

# A European study on rare diseases: the burden of epidermolysis bullosa



**BUR-EB**  
Changes in the Socio-economic  
Burden of Epidermolysis Bullosa in Europe



This project has received funding from the European Union's Horizon 2020 research and innovation programme under the EJP RD COFUND-EJP N° 825575



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## Introduction and objective

We are pleased to announce the start in March 2022 of a European study, **BUR-EB**, that **will seek to evaluate the socio-economic burden of a rare and highly disabling genetic disorder: epidermolysis bullosa**.



## Why epidermolysis bullosa?

Epidermolysis bullosa (EB) is characterised by an **abnormal fragility of skin and mucous membranes** that leads to the formation of blisters and chronic wounds after minimal traumas. Although EB encompasses a wide range of clinical forms of variable severity, it is in most cases **seriously disabling and can even be life-threatening**. Despite a notable research effort over the last two decades, **no curative treatment is yet available for EB, and disease management relies on treating symptoms**.

**Patients and their families face multiple and lifelong challenges** from the need for daily, painful and time-consuming wound dressings to nutritional problems, functional impairment and aesthetic changes in physical appearance. Thus, **EB is exemplificative of the chronicity and complexity of rare diseases and of the difficulties encountered by affected individuals and families**.

Over the last years, National Healthcare Services in most European countries have implemented specific plans for rare diseases.

In parallel, the European Commission has created 24 European Reference Networks (ERNs) for Rare Diseases, including the ERN for Rare and Undiagnosed Skin Diseases, ERN-Skin.

While some studies have evaluated the impact of EB on the quality of life (QoL) of patients and their families, much less is known about the costs of the disease. Moreover, changes in QoL and the socio-economic burden of EB over time have never been assessed.



## Who is involved in the study?

BUR-EB is a joint initiative of expert centres for public health economics and for rare skin diseases, in particular EB, in strict collaboration with the EB patient associations, DEBRA International and the national DEBRAs.

The study is addressed to **individuals affected with EB and their families** and will be carried out **in six European countries** (Bulgaria, France, Germany, Hungary, Italy and Spain).





## How and when will the study be performed?

BUR-EB will collect data on the impact of EB on every-day life via an **anonymous online survey addressed to affected persons and their caregivers**.

The survey will be carried out in Autumn 2023–Spring 2024.

It will assess patients' quality of life and the burden of the disease on their families, as well as their access to and satisfaction with social and healthcare services. The same survey will also evaluate the economic burden of EB, including the costs of healthcare and informal care, the financial impact on families and productivity losses.

Finally, the data obtained will be compared to findings collected 10 years ago in a similar European study on rare diseases including EB, the BURQOL-RD study.

The second main activity of BUR-EB is a qualitative study that will involve patients and caregivers together with physicians in a **co-creation process to design maps of patient care and need trajectories** (patient journey maps) **and to develop capacity to build informational materials for improved self-management**.



## Expected results and impacts

**BUR-EB results will be available in 2025.**

**Thanks to the active participation of individuals affected with EB and their families,**

- BUR-EB will provide an **updated, complete and quantitative assessment of the current socioeconomic burden of EB**.
- A comparison of BUR-EB outcomes with information collected a decade ago by the BURQOL-RD project will **highlight changes in the burden of EB over time in European countries**.
- BUR-EB results could be exploited by **pharmaceutical companies to design trials with novel molecular therapies or repurposed drugs for EB**.
- **BUR-EB results should stimulate the scientific community to promote future socio-economic and clinical research** on rare diseases.
- The **tools and methods** developed by BUR-EB can be applied by patient associations, the ERN-Skin network, the European Union, and national or local authorities to monitor the socio-economic impact of the illness over time and the changes that can be produced by new care models, health policies or novel therapeutic options.
- The **qualitative study** developed in BUR-EB **will identify patients' self-management needs and produce materials to reduce the related burden**.





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[www.bur-eb.com](http://www.bur-eb.com)

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