EB Expert Panel Workshop Report

7 October 2023
Grand Canal Hotel
Dublin

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About Debra

We are Debra, the butterfly skin charity.

We might be a small charity, but make no mistake, our mission and our ambitions are colossal. We are here to tell Ireland and the world beyond about epidermolysis bullosa (EB) - a painful and distressing genetic disorder that affects the body's largest organ; the skin.

We are here to be a positive force for all those living with EB and all those whose lives have been impacted by EB. Our key working areas include EB Support, Advocacy and Research. We are here to help in all kinds of every day ways, to listen, support, empower and alleviate unimaginable pressures. We are here to lobby those in the halls of power. To command attention, demand and drive research and ensure imperative progress is made.

But above all, we are here to bring hope - not that passive, powerless, resigned kind of hope - but the active bold hope that moves things on.

Uniting for change for everyone with skin as fragile as a butterfly wing.

About the EB Expert Panel

We know that there is power in numbers. The more voices we have the stronger our work will be. In November 2022, we launched our EB Expert Panel, a group of those who understand EB the most and are willing to use their experience to shape our work.

The panel advise us on various topics related to what living with EB in Ireland is like now and what it should look like in the future. With their help, we can make sure we can provide support in the way that matters to them. As valued advisors, members of the EB Expert Panel provide advice to our Research, Advocacy and Family Support teams on projects they are working on.

The panel meets as a group twice a year. In between meetings, participants stay connected with Debra through one-on-one conversations with our team, when their advice is needed.

We would like to sincerely thank all members of the EB Expert Panel for their valued contribution to our work. We look forward to working with you further.

Opening Remarks

Sarah Mullins - Research

Sarah opened the workshop by introducing herself and other members of the Debra team who would be managing the EB Expert Panel and facilitating the workshop; Kate Gibney, Sinéad Hickey, Deirdre Callis, Marina Shatskikh and Aimée Longmore.

She thanked those attending for giving up their Saturday in order to make a difference to the work carried out by Debra. She then went on to remind the panel that they meet for a workshop twice a year to discuss bigger topics as a group, with individual panel members being contacted directly on an ad-hoc basis in between workshops for smaller tasks.

All members of the panel are offered payment for tasks they complete, including workshop attendance, with payment levels based on the time and effort it takes to complete a task. Sarah thanked those who had returned after attending previous workshops and welcomed those who were attending their first workshop on the day.

After her opening remarks, Sarah went over some house rules for the workshop and asked everyone to introduce themselves during a short icebreaker activity. She then began to give a short research update before the day's discussions.

Updates

Sarah Mullins - Research

Sarah provided a short research update to inform the panel about how they have been taking their feedback onboard. In the first EB Expert Workshop in 2022, the panel revised Debra's Research Priorities and Criteria. Within this revision, it became clear that EB foot research was a priority that had not yet been addressed by Debra.

With this in mind, over the summer of 2023 the Research team hired a Research Assistant, Alison Hyland, to help Sarah carry out a scoping project titled 'Footcare in Epidermolysis Bullosa: a review of the current scientific evidence and the lived experience in Ireland'. Alison is also a member of the EB Expert Panel and lives with EB herself, so she was able to assist with the PPI in this project as a co-researcher also. Sarah did not go into the results of the study as a report of the findings is currently being developed, however she explained that the project involved a literature review of the current research on this topic, as well as interviewing individuals who link in with Debra to fill in any knowledge gaps. The findings of both parts of the project were combined to draw conclusions and make recommendations.

The results of this project have led to the Research team contacting researchers in Maynooth University about developing a medical device for EB feet. Debra are currently supporting the researchers in Maynooth in applying for funding with the Irish Research Council. Sarah explained that if they are successful with funding the project will not start until next year, however these conversations would not have happened without the input of the EB Expert Panel. Their voice is so important in the work that Debra do and the team are very appreciative of their input. Sarah then introduced the researchers from University College Dublin (UCD) who were this sessions guest speakers.

Research

Members of Prof Wenxin Wang's research team from UCD joined the panel to give a presentation on the EB research they are currently carrying out which is being funded by Debra. Dr Darío Sandoval led the presentation and was accompanied by Albina Bushmalyova and Dr Xianqing Wang.

Darío's interactive presentation explained what gene therapies are and gave the EB Experts a better understanding of how gene therapies have potential to treat EB. Darío also gave an insight into what his team are researching in UCD and how exactly the treatment they are developing could work for Recessive Dystrophic EB.

After the presentation, each researcher sat at one of four discussion tables with 6-7 EB Experts and a facilitator from Debra. The researchers had a discussion with EB Experts on their opinions of gene therapies after learning about them a bit more. The researchers also looked for feedback on how their presentation went, how they could improve the donation process for EB skin cells as this is vital for their lab work and how gene therapies could seem more appealing to those living with EB and their families. A summary of all of the findings is available on the next page.

Overall, both the EB Experts and researchers felt that it was a very productive session with mutual learning taking place. All EB Experts said they enjoyed the session and would be happy to do something like this again.

Summary of UCD discussion

Topic 1 - Impressions of Gene Therapy

1. If Krystal Biotech's VYJUVEK was available for your type of EB in Ireland, would you (or your family member) consider being treated with it?

- The majority of attendees said yes, however there were some reservations amongst the group.
- Reservations included not knowing enough about the therapy, not knowing enough about how safe it was and/or how it might look for each type of EB if ever available.
- Most participants representing RDEB felt it had great potential to improve quality of life.
- Worries around if there are any potential side effects.

2. Do you think your life would change through using a gene therapy? If yes, how do you think a gene therapy could change your life?

- For those living with EBS, they were unsure if it would 'change their life' as they have lived with and managed their condition all of their lives, however they did believe it could change lives for the next generation.
- For those living with EBS, the question was then rephrased to ask how having less blisters might change their life. They responded in saying it would help improve mental health, increase physical activity levels and improve overall quality of life.
- For those representing RDEB and JEB they believed it would certainly have made a difference to quality of life, particularly if it reduced the frequency of bandage changes.

Summary of UCD discussion

Topic 2 - Today's presentation by UCD

1. What did you learn in today's presentation? Did you learn anything new?

- Overall, EB Experts felt they had a much better understanding of science and the research that UCD are working on.
- Gained a better understanding of what gene therapies are and how they could have potential to treat EB.

2. Did you enjoy today's presentation and/or find it useful? Would you be interested in doing something like this again?

- All participants enjoyed the presentation and found it useful. They were open to doing something like this again.

Topic 3 - Help UCD Research to fight EB

1. Would you like to visit the lab in UCD to see what the researchers do?

- A small number of attendees were not interested in visiting the lab.
- The majority of EB Experts would love to visit the lab and see what the scientists are doing to give them an even better understanding of UCD's work.
- Some attendees expressed interest in bringing their children to the lab so that they could gain an understanding also.

Summary of UCD discussion

2. In your experience, what is the number one thing you believe scientists need to address in the treatment of EB?
According to EB Experts, scientists should place a focus on the following areas/topics:

- Speed of scientific discoveries/bringing treatments to the market
- Treatments for itch associated with EB
- Mindfulness-based interventions for pain management
- Pain relief that works but is not as strong as ibuprofen due to associated side effects, suggested perhaps this could be topical
- Would like scientists to put themselves in their shoes - if a scientist or their child had EB, what do they think they should prioritise? and then work from there
- Focus on infections associated with EB
- Nutrition and EB
- Treatments for internal blistering or preventing oesophageal strictures
- Continue focusing on gene therapy development

3. If scientists in UCD were developing a treatment for your type of EB, would you be willing to donate your skin cells to help with their research? If no, why?

- Everyone said that they would, but believe the biobank needs to be better explained to those who attend the EB clinics as a lack of understanding is a barrier to donating skin cells.
- Some wanted clarity on who exactly could donate skin cells.

Family Support

Deirdre Callis - Head of Family Support

Deirdre spoke to the panel about a service review that the family support service is currently undergoing. The primary goal of this review is to gain insights to allow the board to assess the type and level of services Debra should strive to offer people affected by EB. It aims to determine the most effective ways to allocate resources, deliver support, and report on EB support services. Additionally, the review also serves to ensure the service continues to meet the needs of individuals with EB and to identify any gaps in service delivery.

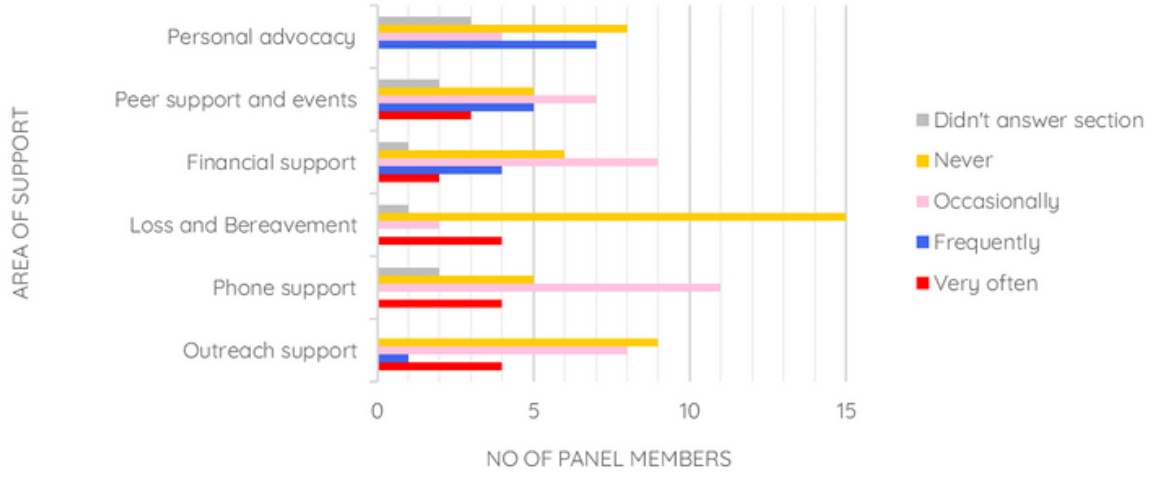
Deirdre then went on to give an overview of the family support service. The service supports all those impacted by EB, encompassing individuals living with EB, those who have lost loved ones to EB, caregivers and family members. The support reaches all counties in Ireland, including Northern Ireland, and supports people throughout their life journey. The service is structured around six core pillars of support, which Deirdre elaborated on in detail: outreach support, phone support, loss and bereavement support, financial support, peer support and events, and personal advocacy.

Following this presentation, Deirdre invited the panel to complete a 10-minute survey to gather their perspectives on each support channel. Subsequently, the panel engaged in roundtable discussions, delving into whether the service adequately addressed the needs and priorities of people impacted by EB and exploring potential areas for improvement. The primary objective of this exercise was to gain feedback from the EB Expert panel regarding the current service, with a specific focus on need, accessibility, delivery, and impact. This feedback will be an important component of the review and influence any required changes in the future, ensuring that Debra's services remain person-led.

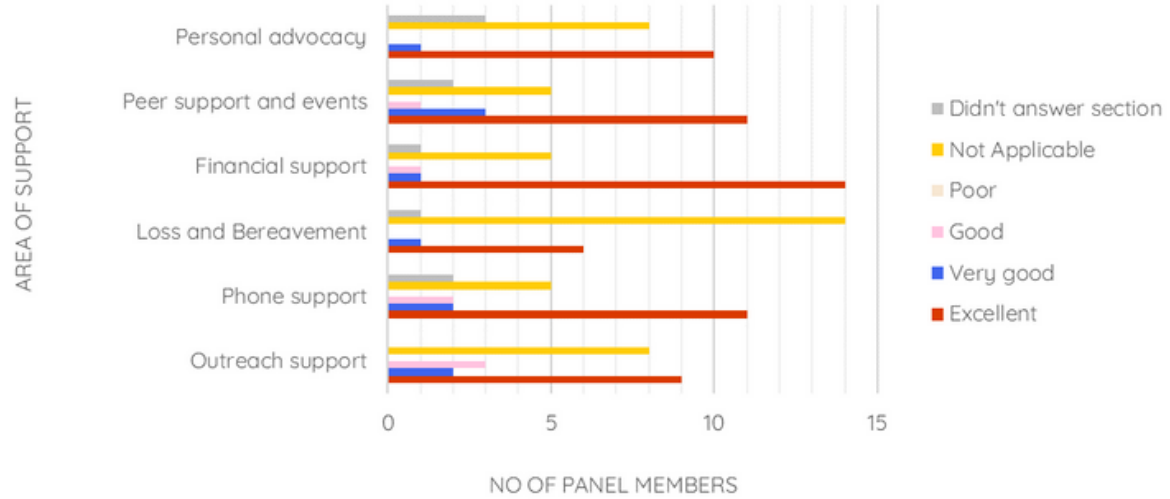
Summary of family support survey: Service review

The following provides only a segment of the broader survey completed by the panel. The full survey will contribute to the service review. 22 individuals participated in the survey.

How often do you access [outreach/ phone/ bereavement/ financial/ peer/ personal advocacy] support?



Describe the quality of support:



Summary of family support discussion: Service Review

Is the family support service meeting the needs of people living with EB?

The panel agreed that the FS service is meeting the needs of people living with EB. comments made were that the service is:

- very accessible
- a great support
- adapted to need of the individual
- well-structured
- vital

Do you think the support service priorities are aligned with yours?

The panel all felt priorities are aligned with theirs.

The following outlines the panel's thoughts on each key area of support discussing needs, accessibility, delivery and suggested improvements.

Outreach

The panel agreed the outreach service is meeting the needs, especially for more severe types.

- Some would like to be offered visits, suggested once a year.
- Some felt it would be of benefit to hire an extra family support worker as there can be a long lead in time for visits.
- There was also a suggestion to offer visit to all the family (extended) at the same time rather than just a household as this might be more cost effective for Debra.
- Other members felt that they did not need the service as the phone service was meeting the need.

Summary of family support discussion: Service Review

Phone support

All agreed the service was meeting the need and is accessible. Some don't use the service after covid or because the need is no longer there.

Loss and Bereavement support

The panel felt this support is very important and meets the need.

- The memorial service is a time to remember and talk about their loved ones.
- There was a suggestion to have the memorial service every year, with one year in September and another early in year so it's more accessible.
- It was also mentioned that the panel is a helpful way for bereaved families to be involved in Debra and focus on something more practical.
- There was also a suggestion for Debra to cover or assist with funeral expenses.

Financial support

The panel were very happy with this support, it's accessibility and delivery.

- It was mentioned that the support allows people to live a fuller life when they're only receiving disability allowance.
- The bereaved famile felt the special circumstance grants are helpful for night nursing, expenses for hospital visits, etc. This support meant they didn't need to worry, and it allowed them to just focus on their family.
- Some members of the panel would like help towards cost of shoes whether that's assisting with getting discounts or offering a grant.
- It was also mentioned that more flexibility with timings on the respite grant would be helpful.

Summary of family support discussion: Service Review

Peer support and events

Members of the panel felt the peer support was excellent mentioning Barretstown and the EB Expert Panel. Bereaved families noted the memorial and a bereaved specific table at the workshop creates a space for them.
Areas to improve:

- older adults would not attend family event.
- events should be more inclusive in terms of location in Ireland.
- it can be apprehensive when you don't know anyone at events. Introduce icebreakers or games for adults at events to facilitate socialising.
- communicate to EB community when Debra fundraisers are on. It was also mentioned walking challenges aren't inclusive for people with EB.

Personal advocacy

Some members of the panel felt this support was perfect with some feeling not all needs were met. Areas needing more advocacy support:

- free travel cards and parking badges
- better communication on advocacy campaigns to people with EB
- accessing adult EB services or more clarity on this t
- accessing bandages and medical supplies
- increase awareness of EB amongst GPs
- increase awareness in schools. Suggestions were:
  - expand/offer alternative school booklets for older ages.
  - advocate for school to not discriminate against hidden EB
  - increase contact with schools as reminder of EB.
  - do an awareness campaign with teachers to get them more involved/understand EB better - like the hot stones walk.
  - school pack to help schools prepare for falls
  - advocate for assisted technology in schools

Family Support

Kate Gibney - Family Support Officer

Kate introduced a new initiative from the family support team to the panel and invited their feedback. The team recently created a draft of an EB medical alert card, intended to be a resource for individuals living with EB. The card is designed to be carried in a wallet and alert medical personnel that the carrier has EB. Kate explained that this is a resource that is currently offered by other international EB organisations and is a common practice across various medical conditions. Before finalising the current draft, the team reviewed and analysed existing cards and collaborated closely with the medical team at CHI.

Kate went on to outline the primary objectives of the card to ensure clarity during the panel's review of the project. These objectives include creating a pause and alerting medical staff treating the person that they have EB, offering a concise overview of EB, and providing guidance on procedures to avoid, with the aim of minimising trauma wherever possible. Additionally, the card will include contact details of the individual's EB clinic, should medical personnel wish to access more information on treating someone with EB and include personal details for identification purposes when communication may not be possible.

Kate highlighted that the primary audience for this card is medical personnel in emergency or hospital settings. Given their likely busy and high-pressure environment, she emphasised the need for the card to be attention-grabbing, clear, concise, and effective in conveying the most crucial information.

The panel went on to discuss the card prompted by questions from the facilitator. For those who did not get time to review the card, Kate followed up after the panel with a questionnaire.

Summary of family support discussion: Medical Alert Cards

1. All participants said they would use the card and felt it was an important initiative, will be useful especially in hospital and feel they will be taken more seriously.

2. The panel agreed the card meets the objective, with a few suggesting additional information needed:

- The card should mention that EB affects internal tissues as well
- Add blood type.
- Add a photo of the person
- A QR code to scan to a Debra webpage describing their specific type of EB and how it can impact a person.
- A "handle with care" note should be included, and it should be obviously positioned at the top of the card.

3. The panel agreed the card works for intended audience however it was suggested that a bracelet alerting to the card may be useful.

4. All participants wanted personal details (name and date of birth) included

5. All participants wanted emergency contact/next of kin details included

6. The panel found the design and colour choices work well, the red worked, and the design was clear and simple. Additionally, there was a suggestion that the Debra orange would also work. It was also emphasised that constructing the card from hard plastic, like a credit card, was important.

7. Most participants would also like a digital version of the card.

Summary of family support discussion: Medical Alert Cards

Additional comments made:

• A suggestion was made to have ambulance services review the card

• Some tables expressed the necessity for a disability card or passport as an additional resource to facilitate their access to disability-related supports, like scooters, wheelchairs, and priority seating on transportation. This card could also serve to inform people, especially airport staff, about the presence of medical supplies, such as needles, carried by the individuals.

Closing Remarks

Sarah Mullins - Research

Before giving the closing remarks, the Debra team played a new organisational video for the EB Experts which has been developed by the Communications team. Sarah then thanked everyone for attending the workshop and said she hoped they enjoyed themselves and that they would continue to engage with the team as EB Experts. She then explained the plan going forward is to hold another workshop in spring of next year, and to approach the panellists individually for smaller tasks between workshops. The frequency of individual tasks will depend on the Debra team's needs as well as on the availability of the EB Experts. Most of these sort of tasks should not take up a lot of time and the majority of them will be remote and will not require travel.

All input provided will be invaluable to the team so Sarah emphasised how grateful the Family Support and Research teams are for everyone's participation. She also reminded the panel that being an EB Expert did not mean they have to say yes to every opportunity that arises and that the Debra team totally understand if they cannot participate in everything that is asked of them. She reminded the panel that for anything they do say 'yes' to, they will be fully supported by a member of Debra staff.

Sarah then asked panel members to fill out a feedback form related to the workshop, which will allow the team to make improvements to these events going forward.

Further Information

For further information on the EB Expert Panel or any information within this report, please contact Sarah:

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Pictured: EB Experts, Researchers and Debra Staff Members who attended the EB Expert Workshop May 2023

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