

EB Research Network

Aiming for a world where EB is treatable

Annual Report 3#2023



Prepared by Sandra Eder EB Resnet Coordinator

August 2024

WWW.EB-RESEARCHNETWORK.ORG

 **eb Research**
Network.

CONTENT

Highlights 2023.....	1
1. Summary.....	1
2. EB Research Network.....	3
Our Mission.....	3
Strategic goals	3
EB Resnet Members & Partners.....	4
3. Research Funding.....	5
Research Grants Statistics.....	5
Research Funding: Calls for Proposals 2023.....	7
Research Funding 2024 - Outlook	8
EB MSAP (EB Medical and Scientific Advisory Panel).....	9
4. Project Overview.....	10
Project types	10
Project Database & Project Map.....	10
Newly funded research projects 2023	11
COSEB - Towards Harmonization of Outcomes in EB	13
5. Clinical trials for EB.....	14
Clinical trials database on EB Resnet's website.....	14
Current clinical trials for EB	15
6. Support us.....	17
There are several ways to support EB Resnet's activities.....	17
7. Outlook.....	18

Highlights 2023

- ✿ First gene therapy VYJUVEK® (by Krystal Biotech) for DEB patients approved in the US
- ✿ Filsuvez® was finally approved by the FDA
- ✿ Joint Funding Call with LifeArc – spearheaded by an expert workshop in May 2023 in London
- ✿ DEBRA Research: new entity emerging in the EB translational research space
- ✿ COSEB consortium: kick-start meeting funded by EJP-RD

1. Summary

The year 2023 will once again go down in the history of EB research. After the world's first-ever EB drug was approved in the EU in 2022, VYJUVEK®, developed by Krystal Biotech, received market authorization in US. This is the first approved topical gene therapy treatment for Dystrophic Epidermolysis Bullosa (DEB) patients. Whether and how the already approved therapy will be reimbursed in countries other than the US is still uncertain and the approval process in Europe has not yet been completed. It is envisageable that further discussion with HTA and the payers will be needed to ensure widespread reimbursement of treatment costs. What is certain is that apart from research funding and the implementation of clinical trials for EB, the EB community is now facing a new issue. The topic of health economics and reimbursement negotiations with authorities will become a major area of action. It will be important for patient organizations such as national DEBRA groups to get actively involved and raise their voices. Projects such as BUR-EB, which collects socio-economic cost data in 7 European countries, could help here. Further details on this

multinational research project, funded by the European Joint Programme on Rare Diseases (EJP-RD) in the social-economic space can be found in the "**Project Overview**" section of this report.

In addition to Krystal, the birch bark-based Filsuvez® gel was also approved for EB by the FDA in the US in 2023. A new overview of the [approved treatment](#) options on EB Resnet's website keeps the community up to date.

The EB Resnet team was kept busy throughout 2023 with a *Special Call: Repurposing Therapy for EB*, co-organized with our strategic partner LifeArc. Together, we were able to provide joint funding of £ 2,5 million to support the call. Find out more about the *Special Call: Repurposing Therapy for EB* in the "**Research Funding**" Section of this report.

With the new entity DEBRA Research – a non-profit limited-liability company founded as a subsidiary of DEBRA Austria – a new player entered the EB research stage in 2023. We are delighted that DEBRA Research intends to become a strategic partner of EB Resnet. This organization focuses on translational research and clinical development

intending to create therapies and cures for EB. Learn more about our network and partners, mission, and strategic goals in our first report section, "**EB Research Network**".

In the "**Project Overview**" section, we present newly funded projects. We wish to mention the COSEB project, even though it has not yet been funded by EB Resnet members. COSEB is a multi-stakeholder consortium built around clinicians, researchers, methodologists, and EB patient organizations. Employing rigorous methods, this initiative aims to establish a broad consensus on meaningful, reproducible, and robust measured outcomes in EB clinical research on all ongoing and completed research projects can be found in the [project database](#) on the EB Resnet website.

The new EB Resnet clinical trials database is updated at least twice a year and provides an overview of ongoing and completed clinical trials for EB. In addition to the overview, there are links to relevant publications on the trials. More on this and statistics on currently ongoing trials are presented in the "**Clinical trials for EB**" section of this report.

Thematically, EB Resnet remains committed to its established priorities of supporting EB research and its clinical translation. Last year's expert round table in London once again underlined the importance of focusing on unmet clinical needs such as chronic inflammation and fibrosis. EB Resnet has launched an All Priorities Call (i.e. open to research proposals meeting one or more of EB

Resnet's priorities) which opened for applications at the end of June 2024. All details can be found on the website under [Research Grants](#). We continuously rethink ways of driving and funding clinical translation for EB patients and in 2024 we start reviewing our current research strategy.

With this report, I would like to take my leave as EB Resnet Coordinator. It has been a great pleasure for me to build up, support, and develop this network over the last 5 years. From November 2024, I will be breaking new ground and handing over coordination to Gaston Sendin as Research Manager for the time being. I know that the possibilities for further EB therapies are increasing every day and wish all stakeholders a lot of strength for the continued fight to help EB patients as quickly as possible.

Thank you for the great collaboration over the past years!



© Ludwig Schedl

Sandra Eder

EB Resnet Coordinator

2. EB Research Network

THE EB RESEARCH NETWORK IS AN ALLIANCE OF PATIENT ORGANIZATIONS WORKING TOGETHER AND IN COLLABORATION WITH PARTNERS TO DEVELOP AND DELIVER EFFECTIVE THERAPIES FOR ALL PEOPLE LIVING WITH EPIDERMOLYSIS BULLOSA (EB).

EB Resnet aims always to present, and evaluate, the up-to-date status of EB research, focusing on challenges such as gaps in knowledge or technology that hinder progress and identifying opportunities to develop therapies and advance their clinical introduction. Researchers, clinicians, patients, and industry partners all have an essential role.

The EB Resnet website provides information on current EB research projects worldwide and highlights collaboration opportunities for and with the industry. In addition, international funding rounds for EB research are announced and processed via EB Resnet's website. The website also provides information about external funding opportunities for EB research.

Our Mission

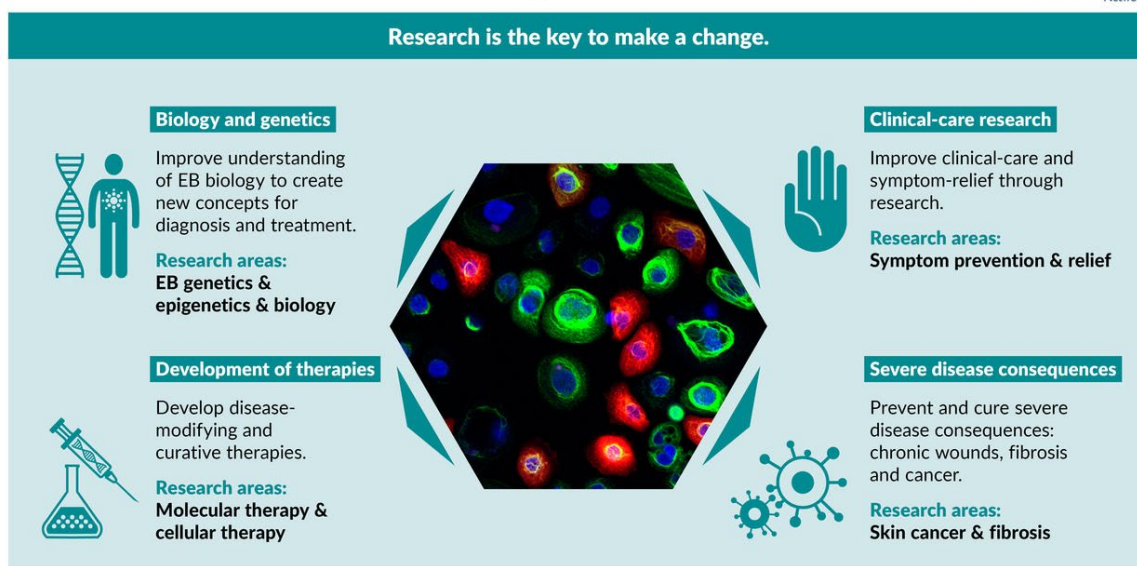
Translating successful research into benefits for EB patients.

Strategic goals

In the current EB Resnet strategy (2020-2024), three strategic aims are defined:

1. **To support research aimed at developing effective and safe treatments**
2. **To develop partnerships that expand research**
3. **To drive clinical translation and the adoption of treatments internationally**

EB research priorities & areas



Current research priorities and areas (EB Resnet 2023)

EB Resnet Members & Partners

In 2023, DEBRA Spain took on its first research project as lead funder. Together with DEBRA Austria, DEBRA Spain is funding the [García-Diez 1](#) project at the University Carlos III in Madrid.

In 2023, a new player emerged in the field of translational EB research. This organization, called DEBRA Research, is a DEBRA Austria subsidiary and operates under German law.

DEBRA Research intends to become a strategic partner of the EB Resnet soon and is primarily focusing on strengthening translation research and bridging the knowledge gap in the area of preclinical research and clinical trials.

Get in contact via the [DEBRA Research](#) website.



EB Resnet member organizations

Funding members

DEBRA Austria, DEBRA UK, DEBRA Ireland, DEBRA Spain, DEBRA France



Ordinary members

EB-LOPPET, DEBRA Australia, DEBRA Switzerland



Partners & Strategic Partners

DEBRA International, EB Clinet, EB House Austria, LifeArc



If you are interested in supporting EB Resnet or have any particular questions about the report or EB Resnet please contact office@eb-researchnetwork.org !

3. Research Funding

EB RESNET SUPPORTS AND COORDINATES RESEARCH FUNDING ON BEHALF OF ITS MEMBER ORGANIZATIONS AND PARTNERS. IT CURRENTLY OFFERS THREE FUNDING SCHEMES: RESEARCH GRANTS THROUGH SCHEDULED CALLS FOR PROPOSALS, AD-HOC GRANTS (OUR FLEXIBLE FUNDING SCHEME TO ACCOMMODATE CO-FUNDING OPPORTUNITIES WITH OTHER FUNDERS), AND CO-FUNDING FOR INDUSTRY-PARTNERING PROJECTS.



© Ludwig Schedl

EB Resnet welcomes proposals for innovative research and clinical development of treatments or diagnostics. All submissions are evaluated for scientific excellence, feasibility, cost-effectiveness, and alignment with the priority needs of people with EB. We undertake peer review to ensure that only the highest quality research is funded.

The actual funding for research support can vary widely from year to year owing to several factors. Firstly, the number of funding rounds held annually varies; while usually two, it has varied from one to

three, depending on funding availability from contributing DEBRAs or other funders, and other commitments. Secondly, the number of research grant applications received per call has varied, usually in the range of 12-25. Thirdly, funding recommendations by the EB Resnet's Medical and Scientific Advisory Panel (EB MSAP) are based solely on research quality and potential patient benefit. Consequently, the proportion of project applications funded has ranged from ~10% - 50%, with an average funding rate of around 25%. If there is a larger than-average number of successful applications in one call, this may reduce funds available for subsequent calls in the same year.

The partnerships with LifeArc and the future collaboration with DEBRA Research enable EB Resnet to provide even more opportunities for support of translational research. Both partners provide know-how and funding possibilities for late-stage clinical development and clinical trials.

Research Grants Statistics

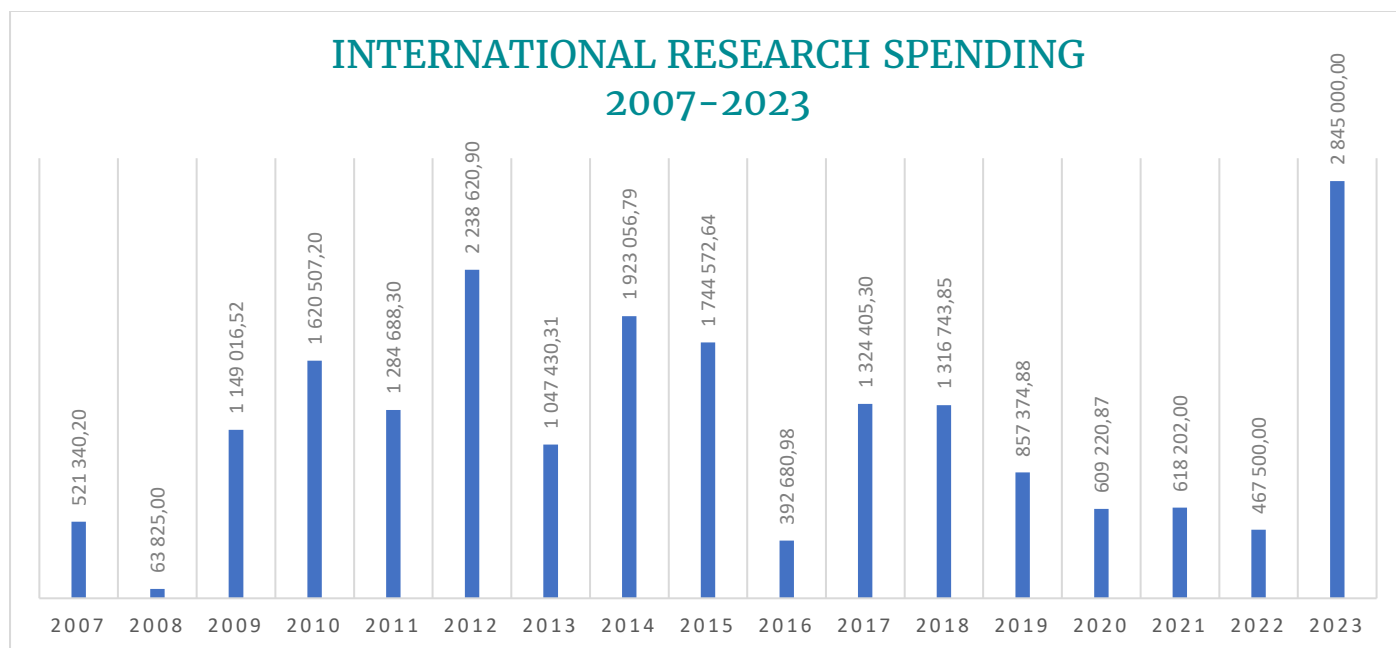
Facts and figures since 2007

The International EB Resnet Research funding rounds include the All Priorities Calls (AP Calls) and the Special Calls. AP Call means that project topics must fit into at least one of the four strategic research priorities of EB Resnet (see page 3). Alternatively, a Special Call may be issued to solicit proposals for important unresolved research

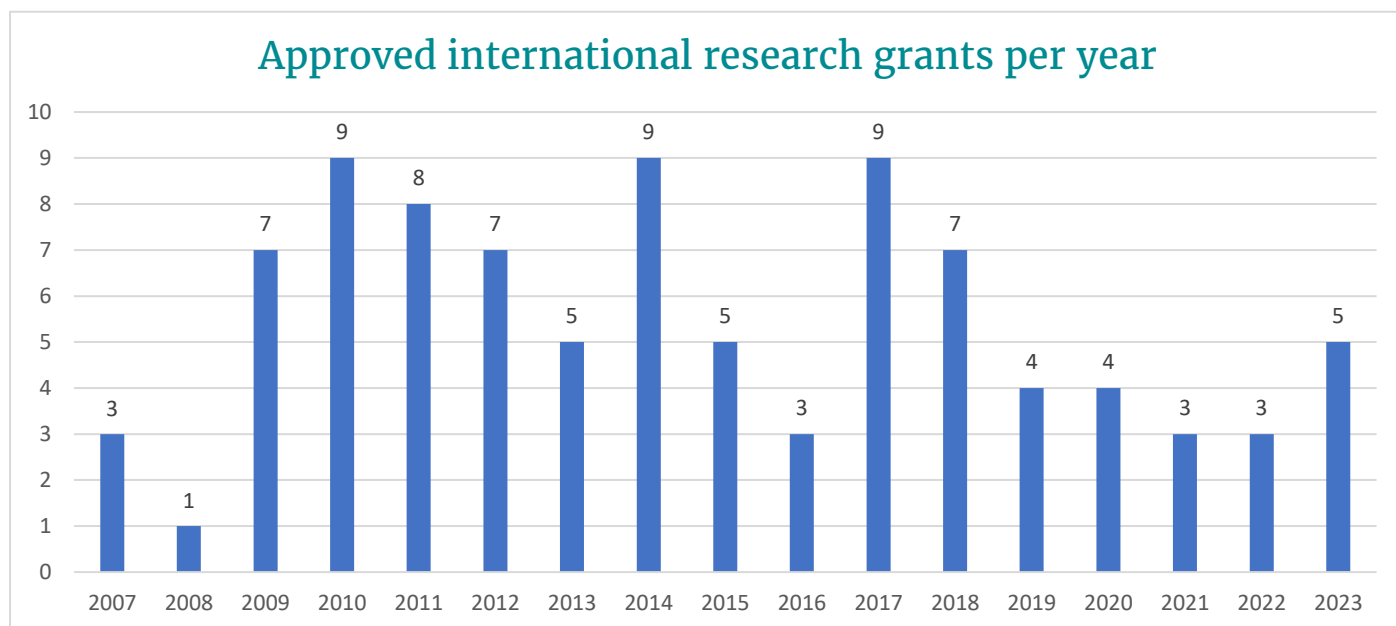
questions, technology needs, or clinical consequences of EB (e.g., chronic inflammation and fibrosis). Since 2007, 20 AP Calls and 6 Special Calls have been organized, and approximately 19 million Euros have been invested in international EB research in the form of Research Grants. These funds have been raised primarily by EB Resnet's

national DEBRA group members and occasionally other EB patient organization funding partners. The Special Funding Call 2023 was co-funded for the first time by LifeArc and gave a huge push to the EB research funding possibilities.

Therefore, 2023 was an exceptional year to date as our strategic partner put £2 million into the funding pot. Together with DEBRA Austria the Special Call: *Repurposing for EB* provided £2.5 million for late-stage clinical EB research. We hope for further similar joint funding opportunities with partners in the future.



Research grants approved in Euros by research funding round since 2007. The amount shown in the 2023 column is not yet contracted – but allocated to 5 projects accepted in 2023

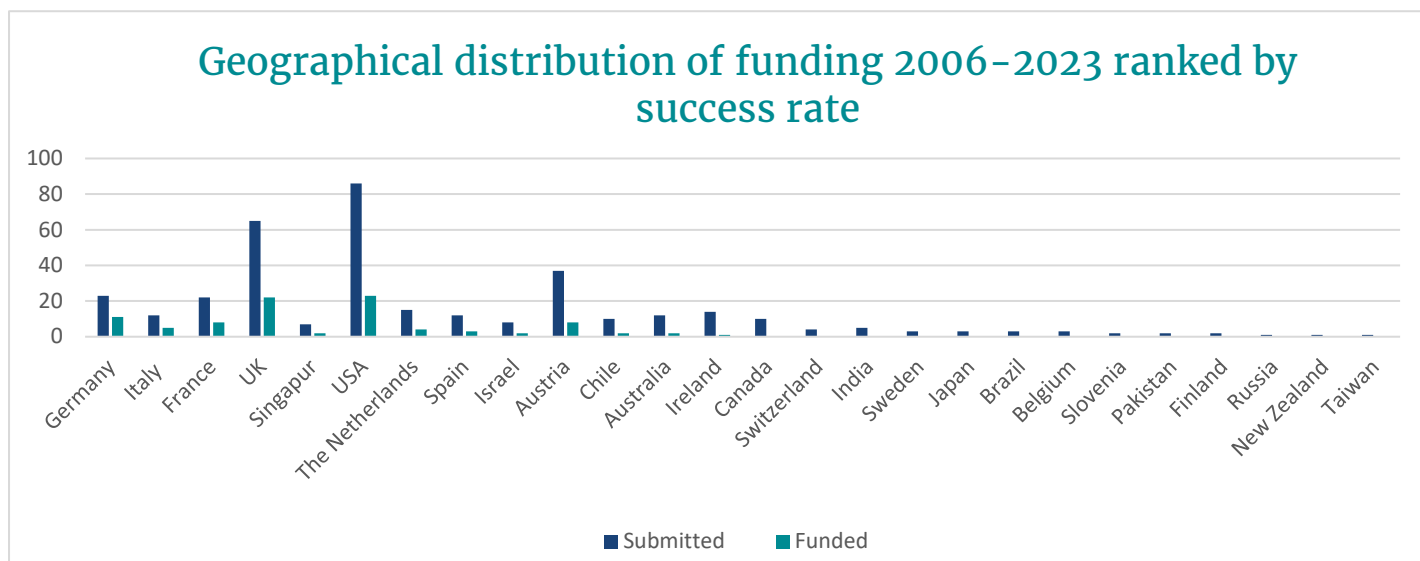


The number of International Research Grants approved per year since 2007. For the funding year 2023, we also included 4 projects which received approval but which have not yet been contracted.

As in previous years, the statistics show that the funding flows to many different countries and is not geographically restricted. Nevertheless, the trend continues that there are traditionally strong EB research groups in the US in particular, but also in European countries such as France, the UK, The Netherlands, and Austria. Submissions from these countries remain relatively high, and the

acceptance rates are also higher. Overall, the US and the UK received the most project funds.

However, the funding rate is highest for German groups (submissions to acceptance ratio). According to our data 363 project submissions have been reviewed by the expert panels through the international EB funding rounds since 2007.



The number of submitted and approved international research projects (2007-2023) by country of research institution, ranked by the success rate of submissions. Only submissions from DEBRA International / EB Resnet funding rounds are included.

Research Funding: Calls for Proposals 2023

Special Call: EB Repurposing Call 2023

The Joint Funding Call with our partner LifeArc culminated in the Expert Panel meeting to assess proposals in March 2024 in Vienna. A total of nine Expression of Interest (EOI) project proposals were selected and invited to submit full proposals. These submissions then underwent an external peer-review process including due diligence provided by LifeArc. The Expert Panel consisted of international experts with backgrounds in both academic/ clinical research and industry. In addition, patient representatives were engaged in the whole process, including the review stage. According to the call's remit, all projects were required to focus on

preclinical to clinical research within the drug repurposing space. Following evaluation, four projects of this joint funding call scored above the threshold for consideration for funding. LifeArc and DEBRA Austria are now discussing various options to support the eligible projects in whole or in part with (smaller/larger) budgetary, content-related, and organizational adjustments. As soon as funding agreements are finalized, we will be able to publicize the funded projects.

The Call's structure and priorities were developed through an EB Expert Workshop in Spring 2023 in London. The goal was to refine and validate current thinking on core unmet medical needs, with experts

in EB and overlapping research disciplines. The review defined the focus areas for the specific call for research proposals in the summer of 2023 and provided a framework for research investment priorities in the near to medium term.

More information including the [workshop report on EB Resnet](#).

DEBRA UK launched an international funding round beginning in 2023. We would be pleased to provide more information on EB projects that have been recommended for funding through the various DEBRA national funding channels. If you know of such projects in your country and wish to include such information, please contact us. More information on the UK call here: [DEBRA UK's website](#).

Ad-hoc Grants 2023

The Ad-hoc funding channel enables small funding grants to be awarded quickly and flexibly. The aim is to allow, for example, co-financing opportunities, new project ideas, or proof-of-concept projects. Of course, all ad hoc project proposals undergo rigorous review. Academic researchers, clinicians, and the industry can contact us outside the official funding rounds to submit research proposals. In 2023, EB Resnet awarded one grant which was an add-on project embedded in a multination EJP RD grant called BUR-EB. More information is provided in the “**Project Overview**” section.

Research Funding 2024 - Outlook

EB Resnet is inviting research proposals for the ‘All-Priorities’ Research Call 2024. The All Priorities Call 2024 is funded by EB Resnet member DEBRA Austria.

All Priorities Call 2024

There is a one-stage application process, which opened on **Monday, June 24, 2024**.

Through this AP Call 2024, research proposals in any of EB Resnet’s priority areas are eligible for support.

Current Research Priorities

- ✿ Improving understanding of **EB biology** to create new concepts for diagnosis and treatment.
- ✿ Developing **disease-modifying and curative therapies**.
- ✿ Preventing and curing **severe disease consequences**: chronic wounds, fibrosis, and cancer.
- ✿ Improving **clinical care and symptom relief** through research.

We would particularly welcome proposals in the following areas:

- Cutting-edge approaches addressing current hurdles in the creation and clinical development of reproducible stem cell therapies for EB (safety, potency, genetic stability, immunogenicity, tumorigenicity, cell reproducibility, and scalability)
- Sophisticated models of EB capable of realistically mimicking complex cell-to-cell interactions, including epithelial-immune

cell crosstalk (organoids, complex skin-equivalents, organ-on-a-chip, bioprinting).

- Predictive, high-throughput, data-driven bioinformatics approaches supported by robust analytics enabling interrogation of EB multimodal data (genetic, molecular, clinical) followed, where possible, by

confirmatory, functional validation assays. These approaches should aim to identify possible points of therapeutic intervention based on specific biomarkers and relevant signaling pathways.

- Breakthrough approaches tackling current problems in delivering repair molecules to internal mucosae of EB patients.

EB Resnet offers to fund individual research projects up to EUR 270,000 over three years.

The closing date for applications is September 09, 2024, at 15:00 GMT

The details of the AP Call 2024 are available under [this link](#).

EB MSAP (EB Medical and Scientific Advisory Panel)

Warm Welcome to Dr Andy South

EB MSAP's members are senior EB researchers and clinicians who jointly reflect the breadth of EB research. For Special Calls for research proposals, which may focus on a particular unmet research need or novel technology, we convene Expert Panels with expertise specifically relevant to the topic of the call, which can involve selected EB MSAP members and additional experts chosen for their specific area of knowledge. Members of our Expert Panels work to act as guardians of scientific quality and relevance to people with EB of the research that EB Resnet funds. Its members review both proposals and progress of research funded by EB Resnet members: this peer-review process is central to maintaining our reputation as a research funder among the academic and clinical research community, bioindustry, and research sponsors and donors. We cordially welcome our new member Prof Andrew South from Jefferson University, US, who recently joined EB MSAP.



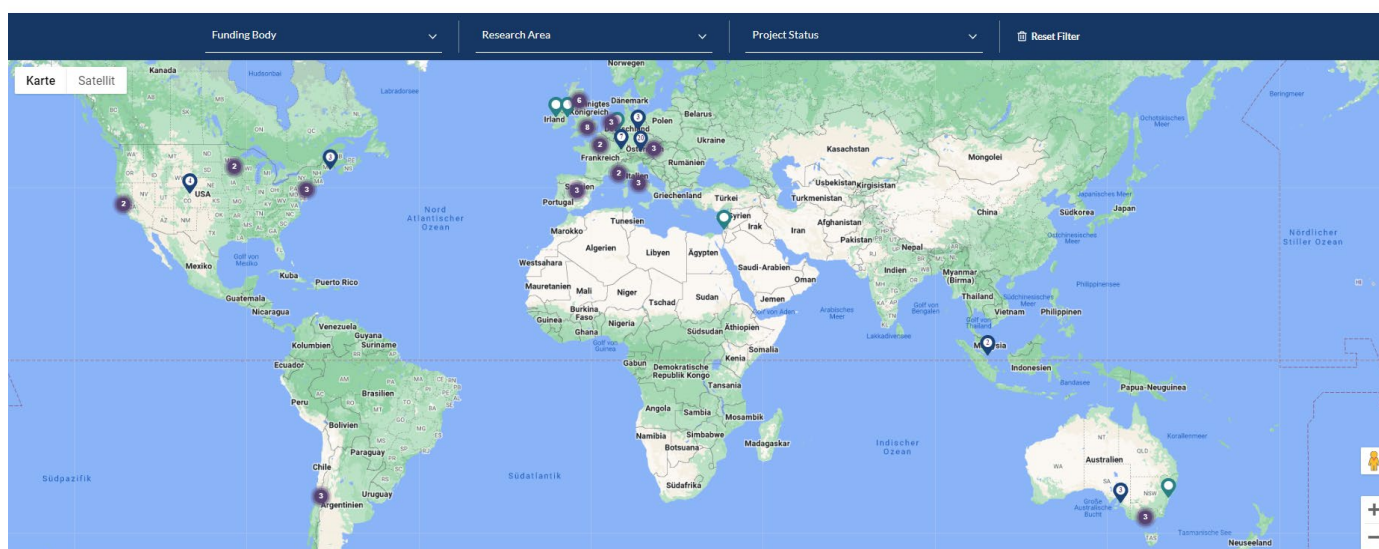
© Ludwig Schedl

Useful links

- [EB Expert Panel Members](#)
- [Review-Process](#)

4. Project Overview

THE EB RESNET PROJECT DATABASE INCLUDES NATIONAL PROJECTS FUNDED BY NATIONAL MEMBER GROUPS, AS WELL AS INTERNATIONALLY FUNDED PROJECTS IN OVER 60 RESEARCH INSTITUTIONS WORLDWIDE. THE PROJECTS COVER ALL CURRENT RESEARCH PRIORITIES AND ALL EB TYPES.



EB Resnet Project Map shows current and completed projects worldwide.

Project types

EB Resnet lists various EB research projects on its website. Those projects funded through EB Resnet research funding rounds, previously known as DEBRA research funding calls, are referred to as Research Grants. The Research Grants are reviewed and evaluated through the international peer review

process with the EB Expert Panel (EB MSAP). The selection process for these projects is subject to a rigorous scientific expert analysis. Projects selected by local members through their national funding streams and own review process are called national research projects.

Project Database & Project Map

There are 138 projects in the EB Resnet project database. 34 projects in the database are ongoing and being conducted at about 30 different research institutions worldwide.

EB Resnet members and partners fund research projects, either jointly or individually.

The Google Map on the EB Resnet website shows the geographic location of all projects. Our database provides a lay summary, a short scientific summary, the strategic relevance for EB, related publications, and a short impact report for each completed project.

[Search our database](#) or [Search our Google map.](#)

Newly funded research projects 2023

The BUR-EB project

As part of an EJP-RD* project (BUR-EB), this new survey aims to assess the socio-economic burden of EB in seven EU countries (Spain, France, Germany, Italy, Hungary, Bulgaria, and Austria) and compare it with data collected ten years ago in the BURQOL-RD project. EB Resnet member DEBRA Austria provided additional funding for data collection in Austria and contributed expertise through DDR Gudrun Salamon from the Sigmund Freud University in Vienna. Other national DEBRAs involved in the study are DEBRA France, DEBRA Spain, DEBRA Italy, DEBRA Germany, DEBRA Hungary, and DEBRA International.

With chronic illnesses such as EB, the financial situation has a major impact on the well-being of both patient and family/ carers. Given the significant amount of medical expenses, coverage by health insurance companies and the possibility of reimbursement play an important role. BUR-EB aims to collect information about the financial resources spent on EB and the impact of the disease on affected persons' quality of life. We are also interested in finding out the burden on the main carer (if applicable). To this end, we have selected patients and their main carers to visualize the financial challenges together.

To ensure that these data are comparable across countries, the BUR-EB project analyses the financial situation of families with EB in seven EU countries simultaneously.

Main objective

Data on the impact of EB on daily life will be collected from affected individuals and their carers via an anonymous survey in collaboration with clinicians and patient organizations (DEBRA). The economic burden takes into account healthcare and informal care costs, the financial burden on families, and the productivity losses of all affected individuals. Quality of life and family burden are also measured. BUR-EB offers the opportunity to observe how the social and economic impact of EB has changed over the last decade and how these changes could be linked to the health and social policies implemented during this time.

The project will provide tools that could be used in clinical trials of new therapeutic options. Find out more on the BUR-EB website: [BUR-EB](#)

The quantitative survey (data collection will close end of August 2024) for each participating country is available via this link: <https://www.bur-eb.com/get-involved/>

Added value

There is currently only limited or no data available on EB-specific indirect or direct costs in the participating countries. These data are urgently needed, especially to speed up future authorization procedures and cost reimbursements for new EB therapies.

*EJP RD European Joint Program for Rare Disease

Research Grants contracted in 2023

Garcia-Diez I: In vivo correction of Recessive Dystrophic Epidermolysis Bullosa by gene editing mediated by adenoviral vectors.

PI: Dr Marta García-Diez

Institution: University Carlos III de Madrid

Funding Round: AP Call 2022 (recommended on international level), funded by DEBRA Spain and DEBRA Austria

Main targeted unmet medical need: Mutation correction and Wound healing

This project targets one of the most severe complications of EB: fibrosis - pathological skin thickening and scarring that occurs mainly in patients with recessive dystrophic EB (RDEB) and

contributes to developing aggressive skin cancer. The proposed research approach is particularly exciting because it is already being tested for another indication in a phase 2/3 clinical trial. Initial data from the research group also indicate an effective reduction of fibrosis in RDEB mouse models. The project relies on a repurposing model of an already-known molecule and could move relatively quickly into clinical implementation if the results are positive.

[Link to the Project description.](#)

Condorelli I: Notch signaling by the gamma-secretase inhibitor PF-03084014 to counteract fibrosis progression in recessive dystrophic epidermolysis bullosa

PI: Dr Angelo Condorelli

Institution: Bambino Gesù Children's Hospital, Rome, Italy

Funding Round: AP Call 2022 (recommended on an international level)

Funding Body: funded by DEBRA Austria

Main targeted unmet medical need: Chronic inflammation and Fibrosis

This project comes from gene therapy and is based on CRISPR/Cas9 technology. The research team proposes the development of an *in vivo* (directly on the skin) gene therapy in the form of a skin gel for RDEB patients. Correction of a prevalent mutation in the collagen 7 (COL7A1) gene could be achieved with a specific adenoviral vector. The aim is to develop a drug for treating chronic wounds, which could also be effective in the digestive tract of EB patients.

[Link to the Project description.](#)

All completed projects are required to submit a brief description of their impact. This is displayed on the website on the respective project description page. For non-confidential details of the individual projects, please contact Gaston Sendin (Gaston.sendin@debra-austria.org) or the PI directly. [Project database.](#)

COSEB - Towards Harmonization of Outcomes in EB

About 50 participants took part in a hybrid working group meeting in December 2023

The COSEB (Core Outcome Sets for Epidermolysis Bullosa) workshop was held as a hybrid meeting in Amsterdam, The Netherlands, on December 15th, 2023. It was organized by the COSEB Steering Committee and DEBRAs of Austria, France, Ireland, Spain, and the United Kingdom. The goal of the COSEB initiative is to establish consensus-based core outcome sets for the major EB types (EB simplex, junctional EB, and dystrophic EB) by identifying the most critical outcome domains (the “what” to measure) and corresponding outcome measurement instruments (the “how” to measure). Such core sets should be used and reported consistently across clinical trials, thereby facilitating accurate comparison and pooling of data to expedite therapy development.

Funded by the European Joint Programme for Rare Diseases (EJP-RD), the workshop aimed to foster collaboration among key stakeholders in EB research to address challenges concerning outcome measurement in EB. Following an intensive literature search, the group compiled a list of over 1,000 clinical outcome measures previously used for clinical EB studies. These different measures will be standardized in the future and a catalog of clinical outcomes made available to clinicians in the form of the core outcome set. In the future, this COS (core outcome set) will be used in all clinical trials and reported in a standardized manner to enable precise comparison and data consolidation to accelerate therapy development.

Currently, the COSEB group’s project submission through the EB Resnet Ad-hoc Channel undergoes peer review. EB Resnet will certainly need funding partners to support this crucial project.



5. Clinical trials for EB

THE NUMBER OF EB CLINICAL TRIALS CONTINUES TO RISE. THE FIRST APPROVED THERAPIES HAVE PAVED THE WAY FOR OTHERS. WE CURRENTLY FIND ALMOST 50 ACTIVE EB CLINICAL TRIALS IN REGISTRIES WORLDWIDE.

Clinical trials database on EB Resnet's website

EB Resnet clinical trials' online tool

The main aim of the tool is to provide a pooled overview of all studies relevant to EB. It combines studies from the following registries: Clinicaltrials.gov, the EU Clinical Trials Registry, the International Clinical Trials Registry Platform (ICTRP), and the UMIN Clinical Trials Registry. It has been available on EB Resnet's website since spring 2023. All registered ongoing and completed interventional and observational studies for EB will be found. The EB Resnet team and the [EB Clinet](#) team will take care of the constant updating of the international database.

The database assigns the studies to the respective therapeutic approach, contains a brief description, and offers further information at a glance, such as the route, the EB subtype, the study phase, and the status. The database's filter functions help you refine your search by therapeutic approach, EB subtype, or study phase. In addition, a search function allows you to search specifically by keyword or, for example, by the sponsor. You can click on the links to the respective study

register and the study sponsor to obtain more detailed information. If you have specific questions about the studies, please get in touch with the respective study center directly. We hope the new tool will be helpful and would be very happy to communicate this to the EB community.



© Dmytro Sheremeta

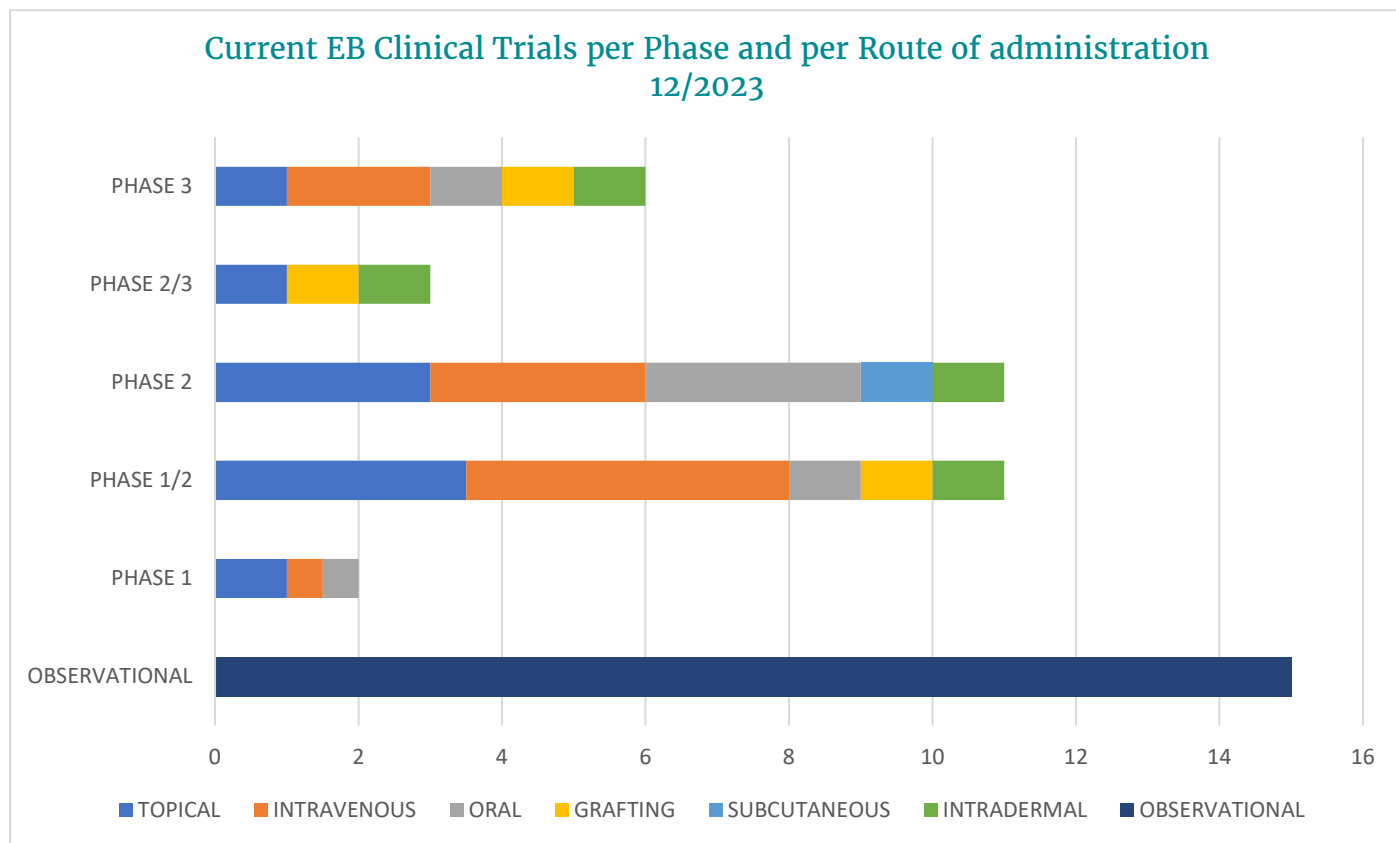
Please note that EB Resnet and DEBRAs cannot guarantee the completeness of the lists, even though they are updated regularly. We are happy to support the maintenance of the database - information about missing studies or updates is very welcome. Thank you very much for your support.

We also included trials with the status indicating „unknown“ which is shown in the status column.

Link to the new clinical trial online tool:

<https://www.eb-researchnetwork.org/clinical-translation/clinical-trials-database/>

Current clinical trials for EB



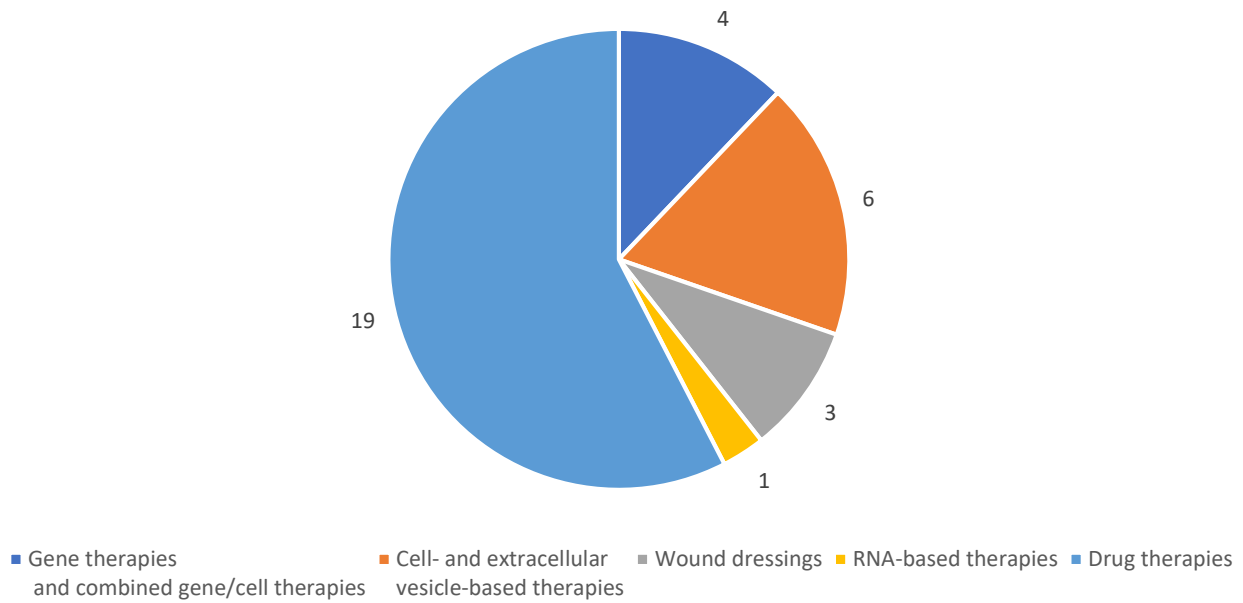
The number of active clinical trials by phase and type of application. Status 12/2023.

We observe a trend of increasing numbers of clinical trials for EB each year. Based on our clinical trials database we did some analysis. We registered an increase in the number of clinical trials from 38 active at the beginning of 2023 to 48 active trials compared to early 2024 (we also display trials with unknown status under active trials). It is interesting to note that the route of administration is very evenly distributed across our main categories of topical, intravenous, oral, grafting, subcutaneous, and intradermal modes of application. There are

many (15) ongoing observational studies for EB - very often these are additional and supporting studies for ongoing clinical trials.

The majority of studies are concentrated in the areas of intravenous (10) and topical applications (9.5). These are followed by oral administration (5.5), intradermal administration (4), and grafting (3). According to our analysis, there is currently only one study in which the treatment is administered subcutaneously 48 studies analyzed.

Active Clinical Trials per Therapeutic Approach 12-2023



The number of active clinical trials by therapeutic approach. Status 12/2023

If you exclude the observational studies and focus solely on the therapeutic approaches in the current list, the significance of drug therapies becomes evident, as they account for 19 ongoing clinical studies.

There are currently no clinical studies in the field of protein therapy, which is why it is not presented here in this chart.

6. Support us

JOINING FORCES TO FIND A CURE AND THERAPIES FOR EB.

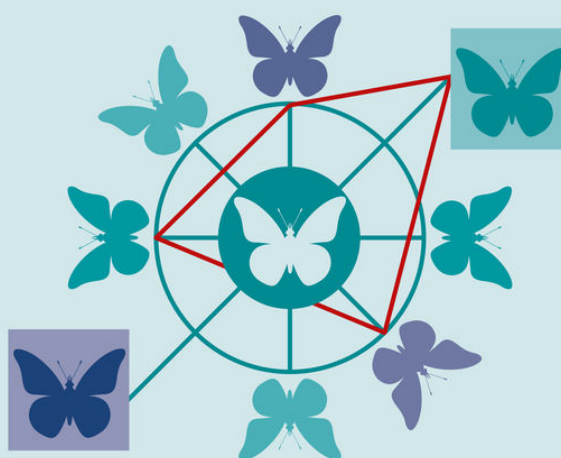
There are several ways to support EB Resnet's activities.

EB-Research Network – member & partner benefits



Patient organizations

- Open to all EB research-active patient organizations
- Clarity for researchers, partners
- Decrease contact-burden of clinicians, patients
- Combine resources to fund larger scale clinical development
- Collaboration with industry, healthcare providers



Industry, healthcare partners

- Coordinated access to patient organization funding, resources
- 'One-stop-shop' for patient perspectives, disease information
- Diverse network members, multiple funding models
- Bespoke collaborations
- Coordinated lobbying on reimbursement, healthcare provision to government and private providers

- ✦ Join the network as a funding member: assist EB Resnet in managing and funding the grant schemes.
- ✦ Join the network as an ordinary member: share your national research projects and provide small research funding for EB Resnet funding channels and profit from the peer-reviewing process provided by EB Resnet.
- ✦ Join the network as a partner: share your research knowledge on EB, your EB

projects, and/or profit on EB patients' access.

- ✦ Join the network as a strategic partner: to help drive research outcomes into clinical application for patient benefit.
- ✦ Help building up the [Industry Partnering Panel – IPP](#).
- ✦ [Donate](#) to increase funds for EB research.

If you are interested in supporting EB Resnet or have any special questions about the report or EB Resnet please contact office@eb-researchnetwork.org!

7. Outlook

Despite promising advances in gene/cell therapies that aim to address the underlying causes of EB and are at various stages in clinical development, EB patients continue to face significant challenges. These include inflammation linked to chronic wounds and consequent development of fibrosis and progression to cancer. Intervention to prevent or slow chronic inflammation and fibrosis (CIF) will improve daily quality of life, and longer-term health prospects for people with EB: CIF therapeutics will be important as standalone treatments, and adjuncts to gene/cell therapies. The goal of the upcoming review process of EB Resnet

strategy is to build on the outputs of the May workshop held in London together with LifeArc. This process will further refine the focus on serious unmet clinical needs, and to identify additional, as yet underresourced or ignored, needs that require prioritization. In addition, significant effort will be dedicated to developing the methodology by which EB Resnet and partners can address strategic priorities and expedite, in a cost-effective way, the clinical translation of promising research.

A Strategy process for the years 2025-2030 will be started during 2024.