Use and Validity of Patient-Reported Outcomes in Schizophrenia Trials

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What is the Methodological Question Being Addressed? This study seeks to understand how often patient-reported outcomes (PROs) are used in Schizophrenia clinical trials and determine if they are consistent with results obtained from clinician-reported outcomes.

Introduction The FDA has been encouraging the use of PROs in clinical trials for many years to augment data from clinician-reported outcomes (ClinROs). PROs have been used to a lesser degree in trials with the severely mentally ill populations due to concerns about deficits in general cognition and insight. We believe that evaluation of the patient's perspective and experience with regard to quality of life, symptom status and healthcare utilization is crucial in Schizophrenia for ascertaining the full picture of treatment efficacy.
In this study we reviewed the depth of PRO instrument use in a sample of clinical trials. We then analysed correlations between two commonly used ClinROs - the Personal and Social Performance Scale (PSP), the Positive And Negative Syndrome Scale (PANSS) and the Quality of Life (QOL) measure EQ-5D-5L, also routinely used for health-economic modelling.

Methods We performed a search of 15 recent Schizophrenia studies. A total of 596 baseline visits in two clinical trials and 5 countries was analysed. Spearman correlations were calculated between individual EQ-5D-5L dimension and VAS score and PSP and PANSS total scores. Level of significance was set at $p < 0.05$.

Results In the 15 schizophrenia trials we reviewed, only 2 trials were identified using the EQ-5D-5L to evaluate QoL, while the 2 other PROs were symptom specific.
Analysis of data from 596 baseline visits resulted in the finding of very weak correlations. For PSP and EQ-5D-5L the Spearman's correlations were very weak ($r = -0.109$ to 0.024 for the EQ-5D-5L dimensions anxiety and depression, mobility, pain and discomfort, and usual activities; $p > 0.01$). Of note, only self-care yielded significant relationships ($p < 0.05$).
A similar trend was observed in the relationship between PANSS total and EQ-5D-5L dimensions. Spearman's correlations ranged from 0.048 to 0.182 for the EQ-5D-5L dimensions. Three dimensions (anxiety and depression, self-care and pain and discomfort) yielded significant relationships ($p < 0.05$), whereas the others did not ($p > 0.05$).
The EQ-5D VAS score showed very weak, but non-significant, correlations with the PSP and PANSS total score (PSP $r = 0.036$; PANSS: $r = - 0.069$; $p > 0.05$).

Conclusion The appropriateness of the EQ-5D-5L for people with schizophrenia has been contested. Here, we demonstrate that the use of the EQ-5D-5L is uncommon in Schizophrenia trials, and most dimensions and VAS scores show no statistically significant relationship with two of the gold standard instruments used in Schizophrenia trials, the PSP and the PANSS, potentially driven by the EQ-5Ds focus on physical functioning rather than mental health.
This study highlights the need to select more appropriate or even develop and validate QOL PROs for use in Schizophrenia trials to add the patients voice and their quality of life to the investigational product efficacy evaluation process as well as health-economic modelling.

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Keywords

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