

The 2023 EB Insights Study: the impact of epidermolysis bullosa on the health-related quality of life of patients and their carers

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Background

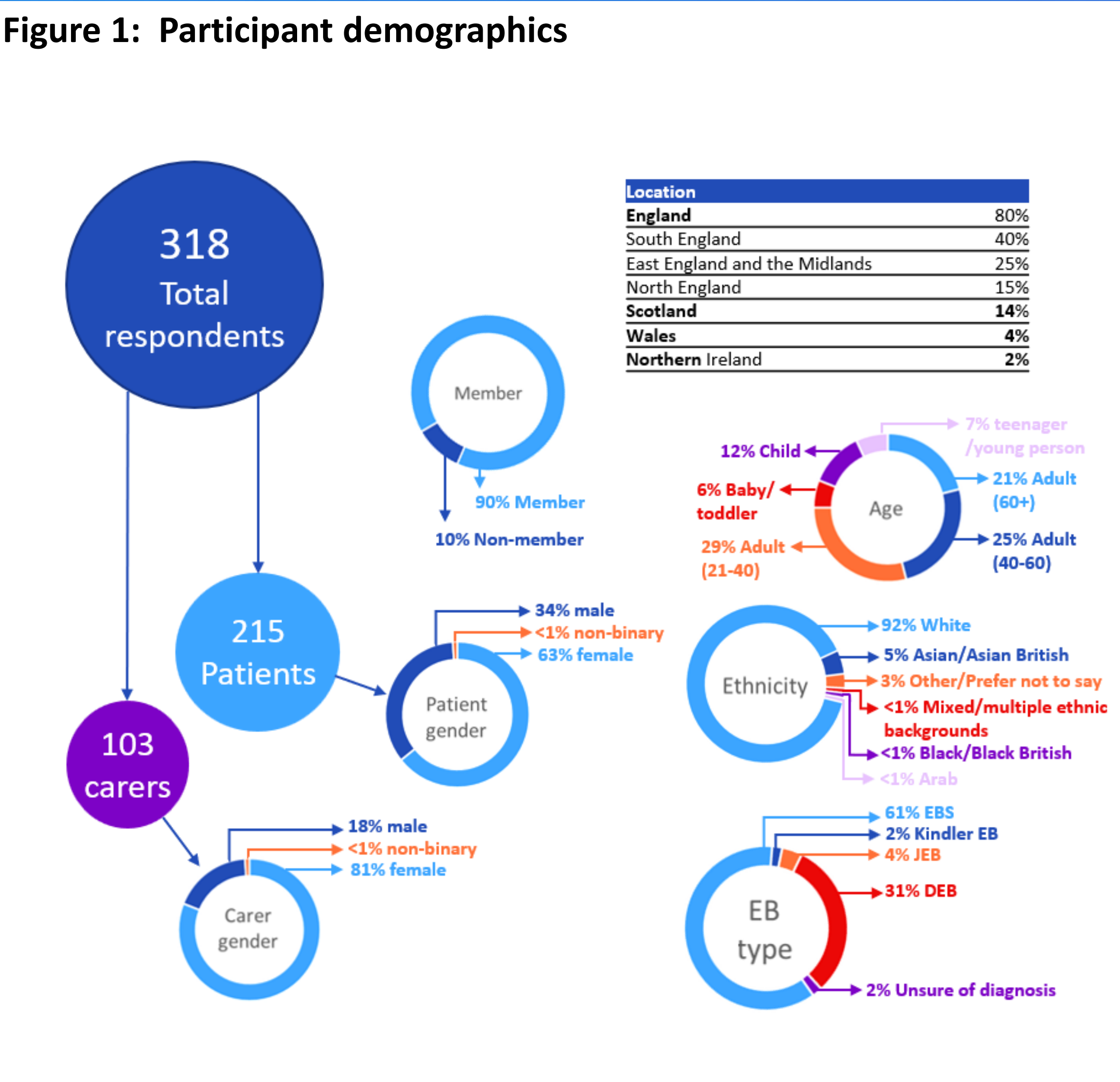
- Epidermolysis bullosa (EB) is a complex group of lifelong, rare genetic disorders which cause the skin to become very fragile, and tear or blister, as a result of minor trauma. They include EB simplex (EBS), dystrophic EB (DEB), junctional EB (JEB), and Kindler.[1]
- Patients have an altered wound 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] Despite this, data on the impact of EB on the health-related quality of life (HRQoL) of both patients and carers is limited.

Methods

- Participants were recruited through DEBRA UK. Inclusion criteria required patients to be aged 16 years or above, live in the UK, and either have EB or be the carer of someone with EB.
- An online survey, developed with input from EB clinical specialists, was completed and included questions relating to:
 - 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
- HRQoL was assessed using the EQ-5D-5L measure and associated visual analogue scale (VAS). EQ-5D index values were calculated using the EQ-5D-5L to 3L crosswalk and the current UK EQ-5D-3L value set.[5] A utility index of 1 reflected full health, and -0.594 reflected a state worse than dead. Assessments were completed by patients and carers twice; once to assess the patient's health 'today', and again for when their EB is 'at its worst'.
- Carers of people with EB were asked several questions around how their caring responsibilities impacted on their daily lives.

Objectives

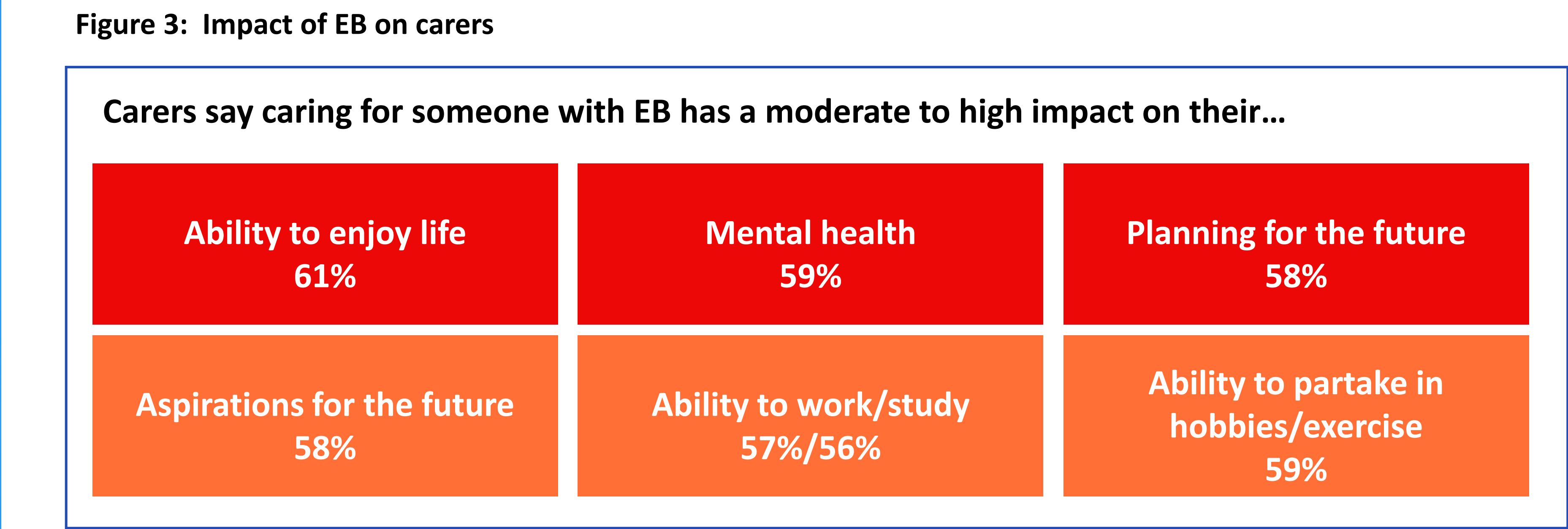
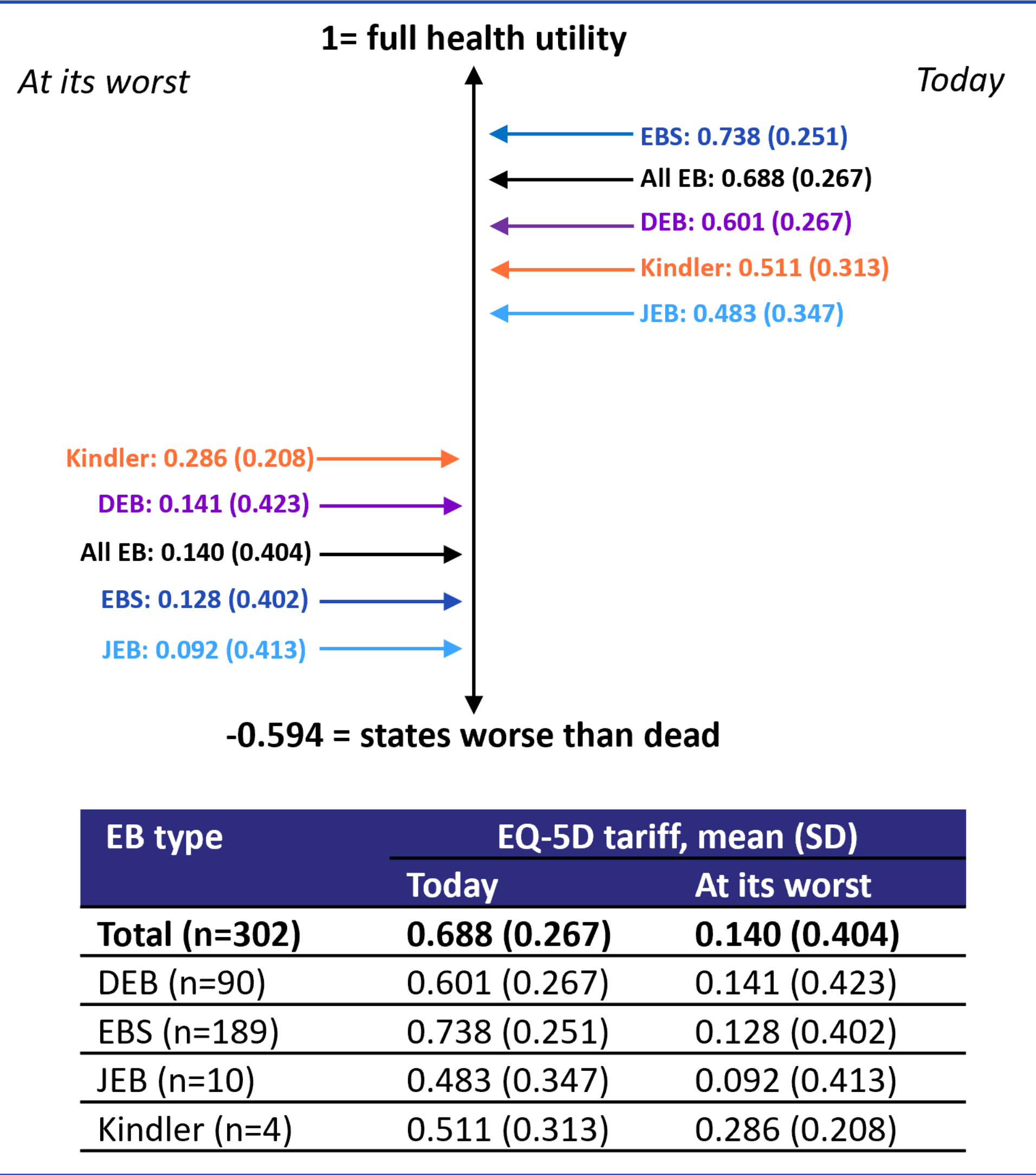
- The 2023 EB Insights Study was commissioned by DEBRA, a UK national medical research charity and patient support organisation for people living with EB. The objective was to provide a foundation of data for patient advocacy groups and to support pharmaceutical companies in evidence generation needs for health technology assessment of new EB therapies.
- Insights reports a body of data across a broad demographic to capture the impact of EB on daily life, including the HRQoL, of patients and their carers/families.



Results

- A total of 215 patients and 103 carers completed the survey between April and May 2023. The baseline characteristics of the participants are summarised in **Figure 1**.
- Average utility score on a typical day and when EB was 'at its worst' is summarised by EB subtype in **Figure 2**.
- Of respondents who are carers of people with EB, most were female (81%), and 71% were parents.
- Carers reported a moderate to high impact on various aspects of their lives (**Figure 3**). Furthermore, 46% of carers strongly agreed that they would like access to mental health support with a professional who understands the unique challenges of EB.

Figure 2: EB patient HRQoL



Conclusion

- The Insights Study 2023 is the most comprehensive, patient-centric research in EB to-date.
- The data reported demonstrate the substantial morbidity associated with living with EB and the impact on patient HRQoL, reducing the average utility tariff on a typical day to 0.688 compared to a UK population norm of 0.806-0.915 (range provided for male and female, age range 20-50 years).[6]
- The lives of carers are also heavily impacted, demonstrating a need for more support and respite for carers.
- These data serve as a foundation for patient advocacy and have the potential to support pharmaceutical companies in their evidence generation activities, as they prepare for the launch of new EB therapies.