Characteristics of Patients with Myotonic Dystrophy Type 1 with Complex Care Needs: Results from the Real-World IMPaCT Study

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BACKGROUND

- Myotonic Dystrophy Type 1 (DM1) is a rare, progressive, neuromuscular disorder associated with high morbidity and early mortality
- DM1 is characterized by marked disease variability and multisystemic manifestations[1,2]
- Critical evidence gaps remain in understanding the healthcare needs of the most medically complex patients with DM1, specifically those with cardiac and respiratory complications, which drive mortality
- The objectives of this study were to describe the characteristics of patients with DM1 who incur the highest total healthcare costs and identify the primary predictors of being a high-cost patient

METHODS

A retrospective, observational cohort study was conducted utilizing the Clarivate Real-world Data Repository of linked electronic health records and administrative healthcare claims from 01/01/2015 to 08/25/2023

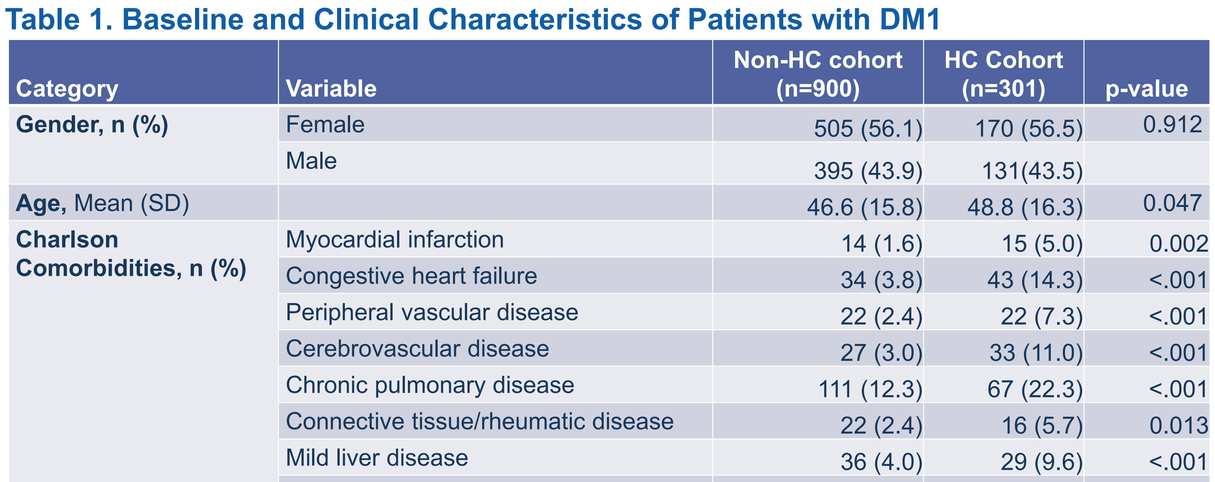
- Patients were selected based on the following eligibility criteria:
  - DM1 diagnosis (SNOMED code: 77956009) (index date)
  - Evidence of data activity during the ≥6 months pre- and ≥12 months post-index period
  - Age ≥12 years at index date
  - Patients with congenital myotonic dystrophy were excluded (SNOMED code: 240104008)
- Patient characteristics, presence of cardiac and respiratory complications, and the Charlson Comorbidity Index (CCI) were calculated utilizing data from the pre-index period. Cardiac and respiratory complications were classified as mild, moderate, or severe based on a combination of diagnostic and procedure codes, informed by clinician input. All outcome variables including health resource utilization (HRU) and costs of care utilized data from the post-index period.
- Costs were calculated using annualized all-cause total costs and adjusted to 2023 US dollars using the medical consumer price index. A cost-to-charge ratio was applied to estimate costs based on the charge data included in the dataset
- Patients with DM1 who incurred high costs of care (HC) were defined as those whose total annual costs fell within the top 25th percentile. These patients were compared to those in the non-high cost (non-HC) group
- Logistic regression models were utilized to predict being an individual with HC based on baseline clinical and demographic characteristics

RESULTS

Table 1. Baseline and Clinical Characteristics of Patients with DM1

Category	Variable	Non-HC cohort (n=900)	HC Cohort (n=301)	p-value
Gender, n (%)	Female	505 (56.1)	170 (56.5)	0.912
	Male	395 (43.9)	131(43.5)	
Age, Mean (SD)		46.6 (15.8)	48.8 (16.3)	0.047
Charlson Comorbidities, n (%)	Myocardial infarction	14 (1.6)	15 (5.0)	0.002
	Congestive heart failure	34 (3.8)	43 (14.3)	<.001
	Peripheral vascular disease	22 (2.4)	22 (7.3)	<.001
	Cerebrovascular disease	27 (3.0)	33 (11.0)	<.001
	Chronic pulmonary disease	111 (12.3)	67 (22.3)	<.001
	Connective tissue/rheumatic disease	22 (2.4)	16 (5.7)	0.013
	Mild liver disease	36 (4.0)	29 (9.6)	<.001
	Moderate/severe liver disease	2 (0.2)	4 (1.3)	0.037
	Diabetes without complications	79 (8.8)	72 (23.9)	<.001
	Diabetes with complications	24 (2.7)	38 (12.6)	<.001
	Paraplegia & hemiplegia	11 (1.2)	11 (3.6)	0.011
	Renal disease	18 (2.0)	18 (6.0)	0.001
	Cancer	42 (4.7)	24 (8.0)	0.012
	Peptic ulcer disease	4 (0.4)	5 (1.7)	0.049
	Dementia	6 (.7)	4 (1.3)	0.28
	Metastatic carcinoma	7 (0.8)	2 (0.7)	0.84
	HIV/AIDS	0 (0.0)	1 (0.3)	0.25
CCI Score, Mean (SD)		0.7 (1.4)	1.7 (2.3)	<.001

Figure 1. Baseline Cardiac & Respiratory Complications by Severity & Cohort



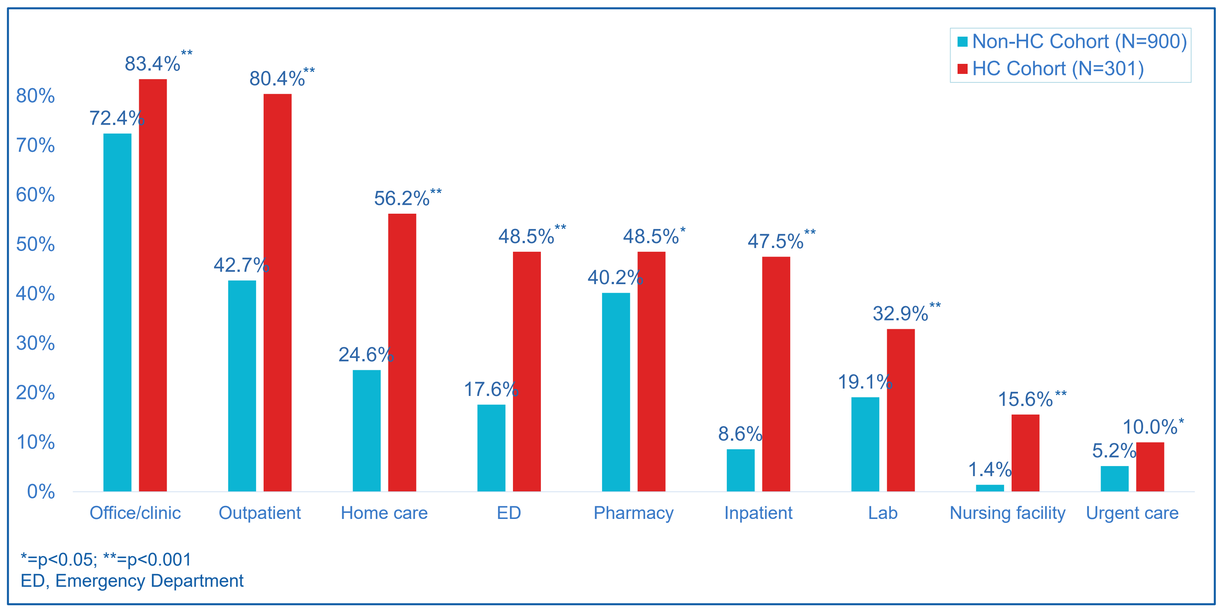
➢ The HC cohort experienced significantly higher rates of mild, moderate and severe cardiac and respiratory complications compared to the non-HC cohort

CONCLUSIONS

- High healthcare utilization in inpatient, emergency and home care settings, along with use of assistive devices/procedures significantly contributes to the high costs of care for individuals with DM1
- Severe cardiac and respiratory clinical complications are significant predictors of increased healthcare costs among patients with DM1
- These findings highlight the substantial unmet treatment needs in DM1 and emphasize the necessity for novel interventions that may address the multi-systemic nature of the disease and mitigate its economic burden

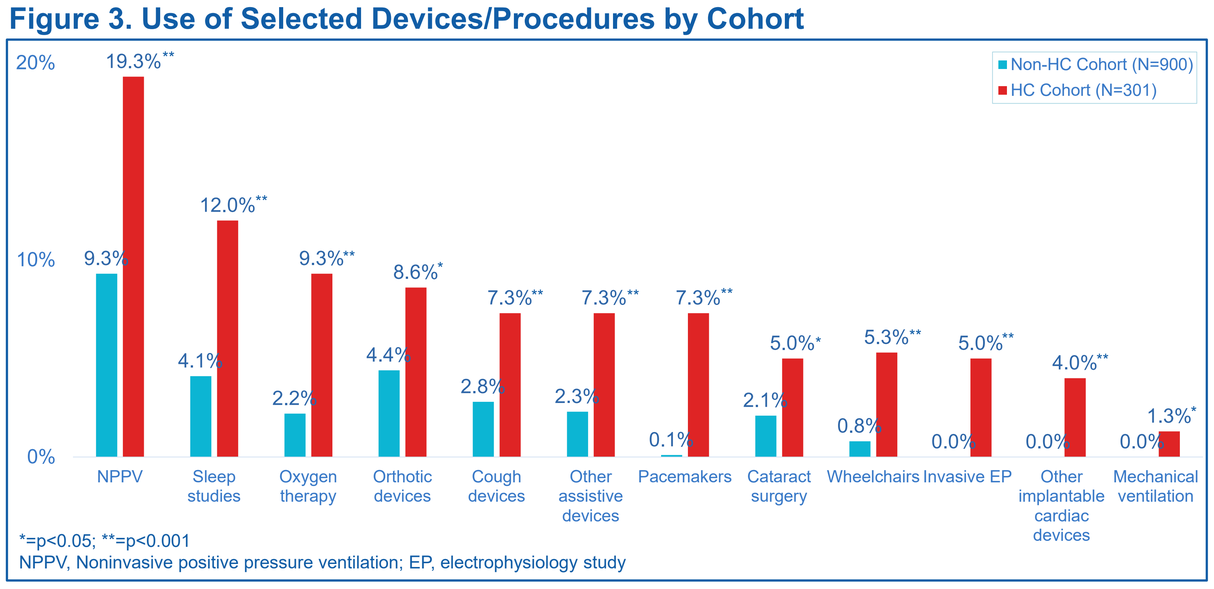
RESULTS

Figure 2. All-cause HRU by Setting & Cohort



➢ The HC cohort observed significantly higher rates of HRU compared to the non-HC cohort, notably, in the inpatient, outpatient, emergency department, and home care settings

Figure 3. Use of Selected Devices/Procedures by Cohort



➢ The HC cohort had significantly higher utilization rates for various medical devices and procedures compared to the non-HC cohort, with notable differences observed in the use of NPPV, pacemakers and oxygen therapy

Table 2. Predictors of Being a Patient with High Costs in 12-Month Follow-up Period

Variable	Estimate**	95% CI		p-value
Cardiac Complications at Baseline*				
Mild	0.96	0.46	2.01	0.917
Moderate	1.71	0.64	4.52	0.283
Severe	**1.81**	**1.14**	**2.88**	**0.012**
Respiratory Complications at Baseline*				
Mild	1.16	0.70	1.93	0.557
Moderate	^			
Severe	**3.77**	**1.93**	**7.36**	**<.001**

*Used hierarchical ordering of cardiac and respiratory severity, where severe supersedes moderate and moderate supersedes mild
**Models adjusted for age, gender, age at index, race/ethnicity, and baseline Charlson comorbidity score and comorbidities
^Unable to estimate due to small sample size

➢ Severe cardiac and respiratory complications at baseline were significant predictors of being a patient with high costs in the 12-month post index period

REFERENCES

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2. Hamel JI, McDermott MP, Hilbert JE, et al. Milestones of progression in myotonic dystrophy type 1 and type 2. Muscle Nerve 2022;66:508-512.

DISCLOSURE INFORMATION

A. Novack & A. Dugar are employees of Dyne Therapeutics Inc. and may hold Dyne stock and/or stock options. M. Samnaliev & D. Ito are employees of Stratevi, a consulting company that received funding from Dyne to conduct this study. J. Hamel served as an advisor to Dyne and Vertex Therapeutics. J Hamel serves as the study principal investigator for Freedom DM1.

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