

A VIGNETTE STUDY TO DERIVE HEALTH STATE UTILITIES FOR AROMATIC L-AMINO ACID DECARBOXYLASE (AADC) DEFICIENCY IN THE UNITED KINGDOM (UK)

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Background: Aromatic L-amino acid decarboxylase (AADC) deficiency is a rare genetic, lifelong condition predominantly manifesting in young infants. Common symptoms are hypotonia, developmental delay, movement disorders, including oculogyric crises. Given the nature and rarity of the condition it is not possible to directly capture health-related quality of life (HRQoL). Therefore, other approaches, such as vignette studies, are required to elicit health utilities for cost-effectiveness evaluations of interventions. The aim of this study was to generate health state utilities for AADC deficiency.

Methods: General population participants were presented with a total of 5 health state vignettes: bedridden, head control, sitting unsupported, standing with assistance and walking with assistance. These had been previously developed based on published literature, clinician input, parent interviews and expert opinion. Health state utilities were elicited using time-trade off (TTO; 10-year time horizon) and the standard gamble (SG). The vignettes were completed online by panel participants drawn from a representative sample of the United Kingdom population.

Results: A total of 1598 participants completed the vignettes. Utilities increased linearly as health states improved for both the TTO and SG, although 37% had incongruent responses (high utilities for the bedridden compared to walking health states). When these were removed from the analysis the mean health utilities (standard deviation) for the TTO were: bedridden state 0.42 (± 0.32); head control 0.48 (± 0.32), sitting unsupported 0.58 (± 0.31); standing with assistance 0.63 (± 0.32); and walking with assistance 0.67 (± 0.33). For the SG, mean utilities were: 0.58 (± 0.27), 0.59 (± 0.27), 0.69 (± 0.24), 0.73 (± 0.22), and 0.79 (± 0.20), respectively. Females had higher utility values compared to males (range: 0.44 to 0.69; 0.39 to 0.64 respectively).

Conclusion: Health state utilities were derived for AADC deficiency through a vignette study. These will be used for a cost-effectiveness model of an AADC deficiency treatment.