

DEBRA International: International cooperation to improve healthcare access for patients with epidermolysis bullosa

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Epidermolysis bullosa (EB) is a group of genetic conditions causing blistering to the skin and body linings which vary in the extent of symptoms but are always painful and disabling and often life threatening. In its most severe form it is fatal in infancy. Currently there is no cure or effective treatment but good management greatly improves quality of life and current research offers good prospects for therapy development.

DEBRA International is the international organisation coordinating the activities of national EB patient support groups, currently working in over 40 countries, with a strong presence in Europe, North and South America and Australasia and a growing membership in Asia, the Middle East and North Africa. The objectives, both internationally and nationally, are to bring about lasting and effective treatments for all forms of EB as quickly as possible and to provide services to help currently affected individuals and families. DEBRA International recognises that these aims can only be met in partnership with clinicians, researchers, industry, other research funders and governments.

Amongst our current priority areas of work are:

- Funding and facilitating research to develop innovative treatments including gene, cell, protein and small molecule therapies; a number of which are in early stage human clinical trials or where such trials are on the horizon. Normally two calls for research grant applications are made each year and, unusually, there is a single system of international peer review used by all of the DEBRAs funding significant amounts of research. On average, we invest around €3 million each year into new research. An invitation-only research planning conference is held every three years, involving

the leading research teams worldwide together with expert patients and industry, to identify opportunities and barriers facing therapy development.

- Identifying potential partners and advisers in industry and venture capital, recognising that expertise in bringing products to market is essential in translating the significant scientific advances being made into available treatments in the clinic.

- The generation of best practice clinical guidelines by groups of specialist clinicians in various areas of importance to people with EB. The guideline on dental care is about to be published and guidelines on cancer management, pain management, nutrition, physical therapies and wound care are in preparation.

- Creating stronger clinical networks of specialist EB centres worldwide to promote sharing of expertise and to facilitate clinical trials. The inaugural meeting of this formal network, EB-CLINET, will be held in Salzburg on 5-7 October 2012.

- The establishment of clinical training opportunities for professionals interested in starting, or improving, a specialist EB clinical service in their own countries including, it is hoped, an online, modular course and mentoring. This programme will be launched at EB-CLINET.

- The creation of an international patient-reported database to understand better the natural history of different forms of EB and the costs of living with the condition. This registry, EBCare, is internet based, www.EBCare.org, and we welcome the help of health professionals in encouraging patients to register. It is currently published in English with Spanish and other languages to follow shortly.

DEBRA International welcomes contact and partnership with researchers, health care professionals, industry and anyone interested in our work. For more information, visit www.debra-international.org.

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