Myotonic Dystrophy Burden of Disease Study Initial Findings

Myotonic Dystrophy Foundation

Drug Development Roundtable, Arlington, Virginia

September 15, 2016

David Herbert, MBA, Mayo Clinic Emeritus Staff

Study Goal and Methods

Goal:
- Study the costs & utilization associated with myotonic dystrophy (DM) in a large, commercially insured population.

Design:
- Retrospective, cohort study

Data Source:
- >100 million, geographically diverse, non-elderly and elderly individuals, privately insured or enrolled in Medicare Advantage (OptumLabs).

Eligible:
- At least 1 diagnosis of Myotonic Dystrophy (ICD-9-CM code 359.21 "myotonic muscular dystrophy") between 2008 and 2014
- ≥12 Months of continuous medical coverage before and after 1st diagnosis

Analytic Methods:
- Descriptive statistics patient demographics and clinical characteristics
- Matched cohort (matched on age, gender, race, census region, enrollment, length of follow-up)
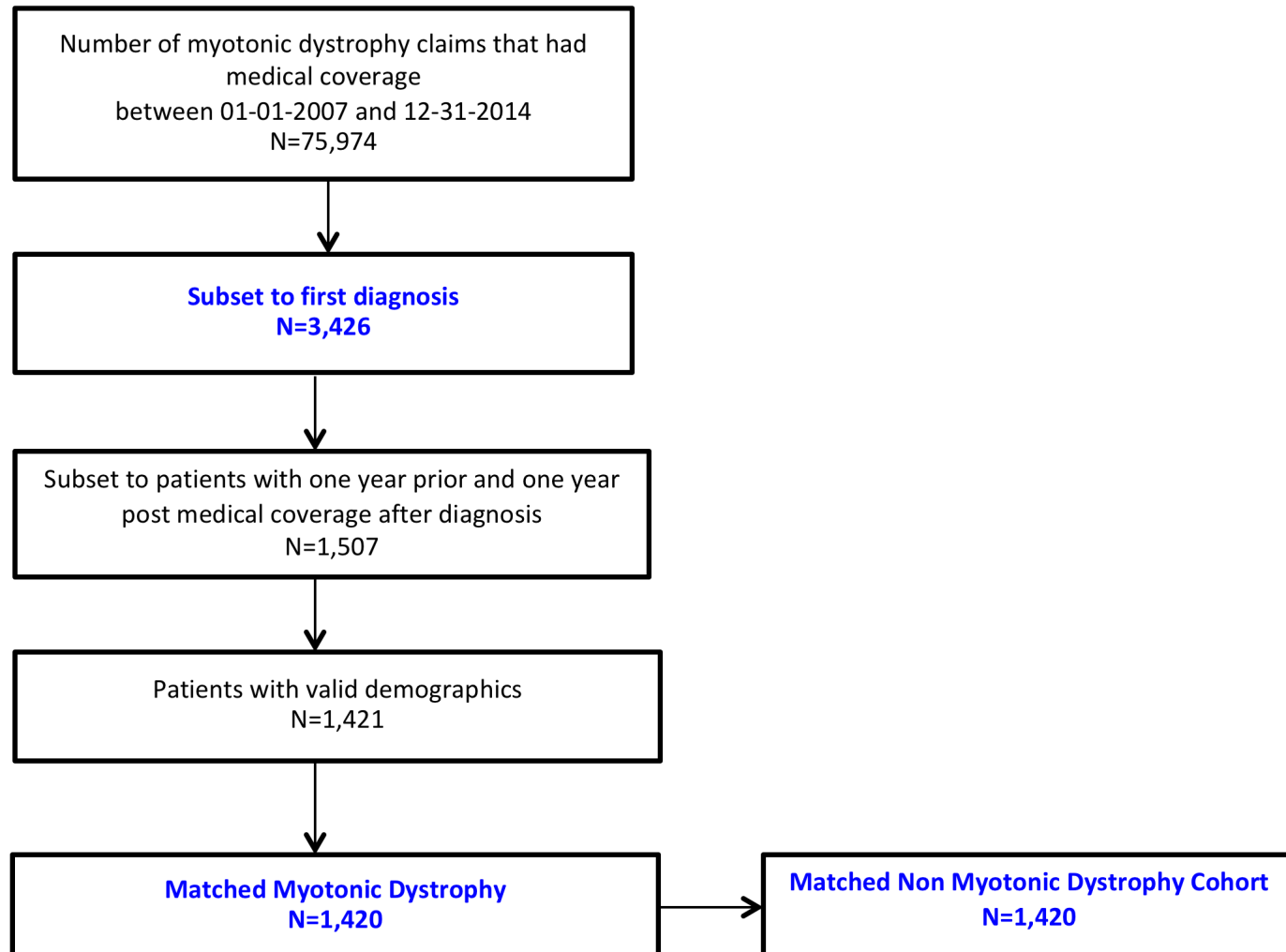
Outcomes of Interest:
- Rates of utilization (hospitalizations, ER visits, office visits)
- Sum of total cost (total paid amounts by patient and health plan) by various lengths of follow up (i.e. 1 year, 2 year, 3 year)

Additional Areas of Interest:
- Reasons for utilization (hospitalizations, ER visits, office visits)
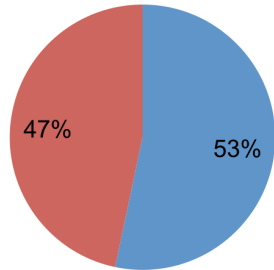- Chronic conditions

Selection of 1,420 DM Subjects

Number of myotonic dystrophy claims that had
medical coverage
between 01-01-2007 and 12-31-2014
N=75,974

Subset to first diagnosis
N=3,426

Subset to patients with one year prior and one year
post medical coverage after diagnosis
N=1,507

Patients with valid demographics
N=1,421

Matched Myotonic Dystrophy
N=1,420

Matched Non Myotonic Dystrophy Cohort
N=1,420

Demographics

% Participants by Gender*

- Female
- Male

53% Female
47% Male

% Participants by Age Group*

- <18
- 18-34
- 35-44
- 45-54
- 55-64
- 65+

12% <18
17% 18-34
17% 35-44
23% 45-54
20% 55-64
11% 65+
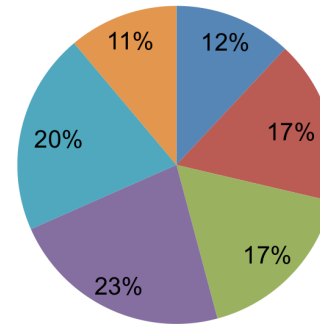
% Participants by Race*

- Asian
- Black
- Hispanic
- White
- Unknown

2% Asian
7% Black
5% Hispanic
58% White
29% Unknown
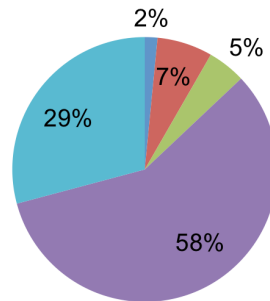
*Both Matched Cohort and DM data

Mean Total Paid Amount, $

	0-1 Years Post Dx	1-2 Years Post Dx	2-3 Years Post Dx
■ DM	$20,233.20	$15,596.80	$13,737.80
■ Matched	$5,846.20	$5,081.90	$5,563.40

Median Total Paid Amount, $

	0-1 Years Post Dx	1-2 Years Post Dx	2-3 Years Post Dx
■ DM	$6,729.00	$4,342.10	$4,139.40
■ Matched	$1,170.40	$1,148.70	$1,288.50

Inpatient Hospitalizations

	0-1 Years Post Dx	1-2 Years Post Dx	2-3 Years Post Dx
■DM	487	227	142
■Matched	126	69	41
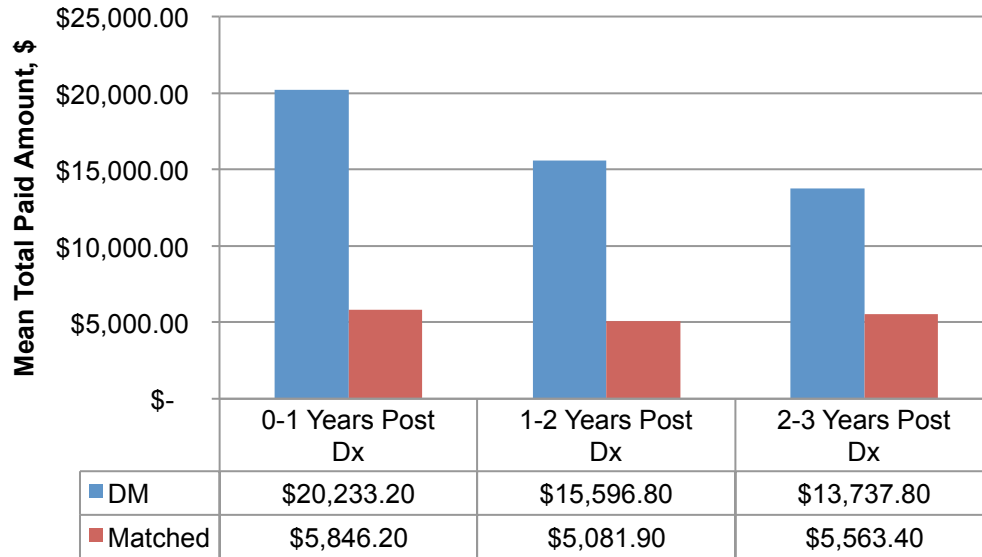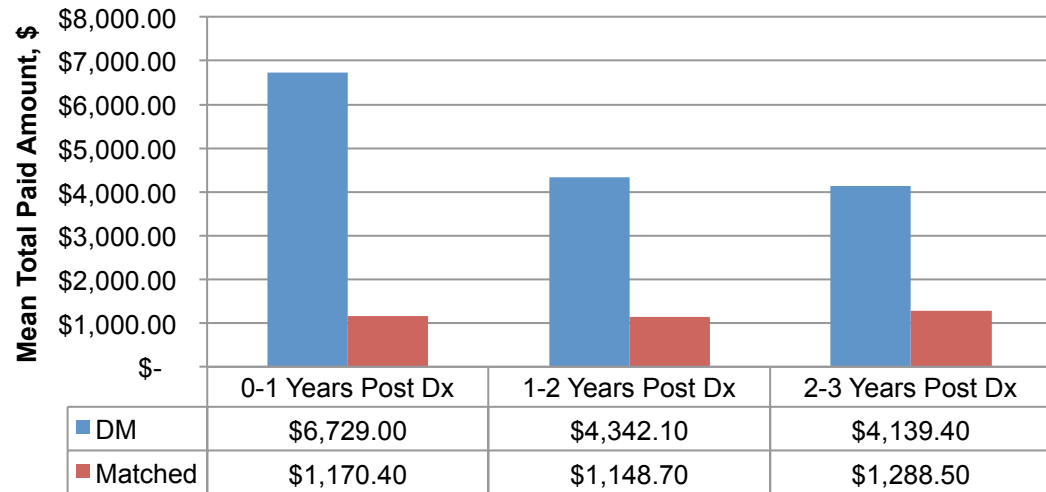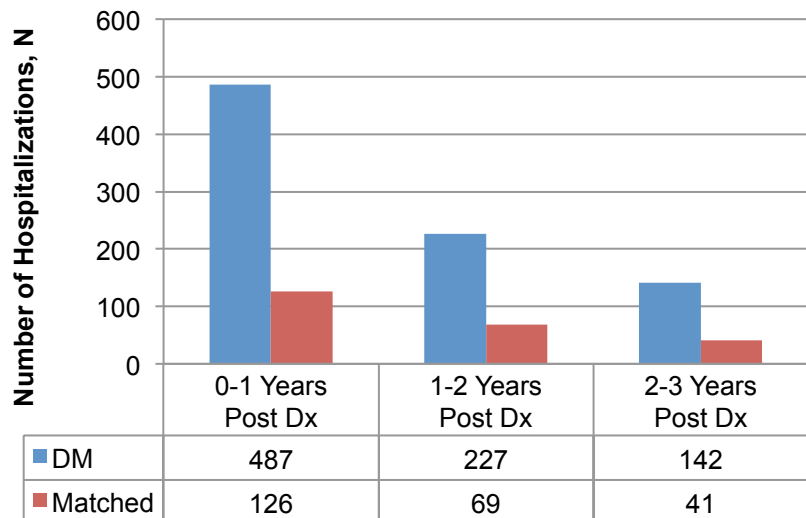
Top Reasons for Hospitalization:

1-year After Diagnosis:

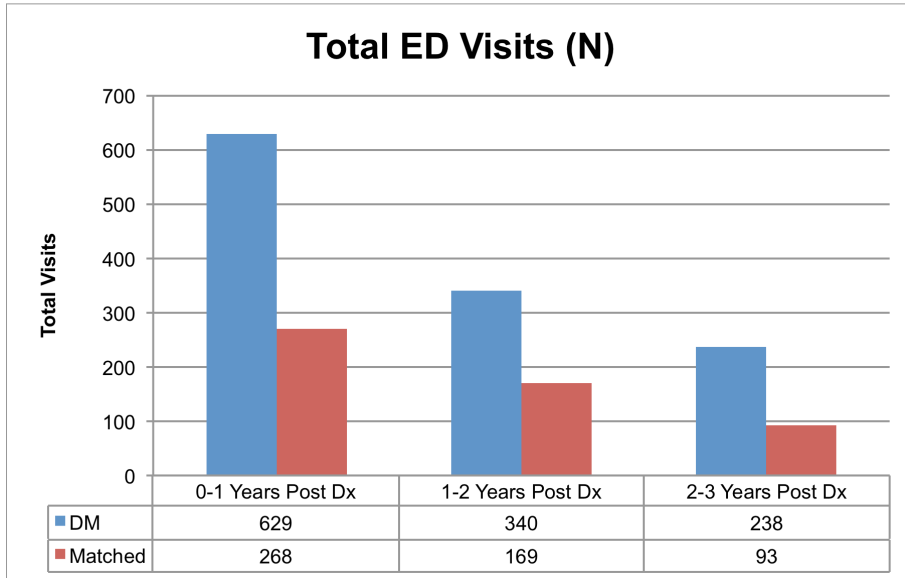| DM Population (N=1420)	Matched Cohort (N=1420)
Pneumonia- Organism Nos (30)	Acute Renal Failure Nos (5)
Myotonic Muscular Dystrophy (16)	Asthma W/ Acute Exacerbation (3)
Food/Vomit Pneumonitis- (13)	Asthma W/ Status Asthmaticus (3)
Acute And Chronic Respiratory Failure (11)	Chest Pain Nos (3)
Hereditary Progressive Muscular Dystrophy (9)	Septicemia (3)

Top Reasons for Hospitalization, 2-year After Diagnosis:

DM Population (N=920)	Matched Cohort (N=920)
Pneumonia- Organism Nos (32)	Localized Osteoarthritis Nos-L/Leg (5)
Food/Vomit Pneumonitis-18 (18)	Chest Pain Nos (4)
Septicemia Nos (12)	Acute Renal Failure Nos (3)
Dehydration (11)	Asthma W/Acute Exacerbation (3)
Atrial Fibrillation (10)	Asthma W/Status Asthmaticus (3)

Top Reasons for Hospitalization, 3-year After Diagnosis:

DM Population (N=628)	Matched Cohort (N=628)
Pneumonia- Organism Nos (29)	Localized Osteoarthritis Nos-L/Leg (6)
Food/Vomit Pneumonitis- (18)	Localized Osteoarthritis Nos-Pelvis (5)
Septicemia Nos (14)	Del W 2 Deg Laceration-Del (4)
Urinary Tract Infection (12)	Asthma W/ Acute Exacerbation (3)
Chest Pain (10)	Asthma W/ Status Asthmaticus (3)

Emergency Room Visits
(Did not result in hospitalization)

Total ED Visits (N)

	0-1 Years Post Dx	1-2 Years Post Dx	2-3 Years Post Dx
DM	629	340	238
Matched	268	169	93

Top Reasons for ED Visit:

1-year After Diagnosis:

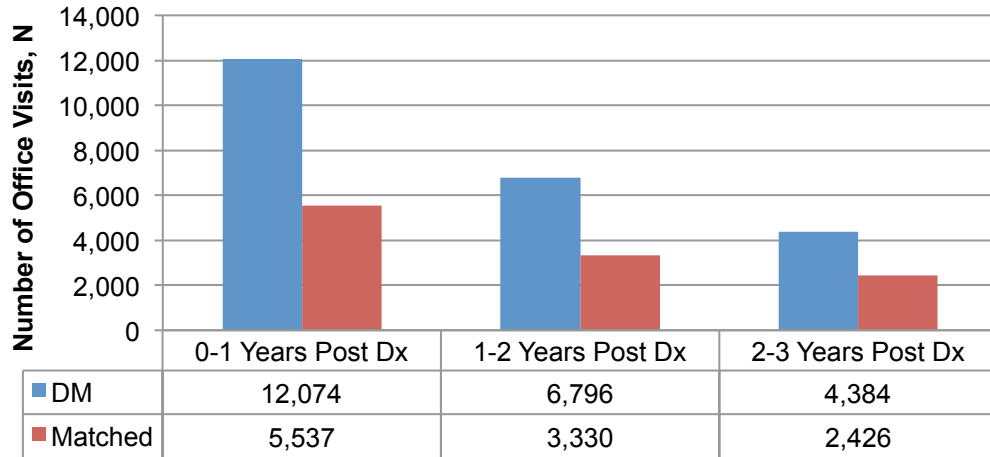| DM Population (N=1420)	Matched Cohort (N=1420)
Chest Pain (45)	Abdominal Pain (16)
Abdominal Pain (37)	Chest Pain (15)
Oth Malaise/Fatigue (13)	Acute Pharyngitis (6)
Lumbago (12)	Fever Nos (5)
Acute Bronchitis (10)	Acute Bronchitis (4)

2-year After Diagnosis:

DM Population (N=920)	Matched Cohort (N=920)
Chest Pain (71)	Chest Pain (19)
Abdominal Pain (39)	Abdominal Pain (17)
Headache (18)	Acute Pharyngitis (7)
Acute Bronchitis (15)	Dizziness And Giddiness (7)
Urinary Tract Infection Nos (12)	Headache (6)

3-year After Diagnosis:

DM Population (N=628)	Matched Cohort (N=628)
Chest Pain (58)	Migraine Nos-Not Intractable (9)
Abdominal Pain (34)	Abdominal Pain Nos (8)
Head Injury - Unspecified (14)	Acute Pharyngitis (7)
Urinary Tract Infection Nos (14)	Chest Pain Nos (7)
Acute Bronchitis (13)	Cellulitis Of Trunk (6)

Total Office Visits - DM and Matched Cohorts

Number of Office Visits, N

	0-1 Years Post Dx	1-2 Years Post Dx	2-3 Years Post Dx
DM	12,074	6,796	4,384
Matched	5,537	3,330	2,426

Top Specialists Seen:

1-year After Diagnosis:

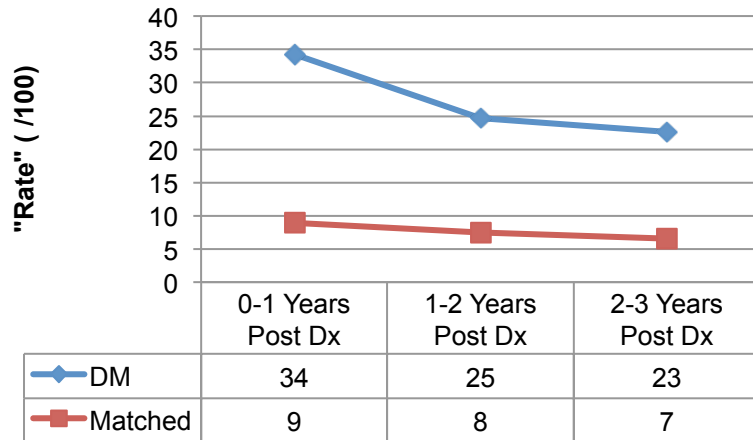| DM Population (N=1420)	Matched Cohort (N=1420)
Family Practice (1983)	Family Practice (1364)
Internist (1913)	Internist (901)
Neurologist (1634)	Dermatologist (337)
Cardiologist (889)	Orthopedist (298)
Pediatrician (531)	Pediatrician (275)

2-year After Diagnosis:

DM Population (N=920)	Matched Cohort (N=920)
Family Practice (2550)	Family Practice (1845)
Internist (2328)	Internist (1089)
Neurologist (1658)	Dermatologist (425)
Cardiologist (957)	Orthopedist (364)
Pediatrician (708)	Pediatrician (33)

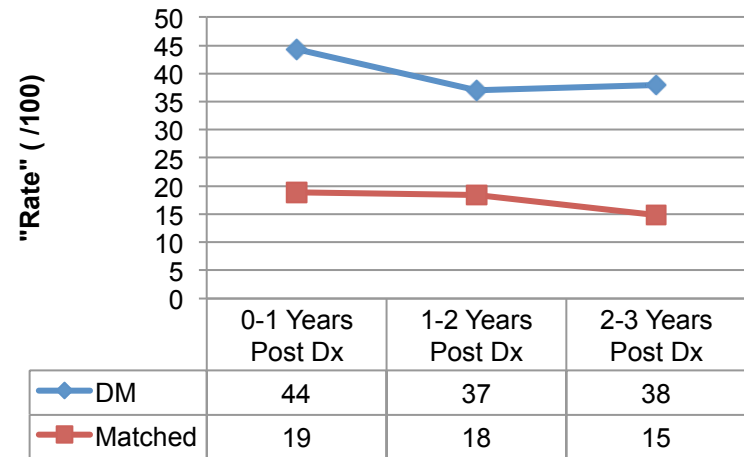Top Specialists Seen, 3-year After Diagnosis:

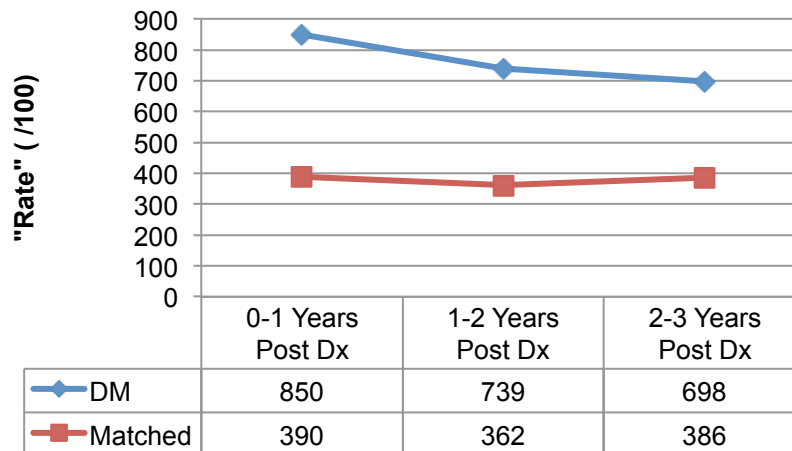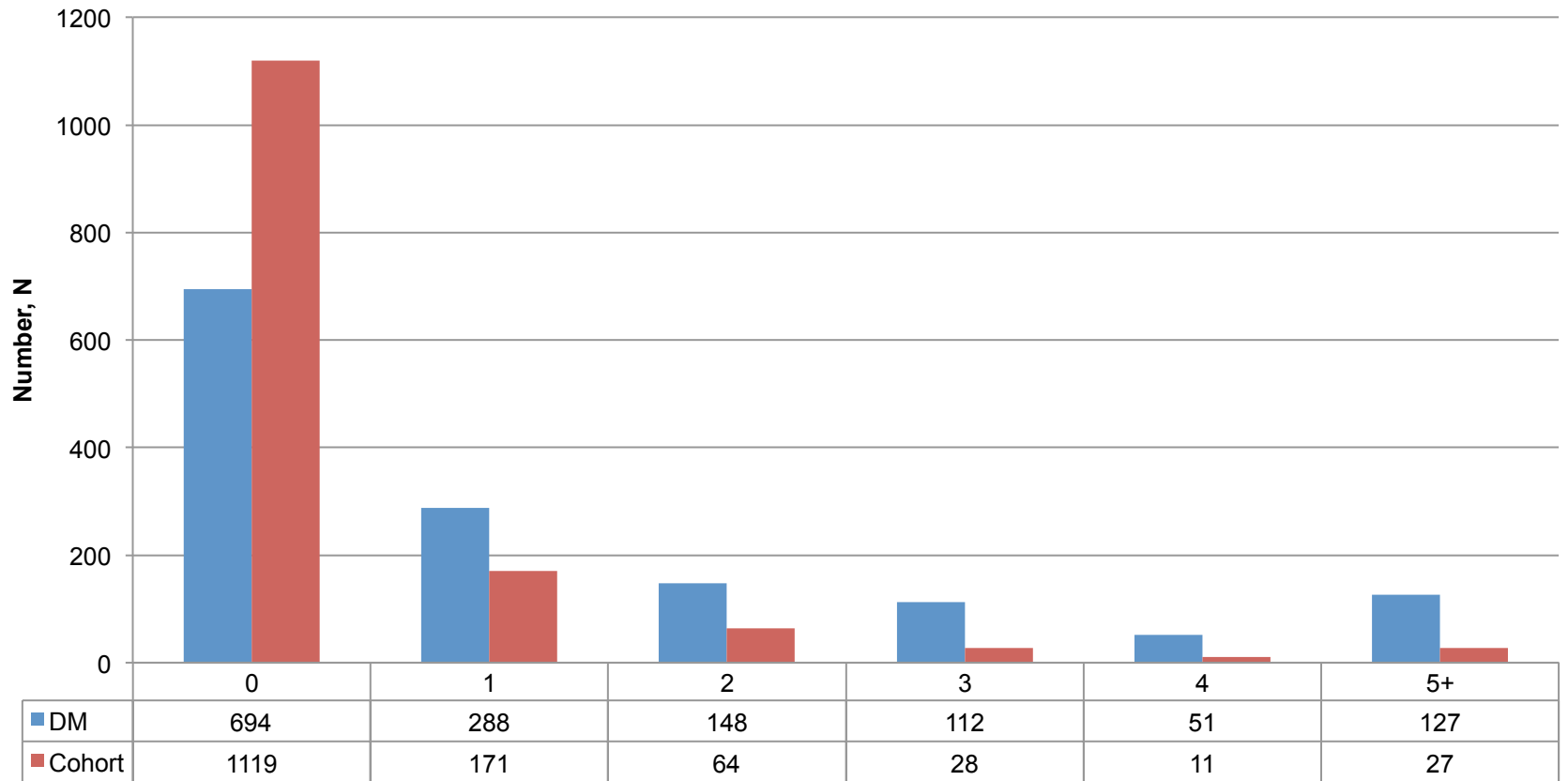| DM Population (N=628)	Matched Cohort (N=628)
Family Practice (2627)	Family Practice (1927)
Internist (2237)	Internist (1042)
Neurologist (1509)	Dermatologist (418)
Cardiologist (822)	Orthopedist (398)
Pediatrician (758)	Pediatrician (349)

"Rate" of Inpatient Hospitalizations

	0-1 Years Post Dx	1-2 Years Post Dx	2-3 Years Post Dx
DM	34	25	23
Matched	9	8	7

"Rate" of ED Visits (/100)

	0-1 Years Post Dx	1-2 Years Post Dx	2-3 Years Post Dx
DM	44	37	38
Matched	19	18	15

Rate of Office Visits

	0-1 Years Post Dx	1-2 Years Post Dx	2-3 Years Post Dx
DM	850	739	698
Matched	390	362	386

Total Chronic Conditions

	0	1	2	3	4	5+
DM	694	288	148	112	51	127
Cohort	1119	171	64	28	11	27

Study Team:

- David Herbert, MBA, Mayo Clinic Emeritus Staff; CEO 46 North, LLC., Treasurer, Myotonic Dystrophy Foundation

- Duygu Selcen, MD, Mayo Clinic Associate Professor of Neurology and Pediatrics, and Consultant - Pediatric Neurology

- Lindsey Sangaralingham, MPH, Assistant Professor of Health Services Research and Principal Health Service Analyst, Mayo Clinic Center for the Science of Health Care Delivery

- Stephanie Schilz, BA, Statistical Programmer Analyst, Mayo Clinic Center for the Science of Health Care Delivery

- Dennis Asante, MS, Statistical Programmer Analyst, Mayo Clinic Center for the Science of Health Care Delivery

- Sharon Hesterlee, Ph.D. - Executive Vice President, Patient Advocacy and Public Affairs, Bamboo Therapeutics, Inc.; former Chief Scientific Officer, Myotonic Dystrophy Foundation.

- John Porter, Ph.D. – Chief Scientific Officer, Myotonic Dystrophy Foundation

- Molly White, CEO, Myotonic Dystrophy Foundation