

## **VALIDATING VIGNETTES FOR A RARE DISEASE USING CLINICIAN INTERVIEWS TO EVALUATE THE IMPACT ON HEALTH-RELATED QUALITY OF LIFE IN AROMATIC L-AMINO ACID DECARBOXYLASE (AADC) DEFICIENCY IN FRANCE**

Adam B. Smith, Andria Hanbury, Igor Beitia, Florence Hammes, Gérard de Pouvourville, Katharina Buesch

**Background:** In the absence of utilities from clinical trials, health related quality of life (HRQoL) can be estimated using health utilities derived through vignette studies and discrete choice experiments (DCE). This enables treatment evaluation in cost utility analysis for HTA assessments. Vignettes should have face and content validity; obtaining input into the design from caregivers/parents of aromatic l-amino acid decarboxylase (AADC) deficiency patients, and clinicians treating these patients is, thus, critical. The study objective was to validate the vignettes and DCE attributes already developed and validated for the UK—this time specifically for France. These will be used in a subsequent study to estimate patient HRQoL in France.

**Methods:** Initial vignettes were developed based on a literature review, interactions with clinicians, as well as parents via interviews, advisory boards and anonymised surveys. To capture improvements on therapy, input into key DCE attributes were also obtained. The health states were bedridden, head control, sitting unsupported, standing with assistance, and walking with assistance aligned to major motor developmental milestones and outcomes in the clinical trials. The vignettes and DCE attributes were translated including full reconciliation. In a second step, the French translation as well as the content of the vignettes were validated using 4 French clinicians' input.

**Results:** The translation process resulted in minor amends to the vignette wordings for each health state. The French clinicians supported the health state descriptions with 6 suggested changes including the addition of 'tiredness' as a symptom to two health states and minor revisions to the language translation.

**Conclusion:** While this project supported the face and content validity of the vignettes and attributes previously developed for the UK, it also showed that it is important to validate with local experts the language when planning to derive utilities for a cost-effectiveness model of an AADC-d treatment in a specific country.