The voice of patients with epidermolysis Bullosa: Redefining the latent construct and scope of PROMs in Health-Related Quality of Life.

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Background

Epidermolysis bullosa (EB) is a rare disease that has a significant impact on Health-Related Quality of Life (HRQoL)

Objective

To assess the scope and latent structure of Patient-Reported Outcome Measures (PROMs) in EB

Methods

- Based on the COSMIN methodology, the precision of the HRQoL instruments was evaluated
- The research focused on the scope of the PROMs previously identified in a scoping review
- Three fundamental aspects were peer-reviewed: i) the construct; ii) the target population and respondents; and iii) the context of use
- The model underlying the latent structure of these PROMs was also evaluated

Results

Two PROMs were identified:

- QoLEB (Frew et al., 2009)
- InToDermQoL-EB (Chernyshov et al., 2019)

	Construct	Target population	Respondents	Context of use	Observations: Structure
QoLEB	QoL	Adults and children over 8 years of age with EB	Self-reported by patients	Clinical studies and monitoring the effects of treatments	Reflective model (with 2 subscales)
InToDermQoL-EB	QoL	Children aged 0 to 4 years with EB	Parents and caregivers	Clinical practice and clinical trials	Formative model (structured into 3 age cohorts).

Conclusion

Both the QoLEB (adults and young people) and the InToDermQoL-EB (children 0-4 years) have adequate scope according to the COSMIN methodology and are aligned with the underlying construct, the target population, and the context of use. This opens the possibility of further analyzing other measurement properties, taking into account that they are reflective and formative models, respectively.

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