

Valuation of Treatments for Rare Diseases: A Systematic Literature Review of Societal Preference Studies

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Supplementary Material

Supplemental Table S1. Search strategy

Databases: EMBASE 1974 to 2020 November 02, Ovid MEDLINE[®] and Epub Ahead of Print, In-Process, & Other Non-indexed

Citations, Daily and Versions (R) 1946 to Nov. 02, 2020

#	Searches	Results
1	("general population" or citizen* or society or (public not "public health") or "general public" or "social preference"* or insuree*).mp.	3301987
2	(rare disease* or orphan disease* or rare disorder* or orphan disorder* or rare condition* or orphan drug* or orphan product* or ultra-rare disease* or highly specialized technology* or orphan medicinal product or ultraorphan drug* or ultraorphan disease* or neglected disease* or orphan medicine* or orphan pharmaceutical* or orphan designation*).mp.	174724
3	(Life-threatening disease* or life-threatening disorder* or debilitating disease* or debilitating disorder* or "severely ill" or "seriously ill" or "serious illness" or "serious disease"* or "serious disorder"* or "severe illness" or "severe disorder"* or "severe disease"*).mp.	129469
4	(Gene therapy* or innovative medicine* or replacement therapy* or regenerative medicine* or advanced therapeutic medicinal product* or ATMP* or curative therapy* or life-extending or potential cure or curative treatment* or curative medicine*).mp.	435204
5	2 or 3 or 4	732448
6	(ranking exercise or ranking exercise or ranking experiment or rating exercise or rating exercise or rating experiment or choice-based or choice experiment or behavioral experiment* or discrete choice experiment or best/worst scaling or conjoint analysis* or conjoint measurement or swing weighting or multiple-criteria decision analysis* or contingent valuation or DCE or BWS or MCDA).mp.	33466
7	(Preference* or Health preference* or stakeholder preference* or public preference* or social preference* or societal preference* or preference value* or preference data or Social value* or societal value*).mp.	408153
8	(Willingness to pay or WTP).mp.	16462
9	(resource allocation or budget allocation or "allocate resources" or "allocate budget").mp.	46936
10	(priority setting or prioritization).mp.	10420
11	(equity or inequity).mp.	44144
12	7 or 8 or 9 or 10 or 11	515656
13	6 or 12	541562
14	1 and 5 and 13	1127
15	remove duplicates from 14	886

Supplemental Table S2. Summary of attributes assessed

Publication details	Type of publication	Elicited attributes			
		Disease & patient-related ^a	Treatment-related ^b	Economic ^c	Other
Bae et al. (2020) [1]	Preference study	Prev, Unm	Eff	Cost	-
Toumi et al. (2020) [2]	Preference study	Prev, Sev, Burd, Unm	Eff, Saf, QoL, Uncert	Cost	-
Hampson et al. (2019) [3]	Preference study	Prev, Sev, Burd	Eff, QoL	-	-
Chim et al. (2019) [4]	Preference study	Prev, Sev, Burd, Unm, Caus, Age	QoL, Innov	Cost, OOP	-
Reckers-Droog et al. (2019) [5]	Preference study	Sev, Age	-	-	-
Olofsson et al. (2019) [6]	Preference study	Prev	Eff	Cost	-
Richardson et al. (2018) [7]	Preference study	Sev, Unm	-	Cost, CE	-
Richardson et al. (2018) [8]	Preference study	Sev	-	Cost (insurance coverage)	-
Bourke et al. (2018) [9]	Preference study	Prev, Sev, Unm	Eff, QoL	Cost	-
Magalhaes et al. (2018) [10]	Preference study	Prev, Sev, Burd	Eff	-	-
Song et al. (2018) [11]	Preference study	Sev	Eff, Obj	Cost	-
Schlander et al. (2018) [12]	Preference study	Prev, Age	Eff, QoL	Cost	-
Richardson et al. (2017) [13]	Preference study	Sev	-	Cost	-
McKie et al. (2017) [14]	Preference study	Sev, Burd	-	Cost	-
Polisena et al. (2017) [15]	Preference study	Prev, Sev, Unm	Eff, QoL	Cost	National equal access
Funagoshi et al. (2017) [16]	Preference study	Prev, Sev, Burd, Unm, Age, SES	Eff, Saf, QoL, Conv, Innov	Cost, CE, OOP	Productivity loss, innovation for societal benefit, guidelines/ expert opinion, organizational requirements
Richardson et al. (2017) [17]	Preference study	Prev, Sev, Burd	-	Cost	-
Shiroiwa et al. (2016) [18]	Preference study	Prev, Sev, Unm, Caus, Age	Obj	-	-
Ramalle-Gomara et al. (2015) [19]	Preference study	Prev, Sev	Eff	Cost	-
Dragojlovic, et al (2015) [20]	Preference study	Prev	-	Cost	-

Publication details	Type of publication	Elicited attributes			
		Disease & patient-related ^a	Treatment-related ^b	Economic ^c	Other
Kolasa et al. (2015) [21]	Preference study	Sev	Eff	Cost	
Wiss et al. (2014) [22]	Preference study	Prev	-		-
Rizzardo et al. (2014) [23]	Preference study	Prev, Sev, Burd, Unm, SES	Eff, Saf, QoL, Adher	-	-
Rizzardo et al. (2014) [24]	Preference study	Prev	-	Cost	-
Linley et al. (2013) [25]	Preference study	Prev, Sev, Burd, Unm	Eff	Cost	-
Desser et al. (2013) [26]	Preference study	Prev	-	Cost	-
Mentzakis et al. (2011) [27]	Preference study	Prev, Sev	Eff	Cost	-
Richardson et al. (2011) [28]	Preference study	Sev	-	-	-
Bae et al. (2010) [29]	Preference study	Prev, Sev, SES	-	-	-
Desser et al. (2010) [30]	Preference study	Prev	-	Cost	-
Whitty et al. (2009) [31]	Preference study	Sev	Eff, QoL, Uncert	Cost	-
Oddsson (2003) [32]	Preference study	Sev	Eff	-	-
Ubel (1999) [33]	Preference study	Sev	Eff	-	-
Richardson et al. (2019) [34]	Review	Prev, Sev, Caus, Age, SES	Eff, Uncert	CE	Type of services
Upton et al. (2017) [35]	Review	Prev	Eff	Cost	-
Gu, et al (2015) [36]	Review	Prev, Sev, Burd, Caus, Age, SES	Eff, QoL, Uncert	Cost	-
Shah (2009) [37]	Review	Sev	-	-	-
Nord (1996) [38]	Review	Sev	Obj	-	-

^aDisease- and patient-related attributes: Age: patient age at disease onset; Burd: disease burden and impact on quality of life; Caus: disease causality (lifestyle or inherited); Prev: prevalence; SES: socioeconomic status of patients; Sev: severity, Unm: disease unmet need.

^bTreatment-related attributes: Adher: treatment adherence; Conv: treatment convenience (ease of use, dosage, route of administration); Eff: efficacy or health improvement; Innov: level of innovation; Obj: treatment objective (symptomatic, life extending, curative); QoL: improvement of quality of life, Saf: safety, Uncert: uncertainty.

^cEconomic attributes: CE: cost effectiveness; Cost: treatment cost and budget impact; OOP: out-of-pocket cost.

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