

EP0645 - The 2023 EB Insights Study; A large comprehensive patient-centric research study to qualify and quantify the impact of epidermolysis bullosa, in the UK

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Introduction & Objective

- Epidermolysis bullosa (EB) is a complex group of lifelong, rare, genetic disorders which cause the skin to become very fragile, and tear or blister, as a result of minor trauma. EB subtypes include EB simplex (EBS), dystrophic EB (DEB), junctional EB (JEB), and Kindler EB.[1]
- Patients have an altered wound healing profile, and can have a high wound burden, as well as debilitating symptoms such as pain and pruritis, and additional systemic complications which can carry considerable morbidity and increased mortality risk.[2-4]
- There is no curative treatment for EB; the mainstay of treatment is wound management.[5]
- The 2023 EB Insights study was commissioned by DEBRA UK, the national charity and patient support organisation for people living with EB.
- The objective was to provide a foundation of data for patient advocacy groups and to support pharmaceutical companies in evidence generation needs for health technology assessment of new EB therapies.

Methods

- Data were collected on the impact of EB on daily life, healthcare resource use (HCRU), and health-related quality of life (HRQoL) of patients and their carers/ families. The study overview is depicted in (Figure 1).
- Part 1 consisted of depth qualitative interviews with n=24 patients/ carers, and n=9 EB specialists. These were followed by 20-minute online surveys of EB patients and carers (Part 2), and 15-minute online surveys of GPs and dermatologists (Part 3).
- The results of the Part 2 and Part 3 surveys are discussed here.
- Survey questions covered the following topics: what it is like to live with EB, symptom management, impact on daily living, aspirations for the future in terms of healthcare and treatment, awareness of the condition, and barriers to optimal EB patient care.

Results

- 215 patients, 103 carers, 50 dermatologists, and 100 GPs were interviewed between April and May 2023.
- The patient and carer respondents represented a broad demographic, encompassing different EB subtypes, (61% EBS, 31% DEB, 4% JEB, 2% Kindler EB), genders (patient: 70% female, 29% male, <1% non-binary; carer: 81% female, 18% male, <1% non-binary), ethnicities (92% white), and ages (25% children, 75% adult) (Figure 2).
- Dermatologist respondents included both consultants (88%) and specialist registrars (12%); 74% worked in a University Teaching Hospital and 26% worked in a non-University Teaching Hospital. None of the dermatologists worked at an EB specialist centre, however 82% had seen at least one EB patient in the previous five years (Figure 2).
- GP respondents were from a range of practice areas (52% suburban, 40% urban, and 8% rural) and 58% reported having seen at least one EB patient in the previous 5 years (Figure 2).
- Responses collected could be grouped into four output themes: HRQoL, EB symptoms, treatment landscape, and carers (Figure 3), from which seven key issues have been identified which comprise a call for action (Table 1).

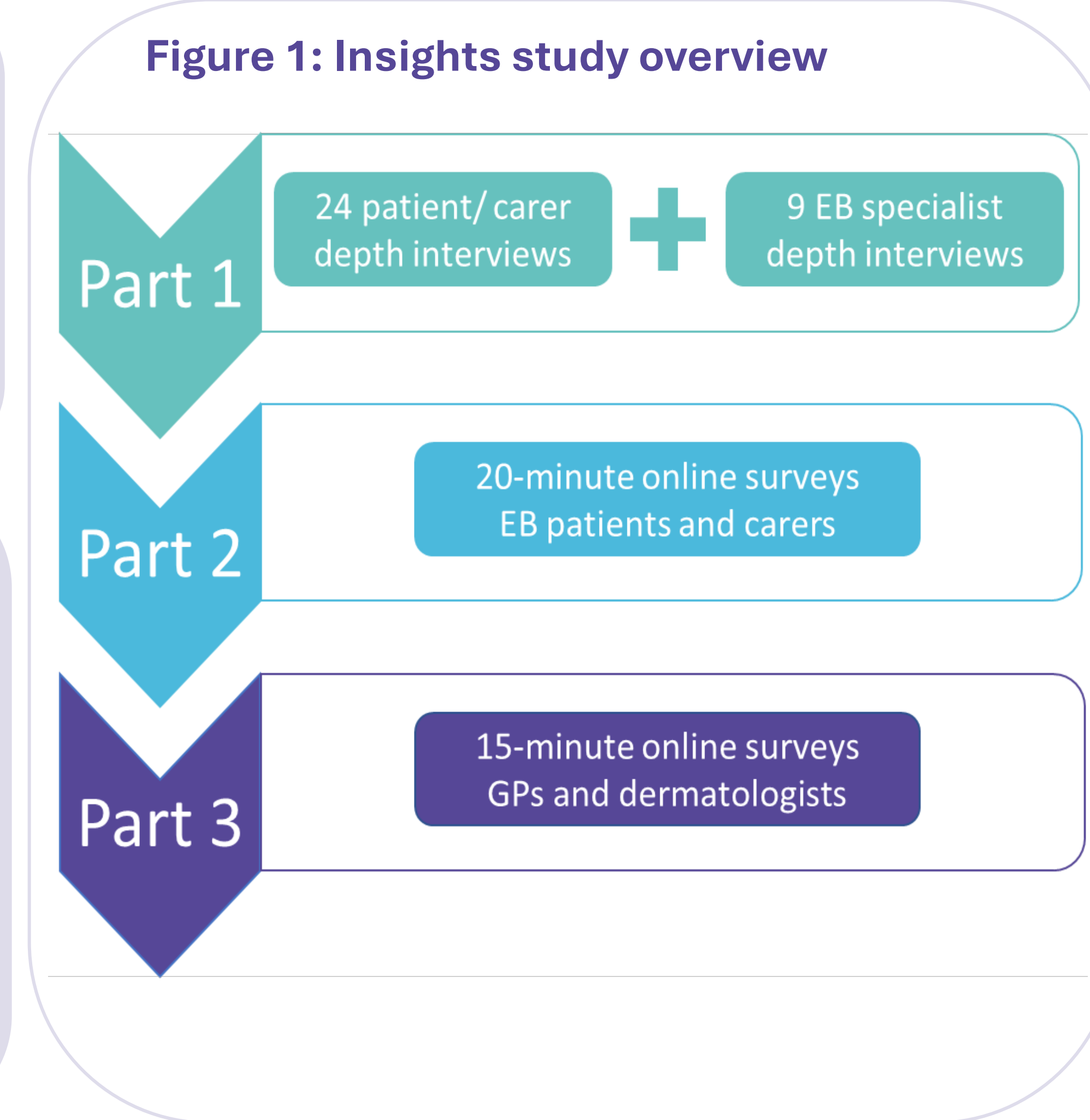


Figure 2: Participant demographics

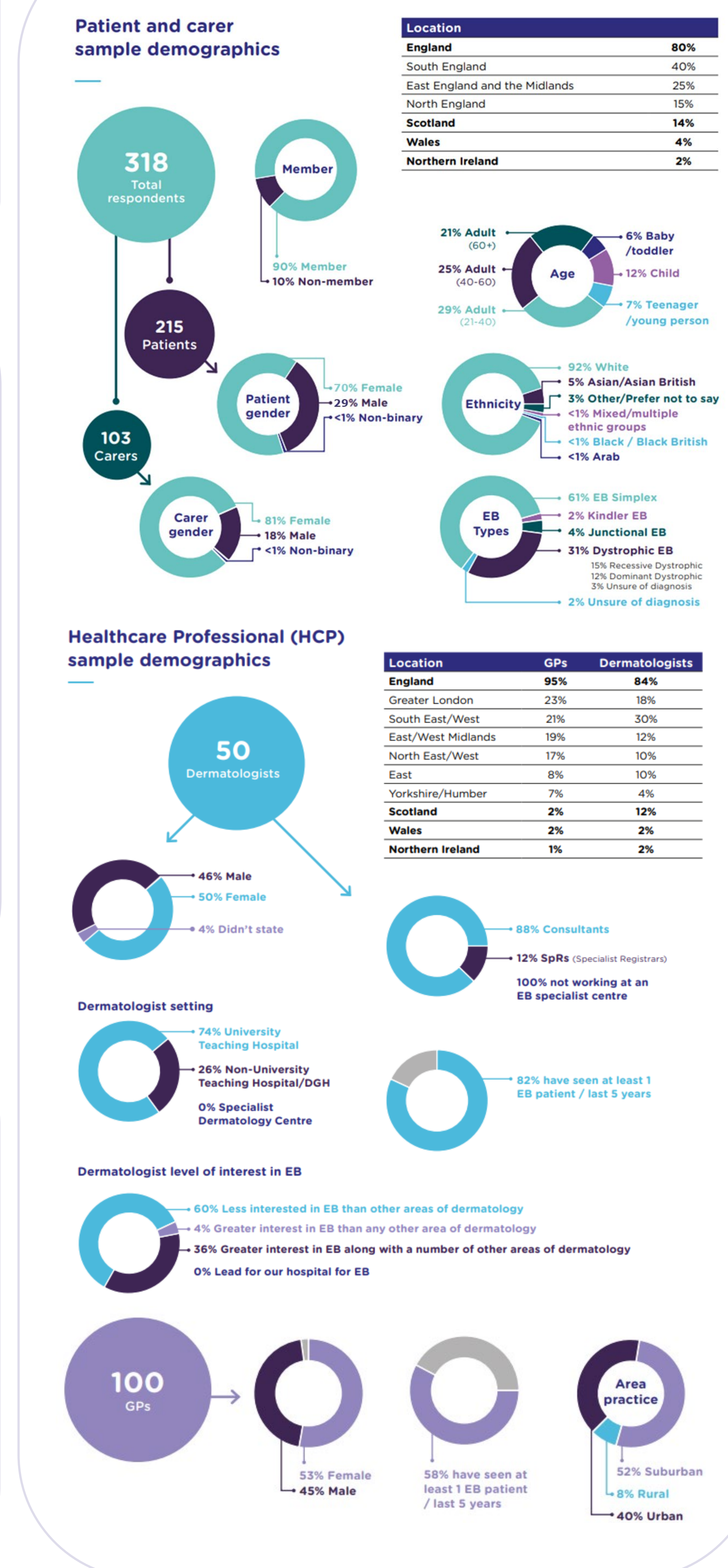


Figure 3: Insights study output themes

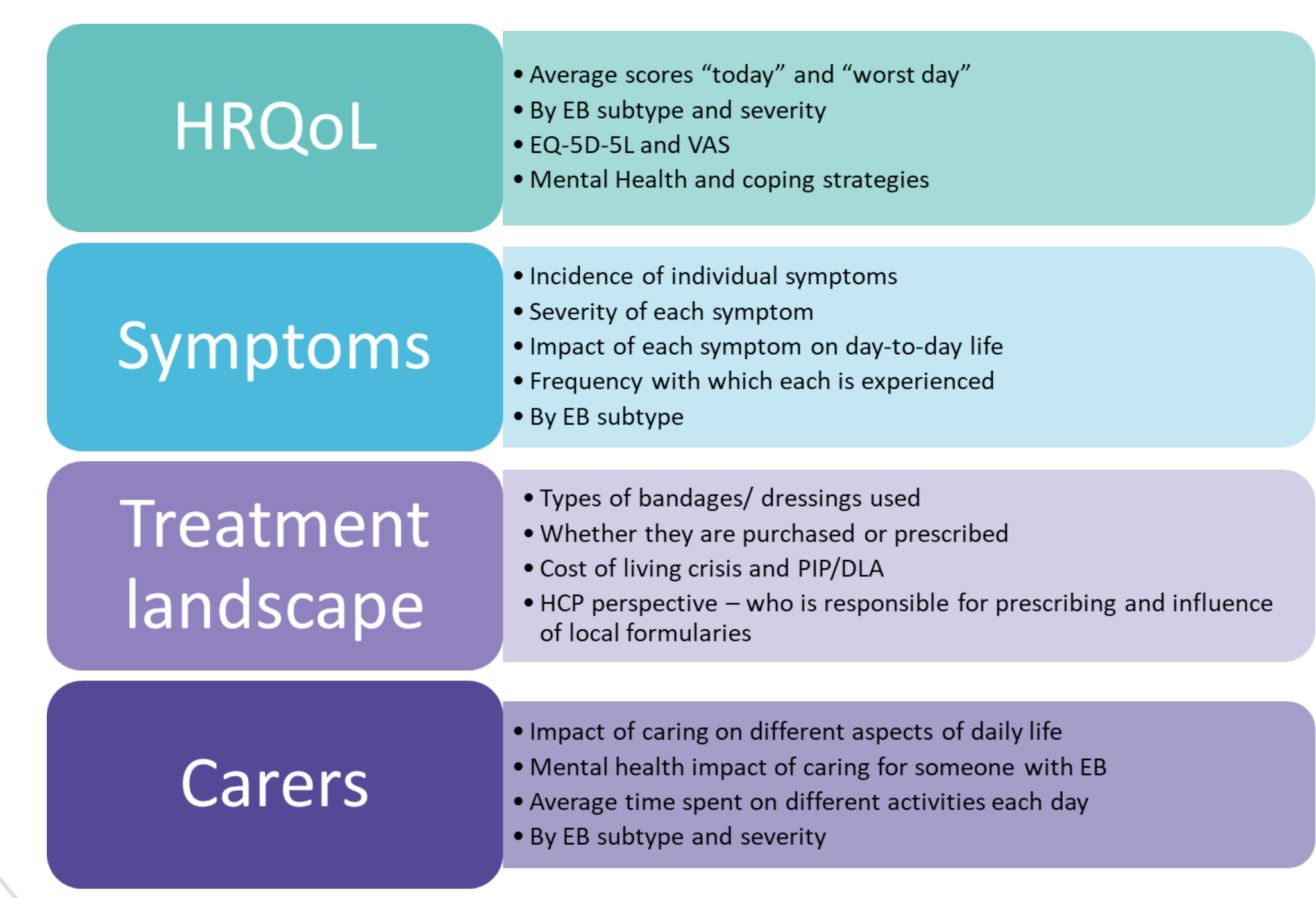


Table 1: Key issues – call for action

Key findings and call for action	
1	There is a need for improved awareness and understanding of EB
2	There is a geographical imbalance of EB specialist healthcare
3	The cost-of-living crisis is exacerbating the financial burden of living with EB
4	The current mental health provision for EB patients/ carers is inadequate
5	EB patients/ carers want treatment recommendations from other EB patients
6	EB patients/ carers want effective treatments as a priority
7	EB patients want more interaction with DEBRA's community support team

Conclusions

- More than 200 people with different types of EB, over 100 carers, 50 dermatologists, and 100 GPs contributed to the 2023 EB Insights study, forming the most comprehensive, patient-centric research in EB to-date.
- The opinions of patients and families impacted by EB is essential to our understanding of this debilitating condition.
- The Insights Study provides a body of quantitative and qualitative data across a broad demographic, and reports on many areas of importance to families impacted by EB including: HRQoL, incidence and prevalence of symptoms, treatment landscape, and the impact on carers.
- These data serve as a foundation for patient advocacy and to support pharmaceutical companies in their evidence generation activities, as they prepare for the launch of new EB therapies.

References 1. DEBRA UK. What is EB? [cited 2024; Available from: <https://www.debra.org.uk/what-is-epidermolysis-bullosa-eb>]; 2. Bardhan, A., et al., Epidermolysis bullosa. Nat Rev Dis Primers, 2020. 6(1): p.78; 3. Cianfarani, F., et al., Pathomechanisms of Altered Wound Healing in Recessive Dystrophic Epidermolysis Bullosa. Am J Pathol, 2017. 187(7): p.1445-1453; 4. Fine, J.D., Cumulative life course impairment by epidermolysis bullosa. Curr Probl Dermatol, 2013. 44: p.91-101; 5. Denyer J, Pillay E, and C. J, Best practice guidelines - Skin and wound care in epidermolysis bullosa: An International Consensus, Wounds UK. 2017.

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