DEBRA UK Research Impact Report 2021

Pioneering research for a future free of EB

www.debra.org.uk

debra
for people whose skin doesn't work we do

“As a parent of a child living with EB, it is incredible to see that research that was science fiction when Isla was born is now part of the solution. Isla has faced many challenges with her pain and she is tired; she wants a cure, as do many others who suffer from this condition. This is an upsetting and painful fight to go through and to watch.

The EB scientific and medical community increasingly believe they can solve the puzzle, that they can and will cure EB. They increasingly know where they need to be heading and are working out how and what they need to get there.

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 & Isla, DEBRA Members, Scotland

Andy & Isla, DEBRA Members, Scotland

It’s time for a cure

Join our journey to change lives forever

Our vision is a world where no one suffers from the painful skin condition Epidermolysis Bullosa

From gene discovery to cutting edge clinical trials of therapies to control symptoms and better ways to manage complications like cancer, the research journey for EB over the past 40 years has been remarkable.

During this time, we have invested over 20m and 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 15 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 more well-known conditions, such as cancer and heart disease. That means taking longer to find a cure. But patients living with EB can't wait. They need effective control of symptoms, a better quality of life and real hope that a cure will be found soon. For themselves and their children.

We are living in an era of enormous scientific and medical innovation. There are real scientific advances, new technologies, new science with EB at the cutting edge. It is a time of real opportunity for EB research. Breakthrough in research comes from supporting outstanding projects that encourage the best of talented clinicians and scientists.

We need to accelerate the pace and breadth of our research. This means 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
Chief Executive Officer

“The worst thing about EB is the pain. The pain is incredible. I have to take so many painkillers every day –sometimes the medication is so extreme it puts me to sleep. Sometimes I have days where I just can’t stop itching. I find it difficult to eat a lot because that also causes blistering. My skin scarring, the fusion of my fingers and the depletion of my skin tissue all increase as I get older which will make life even trickier for me.

Some people living with Recessive DEB (RDEB) have a high chance of developing a squamous cell carcinoma (a form of aggressive skin cancer) before the age of 35. I want to find a cure for EB if DEBRA hasn’t found one by the time I’m old enough to be a doctor.”

Fazeel, living with Recessive Dystrophic EB (RDEB)

What is EB?

Skin as fragile as a butterfly wing

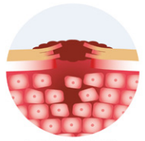
EB, Epidermolysis Bullosa, is a complex and rare group of genetic skin conditions that cause the skin to tear and blister at the slightest touch. The name comes from ‘epiderm’ - the outer layer of skin, ‘lysis’ - the breakdown of cells and ‘bullosa’ - blisters. EB can impact the external skin and also internal linings e.g. mouth, throat, eyes, anus and organs.

Can affect internal organs

People living with EB have a faulty gene which means the skin cannot bind together so that any friction causes it to tear apart. Every person has two copies of each gene – one passed on from each parent. EB may be inherited as either dominant (only one copy of the gene is faulty) or recessive (both copies of the gene are faulty). Parents have a 50% chance of passing on a dominant form of EB to their child, whereas the chance of passing on a recessive form of EB drops to 25%. Both parents may carry the gene without knowing or displaying any symptoms.

Genetic condition

There are four main types of EB – EB Simplex (EBS), Junctional EB (JEB), Dystrophic EB (DEB) and Kindler Syndrome.



Layers of skin tear and blister

Symptoms vary from mild to severe depending on the type of EB. In its most severe forms, EB is fatal; even in its mildest forms it can cause lifelong disability and pain. Blisters continuously form and have to be drained and dressed daily, a painful process that can take several hours. The build-up of scar tissue can cause fingers and toes to fuse together. It can mean a higher risk of developing skin cancer.

Not contagious

People living with EB may require treatment from a range of medical specialists aimed at symptom relief. The most common challenge amongst all types of EB is the pain and itching that occurs as a result of the blistering.

No cure

There are an estimated 5,000 people affected by EB in the UK and 500,000 worldwide. It is currently incurable.

Who we are

DEBRA is the national charity supporting those 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.

DEBRA was the world's first EB patient support group, founded in 1978 by Phyllis Hilton whose daughter Debra had Dystrophic EB. 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 of EB is routine and DEBRA is now international in scope with a network of nearly 50 DEBRA groups worldwide sharing information 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.

Investing in a future free of EB, DEBRA UK funds:

- Pioneering research
- Specialist healthcare
- Lifelong community support
- International collaboration and best practice

A journey of discovery

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 been responsible, through funding pioneering research and working internationally, for establishing much of what is now known about EB.

Now is the time to accelerate the pace of discovery, reduce the pain and challenges of life with EB and save lives.

40 Years
Funding EB research

134
Research projects

107
Researchers

52
Research sites

12
Countries

£20,000,000
invested to date.

40 years of pioneering EB research

1978
First EB patient group set up

1981
First EB research project funded

1992
First EB genes identified

1995
DNA-based prenatal diagnosis developed

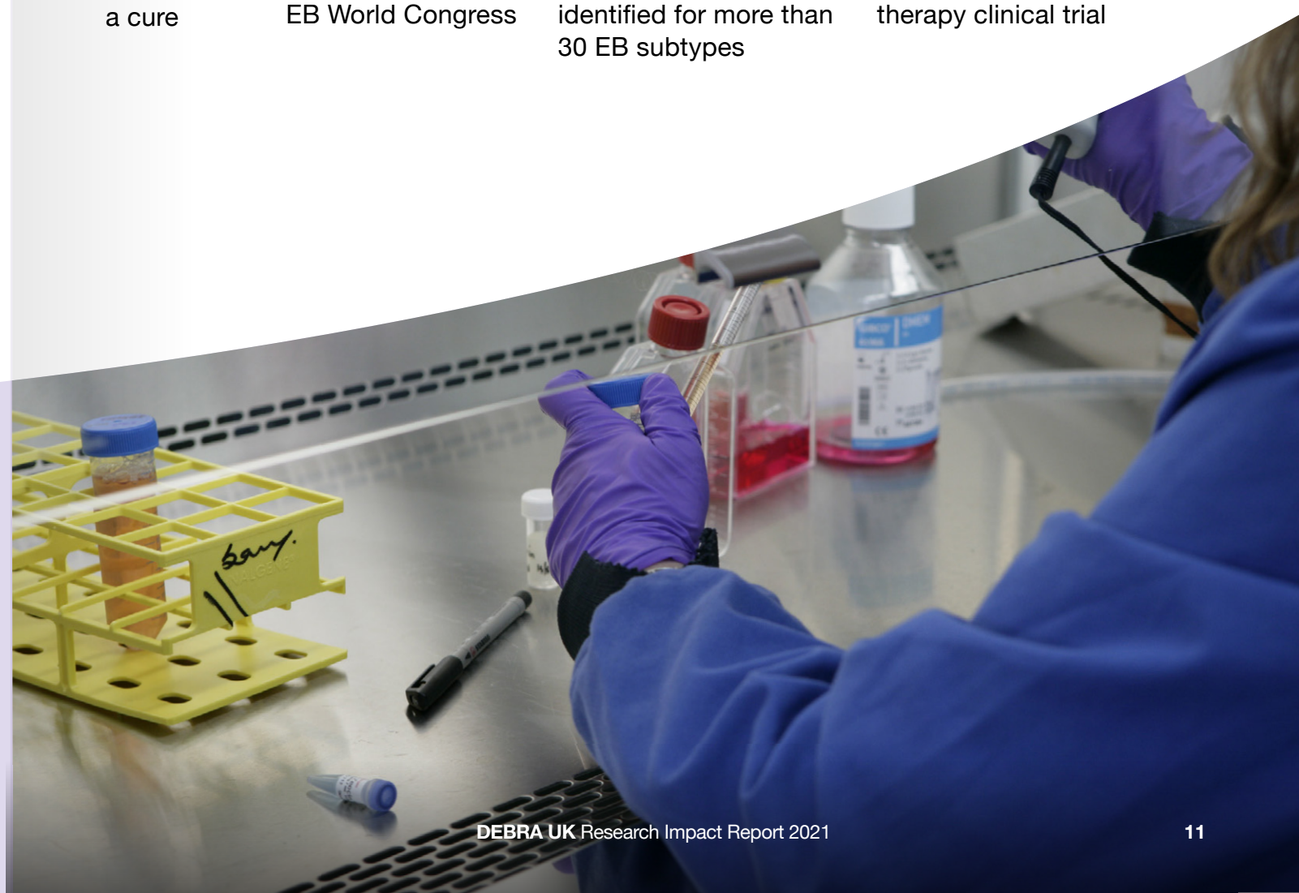
2000
First EB cancer projects funded

2002
First EB gene therapy clinical trial

2012
Up to 18 genes now identified for more than 30 EB subtypes

2020
First international EB World Congress

2021
Time to find a cure



EB research changes lives

Laboratory diagnosis is crucial in determining the type of EB and understanding how to treat and manage the condition. Our funding has helped establish a model EB diagnostics centre in London, which has made it possible to receive prenatal diagnostic testing if they are at risk for passing on EB, ensuring a parental choice.

“EB is inherited and we were both carriers of the gene. This meant that it was likely our baby would have the same type of EB as Dylan had. Thanks to advances in EB research and funding from DEBRA, we were able to test our baby for EB before she was born. Knowing we would not have to see our new-born go through the same pain and distress Dylan did was a massive relief. Our baby girl was born on 4 November 2018, EB free.

We promised Dylan before he passed away that we would always continue to #FightEB. Research into EB is so very important and gives us hope that one day there will be a cure into this devastating condition.”

Karen and Simon, son Dylan and daughter Katie Rose

Our research priorities

We want everyone living with EB to have the best care, support and treatment available right now. We are also committed to finding a cure and to ending this disease.

Cure, control and quality of life

The three pillars of our research strategy are cure, control and quality of life. 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 for all types of EB. In parallel we are committed to driving forward research to improve the quality of life for patients. We have achieved so much in the past 40 years, with successes such as pre-natal diagnosis available now. But we still need better treatments to help ease and control symptoms such as wound healing, pain relief and cancer therapy, reduce itch and prevent risk of complications such as infection.

The voice of the EB Community

Patients and their family members through their own experiences are the experts on the impact 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.

High quality research

We are proud members of the Association of Medical Research Charities (AMRC), which demonstrates that our research governance is robust and we're confident every project we support is bringing us a step closer to a treatment for EB. All projects undergo a thorough application and review process by a panel of medical and scientific experts (MSAP) to ensure they meet our priorities and are judged on potential results, guaranteeing that we only fund high-quality projects. This process ensures new research projects build on existing knowledge, which helps us to invest only in projects that give the best chance of breakthroughs leading to change.

The more projects we can fund that meet these criteria, the sooner we can find a cure(s) and more treatments to improve the quality of life for people living with EB.

Dr Sagair Hussein, Director of Research, DEBRA UK

We will strive to:

- ✓ Discover the causes of EB and its impact for people living with the condition.
- ✓ Develop better treatments that will improve the quality of life for EB patients.
- ✓ Evaluate every research project and review progress regularly to ensure we fund high-quality projects.
- ✓ Boost opportunities for success by encouraging more investment, increasing our research programme and working with more EB patients.

International collaboration

The research we fund is world-class, and that's because we don't just fund UK scientists and clinicians but the best in the world. Many of the projects we fund combine knowledge and skill from researchers at multiple research sites in the UK and internationally.

The tremendous effort of researchers looking into 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 supporting 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 the EB community and a commitment to share state-of- the-art knowledge on EB.

It is vital for us to continue to drive the global effort to fight EB.

Some of the 52 research institutions we have funded include:

- **King's College,** London, England
- **Cancer Research UK Beatson Institute,** University of Glasgow
- **Thomas Jefferson University,** Philadelphia, USA
- **University of Dundee,** Scotland
- **University Medical Center Groningen**, Netherlands
- **Massachusetts Institute of Technology (MIT),** USA
- **Great Ormond Street Hospital,** London, England
- **University of Oxford,** England
- **Medizinische Hochschule,** Hanover, Germany
- **University College London,** England
- **Netherlands Cancer Institute (NKI),** Amsterdam
- **Istituto Nazionale Genetica Molecolare,** Milan, Italy
- **Swiss Federal Institute of Technology,** Zurich, Switzerland
- **FIBRX Derm Inc. (Biotech),** Boston, USA
- **University College Dublin,** Eire
- **University of Freiberg,** Germany
- **Istituto Dermopatico dell'Immacolata,** Rome, Italy
- **University of Santiago,** Chile
- **Birmingham Dental School & Hospital,** England

"Global collaboration is absolutely vital because we have so many experts around the world across various continents working on not just EB but also people from different specialities such as oncology and haematology and combining different approaches. Thinking outside the box and working together will speed up the research and the effectiveness of what we can achieve together."

Dr Su Lwin, Researcher

Our current projects

Our current portfolio of research projects includes pre-clinical laboratory work, research into gene and cell therapies and drug re-purposing, as well as projects driving a change in symptom relief for wound healing and cancer therapy.

It is critical to ensure there is a balance of research into all types of EB represented in our community. We also support fellowships, helping to develop and shape our researchers and clinical experts for the future.

Find out more about our research at debra.org.uk/research

We receive no statutory government funding but our amazing supporters, volunteers and staff give us unwavering support, generating income from fundraising and our retail shops to prioritise and deliver investment for research into cure and care.

In 2020, we made our first investment into a biotechnology firm, FIBRX Derm Inc. in support of a study that aims to continue research (originally funded by DEBRA) investigating a naturally occurring protein that may serve as a viable anti-scarring therapy for people living with DEB (Dystrophic EB) and which could be administered topically. The company has received financial support from the US Department of Defense and multiple EB groups across the world, supporting our desire to take the lead in international collaboration.

In the next few pages are some of the pioneering projects we are currently funding in essential areas:

- Symptom relief
- Wound healing
- Cancer therapies
- Gene & cell therapy
- Data collection

5,000

people with EB in the UK

500,000

people worldwide

Gene and cell therapy

A revolutionary gene therapy using spray-on cells with a protein that glues the skin together.

Researcher – Dr Su Lwin

Research site – Kings College London, England, UK

Total awarded – £174,023

DEBRA website link: https://bit.ly/2SAV7T5

Recessive dystrophic epidermolysis bullosa (RDEB) is caused by damage in a gene called COL7A1 that controls the production of a protein – type 7 collagen (C7) in the skin. C7 is an important sticky protein that glues the skin together, in RDEB the lack of C7 leads to blisters.

Dr Su Lwin and her group are planning to restore the COL7A1 gene using gene therapy to deliver it into the patient's own skin cells called fibroblasts. Fibroblasts cells are found in the skin and they make collagen and other proteins that make the skin healthy. They plan to test this new gene therapy by spraying the restored cells onto the patient thereby avoiding more invasive procedures.

"The vision is that the patient would come to the clinic. We would then clean the wound sites, which are chronically infected and prone to all sorts of pain, itch, and further infections. And then spray this gene-corrected cells back onto the skin with the potential for faster healing and prevention of infections and so on."
Dr Su Lwin, Researcher (England)

Protein therapy

A skin cream using a human protein that could improve healing and stop wounds scarring. Wounds and pain and the risk of infection they cause are one of the most debilitating aspects of EB.

Researchers – Dr Mark de Souza / Professor Jean Tang

Research site – FIBRX Derm Inc., Berkeley, CA, USA

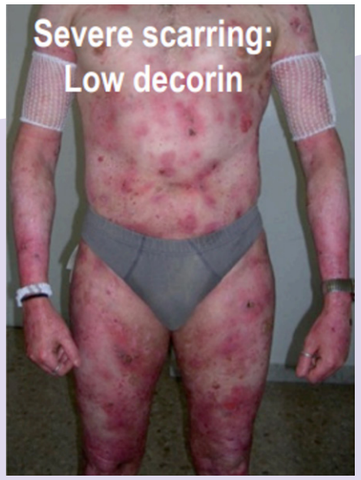
Investment - £192,000

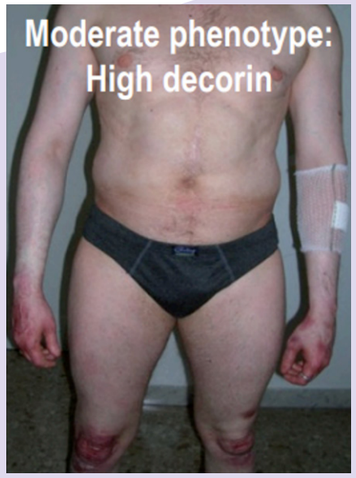
DEBRA website link: https://bit.ly/3f1sqWX

Funded jointly with DEBRA and other organisations, the Fibrx team are exploring a new topical anti-scarring therapy for Dystrophic Epidermolysis Bullosa (DEB) using the human protein decorin.

In an amazing case study of identical twins with RDEB, one of the twins had twice as much decorin protein in his skin and moderate skin scarring compared to his twin brother who had severe skin scarring.

Decorin has been shown to reverse skin scarring by inhibiting factors that cause scarring and decrease inflammation. The Fibrx team are exploring the opportunity of using decorin in a cream/gel, which patients can apply onto the wound to stop scarring and improve healing in RDEB patients.





Gene therapy

A potential treatment for EB Simplex using gene ‘silencing’ technology to switch off a faulty gene to allow the normal gene to work properly.

Researchers – Professor Robyn Hickerson / Dr Peter van den Akker

Research site – University of Dundee, Dundee, Scotland, UK

Total awarded – £635,251

DEBRA website link: https://bit.ly/3eYVG0p

About 70% of EB cases are classified as EB Simplex (EBS), which is caused by mutations in the genes that manufacture proteins called keratin 5 and keratin 14 (KRT5 and KRT14). Keratins are vital to ensure a strong and healthy skin.

EBS is characterised by persistent blistering and poor healing of the skin both internally and externally. Genes are inherited, one copy from each parent. Only one copy of the gene needs to contain a mutation to cause EBS – these are called dominant genes. By selectively suppressing the expression of the faulty copy of the gene, this allows the normal copy of the gene to work properly, a strategy that could be developed into a treatment for EBS.

Dr van den Akker and his team have been working with the pharmaceutical company WAVE Life Sciences to develop methods to use gene silencing technology to switch off the mutant gene. They have been able to silence KRT14 in human skills cells grown in the lab and hope to eventually take it to clinic.

> “The blisters are likened to third degree burns and can form anywhere on my feet, in between my toes and under my toe nails. My feet are often covered so on the face of it nothing appears to be wrong with me as my symptoms aren’t visible. EB isn’t just a physical condition, it’s all consuming and mentally draining.”
>
> **Tom, living with EB Simplex (EBS)**

Drug therapy

A cancer treatment drug with the potential to stop the growth of squamous cell carcinoma.

Researchers – Dr Andrew South (United States) and Professors Johann Bauer (Austria) and Jemima Mellerio (United Kingdom)

Research site – Thomas Jefferson University, Philadelphia, USA; Paracelsus Medical University, Salzburg, Austria; St John’s Institute of Dermatology, St Thomas’ Hospital, London, UK

Total awarded - £470,490

DEBRA website link: https://bit.ly/3vIcBLs

The skin cancer Squamous cell carcinoma (SCC) is the biggest cause of death in patients with recessive dystrophic epidermolysis bullosa (RDEB). Although, we are beginning to understand why patients develop this fatal cancer, therapies which target RDEB SCC are urgently required.

Following on from a previous DEBRA-funded study, Dr. Andrew South (United States) and Professors Johann Bauer (Austria) and Jemima Mellerio (United Kingdom) began a Phase II trial to test the safety of Rigosertib, a drug which has the potential to stop the growth of SCC – one of the current research priorities set by the EB Community.

Rigosertib also has wider clinical reach and is being used in a number of trials for other cancers. The purpose of this study in EB is to test if the drug can be tolerated by EB patients and administered without significant impact to daily routines.

Researchers are working alongside the biopharma industry to see if this drug could potentially alter the course of treatment and prognosis of EB patients diagnosed with skin cancer. If successful, this trial could be the first step in finding an effective treatment for SCC in RDEB. An effective treatment may make a real difference to the quality of life for people facing this cancer.

PROJECTS

Symptom relief

A study into the potential use of cannabinoid-based medicines to manage pain and itch without the side effects of some treatments.

Researchers – Professor André P. Wolff / Dr Nicholas Schräder (initiated by Professor Marcel Jonkman, deceased)

Research site – University Medical Center Groningen, Groningen, Netherlands

Total awarded – £159,025

DEBRA website link: https://bit.ly/3vRPkGS

Even the smallest changes can make a massive difference to improving someone's quality of life, which is why we continue to fund research for effective treatments and therapies to help manage the symptoms brought on by EB. The most common and bothersome symptoms of EB, no matter the type, are pain and itch and most people living with EB will suffer from both to some degree due to the constant blistering. Tackling these symptoms is a high research priority so that we can help reduce, or even eliminate, some of the suffering.

Researching symptoms comes with a range of challenges – one of which is that everyone is unique, making it difficult to compare individuals who have varying levels of thresholds and tolerability (e.g. what causes pain for one person may not cause pain for someone else). Much of this research relies on first-hand experience provided by EB patients, which is why many of the symptom relief projects directly involve members of the EB Community.

Though symptom relief was designated a priority topic in 2017, we have been funding research into this area – including pain and itch – for more than 15 years, and we are regularly reviewing new ideas and approaches to treatment. Many people living with EB require more than one medicine to treat their symptoms, which can often lead to unwanted side effects. Unofficial reports from some EB patients suggest cannabinoid (a component of the cannabis plant) based medicines (CBMs) may be an effective way to manage both pain and itch and avoid other side effects.

Requested by the EB Community, we are funding a study based in the Netherlands (where cannabis is legal) that aims to review the safety and effectiveness of CBMs to treat the symptoms caused by EB. This proof-of-concept study (a project designed to see if something works in a small number of patients before including a larger group) aims to gain insight whether CBMs can ultimately improve the quality of life for people living with EB with hopes of producing evidence-based guidelines for managing symptoms with CBMs.

Wound healing

Project to understand the bacteria which in EB may cause infections, delay wound healing and result in scarring and the potential to re-establish the 'healthy' bacteria in the immune system.

Researcher – Professor Iain Chapple

Research site – Birmingham Dental School and Hospital, Birmingham, England, UK

Total awarded – £296,289

DEBRA website link: https://bit.ly/3f2FKu6

Professor Iain Chapple and his group are investigating the different bacteria that are present on the skin of people with Epidermolysis Bullosa (EB). The human body has twice the number of bacterial cells compared with human cells hence we are actually a complex mix of human and bacterial elements, and health requires our immune system to live in harmony with our bacteria. Most of these bacteria are friendly. However, in EB wounds, bacteria may change and cause infections, delay wound healing, resulting in scarring.

The human body has specialized immune cells to protect it against infections. In health, we have health-promoting bacteria, that live quite happily with our immune system, however, if the environment changes (by for example trauma that causes a blister), different bacteria can start to grow and this can also upset our immune system. n some diseases, when this happens, the immune cells do not function as they should and can over-react to certain bacteria, in a way that also damages our tissues and can delay wound healing.

The project is to understand whether particular immune cells, called neutrophils, work properly in Epidermolysis Bullosa and this may help with treatments that can improve their ability to get rid of the bad bacteria, allowing the healthy bacteria to come back and re-establish that important balance between our "healthy" bacteria and our immune system.

> "EB is a painful, difficult condition which causes wounds that are like second degree burns but never heal."
>
> **Myra, DEBRA member, England**

PROJECTS

Cancer treatments

A ground-breaking study into blocking molecules ‘signalling’ to stop cancer growth.

Researcher – Professor Gareth Inman

Research site – Cancer Research UK Beatson Institute, University of Glasgow, Scotland, UK

Total awarded – £221,139

DEBRA website link: https://bit.ly/3f3AGpd

A high proportion of RDEB patients develop the skin cancer, cutaneous squamous cell carcinoma (cSCC), which is often fatal. Cells within the body rely on specific signals for survival and growth. In cancer these signals are either lost or can be increased causing cancer and its spread.

Professor Gareth Inman and his group have shown a specific signalling system, involving a molecule called Transforming Growth Factor- beta (TGF-β), is increased in the skin of people with RDEB. Importantly, blocking TGF-β signalling can stop cancer cell growth in 50% of RDEB samples tested. Interestingly, blocking the signaling pathway can also promote cancer cell growth in certain RDEB cSCC samples.

The group are trying to understand how TGF-β stops cancer growth in some RDEB patients whilst helping cancer growth in others. This will help to identify the right treatment for the right patient at the right time.

Data collection

Evaluation into long-term disease progression of RDEB that will inform the use of clinical trials to measure new treatments for all types of EB.

Researcher – Professor Jemima Mellerio

Research site – Guy’s and St Thomas’ NHS Foundation Trust, London, England, UK

Total awarded – £734,790

DEBRA website link: https://bit.ly/33oqqTq

A team of DEBRA-funded researchers across England is evaluating the long-term impact of RDEB in efforts to better understand the progression of the condition. The project, led by Professor Jemima Mellerio in London, is helping to identify how EB clinical trials could be measured to determine how well a new treatment is doing. The project has highlighted a need for better understanding of the disease progression including assessment of laboratory, clinical, quality of life and social and economics.

Clinical data are now being collected in a consistent way and the information stored on an electronic database to monitor and review many aspects of an RDEB patient’s life: demographic details, growth and development information, clinical treatments and therapies, and more.

In obtaining these data, this study will ultimately help identify meaningful endpoints to form future clinical trials needed for all types of EB.

You can find out more about our research priorities and projects at debra.org.uk/research

PROJECTS

Clinical trials

Trials using cell therapy to improve understanding and treatment of severe itch.

Researcher – Professor John McGrath and Professor Jemima Mellerio

Research site – Guy's and St Thomas' NHS Foundation Trust, London, England, UK

Total awarded – £497,360

DEBRA website link: https://bit.ly/3tw7Yme

Itch is a common and distressing symptom and better control of it is a research priority. Currently, we do not know what causes the skin to be itchy in EB patients although we recognise the current treatments are not particularly effective.

Researchers have previously shown using cells bone marrow (mesenchymal stromal cells, MSCs) from unrelated healthy donors and infusing them into children and adults with RDEB had a positive benefit on wound healing, skin pain and itching. They now plan to use the same cell therapy to people who have the very itchy form of dystrophic EB known as EB pruriginosa.

The overall aim of the work is to improve understanding about (a) what causes itch in EB pruriginosa (and potentially other forms of EB), (b) whether intravenous infusions of MSCs offer a helpful treatment for people with EB pruriginosa and (c) how do the MSCs work in reducing itch in EB pruriginosa.

"It's like my whole body is on fire and I have ants crawling all over me. It's so overwhelming and no one without EB can understand how bad the itch actually is."

Maya, living with Recessive Dystrophic EB (RDEB)

Clinical fellowships

A multi-year project investigating inflammation, scarring and wound healing.

Researchers – Professor Adrian Heagerty / Dr Ajoy Bardhan

Research site – University Hospitals Birmingham NHS Foundation Trust, Birmingham, England, UK

Total awarded – £125,263

DEBRA website link: https://bit.ly/3xXjsCW

A multi-year project in Birmingham, England with Dr Ajoy Bardhan is currently underway. Working with other prominent and DEBRA-funded EB researchers, Dr Bardhan investigates wound healing in EB with a focus on inflammation and scarring. Through accessing and making use of cutting-edge facilities, his work aims to develop new therapies and methods of support for people living with EB.

"The EB community has clearly identified wound healing as an area of focus where advances in research could be more readily and rapidly translated into improvements in quality of life for patients. We are fortunate in Birmingham to now have a number of facilities dedicated to these very processes and will hopefully bring real benefits to patients in the near future."

Dr Ajoy Bardhan

The future for our EB research

Accelerating the pace and remit of our research is essential if we are to make life for the 500,000 people with EB not just endurable but pain free and bring the reality of a cure closer. This means increasing our funding to drive a programme of ambitious, world-class research to change lives and end suffering.

We are living in an era of enormous scientific and medical innovation. It is a time of real opportunity for EB research. We need to support outstanding projects that encourage the best of talented clinicians and scientists. However, EB is a rare condition, with significantly fewer dedicated EB researchers than there are for more well-known conditions, such as cancer and heart disease.

Continuing and accelerating our EB research will rely on:

- new scientists entering the field of EB research
- current researchers using existing skills in other areas to undertake research relevant for EB
- clinicians using their medical knowledge and further training in research.

As part of our strategy to encourage those who have not previously been involved in EB research, we also fund fellowships, i.e. research conducted by a doctor to explore a particular subject area or special interest. Since the early 2000s we have funded EB research fellowships covering key areas including diagnosis, cancer, gene therapy, ophthalmology and wound healing.

With your help we can do more. We will encourage the involvement of the EB community, increase the collaboration with the best institutions and researchers worldwide and, together, create a world where no one has to suffer from EB.

We will continue to innovate, accelerate and collaborate to work towards a future free of EB.

On top of our current work in EB research, we are committed to doing more to:

Ensure the patient voice is central to all EB research.
We want to grow the pool of patients taking part in the DEBRA International Research Involvement Network and helping to develop research by providing lived experiences, giving feedback on research proposals, contributing to developing training materials and making sure research information is easy to understand.

Help EB professionals understand how EB progresses over a lifetime.
We want to invest more in the international EB patient registry, which collects data on the demographics of EB (e.g. type of EB, symptoms, pain levels, etc.) to understand trends and commonalities amongst EB patients. This data will help support research studies, identify patients for clinical trials and give more accurate prognosis (e.g. survival rates) for people living with the condition.

Partner with biotech and pharmaceutical companies to speed discoveries.
We want to work more closely with industry players to take academic projects, those mainly based in the lab, to real-world clinical trials with patients, which could lead to ground-breaking treatments in the next few years. When we invest as an industry partner on a project, we take a calculated risk in hopes of increasing our own income to put back into further EB research studies, as well as care and support for our Members.

Fund the best projects that will deliver the greatest impact.
We want to continue funding the best in EB research – those projects most likely to provide direct benefit to people living with EB. Some treatments are limited by country-specific regulations, so we work with organisations (including other EB groups, charities and industry) all over the world to co-fund the best projects so that our funding will have a wider impact for everyone living with EB.

Encourage creative thinking for ground-breaking discoveries.
We want to fund innovative projects and encourage researchers to approach EB in new ways whilst building on existing knowledge. These methods may include partnering with experts in other fields to look at specific areas of EB (e.g. tissue regeneration) or using the latest technologies to create innovative experimental treatments and therapies

Together we #FightEB, together we will beat EB.

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

Thank you for joining us on our journey. In just 40 years, we have achieved so much. Where once a parent struggled for information and help, diagnosis, support and information are widely available and ground-breaking clinical discovery has led to breakthroughs in current and potential treatments and understanding of EB.

But patients with EB are still suffering from devastating symptoms - pain, itch, cancer, wounds that require hours of dressings, blisters that can lead to infection. The EB community is at the heart of all we do. Their voices drive 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.

We can't do it without you. With your support we can invest in more EB research, encourage more world-class EB researchers, reach more EB patients.

Please join us. Help us to to #FightEB.

To find out more about our research, to donate or to get involved, please go to our website: www.debra.org.uk or scan the code:

Make the impossible, possible

DEBRA funds pioneering research, specialist healthcare, international best practice and lifelong 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 #FightEB.

Patron: HRH The Countess of Wessex GCVO

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