Title: Cataloging Health State Utility Estimates for Duchenne Muscular Dystrophy and Related Conditions

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Supplemental Materials

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Table S1: Search terms for Analysis Two

ambulatory; ambulation; ambulate; ambulating
delayed growth
difficulty; trouble; problems [walking; jumping; hopping; standing; rising; lifting; running; jogging]
disability; disabled; disabling
frequent [falling; tripping]
waddle; waddling
gait
enlarged calves
sway back; saddle back; arched back
wheelchair; wheel chair; carriage; scooter
muscle [pain; stiffness; tightness; weakness; wasting]
joint [pain; stiffness; tightness; weakness; wasting]
limp; limping
mobility; mobile
assistance with walking
limb function
Gower's maneuver

Table S2: Data extracted on included utility records

Variable	Description
Source	
Publication Reference	Standard APA reference format as for a journal article (Author, Title, Journal, Volume, Issue, Pages, Year)
PMID	PubMed ID of publication
PubMed Link	Clickable hyperlink to the appropriate PubMed source
Publication Year	Self-explanatory
Tufts Source	Primary – Authors of the study measured the particular health-state utility Secondary – Authors of the study used a previously published source for health-state utility value
Secondary Source (PMID)	Self-Explanatory
Population Studied	
Indication/Disease State	Brief description of health state from the CEA Registry
Tufts Population	Sample used to measure health-state utility
Gender	Self-Explanatory
Age Range (low - high)	Self-Explanatory
Number Patients Studied	Self-Explanatory
Inclusion Criteria	Self-Explanatory
Exclusion Criteria	Self-Explanatory
Other Population Details	Self-Explanatory *May include prevalence of the disease or condition, fatality rate, and/or any other details provided by the relevant sources*
Endpoint	
Endpoint Assessed	Full description of health state (endpoint) from CEA Registry
Other Endpoint Details	Self-Explanatory
DMD Endpoint: Early ambulatory (Study Objective 1)	Health state utility value falls within the utility range for DMD early ambulatory stage (0.70–0.76)
DMD Endpoint: Late ambulatory (Study Objective 1)	Health state utility value falls within the utility range for DMD late ambulatory stage (0.61–0.67)
DMD Endpoint: Early non-ambulatory (Study Objective 1)	Health state utility value falls within the utility range for DMD early non-ambulatory stage (0.18–0.24)
DMD Endpoint: Late non-ambulatory (Study Objective 1)	Health state utility value falls within the utility range for DMD early ambulatory stage (0.15–0.21)
DMD Endpoint: Tier 1 (Study Objective 2)	Broad categorization of ambulatory condition: (1) Ambulatory, (2) Non-ambulatory
DMD Endpoint: Tier 2 (Study Objective 2)	Relevancy score of the health state to4 major DMD phases [(1) Early ambulatory stage, (2) Late ambulatory stage, (3) Early non-ambulatory stage, (4) Late non-ambulatory stage] or (5) could not be determined. *Health states will be categorized by its similarity to the clinical outcomes of each of the four ambulatory conditions*
Utility Value	
Utility Point Estimate	Self-Explanatory
Utility Point Estimate Type	E.g., mean or median
Utility Uncertainty Assessed	E.g., 95% confidence interval
Utility Confidence Interval (CI) Low	Self-Explanatory
Utility CI High	Self-Explanatory
Utility Standard Deviation	Self-Explanatory

Methodology	
Utility Assessment Method / Instrument	Broad categorization of measurement method (e.g. Time Trade Off, Standard Gamble, SF-36, EQ-5D, etc.)
Utility Assessment Details	Specific details that elucidate the methods used, (e.g. Subjects explained TTO with a sample case, then tested)
Time Period (over which utility applies)	Self-Explanatory
Comments	Any details Tufts team perceives important, but not well-captured in the other variables

Table S3. Analysis One Results—Health states estimated from HUI instrument and with comparable utility estimates to published DMD utility estimates

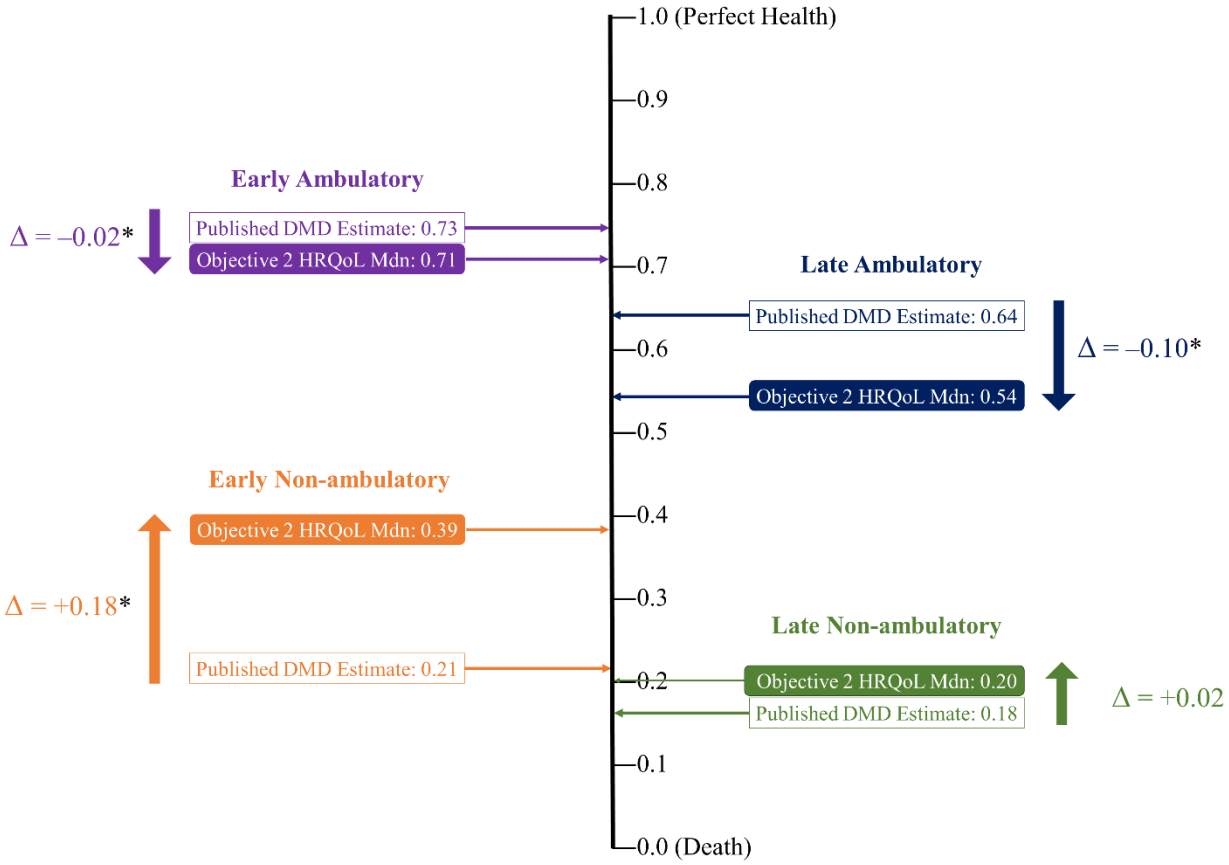
DMD Health Phase (# of health states)	Health State	CEA Study Context (Country)	Utility Estimate	Utility Assessment Method	Utility Assessment Details
Early Ambulatory (n=16)	cerebral palsy	US	0.7	HUI	
	myocardial infarction	US	0.7	EQ-5D; HUI	HUI - 3
	post-orthotopic liver transplantation	US	0.7	HUI	
	single amputation	US	0.71	HUI	
	acute bacterial sinusitis with side-effects from antibiotics	US	0.72	HUI	
	hepatocellular carcinoma	US	0.72	EQ-5D; HUI	HUI - 3
	post trochlear implant hearing loss	US	0.72	SF-36-12; HUI	
	hearing loss	US	0.723	HUI	
	chronic hepatitis (f0-f3)	US	0.73	HUI	
	liver transplant	US	0.73	EQ-5D; HUI	HUI - 3
	compensated cirrhosis	US	0.74	HUI	
	stage 2 chronic obstructive pulmonary disease + alpha-1 antitrypsin deficiency	US	0.75	HUI	Expert opinion; Rating Scale
	survivors of cardiopulmonary resuscitation	US	0.75	HUI	
	survivors of salvage cardiac extracorporeal membrane oxygenation	US	0.75	HUI	
	medulloblastoma tumor, nonmetastatic	US	0.76	HUI	Rating Scale
	permanent mild disability from subarachnoid hemorrhage	US	0.76	HUI	Expert opinion; Rating Scale
	hearing impairment (post-implantation)	US	0.61	HUI	HUI-3
	lower bound single mutation multiple mutations	US	0.61	HUI	Rating Scale
	osteoporosis-related wrist fracture: emergency room	Canada	0.61	HUI	HUI-3; Rating Scale
	population with hearing complaints	Netherlands	0.61	HUI	HUI-3; Rating Scale
	multiple amputations due to bacterial meningitis	US	0.613	HUI	HUI-3
	hearing-impaired (severe) - ahl 71-95 db	US	0.616	HUI	HUI-3; Person Trade-Off

Late Ambulatory (n=22)	cd4 count >100 cells/mm3, viral load at and no previous serious adverse event	US	0.62	HUI	HUI-3
	hearing aid with large vestibular aqueduct syndrome	US	0.62	HUI	HUI-3; QOL scores for 7 of 20 patients done by MD proxy
	otosclerosis detected in hearing aid user	Netherlands	0.62	HUI	HUI-3; Rating Scale
	survivor of neisseria meningitides with sequel (chronic sequel)	South Africa	0.62	HUI	Standard Gamble; Rating Scale
	colonic necrosis	US	0.624	HUI	
	acute illness	US	0.627	HUI	HUI-2
	psychosocial problems detected with no hearing aid	Netherlands	0.63	HUI	HUI-3; Rating Scale
	depression (4 month follow up, no intervention)	US	0.6349	EQ-5D; HUI	HUI-3
	descemet stripping automated endothelial keratoplasty	Netherlands	0.64	HUI	HUI-3; Rating Scale
	mild pain	Spain	0.64	HUI	HUI-3
	using hearing aids	Japan	0.644	HUI	HUI-3
	clinical pelvic inflammatory disease	Australia	0.65	HUI	HUI-2; Person Trade-Off
	colon cancer, last year of life	United Kingdom	0.65	HUI	HUI-3; Person Trade-Off
	ixabepilone + capecitabine, stable metastatic breast cancer	US	0.65	HUI	HUI-3; Rating Scale
	urgent reoperation	US	0.66	EQ-5D; HUI	HUI-3
	depression (post beat the blues intervention)	US	0.6651	EQ-5D; HUI	HUI-3
Early and Late Non-Ambulatory (n=9)	moderate Alzheimer's disease, community	US	0.18	HUI	
	expanded disability status scale 4-6: moderate limitations in mobility	Iran	0.18	HUI	
	survivor of colorectal cancer with recurrence	US	0.2	HUI	Rating Scale
	moderate Alzheimer's disease	US	0.21	HUI	
	profoundly deaf	US	0.22	HUI	Standard Gamble; Time Trade-Off; Rating Scale
	moderate Alzheimer's disease	Japan	0.16	HUI	

	clinical dementia rating 2	Japan	0.16	HUI	Rating Scale
	advanced dementia	US	0.165	HUI	
	moderate Alzheimer's disease, community	US	0.18	HUI	

Abbreviations: DMD, Duchenne muscular dystrophy; n, Number of identified utility records; US, United States; HUI, Health Utility Index; EQ-5D, EuroQoL 5th Dimension;

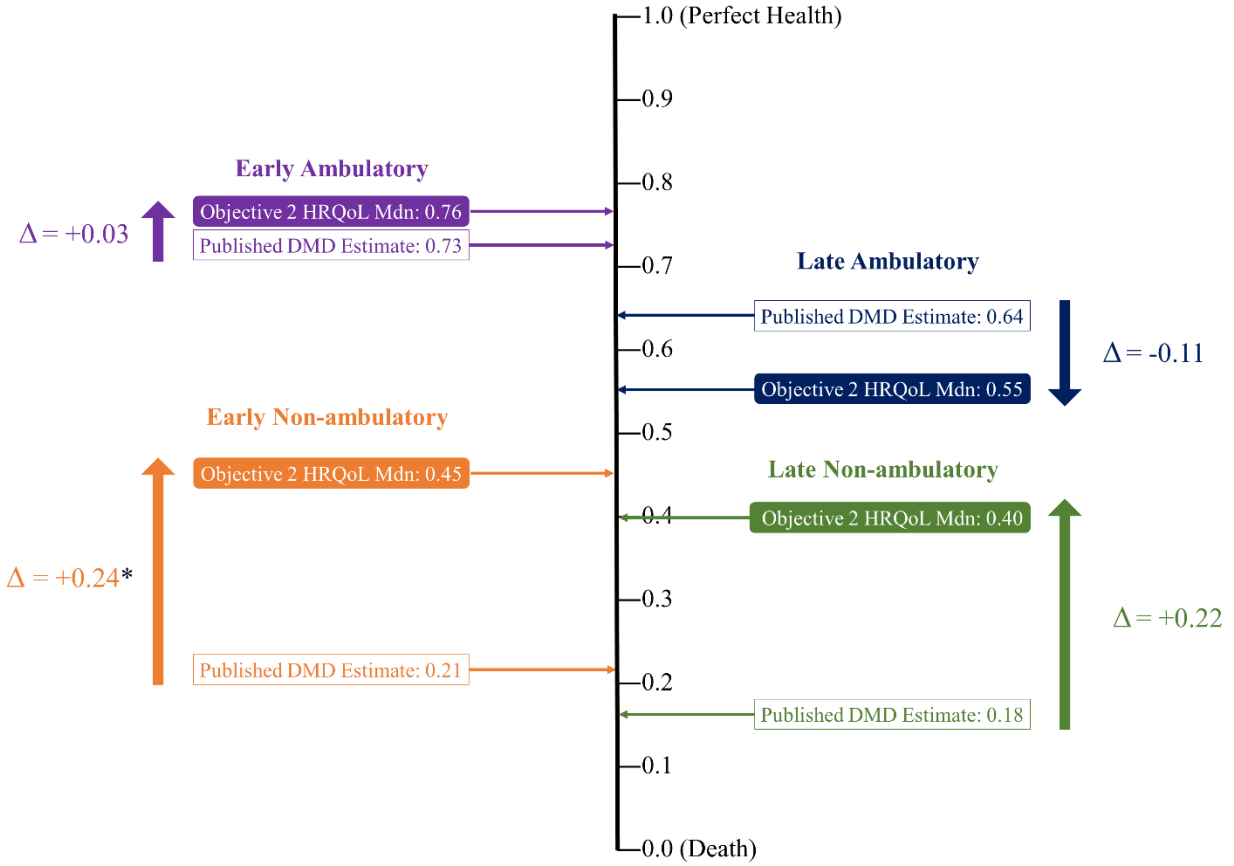
Figure S1. Differences in utility estimates for health states clinically similar to DMD and existing DMD utility estimates (Relevancy Score ≥8)



Abbreviations: HRQoL, Health-related quality of Life; Δ, change in utility estimate; Mdn, median

Notes: *No change compared to relevancy score =10

Figure S2. Differences in utility estimates for health states clinically similar to DMD and existing DMD utility estimates and extracted from US-based cost-effectiveness analysis studies (Relevancy Score ≥8)



Abbreviations: HRQoL, Health-related quality of Life; Δ, change in utility estimate; Mdn, median

Notes: *No change compared to relevancy score =10;

Figure S3: Distribution of relevancy scores for health states clinically similar to DMD

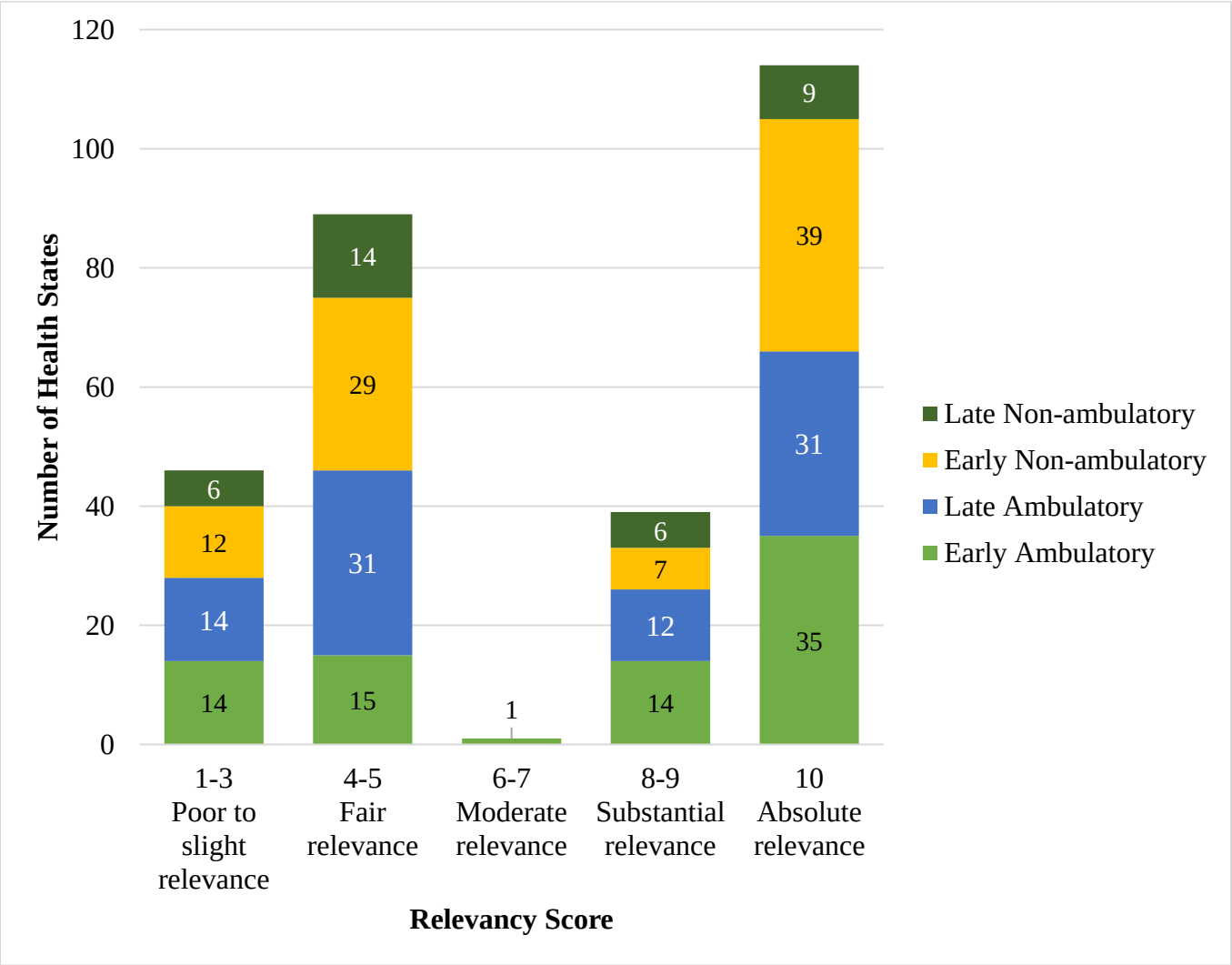
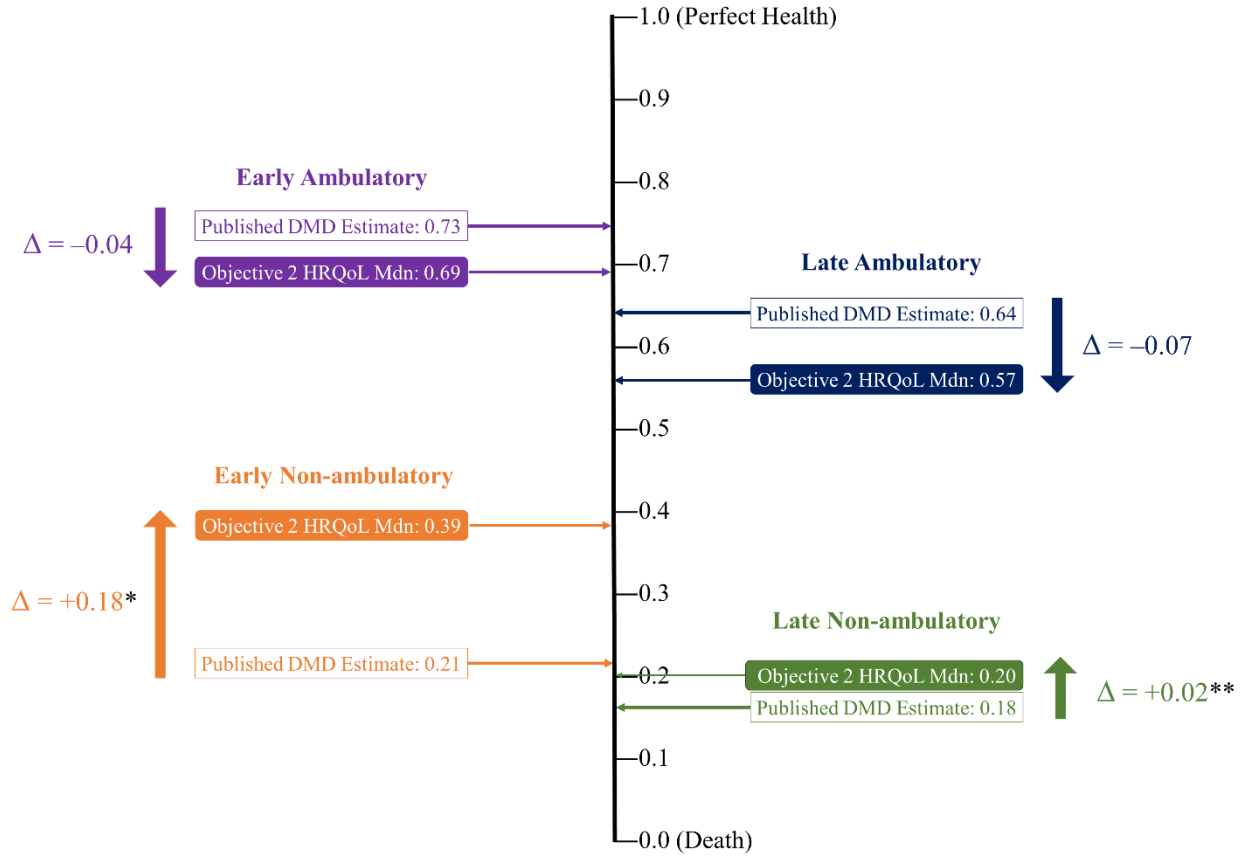


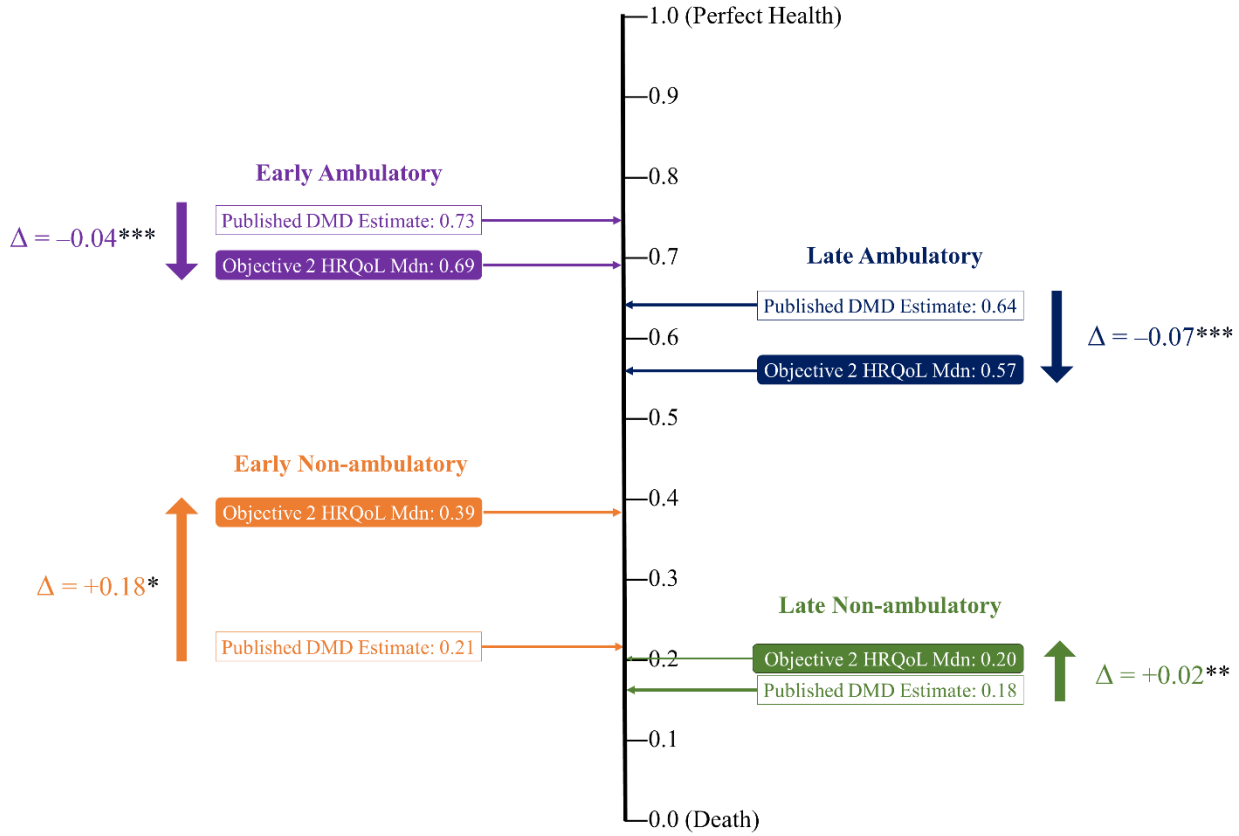
Figure S4. Differences in utility estimates for health states clinically similar to DMD and existing DMD utility estimates (Relevancy Score ≥6)



Abbreviations: HRQoL, Health-related quality of Life; Δ, change in utility estimate; Mdn, median

Notes: * No change compared to relevancy score =10; **No change compared to relevancy score ≥8

Figure S5. Differences in utility estimates for health states clinically similar to DMD and existing DMD utility estimates (Relevancy Score ≥4)



Abbreviations: HRQoL, Health-related quality of Life; Δ, change in utility estimate; Mdn, median

Notes: * No change compared to relevancy score =10; **No change compared to relevancy score ≥8; ***No change compared to relevancy score ≥6

Table S4. Summary Statistics for Analysis Two—Utility estimates for health states clinically similar to DMD (Relevancy Scores ≥6 and ≥4)

Relevancy Score	DMD Ambulatory phase	Total number of identified utility records	Mean	Median	Standard Deviation	Interquartile Range
≥6: Moderate to absolute relevance) (n=239)	Early Ambulatory	65	0.66	0.69	0.18	0.65–0.76
	Late Ambulatory	72	0.54	0.57	0.19	0.49–0.65
	Early Non-ambulatory	72	0.37	0.39	0.23	0.21–0.50
	Late Non-ambulatory	30	0.23	0.18	0.20	0.05–0.38
≥4: Fair to absolute relevance (n=243)	Early Ambulatory	65	0.66	0.69	0.18	0.65–0.76
	Late Ambulatory	74	0.53	0.57	0.20	0.49–0.65
	Early Non-ambulatory	75	0.36	0.39	0.24	0.20–0.50
	Late Non-ambulatory	29	0.23	0.18	0.21	0.05–0.38