

Rt Hon Sajid Javid MP  
Secretary of State for Health and Social Care  
Department for Health and Social Care  
39 Victoria Street  
London  
SW1H 0EU

10 May 2022

Dear Secretary of State,

**Strengthening the UK's position as a global leader in developing treatments and finding cure(s) that will end suffering for thousands of people living with Epidermolysis Bullosa (EB).**

**REQUEST FOR FINANCIAL SUPPORT.**

DEBRA is the national charity supporting people living with or directly affected by Epidermolysis Bullosa (EB) a group of rare, currently incurable, genetic disorders that result in extreme fragility of the skin, blisters, open recurring wounds and sores which can form with the slightest touch or rub. The condition is often referred to as 'butterfly skin' as the skin is as fragile as a butterfly's wing.

EB sufferers live in constant debilitating pain, often with significant scarring, disability, disfigurement, and a considerable risk of skin cancer, which is usually fatal in early adulthood. The most severe form of EB is usually fatal in early infancy. There is currently no cure for EB and management is supportive toward improving quality of life.

DEBRA was founded in 1978 and was the worlds' first EB patient support group. There is now a DEBRA organisation in over 50 countries and DEBRA is the only UK charity specifically aimed at supporting anyone living with EB from initial diagnosis and throughout their lives. We currently have 3,279 members but estimate there are at least 5,000 people suffering with EB in the UK and 500,000 worldwide.

Not only does EB cause suffering to thousands of people significantly impacting their health-related quality of life but it also has a high socioeconomic cost in terms of health resource utilisation, informal care, and labour productivity losses. A 2016 study, titled 'Social/economic costs and health-related quality of life in patients with epidermolysis bullosa in Europe' led by the London School of Economics looking at patients with EB within EU member states<sup>1</sup> revealed that the average annual cost of direct healthcare and non-healthcare per patient with EB was estimated at £26,000, suggesting that the annual cost to the NHS of managing EB in the UK could be up to £130m per annum.

The same study revealed a high prevalence of psychological problems and psychiatric symptoms indicating the importance and associated cost of providing appropriate psychological and peer support, in tandem with pain management and nursing support.

---

<sup>1</sup> Eur J Health Econ (2016) 17 (Suppl 1):S31–S42

## **Our activities and impact:**

- DEBRA works in partnership with the NHS to deliver an enhanced healthcare service for people suffering with EB.
- DEBRA funds pioneering research and work with researchers at the best institutions in the UK and globally to improve treatments and toward finding cures for all types of EB.
- DEBRA has invested over £20 million to support 134 research projects and 107 researchers at 52 research sites across 12 countries over the last 20 years.
- DEBRA collaborates internationally with our network of DEBRA groups and organised *EB 2020*, the first EB world congress, which brought together experts in the field of EB research, clinicians, patients, support groups and over 20 biotechnology companies to share state-of-the-art knowledge and best practice on EB.

Through our international approach we have been responsible for establishing much of what is known about EB, all with a focus on management strategies, treatments, and cures. We continue to mobilise the EB community and build strong partnerships to deliver for people living with EB, driven by our pursuit of impact and patient outputs.

## **Our vision**

Our vision is a world where no one suffers with EB. We have a path to make our vision a reality but to achieve this we need Government support.

## **How we will end suffering from EB**

Drug repurposing presents a clear, achievable path to end suffering and improve quality of life for people living with EB.

As could be seen in the fight against COVID-19 where Tocilizumab, a drug originally developed to treat arthritis, was repurposed to provide an effective treatment for hospitalised patients.

Repurposing drugs already approved by the MHRA and NICE offers the potential to release a rich source of safe, effective and potentially life changing treatments for EB patients at a much lower cost than traditional drug discovery and in a much shorter timeframe.

Research has shown that EB is an inflammatory condition like Psoriasis and Atopic Dermatitis (severe eczema) which are treated with anti-inflammatory drugs (biologics) and initial work has shown us that treating EB patients with some of these psoriasis and eczema drugs can significantly improve their blistering and patients' quality of life.

DEBRA have already identified candidate anti-inflammatory drugs for re-purposing which we think could be successful in slowing, stopping, or reversing the progression of EB and we have committed to raise £10 million by 2026 to find treatments to improve the quality of life of EB patients.

## **We seek Government help – of £10m in matched funding to accelerate drug repurposing**

**With your support and commitment to match fund our overall £10m fundraising target, we can accelerate our clinical trial programme and commit to testing up to 20 drugs, which will greatly increase the potential to offer treatment and provide hope to thousands of EB sufferers in the UK and around the world.**

To provide greater clarity, and by way of a follow up to the study in 2016 led by the LSE and published in the European Journal of Health Economics, DEBRA is funding a project with the National Congenital Anomaly and Rare Disease Registration Service (NCARDRS, part of NHS Digital) to better understand all parts of the patient journey from initial diagnosis through to treatment and to obtain an accurate estimate of costs incurred in supporting those living with EB in the UK.

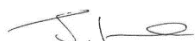
Based on results of the previous study, we currently believe that supporting those living with EB costs the NHS up to £130m per annum creating significant scope for potential savings that successful drug repurposing might bring. Each drug costs the NHS approx. £10,000 per patient per year creating a potential saving of £16,000 per patient per year or in total £80m per year.

Providing significant improvements in quality of life and real hope toward finding cures will simultaneously lift many of the mental stresses placed on EB sufferers and their families.

We need to act now.

DEBRA would welcome the opportunity to talk to you or your ministerial colleagues about how we **#FightEB** and how Government can support in accelerating our journey.

Yours sincerely



Jim Irvine  
Chair of Trustees  
DEBRA UK



Tony Byrne  
CEO  
DEBRA UK