

Caregiving Impacts in Economic Evaluation

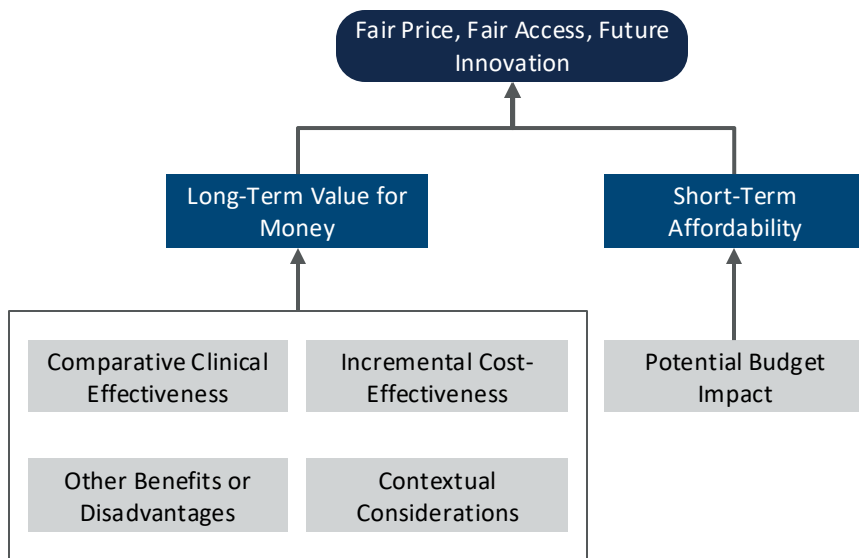
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HTA Value Assessment Framework

US Institute for Clinical & Economic Review



Qualitative and quantitative information on caregiving may be found:

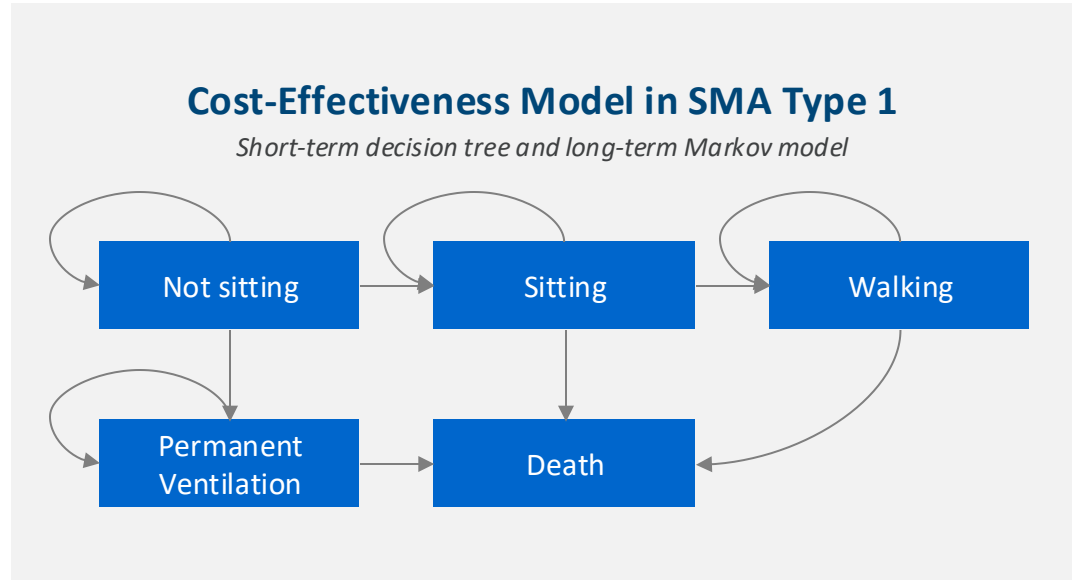
- Summary text throughout assessment
- Other benefits and disadvantages
- Contextual considerations
- **Cost-effectiveness model**

Caregiving impacts are included in ~75% of ICER Assessment, but fewer than 50% of ICER's economic evaluations

What gets measured, gets done

- What elements of caregiving impact lead to the largest change in measurement of treatment value?
- Given current tools and data available, can economic evaluation capture caregiving impacts meaningfully?

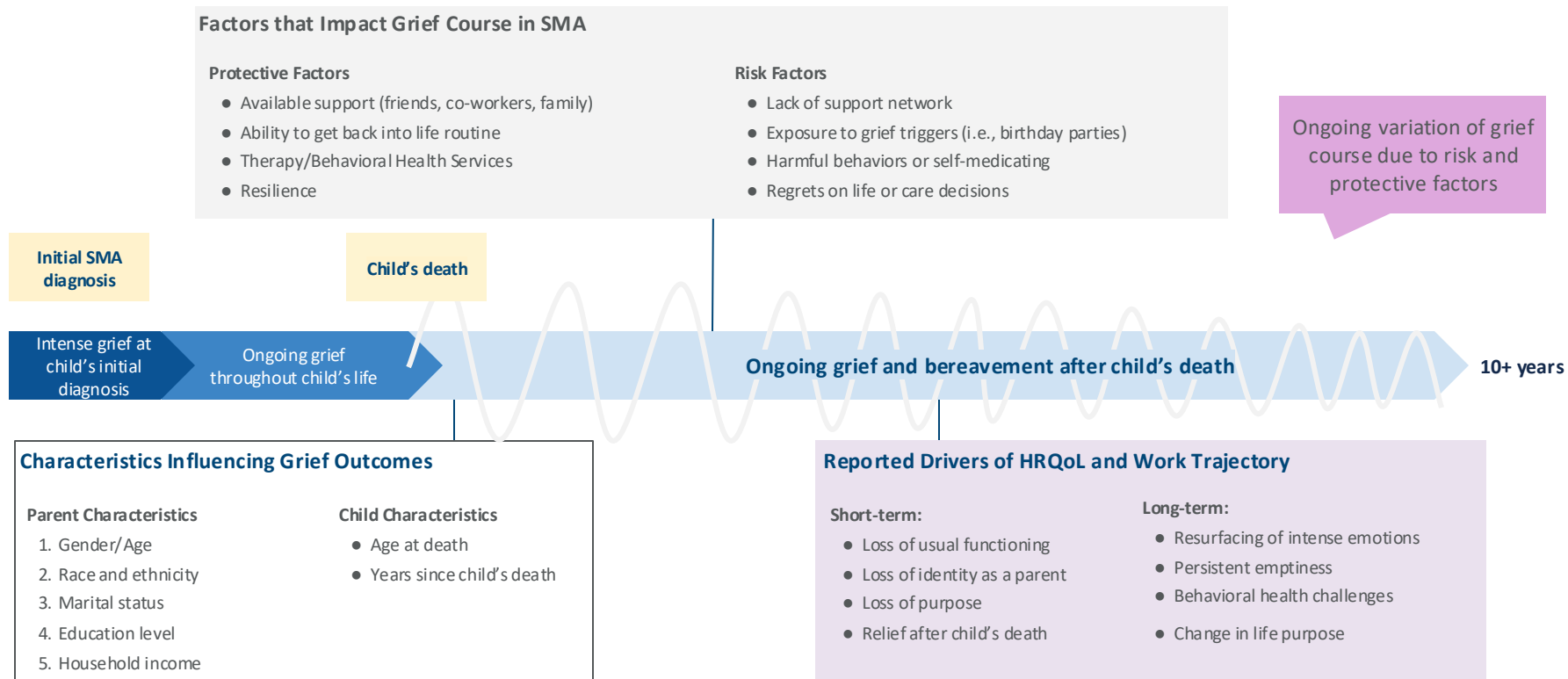
- Indirect and direct non-medical costs of SMA are high and are much larger in magnitude than direct medical costs^{1,2,7}
- SMA has significant impact on caregivers' daily activities, work productivity, and quality of life¹⁻⁷
- Disease severity is associated with increased caregiver burden¹⁻³
- Bereaved parents experience significant mental and psychosocial sequelae⁸



1. Belter, L., Cruz, R. & Jarecki, J. Quality of life data for individuals affected by spinal muscular atrophy: a baseline dataset from the Cure SMA Community Update Survey. Orphanet J Rare Dis 15, 217 (2020).
2. Er Chen, Komal Bawa, Josh Noone et al. Impact of Spinal Muscular Atrophy on Caregivers' Daily Activities and Health-Related Quality of Life, 02 August 2020,
3. Klug C, Schreiber-Katz O, Thiele S, et al. Disease burden of spinal muscular atrophy in Germany. Orphanet J Rare Dis. 2016;11(1):1-9
4. Kocova H, Dvorackova O, Vondracek P, Haberlova J. Health-Related Quality of Life in Children and Adolescents With Spinal Muscular Atrophy in the Czech Republic. Pediatr Neurol. 2014 Aug;50(6):591-4.
5. López-Bastida J, Peña-Longobardo LM, Aranda-Reneo I, et al. Social/economic costs and health-related quality of life in patients with spinal muscular atrophy (SMA) in Spain. Orphanet J Rare Dis. 2017;12(1):1-7
6. Peña-Longobardo LM, Aranda-Reneo I, Oliva-Moreno J, et al. The economic impact and health-related quality of life of spinal muscular atrophy: an analysis across Europe. Int J Environ Res Public Health. 2020;17(16):e5640.
7. The Lewin Group Inc. Cost of Amyotrophic Lateral Sclerosis, Muscular Dystrophy, and Spinal Muscular Atrophy in the United States. 2012;
8. Burden C, Bradley S, Storey C, et al. From grief, guilt pain and stigma to hope and pride - a systematic review and meta-analysis of mixed-method research of the psychosocial impact of stillbirth. BMC Pregnancy Childbirth. 2016;16:9.

New research: longitudinal impact of bereavement in SMA

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1

Replicated US ICER's 2019 SMA cost-effectiveness model¹

- Inflated costs to current year
- New hypothetical arm (reflects average performance of available DMTs)

2

Added caregiving impacts during active caregiving & bereavement

- Aligned to new SHEER taskforce recommendations²
- Patient, one parental carer

3






Robust scenario analyses to pressure test impacts

1. Pearson, S.D., et al., *The Effectiveness and Value of Treatments for Spinal Muscular Atrophy*. J Manag Care Spec Pharm, 2019. **25**(12): p. 1300-1306.

2. Henry E, Al-Janabi H, Brouwer W, Cullinan J, Engel L, Griffin S, Hulme C, Kingkaew P, Lloyd A, Payakachat N, Pennington B. Recommendations for emerging good practice and future research in relation to family and caregiver health spillovers in health economic evaluations: a report of the SHEER task force. *Pharmacoeconomics*. 2024 Mar;42(3):343-62.

Caregiving impacts included in SMA case study

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	<u>Active caregiving</u>	<u>Bereavement</u>
 Caregiver quality of life	Caregiver survey (EQ-5D)	Caregiver survey (SF-12)
 Caregiver productivity loss impact	Proxy algorithm (Jiao)	Caregiver survey (WLQ)
 Caregiver non-healthcare sector costs	Surveys, assumptions based on interviews	
 Caregiver healthcare sector costs	Surveys, assumptions based on interviews, limited RWE	
 Broader spillover effects beyond primary caregiver	Surveys, proxy data (Gross) or algorithms (Jiao), assumptions based on interviews	

Jiao, B. and A. Basu, Associating Health-Related Quality-of-Life Score with Time Uses to Inform Productivity Measures in Cost-Effectiveness Analysis. *Pharmacoeconomics*, 2023. 41(9): p. 1065-1077.

Grosse SD, Krueger KV, Pike J. Estimated annual and lifetime labor productivity in the United States, 2016: implications for economic evaluations [published correction appears in *J Med Econ*. 2019 Jun;22(6):612. doi: 10.1080/13696998.2019.1584497.]. *J Med Econ*. 2019;22(6):501-508.

Framework from: Shafrin J, Kim J, Cohen JT, et al. Valuing the Societal Impact of Medicines and Other Health Technologies: A User Guide to Current Best Practices. *Forum Health Econ Policy*. 2024;27(1):29-116. Published 2024 Nov 8. doi:10.1515/fhep-2024-0014

Health State (Active Caregiving) ^{1,2}	Annual Utility Value	Difference from population norm
Not Sitting	0.48	-0.38
Sitting	0.63	-0.22
Walking	0.92	0.00
Permanent Ventilation	0.48	-0.38

Health State (Bereavement) ³	Annual Utility Value	Difference from population norm
Bereavement, Death <5 Years Past	0.64	-0.22
Bereavement, Death 5 - 10 Years Past	0.70	-0.14
Bereavement, Death +10 Years Past	0.73	-0.09

1. López-Bastida, J., et al., *Social/economic costs and health-related quality of life in patients with spinal muscular atrophy (SMA) in Spain*. Orphanet J Rare Dis, 2017. **12**(1): p. 141.

2. Pearson, S.D., et al., *The Effectiveness and Value of Treatments for Spinal Muscular Atrophy*. J Manag Care Spec Pharm, 2019. **25**(12): p. 1300-1306.

3. Lavelle et al. *In peer review: Value in Health; Mental Health, Quality of Life, and Work Functioning of Parents of Children Who Have Died from Spinal Muscular Atrophy*

Health State (Active Caregiving)^{1,2,3}

Annual Productivity Loss Costs

Not Sitting	\$40,644
Sitting	\$23,372
Walking	\$0
Permanent Ventilation	\$40,644

Health State (Bereavement)⁴

Annual Productivity Loss Costs

Bereavement, Death <5 Years Past	\$26,172
Bereavement, Death 5 - 10 Years Past	\$19,920
Bereavement, Death >10 Years Past	\$16,444

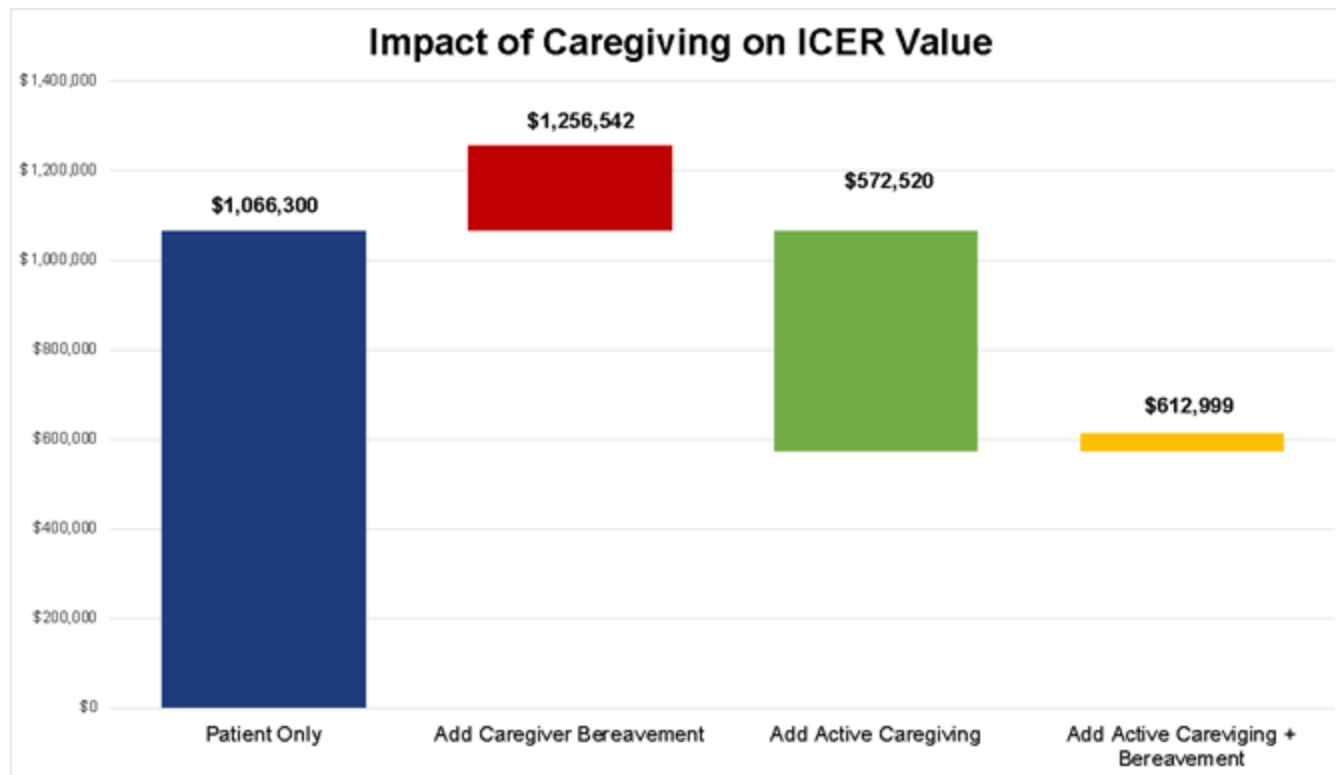
1. López-Bastida, J., et al., *Social/economic costs and health-related quality of life in patients with spinal muscular atrophy (SMA) in Spain*. Orphanet J Rare Dis, 2017. **12**(1): p. 141.

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4. Lavelle et al. *In peer review: Value in Health; Mental Health, Quality of Life, and Work Functioning of Parents of Children Who Have Died from Spinal Muscular Atrophy*

How did adding caregiving change the ICER?



What had the biggest impact?

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- HRQoL for active caregiving
- HRQoL during bereavement
- Productivity in active caregiving
- Productivity in bereavement

Impact of adding bereavement did not always increase the ICER

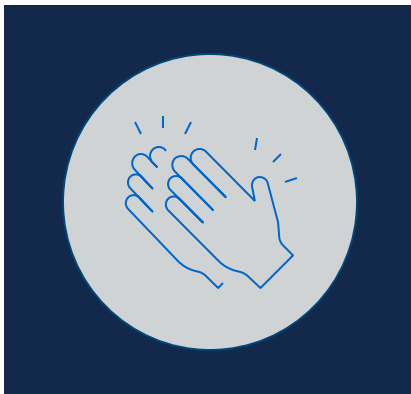
Scenarios where bereavement reduced ICER:

- Shorter duration of bereavement
- Higher treatment efficacy
- Higher career utilities

Depending on the disease and inputs, considering long-term caregiving impacts may increase, or decrease the ICER

It is uncertain whether CEA outputs can adequately capture the value of life extending therapies on caregivers given:

- Data gaps for important caregiving impacts
- Potential counterintuitive insights (carer QALY trap)



Recommendations

- Summarize impacts quantitatively within – and outside of – the model
- Don't minimize the impact of:
 - Non-quantitative impacts
 - Robust scenario analyses and pressure testing
 - Structured deliberation to better understand the full picture