Epidemiology, disease burden and costs of Duchenne Muscular Dystrophy in Germany: an observational, retrospective health claims data analysis

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Supplementary Materials

Supplemental table 1 Inclusion and exclusion criteria applied to identify DMD patients with sources from which algorithm was adapted

Criteria (inclusion)	Rationale
M2Q of G71.0 diagnosis code (the first year in which this is fulfilled)	ICD-10 code G71.0 used for muscle dystrophies in Germany [1]. We implemented M2Q as an internal validation step to minimize possible miscoding [2, 3]
At least 1 intermediate/final diagnosis of G71.0 (M1Q) At least one G71.0 diagnosis in at least two different clinics/hospitals in the first year	Since DMD patient care extends across several specialties [4], these criteria capturing the diagnosis code for muscle dystrophies across medical practices were a further internal validation step [3].
Age < 40	Considering the median life expectancy of DMD patients is 28.1 years (95% CI 25.1, 30.3) [5] or up to early 40s [6], this limit was intended to allow us to capture the full range of DMD stages [2].
At least one of the following age-related additional criteria (fulfilled at least once in the observation period): ▪Long-term therapy with glucocorticoids between 4 and 16 years; at least two consecutive quarters of prescription from one of either deflazacort, prednisolone, or prednisone ▪Wheelchair use < 16 years of age ▪Evidence of walking aid < 13 years of age ▪Cardiomyopathy/ACE inhibitors/Beta-blocker prescription and < 26 years of age ▪Ventilation ≥ 10 years old	Multiple criteria are considered here to capture patients across the disease course [7] ▪Long-term glucocorticoid therapy is normally prescribed to DMD patients (80-85% of patients aged 6-8) but not to patients of other muscle dystrophies [2, 7] ▪Independent mobility is typically lost in DMD between the ages of 6 and 13 years [7–9] ▪A walking aid with age limitation was intended to different from BMD patients, who typically walk normally until the age of 15 [8, 10, 11] ▪Cardiomyopathy/ACE inhibitors/Beta-blocker prescriptions at this age to differentiate DMD patients from other muscular dystrophies, was also used by [7, 12]. ▪Ventilation at this age was to differentiate DMD patients from patients with other muscular dystrophies (for example, limb girdle) [8, 12]), and from patients who may have a more severe disorder if ventilation is needed before age 10 years [13]. Criterion also used by [2, 7]
Criteria (exclusion)	Rationale
Exclusion of patients receiving ventilation ≤ 3 years of age	It is unlikely in DMD that ventilation would be needed at such an early age and this criterion was used to exclude patients with more severe disorders at this age [7, 14, 15]

Exclusion of patients with no ventilation at the age of ≥30 years of age	DMD patients typically require ventilation by the age of 30; criterion was also used by Schrader et al (2023) [2]
Exclusion of females	DMD is an x-linked disease and was used to exclude females with other muscular dystrophies [7, 14]

Supplementary table 2 Codes used in the algorithm to identify DMD patients in the WIG2 database claims data

Codes in claims data used to identify DMD patients population		
Codes used to determine glucocorticoid therapy		
Code type	Code	Description
ATC code	H02AB06	prednisolone
	H02AB07	prednisone
	H02AB13	deflazacort
Codes used to determine ventilation		
Code type	Code	Description
ICD-10-GM	Z99.1	Dependence (long-term) on a respirator
	J96.10	Chronic respiratory failure, not elsewhere classified: Type I
	J96.11	Chronic respiratory failure, not elsewhere classified: Type II
	J96.19	Chronic respiratory insufficiency, not elsewhere classified: Type unspecified
	J96.90	Respiratory insufficiency, unspecified: Type I
	J96.91	Respiratory insufficiency, unspecified: Type II
	J96.99	Respiratory insufficiency, unspecified: Type unspecified
	J95.0	Dysfunction of a tracheostoma
	Z43.0	Supply of a tracheostoma
	Z93.0	Presence of a tracheostoma
OPS	5-312	Permanent tracheostomy
	8-711	Mechanical ventilation and respiratory support for newborns and infants
	8-712	Mechanical ventilation and respiratory support in children and adolescents
	8-713	Mechanical ventilation and respiratory support for adults
	8-714	Special procedures for mechanical ventilation in severe respiratory failure
	8-716	Adjustment of home mechanical ventilation
SHI medical aids/devices	14.24.01	Aerosol inhalation devices for lower respiratory tract
	14.24.02	Aerosol inhalation devices for upper respiratory tract

	14.24.04	Oxygen therapy devices, pressurized gas filling units	
	14.24.05	Oxygen therapy devices, pressurized and liquid gas	
	14.24.06	Oxygen therapy devices, oxygen concentrators	
	14.24.08	Respiratory therapy for mucus loosening/elimination	
	14.24.09	Respirators for intermittent ventilation up to 30 hPA ventilation pressure	
	14.24.12	Ventilators for life-sustaining ventilation	
	14.24.13	Ventilators for intermittent ventilation with a ventilation pressure > 30 hPA	
	14.24.19	Customized masks for respiratory systems	
	14.24.20	CPAP systems for the treatment of sleep-related breathing disorders	
	14.24.21	Auto-CPAP systems for the treatment of sleep-disordered breathing	
	14.24.22	Bilevel systems for the treatment of sleep-disordered breathing	
	14.24.23	Auto-bilevel CPAP systems for the treatment of sleep-disordered breathing	
	14.24.24	Bilevel CPAP systems for the treatment of sleep-disordered breathing with ST function	
	14.24.25	Special devices for the treatment of sleep-disordered breathing	

Codes used to determine wheelchair use

Code type	Code	Description	
ICD-10-GM	R26	Reduced mobility	Abnormalities of gait and mobility
	R26.1	Reduced mobility	Paralytic gait
	R26.2	Reduced mobility	Difficulty in walking, not elsewhere classified
	R26.8	Reduced mobility	Other abnormalities of gait and mobility
	R26.81	Reduced mobility	Unsteadiness on feet
	R26.89	Reduced mobility	Other abnormalities of gait and mobility
	R26.9	Reduced mobility	Unspecified abnormalities of gait and mobility
	Z74.09	Reduced mobility	Other reduced mobility
SHI medical aids/devices	22.29.01	Mobility assistance, full body, lifting aid	
	22.29.02	Mobility assistance, full body, stand up aid/device for armchair/chairs	
	22.40.01	Mobility assistance, domestic use, Lift, mobile for external operation	
	22.40.02	Mobility assistance, domestic use, Lifter for external operation, wall mounted	
	22.40.04	Mobility assistance, domestic use. Accessories for lift	
	22.40.05	Mobility assistance, domestic use, Stationary lift	
	22.40.06	Mobility assistance, domestic use, ceiling lift	
	22.50.01	Mobility assistance, domestic and outdoor use/road traffic use, ramp system	

	22.50.02	Mobility assistance, domestic and outdoor use/road traffic use, orthopedic scooter
	22.50.03	Mobility assistance, domestic and outdoor use/road traffic use
	22.50.04	Mobility assistance, domestic and outdoor use/road traffic use
	22.50.05	Mobility assistance, domestic and outdoor use/road traffic use
	22.51.01	Mobility assistance, road traffic, two-wheelers for children and adolescents
	22.51.02	Mobility assistance, road traffic, tricycles for children and teenagers
	22.51.03	Mobility assistance, road traffic, accessories for two- / three-wheelers for children and adolescents
	22.51.04	Mobility assistance, road traffic, residual power tricycles for children and adolescents
	22.51.05	Mobility assistance, road traffic, walking bikes
	22.51.99	Mobility assistance, road traffic
	18.46.01	Vehicles for the sick / disabled, indoor, Select shower / toilet wheelchairs
	18.46.02	Vehicles for the sick / disabled, indoor, toilet wheelchairs
	18.46.03	Vehicles for the sick / disabled, indoor, shower wheelchairs
	18.46.04	Vehicles for the sick / disabled, indoor, Wheelchairs with single arm drive
	18.46.05	Vehicles for the sick / disabled, indoor, Indoor power wheelchairs
	18.46.06	Vehicles for the sick / disabled, indoor, power wheelchairs dismountable, foldable for indoor use
	18.46.07	Vehicles for the sick / disabled, indoor, power wheelchairs dismountable, foldable for indoor use
	18.50.01	Vehicles for the sick / disabled, indoor/outdoor/road traffic, Sliding wheelchairs
	18.50.02	Vehicles for the sick / disabled, indoor/outdoor/road traffic, wheelchairs with push rim drive
	18.50.03	Vehicles for the sick / disabled, indoor/outdoor/road traffic, adaptive wheelchairs
	18.50.04	Vehicles for the sick / disabled, indoor/outdoor/road traffic, Mobile walking aids, indoor and outdoor power wheelchairs
	18.50.05	Vehicles for the sick / disabled, indoor/outdoor/road traffic, Mobile walking aids with wheelchair function, Select power wheelchairs for children and adolescents
	18.50.06	(planned product subgroup: power wheelchairs dismountable, foldable for indoor use)
	18.50.07	Vehicles for the sick / disabled, indoor/outdoor/road traffic, Wheelchairs with fixed, residual crane-assisting drive, power wheelchairs dismountable, foldable for indoor and outdoor use / road transport select
	18.51.01	Vehicles for the sick / disabled, road traffic, Electric wheelchairs for outdoor use

	18.51.02	Vehicles for the sick / disabled, road traffic, Electric scooters
	18.51.03	Vehicles for the sick / disabled, road traffic
	18.51.04	Vehicles for the sick / disabled, road traffic, Hand crank drive pretensioned/hang-in bicycles for children and teenagers
	18.51.05	Vehicles for the sick / disabled, road traffic, Electromobiles
	18.65.01	Vehicles for the sick / disabled, stairs, staircase vehicle
	18.65.02	Vehicles for the sick / disabled, stairs
	18.65.03	Vehicles for the sick / disabled, stairs
	18.65.04	Vehicles for the sick / disabled, stairs
	18.65.05	Vehicles for the sick / disabled, stairs
	18.65.06	Vehicles for the sick / disabled, stairs
	18.65.07	Vehicles for the sick / disabled, stairs
	18.65.08	Vehicles for the sick / disabled, stairs
	18.65.09	Vehicles for the sick / disabled, stairs
	18.65.10	Vehicles for the sick / disabled, stairs
	18.65.11	Vehicles for the sick / disabled, stairs
	18.65.12	Vehicles for the sick / disabled, stairs
	18.65.13	Vehicles for the sick / disabled, stairs
	18.65.99	Vehicles for the sick / disabled, stairs

Codes used to determine use of mobility assistance

Code type	Code	Description
ICD-10-GM	Z99.3	Dependence (long-term) on assistive devices, medical equipment, or supplies, not elsewhere classified.
	Z46.7	Providing with and fitting other medical devices or aids
	Z46.8	Providing and fitting an unspecified medical device or appliance.
	Z46.9	Providing and fitting an unspecified medical device or appliance
SHI medical aids/devices	10.46.01	Walking aid, indoors, Walking frames
	10.46.02	Walking aid, indoors, Walking frames
	10.46.04	Walking aid, indoors, Mobile walking frames with forearm supports
	10.50.04	Walking aid, indoors/outdoors/street traffic, Mobile walking aids
	10.50.05	Walking aid, indoors/outdoors/street traffic, Mobile walking aids with wheelchair function

Codes used to determine cardiomyopathy

Code type	Code	Description
ICD-10-GM	I42	Cardiomyopathy (all)
ATC	C09A	ACE inhibitors or angiotensin
	C09B	ACE inhibitors or angiotensin
	C07A	Beta Blocking Agents
	C09C	Angiotensin II receptor blockers

Supplementary table 3 Codes (by category) used to determine disease stage allocation and pre-defined healthcare resource use

Disease/ Treatment	Source of healthcare data					
	ICD	EBM	DRG	OPS	SHI medical aids/devices	ATC Codes
Codes for approximation of DMD disease stage 1: Early ambulatory						
No codes defined						
Codes for approximation of DMD disease stage 2: Late ambulatory						
Long-term therapy with glucocorticoids (prednisolone, prednisone or deflazacort)						H02AB06 H02AB07 H02AB13
Scooter or wheelchair prescription (manual) or walking aid assistance	Z99.3; Z46.7; Z46.8; Z46.9;				Manual 18.50.02; 18.50.03; 18.51.01; 18.51.04; 10.46.01; 10.46.02 10.46.04; 10.50.01; 10.50.02; 10.50.03; 10.50.04; 10.50.05 22.50.02 22.50.03 22.50.04 22.50.05 22.51.01 22.51.02 22.51.03 22.51.04 22.51.05 22.51.99	
Orthotic or prosthetic therapy (footplate, ankle motion, inner boot)					24.01 24.03 31.03	

ACE/ARB, Beta-Blocker prescription or cardiomy-opathy diagnosis	I42					C09A; C09B; C09C; C01CX09 C07A
Codes for approximation of DMD disease stage 3: Early non-ambulatory						
Powered wheelchair					18.46.05; 18.50.04; 18.50.05; 18.50.06; 18.51.02; 18.51.05; 18.46.04 18.46.06 18.46.07	
Mobility					22.29.01 22.29.02 22.40.01 22.40.02 22.40.04 22.40.05 22.40.06 22.50.01 18.46.01 18.46.02 18.46.03 18.50.01 18.50.07 18.51.03 18.65.01 18.65.02 18.65.03 18.65.04 18.65.05 18.65.06 18.65.07 18.65.08 18.65.09 18.65.10 18.65.11 18.65.12 18.65.13 18.65.99	

Pulmonary management; nasal-, positive airway and breathing devices					14.24.01 14.24.02 14.24.04 14.24.05 14.24.06 14.24.19 14.24.20 14.24.21 14.24.22 14.24.23 14.24.24 14.24.25	
Scoliosis therapy	M41			5-83X	23.15	
Hospital bed or mattress					50.45.01 50.45.02 50.45.03 50.45.04 50.45.06	
ACE/ARB, Beta-Blocker prescription or cardiomy-opathy diagnosis	I42					C09A; C09B; C09C; C01CX09 C07A
Codes for approximation of DMD disease stage 4: Late non-ambulatory						
Defibrillator, ventricular support, heart transplant				5-375 5-377 5-378		
Tracheostomy and rela-ted products	Z93.0 Z43.0 J95.0				12.24	
Ventilation support and related devices	J96.10 J96.11 J96.19 J96.90 J96.91 J96.99 Z99.1			5-312; 8-711 8-712; 8-713; 8-714 8-716	14.24.09 14.24.12 14.24.13	
Enteral formula, nutri-tion infusion pump				8-017	03.36	
PEG/PEJ tube	T85.74			5-445 5-449.n		
Pre-defined variables						
Ataluren (Translarna®)						M09AX03
Scoliosis	M41			5-83X	23.15	

PEG/PEJ tube	T85.74			5-445 5-449.n		
Respiratory management						
Codes used to identify respiratory management costs	Z99.1			5-312	14.24.01	
	J96.10			8-711	14.24.02	
	J96.11			8-712	14.24.04	
	J96.19			8-713	14.24.05	
	J96.90			8-714	14.24.06	
	J96.91			8-716	14.24.08	
	J96.99				14.24.09	
	J95.0				14.24.12	
	Z43.0				14.24.13	
	Z93.0				14.24.19	
					14.24.20	
					14.24.21	
					14.24.22	
					14.24.23	
					14.24.24	
					14.24.25	
Cardiac management						
Codes used to identify cardiac management costs	I42					C09A
						C09B
						C07A
						C09C
						C01CX09

Supplementary table 4 The number of patients (N) included in the DMD study population in total by year, by inclusion/exclusion step

	First G71.0, M2Q (first year in which this is fulfilled)						
Criteria	Patients (N)	2016	2017	2018	2019	2020	2021
G71.0, M2Q (first year in which this is fulfilled)	2,517	1,264	366	231	242	206	208
At least 1 "intermediate/final diagnosis" G71.0-M1Q*	2,062	1,117	284	164	157	132	208
At least one G71.0 diagnosis in at least two different clinics/hospitals in the first year	662	423	69	44	45	33	48
Age < 40	317	214	29	15	14	18	27
Exclusion of patients receiving ventilation ≤ 3 years of age	315	213	29	15	13	18	27
At least one of the following age-related additional criterion (fulfilled in at least one year of age)	165	123	11	6	7	10	8

Long-term therapy with glucocorticoids between 4 and 16 years; at least two consecutive quarters of prescription from one of either deflazacort, prednisolone, or prednisone	35	25	< 5	< 5	< 5	< 5	< 5
Wheelchair use < 16 years of age	66	43	7	< 5	< 5	6	< 5
Evidence of walking aid < 13 years of age	21	13	< 5	< 5	< 5	< 5	< 5
Cardiomyopathy/ACE inhibitors/Beta-blocker prescription < 26 years of age	77	63	< 5	< 5	< 5	< 5	< 5
Ventilation ≥ 10 years old	93	80	5	< 5	< 5	< 5	< 5
Exclusion of patients with no ventilation at the age of ≥30 years of age	164	122	11	6	7	10	8
Exclusion of females	140	108	9	6	< 5	9	<5
At least 12 months of continuous observation data following the first G71.0-M2Q diagnosis	134	107	9	6	< 5	8	0

Supplementary table 5 Longitudinal analysis of the most frequently documented 3-digit ICD-10 GM codes from 2017–2021, by DMD disease stage approximation

ICD code	Description	Stage 1	Stage 2	Stage 3	Stage 4
Z00	General examination and investigation of persons without complaint and reported diagnosis	5 (83.33%)			
Z01	Other special examinations and investigations of persons without complaint or reported diagnosis		25 (55.56%)	19 (38.00%)	36 (57.14%)
H52	Disorders of refraction and accommodation	*	24 (53.33%)	11 (22.00%)	
Z25	Need for immunization against other single viral diseases	*	20 (44.44%)	20 (40.00%)	33 (52.38%)
J06	Acute upper respiratory infections of multiple and unspecified sites	*	19 (42.22%)	22 (44.00%)	37 (58.73%)
F82	Specific developmental disorder of motor function	*	19 (42.22%)	13 (26.00%)	
F83	Mixed specific developmental disorders	*	19 (42.22%)		
F80	Specific speech articulation disorder	*	19 (42.22%)		
Z27	Need for immunization against combinations of infectious diseases		19 (42.22%)		

Q66	Congenital deformities of feet		15 (33.33%)	16 (32.00%)	
A09	Other gastroenteritis and colitis of infectious and unspecified origin	*	14 (31.11%)		
Z11	Special screening examination for infectious and parasitic diseases		13 (28.89%)		36 (57.14%)
U99	Unassigned key number		13 (28.89%)		35 (55.56%)
E55	Vitamin D deficiency		13 (28.89%)		
H50	Other strabismus		13 (28.89%)		
M21	Other acquired deformities of limbs		12 (26.67%)		
Z99	Dependence on enabling machines and devices, not elsewhere classified		11 (24.44%)	19 (38.00%)	59 (93.65%)
M62	Other disorders of muscle		11 (24.44%)	12 (24.00%)	31 (49.21%)
M24	Other specific joint derangements		11 (24.44%)	12 (24.00%)	
R26	Abnormalities of gait and mobility		11 (24.44%)	11 (22.00%)	24 (38.1%)
H10	Conjunctivitis	*	11 (24.44%)		
M41	Scoliosis			19 (38.00%)	46 (73.02%)
I42	Cardiomyopathy			14 (28.00%)	31 (49.21%)
K59	Constipation			12 (24.00%)	36 (57.14%)
Z26	Need for immunization against other single infectious diseases	*		12 (24.00%)	
E66	Obesity			12 (24.00%)	
G47	Sleep disorders			11 (22.00%)	24 (38.1%)

I50	Heart failure			10 (20.00%)	26 (41.27%)
F43	Reaction to severe stress, and adjustment disorders			10 (20.00%)	
Z76	Persons encountering health services in other circumstances			10 (20.00%)	
R11	Nausea and vomiting			10 (20.00%)	
J96	Respiratory failure, not elsewhere classified				60 (95.24%)
Z74	Problems related to care-provider dependency				43 (68.25%)
I10	Essential (primary) hypertension				35 (55.56%)
E87	Other disorders of fluid, electrolyte, and acid-base balance				33 (52.38%)
G82	Paraplegia and tetraplegia				31 (49.21%)
R06	Abnormalities of breathing				26 (41.27%)
R13	Dysphagia				24 (38.10%)

*Note: diagnosis codes for which < 5 patients (n) were documented as 20 most frequent were not reported, however codes for which a number could be reported in other stages, and it was recorded in < 5 in other stages were marked with an * when these were under the 20 most frequent codes for patients of other stages

Supplementary table 6: Longitudinal analysis of the most frequently documented 5-digit ATC codes from 2017–2021, by DMD disease stage approximation

5-digit ATC code	Medications	Stage 1	Stage 2	Stage 3	Stage 4
A11CC	Vitamin D and analogs	*	31 (68.89%)	18 (36.00%)	22 (34.92%)
H02AB	Glucocorticoids	*	31 (68.89%)	12 (24.00%)	
M01AE	Propionic acid derivatives	*	22 (48.89%)	18 (36.00%)	27 (42.86%)
R01AA	Sympathomimetics, pure	*	17 (37.78%)	8 (16.00%)	
A12AA	Calcium		11 (24.44%)		
R05CP	Herbal expectorants	*	10 (22.22%)		
C09AA	ACE inhibitors, pure		9 (20.00%)	22 (44.00%)	38 (60.32%)

A06AD	Osmotic laxatives	*	7 (15.56%)	12 (24.00%)	32 (50.79%)
N02BE	Anilide	*	7 (15.56%)		
B05BB	Solutions with an effect on the electrolyte balance		5 (11.11%)	7 (14.00%)	26 (41.27%)
N02BB	Pyrazolones		5 (11.11%)	6 (12.00%)	30 (47.62%)
R05CB	Mucolytics	*	5 (11.11%)	5 (10.00%)	13 (20.63%)
R05DB	Other antitussives	*	5 (11.11%)		
D06AX	Other antibiotics for topical use		*	6 (12.00%)	
J01CA	Penicillins with extended spectrum of activity		*	5 (10.00%)	17 (26.98%)
C07AB	Beta-adrenoceptor antagonists, selective			18 (36.00%)	34 (53.97%)
A02BC	Proton pump inhibitors			11 (22.00%)	27 (42.86%)
R03AC	Salbutamol-ratiopharm;Sultanol;Berotec;SalbuHEXAL;Salbutamol AL			8 (16.00%)	17 (26.98%)
B01AB	Selective beta2-adrenoceptor agonists			8 (16.00%)	
J01FA	Macrolides			6 (12.00%)	11 (17.46%)
J01MA	Fluoroquinolones			5 (10.00%)	
J01CR	Combinations of penicillins, incl. beta-lactamase inhibitors			*	21 (33.33%)
J01DC	2nd generation cephalosporins	*			16 (25.4%)
R04AX	Other inhalants				13 (20.63%)
C03CA	Sulfonamides, pure				13 (20.63%)
D07AC	Corticosteroids, highly effective (group III)				13 (20.63%)
S01CA	Corticosteroids and anti-infectives in combination				11 (17.46%)
C03DA	Aldosterone antagonists				11 (17.46%)
D01AC	Imidazole and triazole derivatives				11 (17.46%)

*Note: medications for which <5 patients (n) were documented as 20 most frequent were not reported, however those medications for which a number could be reported in other stages, and it was recorded in <5 in other stages were marked with an * when these were under the 20 most frequent medications for patients of other stages

Supplementary table 7: Longitudinal analysis of the most frequently documented 4-digit SHI medical aid codes from 2017–2021, by DMD disease stage approximation

SHI Code	Medical Aid English	Stage 1	Stage 2	Stage 3	Stage 4
1899	Patient/disabled vehicle - Without special application location/additions		27 (60.00%)	34 (68.00%)	56 (88.89%)

1850	Patient/disabled vehicle - Interior and exterior/road traffic		26 (57.78%)	26 (52.00%)	39 (61.90%)
2303	Orthoses/splints - Foot	*	20 (44.44%)	10 (20.00%)	
803	Insoles - Foot	*	14 (31.11%)	5 (10.00%)	
3103	Shoes - Foot	*	12 (26.67%)	11 (22.00%)	12 (19.05%)
2399	Orthoses/splints - Without special application site/additions		11 (24.44%)		
2521	Visual aids - Eye/vision		9 (20.00%)	6 (12.00%)	
2251	Mobility aids - Road traffic		9 (20.00%)		
2611	Seating aids - Body/trunk		8 (17.78%)	14 (28.00%)	26 (41.27%)
440	Bathing and showering aids - Domestic use		6 (13.33%)	12 (24.00%)	12 (19.05%)
1050	Walking aids - Indoor and outdoor/road traffic		6 (13.33%)		
2302	Orthoses/splints - Ankle		5 (11.11%)		
2699	Seating aids - Without special application site/additions		*	11 (22.00%)	15 (23.81%)
1940	Nursing articles - Domestic use		*	23 (46.00%)	44 (69.84%)
1424	Inhalation/respiratory therapy devices - Respiratory organs		*	18 (36.00%)	61 (96.83%)
1599	Incontinence aids - Without special application site/additions		*	11 (22.00%)	31 (49.21%)
899	Insoles - Without special application site/additions		*	5 (10.00%)	
2240	Mobility aids - Domestic area			13 (26.00%)	36 (57.14%)
3340	Toilet aids - Domestic use			11 (22.00%)	16 (25.40%)
1129	HiMi against decubitus - Whole body			8 (16.00%)	31 (49.21%)
1499	Inhalation/respiratory therapy devices - Without special application site/additions			6 (12.00%)	48 (76.19%)
2229	Mobility aids - Whole body			5 (10.00%)	
2307	Orthoses/splints - Hand			5 (10.00%)	
1846	Patient/disabled vehicle - Interior			*	14 (22.22%)
2130	Measuring devices for body conditions - Skin				24 (38.10%)
1139	HiMi against decubitus - Buttocks				20 (31.75%)

124	Suction devices - Respiratory organs				18 (28.57%)
1525	Incontinence aids - Urinary/digestive organs				15 (23.81%)
399	Application aids - Without special application site/additions				15 (23.81%)
2299	Mobility aids - Without special application site/additions				14 (22.22%)

*Note: medical aids for which <5 patients (n) were documented as 20 most frequent were not reported, however patients for whom medical aids were documented that were also recorded under the 20 most frequent medications for which ≥5 patients were recorded in other disease stages, was marked with an *

Supplementary table 8: Longitudinal analysis of the most frequently documented operations/procedures, by 3-digit OPS codes from 2017–2021, by DMD disease stage approximation

Most frequent 20 documented operations/procedures (by OPS code) from 2017-2021, by DMD disease stage allocation

OPS Code	Operation/Procedure English	Stage 2	Stage 3	Stage 4
9-98	Need for care and day-care pediatric treatment	14 (31.11%)	15 (30.00%)	52 (82.54%)
1-79	Physiological function tests	11 (24.44%)	15 (30.00%)	29 (46.03%)
1-71	Pneumological functional examinations	8 (17.78%)	9 (18.00%)	26 (41.27%)
5-85	Operations on muscles, tendons, fasciae, and bursae	7 (15.56%)	*	
8-31	Immobilization with plaster cast	5 (11.11%)		
8-93	Monitoring of respiration, heart, and circulation	*	9 (18.00%)	43 (68.25%)
9-40	Psychosocial, psychosomatic, and neuropsychological therapy	*	*	9 (14.29%)
3-99	Additional information on imaging procedures	*	8 (16.00%)	9 (14.29%)
8-83	Therapeutic catheterization and cannulation of vessels	*	*	15 (23.81%)
8-80	Transfusion of blood cells	*	*	9 (14.29%)
8-90	Anesthesia	*		8 (12.7%)
8-98	Other multimodal complex treatment		*	31 (49.21%)
3-20	Computed tomography [CT], native		*	13 (20.63%)
3-22	Computed tomography [CT] with contrast medium		*	12 (19.05%)
8-39	Positioning treatment		*	9 (14.29%)
8-71	Mechanical ventilation and respiratory support via mask or tube and ventilation weaning			51 (80.95%)
8-70	Access for mechanical ventilation and measures to keep the airways open			31 (49.21%)
1-62	Diagnostic tracheobronchoscopy			16 (25.4%)

1-63	Diagnostic endoscopy of the upper digestive tract			10 (15.87%)
1-61	Diagnostic endoscopy of the upper respiratory tract			10 (15.87%)
1-84	Diagnostic puncture and aspiration			8 (12.70%)
1-20	Neurological examinations			7 (11.11%)

*Note: codes for which < 5 patients (n) were documented as 20 most frequent were not reported, however codes for which a number could be reported in other stages, and it was recorded in < 5 in other stages were marked with an * when these were under the 20 most frequent codes for patients of other stages

No OPS codes were documented for patients allocated to DMD stage 1

Supplementary table 9 Mean number of HCRU by sector per patient year (PPY), among all patients and among patients with at least 1 HCRU, stratified by disease stage and across the timeframe 2017–2021

Sector	DMD stage (n)	Mean HCRU PPY (SD) - All patients	Patients ≥ 1 HCRU (%)	Mean HCRU PPY (SD) - Patients with ≥1 HCRU
Inpatient Days	Stage 1 (n=6)	- (-)	0 (0.00%)	0 (-)
	Stage 2 (n=45)	2.80 (19.82)	24 (53.33%)	5.11 (26.48)
	Stage 3 (n=50)	1.39 (4.26)	24 (48.00%)	2.47 (4.83)
	Stage 4 (n=63)	7.22 (21.70)	60 (95.24%)	7.56 (21.70)
Hospital admissions	Stage 1 (n=6)	- (-)	< 5 (-)	- (-)
	Stage 2 (n=45)	0.63 (2.63)	26 (57.78%)	1.05 (3.29)
	Stage 3 (n=50)	0.38 (0.92)	26 (52.00%)	0.64 (0.97)
	Stage 4 (n=63)	1.66 (3.66)	60 (95.24%)	1.74 (3.60)
Outpatient visits	Stage 1 (n=6)	8.91 (11.88)	6 (100.00%)	8.91 (11.88)
	Stage 2 (n=45)	12.69 (20.59)	45 (100.00%)	12.69 (20.59)
	Stage 3 (n=50)	9.63 (15.44)	50 (100.00%)	9.63 (15.44)
	Stage 4 (n=63)	10.46 (19.07)	63 (100.00%)	10.46 (19.07)
ED visits	Stage 1 (n=6)	- (-)	< 5 (-)	- (-)
	Stage 2 (n=45)	0.24 (0.73)	16 (35.56%)	0.58 (0.99)
	Stage 3 (n=50)	0.24 (0.66)	21 (42.00%)	0.47 (0.79)
	Stage 4 (n=63)	0.42 (1.24)	39 (61.90%)	0.68 (1.47)
Number of prescriptions (outpatient pharmacy)	Stage 1 (n=6)	6.82 (13.93)	6 (100.00%)	6.82 (13.91)
	Stage 2 (n=45)	8.06 (15.84)	41 (91.11%)	8.31 (15.89)
	Stage 3 (n=50)	7.02 (12.49)	49 (98.00%)	7.06 (12.43)
	Stage 4 (n=63)	15.39 (37.10)	62 (98.41%)	15.58 (37.23)
Number of cases	Stage 1 (n=6)	2.36 (4.47)	5 (83.33%)	3.25 (4.87)

(Physio- and occupational therapy)	Stage 2 (n=45)	4.65 (7.26)	42 (93.33%)	4.80 (7.11)
	Stage 3 (n=50)	5.06 (10.47)	42 (84.00%)	5.75 (10.87)
	Stage 4 (n=63)	7.84 (20.03)	58 (92.06%)	8.41 (20.55)
Number of prescriptions (SHI medical aids)	Stage 1 (n=6)	- (-)	< 5 (-)	- (-)
	Stage 2 (n=45)	3.63 (7.64)	43 (95.56%)	3.74 (7.70)
	Stage 3 (n=50)	6.16 (10.92)	44 (88.00%)	7.0 (11.08)
	Stage 4 (n=63)	26.10 (73.91)	63 (100.00%)	26.10 (73.63)

Abbreviations: DMD: Duchenne muscular dystrophy**; ED:** emergency department**; HCRU:** healthcare resource use**; PPY** per patient year; **SD**: standard deviation.

Supplementary table 10 Mean PPY costs by sector (€, with SD) among all patients and among patients with at least 1 HCRU, stratified by disease stage and across the timeframe 2017–2021

Sector	DMD stage (n)	Mean costs in € PPY (SD) - All patients	Patients ≥1 HCRU (%)	Mean costs in € PPY (SD) - Patients with ≥1 HCRU
Inpatient costs	Stage 1 (n=6)	- (-)	<5 (-)	- (-)
	Stage 2 (n=45)	2,501.28 (17,107.62)	24 (53.33%)	4,243.64 (21,891.10)
	Stage 3 (n=50)	1,428.44 (5,154.38)	24 (48.00%)	2,508.59 (5,915.14)
	Stage 4 (n=63)	10,719.94 (61,962.38)	60 (95.24%)	11,226.56 (63,075.13)
Outpatient costs	Stage 1 (n=6)	1,039.58 (1,455.09)	6 (100.00%)	1,039.58 (1,455.09)
	Stage 2 (n=45)	1,746.56 (3,298.89)	45 (100.00%)	1,746.56 (3,298.89)
	Stage 3 (n=50)	1,032.33 (1,818.11)	50 (100.00%)	1,032.33 (1,818.11)
	Stage 4 (n=63)	1,291.77 (2,759.27)	63 (100.00%)	1,291.77 (2,759.27)
ED visits costs	Stage 1 (n=6)	- (-)	<5 (-)	- (-)
	Stage 2 (n=45)	10.26 (39.26)	16 (35.56%)	24.84 (54.56)
	Stage 3 (n=50)	9.95 (28.32)	21 (42.00%)	19.78 (32.34)
	Stage 4 (n=63)	30.37 (119.00)	39 (61.90%)	49.35 (146.76)
Outpatient pharmacy costs	Stage 1 (n=6)	123.91 (1,629.01)	6 (100.00%)	123.91 (1,673.00)
	Stage 2 (n=45)	2,577.45 (22,189.92)	41 (91.11%)	2,658.93 (22,528.86)
	Stage 3 (n=50)	591.08 (1,678.10)	49 (98.00%)	595.01 (1,679.40)
	Stage 4 (n=63)	3,785.82 (24,149.38)	62 (98.41%)	3,832.57 (24,289.59)
Physio- and occupational therapy costs	Stage 1 (n=6)	763.15 (1,998.11)	5 (83.33%)	1,049.23 (2,200.72)
	Stage 2 (n=45)	1,757.34 (2,734.19)	42 (93.33%)	1,812.93 (2,659.67)
	Stage 3 (n=50)	2,149.06 (4,590.37)	42 (84.00%)	2,439.94 (4,750.22)

	Stage 4 (n=63)	4,686.42 (13,411.42)	58 (92.06%)	5,027.43 (13,798.80)
SHI medical aids costs	Stage 1 (n=6)	- (-)	<5 (-)	- (-)
	Stage 2 (n=45)	5,006.94 (9,434.04)	43 (95.56%)	5,165.33 (9,387.39)
	Stage 3 (n=50)	9,261.22 (21,117.64)	44 (88.00%)	10,514.71 (22,098.56)
	Stage 4 (n=63)	21,374.37 (47,346.29)	63 (100.00%)	21,374.37 (47,022.27)
Cardiac management costs	Stage 1 (n=6)	- (-)	<5 (-)	- (-)
	Stage 2 (n=45)	36.32 (628.20)	13 (28.89%)	126.72 (982.42)
	Stage 3 (n=50)	1,422.77 (14,669.84)	32 (64.00%)	2,064.71 (17,592.19)
	Stage 4 (n=63)	1,673.17 (21,970.58)	47 (74.60%)	2,226.16 (25,281.12)
Respiratory management costs	Stage 1 (n=6)	- (-)	<5 (-)	- (-)
	Stage 2 (n=45)	- (-)	<5 (-)	- (-)
	Stage 3 (n=50)	146.88 (9,455.75)	13 (26.00%)	504.58 (16,812.35)
	Stage 4 (n=63)	38,482.11 (124,361.79)	62 (98.41%)	38,526.31 (120,279.06)
Total costs (€)*	Stage 1 (n=6)	2,180.73 (16,258.90)	6 (100.00%)	2,180.73 (16,258.90)
	Stage 2 (n=45)	13,599.83 (33,756.07)	45 (100.00%)	13,599.83 (33,756.07)
	Stage 3 (n=50)	14,472.08 (27,245.78)	50 (100.00%)	14,472.08 (27,245.78)
	Stage 4 (n=63)	41,888.70 (117,718.13)	63 (100.00%)	41,888.70 (117,718.13)

Abbreviations: DMD: Duchenne muscular dystrophy**; ED:** emergency department**; HCRU:** healthcare resource use**; PPY** per patient year; **SD**: standard deviation.

Supplementary table 11 Mean PPY costs by sector (€, with SD) among all patients and among patients with at least 1 HCRU, stratified by age group and across the timeframe 2017–2021

Sector	Age group, years (n)	Mean costs in € PPY (SD) - All patients	Patients ≥1 HCRU (%)	Mean costs in € PPY (SD) - Patients with ≥1 HCRU
Inpatient costs	00-03 (n=6)	- (-)	<5 (-)	- (-)
	04-07 (n=26)	528.59 (3,584.18)	10 (38.46%)	1,287.68 (3,635)
	08-12 (n=43)	7,126.16 (62,130.06)	22 (51.16%)	11,532.31 (78,772.29)
	13-17 (n=54)	7,870.95 (46,966.73)	36 (66.67%)	11,206.96 (55,717.25)
	≥18 (n=79)	5,466.32 (28,891.14)	66 (83.54%)	6,205.79 (30,503.03)
Outpatient costs	00-03 (n=6)	1,496.59 (944.1)	6 (100.00%)	1,496.59 (944.1)
	04-07 (n=26)	1,406.15 (1,802.57)	26 (100.00%)	1,406.15 (1,802.57)
	08-12 (n=43)	1,790.08 (3,400.96)	43 (100.00%)	1,790.08 (3,400.96)
	13-17 (n=54)	1,394.7 (2,312.51)	54 (100.00%)	1,394.7 (2,312.51)

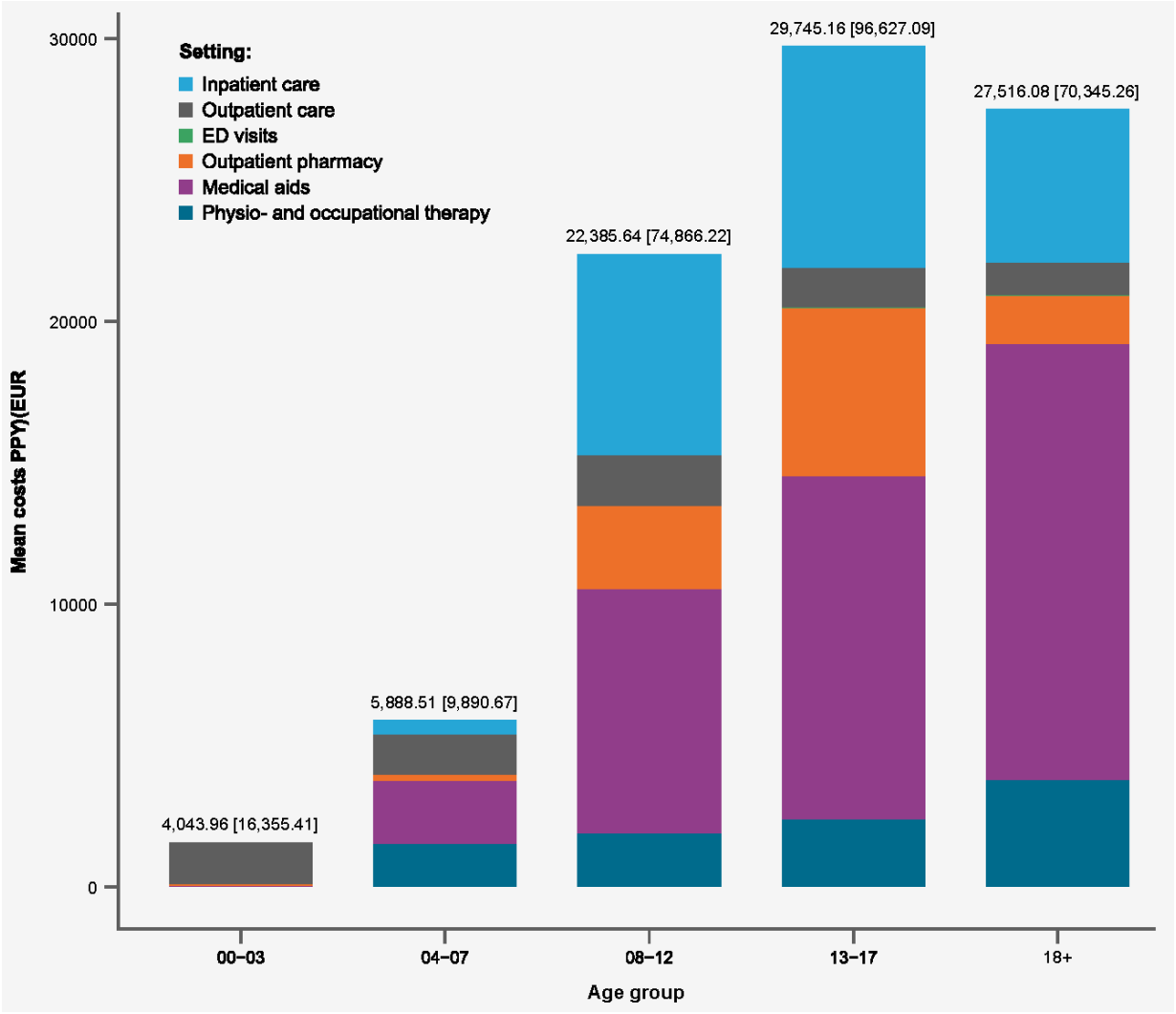
	≥18 (n=79)	1,127.47 (2,663.23)	79 (100.00%)	1,127.47 (2,663.23)
ED visits costs	00-03 (n=6)	- (-)	<5 (-)	- (-)
	04-07 (n=26)	15.2 (54.53)	9 (34.62%)	34.16 (74.54)
	08-12 (n=43)	8.96 (24.78)	19 (44.19%)	18.19 (27.55)
	13-17 (n=54)	21.17 (75.64)	20 (37.04%)	48.07 (106.87)
	≥18 (n=79)	28.7 (125.13)	45 (56.96%)	47.33 (156.07)
Outpatient pharmacy costs	00-03 (n=6)	79.65 (2,194.45)	6 (100.00%)	79.65 (2218.73)
	04-07 (n=26)	193.45 (1,551.38)	25 (96.15%)	197.12 (1542.64)
	08-12 (n=43)	2,942.51 (26,314.86)	43 (100.00%)	2,942.51 (26312.64)
	13-17 (n=54)	5,944.41 (37,986.92)	51 (94.44%)	6,118.27 (38532.31)
	≥18 (n=79)	1,706.25 (7,145.65)	77 (97.47%)	1,731.49 (7,190.5)
Physio- and occupational therapy costs	00-03 (n=6)	- (-)	<5 (-)	- (-)
	04-07 (n=26)	1,538.48 (2,177.4)	24 (92.31%)	1,593.35 (2,103.82)
	08-12 (n=43)	1,906.41 (3,346.68)	40 (93.02%)	2,004.05 (3,323.4)
	13-17 (n=54)	2,393.2 (7,095.76)	46 (85.19%)	2,662.31 (7,391.11)
	≥18 (n=79)	3,799.32 (11,545.88)	70 (88.61%)	4,241.66 (12,111.12)
SHI medical aids costs	00-03 (n=6)	- (-)	<5 (-)	- (-)
	04-07 (n=26)	2,206.64 (6,241.55)	19 (73.08%)	2,741.15 (5,515.83)
	08-12 (n=43)	8,611.51 (17,620.55)	36 (83.72%)	9,693.53 (18,137.55)
	13-17 (n=54)	12,120.73 (30,660.27)	48 (88.89%)	13,558.85 (32,054.16)
	≥18 (n=79)	15,388.02 (39,772.16)	76 (96.20%)	15,882.2 (40,081.22)
Cardiac management costs	00-03 (n=6)	- (-)	<5 (-)	- (-)
	04-07 (n=26)	- (-)	<5 (-)	- (-)
	08-12 (n=43)	82.23 (708.19)	13 (30.23%)	215.63 (925.19)
	13-17 (n=54)	137.65 (680.46)	28 (51.85%)	254.39 (891.49)
	≥18 (n=79)	1,838.46 (21,015.65)	55 (69.62%)	2,487.06 (24,383)
Respiratory management costs	00-03 (n=6)	- (-)	<5 (-)	- (-)
	04-07 (n=26)	- (-)	<5 (-)	- (-)
	08-12 (n=43)	1,373.09 (13,749.29)	6 (13.95%)	8,859.5 (22,584.55)
	13-17 (n=54)	8,473.41 (33,286.17)	21 (38.89%)	19,038.3 (45,087.04)
	≥18 (n=79)	31,457.51 (151,826.17)	65 (82.28%)	36,727.62 (161,573.28)
Total costs (€)*	00-03 (n=6)	4,043.96 (16,355.41)	6 (100.00%)	4,043.96 (16,355.41)

	04-07 (n=26)	5,888.51 (9,890.67)	26 (100.00%)	5,888.51 (9,890.67)
	08-12 (n=43)	22,385.64 (74,866.22)	43 (100.00%)	22,385.64 (74,866.22)
	13-17 (n=54)	29,745.16 (96,627.09)	54 (100.00%)	29,745.16 (96,627.09)
	≥18 (n=79)	27,516.08 (70,345.26)	79 (100.00%)	27,516.08 (70,345.26)

Abbreviations: ED: emergency department**; HCRU:** healthcare resource use**; PPY** per patient year; **SD**: standard deviation.

Supplementary table 12 The proportion (%) of males remaining in the DMD population formed by the algorithm by inclusion/exclusion criteria

Criteria	Males	Females	% Males
G71.0, M2Q (first year in which this is fulfilled)	1402	1115	56%
At least 1 "intermediate/final diagnosis" G71.0-M1Q*	1159	903	56%
At least one G71.0 diagnosis in at least two different clinics/hospitals in the first year	421	241	64%
Age <40	242	75	76%
Exclusion of patients receiving ventilation ≤ 3 years of age	240	75	76%
At least one of the following age-related additional criteria (fulfilled in at least one year of age)	141	24	85%
Long-term therapy with glucocorticoids between 4 and 16 years; at least two consecutive quarters of prescription from one of either deflazacort, prednisolone, or prednisone	35	0	100%
Wheelchair use <16 years of age	58	8	88%
Evidence of walking aid <13 years of age	17	4	81%
Cardiomyopathy/ACE inhibitors/Beta-blocker prescription <26 years of age	72	5	94%
Ventilation ≥10 years old	78	15	84%
Exclusion of patients with no ventilation at the age of ≥30 years of age	140	24	85%
Exclusion of females	140	0	100%
At least 12 months of continuous observation data following the first G71.0-M2Q diagnosis	134	0	100%



Supplementary figure 1 Mean direct healthcare costs (€) by sector and age group, per patient year (2017-2021)

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