

JOINT FUNDING CALL 2023: Repurposing therapies for Epidermolysis Bullosa (EB)

GUIDANCE DOCUMENT FOR APPLICANTS

Chronic inflammation and non-healing wounds, and the consequent **fibrosis and skin cancer initiation** in severe disease subtypes, **are a clear unmet need for people with epidermolysis bullosa (EB)**, LifeArc and DEBRA Austria are holding a joint call for projects to progress well-evidenced therapeutics that can be repurposed / repositioned towards clinical testing, to deliver:

- Increased resolution of non-healing wounds
- Prevention and / or reduction of chronic inflammation
- Inhibition and / or reduction of fibrosis
- Decreased risk of squamous cell carcinoma initiation in EB

What we are looking for

Proposals should include:

- A substantial, ambitious programme of work with a translational focus and the potential to deliver future patient benefit
- A clear strategy for progress to clinical application in EB, including patient access
- Consideration of the intellectual property (IP) position
- Details of engagement (if any) with owner / manufacturers of the proposed intervention (for example generic or branded medicines being considered)
- Details on ownership of and access to the therapeutic/s or other required materials, current authorisation status of the intervention and plans for further development of IP

We welcome applications across multiple modalities, including small-molecule compounds or biologics. These should be supported by a solid evidence base obtained through functional studies in relevant disease models, as well as strong molecule characterisation from prior development in a primary indication.

Applications must be progressing an identified, existing molecule associated with a minimum of an understanding of pre-clinical toxicity and PK / PD package from prior development in a primary indication. A broad range of drug nomination strategies are welcome, including in-silico prediction; however, **expectations for experimental validation remain consistent across all approaches.**

In addition to developing the therapeutic agents, projects could include a strategy to develop and validate biomarkers to allow targeted treatment and report outcome; however, standalone model development and biomarker discovery will not be considered.

Immunotherapeutics and cell / cell-derived treatments, along with small-molecule drugs, peptides and other biologicals, addressing non-resolving inflammation and infection and progression to fibrosis and cancer initiation are eligible. Treatments designed to be used in conjunction with regenerative medicine therapies, in order to address the inflammation and fibrosis directly and thereby enhance the

effectiveness of the regenerative medicine therapy, are also eligible. However, gene / cell therapies addressing the underlying gene defect causing EB are not.

New drug discovery projects, high-throughput screening, hit to lead discovery and medicinal chemistry development, will not be considered. Non-specific wound treatments (including gels, ointments, and dressings) where the mode of action of one or more ingredients is not known are also not eligible.

We wish to support multi-disciplinary collaborations that, ideally, also demonstrate adequate familiarity with the challenges of developing therapies for conditions such as EB. We expect applications to include evidence of patient and public involvement in the proposed workplan.

We will not fund:

- Fundamental 'discovery' research
- New drug discovery, high throughput screening, hit to lead discovery and medicinal chemistry development, is not eligible for support
- Generic wound-healing and oncology drugs that lack a robust rationale for their use in EB
- Standalone development of either new models or biomarkers
- Gene / cell, protein, or small-drug or molecular therapies targeting underlying gene / protein defect of EB
- Industry R&D costs

Guidance on repurposing medicines is available here:

- www.repurposingmedicines.org.uk

Applicant Eligibility

Research groups employed at academic / non-profit research institutions, or hospitals, worldwide, are eligible to apply for funding. Collaboration among research groups is encouraged for all projects to ensure all expertise needed is optimised, and familiarity with EB as a disease is included. Collaboration with industry is also encouraged, but the lead organisation must be non-commercial. SMEs and larger bio/pharma can apply as co-investigators in collaboration with an academic institution leading the application but may not request funding: the costs of commercial companies cannot be funded.

Applicants will be required to demonstrate that the assembled team has the necessary experience, expertise and access to facilities to deliver the proposed research plan. The Principal Investigator (PI) for any collaborative project should have demonstrable experience in leading multi-investigator, multidisciplinary, consortia or, at a minimum, applicants should demonstrate their potential to lead and manage a large-scale collaborative project.

Process and timelines

The number and scale of projects funded is not pre-defined, nor is there a preference for small or large projects. Appropriately budgeted projects will be prioritised according to their targeting a severe unmet need, level of patient benefit, and likelihood that they can be brought to clinical application within a reasonably short timeframe.

Small projects (<£100K, 12 months duration maximum) through to larger projects (£1M max; 3 years duration maximum) are all eligible, and will be subject to the same criteria of potential to address a severe unmet need in clinical treatment of EB, feasibility and research quality, and a well-defined pathway to further development and delivery. Demonstration of adequate resourcing, expertise and appropriate budgeting at expression-of-interest (EOI) stage will influence invitation to progress to full application status.

Funded projects will have a clearly defined scope with key deliverables and milestones with clear go / no-go criteria and risk mitigation strategy. Project progress will be assessed by achievement of agreed milestones, and monitored by a joint steering group with LifeArc and DEBRA Austria representation.

Applicants are referred to these guidelines, and will have an opportunity to seek advice and clarification during preparation of their full application.

Application process and deadlines

A two-stage process will be used to select projects, with an initial EOI, followed by an invited full application. No full applications that have not undergone the EOI stage will be permitted. Confidential external Expert Panel review and Funders’ Diligence will be undertaken on all full applications.

Application step	Date
Call for EOI opens	10 July 2023
Deadline for EOI submission	10 September 2023
EOI applicants notified of outcome; full applications open (by invitation)	04 October 2023
Deadline for full application submission	04 December 2023
Funding decision, and successful applicants notified of outcome	By 29 March 2024
Expected Project start	2Q 2024

Terms and conditions

Recipient of the grant, the Grantholder, will be expected to agree to the terms and conditions of this award that will include milestones-driven payments, reporting obligations and provisions on the management of the funded outputs / arising IP.

Full terms and conditions will be made available to applicants upon invitation to full application.

The Grantholder will own the outputs / arising IP generated as part of the funded project.

The Grantholder will be required to use the generated outputs / arising IP in order to maximise the public and patient benefit and in accordance with LifeArc’s and DEBRA Austria’s charitable purposes.

The Grantholder will be expected to consider what form of IP management would best promote this, and will take steps to implement this.

There will be an expectation that Grantholders will seek funders consent before commercialising any funded outputs / arising IP and with an expectation of sharing of revenue generated from such commercialisation (similar to AMRC revenue-sharing principles).

If the funded outputs / arising IP are not diligently protected or exploited, the funders will have step-in rights.

Please note: for successful applications from collaborative proposals involving industry (the PI must in all cases be an academic partner), additional side-agreements may be required at the point of contract.

The application process and subsequent peer review are subject to strict conditions of confidentiality. Following final approval of funding and the issue of contracts, limited details of funded projects will be published on the Charities' websites and may be published in EB community newsletters; all EOI and unsuccessful full applications remain confidential.

Applicants should provide the information required in the application form fully and clearly, completing each section of the form. Forms that are incomplete or completed incorrectly cannot be considered.

Some important definitions

Co-applicant names and Institutions: Use full titles, all initials and surnames and include Institution name	Means any individual named as such on the Grant Application and who will be involved in managing execution of the Research Project in conjunction with the Principal Investigator.
Collaborators / Project Partners	Means a third party who performs any part of the project for the Grantholder being a collaborator, subcontractor or any other form of provider, except the Principal Investigator and any other employee of the Grantholder, providing, among others, services, materials, technical information or IP to the Grantholder to enable the Grantholder to carry out the project.
Institution / Grantholder	Means the institution at which the Principal Investigator is employed, either tenured, or for at least the duration of the project, and which takes responsibility for administering the grant.
IP or Intellectual Property	Means patent rights, trademarks, copyright, database rights, rights in designs, rights to use and protect confidential information, and all or any other intellectual property rights whether or not registered or capable of registration anywhere in the world (including the right to apply for and applications for the foregoing).
PI or Principal Investigator	Means the person named as such in the award letter and who is employed or otherwise engaged by the Grantholder being responsible for managing execution of the funded project.
Role and expertise of Principal Investigator	Role within project and proportion of time (% total employment) devoted to project.

LifeArc

LifeArc accelerates scientific innovation so patients can benefit from medical breakthroughs sooner. Our in-house scientists, technology transfer and other experts help academics, charities, and others to overcome commercial barriers and develop new diagnostics, therapeutics and devices.

An independent self-financing charity, we're driven by patient need rather than profit. We've helped develop life-changing treatments for cancer (Keytruda®), Crohn's disease (Entyvio®), multiple sclerosis (Tysabri®), rheumatoid arthritis (Actemra®) and Covid-19.

DEBRA Austria / EB Resnet

DEBRA Austria is a patient organization charity dedicated to support people with epidermolysis bullosa. It is a member of EB Resnet, an international network of epidermolysis bullosa (EB) patient organisations funding promising EB research worldwide and partnering with the industry, academics, clinicians, and other funders to drive its translation to clinical benefit. Our mission is to support the development of efficacious, safe, and affordable medicines for people living with EB.