RWD91

Healthcare Resource Use Among Children with Mucopolysaccharidosis type III (MPS III): A retrospective claims database analysis in the US

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INTRODUCTION

- Mucopolysaccharidosis type III (MPS III), also known as Sanfilippo syndrome, is a rare, progressive, inherited lysosomal storage disorder caused by mutations in the *SGSH* gene. Patients with this disease are unable to produce sufficient sulfamidase to break down heparan sulfate, leading to accumulation in the brain, resulting in progressive and irreversible brain damage, and global developmental arrest, decline, and early death.
- MPS III has a global incidence of 1:70,000.[1] Children often appear healthy at birth, but begin to develop symptoms – including developmental delays, speech difficulties, and behavioral issues such as hyperactivity, aggression, and sleep disturbances – between the ages of 2 and 6. As disease progression continues, children begin to experience severe cognitive decline, loss of communication skills, and loss of mobility.
- MPS III is progressive and ultimately leads to death. The median life expectancy of patients living with MPS III is between 10 to 20 years.[2] Currently, there are no approved disease-modifying therapies for MPS III and most treatment is geared towards supportive care to manage symptoms and improve quality of life. However, there are some enzyme replacement therapies and gene therapies that are under development that may potentially impact the progression of disease through the restoration of enzyme activity.

OBJECTIVE

- The primary objective of this study was to better understand the economic burden and impact of MPS III on children, including direct medical costs like hospital stays, specialist visits, supportive care, and medical procedures, to build a foundation for future evidence-based decisions in healthcare policy, reimbursement strategies, and the development of future treatments.

METHODS

- This was a retrospective, non-interventional cohort study using the claims database, IQVIA PharMetrics® Plus. Children diagnosed with MPS III using the ICD-10 code E76.22 in any billing position of a claim from 01-Jan-2016 to 31-Mar-2024 were included in the study. Patients had ≥12 months of continuous enrollment (CE) during the study period. Patients were excluded if there was any evidence of clinical trial participation, pregnancy, or data quality issues.
- The control cohort, patients without the ICD-10 code for MPS III, were matched on age, gender, payer type on CE start month, and CE start year (1:20 of MPS III to the control group). Patients were also excluded if there was any evidence of clinical trial participation, pregnancy, or data quality issues. Supportive or adjunctive therapies, clinical manifestations, and Health Care Resource Utilization (HCRU) were assessed during all time periods available after the first diagnosis code date and were identified using ICD-10 codes in any care setting.

RESULTS

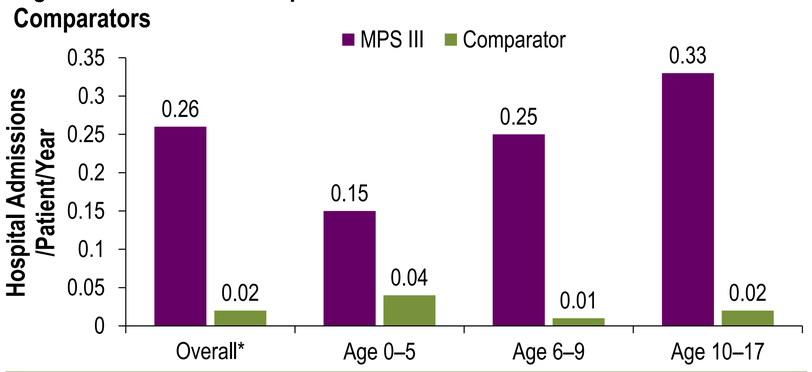
Table 1. Baseline Demographics for Pediatric MPS III Base Case and the Comparator

- A total of 204 patients with MPS III were identified in IQVIA PharMetrics® Plus database utilizing ICD-10 code E76.22, 113 children and 91 adults.
  - Given this database covers approximately 1/3 of the US total population, we can estimate >600 patients with MPS III in the US.
  - Assuming between 55–70% of all patients with MPS III have subtype IIIA,[1,3] we can estimate there to be around 300–400 patients with MPS IIIA in the US.
- Within 113 children for MPS III, 35 (30.9%) of them were age ≤5, 27 (24.0%) of them were age 6–9, and 51 (45.1%) of them were age 10–17. 56 (49.6%) were female.

	MPS III Base Case			Comparator Group		
Characteristic	Age ≤5	Age 6–9	Age 10–17	Age ≤5	Age 6–9	Age 10–17
Total Number of Patients	35	27	51	700	540	1,020
Sex						
Female, n (%)	17 (48.57%)	16 (59.26%)	23 (45.1%)	340 (48.57%)	320 (59.26%)	460 (45.1%)
Age (years)						
Mean (SD)	3.2 (1.71)	7.59 (1.22)	13.29 (2.29)	3.2 (1.69)	7.59 (1.2)	13.29 (2.27)
Median (IQR)	3 (2, 5)	8 (6, 9)	13 (11, 16)	3 (2, 5)	8 (6, 9)	13 (11, 16)
Min, Max	0, 5	6, 9	10, 17	0, 5	6, 9	10, 17
Payer Type, n (%)						
Commercial fully insured, n (%)	24 (68.57%)	18 (66.67%)	29 (56.86%)	331 (47.29%)	259 (47.96%)	485 (47.55%)
Commercial self-Insured, n (%)	6 (17.14%)	5 (18.52%)	12 (23.53%)	269 (38.43%)	201 (37.22%)	335 (32.84%)
Medicaid, n (%)	5 (14.29%)	4 (14.81%)	10 (19.61%)	100 (14.29%)	80 (14.81%)	200 (19.61%)
Medicare, n (%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)
Geographic Region, n (%)						
Midwest, n (%)	8 (22.86%)	10 (37.04%)	26 (50.98%)	231 (33%)	158 (29.26%)	318 (31.18%)
Northeast, n (%)	1 (2.86%)	5 (18.52%)	5 (9.8%)	108 (15.43%)	83 (15.37%)	148 (14.51%)
South, n (%)	18 (51.43%)	10 (37.04%)	12 (23.53%)	238 (34%)	214 (39.63%)	357 (35%)
West, n (%)	8 (22.86%)	2 (7.41%)	8 (15.69%)	120 (17.14%)	83 (15.37%)	190 (18.63%)
Unknown, n (%)	0 (0%)	0 (0%)	0 (0%)	3 (0.43%)	2 (0.37%)	7 (0.69%)
Follow Up Time (month)						
Mean (SD)	4.17 (2.31)	3.66 (2.12)	3.67 (2.54)	3.47 (2.13)	3.46 (2.2)	3.31 (2.15)
Median (IQR)	3.58 (2.08, 6.33)	3.33 (2, 5.42)	2.67 (2, 4.92)	3 (1.67, 4.96)	2.83 (1.5, 5.33)	2.5 (1.42, 4.92)
Min, Max	1, 8.33	1, 8.33	1, 8.33	1, 7.83	1, 7.75	1, 7.75

- Hospital admissions for children age 0–17 with MPS III were largely due to MPS III-related causes.
- There was an increase in hospital admissions and ER visits as children age.
- Outpatient visits decreased as children age, likely due to the increased family management as the child becomes less mobile, and in the end, palliative care in the home.
- There was much more HCRU when it comes to outpatient hospital visits compared to the other uses of HCRU.

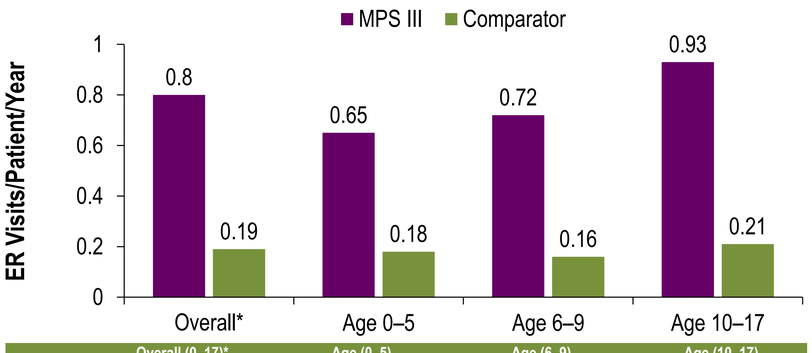
Figure 1a. Annualized Hospital Admissions for Children with MPS III and Comparators



Hospital Admissions/ Patient/ Year	Overall (0–17)* Total Number of Patients	Mean (SD)	Median (IQR)	Age (0–5) Total Number of Patients	Mean (SD)	Median (IQR)	Age (6–9) Total Number of Patients	Mean (SD)	Median (IQR)	Age (10–17) Total Number of Patients	Mean (SD)	Median (IQR)
Comparator	2,260	0.02 (0.17)	0 (0, 0)	700	0.04 (0.23)	0 (0, 0)	540	0.01 (0.08)	0 (0, 0)	1,020	0.02 (0.16)	0 (0, 0)
MPS III	113	0.26 (0.62)	0 (0, 0.35)	35	0.15 (0.29)	0 (0, 0.19)	27	0.25 (0.55)	0 (0, 0.35)	51	0.33 (0.79)	0 (0, 0.44)

*There was a significant difference in MPS III children and their comparators age 0–17 in annualized hospital admissions.

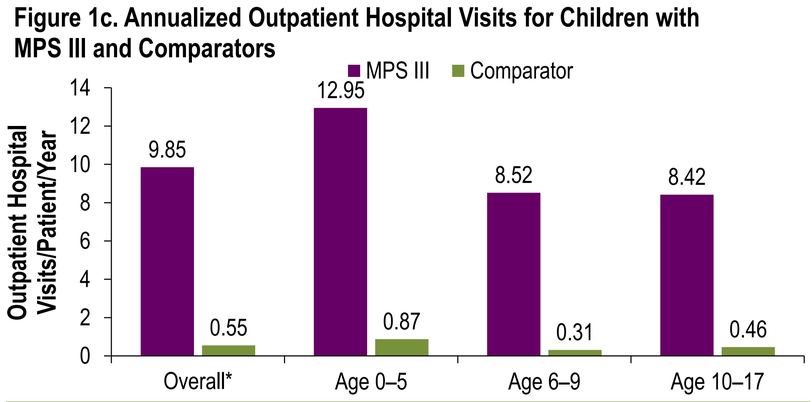
Figure 1b. Annualized ER Visits for Children with MPS III and Comparators



ER Visits /Patient/ Year	Overall (0–17)* Total Number of Patients	Mean (SD)	Median (IQR)	Age (0–5) Total Number of Patients	Mean (SD)	Median (IQR)	Age (6–9) Total Number of Patients	Mean (SD)	Median (IQR)	Age (10–17) Total Number of Patients	Mean (SD)	Median (IQR)
Comparator	2,260	0.19 (0.5)	0 (0, 0.19)	700	0.18 (0.42)	0 (0, 0.21)	540	0.16 (0.39)	0 (0, 0.17)	1,020	0.21 (0.59)	0 (0, 0.2)
MPS III	113	0.8 (1.06)	0.5 (0, 1)	35	0.65 (0.65)	0.59 (0, 0.92)	27	0.72 (1.04)	0.41 (0, 0.86)	51	0.93 (1.27)	0.44 (0, 1.02)

*There was a significant difference in MPS III children and their comparators age 0–17 in annualized ER visits.

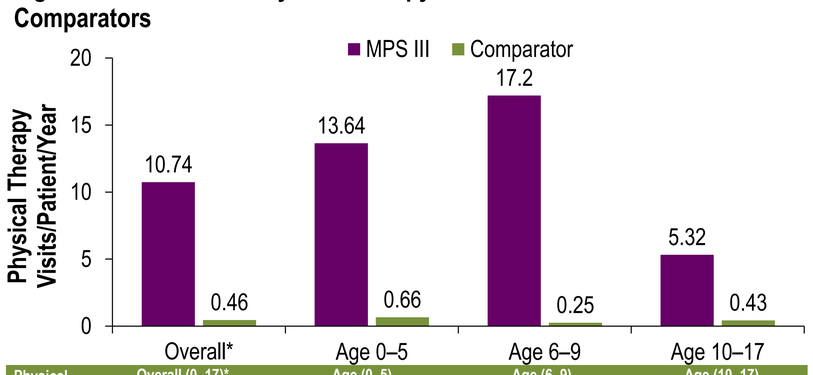
Figure 1c. Annualized Outpatient Hospital Visits for Children with MPS III and Comparators



Outpatient Hospital Visits/ Patient/Year	Overall (0–17)* Total Number of Patients	Mean (SD)	Median (IQR)	Age (0–5) Total Number of Patients	Mean (SD)	Median (IQR)	Age (6–9) Total Number of Patients	Mean (SD)	Median (IQR)	Age (10–17) Total Number of Patients	Mean (SD)	Median (IQR)
Comparator	2,260	0.55 (5.44)	0 (0, 0.33)	700	0.87 (9.52)	0 (0, 0.39)	540	0.31 (0.99)	0 (0, 0.26)	1,020	0.46 (1.67)	0 (0, 0.33)
MPS III	113	9.85 (19.02)	3.33 (1.15, 9.83)	35	12.95 (17.74)	7.58 (1.5,13.02)	27	8.52 (23.49)	2.82 (1.09, 5.4)	51	8.42 (17.31)	3 (1, 9.18)

*There was a significant difference in MPS III children and their comparators age 0–17 in annualized outpatient hospital visits.

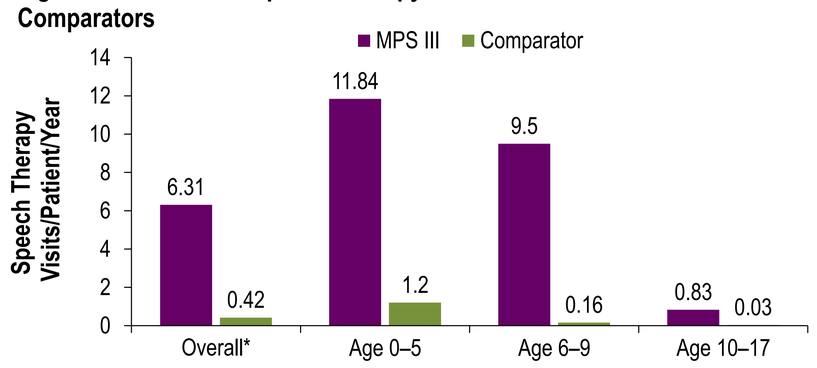
Figure 1d. Annualized Physical Therapy Visits for Children with MPS III and Comparators



Physical Therapy/ Visits /Patient/ Year	Overall (0–17)* Total Number of Patients	Mean (SD)	Median (IQR)	Age (0–5) Total Number of Patients	Mean (SD)	Median (IQR)	Age (6–9) Total Number of Patients	Mean (SD)	Median (IQR)	Age (10–17) Total Number of Patients	Mean (SD)	Median (IQR)
Comparator	2,260	0.46 (2.97)	0 (0, 0)	700	0.66 (4.57)	0 (0, 0)	540	0.25 (2.01)	0 (0, 0)	1,020	0.43 (1.74)	0 (0, 0)
MPS III	113	10.74 (20.74)	1 (0, 8.55)	35	13.64 (18.63)	3.2 (0,18.75)	27	17.2 (31.9)	2.08 (0, 10)	51	5.32 (11.84)	0 (0, 3.5)

*There was a significant difference in MPS III children and their comparators age 0–17 in annualized physical therapy visits.

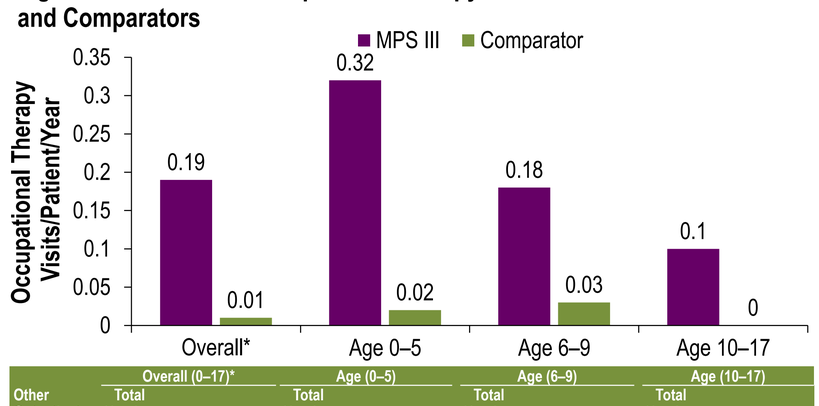
Figure 1e. Annualized Speech Therapy Visits for Children with MPS III and Comparators



Other Outpatient Visits/ Patient/year	Overall (0–17)* Total Number of Patients	Mean (SD)	Median (IQR)	Age (0–5) Total Number of Patients	Mean (SD)	Median (IQR)	Age (6–9) Total Number of Patients	Mean (SD)	Median (IQR)	Age (10–17) Total Number of Patients	Mean (SD)	Median (IQR)
Comparator	2,260	0.42 (3.71)	0 (0, 0)	700	1.2 (6.45)	0 (0, 0)	540	0.16 (1.45)	0 (0, 0)	1,020	0.03 (0.53)	0 (0, 0)
MPS III	113	6.31 (16.21)	0 (0, 2.47)	35	11.84 (18.08)	3.77 (0, 19.58)	27	9.5 (23.89)	0 (0, 2.54)	51	0.83 (3.41)	0 (0, 0.49)

*There was a significant difference in MPS III children and their comparators age 0–17 in annualized speech therapy visits.

Figure 1f. Annualized Occupational Therapy Visits for Children with MPS III and Comparators

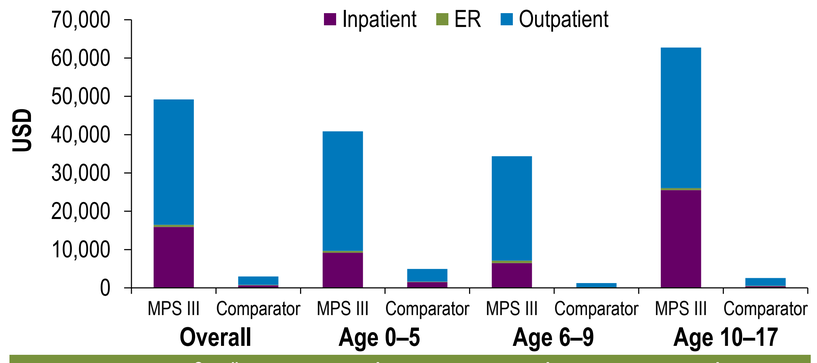


Other Outpatient Visits/ Patient/year	Overall (0–17)* Total Number of Patients	Mean (SD)	Median (IQR)	Age (0–5) Total Number of Patients	Mean (SD)	Median (IQR)	Age (6–9) Total Number of Patients	Mean (SD)	Median (IQR)	Age (10–17) Total Number of Patients	Mean (SD)	Median (IQR)
Comparator	2,260	0.01 (0.22)	0 (0, 0)	700	0.02 (0.13)	0 (0, 0)	540	0.03 (0.42)	0 (0, 0)	1,020	0 (0.05)	0 (0, 0)
MPS III	113	0.19 (0.36)	0 (0, 0.23)	35	0.32 (0.48)	0.13 (0, 0.38)	27	0.18 (0.27)	0 (0, 0.37)	51	0.1 (0.27)	0 (0, 0)

*There was a significant difference in MPS III children and their comparators age 0–17 in annualized occupational therapy visits.

Figure 2. Annualized Medical Paid Costs by Setting of Care for Children with MPS III and Comparators

- Children with MPS III had higher annual medical costs compared to their control cohort counterparts across all the age groups.
- The bulk of the medical costs were outpatient costs.
- There was a large increase in inpatient costs for the children in the age 10–17 group.



	Overall (0–17)		Age (0–5 years)		Age (6–9 years)		Age (10–17 years)	
Inpatient	15,902	691	9,160	1,529	6,469	99	25,253	428
ER	537	95	481	80	642	68	519	120
Outpatient	32,724	2,202	31,234	3,324	27,272	1,096	36,693	2,018

LIMITATIONS

- In claims data, payment records are based on diagnostic coding that may be driven by reimbursement concerns and may or may not accurately reflect the true medical condition.
- There were no commercially available treatments for MPS III and no national newborn screening program in the USA during the study period; therefore, MPS III may have been underdiagnosed or misdiagnosed.
- The study's retrospective design required patients to have continuous enrollment during the observation period. This could lead to the introduction of survival bias as patients who didn't live long enough to meet the requirement were excluded.
- The claims database did not include information on patient deaths, making it not possible to assess fatal outcomes or long-term survival trends in patients with MPS III.

DISCUSSION

- MPS III is associated with substantial burden of illness for children and the health care system.
- Children with MPS III required more HRU than their cohort control comparators, especially in the outpatient hospital setting leading to substantially higher medical costs.
  - Children with MPS III in the older age bracket of 10–17 also required more visits in the inpatient hospital setting.
- HRU and costs were much higher in the 10–17 pediatric patient MPS III population.

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