Severity Index for Children With Developmental Epileptic Encephalopathy and Quality of Life of Carers

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ISPOR AP Summit 2026
6-8 Sep 2026 | Bangkok

Poster Code: MSR17
Monday, 7 September, 12:15 - 16:15

Background

There are several different rare developmental and epileptic encephalopathies (DEE) for children, such as Dravet syndrome, and CDKL5 deficiency disorder. Incidence estimates for Dravet syndrome have ranged from 1 in 15,700 births in the USA to 1 in 40,000 in Europe, and CDKL5 DEE is estimated at between 1 in 40,000 and 1 in 60,000 live births. This means that DEE are typically categorised as rare diseases. Carers living with children who are suffering from Developmental Epileptic Encephalopathy (DEE) have experienced symptoms broadly categorised into epilepsy, cognitive and developmental delay, and orthopaedic and movement disorders, with higher chances of experiencing certain comorbidities (i.e., sleep impairment, infection, blood abnormalities, and psychiatric disorders) and sudden unexpected death in epilepsy. The unique nature of paediatric conditions places a burden on caregivers and family, predominantly affecting emotional, employment, and leisure time..

Objective

To develop a latent DEE severity indicator and examine its association with carers' EQ-5D-5L health-related quality-of-life utility.

Methods

To develop a latent severity indicator, we first selected five clinical variables: patient age, age at first seizure onset, seizure frequency, current treatments, and previous treatments. The mathematical expression for the categorical outcome was constructed as follows. We applied Principal Component Analysis (PCA) on set of X_i. Two orthogonal principal components were selected based on the eigenvalue criterion (eigenvalues greater than 1). Mathematically, the principal component scores for individual *i* are given by:$PC_i = W^{'}X_i$ where, W is the matrix of eigenvectors corresponding to the selected components. To identify the underling subgroups of DEE severity, we conducted Latent Class Analysis (LCA) maximises the following likelihood function with two ordered categorical variables (PC_{i1}^{cat} and PC_{i2}^{cat}) derived from PC scores.

$$\max(\theta)\prod_{i=1}^{n}\sum_{k=1}^{K}\pi_k\prod_{j=1}^{2}P\left(PC_{ij}^{cat}\,|Class\ k,\theta\right)$$

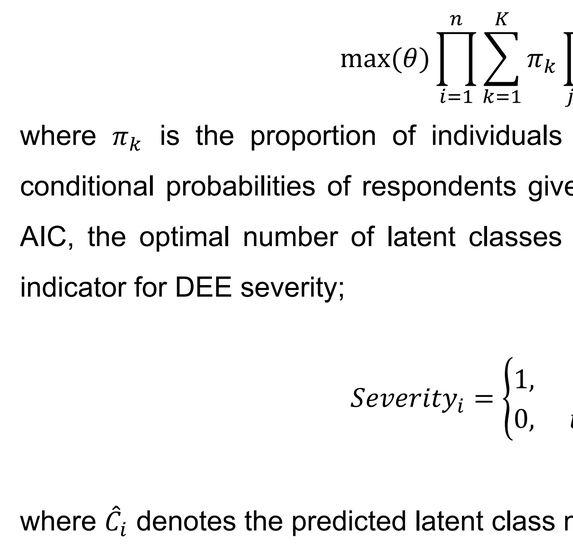
where π_k is the proportion of individuals in the latent class *k* and *P(.)* represents the conditional probabilities of respondents given latent class membership. Based on BIC and AIC, the optimal number of latent classes is two, leading to the identification of a binary indicator for DEE severity;

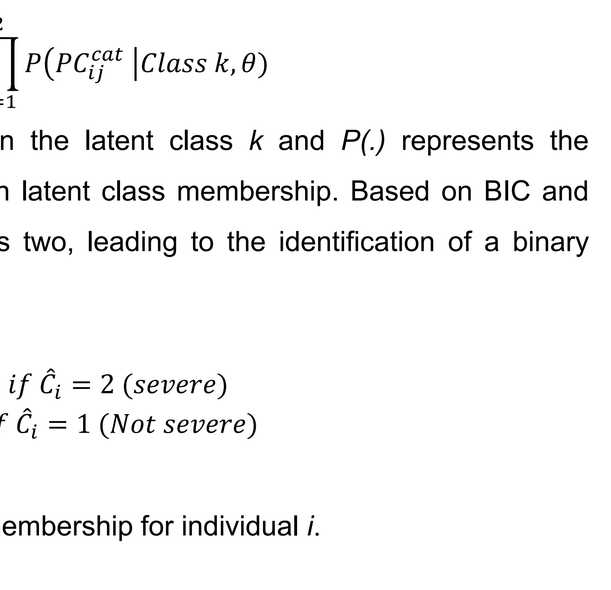
$$Severity_i = \begin{cases} 1, & if\ \hat{C}_i = 2\ (severe) \\ 0, & if\ \hat{C}_i = 1\ (Not\ severe) \end{cases}$$

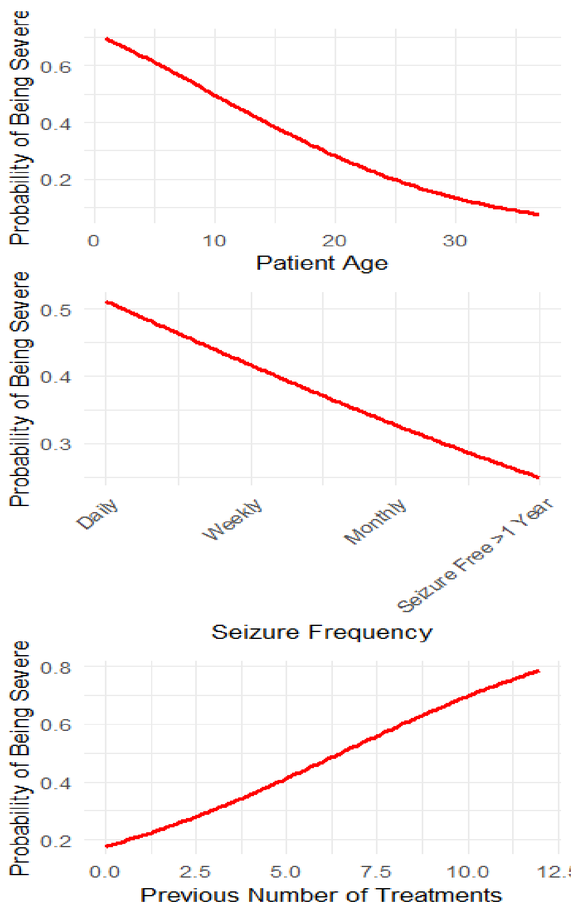
where $\hat{C}_i$ denotes the predicted latent class membership for individual *i*.

Results

Patients aged over 50 years were excluded (1 observation). PCA show that the first two components (explaining 62% of the variance) were used in the LCA. Based on BIC and AIC, a two-class model was selected, classifying individuals into “Severe” and “Not Severe” groups. Of the five estimated PCA components, two explained 62% of the variability (see figures below).



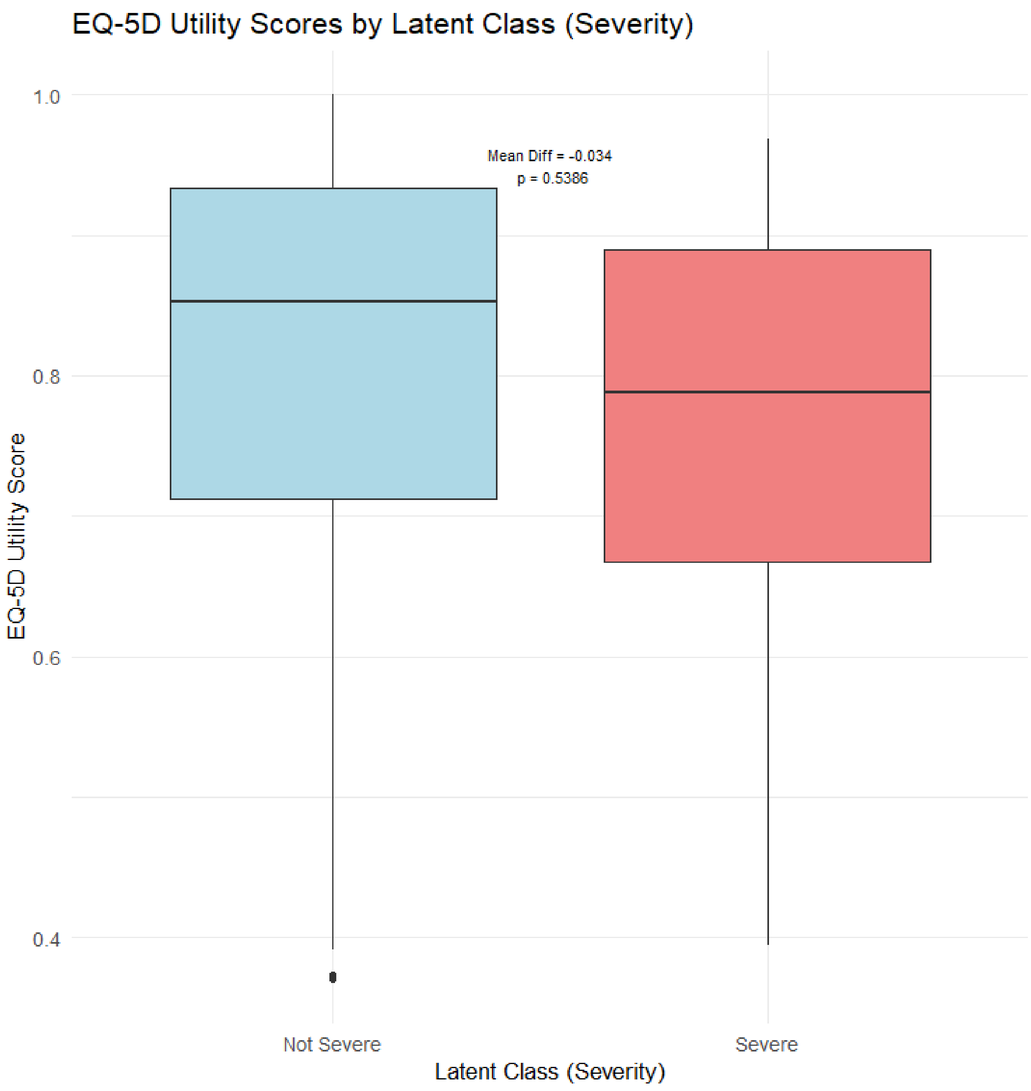




Patients classified within the severe disease class were characterised by a distinct clinical profile indicative of greater treatment burden and disease complexity. Higher predicted severity was associated with younger age, earlier seizure onset (before 1 year of age), and high seizure frequency, particularly weekly or daily seizures. Individuals in this class were also more likely to be receiving multiple concurrent antiseizure medications, with four or more current therapies, and to have an extensive history of medication trials, having previously used more than seven antiseizure medications. Collectively, these features reflect a population with early-onset, highly active, and treatment-resistant epilepsy.

Carer health utility

Reported Mean EQ-5D-5L Not severe 0.79 (0.20) vs severe: 0.75 (0.16). Mean difference: -0.034; p=0.539.



Conclusion

This study presents the first severity index for Developmental and Epileptic Encephalopathy and demonstrates that carers for children with severe disease rate their quality of life 4.27% lower than carers for children with non-severe disease.

AIC, Akaike information criterion; BIC, Bayesian information criterion; DEE, Developmental Epileptic Encephalopathy; EQ-5D-5L, EuroQol 5-Dimension 5-Level; LCA, latent class analysis; PCA, principal component analysis.