

Supplementary Table 1: Search strategy

MEDLINE	Search term	Results	
Population	1 DMD.ti,ab,ot.	16648	
	2 (duchenne\$ adj3 (syndrome\$ or morbus)).ti,ab,ot,hw.	142	
	3 ((duchenne\$ or pseudo hypertrophic or pseudohypertrophic) adj3 dystroph\$).ti,ab,ot,hw.	28315	
	4 Duchenne muscular dystrophy/	19408	
	5 or/1-4	32117	
	6 (caregiver or caregiv* or care-giv*).ti,ab,ot,hw.	179019	
	7 5 and 6	259	
	8 (carer or carer* or caring).ti,ab,ot,hw.	111680	
	9 5 and 8	119	
	10 proxy/	2167	
	11 5 and 10	5	
	12 (parent or mother or father or son or family).ti,ab,ot,hw.	2559270	
	13 5 and 12	2630	
	14 7 or 9 or 11 or 13	2811	
	15 animal/	7727104	
	16 animal experiment/	2313035	
	(rat or rats or mouse or mice or murine or rodent or rodents or hamster or hamsters or pig or pigs or porcine or rabbit or rabbits or animal or animals or dogs or dog or cats or cow or bovine or sheep or ovine or monkey or monkeys).ti,ab,ot,hw.	13202997	
	or/15-17	13202997	
	19 14 not 18	2498	
	20 5 not 18	22414	
Outcomes	21 "Value of Life"/	129109	
	22 quality of life/	581879	
	23 quality adjusted life/	0	
	24 quality adjusted life year/	33619	
	25 quality of life index/	2552	
	26 short form 6/ or short form 36/	24512	
	27 sickness impact profile/	9232	
	28 (quality adj2 (wellbeing or well-being)).ti,ab.	3948	
		(quality of wellbeing or quality of well being or index of wellbeing or index of well being or qwb).ti,ab.	1190
	29	sickness impact profile.ti,ab.	2239
	30	disability adjusted life.ti,ab.	6048
	31		

	(qal* or qtime* or qwb* or daly* or qtime* or life year or life years).ti,ab.	48476
	32	
	(euroqol* or eq5d* or eq 5d*).ti,ab.	25792
	33	
	(qol* or hql* or hqol* or h qol* or hrqol* or hr qol*).ti,ab.	129374
	34	
	(pqol or qls).ti,ab.	937
	35	
	(health utility* or utility score* or disutilit*).ti,ab.	6418
	36	
	(hui or hui1 or hui2 or hui3).ti,ab.	3329
	37	
	health* year* equivalent*.ti,ab.	80
	38	
	(health* adj2 year* adj2 equivalent*).ti,ab.	99
	39	
	(health adj3 (utilit* or status)).ti,ab.	145359
	40	
	(utilit* adj3 (valu* or measur* or health or life or estimat* or elicit* or disease or score* or weight)).ti,ab.	27486
	41	
	(preference* adj3 (valu* or measur* or health or life or estimat* or elicit* or disease or score* or instrument or instruments)).ti,ab.	21552
	42	
	rosser.ti,ab.	200
	43	
	(hye or hyes).ti,ab.	183
	44	
	(willingness to pay or time tradeoff or time trade off or tto or standard gamble*).ti,ab.	16748
	45	
	(sf36 or sf 36 or short form 36 or shortform 36 or short form36 or shortform36 or sf thirtysix or sfthirtysix or sfthirty six or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six).ti,ab.	60639
	46	
	(sf6 or sf 6 or short form 6 or shortform 6 or sf six or sfsix or shortform six or short form six or shortform6 or short form6).ti,ab.	4005
	47	
	(sf6D or sf 6D or short form 6D or shortform 6D or sf sixD or sfsixD or shortform sixD or short form sixD or shortform6D or short form6D).ti,ab.	2097
	48	
	nottingham health profile*.ti,ab.	2594
	49	
	sickness impact profile.ti,ab.	2239
	50	
	exp health status indicators/	303835
	51	
	duke health profile.ti,ab.	193
	52	
	functional status questionnaire.ti,ab.	277
	53	
	dartmouth coop functional health assessment*.ti,ab.	23
	54	
	or/21-54	1216892
	55	
DMD carers and outcomes	56 19 and 55	277
DMD and outcomes	57 20 and 55	1042
EMBASE		
Population	58 56 use oomezd	191
	59 57 use oomezd	698

60	DMD.ti,ab,ot.	16648
61	(duchenne\$ adj3 (syndrome\$ or morbus)).ti,ab,ot,hw.	142
62	((duchenne\$ or pseudo hypertrophic or pseudohypertrophic) adj3 dystroph\$).ti,ab,ot,hw.	28315
63	Muscular Dystrophy, Duchenne/	12810
64	or/60-63	32117
65	(caregiver or caregiv* or care-giv*).ti,ab,or,hw.	179017
66	64 and 65	259
67	(carer or carer* or caring).ti,ab,ot,hw.	111680
68	64 and 67	119
69	proxy/	2167
70	64 and 69	5
71	(parent or mother or father or son or family).ti,ab,ot,hw.	2559270
72	64 and 71	2630
73	66 or 68 or 70 or 72	2811
74	exp animals/ not (exp animals/ and humans/)	16132559
75	73 not 74	2057
76	64 not 74	20830
77	"Value of Life"/	129109
78	quality of life/	581879
79	quality adjusted life/	0
80	quality-adjusted life years/	33619
81	sickness impact profile/	9232
82	(quality adj2 (wellbeing or well-being)).ti,ab.	3948
83	(quality of wellbeing or quality of well being or index of wellbeing or index of well being or qwb).ti,ab.	1190
84	sickness impact profile.ti,ab.	2239
85	disability adjusted life.ti,ab.	6048
86	(qal* or qtime* or qwb* or daly*).ti,ab.	33298
87	(euroqol* or eq5d* or eq 5d*).ti,ab.	25792
88	(qol* or hql* or hqol* or h qol* or hrqol* or hr qol*).ti,ab.	129374
89	(pqol or qls).ti,ab.	937
90	(health utility* or utility score* or disutilit*).ti,ab.	6418
91	(hui or hui1 or hui2 or hui3).ti,ab.	3329
92	health* year* equivalent*.ti,ab.	80
93	(health* adj2 year* adj2 equivalent*).ti,ab.	99
94	(health adj3 (utilit* or status)).ti,ab.	145359
95	(utilit* adj3 (valu* or measur* or health or life or estimat* or elicit* or disease or score* or weight)).ti,ab.	27486

Outcomes

		(preference* adj3 (valu* or measur* or health or life or estimat* or elicit* or disease or score* or instrument or instruments)).ti,ab.	21552
	96		
	97	rosser.ti,ab.	200
	98	(hye or hyes).ti,ab.	183
		(willingness to pay or time tradeoff or time trade off or tto or standard gamble*).ti,ab.	16748
	99		
	100	(sf36 or sf 36 or short form 36 or shortform 36 or short form36 or shortform36 or sf thirtysix or sftirtysix or sfthirty six or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six).ti,ab.	60639
	101	(sf6 or sf 6 or short form 6 or shortform 6 or sf six or sfsix or shortform six or short form six or shortform6 or short form6).ti,ab.	4005
	102	(sf6D or sf 6D or short form 6D or shortform 6D or sf sixD or sfsixD or shortform sixD or short form sixD or shortform6D or short form6D).ti,ab.	2097
	103	nottingham health profile*.ti,ab.	2594
	104	sickness impact profile.ti,ab.	2239
	105	exp health status indicators/	303835
	106	duke health profile.ti,ab.	193
	107	functional status questionnaire.ti,ab.	277
	108	dartmouth coop functional health assessment*.ti,ab.	23
	109	or/77-108	1207845
DMD carers and outcomes	110	75 and 109	167
DMD and outcomes	111	76 and 109	699
MEDLINE	112	110 use ppezv	87
MEDLINE	113	111 use ppezv	356
	114	112 or 56	279
	115	113 or 57	1056
(DMD + CG terms) + OUTCOMES	116	remove duplicates from 114	222
All DMD terms + OUTCOMES	117	remove duplicates from 115	888

Supplementary Table 2: Study quality assessment

Within the study quality assessment, one point was awarded for each of the following criteria: (1) sample size ≥ 100 ; (2) description of respondent selection and recruitment; (3) description of inclusion/exclusion criteria; (4) response rate $\geq 60\%$; (5) reporting of attrition/loss to follow-up (for longitudinal studies only); (6) reporting of missingness of data and approaches to deal with it; (7) appropriateness of measure (based on the authors' judgment). Lastly, the scores were summed for each article to yield an overall quality score, ranging from 0 to 7 (for longitudinal studies) or 0 to 6 (for cross-sectional studies) where higher scores indicated higher quality.[26]

Citation	n	Recruitment	IC/EC	Response	LFU	Missing data	Measure	Total Score
Cavazza et al, 2016 [29]	1	1	1	0	C	1	1	5/6
Landfeldt et al, 2014, 2015, 2016 [15; 17; 27; 28]	1	1	1	0	C	1	1	5/6
Landfeldt et al, 2018 [30]	1	1	1	0	C	0	1	4/6
Pangalila et al, 2011 [32]	1	1	1	0	C	1	1	5/6

C = Cross-sectional study, criterion not applicable

n=Appropriate sample size; Recruitment= Appropriate and robust respondent recruitment strategy; IC/EC=Inclusion/exclusion criteria clearly specified; Response=Response rates reported; LFU=Loss to follow-up reported; Missing data=Amount and nature of missing data described; Measure=Measure appropriateness

The quality of the utilities included in Magnetta et al.,[31] could not be rated due to that only few details were reported.

Supplementary Table 3: Utilities for DMD patients with heart failure [31]

	Mean utility
NYHA class I	0.7
NYHA class II	0.63
NYHA class III	0.55
NYHA class IV	0.44

NYHA: New York Heart Association

Supplementary Table 4: Stratified utility values for (top) DMD patients; and (bottom) DMD caregivers [27; 28]

Health state	n	Mean HUI-3 utility
<i>Caregiver-rated patient physical health</i>		
Excellent	145	0.62
Very good	321	0.5
Good	228	0.37
Fair	70	0.22
Poor	6	0
<i>Caregiver-rated patient mental health</i>		
Happy/interested	455	0.56
Somewhat happy	239	0.36
Somewhat unhappy	63	0.22
Very unhappy	13	0.04

Health state	n	Mean EQ-5D utility
<i>Caregiver-rated patient physical health</i>		
Excellent	145	0.88 (0.15)
Very good	321	0.83
Good	228	0.77
Fair/poor	76	0.71
<i>Caregiver-rated patient mental health</i>		
Happy/interested	455	0.84
Somewhat happy	239	0.79
Somewhat unhappy	63	0.69
Very unhappy	13	0.57 (0.32)

DMD: Duchenne muscular dystrophy; HUI: Health Utilities Index