

Supplementary Materials

Cost-Effectiveness of Teduglutide for Pediatric Patients with Short Bowel Syndrome in Japan, Including Caregiver Burden

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Supplementary Table 1 Model inputs for patients

	Base value	DSA range		PSA distribution	Source
		Lower	Upper		
Discount rate (per year)					
Cost	2%	0	4	N/A	Japanese guideline for preparing cost-effectiveness evaluation (1)
Effectiveness	2%	0	4	N/A	Japanese guideline for preparing cost-effectiveness evaluation (1)
Patient background					
Mean age (years) at start of model	6	N/A	N/A	N/A	TED-C14-006 (2, 3)
Proportion of female patients	30.5%	N/A	N/A	N/A	TED-C14-006 (2, 3)
Proportion of patients by PS distribution at baseline					TED-C14-006 (2, 3)
High PS	85.7%	N/A	N/A	N/A	
Mid PS	11.4%	N/A	N/A	N/A	
Low PS	2.9%	N/A	N/A	N/A	
No PS	0.0%	N/A	N/A	N/A	
Transition probabilities					
Discontinuation at Year 2(2, 3)					TED-C14-006 (2, 3)
No PS [†]	0.85	0.68	1.00	Beta	
Low–High PS [†]	0.85	0.68	1.00	Beta	
Discontinuation due to nonresponse					TED-C14-006 (2, 3)
No PS	0.00	N/A	N/A	N/A	
Low PS	0.00	N/A	N/A	N/A	
Mid PS	0.00	N/A	N/A	N/A	
High PS [†]	0.50	0.40	0.60	Beta	

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Prevalence of IFALD at 2 years					NICE TA804 (4)
High PS	1.00%	N/A	N/A	N/A	
Mid PS	0.67%	N/A	N/A	N/A	
Low PS	0.33%	N/A	N/A	N/A	
No PS	0.00%	N/A	N/A	N/A	
Prevalence of IFALD at 6 years					NICE TA804 (4)
High PS	2.00%	N/A	N/A	N/A	
Mid PS	1.33%	N/A	N/A	N/A	
Low PS	0.67%	N/A	N/A	N/A	
No PS	0.00%	N/A	N/A	N/A	
Prevalence of IFALD at 10 years					NICE TA804 (4)
High PS	3.00%	N/A	N/A	N/A	
Mid PS	2.00%	N/A	N/A	N/A	
Low PS	1.00%	N/A	N/A	N/A	
No PS	0.00%	N/A	N/A	N/A	
Development rates from nonprogressive liver disease to liver fibrosis (/month)					Cavicchi <i>et al.</i> (5)
0–2.167 years	2.38%	N/A	N/A	N/A	
2.167 years and above	0.98%	N/A	N/A	N/A	
Development rates from liver fibrosis to cirrhosis (/month)					Cavicchi <i>et al.</i> (5)
0–3.083 years	1.30%	N/A	N/A	N/A	
3.083 years and above	1.20%	N/A	N/A	N/A	
AE incidence (/month) (TED)					TED-C14-006 (2, 3)
Abdominal pain [†]	4.5%	3.6	5.4	Beta	
Catheter site erythema [†]	0.6%	0.5	0.8	Beta	
Cough [†]	7.1%	5.6	8.5	Beta	
Nasopharyngitis [†]	5.8%	4.6	6.9	Beta	
Fever [†]	10.9%	8.7	13.1	Beta	
Upper respiratory tract infection [†]	6.4%	5.1	7.7	Beta	

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Vomiting [†]	10.9%	8.7	13.1	Beta	
AE incidence (/month) (SOC)					TED-C14-006 (2, 3)
Abdominal pain	0.0%	N/A	N/A	N/A	
Catheter site erythema [†]	3.7%	3.0	4.4	Beta	
Cough [†]	7.4%	5.9	8.9	Beta	
Nasopharyngitis [†]	3.7%	3.0	4.4	Beta	
Fever [†]	13.0%	10.4	15.6	Beta	
Upper respiratory tract infection [†]	9.3%	7.4	11.1	Beta	
Vomiting [†]	13.0%	10.4	15.6	Beta	
Cost					
Drug cost (JPY/day)	79 300	N/A	N/A	N/A	Drug price standard(6)
Management fee for self-injection at home (JPY/time)	7 500	N/A	N/A	N/A	Medical service fee point summary (7)
Management fee for self-injection at home (Introduction fee) (JPY/time)	5 800	N/A	N/A	N/A	Medical service fee point summary (7)
Colonoscopy (JPY/time)	13 375	N/A	N/A	N/A	Medical service fee point summary (7)
PS cost per month by PS health state (JPY)					Medical resource consumption survey (8)
Patient aged < 18 years old					
High PS‡	496 175	263 981	728 369	Gamma	
Mid PS‡	247 192	201 715	292 669	Gamma	
Low PS‡	196 279	187 125	205 433	Gamma	
No PS‡	90 670	26 879	154 461	Gamma	
Patient aged ≥ 18 years old					
High PS [†]	350 339	280 271	420 407	Gamma	
Mid PS [†]	240 299	192 239	288 359	Gamma	
Low PS [†]	171 474	137 179	205 769	Gamma	

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No PS [†]	102 981	82 385	123 577	Gamma	
AE treatment cost (JPY/time)					Medical service fee point summary (8), assumption
Abdominal pain [†]	1 775	1 420	2 130	Gamma	
Catheter site erythema [†]	735	588	882	Gamma	
Cough	0	N/A	N/A	N/A	
Nasopharyngitis	0	N/A	N/A	N/A	
Fever [†]	1 015	812	1 218	Gamma	
Upper respiratory tract infection [†]	735	588	882	Gamma	
Vomiting [†]	2 345	1 876	2 814	Gamma	
IFALD treatment cost (JPY/month)					Ikeda <i>et al.</i> (9)
Nonprogressive liver disease [†]	9 524	7 619	11 429	Gamma	
Fibrosis [†]	24 090	19 272	28 908	Gamma	
Cirrhosis [†]	38 563	30 850	46 276	Gamma	
Utilities					
Health state utility values by PS health state					Ballinger <i>et al.</i> (10)
No PS‡	0.820	0.777	0.863	Beta	
Disutility Low PS [†]	0.103	0.083	0.124	Beta	
Disutility Mid PS [†]	0.275	0.220	0.330	Beta	
Disutility High PS [†]	0.435	0.348	0.522	Beta	
Utility with IFALD [†]	0.830	0.664	0.996	Beta	Hirao <i>et al.</i> (11)
Disutility due to AE					
Abdominal pain [†]	0.0512	0.041	0.061	Beta	Sullivan <i>et al.</i> (12)
Catheter site erythema [†]	0.03	0.024	0.036	Beta	NICE TA352 (13)
Cough	0	N/A	N/A	N/A	Assumption
Nasopharyngitis	0	N/A	N/A	N/A	Assumption

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Fever	0	N/A	N/A	N/A	Assumption,
Upper respiratory tract infection [†]	0.09	0.07	0.11	Beta	NICE CG139 (14)
Vomiting [†]	0.0512	0.04	0.06	Beta	Sullivan <i>et al.</i> (12)

[†]Assuming $\pm 20\%$ for DSA and 10% of the value of the base-case analysis for PSA as SE.

[‡]Parameters for sensitivity analysis were set with 95% CI and SE of parameters.

AE, adverse event; CI, confidence interval; DSA, deterministic sensitivity analysis; IFALD, intestinal failure-associated liver disease; JPY, Japanese yen; N/A, not applicable; PS, parenteral support; PSA, probabilistic sensitivity analysis; SE, standard error; SOC, standard of care; TED, teduglutide.

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Supplementary Table 2 Model inputs for caregivers

	Base value	DSA range		PSA distribution (parameter)	Source
		Lower [†]	Upper [†]		
Caregiver utilities[‡]					
Patient aged < 18 years old					UK study (15)
No PS	1.405	N/A	N/A	N/A	
Disutility Low PS	0.052	0.041	0.062	Beta	
Disutility Mid PS	0.183	0.146	0.219	Beta	
Disutility High PS	0.162	0.129	0.194	Beta	
Patient aged ≥ 18 years old					UK study (15)
No PS	0.749	0.599	0.899	Beta	
Disutility Low PS	0.027	0.022	0.033	Beta	
Disutility Mid PS	0.097	0.078	0.117	Beta	
Disutility High PS	0.086	0.069	0.103	Beta	
Caregiver utilities[‡] (used for scenario analyses 2 and 4)					
Patient aged < 18 years old					Delphi panel (16)
No PS	1.405	N/A	N/A	N/A	
Disutility Low PS	0.155	N/A	N/A	N/A	
Disutility Mid PS	0.239	N/A	N/A	N/A	
Disutility High PS	0.464	N/A	N/A	N/A	
Patient aged ≥ 18 years old					Delphi panel (16)
No PS	0.749	N/A	N/A	N/A	
Disutility Low PS	0.082	N/A	N/A	N/A	
Disutility Mid PS	0.127	N/A	N/A	N/A	
Disutility High PS	0.247	N/A	N/A	N/A	
Productivity loss					

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WPAI OWI (%)					UK study (15)
No PS	0.00%	N/A	N/A	N/A	
Low PS	13.68%	10.94%	16.41%	Beta	
Mid PS	31.08%	24.86%	37.30%	Beta	
High PS	52.92%	42.34%	63.50%	Beta	
WPAI AI (%)					UK study (15)
No PS	0.00%	N/A	N/A	N/A	
Low PS	20.00%	16.00%	24.00%	Beta	
Mid PS	53.45%	42.76%	64.13%	Beta	
High PS	49.87%	39.89%	59.84%	Beta	
Employment (%)					Labor force survey (17)
Male	91.98%	73.58%	100.00%	Beta	
Female	78.80%	63.04%	94.56%	Beta	
Percentage of full-time employees					Labor force survey (17)
Male	91.65%	73.32%	100.00%	Beta	
Female	49.86%	39.89%	59.83%	Beta	
Average wages (JPY per month)					Basic survey on wage structure (18)
Male, full-time	577 242	461 793	692 690	Gamma	
Male, part-time	287 225	229 780	344 670	Gamma	
Female, full-time	403 850	323 080	484 620	Gamma	
Female, part-time	219 183	175 347	263 020	Gamma	
Percentage of full-time female stay-at-home parents	1.27%	1.02%	1.52%	Beta	Labor force survey (17)

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Percentage of full-time male stay-at-home parents	17.56%	14.05%	21.07%	Beta	Labor force survey (17)
Unpaid work (JPY per month)					Unpaid work evaluation (19)
Full-time female stay-at-home parents	174 250	139 400	209 100	Gamma	
Full-time male stay-at-home parents	307 583	246 067	369 100	Gamma	

†The DSA range of each parameter was $\pm 20\%$.

‡Values based on 1.5 caregivers for the patients under 18 years of age and 0.8 caregivers for patients 18 years of age and older.

AI, activity impairment; DSA, deterministic sensitivity analysis; JPY, Japanese yen; N/A, not applicable; OWI, overall work impairment; PS, parenteral support; PSA, probabilistic sensitivity analysis; WPAI, Work Productivity and Activity Impairment.

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