

Research Strategy 2022-2026

DEBRA UK

Changing lives faster together



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DEBRA UK Research Strategy 2022-2026

Foreword

From gene discovery and cutting-edge clinical trials of therapies to control symptoms and better ways to manage complications like cancer, the research journey for epidermolysis bullosa (EB), also known as ‘butterfly skin’ over the past 40+ years has been remarkable.

During this time, we have invested over £20m and have been responsible, through funding pioneering research and working internationally, for establishing much of what is now known about EB. Our research is now amongst the top 501 UK-based research funders across all diseases and conditions investing in global research.

However, EB is a rare condition, with significantly fewer dedicated EB researchers than there are for better-known conditions, such as cancer and heart disease. That means it takes longer to find a cure. But patients living with EB can’t wait. They need effective control of symptoms, better quality of life and real hope that a cure will be found soon.

We live today in an era of enormous scientific and medical innovation. There are real scientific advances, new technologies, and new science - it is a time of real opportunity for EB research. Breakthrough in research comes from supporting outstanding projects that encourage the best from talented clinicians and scientists.

We need to accelerate the pace and breadth of our research. To achieve this we are committed to increasing our funding and driving a programme of world-class, innovative and collaborative research that will bring hope and improved outcomes for everyone living with EB. Together we can achieve this ambitious and essential journey to change lives and end suffering.



Tony Byrne
CEO, DEBRA UK

¹ Association of Medical Research Charities (AMRC).2021



Our history

DEBRA was the world’s first EB patient support organisation, founded in 1978 by Phyllis Hilton whose daughter Debra had dystrophic epidermolysis bullosa (DEB). When told that nothing could be done for her baby, she set out to find ways to treat Debra’s skin using cotton dressings. We have come a long way since then. All UK patients have access to state-of-the-art dressings, diagnosis is for DEB is routine (although diagnosis of the less severe types of EB is still challenging), and DEBRA is now international in scope with a network of 50 DEBRA groups worldwide sharing information and best practice.

Investing in a future free of EB, DEBRA funds:

- pioneering research
- specialist healthcare
- lifelong community support
- international collaboration and best practice

We work in partnership with the NHS to deliver an enhanced EB healthcare service for people with EB. We fund pioneering research, working with researchers at the best institutions across the world to improve treatments and to search for a cure. We invest in the development of clinical best practice guidelines. We provide practical, financial and emotional support and advocacy for people with EB and their families, connecting patients with the specialist services they need and a support network across the EB community.



Our track record

We are the largest UK funder of EB research, and we are consistently in the top 50 UK funders of research across all diseases and conditions. We have invested over £20m and have been responsible, through funding pioneering research and working internationally, for establishing much of what is now known about EB.

The millions of pounds we have invested in research is all thanks to our incredible supporters who expect us to take advantage of these opportunities to deliver benefits to patients in the short and long term. We fund the best scientists and clinicians from around the world and many of the projects we fund combine the knowledge and skill of researchers from multiple sites across the UK and internationally.

The tremendous efforts of researchers that went into finding treatments, vaccines, and cures for COVID-19 has demonstrated that a global and collaborative approach to research can lead to outstanding results. This approach is also the most effective for EB research, which is why we fund research in partnership with other EB research groups, academia (i.e. universities) and industry (i.e. biotechnology and pharmaceutical). Working together ensures that research is not duplicated or repeated and allows us to spread our funding across more projects around the world.

As a key organisation involved in EB research, we have continued to prove our desire to collaborate through support of global meetings, including the 2019 EB reclassification meeting during which leading experts reviewed the different EB types to better define their characteristics and effects on patients. We organised the first EB World Congress, EB 2020, bringing together the world’s experts in research, clinicians, patients, EB support groups and industry, including over 20 biotechnology companies, all with a focus on cures, treatment and management strategies for EB and a commitment to share state-of-the-art knowledge on EB.

We will continue to mobilise the EB community and build strong partnerships to deliver for people living with EB, driven by our pursuit of impact and patient outputs.



► Our vision

A world where no one suffers with epidermolysis bullosa (EB).

► Our mission

Provide life-long care, while seeking cures, for all those affected/impacted by living with EB.

► Our ambition

DEBRA is the national charity and patient support organisation for people living with or directly affected by EB. We have achieved global recognition as a leader on EB, and are the only UK charity specifically aimed at continuously supporting the EB community. This is our first research strategy to focus on impact and on what matters to people living with EB.

Our ambition is to find and fund treatment(s) to lessen the day-to-day impact of EB and cures to eradicate EB. To achieve this, we are embarking on a new, patient-centred journey from 2022 to 2026, which will be underpinned by our new research strategy.

We will fund science of the highest quality across the world that has the potential to deliver for EB patients.

Executive summary

We have a bold ambition, and we can only achieve it by working together. By encouraging collaborative approaches and seeking partnerships towards patient-driven outputs, we will deliver improvements in the quality of life for EB patients whilst seeking a cure(s) for all types of EB.

DEBRA UK will achieve its ambitions by focussing on research with clear deliverables in the coming years.

Our four overarching research priorities are those we view most likely to deliver outputs for people living with EB. They are:

- invest in drug re-purposing and develop drug discovery programmes to accelerate finding treatments
- increase investment in patient-centred research themes
- continue to invest to understand better the causes and progression of EB and the role of the immune system
- invest heavily in the next generation of EB researchers

To deliver these priorities we will:

- actively seek partnerships with other charities, industry, government agencies and other partners who share common goals in the UK and internationally
- launch new funding schemes to address major questions in EB
- encourage collaborative/multi-disciplinary approaches to take progressive steps forwards
- develop an EB research workforce, attracting, supporting and developing the brightest researchers, at all stages of their careers
- empower patients to engage with research
- communicate research findings to people with EB and their families with accessible, up-to-date and accurate information



Our strategy



The devastating nature of EB drives our determination to do more than we have ever done to achieve our vision and end the suffering that EB brings.

This strategy is the start of our new journey. We want to highlight EB research and EB researchers to the wider world. We are calling on the scientific community, funders and our industry partners to come and join us on this journey to accelerate EB research innovation. To achieve this, we need a step change in our approach to research and how we work with individuals, organisations and government bodies to achieve what we have been working towards for over 40 years.

We will increase our funding and drive a programme of world-class, innovative and collaborative research that will bring hope and improved outcomes for everyone living with EB.

Sagair Hussain

Director of Research, DEBRA UK



A chronic and debilitating skin condition, EB is not constrained by boundaries. As the patient voice organisation for those with EB in the UK, we also cannot be constrained by boundaries in our ongoing search for cutting-edge treatments that will make EB manageable and liveable. Our new strategy puts patient outputs front and centre, with a focus on translational research that will have a positive impact on those with EB today.

Our strategy puts the patient first, concentrating on tangible outcomes and rapid results. In direct consultation with those with EB, DEBRA will ensure that funding is focused on patient outcomes relevant to the day-to-day challenges they face. This strategy also embraces partnerships with specialists in fields that relate to EB, including dermatologists, ophthalmologists, podiatrists, and oncologists, in addition to strengthening our relationships with the pioneering EB centres of the UK. We know that beating EB can be achieved faster working collaboratively and we welcome anyone to join our fight against EB.

Carly Fields

Chair of DEBRA Charitable Purposes Committee (CPC)

Our core research principles

EB patients at the heart of research

Our research strategy is focused on impact and what matters to people living with EB. With their lived experiences, patients and their family members are the experts on the effects of EB.

The key priorities of the EB community - those living and working with the conditions - are essential in shaping the research selection process and driving the future of EB research, balancing short-term benefits for patients and support for long-term research into a potential cure or cures.

People

To achieve our vision, we need creativity and the expertise of scientists, clinicians and health care professionals. Supporting these people at all stages of their careers will lead to high quality research. We will continue to support the best individuals so they can tackle the big questions in EB research.

“The research and dedication of those finding a cure for EB is the hope we, with EB, need. It allows us to think of a time where our days are not dependent on how our skin is managing; where life could be easier.”

Chloe
DEBRA member, Scotland

National and international partnerships

Partnerships are essential to deliver the changes we want to see for EB patients. We share common goals and objectives with other organisations.

We will continue to work with our existing partners whilst engaging with new ones, both nationally and internationally within all sectors: universities, NHS, governmental, industry and other charities who share our goal of beating EB.

Global collaborations

Tackling the big questions in EB research can only be done through a multidisciplinary collaborative approach. Fostering research innovation across disciplines has the potential to leverage knowledge and bring in new and different perspectives in the fight against EB. Networking nationally and internationally will be crucial to achieve the maximum impact from our research.

Our approach

By 2026 we want to see a new EB world, a world where no one suffers with EB. To achieve this we need treatments so that we can improve the quality of life for those living with EB.

Our research strategy outlines what we will do to address areas of high patient need. We will continue to support what we do well and invest heavily in areas where we need to build capacity.

Develop a treatment pipeline to slow, stop and/or reverse EB

EB patients are living with a degenerative condition yet there are no treatments within the NHS, representing an unmet need.

For EB to be stopped it is important we tackle the immune component of the condition. With numerous immune modulatory drugs on the market to treat other conditions, we believe efforts should be put into understanding, identifying, developing and testing potential anti-inflammatory treatments for people with EB. As well as understanding, how the cost of the new treatment can be covered by the healthcare system.

What we'll do

- Fund work to identify and select candidate repurposed drugs for clinical trials.
- Grow our clinical trial programme with the aim of slowing, stopping or reversing progression.

The sooner we do this, the sooner people could feel the benefits in their day-to-day life.

Translational research

The research we fund will only help patients if it can be translated into interventions that benefit them. Successful translational research relies on a collaborative multidisciplinary approach between laboratory and clinical researchers as well as life scientists, engineers, clinicians, data analysts, and commercial partners to accelerate health innovation.

What we'll do

- Set up new funding schemes to support multidisciplinary translational research.
- Develop a therapeutic discovery model.
- Support a broad portfolio of clinical research.
- Launch challenge funding projects to help EB patients gain greater independence.
- Develop partnerships with national and international organisations.

Our approach continued

Increase our understanding of EB

To continue making progress we need to understand more about the drivers of EB, the relationship between genes and proteins, the role of the immune system and the progression of cancer. This will determine everything we do, from developing diagnostics to designing treatments and cures.

What we'll do

- Continue to invest and strengthen this area.
- Fund research into the various types of EB – Simplex, Dystrophic, Junctional and Kindler
- Seek to understand the causes, nature and ultimately find a cure for EB

EB therapies - cure

EB is a complex group of skin disorders. Each EB type and sub-type requires different treatments, and each patient may need a range of therapies. Our aim is, ultimately, to find a cure(s) for all types of EB.

What we'll do

- Continue to fund pioneering research into gene, protein, and cell therapies.
- Seek partnerships opportunities in these areas so we can leverage funds.
- Actively seek collaborations with national and international organisations with common goals.

Develop EB research leaders for tomorrow

To support EB research, we need to build a strong community of highly trained, innovative researchers. This means there needs to be a clear career pathway into and through EB research at each step of the way.

We will provide support at every stage in order to attract, retain and support not only existing researchers but also new researchers into the field. This also applies to researchers from other fields who wish to move to EB, ensuring that they also have a clear pathway to enter EB research.

This will ensure we inspire the next generation of EB researchers to become leaders of the future.

What we'll do

- Introduce grant rounds to fund PhD students and career development fellowships
- Increase capacity where it is needed

Partnerships

The tremendous effort of researchers investigating treatments, vaccines and cures for COVID-19 has demonstrated that a global and collaborative approach to research can lead to outstanding results. This approach is also the most effective for EB research, which is why we fund research in partnership with other EB research groups, academia and industry.

We can all achieve more by working together.

What we'll do

- Continue to work with our existing partners to increase investment in EB research
- Seek partnerships opportunities in areas of strategic priorities so we can leverage funds
- Foster opportunities to collaborate nationally and internationally.
- Partner with organisations outside of our traditional focus to bring in new ways of thinking, ideas and expertise into EB

Ultimately, we will work with national and international partners to make the biggest difference to people living with EB.

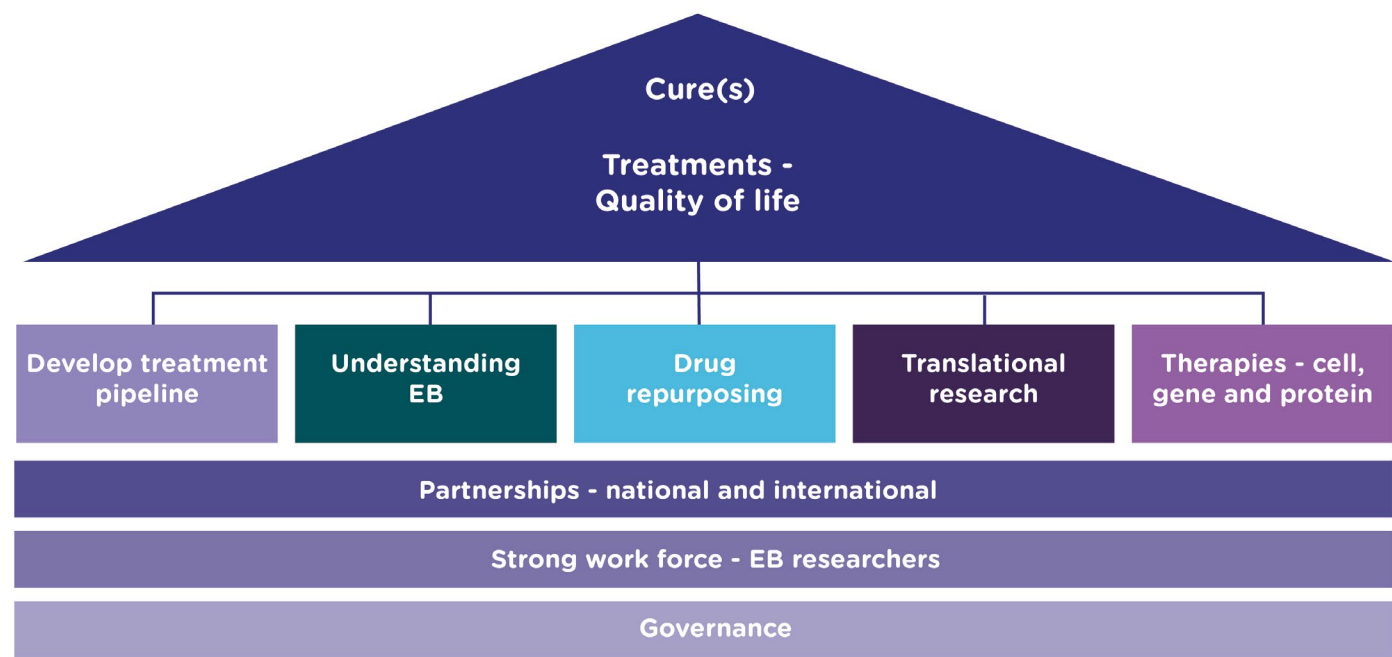
Governance

As a member of the Association of Medical Research Charities (AMRC), it is essential we uphold the highest standards of best practice in research governance. Peer review is therefore essential to DEBRA as a funding organisation. All research proposals will be examined by independent experts who will consider the importance and potential of the proposal as well as the cost effectiveness of the research proposed.

What we'll do

- Our Charitable Purposes Committee (CPC) will provide advice on the strategic direction
- We will develop milestones against our strategic priorities and the CPC committee will input on the progress on a regular basis
- We will seek independent external advisors to remain responsive to innovations and new opportunities in EB research
- The research we fund will be reviewed by an independent medical and scientific advisory panel.
- Our research portfolio will be overseen by the CPC committee consisting of experts within and outside of the EB field including people living with EB.
- We will continue to be a member of the AMRC to uphold the highest standards of best practice in research governance.

Summary of our research strategy



“As a parent of a child living with EB, it is incredible to see that research that was science fiction when my daughter was born is now part of the solution. Isla has faced many challenges with her pain; she wants a cure, as do many others who suffer from this condition. My hope for EB research is to make what was once impossible, possible. I want a brighter future for Isla. I want a cure to happen in her lifetime.”

Andy Grist
DEBRA member and parent



Our funding programmes

Applications are welcome from all disciplines committed to improving the lives of people with EB. Funding for researchers will be provided through the following schemes.

Project grants

Awards up to three years will be available to researchers through our research calls. Funding will be judged on relevance to EB, scientific merit and novelty. Applicants must demonstrate the potential benefit to EB patients in the research proposal.

Programme grants

Awards will be provided where collaboration between several groups is required such as clinical projects adopting a multicentre approach.

PhD studentships

PhD grant awards for 3-year projects to develop degree graduates into EB researchers. The criteria will be the same as for project grants with the addition of quality of research environment and training for students.

Clinical research fellowships

Awards for registered healthcare professionals to establish themselves as independent investigators to run their own group and develop their own research interest in EB. The aim is to create future leaders in the EB field.

Non-clinical research fellowships

Awards to support post-doctoral researchers at early and intermediate stages of their career to establish themselves as independent investigators to run their own group and develop their own research interest in EB. The aim is to create future leaders in the EB field.

Small grants

Small grant awards support small pilot studies such as generation of preliminary data or feasibility studies which would not normally attract funding. The aim is to make novel ideas more competitive for larger follow up funding grants.

“ We will increase our funding and drive a programme of world-class, innovative and collaborative research that will bring hope and improved outcomes for everyone living with EB. We are calling on the scientific community to join us on this journey to accelerate EB research innovation.

Dr Sagair Hussain
Director of Research, DEBRA UK

General principles

The following are broad general principles:

Work being funded

- All applications will be judged on scientific merit. Successful applications must be of the highest scientific excellence, including the research team and publication record
- The key criteria will be scientific and treatment potential in relation to EB. Applications must have clear aims and objectives to advance scientific understanding of EB and therapeutic interventions. Projects must be feasible and achievable in the stated time period
- Researchers will provide annual scientific and financial reports which compare research progress to the original aspirations. A lay report will also be required as part of the annual progress report
- At the end of the grant period, a final report will be required, including a lay summary, along with publications in peer-reviewed journals

Assessment and award process

- All applications will be considered by the Charitable Purposes Committee (CPC) and External Peer Reviewers of DEBRA
- The membership of the CPC consists of experts within and outside of the EB field including people living with EB
- External reviewers will be recruited from across the academic community
- The Medical and Scientific Advisory Panel will adhere to the Terms of Reference outlining membership, tenure, quoracy and conflicts of interests

- The Chair of the CPC will take recommendations to the trustees of DEBRA who will make the final decision on awarding grants
- Grants will be advertised between 1st February and the 31st of March each year and will then be reviewed by DEBRA’s Scientific Grants Advisory Panel with funding recommendations then presented to the Trustees of DEBRA for approval or further comment. Applicants will receive a final funding decision in November. Projects will therefore start the following year.
- Payments will only be paid quarterly in arrears upon receipt of an appropriate invoice

Publication

- Copies of all published papers, submitted manuscripts, conference abstracts and posters should be sent to DEBRA through the grant period

Publicity, engagement and communication with DEBRA

- Publicity is vital to DEBRA in helping to raise funds and communicate our message. It is therefore expected researchers will work with DEBRA to co-ordinate and maximise publicity and communication opportunities arising from the grant

Future developments

- Develop a Lay Panel to help review applications from a patient perspective
- Establish EB patient research priorities

Measuring progress and success

Our research strategy will be guided by a focus on impact and what matters to people living with EB. Through their lived experience, patients and their family members are the experts on the effects of EB.

Assessing the impact of our research strategy on people living with EB will ensure we are delivering for the EB community. We will review and refine our approach over time to drive the future of EB research, balancing short-term benefits for patients and support for long-term research into a potential cure or cures.

In 2024 and 2026 we will publish a research impact report assessing progress against our strategy, to evaluate achievements, and to detail any changes in approach that may be required.

Investing in research is investing in the future.



General principles

At DEBRA, we can't change the future of epidermolysis bullosa (EB) in an instant. But we do know that an instant can help change the future of people on a life-long journey with EB.

We believe that anyone can effect change with one small action:

- One action on behalf of the EB community could bring invaluable insight that...
- Builds our relationships with experts who share our passion for finding life-changing solutions so that...
- Directs our energy and focus onto an essential unmet need which...
- Our community becomes more hopeful, connected and mutually supportive and we...
- Enables us to tailor our support and services effectively so that...
- Strive to make life-long improvements that enable anyone affected to enjoy pain-free, fully-lived lives.
- Our members feel empowered to take as much control over their lives as possible which...

Let us never underestimate the simple things we can do to spread ripples of hope for people living with EB. Let us all take one action to fight EB so that, one day, EB no longer has any fight left to give.

Help us begin... the DEBRA effect.

The EB community is at the heart of all we do. Their lived experience drives our commitment to do more to increase our programme of world-class research and make what was once impossible, possible. We want a future where everyday treatments end the pain and suffering of EB and a cure becomes a reality.

To find out more about our research:

- ✉ please contact research@debra.org.uk
- 🌐 or visit our website: www.debra.org.uk



DEBRA funds pioneering research, specialist healthcare, international best practice and lifelong care and support to the EB community.

We can't change the future of epidermolysis bullosa (EB) in an instant. But we do know that an instant can help change the future of people on a life-long journey with EB. Let us all take one action to fight EB so that, one day, EB no longer has any fight left to give.

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