Patient Organizations and the Investigative Dermatology Community as Partners: DEBRA and Epidermolysis Bullosa Research

DEBRA International actively encourages partnership between patients and the research community to develop treatments for epidermolysis bullosa (EB). Triennial conferences, such as EB2012, are important for identifying challenges and opportunities, establishing a research strategy, and, increasingly, bringing together all stakeholders to create new partnerships.

DEBRA International (http://www.debra-international.org) is the alliance of national EB patient support groups working in well over 40 countries to support people with the condition. Our main programs are the promotion and funding of research into finding effective treatments, working with clinical experts to improve the day-to-day care of patients, and assisting people with EB as well their organizations to be more effective on a national level.

The triennial research conferences organized by DEBRA International are a vital part of our relationship with clinicians and nonclinical researchers. Similar conferences had been held irregularly in the past, but they have been scheduled regularly since 2006, when EB2006 (Featherstone, 2007) was held in Dublin, followed by EB2009 (Uitto *et al.*, 2010) in Vienna and EB2012 in Marbella. These by-invitation meetings bring together the leading international players in EB research and are exceptionally effective in identifying both potential opportunities and barriers to progress, thereby determining DEBRA's research strategy and priorities for the following three years. We are grateful to the Scientific Programme Committee, speakers, and delegates for consistently supporting these meetings and for the groundbreaking work they are doing in taking us toward treatments for EB. Anyone reading the EB2012 report in this issue will be impressed by the great progress being made in many different potential therapies.

Because EB is a rare condition—or, more strictly a collection of even rarer conditions—people living with the disorder feel a strong responsibility to play a full part in the search for treatments. Patient-organization funding for research has been, and continues to be, an important driver in improving the understanding of EB, particularly in earlier days, when alternative funding sources were few and far between. Our aim then, as now, was to attract innovative researchers into the field, maintain their commitment to EB as they progress in their careers, and do all we can to ensure that funding is available to keep up the momentum of research. One of the most pleasing aspects of EB2012 was the number of senior researchers participating who received DEBRA support as young researchers and have since become leaders in the field. National initiatives independent of research-project grants, such as the DEBRA UK Fellowships and the DEBRA Austria–inspired EB House in Salzburg (Pohla-Gubo and Hintner, 2010), have also played a part in supporting the EB research community.

From its earliest days, the members of DEBRA International have seen the importance of an international perspective in the search for treatments. The mantra has always been that "we want to fund the best research, not just the best national research." Our centralized international peer review system, overseen by DEBRA's International Medical & Scientific Advisory Panel, gives member groups the ability to pool funding, avoid duplication of effort, and maintain a common strategy. We are grateful to the many eminent researchers who have served on the panel over the years.

We now face new challenges. As the conference report shows (Bruckner-Tuderman *et al.*, 2013), exciting work is opening up the prospect of gene, cell, protein, and small-molecule therapies that may, for the first time, transform the lives of patients. Although funding or facilitating

Journal of Investigative Dermatology (2013) **133**, 2116–2117. doi:10.1038/jid.2013.257

basic and preclinical research and early-stage clinical studies continues to be at the core of DEBRA International's mission, we know that this is not enough to take therapies into the clinic. The funding and expertise required to take these new therapies to market do not lie in the patient group or, in most cases, in academia. New partners are needed from the pharmaceutical and biotechnology industries, as well as from venture capital, to take therapies forward. EB2012 gave us the opportunity to build on the foundations created by EB2009 and invite more industry representatives to participate as speakers and delegates and provide their perspective on the way forward. In previous conferences, contacts made between researchers and industry have led to some fruitful collaborations, such as the fibroblast-therapy (Wong *et al*., 2008) trial led by John McGrath in conjunction with Intercytex. Our expectation is that EB2012 will lead to more such partnerships.

This increased interest in EB shown by industry has been a welcome development for the patient group; companies are now coming to us rather than our having to pursue them. The presentation made during EB2012 by Mark De Souza of Lotus Tissue Repair (since acquired by Shire HGT) on his company's program to take protein therapy from interesting results in animal models into the clinic gave valuable insights into how the various elements—science, epidemiology, production capacity, regulation, partnership with patients, and reimbursement—must be woven together from the beginning.

Another key element of DEBRA International's strategy is to fund or facilitate initiatives that support and accelerate the research effort or the development of clinical trials: such a commitment will also make it more likely that external partners will see EB therapy development as an attractive and viable investment. Our advisers on DEBRA's Translational Research Advisory Panel and elsewhere have emphasized the importance of (i) a validated and well-documented natural history of the various types of EB in establishing robust clinical end points, (ii) good measures of disease severity and quality of life, and (iii) health-economics data and patient registers specific to the condition. DEBRA International is already taking many of these forward; in addition to having commissioned clinical–end point validation studies, DEBRA hosted working groups at EB2012 on the development of clinical tools such as quality-of-life measures and severity scores as early steps in achieving EB community–wide consensus. These discussions continue.

DEBRA is very conscious that the main drivers in therapy development are innovative researchers and developments in technology. Our role will continue to be to work closely with our research colleagues to support them in their work and to help create a framework that fosters the advent of effective treatments in the clinic as quickly as possible.

The next conference—EB2015, to be held in the United States—is already in preparation. We are excited by the prospect of what the next three years will bring.

Rainer Riedl and John Dart

DEBRA International, Vienna, Austria

Correspondence: Rainer Riedl, DEBRA International, Am Heumarkt 27/1, 1030 Vienna, Austria. E-mail: rainer.riedl@debra-austria.org

REFERENCES

Bruckner-Tuderman L, McGrath JA, Robinson EC *et al*. (2013) Progress in epidermolysis bullosa research: summary of DEBRA International Research Conference 2012. *J Invest Dermatol* 133:2121–26

Featherstone C (2007) Epidermolysis bullosa: from fundamental molecular biology to clinical therapies. *J Invest Dermatol* 127:256–9

Pohla-Gubo G, Hintner H (2010) Epidermolysis bullosa care in Austria and the epidermolysis bullosa House Austria. *Dermatol Clin* 28:415–20

Uitto J, McGrath JA, Rodeck U *et al*. (2010) Progress in epidermolysis bullosa research: toward treatment and cure. *J Invest Dermatol* 130:1778–84

Wong T, Gammon L, Liu L *et al*. (2008) Potential of fibroblast cell therapy for recessive dystrophic epidermolysis bullosa. *J Invest Dermatol* 128:2179–89