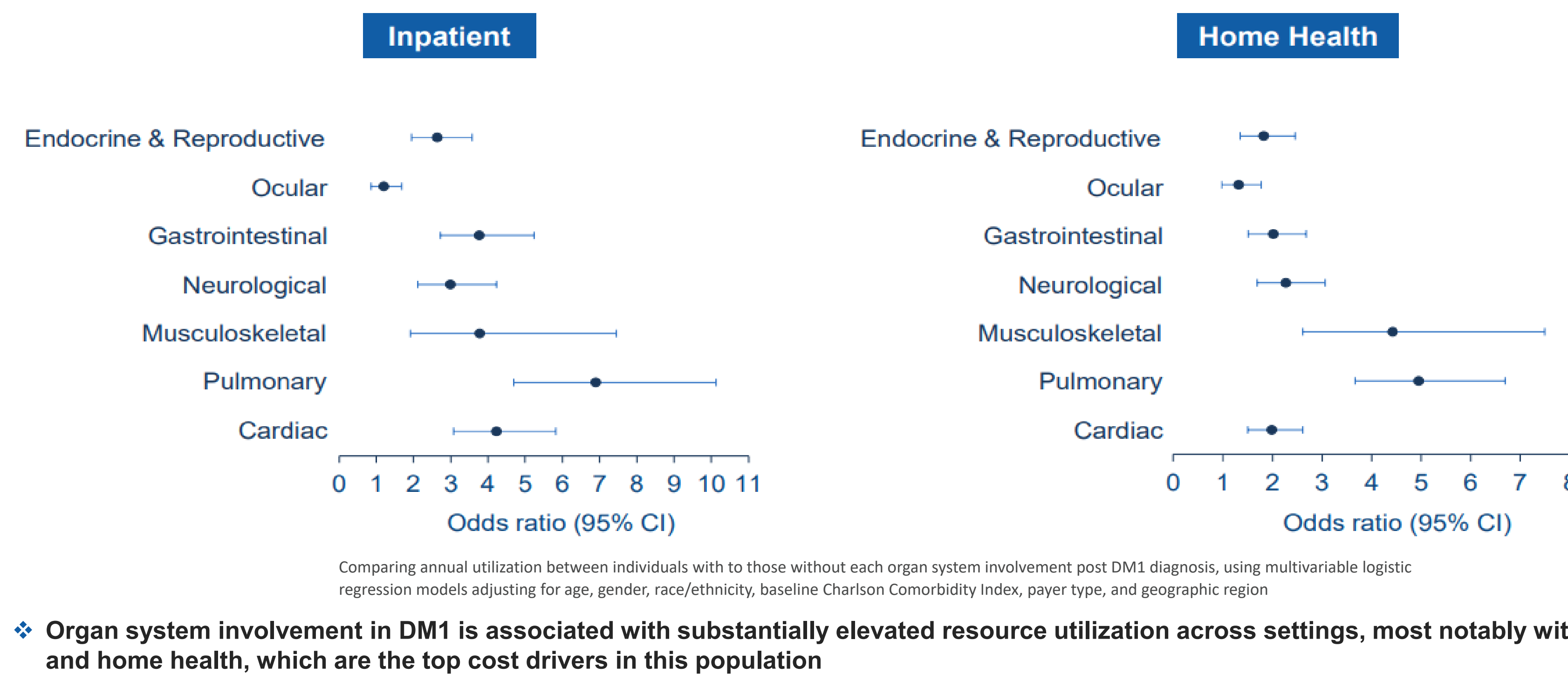


Figure 7. Regression-adjusted Utilization Associated with Organ System Involvement After DM1 Dx



## 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

1. Ashizawa, T., et al. Neurology: Clinical Practice 2018;8:507-520. 2. Orphanet. Steinert myotonic dystrophy: <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;17:79. 4. Larkindale J, et al. Muscle Nerve. 2014;49:431-8. 5. Mortensen K., et al. Med Care. 2021;59:704-710.

## 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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