

Exploratory psychometric analysis of the EQ-5D in a Myelofibrosis population

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INTRODUCTION

The EQ-5D is a generic preference-based measure of health-related quality of life (HRQoL) applicable to a wide range of health conditions and treatments.¹ Myelofibrosis (MF) is a rare but serious bone-marrow cancer in which the proliferation of an abnormal type of bone marrow stem cell results in fibrosis, or the replacement of the marrow with collagenous connective tissue fibres. Massive spleen enlargement is a consequence of the resulting extra-medullary haematopoiesis and can cause symptoms (typically abdominal pain, early satiety and difficulty in breathing); and can lead to complications such as portal hypertension, splenic infarction and vascular events. Patients commonly have symptoms such as fever, night sweats, and weight loss.

There is no evidence about the appropriateness of the EQ-5D in MF; thus, this study aimed to provide psychometric evidence of its appropriateness.

METHODS

The psychometric performance of the EQ-5D was assessed relative to that of a condition-specific measure, the modified MF Symptom Assessment Form (MF-SAF) version 2.0 using data from the ROBUST UK study.

The ROBUST UK² study collected data on both EQ-5D (5 levels) and modified MF-SAF v2.0 in a small sample of UK patients (n=48) with MF, post-polycythemia vera myelofibrosis (PPV-MF), or post-essential thrombocythemia myelofibrosis (PET-MF) with repeated measurement over 48 weeks (baseline, week 4, 12, 24 and 48 weeks).

- The EQ-5D (5 levels) classification consists of five dimensions (mobility, self-care, usual activities, pain/discomfort, and anxiety/depression), each with five levels of response (no problems, slight problems, moderate problems, severe problems, and extreme problems). A single summary score is generated and range from 1 to -0.594 with 1 representing full health and 0 for dead.
- The MF-SAF v2.0³ has seven symptoms (abdominal discomfort, pain under left ribs, early satiety, night sweats, itching, bone or muscle pain and inactivity) each scored from 0 (absent) to 10 (worst imaginable). A total symptom score (TSS) is generated from the first 6 symptoms.

In order for the EQ-5D to be appropriate, the measure should be associated with key symptoms in MF captured by the MF-SAF, at both baseline and over time.

Analysis consisted of:

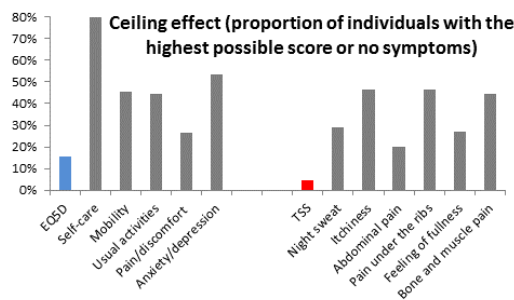
- Convergent validity.** Convergent validity was assessed by examining how well the EQ-5D preference-based measure and health state classification dimensions were associated with the MF-SAF total score and individual symptoms. Pearson's correlations were used for continuous variables (e.g preference based and total clinical score); whilst spearman rank correlation was used for dimensions. Correlations were analysed using data at baseline only. The following cut-offs are used to determine the strength of the association: very strong >|0.6|, strong: <|0.6 to ≥ 0.5|, moderate: <|0.5 to ≥ 0.3| and weak: <|0.3|. Given the small sample size, further assessment of convergent validity was undertaken using data from all the time points. To account for repeated measures from the same individuals, this was examined using a mixed effect model using the EQ-5D preference based as the dependent variable and MF-SAF individual symptoms as covariates. For a symptom to be considered associated with the EQ-5D, the regression coefficient needs to be significant and negative and larger coefficients indicate stronger relationship.
- Responsiveness.** Responsiveness assesses how well the EQ-5D captures changes in health compared to the MF-SAF. The proportion of participants at the floor and/or ceiling on both the EQ-5D and MF-SAF was compared as large proportions at either end of the scale means the measure cannot assess deterioration or improvement. In the absence of established cut-offs, a cut off of less than 10% at the floor or ceiling was considered acceptable. The magnitude of the change between the time points was assessed using the standardised response mean (SRM), calculated by dividing the mean change on the measure by the standard deviation of the change. The number of patients with both data at baseline and follow-up available for analysis reduced with time. The following cut-offs were used to categorise the SRMs: $|\geq 0.2$ to $< 0.5|$, $|\geq 0.5$ to $< 0.8|$ and $|\geq 0.8|$ denote small, medium and large SRMs respectively.

RESULTS

Convergence validity:

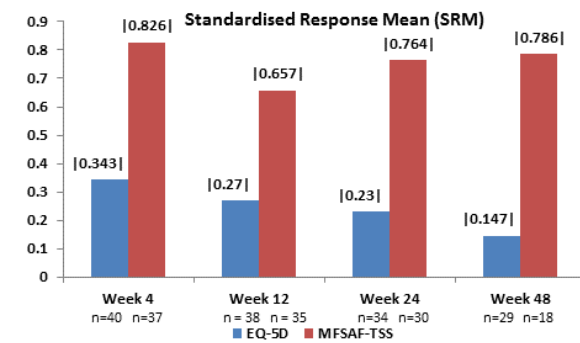
- At baseline, the correlation between the EQ-5D preference based and the MF-SAF Total score was strong ($<|0.6$ to $\geq 0.5|$). However,
- The correlation between the EQ-5D preference-based (and all EQ-5D health dimensions) and night sweat and itchiness was weak ($<|0.3|$).
- The correlation of the EQ-5D health dimensions with other symptoms from the MF-SAF were weak to moderate ($<|0.5|$) with the exception of the correlation between the EQ-5D pain/discomfort health dimension and abdominal discomfort and EQ-5D usual activities health dimension with feeling of fullness.
- When accounting for repeated measurement, only feeling of fullness and bone and muscle pain were significantly ($p < 0.05$) associated with the EQ-5D preference based.

Ceiling effects: The MF-SAF total score did not show comparable ceiling effect (4.76% with a score of 0; n=2) compared with the EQ-5D preference based (15.56%; n=7).



Responsiveness: The SRM for the EQ-5D preference based was small ($<|0.5|$).

In comparison the SRM for the MF-SAF total score was large ($>|0.8|$) at 4 weeks indicating that participants had large improvement in MF key symptoms at week 4.



CONCLUSION

- This exploratory analysis suggests that the EQ-5D preference based and health dimensions had poor association with key symptoms in MF, except for the 'pain/discomfort' and 'anxiety/depression' health dimensions in some respect.
- Although the EQ-5D captured some changes, these changes were much smaller than when assessed using the MF-SAF.
- This exploratory analysis suggests that the EQ-5D measures' ability to capture the effect of key symptoms in MF is limited to pain rather than the specific MF symptoms such as night sweat, itchiness.
- However, results of this analysis need to be interpreted with caution due to the small number of patients and the potentially non-representative nature of the sample.

References

- www.euroqol.org
- Mead et al. 2014 abstract presented at EHA, 15-19 June 2014.

3 Mesa et al. 2009 *Leukemia research*, 33, (9) 1199-1203)



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