Estimating health state utilities from a longitudinal SMA cohort

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Background

- Spinal muscular atrophy (SMA) is a rare neurodegenerative disease that impacts about 1 in 15,000 individuals.
- The past five years have yielded many important advances in care, treatment and lifespan in individuals living with spinal muscular atrophy (SMA).
- Despite improvements in research and innovation, it is important to understand outcomes that matter most to patients and ways to measure changes that can be used in clinical care and research settings.
- Health-related quality of life (HRQOL) instruments enable benchmarking and assessing changes across populations.
- One such instrument is the Health Utilities Index (HUI). The HUI is a family of generic health profiles and preference-based systems for the purposes of measuring health status, reporting health-related quality of life, and producing utility scores.[1]
- The HUI consists of two systems, the HUI2 and HUI3, which describe almost 1,000,000 unique health states. The HUI3 is the more detailed descriptive system and is the focus of this analysis.
- The purpose of this analysis is to evaluate factors that impact the HRQOL in individuals with SMA using the HUI3.

Methods

- Since 2017, Cure SMA, a patient advocacy organization that provides support and funding for care and treatment for those with spinal muscular atrophy (SMA), has hosted an annual community update survey (CUS) for caregivers and affected individuals.
- The CUS captures data such as demographics, treatment experience and health related quality of life measures, including the HUI.
- The CUS specifically captured the HUI3 in 2019 and 2021-2023.
- Scores on the HUI3 range from -0.36 (representing a health state worse than death) to 1 (representing perfect health).
- The HUI3 was self-completed by those over the age of 18 and completed by caregivers for those both under and over the age of 18.
- This study utilized a mixed-effects linear regression model to explore associations between HUI3 scores and multiple covariates in individuals diagnosed with SMA.
- Affected individuals that were younger than 5 at the time of the survey,[2] non U.S. residents, respondents affected with a non-5qSMA, and individuals that did not complete the HUI were removed from the analysis.

Results

- The final sample size included 1,089 surveys completed for 750 unique individuals.
- 510 individuals had completed only one CUS and 240 individuals has more than one CUS completed.

References

1. Horsman J, Furlong W, Feeny D, Torrance G. The Health Utilities Index (HUI): concepts, measurement properties and applications. Health Qual Life Outcomes. 2003 Oct 16;1:54.
2. The HUI only applies to those over the age of 5. See above reference.

Demographics

- **Table 1** describes the study sample by year of survey completion.
  - The majority of participants were female, had commercial insurance, 3 *SMN2* copies, and had been treated with a single FDA-approved treatment.
  - The mean age of the individuals at first survey completion was 28.9 (17.9) years and the mean age at diagnosis was 84.0 (147.9) months.

Table 1: Demographics of study sample by survey completion year					
	Total Unique Individuals[1]	2019	2021	2022	2023
n (%)	750	315 (28.9)	338 (31.0)	206 (18.9)	230 (21.1)
Male, n (%)	293 (39.1)	132 (41.9)	136 (40.2)	71 (34.5)	78 (33.9)
Age during survey in years, mean (sd)	28.9 (17.9)	25.3 (16.4)	27.6 (17.6)	36.6 (16.8)	37.8 (16.9)
Insurance, n (%)					
Public	303 (40.4)	115 (36.5)	146 (43.2)	78 (37.9)	97 (42.2)
Commercial	407 (54.3)	177 (56.2)	182 (53.9)	121 (58.7)	124 (53.9)
No insurance	7 (0.9)	0	7 (2.1)	2 (1.0)	2 (0.9)
Unknown	33 (4.4)	23 (7.3)	3 (0.9)	5 (2.4)	7 (3.0)
SMA Type, n (%)					
Type 1	88 (11.7)	40 (12.7)	49 (14.5)	11 (5.3)	12 (5.2)
Type 2	379 (50.5)	163 (51.8)	168 (49.7)	104 (50.5)	121 (52.6)
Type 3/4	283 (37.7)	112 (35.6)	121 (35.8)	91 (44.2)	97 (42.2)
SMN2 Copy Number, n (%)					
1-2 copies	141 (18.8)	53 (16.8)	69 (20.4)	28 (13.6)	35 (15.2)
3 copies	287 (38.3)	129 (41.0)	131 (38.8)	86 (41.8)	91 (39.6)
4-5 copies	109 (14.5)	51 (16.2)	54 (16.0)	39 (18.9)	43 (18.7)
Unknown	213 (28.4)	82 (26.0)	84 (24.9)	53 (25.7)	61 (26.5)
Age at diagnosis in months, mean (sd)	84.0 (147.9)	68.9 (121.0)	82.6 (155.9)	110.3 (179.9)	109.0 (160.7)
Treatment status with an SMA treatment[2], n (%)					
Unknown[3]	183 (24.4)	94 (29.8)	60 (17.8)	52 (25.2)	49 (21.3)
Mono	539 (71.9)	217 (68.9)	259 (76.6)	141 (68.5)	171 (74.4)
Combo	28 (3.7)	4 (1.3)	19 (5.6)	13 (6.3)	10 (4.4)

sd=Standard Deviation
[1] Data shown for unique individuals is based on the earliest survey completed
[2] SMA treatments included nusinersen, onasemnogene abeparvovec, and risdiplam
[3] Unknown treatment also includes those that reported never receiving and SMA treatment at time of the survey

Predictors of HUI3 scores

- **Table 2** shows the results from the mixed-effects linear regression model.
- Key findings from the model includes:

  SMA Type:

  Individuals with Type 2 or Type 3/4 have higher HUI3 scores than those with Type 1.

  Age during survey:

  There was an inverse relationship between the age of the affected individual and HUI3 scores indicating that HUI3 scores declined as the individual got older.

  Unknown/no SMA treatment:

  Individuals with an unknown or no SMA treatment history had lower HUI3 scores than those that had been treated with a single FDA approved SMA treatment. There was no difference in HUI3 scores among those that have received one or multiple SMA treatments.

Table 2. Predictors of Change in HUI3 Scores			
Independent Variables	Reference Category	Outcomes: Coefficients and p-values	
		Coef.	P>|z|
SMA Type			
Type 2	Type 1	0.19	**0.000**
Type 3/4	Type 1	0.40	**0.000**
Age at diagnosis, in months (continuous)		0.0003	**0.000**
Age during survey, in years (continuous)		-0.004	**0.000**
Insurance			
Commercial	Public	0.06	**0.000**
No insurance	Public	0.04	0.473
Unknown	Public	0.06	**0.023**
Treatment status with an FDA treatment			
Unknown	Mono	-0.03	**0.025**
Combo	Mono	0.04	0.149
Survey Year[1]			
2021	2019	-0.004	0.723
2022	2019	-0.0004	0.976
2023	2019	-0.03	**0.034**

- Survey year, age at diagnosis and insurance also were significant factors related to HUI3 scores although the overall impact on the HUI3 scores was minimal.

Conclusion

- This model underscores the influence of SMA type, treatment modality, age, insurance category, and survey year on HUI3 scores, providing valuable insights into factors impacting HRQOL in individuals with SMA.
- Although not assessed here, future analyses will evaluate how self and caregiver reported HUI# can impact overall scores.
- The model could be useful for estimating the value of different interventions and treatments used in SMA.

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