debra International.

Annual Review 2015

Working Together for People Living with EB
Contents

DEBRA Worldwide Map                        Page 1 & 2
DEBRA International                        Page 3
Our Achievements                           Page 4
The Role of the Executive Committee        Page 5
Research                                   Page 6 & 7
Patient Care                               Page 8 & 9
Mentoring                                  Page 9
External Partnerships                      Page 10
Executive Officer Report                   Page 10
Communications                            Page 11
Financial Accounts                         Page 13 - 15
DEBRA Worldwide

Argentina, Australia, Austria, Belgium, Bosnia and Herzegovina, Brazil, Bulgaria, Canada, Chile, China, Colombia, Costa Rica, Croatia, Czech Republic, Finland, France, Germany, Greece, Hong Kong, Hungary, Iran, Ireland, Italy, Japan, Korea, France, New Zealand, Netherlands, Norway, Peru, Poland, Portugal, Romania, Russia, Serbia, Slovakia, Slovenia, South Korea, Spain, Sweden, Switzerland, Taiwan, Turkey, Ukraine, UK, USA, Vietnam, International.
Dear DEBRA members,

Looking back at this past year, I am grateful to see that we – DEBRAs and EB support groups all over the world – have yet again taken some more steps to improve the lives of people living with EB.

This review covers just a small part of what is going on, and there is still so much more to do – together.

DEBRA International is a not for profit worldwide charity working on behalf of those affected by the genetic skin blistering condition Epidermolysis Bullosa (EB).

Our vision is to ensure that people living with EB have access to the best quality support and medical care, while also funding development of effective treatments and cures for EB.

With a growing membership of over 50 National DEBRA groups, the membership has set the following priorities during a survey in late 2014:

- Clinical Practice Guidelines
- Communications
- EB Without Borders (Mentoring)
- Professional Training & Development
- Operations & Administration
- Small Grants Scheme

The 2014 member survey showed an overall satisfaction for DEBRA International above 72%

Dr. Rainer Riedl
President, DEBRA International

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Our Achievements

Over the years, our achievements include:

- In promoting care for people living with EB, the international focus allows the development of better clinical care by sharing expertise and best practice and by promoting opportunities for joint training.

- The organisation of multi-centre clinical trials also depends heavily on these international clinical collaboration. Finally, progress in research and access to clinical care globally can be affected positively by this participation and guidance by the patient lead support group.

- Assisting in the establishment of DEBRAs in different continents. DEBRAs EB Without Borders Team continues to work with EB CLINET to make effective connections in developing new DEBRAs.

- Recognition in the international rare disease world as a major player, with significant influence in international patient associations and policy development.

- Clinical Practice Guidelines on Dental Care, Wound Management and Pain Management have now been completed with great reviews.

- Ongoing capacity building sees more countries taking part in research and care initiatives.

- Becoming the main point of contact with the worldwide Research and Care community. DEBRA has great experience at bringing all stakeholders together.

- Cancer Care Guidelines will be available very soon.

- A single updated system of peer review research grant applications and shared funding of projects with national DEBRA groups.

- For more information or to keep up to date go to: www.debra-international.org
The Executive Committee has representatives from National DEBRAs. Committee Members are elected on a rotational basis every three years and in line with good governance the charity is committed to accountability and transparency for its decisions and activities. Committee members have responsibility for setting the strategic direction as well as directing the activities of the organisation. The Committee meets annually at the DEBRA International conference, has monthly conference calls and also devote a day to setting out its aims for the following 12 months.

**DEBRA International Work Streams**

The key priorities for the organisation are driven by the leaders of the following work streams:

- **Research**
- **Patient Care**
- **External Partnerships**
- **Mentoring**
- **Communications**
- **Revenue Generation**

**Research**

DEBRA International (DI) has re-developed its research strategy for the period 2015-2019, to provide greater focus – and funding streams – to support symptom-relief research. This is in response to consultation by DI and some national DEBRA groups with EB clinician-researchers, as well as with the DEBRA membership over the past year or so: both clinicians and people with EB expressed a wish for increased prioritization of research into treatments to improve quality of life. This will complement continuing strategic support for discovery-research to better understand EB disease mechanisms, with the aim of developing novel concepts for improved therapies, and support for clinical trials of promising therapies targeting the causes of EB.

In line with the strengthened strategic priority for clinical research, DI launched a major Clinical Research Program call in Spring 2015. This welcomed proposals for clinical studies or clinical trials of therapies that address either the underlying causes of EB, or the severe life-limiting consequences of EB, or symptom-relief treatments to address clinical consequences of EB that impact upon daily quality of life. With a two-stage application process, a specially convened Expert Panel will meet to assess peer-reviewed proposals in December 2015. The program will offer up to €500K each to successful projects, and anticipates making awards before the end of 2015.

Another new strategic priority, to support the next generation of clinician-researchers, and development of research infrastructure, will be addressed by additional initiatives to be developed in 2016 and beyond.

A further strategic priority is outreach to future partners in therapy development and delivery. DI provides information about EB and patient priorities to those – primarily biopharma companies – planning clinical trials. DI staff continued to respond to enquiries, and reach out to potential partners interested in EB. Notable interactions over the past year have been with Scioderm (re clinical sites for the Phase 3 trial of a topical treatment for all EB types), and Shire (re socioeconomic impact of living with EB, to inform development of a protein therapy for RDEB).

Over the past year, DI has committed just over €2.2M, funded by research-active national DI members, (primarily DEBRA Austria and DEBRA UK), to 11 new research projects. These have been identified through DI’s ‘Ordinary calls’ for proposals across all four of DI’s research priorities (EB disease mechanisms, wound healing and cancer, symptom relief and clinical care, and development of therapies). These ‘Ordinary’ calls for research proposals, across DI’s research priorities, will continue to be held once or twice each year, according to funding availability.
To enhance awareness of patient priorities among clinical researchers, DI introduced for the first time the opportunity for people with EB to comment on research proposals. A ‘Patient Panel’ considered outline proposals received for the 2015 Clinical Research Program call, noting their thoughts on likely benefits of the research, and patient participation in proposed studies, together with any issues or further information they would like to be included. These perspectives were communicated back to researchers to consider in the design of their research and clinical trials. Eleven people with EB from ten countries provided valuable comment; review of their experience will lead to further development of this initiative.

DI provides a centralized peer-review process, to enable national member DEBRAs to combine resources on support for our research priorities and to avoid duplication of research effort or funding. This year, four new members have been appointed to DI’s Medical & Scientific Advisory Panel (MSAP) to bring in expertise in EB cancer research, cell biology of the skin, and in the development of gene- and cell therapies, to reflect the evolving direction of research.

DEBRA International’s triennial research conference, EB2015, was hosted by DEBRA of America in Atlanta, 3-6 May 2015. The conference evidenced the incremental steady progress being made towards development of therapies for EB. Whereas earlier conferences noted breakthroughs in one or a few areas, EB2015’s presentations reflected the breadth of work in cell-, molecular- and drug therapies, all moving towards clinical application. A report on the conference outcomes will be published in the scientific press, and will be used to help guide future research-support by DI.

Clinical Practice Guidelines

Clinical practice guidelines are the best way to ensure that both the outcomes of clinical research and the specialist knowledge of EB experts are used to shape the care of people with EB.

As many of you will know, DEBRA International is focused on developing a series of such guidelines that will guide the care of the different clinical aspects of EB. Some of the highlights of the past year are listed along the right.

Dr. Avril Kennan
Head of Research & Advocacy, DEBRA Ireland

Dr. Clare Robinson
Head of Research, DEBRA International

Highlights

- An excellent guideline on pain care in EB was published and is freely available on the DEBRA International and EB-CLINET websites. This guideline is relevant for all forms of EB and for all clinicians and families who care for people with EB. It is the fruition of many years of hard work by the development team, led by Dr. Kenneth Goldschneider and was funded by DEBRA of America.

- Dr. Jemma Mellerio and colleagues have made great progress on the cancer in EB guideline. We are delighted to report that it is now about to be published and will also be made freely available.

- While clinical practice guidelines are incredibly worthwhile, the task of developing them is not straightforward and can be challenging to those involved. With this in mind, we recently organised for eight people involved in the project to attend a course on guideline development. EB guideline developers (and potential developers) from the areas of nutrition, wound care and hand therapy, myself from DEBRA together with Emma Batten attended and came away inspired.

- Over the course of the year we established strong links with a European initiative, RARE-BestPractices, which aims to improve the methods of developing rare disease guidelines. I was kindly funded by Eurordis to attend two of their excellent and informative events, in Edinburgh and Rome and what I learned there has been very valuable in shaping our future plans.
**External Partnerships**

Partnerships with other organisations with complementary objectives is an important part of DEBRA International’s strategy in the fields of advocacy, research, care and fundraising.

National DEBRAs advocate on behalf of people with EB with their own national institutions. DEBRA International’s role is to advocate regionally and internationally on issues with an international dimension. In a globalised world, many policy decisions made by international institutions affect people with EB. In addition, many awareness raising actions benefit from a cross-border approach, such as EB Awareness Week.

DEBRA International is active in many of the most influential multinational organisations relevant to people with EB.

We welcome the opportunity to invite organisations to talk to us about an effective partnership and such partnerships will be a priority for the Executive Committee over the next 12 months.

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**Executive Officer Report**

I had the privilege of taking up the Executive Officer role at the start 2015. The first few months saw a transfer of a number of tasks from John Dart who is standing down from paid employment with the organisation. We are however fortunate that John has agreed to take on the voluntary role of senior advisor to the Executive. John has been a pivotal person in the ongoing development of the organisation and we wish him all the best for the future.

The last 12 months has been a busy period in preparation for the EB2015 Research Symposium in Atlanta along with EB-CLINET and the DEBRA International Congress in London. We have also seen the increase in Therapies moving forward as outlined in the Research Managers report. We have seen the introduction of the patient panel to review possible research projects as part of the review process.

EB Without Borders team continues to work with developing groups and to support those in places where there is no DEBRA group. A priority of the Executive to work on is the long term sustainability of the organisation while developing robust policy and procedures. We envisage this will lead to improved initiatives along with staff that will benefit DEBRA members and ultimately the quality of life outcomes for people living with EB. I encourage all groups to ensure that their contact details are kept up to date with DEBRA International to ensure groups can be kept up to date on all activities and communications.

Michael Fitzpatrick
CEO, DEBRA International

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**Patient Care**

**Professional Training**

DEBRA International aims to work with, and support, existing centres and to assist interested clinicians to develop their expertise especially in countries where no specialist centre currently exists. During 2014/15 we have worked closely with our clinical colleagues.

**Mentoring**

**EB Without Borders**

The EBWB team have had a busy and productive year working across geographic and political borders to help people with EB, families and professionals in countries where there is little or no information, support or health care. The EBWB team have also supported and helped develop new DEBRA groups to become established. DEBRA and EB are well represented at regional levels in rare disease alliances, with members of the EBWB team on the boards of Eurordis, Radiorg, PARDO, Cord and CARD.

The Spanish speaking countries are well supported by Regional Ambassadors Loreto Moore in Chile and Evanina Makow in Spain. In the past year they have responded to requests from Venezuela, Guatemala, Peru, Puerto Rico, Mexico, Paraguay, Argentina, Dominican Republic, Uruguay, Ecuador, Bolivia, Panama, and Cuba and given particular mentoring support for DEBRA Colombia. As well, Evanina has assisted people in Israel, Lebanon, Poland and Morocco.

Regional Ambassador for South East Europe, Vlasta Zmazek, with the support of Polona Zakosek, has been networking steadily with Bosnia, Serbia, Albania, Montenegro, Kazakhstan and Slovenia building a solid support base for people in that region with EB.

With Anna Kemble Welch’s leadership, the emergence of DEBRA Malaysia and DEBRA Singapore, the flourishing DEBRAs of Japan and Taiwan and the developing DEBRA China, DEBRA now has a strong presence in Asia and Pacific. DEBRA Singapore recently held their inaugural member meeting and is already involved in outreach programs supporting neighbouring countries. EBWB with DEBRA New Zealand will have the first clinical training visit to Sri Lanka this October and is planning a visit to China. EBWB has contact with many other countries including Syria and Pakistan. In the coming year we expect to welcome a DEBRA India, DEBRA Turkey and possibly DEBRA Algeria and DEBRA Tunisia.

**Lea Prujean**
International Coordinator, EBWB

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Communications

Good communications can even bring the most widespread community together. Such is the mission of the DI communications team: to allow for fruitful exchange of ideas and knowledge through a constant flow of information, and to make resources easily accessible for those needing them.

Website

This is our core communication platform: a central hub for the DEBRA community as well as external associates. It provides EB-related information to patients, their families, DEBRA delegates, medical staff, the medical research community and all other groups.

www.debra-international.org

News

We have maintained a steady stream of news articles in the last 12 months, which feed both the news page of the website and the DI newsletter. News include reports on the latest medical research, advances in care and reports on relevant conferences or major events, as well as frequent stories from national groups.

We particularly encourage articles submitted by people outside of the core communications team and so, if you have a story, or would like your group to feature, please get in touch! A news template is available on the website for your use.

Have a look and subscribe to the DI newsletter; you will find a ‘sign up’ button on the homepage of the website.

Social Media

DEBRA International has a strong social media presence, and we encourage you to join us continuing to grow the international EB community on Facebook, YouTube and other channels.

www.facebook.com/DebraInternational
www.youtube.com/user/DEBRAinternational

International EB Awareness Week

The IEBAW takes place in the last week of October each year. Our work simply couldn’t happen without amazing supporters who raise the money that funds it. During this week, DEBRA groups around the world aim to raise awareness and funds for people living with EB. Last year’s IEBAW was the biggest one yet, with many groups around the world harnessing the power of social media to spread the word. Be inspired by a few examples of awareness campaigns on the DI website.

Barbara Dissauer

Communications & Research Management
DEBRA Austria

DEBRA International Accounts 2014
## Profit & Loss Statement
### January 1st 2014 - December 31st 2014

### 1. Revenues

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>68,977.66</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>a) Membership Fees</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Membership Fees</td>
<td>49,392.00</td>
<td>46,248.84</td>
</tr>
<tr>
<td>Research co-funding</td>
<td>0.00</td>
<td>22,364.03</td>
</tr>
<tr>
<td><strong>Total Membership Fees</strong></td>
<td>49,392.00</td>
<td>68,612.97</td>
</tr>
<tr>
<td><strong>b) Other Revenues</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Donations</td>
<td>10,557.27</td>
<td>26,105.68</td>
</tr>
<tr>
<td>Donations via CAP</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>EB Care registry earnings</td>
<td>12,000.00</td>
<td>25,378.96</td>
</tr>
<tr>
<td><strong>Total Other Revenues</strong></td>
<td>22,557.27</td>
<td>51,484.64</td>
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### 2. Other Income

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>50,262.88</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>a) Income from the reversal of accruals</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Dissolution other accruals</td>
<td>843.00</td>
<td>184.00</td>
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</table>

### 3. Other Expenses

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>66,710.51</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>a) Others</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Distribution costs</td>
<td>19,807.78</td>
<td>3,000.00</td>
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<tr>
<td>Advertising</td>
<td>0.00</td>
<td>79.97</td>
</tr>
<tr>
<td>Best practice guidelines</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>EB without borders</td>
<td>35,582.98</td>
<td>19,800.00</td>
</tr>
<tr>
<td>EB CLINET</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td><strong>Research</strong></td>
<td>55,390.76</td>
<td>22,879.97</td>
</tr>
<tr>
<td>Research co-funding</td>
<td>0.00</td>
<td>7,518.84</td>
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<tr>
<td>Professional training</td>
<td>9,332.88</td>
<td>699.21</td>
</tr>
<tr>
<td>Small grants</td>
<td>11,930.00</td>
<td>10,200.00</td>
</tr>
<tr>
<td><strong>Fees &amp; contributions</strong></td>
<td>21,262.88</td>
<td>18,418.05</td>
</tr>
<tr>
<td>EB Care registry expenses</td>
<td>0.00</td>
<td>11,100.00</td>
</tr>
<tr>
<td>Legal &amp; Consulting costs</td>
<td>5,082.35</td>
<td>1,200.00</td>
</tr>
<tr>
<td>Bookkeeping costs</td>
<td>1,399.50</td>
<td>2,321.50</td>
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<tr>
<td><strong>Total Legal &amp; Consulting costs</strong></td>
<td>6,481.85</td>
<td>3,521.50</td>
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### 4. Result of association activities

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>48,601.29</strong></td>
<td></td>
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</table>

### 5. Interest and similar income

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Interest proceeds from bank credits</td>
<td>165.75</td>
<td>364.04</td>
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</table>

### 6. Financial result

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Financial result</td>
<td>-62,832.31</td>
<td>-27,052.65</td>
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</tbody>
</table>

### 7. Profit from association activities

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Profit from association activities</td>
<td>-14,231.02</td>
<td>59,580.59</td>
</tr>
</tbody>
</table>

### 8. Taxes on income

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Capital gain tax</td>
<td>41.44</td>
<td>91.01</td>
</tr>
</tbody>
</table>

### 9. Net Income

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Net income</td>
<td>-14,272.46</td>
<td>59,489.58</td>
</tr>
</tbody>
</table>

### 10. Profits carried forward from the previous years

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Profit carried forward</td>
<td>160,934.65</td>
<td>101,445.07</td>
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</table>

### 11. Balance sheet profit

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 (EUR)</th>
<th>2013 (EUR)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Balance sheet profit</td>
<td>151,848.12</td>
<td>160,934.65</td>
</tr>
</tbody>
</table>
# Revenue Generation

## Financial Review

DEBRA International has a relationship with CAP Partners who act as fundraisers for the organisation in seeking corporate sponsorship and gift in kind support from the healthcare industry.

### Incoming Resources

- **EB Care Registry**: 68% (49,392)
- **Donations**: 17% (12,000)
- **Membership Fees**: 15% (10,557.27)

### Outgoing Resources

- **Internet**: 23% (19,807.78)
- **Membership**: 23% (19,807.78)
- **Financial Transactions**: 40% (35,582.98)
- **Bookkeeping**: 14% (11,930)
- **Travel**: 11% (9,332.88)
- **Professional Training**: 11% (9,332.88)
- **Legal & Consulting**: 6% (5,082.35)
- **Small Grants**: 15% (12,000)
- **Communications**: 15% (12,000)
- **EBWB**: 15% (12,000)