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UTILITY ELICITATION IN EPIDERMOLYSIS BULLOSA: CROSS SECTIONAL STUDY

Context

Epidermolysis Bullosa (EB) is a group of rare genetic skin conditions that cause the skin to blister and tear[1].

Current standard of care is highly varied and includes the use of emollients, management of pain, daily wound care, and protective bandaging. Therapeutic options are urgently needed to assist in reducing the number of wounds and the severity of those wounds[2].

Accurate estimates of health related quality of life (HRQoL) associated with different EB management strategies are needed to better inform resource allocation.

Methods

Type of study design: Cross-Sectional Study
Countries: USA, UK & Republic of Ireland (ROI)
Eligibility: Individuals with DEB, JEB or Kindler EB and their carers
Data collection:

- Electronic survey (via self-report or proxy)
- Questions upon demographics; clinical (health) impact and disease & treatment management
- Period: September 2021 to February 2022

Measures of HRQoL & health status collected:

EQ-5D-5L[3] & EQ-VAS[3] | CHU9D* [4] | iscorEB** [5] | CarerQol -7D[6] & CarerQOL VAS [6]

*The domain 'Schoolwork/homework' was altered to 'Work' for 18+ to enable completion by the full sample
**For the iscorEB only the patient/family section was completed to provide a sub-score

Data analysis:

- Data was summarised descriptively; continuous variables being presented alongside the mean and standard deviation (SD) and categoric variables alongside the sample size (n) and percentage
- Analysis of HRQoL by self-reported disease severity groups was split into mild, moderate and severe for patients & caregivers completes
- Analysis of HRQoL by self-reported body surface area percentage (BSAP) effected which was split into six groups (<=4, >4 to <=7, >7 to <=10, >10 to <=18, >18 to <=24, >24) for patient completes
- Associations were determined using scatter plots with linear prediction lines

Results

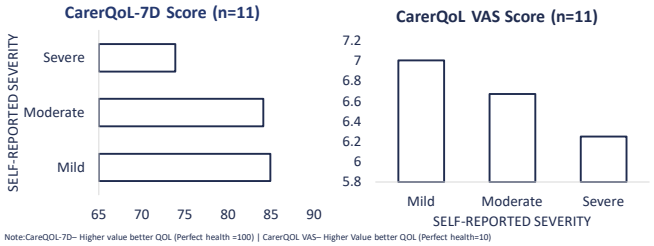
Patients Recruited: n= 67
USA: n=56 UK: n=9 ROI: n=2

Caregiver Recruited: n=11
USA: n=10 UK: n=1 ROI: n=0

Patient Characteristics

Characteristics		Patient Complete (n=67)	Caregiver Complete (n=11)
Gender n (%)	*Male*	38 (56.72)	2 (18.18)
	Female	29 (43.28)	9 (81.82)
EB Type n (%)	*Dominant, dystrophic*	30 (44.78)	0 (0)
	Recessive, dystrophic	30 (44.78)	3 (27.27)
	Junctional	6 (8.96)	8 (72.73)
	Kindler	1 (1.49)	0 (0)
Severity n (%)	*Mild*	18 (26.87)	1 (9.09)
	Moderate	39 (58.21)	6 (54.55)
	Severe	10 (14.93)	4 (36.36)
Age Mean (SD)		26.22 (9.00)	7.91 (5.07)

Mean utility score for caregivers by EB self-reported severity



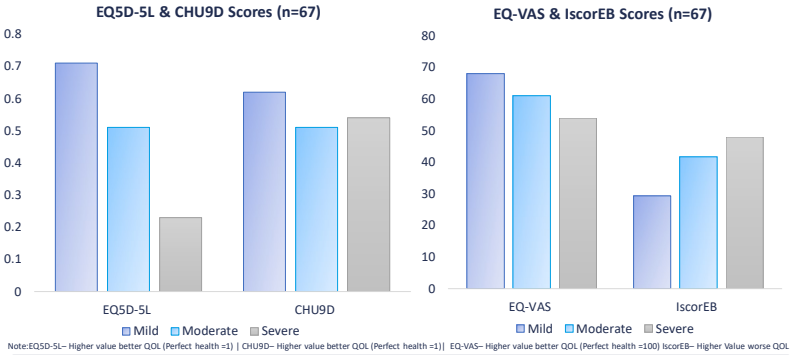
Note:CareQOL-7D– Higher value better QOL (Perfect health =100) | CarerQOL VAS– Higher Value better QOL (Perfect health=10)

Aim

To elicit the HRQoL from EB patients and their caregivers using validated generic and disease specific instruments. Explore the relationship between HRQoL and disease severity from EB patients and their caregivers.

Results

Mean utility score for patients by EB self-reported severity



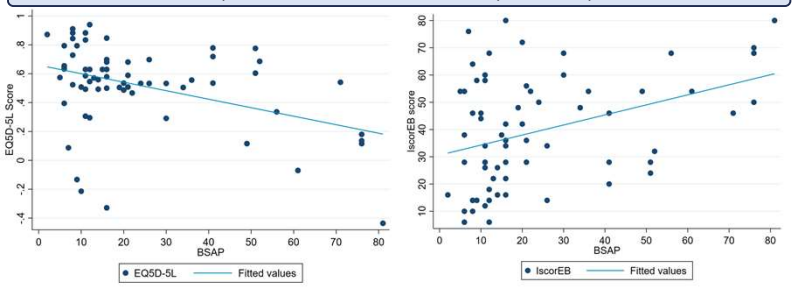
Note:EQ5D-5L– Higher value better QOL (Perfect health =1) | CHU9D– Higher value better QOL (Perfect health =1)| EQ-VAS– Higher value better QOL (Perfect health =100) IscorEB– Higher Value worse QOL

Mean utility score for patients by self reported BSAP group

QoL Instrument	BSAP %					
	<4 Mean (n, SD)	5-7 Mean (n, SD)	8-10 Mean (n, SD)	11-18 Mean (n, SD)	19-24 Mean (n, SD)	25+ Mean (n, SD)
Patient						
EQ5D-5L	0.87 (1, NA)	0.54 (7, 0.23)	0.54 (9, 0.43)	0.59 (22, 0.27)	0.54 (8, 0.07)	0.41 (20, 0.32)
EQ-VAS	80 (1, NA)	59 (7, 20.89)	63 (9, 24.04)	71 (22. 17.57)	54 (8, 14.49)	55 (20, 20.84)
CHU9D	0.77 (1, NA)	0.57 (7, 0.16)	0.59 (9, 0.14)	0.55 (22, 0.12)	0.49 (8, 0.07)	0.53 (20, 0.17)
iScorEB	16 (1, NA)	38 (7, 25.38)	34.44 (9, 21.33)	32.37 (22, 19.08)	48.25 (8, 13.41)	47.30 (20, 18.82)

Note:EQ5D-5L– Higher value better QOL (Perfect health =1) | CHU9D– Higher value better QOL (Perfect health =1)| EQ-VAS– Higher value better QOL (Perfect health =100) IscorEB– Higher Value worse QOL

Scatter plot of BSAP & EQ5D-5L/IscorEB for patient completes



Note:EQ5D-5L– Higher value better QOL (Perfect health =1) | IscorEB– Higher Value worse QOL

Conclusions

Patient

An association was observed between increasing disease burden (in terms of self-reported severity and BSAP) and greater disutility as measured using the EQ5D-5L and EQ-VAS. However, no clear association was observed between increasing disease burden (in terms of self-reported severity and BSAP) using the CHU9D. An association was observed between increasing disease burden (in terms of self-reported severity and BSAP) and higher iscorEB scores.

Caregiver

The observed trend for the CarerQOL-7D showed carers of more severely impacted patients had lower scores in comparison to those caring for patients in mild or moderate severity groups. The CarerQOL VAS also showed this association but with carers of mild patients having a higher score than those of carers with moderate patients.

Limitations

The data in this study was self-reported. Disease severity and BSAP effected were determined by the respondent and may differ from that assessed by a clinician. The sample was largely US based, with a large difference in size between patient and caregiver subgroups.

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