Global EB Priority Setting Partnership

identifies research priorities for the four main types of epidermolysis bullosa

A word from our Director of Member Services

"In the 2023 Epidermolysis Bullosa (EB) Insights Study, EB patients and carers told us that they wanted to see funding for research into potential EB treatments as our top priority. In response to this and to ensure that our future EB research addresses the key needs of the global EB community, we set up an EB Priority Setting Partnership (EB PSP) with the objective of identifying the research priorities for the four main types of EB.

The project was led by DEBRA UK but involved close collaboration with other DEBRA patient support organisations (DEBRA Ireland, DEBRA Canada, and DEBRA International) plus EB charities, EB Research Partnership and Cure EB, to form a truly global research alliance.

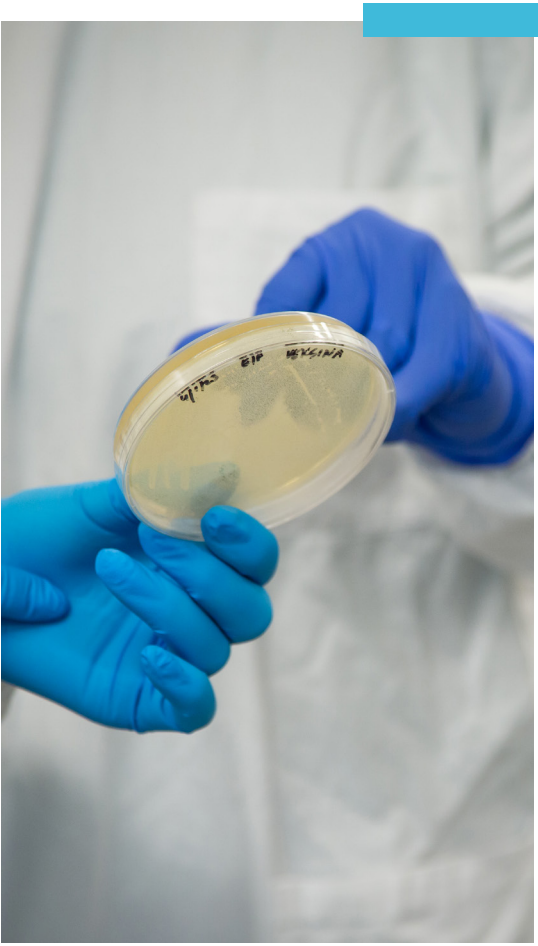
The James Lind Alliance (JLA), a non-profit-making initiative funded by the National Institute for Health and Care Research (NIHR), facilitated the project and Synergy Healthcare Research, a leading healthcare research agency, supported the data gathering.

We are so grateful to the members of the EB community, living and working with EB, who participated in this study and to all the organisations who partnered with us to make this happen.

I hope you enjoy reading this summary of the study and more importantly the outcomes and how this will shape our future EB research strategy".

Claire Mather - DEBRA UK Director of Member Services, June 2025

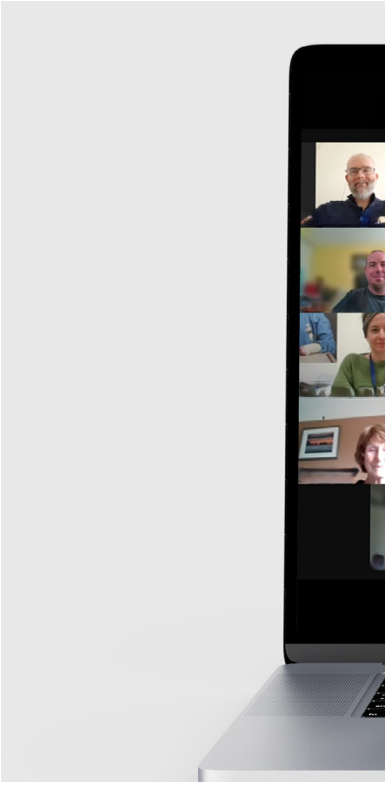
Why was the EB Priority Setting Study needed?



Often medical research can overlook the questions that patients, families/friends, carers, and health and social care professionals consider most important. This can result in areas of potentially important research being neglected and funding wasted.

The JLA priority setting partnership method enables partnership working between patients, families/friends, carers, and healthcare professionals to help identify and prioritise the unanswered research about prevention, diagnosis, treatments and service delivery for a specific disease of interest, in this case, EB.

With an agreed list of research priorities for each type of EB we can ensure that future EB research projects address the issues that are most important to the EB community.



How was the research conducted?

In summer 2024, research questions were gathered through an online survey of 537 people from 41 countries. The survey was completed by people living with EB, family members/ friends, carers, and EB health and social care professionals. A total of 2800 questions were received, and each was then reviewed by the JLA steering group, which comprised representatives from the partner organisations, people with lived experience of EB, and healthcare experts. The steering group advised and guided the project from beginning to end.

The JLA research method defines a question as being unanswered if there are no up-to-date, reliable reviews of research evidence available that fully answer the question.

From this stage, 304 questions were removed due to there being research evidence already available that answers that question. We will ensure that the EB community is aware and able to easily access this research/evidence.

The verified questions were then summarised to produce 62 questions that reflected the overall themes of the original questions submitted.

An online prioritisation survey was conducted in November 2024 which asked participants to select up to ten of the 62 shortlisted questions that were most important to them.

This survey was completed by 865 members of the global EB community; respondents came from 55 countries and represented all EB types. There was also good representation from EB health and social care professionals including dermatologists and EB nurses.

The results of this second survey identified 17 research questions for each type of EB that were of key importance to the EB community. The final stage of the EB-PSP study was to create a top ten for each type of EB from these 17. This was conducted through several small group workshops in February and March 2025, each specific to the type of EB.

The small number of people living with kindler epidermolysis bullosa (KEB) meant that a KEB specific workshop was not possible therefore, the top 18 research priorities for KEB are presented, based solely on the uncertainties gathered in the first survey and prioritised in the second survey.

What were the results?

Below are the top research priorities for each type of EB determined by the global EB community through the EB PSP.

Epidermolysis bullosa simplex (EBS) – 70% of all cases	
1	What are the best strategies for **pain relief** (including long-term) for people with EB? Including barriers that stop people with EB taking painkillers, and how to overcome these, and why some forms of pain relief are ineffective.
2	What are the best strategies to **prevent and manage blisters** to produce better outcomes for people with EB, including use of medication, lifestyle changes, and methods to reduce friction and impacts?
3	What is the best way to **manage and prevent foot problems** in EB? For example - blistering, sweating, itching, shoe / sock choice.
4	What are the best strategies for **improving mental health for children and young people** with EB, by age group, including skills to help equip them for later life?
5	What is the best way to **reduce itching in EB**, including both medical treatment and non-medical treatments and interventions?
6	**Is there a relationship between EB and other conditions** (e.g. heart, bone, immune system, nervous system, other skin and lung conditions, obesity and arthritis)?
7	What is the best way of **managing heat and humidity** for people with EB, and related blistering?
8	**How does EB change with age** (e.g. changes to wound healing, skin sensitivity, risk of infection), and how are changes / progression of EB best managed?
9	**How effective are gene therapy options for treating EB**, and how does this vary by EB subtype?
10	What is the **best practice for wound prevention, management and healing** in EB, including the use of creams, steroids, antibiotics and other treatments, and bathing - taking into account the wound location, severity, new and long-term wounds.

Dystrophic epidermolysis bullosa (DEB) – 25% of cases

1	What are the **best strategies for pain relief** (including long-term) for people with EB? Including barriers that stop people with EB taking painkillers, and how to overcome these, and why some forms of pain relief are ineffective.
2	What is the **best way to reduce itching in EB**, including both medical treatment and non-medical treatments and interventions?
3	What are the **best strategies for improving mental health for children and young people** with EB, by age group, including skills to help equip them for later life?
4	What is the **impact of inflammation (swelling/redness) on EB**, and what are the best ways to manage inflammation (including anti-inflammatory treatments)?
5	What is the **best way to manage gastrointestinal (digestive system) problems in EB**? This **includes managing difficulty swallowing and preventing/managing strictures** (narrowing) in the oesophagus (food pipe) and gastrointestinal tract, through methods such as **tube feeding, drug treatments and gene therapy.**
6	What are the **best strategies for earlier diagnosis and prevention of cancers** for people with EB?
7	What are the **best strategies to prevent and treat eye damage and vision loss** for people with EB, including preventing blisters and corneal abrasions (scratches on the eye)?
8	What is the **best diet for good nutrition in EB**, including use of supplements?
9	What is the **best practice for wound prevention, management and healing** in EB, including the use of creams, steroids, antibiotics and other treatments, and bathing - taking into account the wound location, severity, new and long-term wounds.
10	What are the **best ways for people with EB to manage their dental health** including maintaining healthy teeth, cleaning the teeth, maintaining tooth enamel, and getting dental work done (including best methods for replacing lost teeth)?

Junctional epidermolysis bullosa (JEB) – 5% of cases

1	What is the **best practice for wound prevention, management and healing** in EB, including the use of creams, steroids, antibiotics and other treatments, and bathing - taking into account the wound location, severity, new and long-term wounds.
2	What are the **best strategies for managing issues with the respiratory tract** (lungs and windpipe) and respiratory distress (breathing difficulties) in people with EB?
3	What are the **best strategies for improving mental health for children and young people** with EB, by age group, including skills to help equip them for later life?
4	What is the impact of inflammation (swelling/redness) on EB, and what are the **best ways to manage inflammation** (including anti-inflammatory treatments)?
5	What is the **best way to reduce itching in EB**, including both medical treatment and non-medical treatments and interventions?
6	**How effective are gene therapy options for treating EB**, and how does this vary by EB subtype?
7	What are the **best strategies for pain relief** (including long-term) for people with EB? Including barriers that stop people with EB taking painkillers, and how to overcome these, and why some forms of pain relief are ineffective.
8	What are the **best strategies to prevent and treat eye damage and vision loss** for people with EB, including preventing blisters and corneal abrasions (scratches on the eye)?
9	What is the **best way to manage gastrointestinal (digestive system) problems** in EB? This **includes managing difficulty swallowing and preventing/managing strictures** (narrowing) in the oesophagus (food pipe) and gastrointestinal tract, through methods such as **tube feeding, drug treatments and gene therapy**.
10	What is the **best way to manage toileting problems** in people with EB, such as constipation, pain when defecating (having a poo), or anal fissures (a tear inside the anus)?

Kindler epidermolysis bullosa (KEB) – less than 1% of cases

1	What are the **best strategies for preventing or slowing skin atrophy (skin thinning)** in people with Kindler EB?
2	What are the **best strategies for earlier diagnosis and prevention of cancers** for people with EB?
3	What is the **best diet for good nutrition in EB**, including use of supplements?
4	**How does being in the sun impact people with EB**, and how important is sun protection? What are the best sun protection practices for people with EB?
5	What are the **best strategies to prevent and treat eye damage and vision loss** for people with EB, including preventing blisters and corneal abrasions (scratches on the eye)?
6	**What is the best way to manage genitourinary complications and toileting problems in people with EB**, such as urethral strictures, constipation, pain when defecating (having a poo), or anal fissures (a tear inside the anus)?
7	What is the **best way to manage gastrointestinal (digestive system) problems** in EB? This includes **managing difficulty swallowing and preventing/managing strictures** (narrowing) in the oesophagus (food pipe) and gastrointestinal tract, through methods such as **tube feeding, drug treatments and gene therapy.**
8	What are the **best strategies for nail care** for people with EB?
9	What is the **best way to maintain oral (mouth) health in people with EB**, including ulcer and wound management, dryness and saliva (spit) production?
10	What is the **best type of cream / ointment to use for people with EB** for daily use?
11	**Is there a relationship between EB and other conditions** (e.g. heart, bone, immune system, nervous system, other skin and lung conditions, obesity and arthritis)?
12	**To what extent do people with EB have an increased risk of getting cancer** (e.g. melanoma, squamous cell carcinoma, mouth cancer etc)?
13	What are the **best strategies for scalp and hair care** (including hair regrowth) for people with EB?
14	What are the **best strategies to prevent and manage blisters** to produce better outcomes for people with EB, including use of medication, lifestyle changes, and methods to reduce friction and impacts?
15	What are the **best strategies for improving mental health for children and young people with EB**, by age group, including skills to help equip them for later life?
16	What is the **best way to reduce itching in EB**, including both medical treatment and non-medical treatments and interventions?
17	What is the **best practice for wound prevention, management and healing in EB**, including the use of creams, steroids, antibiotics and other treatments, and bathing - taking into account the wound location, severity, new and long-term wounds?
18	What are the **best strategies for pain relief (including long-term) for people with EB?** Including barriers that stop people with EB taking painkillers, and how to overcome these, and why some forms of pain relief are ineffective.

Conclusion and next steps

This is a hugely important piece of global research, a first of its kind and the first global PSP for a rare condition that follows the JLA research method.

The 2023 EB Insights Study identified that research into treatments for EB was a top priority for the EB community. However, it didn't give the depth of insight needed to support research commissioners, like DEBRA UK, in prioritising the various research opportunities that exist for EB, ensuring that they satisfy the key needs of the EB community. We now have that insight.

DEBRA UK is one of the largest funders of EB research globally having invested over £22m and established much of what is now known about EB.

Some of our existing research programmes will address some of the unanswered research questions that the EB community has, but there will be gaps.

We will now use the data from the EB PSP to review our current research programme. We will check whether our existing research projects address the key unanswered questions identified through this study for each of the four main types of EB.

If they don't, then we will seek to commission research that does, whether this is research that we commission and fund ourselves, or research programmes for which we seek collaboration and co-funding to be able to commission.

We hope that other organisations active in the EB research space will also use the insight gained from this study to help ensure any EB specific research they commission also directly addresses the key needs of the EB community.

We can only fund research that could be transformational for people living with EB by working together. If you can support us on our journey to a world where no one suffers with EB, we would love to hear from you.

research@debra.org.uk

01344 771961

To read the full study findings, please scan the QR code

debra.org.uk/EB-priorities-report

Thank you

Our partners

This study is another great example of community engagement, working together as a global EB community to improve outcomes for everyone directly affected by EB.

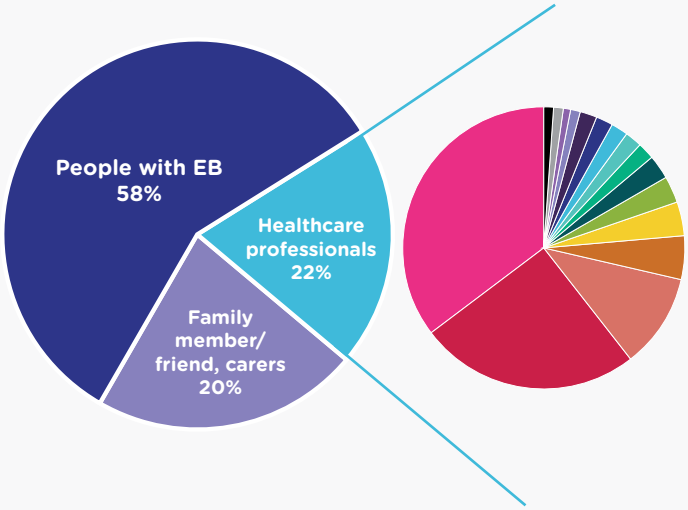
Without the involvement of people living with all types of EB, their families/friends, carers, EB healthcare professionals and social care professionals this study simply would not have been possible.

Thank you to everyone who gave up their time to participate in this study.

Thank you also to all our partners who co-funded this study, to the members of the EB PSP steering group who gave their time and provided expert input, and to the teams at the JLA and Synergy Healthcare Research, for your help in delivering this important study.

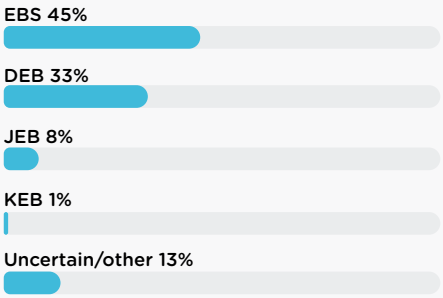
Study demographic data

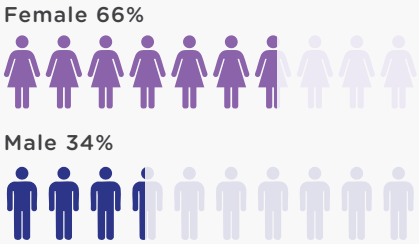
First online survey: 537 respondents

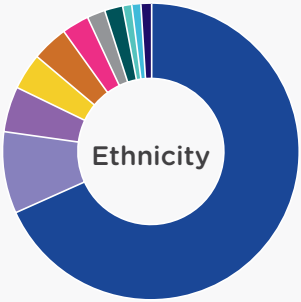


- **36%** Other healthcare professionals
- **26%** Dermatologists
- **11%** EB nurse
- **5%** Mental health professionals
- **4%** Social workers
- **3%** Dentist
- **3%** Dermatology nurse
- **2%** Dietician
- **2%** Geneticist
- **2%** Ophthalmologist
- **2%** Pharmacist
- **2%** Professional care worker
- **1%** GP
- **1%** Occupational therapist
- **1%** Paediatrician
- **1%** Surgeon

EB Types



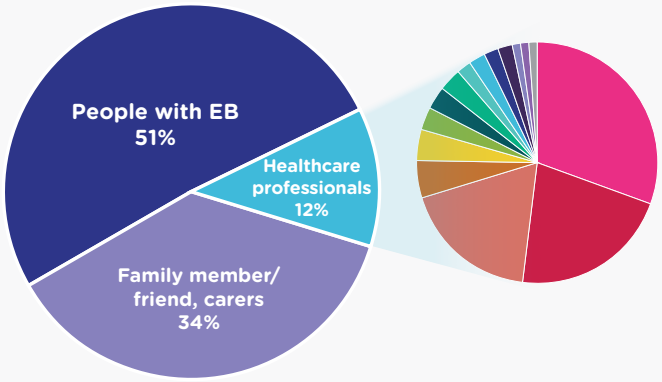




- **69%** White
- **9%** Arab
- **5%** Hispanic/Latino
- **4%** South Asian
- **4%** Mixed/multiple ethnic groups
- **3%** Southeast Asian
- **2%** East Asian
- **2%** Another ethnic group
- **1%** Black
- **1%** Indigenous Peoples
- **1%** Prefer not to say

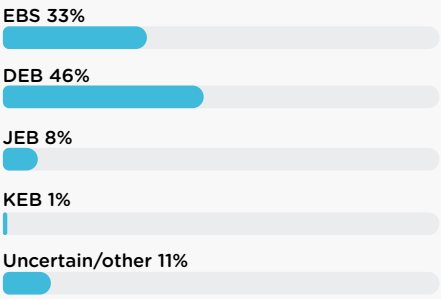
Study demographic data

Second online survey: 865 respondents



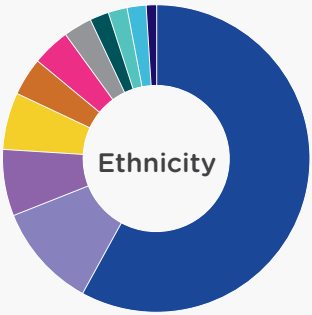
- **30%** Dermatologists
- **21%** Other healthcare professionals
- **18%** EB nurse
- **5%** Geneticist
- **4%** Mental health professionals
- **3%** Social workers
- **3%** Pharmacist
- **3%** Physiotherapist
- **2%** Professional care worker
- **2%** Occupational therapist
- **2%** Paediatrician
- **2%** Surgeon
- **1%** Dentist
- **1%** Dermatology nurse
- **1%** Podiatrist

EB Types



Gender

- **64%** Female
- **35%** Male
- **<1%** Non-binary
- **<1%** Prefer not to say



- **58%** White
- **11%** East Asian
- **7%** Arab
- **6%** Prefer not to say
- **4%** Hispanic/Latino
- **4%** South Asian
- **3%** Southeast Asian
- **2%** Mixed/multiple ethnic groups
- **2%** Another ethnic group
- **2%** Indigenous Peoples
- **1%** Black

Feedback from members of the global EB community who participated in this study

"I felt every discussion we had about the priorities was done with extreme consideration by each and every participant and it was all done in a very respectful way".

"The workshops were fantastic. This was the first of its type I participated in, but I feel even as a patient I learned a great deal and it was an extremely valuable experience that I am so grateful to have been a part of. Also, a huge thank you to the facilitators and all those helping drive these goals forward".

"It was my first time participating in activities like this, but I felt very comfortable and welcomed".

"This workshop also pushed my self-confidence. I felt so heard and appreciated. I really had the feeling that my opinion was wanted and that was an empowering experience for me".

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Help stop the pain of EB

DEBRA funds pioneering research, specialist healthcare, international best practice and life-long care and support to the EB community. We make a difference today with the aim of a better future for those suffering from the condition.

Together we will #StopThePain.

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