

Project ID: 2019-2.1.7-ERA-NET-2022-00055

Project title: Changes in the Socio-economic Burden of Epidermolysis Bullosa in Europe - BUR-EB research project (2022-2025)”

RESEARCH REPORT

Changes in the Socio-economic Burden of Epidermolysis Bullosa in Europe - BUR-EB research project (2022-2025)

Project description

Prof. Dr. Márta Péntek

Consortium:

Austria
Bulgaria
France
Germany
Hungary
Italy
Spain

October 26, 2025.



ÓBUDAI EGYETEM
ÓBUDA UNIVERSITY



NATIONAL RESEARCH, DEVELOPMENT
AND INNOVATION OFFICE
HUNGARY

PROJECT
FINANCED FROM
THE NRDI FUND

CHANGES IN THE SOCIO-ECONOMIC BURDEN OF EPIDERMOLYSIS BULLOSA IN EUROPE

BUR-EB RESEARCH PROJECT (2022-2025)

PARTICIPATING PARTNER FROM HUNGARY

Health Economics Research Center (HECON), University Research and Innovation Center,
Obuda University

Leader: Prof Dr. Márta Péntek DSc

Researchers: Prof Dr. László Gulácsi DSc, Dr. Zsombor Zrubka PhD, Dr. Áron Hölgyesi PhD

FUNDING

European Union's Horizon 2020 research and innovation programme under the EJP RD
COFUND-EJP N° 825575

National Research Development and Innovation (NRDI) Fund (2019-2.1.7-ERA-NET)

PROJECT HOMEPAGE

<https://www.bur-eb.com/>

BACKGROUND

Health problem

Epidermolysis Bullosa (EB) is a rare genetic skin disorder with no cure that places an enormous social and economic burden on patients and their families. The disease is characterized with fragile skin that leads to blistering and tearing and patients often suffer from pain. The primary aim of the treatment is to promote wound healing, comfort and prevent pain and re-injury. There are several types and severity levels of the disease, but overall EB can cause lifelong disability that needs ongoing medical care.

Medical devices, bioengineering

Medical devices provide a major contribution to the management of EB and thus the health-related quality of life and life expectancy of patients. Bioengineering (in multidisciplinary teams involving biomedical and biomaterial engineers and clinicians) focuses on the development of skin substitutes (called dressing) over the damaged skin areas to treat healing-resistant wounds.

Disease burden studies, the BURQOL-RD (2010-2013) project

Studies on the disease burden of rare disease, and specifically on the burden of epidermolysis bullosa on the patient, family and society are scarce. Around a decade ago (2010-2013) the so called BURQOL-RD project was funded and conducted in Bulgaria, Hungary, France, Germany, Italy, Spain, Sweden and the UK.¹ The main aim of the BURQOL-RD project was to assess the health-related quality of life (HRQOL) of patients with epidermolysis bullosa (EB), mucopolysaccharidosis (MPS), Duchenne muscular dystrophy (DMD), haemophilia, cystic fibrosis, fragile X syndrome, histiocytosis, juvenile idiopathic arthritis (JIA), Prader-Willi syndrome, scleroderma and also of their caregivers, and to quantify the disease-related costs from a societal perspective.

The leader of the Hungarian project was Prof Márta Péntek DSc.

Results of the project were published in a Supplement of the European Journal of Health Economics (<https://link.springer.com/journal/10198/volumes-and-issues/17-1/supplement>)

Prevalence and long-term health care and economic commitment

As a result of more and more effective health care technologies, drugs, devices and other pieces of technology, the life expectancy of the patients living with different rare diseases is continuously and significantly increasing. The prevalence of rare diseases is growing. Longer life expectancy of the patients means long term need for health care services and increasing economic commitment. The underlying hypothesis of the BUR-EB project is that the prevalence and societal burden of rare diseases are growing, the public health importance of rare diseases is increasing. More attention and more innovation are needed, the cost, cost-effectiveness of the disease management is changing and the health policy and financing situation might be changing as well.

RESEARCH AIMS

This international research project (BUR-EB) funded by the European Union's Horizon 2020 programme and NRDI Fund (Hungary) aims to estimate the socio-economic burden of EB in 7 EU countries (Spain, France, Germany, Italy, Hungary, Austria and Bulgaria), and to compare it to data collected 10 years ago in a previous project (named BURQOL-RD).

Data related to the impact of EB on every-day life are collected from affected persons and their caregivers via an anonymous survey in collaboration with clinicians and patient organizations (DEBRA). The economic burden takes into account costs of healthcare and informal care, financial burden for families and productivity losses of all affected persons. Quality of life and family burden are also measured. Besides, affected persons will co-create a map of their trajectories of care and needs. Based on these needs, information materials will be developed to help patients and their families cope better with the illness.

¹ Funding: European Union in the framework of the Health Programme (grant A101205);
<http://www.burqol-rd.com/uk.html>

Title of the research project: Social economic burden and health-related quality of life in patients with rare diseases in Europe (BURQOL-RD)

Type of research grant application: EU Health Programme -2013, year: 2010-2013; Project budget: 50 000 EUR (Hungary)

HECON team (Obuda University) leads the data analysis and cost-of-illness assessment in all the 7 countries of the project and participates in all work packages offering health economics expertise.

EXPECTED RESULTS AND IMPACT

BUR-EB offers an opportunity to observe how the social and economic impact of EB has changed over the last decade and how these changes could be connected to the health and social policies implemented during that period. The project will provide tools that could be used in clinical studies of novel therapeutic options. BUR-EB is backed-up by a balanced multidisciplinary group of internationally recognized clinical dermatologists, health economists, qualitative research experts, biostatisticians and representatives from patient organizations.

EXTENDED PROJECT DURATION

The international research project has been extended until 31 December, 2025.

Justification:

The BUR-EB international research project has started patient recruitment and data collection for the quantitative study with a significant delay. This is due to reasons outside our working group (HECON, Obuda University). In several of the participating countries, obtaining ethical approval took longer than planned. In addition, the programming (lead by the consortium leaders) and finalisation of the questionnaire took longer than planned, with delays in the completion of the online questionnaires in different language versions.

As a result, the data collection (patient survey) in the quantitative questionnaire study started in May 2024 instead of the planned start in September 2023 and was completed in December 2024.

Our working group (HECON, Obuda University) is leading the 4th work package (WP4). The WP4 cleaning the data and database received from the quantitative questionnaire survey mentioned above, analysing the data, calculating the burden and cost of disease, synthesising the results for the participating countries of the consortium. The preparatory work has been done (development of unit costs for the seven participating countries, data processing plan), and the incoming data are continuously monitored. However, the actual data cleaning and analysis can only start after the data collection is completed.

The consortium leader and the participating countries are responsible for the implementation of the data collection and evaluation process. The consortium leader and the participating countries have submitted an extension request to their funding agencies. As our work, data analysis, is key to achieving the main objectives of the research programme, analysing patient data and producing the final report, it seems appropriate to accommodate the extension requests from the consortium countries. If we exit earlier than the other partner countries, we would not be able to participate in answering new questions as they arise, in preparing and finalising the final research report, in the final technical conference (7 October 2025) and would have a short timeframe for disseminating our results.

The extension will not jeopardise the implementation of the project, the original commitments will continue to be met and no additional costs will be incurred.

RESULTS

BUR-EB: Changes in the socio-economic burden of Epidermolysis Bullosa in Europe:

Situational analysis

Available:

<https://drive.google.com/file/d/1Xf5uCI0pxWQkLUkGBgEU9nxWbmMV7AAI/view>

Cross-sectional questionnaire survey:

Data collection was finished in December 2024.

Number of adult patients by countries:

Austria	Bulgaria	France	Germany	Hungary	Italy	Spain
N = 27	N = 16	N = 74	N = 34	N = 9	N = 27	N = 20

Number of child patients by countries:

Austria	Bulgaria	France	Germany	Hungary	Italy	Spain
N = 19'	N = 15'	N = 36'	N = 24'	N = 13'	N = 20'	N = 15'

Informal caregivers of adult patients

Austria	Bulgaria	France	Germany	Hungary	Italy	Spain
N = 11	N = 6	N = 8	N = 8	N = 3	N = 15	N = 9

We would like to express our gratitude to Dr Márta Medvecz associate professor (Semmelweis University, Budapest, Hungary) for her work in the data collection in Hungary, as well as to the participating patients and their families.

Data processing

Data cleaning has been performed, the cost database has been finalised, and analyses are ongoing.

Publications

Arias-Merino, G., Benito-Lozano, J., Linertová, R., Verónica Alonso-Ferreira on behalf of the BUR-EB Study Group. Health-related quality-of-life evaluation in epidermolysis bullosa: a scoping review protocol. *Syst Rev* 14, 159 (2025). <https://doi.org/10.1186/s13643-025-02918-9>

Arias-Merino G, Benito-Lozano J, Gómez-Martínez M, Villaverde-Hueso A, Linertová R, Alonso-Ferreira V, BUR-EB Study Group. *British Journal of Dermatology*, Volume 193, Issue 5, November 2025, Pages 856–864, <https://doi.org/10.1093/bjd/ljaf274>

El Hachem M, Diociaiuti A, Zambruno G, Samela T, Ferretti F, Carnevale C, Linertová R, Bodemer C, Murrell DF, Abeni D. “Quality of Life in Epidermolysis Bullosa” and “Epidermolysis Bullosa Burden of Disease”: Italian translation, cultural adaptation, and pilot testing of two disease-specific questionnaires. *Ital J Pediatr*. 2024 Apr 19.

Linertová R, Péntek M, Rodríguez-Díaz B, Bodemer Ch, Hübl V, El Hachem M, Salamon G, Alonso-Ferreira V, Stefanov G, Jain R, Ramallo-Fariña Y, García-Pérez L, BUR-EB Study Group. Health-related quality of life in adults with epidermolysis bullosa: a cross-sectional study in seven European countries using EQ-5D-5L. (Submitted to *Orphanet Journal of Rare Diseases* on the September 29, 2025)

Planned original articles:

- Cost-of-illness of EB in seven European countries - *The European Journal of Health Economics*, original article (lead by HECON)
- Quality of life in EB, results with the EQ-5D-5L questionnaire (HECON with the Spanish Group)
- Validation of Hungarian version of the QOLEB questionnaire (HECON and Austrian Group)
- Validation of Hungarian version of the EB-BoD questionnaire (HECON and the Austrian Group jointly with the BUR-EB Study Group)
- Out of pocket (led by Spanish Group, HECON is partner jointly with the BUR-EB Study Group)

Conference abstracts:

- - ISPOR Europe 2025 (<https://www.ispor.org/conferences-education/conferences/upcoming-conferences/ispor-europe-2025>)

We have submitted three abstracts to the ISPOR Europe 2025 conference (The International Society for Pharmacoeconomics and Outcomes Research - ISPOR) held in Glasgow (Scotland) on 9-12 November 2025.

- Áron Hölgyesi, Renata Linertová, Lidia García-Pérez, László Gulácsi, Zsombor Zrubka, Márta Péntek and the BUR-EB Study Group. Resource Utilization Among Patients with Epidermolysis Bullosa: Preliminary Findings from Seven European Countries
- Márta Péntek, Márta Medvecz, László Gulácsi, Zsombor Zrubka, Renata Linertová, Áron Hölgyesi and the BUR-EB Study Group. Health-related Quality of Life of Patients with

Epidermolysis Bullosa in Hungary: Results with the EQ-5D-5L and EQ-5D-Y-3L
Preference-based Measurement Tools

- Renata Linertova, Christine Bodemer, May El Hachem, Cristina Has, Gudrun Salamon, Márta Péntek, Georgi Iskrov, Olivia Mullins, Verónica Alonso-Ferreira, Benjamin Rodriguez-Díaz, Aránzazu Hernández Yumar, Lidia García-Perez and the BUR-EB Study Group. Out of pocket expenditure of families affected by epidermolysis bullosa in Europe.

Accepted abstracts will be published in Value in Health journal.

Conferences

On 6 June 2025, we held a mini-conference at the Obuda University to present and discuss the results of BUR-EB. In addition to HECON researchers from Obuda University, the event was attended by Renata Linertova (Consortium Leader, Spain), Dr. Márta Medvecz (Associate Professor, Department of Dermatology, Venereology and Dermatoooncology, Faculty of Medicine, Semmelweis University, Budapest, Hungary) the programme's Hungarian dermatology expert, and Dr. Gábor Pogány, representing the National Association of People with Rare and Congenital Disorders (RIROSZ).

Mini-conference presenters and contributors, 26 June 2025, Obuda University



*Prof. Dr. László Gulácsi László (HECON), Dr Márta Medvecz, Prof. Dr. Márta Péntek , Renata Linertova (Spain), Dr. Áron Hölgyesi (HECON)
Dr. Gábor Pogány (RIROSZ), Dr Zsombor Zrubka (HECON)*

FINAL CONFERENCE

The final conference has taken place on the 7th October 2025, in Spain (Santa-Cruz de Tenerife; Fundación Canaria Instituto de Investigación Sanitaria de Canarias, FIISC).

<https://www.bur-eb.com/2025/10/14/final-conference-of-the-european-project-bur-eb-in-santa-cruz-de-tenerife/>

The Final Conference of the European project “Changes in the Socio-economic Burden of Epidermolysis Bullosa in Europe” (BUR-EB), coordinated by Renata Linertová, was held on October 7th, 2025, in Santa Cruz de Tenerife, with the participation of representatives from all project partners and patient associations, DEBRA International and national DEBRAs.

The meeting focused on the achievements related to the two aims of the project: 1) assessment of EB socio-economic costs and impact on quality of life through an online survey addressed to affected individuals and their caregivers in seven European countries, and 2) a qualitative study focusing on the development of educational materials and patient journey maps in collaboration with patient associations.

Thanks to the dissemination activity by International and national DEBRAs, the survey resulted in a high participation rate with 349 complete questionnaires (207 adults and 142 children), representing the largest dataset on EB available to date.

Áron Hölgyesi (HECON, OU) presented the costing methods and results. Analysis of direct and indirect healthcare costs showed the major contribution of caregiver care, accounting for about 60% of total costs. Though with differences between European countries, costs were higher for children and severe EB types. Most costs were covered by National Health Services, but some expenses continued to be paid by affected individuals and families with a significant financial impact.



Presentation of Áron Hölgyesi PhD (HECON, OU) at the BUR-EB final conference.

Evaluation of quality of life with different generic and disease-specific instruments confirmed the major burden of EB and its correlation with disease severity. At the same time, findings highlighted a profound emotional and psychosocial impact also in individuals with less severe EB types. Of note, the BUR-EB project allowed the translation and validation in four additional European languages of an EB-specific questionnaire which will represent a useful instrument for patient-reported outcome measures in future clinical trials.

The qualitative study was carried out in strict collaboration with patient associations, EB individuals and their careers. Fourteen educational materials were produced representing:

- i) useful and accessible empowerment tools helping affected individuals and their families to cope with EB multifaced challenges, and
- ii) informative and clear instruments to disseminate disease knowledge in order to prevent social isolation.

In parallel, three Patient Journey Maps were developed illustrating patient and caregiver specific experiences and challenges during disease course.

Final discussion focused on the dissemination strategy of both project results and educational materials, in which patient organizations will play a key role.



Representatives of the BUR-EB Study Group at the Final Conference

Scientific visit of Áron Hölgyesi PhD at SESC/FIISC

<https://www.bur-eb.com/2025/10/16/scientific-visit-of-researcher-aron-holgyesi-hecon-budapest/>

On October 8, SESCO/FIISC welcomed researcher Áron Hölgyesi, member of the Health Economics Research Center (HECON) at Óbuda University (Budapest, Hungary).

During his visit, Dr. Hölgyesi gave a scientific talk presenting the main research lines of his center, focused on health economic evaluation and the measurement of disease impact on quality of life.

He also shared the results of the cost study on Epidermolysis Bullosa (#EB) conducted within the framework of the European BUR-EB project, coordinated by researcher Renata Linertová (SESCS/FIISC). His presentation sparked discussion on the applied methodologies and the implications of the findings for health policies and rare disease management in Europe.

The visit helped strengthen collaboration between SESCO and HECON, promoting the exchange of knowledge and experience in health economics and European research on rare diseases.



Both SESCC and BUR-EB has reported on Dr Hölgyesi's visit on their websites.

SESCC web:

← → ↻ sescs.es/en/ [Icons: Home, About, Projects, Reports, CPG/OEBP, Scientific Production, Networks, News and Communications, Contact]

Using electro-anatomical mapping system for mapping vs. fluoroscopy: a systematic review, meta-analysis and economic evaluation

Beatriz León-Salas, Diego Infante-Ventura, Aránzazu Hernández-Yumar, Renata Linertová, Estefanía Herrera-Ramos, Alejandra Torres-Castaño, Hilmar González-Pacheco, Analia Abt-Sacks, Javier García-García, Raúl Quirós-López, ...

3 DAYS AGO

NEWS

New Version of the Clinical Practice Guideline on Autism Spectrum Disorder in Primary Care

The Andalusian Agency for Health Technology Assessment (AETSA) has updated the Clinical Practice Guideline for the care of Autism Spectrum ...

OTHER REPORTS

Scientific visit of researcher Áron Hölgyesi (HECON, Budapest)

On October 8, SESCC/FIISC welcomed researcher Áron Hölgyesi, member of the Health Economics Research Center (HECON) at Óbuda University (Budapest, Hungary). ...

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BUR-EB web:



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Next conference:

Hungarian Science Festival, 2025 November