A DISCRETE CHOICE EXPERIMENT TO DERIVE HEALTH STATE UTILITIES FOR AROMATIC L-AMINO ACID DECARBOXYLASE (AADCd) IN THE UNITED KINGDOM

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Background: Deriving health utilities for ultra-rare medical conditions such as aromatic L-amino acid decarboxylase deficiency (AADCd) poses challenges. The rarity of AADCd and the fact that this genetic condition is predominantly manifested in infants means that robust utility values cannot be derived from the child or their parent/caregiver. Alternative approaches, e.g. discrete choice experiments (DCE), are required in order to provide health utilities for cost-effectiveness evaluations of AADCd interventions. The study aim was to generate health utilities for AADCd using a DCE.

Methods: An orthogonal design was created (NGene). The DCE comprised 6 key AADCd attributes (2-6 levels): mobility, muscle weakness, oculogyric crises, feeding ability, cognitive impairment and screaming. These had been identified from published literature, clinician input, parent interviews and expert opinion. Participants were presented with 10 choice sets, including 1 with reversed levels to evaluate choice consistency. Participants were presented with 5 health state vignettes prior to the DCE. These were used to elicit utilities using time-tradeoff. The utilities for the worst/best health states were used as anchors to convert indirect DCE part-worth utilities to health utilities. Multinomial logit models were estimated (NLogit6). The DCE was completed online by panel participants from a UK representative sample.

Results: A total of 1596 participants completed the DCE. The majority (70.7%) gave consistent responses to the repeated choice task; only 1.7% (27) always chose the same alternative for every choice set. Five models were evaluated. There was one preference reversal ("sitting unaided"/"standing with assistance") occurring in all models; these 2 mobility level coefficients were set to be equal in the final model. Rescaled utilities ranged from 0.4217 to 0.6703, corresponding to the worst (633233) and best (111111) health states.

Conclusion: Health utilities were derived for AADCd through a DCE. These will be used for a cost-effectiveness model of an AADCd treatment.