WILL NICE'S NEW SEVERITY WEIGHTING CRITERIA BE CAPABLE OF IDENTIFYING SEVERE CONDITIONS?

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BACKGROUND

- NICE's revised technology appraisal methods[1] include the introduction of QALY severity weights of 1.2 and 1.7, leading to potential upper cost-effectiveness thresholds of £35,000 and £50,000, respectively.
- Simply, QALYs are multiplied by the preferred weighting which leads to a reduced Incremental Cost-Effectiveness Ratio. In other words, the willingness-to-pay (WTP) threshold is, effectively, increased.

$$ICER_{weighted} = \frac{Costs_{New\ Treatment} - Costs_{Status\ Quo}}{[QALYs_{New\ Treatment} - QALYs_{Status\ Quo}] \times Severity\ Weighting}$$

- The weighting is calculated by assessing the absolute and proportional shortfall in discounted QALYs between people with the condition and the general population.
- The use of discounted QALYs in the severity calculation contrasts with the previous criteria for End-of-Life (EoL) QALY weighting, which were based on undiscounted life years, and the weighting applied in the Highly Specialised Technology (HST) process, which is determined by undiscounted QALY gains[2].

Table 1: A comparison of EoL, HST and severity modifier criteria. All weightings explicitly weight QALY gains, thereby implicitly increasing WTP

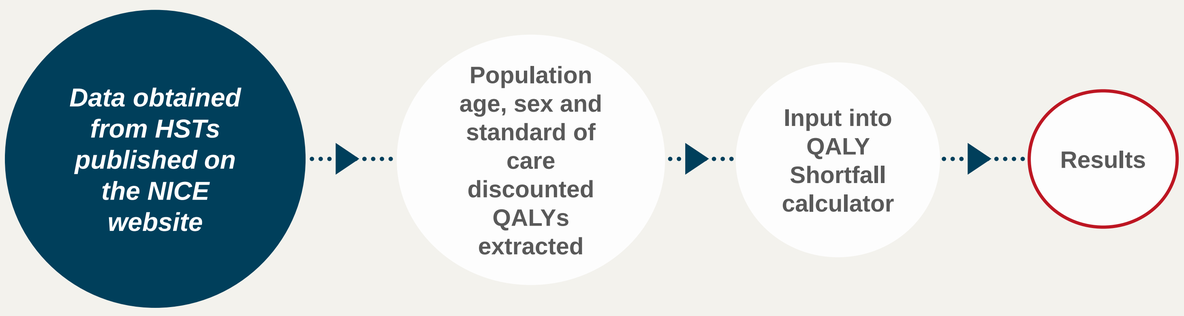
Comparative criteria	EOL		HST			Severity Modifier		
	No additional weight	High weight	No additional weight	Intermediate weight	Highest weight	No additional weight	Medium weight	High weight
Determinant	Undiscounted survival (LYs) delivered by current SoC and Incremental LYs delivered by intervention		Undiscounted incremental QALYs delivered by intervention over lifetime horizon			Discounted QALYs delivered by current standard of care over lifetime horizon		
Criteria	None	< 24 months life expectancy & treatment offers extension > 3 months	≤ 10 QALYs	11-29 QALYs	≥ 30 QALYs	Proportional shortfall < 0.85 Absolute shortfall < 12	Proportional shortfall = 0.85-0.95 Absolute shortfall = 12-18	Proportional shortfall ≥ 0.95 Absolute shortfall ≥ 18
QALY weighting	x1.0	x1.7	x1.0	x1.0-x3.0	x3.0	x1.0	x1.2	x1.7
Effective Threshold	£20,000-£30,000	£50,000	£100,000	£100,000-£300,000	£300,000	£20,000	£35,000	£50,000

OBJECTIVE(S)

- To assess the new NICE severity weight criteria by using the results of published HSTs as proxies for severe disease, since technologies can only be appraised via HST if the condition is considered "chronic and severely disabling".

METHODS

- Data were extracted from Highly Specialised Technology appraisals published on the NICE website.
- Where possible, data on the Evidence Review Group's preferred QALYs for the comparator arm, discount rate used, whether the QALYs included carer disutility, age and proportion female at model entry, time horizon, and whether a QALY weighting was allocated by the appraisal committee were collated.
- These data were then inputted into the ScHARR online R Shiny QALY shortfall calculator[3] and the severity weight was recorded.
- Thereafter, a review of the outcomes of these data was conducted. The interpretation of these findings is reported in the '*Results*' section.



DISCUSSION

- We were unable to calculate a QALY shortfall excluding carer QALYs for all HSTs used in this analysis due to the high frequency of redaction. As carer disutility would be expected to increase the shortfall, the proportions achieving the severity modifier criteria are potentially over-reported.
- Moreover, since undiscounted QALYs most often remain confidential, we were unable to produce a robust proportional or absolute shortfall analysis comparing the weighting results of discounted and undiscounted QALYs.

RESULTS

- 20 HSTs were analysed, of which 14 had sufficient data to calculate a severity weight.
- Of these, 7 (50%), 2 (14%) and 5 (36%) achieved a severity weight of 1.7, 1.2 and 1, respectively.

Table 2: A summary of the distribution of data for age at model entry, the proportion of females, the discount rate, discounted QALY gains, whether a weighting was allocated at HST and, lastly, the severity weight calculated using these data

Highly Specialised Technology Appraisal (HST)	Condition	Age at model entry	% Female	Discount rate	Discounted QALYs (Status Quo)	Allocated QALY weighting in HST	Severity Weighting
HST18	Metachromatic leukodystrophy	4	51%	1.5%	-3.97	✓	1.7
HST16	Acute hepatic porphyria	41.6	86%	3.5%	4.04	✓	1.2
HST15	Spinal muscular atrophy	0	53%	3.5%	0.21	✓	1.7
HST12	Neuronal ceroid lipofuscinosis type II	4.78	50%	3.5%	-1.3	✓	1.7
HST11	Inherited retinal dystrophies caused by RPE65 gene mutations	15.1	58%	3.5%	3.64	✓	1.7
HST10	Hereditary transthyretin amyloidosis	59	29.5%	3.5%	0.32	✗	1.7
HST8	X-linked hypophosphataemia	6.5	49.2%	3.5%	16.18	✓	1
HST7	Severe combined immunodeficiency caused by adenosine deaminase deficiency	1	50%	3.5%	12.1	NR	1
HST6	Paediatric-onset Hypophosphatasia	0	46.7%	3.5%	4.62	NR	1.7
HST5	Type I Gaucher disease	35	60%	3.5%	12.71	NR	1
HST4	Fabry disease	48	50%	3.5%	10.66	NR	1
HST3	Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene	8.5	0%	3.5%	3.8	NR	1.7
HST2	Mucopolysaccharidosis type IV-A	14.5	52.9%	3.5%	7.67	✓	1.2
HST1	Atypical haemolytic uraemic syndrome	28	65%	3.5%	11.69	NR	1

NR: Not reported

Figure 1 A summary of the proportions that achieved the shortfall thresholds and severity weightings that were assigned based on the HSTs analysed

CONCLUSIONS

- One-third of conditions previously considered severe by NICE may not be allocated a QALY weighting using the new severity weighting criteria. This includes conditions previously allocated weights under HST weighting criteria, underpinned by gains of >10 undiscounted QALYs vs. current standard of care,.
- Based on the reasonable assumption that the validity of models was appraised by NICE, our findings suggest that the new severity weighting approach may be unable to consistently identify severe diseases.
- We believe that a key determinant of the above is largely due to the use of discounted rather than undiscounted QALYs in assessing QALY shortfall, and that there is a need to reconsider how QALYs are valued in shortfall analyses.

REFERENCES

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2. NICE Highly Specialised Technology Appraisals: Published guidance, NICE advice and quality standards | Guidance | NICE
3. Schneider P., McNamara S., Love-Koh J., Doran T., Gutacker N. QALY Shortfall Calculator. 2021. https://r4scharr.shinyapps.io/shortfall/