DebRA AUSTRALIA working for a life free of pain

Submission to the Federal Government of Australia For Consideration of a Nationally Funded Dressing Supply Scheme To assist children & adults with Epidermolysis Bullosa

Sample picture from successful EB awareness campaign. Used by DebRA in Austria and Ireland

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Contents

Executive Summary

Epidermolysis Bullosa (EB) is a rare genetic condition which is incurable, and in its most severe forms can be fatal in infancy and childhood. Due to a genetic spelling mistake in certain genes, children and adults with EB do not manufacture the proteins that hold the skin layers together. They have skin as fragile as butterfly's wings so we call them the "butterfly children" or "cotton wool babies". In Australia it is estimated that approximately 1,000 people may be affected by this condition, of whom 100-150 have the most severe form.

This submission describes how EB deeply affects lives. Access to suitable affordable dressings for people with EB is as essential and is as vital to them as is many other medications and treatments that are currently funded by various health programs available to all Australians.

DebRA (National Dystrophic Epidermolysis Bullosa Research Association) Australia and its member State Branches is seeking the support of the Federal Government to fund and implement a national dressing supply scheme for people with EB. This will ensure that all Australians have access to care which is medically accepted as current best practice and address current inequities beacuse the present healthcare system does not have a clear mechanism to deal with patients such as these .

The impact of EB on patients and their families

EB makes the skin as fragile as wet tissue paper. The slightest knock or rub, even rolling over in bed or a mother's hug can cause blistering. The constant damage can cause fingers and toes to completely fuse together. Infection is a constant problem in blistered and eroded areas, and may result in repeated hospitalisation to treat infection and manage ulcerated wounds. Many children are unable to enjoy a normal childhood with all the physical contact it brings. For those children who make it in to adulthood, fatal skin cancer becomes an added threat which may cut short their life expectancy.

Every day children and adults with EB need their blisters pierced, drained and dressed in an endless routine lasting up to 3 hours in the most severe cases. All the mucus membranes maybe affected. What you see on the skin is really just the tip of the iceberg. Even the eating of solids can have a devastating effect on the mouth and oesophagus. As a result many take their food, medication and painkillers through a feeding tube directly into their stomach. Often damage to the eyes can result in a child having to sit in the dark for up to 2 or 3 days until they have healed. They require access to many medical professionals for support and this is done best through a multi disciplinary team approach.

In most cases the care of the person living with EB falls to the parents. This can be a 24 hour a day, 7 day a week job with little to no support or respite options available to many families. This has significant impacts on the family with many living in isolation they become disconnected from their friends and community as well as the ongoing financial burden. Relationships can and do break down. There are significant lifestyle changes required with at least 1 parent needing to give up paid employment in many cases to take on the role of carer. Children need support with access to education and often miss blocks of learning, many needing to attend school with a teacher's aide. Parents are always on call if needed and must spend large amounts of time assisting children catch up on missed learning opportunities and assisting schools care and adapt to the children's needs.

Access to treatment

The current standard of care for dressing the skin lesions of patients with EB is to use dressings such as silicone based dressings , which are much easier to apply and remove, protect the area from infection and create a favourable environment for wound healing. Unfortunately these are relatively expensive and dressings for severely affected patients can cost thousands each month.. However, there are also potential cost-offsets due to the reduced need for antibiotics, medical visits and hospital stays to manage infected wounds.
There is currently no uniform national system to provide patient access to these dressings and many public hospitals are unwilling to provide them. Many families are forced to pay for dressings themselves or seek financial support from charitable organisations like DebRA to help fund ongoing access to dressings. There have even been situations where one patient received the dressings and another did not, within the same hospital. Therefore it is currently a matter of geography and/or good luck, rather than good medicine, which determines whether an EB patient has access to the most appropriate care.

DebRA's Proposal for a National Dressing Supply Scheme for EB

To address the inadequacies and inequities of the current system, DebRA proposes that a national scheme is set-up, with an indexed budget of $5 million in year 1, whereby dressings are made available to eligible patients from a designated scheme. DebRA is willing to coordinate and assist with the administration of the scheme.

This would also bring Australia into line with countries such as New Zealand, United Kingdom and other developed countries which have a long-established National scheme.

Benefits of the Proposed National Scheme

This scheme will provide an equitable framework to make available dressings regarded as best practice for patients with EB. It will improve the **quality of life** for individuals and families through:

- Improved rates of healing
- Reduced pain during dressing changes
- Reduction in use of other medications
- Greater patient compliance
- Fewer hospital admissions
- Cost savings through economy of scale
- Greater economic productivity through increased school attendance & work participation
- Reduce the financial burden on families already struggling with the added cost of caring for a person with a major medical condition or disability
- A reliable system that is not constantly at the risk of review, change or termination
- Improved social and community involvement

Debra receives no direct income from the government so every single dollar has to be raised by DebRA itself, through corporate support, grants or individual donors. This is insufficient to meet the dressing needs of Australians with EB. A national dressing supply scheme, would allow DebRA to redirect its financial support into other major areas of need, such as education, respite, family support and research.

Introduction

The purpose of this submission is to bring to the attention of the Federal Government the need for a Nationally funded dressing supply scheme to assist individuals with the rare genetic condition of Epidermolysis Bullosa.

This submission will explain what is known about Epidermolysis Bullosa, how people are affected, quality of life impacts for individuals and families, as well as the benefits for a national dressing supply scheme.

We will also detail how individuals are currently supported with their dressing and treatment requirements as well as the role of medical professionals in a multi disciplinary team.

Best practice dressing supply schemes are available in other developed countries as a model. The current role of DebRA Australia and its member State branches in supporting individuals, families and professionals living and working with Epidermolysis Bullosa will also be outlined.

Outline of a proposed costing and model with supporting information.

Acknowledgements

DebRA Australia Inc
DebRA A Queensland
DebRA South Australia
DebRA A New South Wales
DebRA Victoria
DebRA New Zealand

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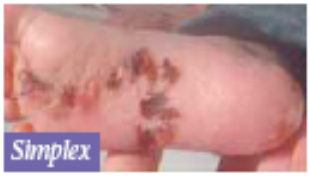
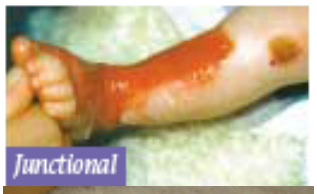
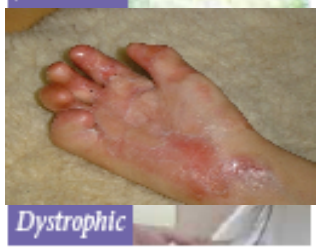
What is Epidermolysis Bullosa (EB) ?







Epidermolysis Bullosa (EB) is a rare genetic skin disorder which is characterised by skin fragility with blister formation occurring spontaneously or following minor trauma. This sometimes involves the mucous membranes. EB can be broadly divided into three major categories that are recognized accordingly: **Simplex**, where cell lysis occurs in the epidermis; **Junctional** where the separation occurs in the dermal-epidermal junction, and **Dystrophic**, in which the plane of the cleavage is below the basement membrane in the dermis. These categories can be further sub-typed based on the inheritance and clinical features. You can not change from one type of EB to another.

EB ranges from "mild" to severe and can require major adjustments in to the lifestyle of both the EB patient and his or her family. In severe EB, blisters are not confined to the outer skin. They may develop on the soft tissues (mucous membranes) inside the body such as the linings of the mouth, esophagus, stomach, intestines, lungs, airway, eyes and bladder. The extent of tissue involvement experienced by an individual is usually determined by the severity of the condition and the subtype present.

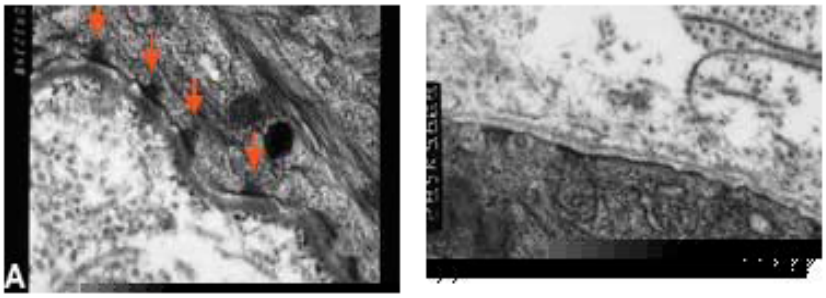
What Causes EB?

A genetic change or mutation in one of the genes that code for the proteins that "glue" the skin together. The particular protein affected is then reduced or missing in a specific layer of the skin, causing areas of structural weakness. As a result, the fragile skin is vulnerable to damage from mild friction, causing the blisters, which are the characteristic feature of EB. Sometimes these same proteins are also important for "glueing" internal tissues together, and hence in these cases, internal blistering and damage can result as well.

Diagnosis of EB

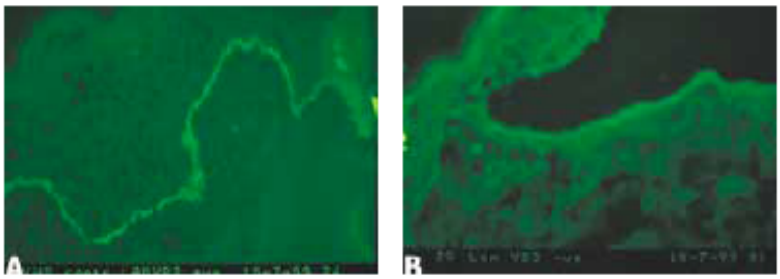
Diagnosis of EB is first dependant on the patient's clinical presentation and family history.

Information from electron microscopy and immunofluoresence mapping from a skin biopsy is required to make the diagnosis. Electron microscopy involves the use of a highly powered microscope that evaluates the skin sample by looking at the level of skin separation present and also noting the number and appearance of specific structures, such as anchoring fibrils or hemidesmosomes, located within the skin. This will assist in diagnosing the correct subtype of EB.



Electron micrographs of the dermal-epidermal junction of skin samples obtained by a skin biopsy and found to be normal (A) and affected with JEB (B). The hemidesmosomes (arrows) are formed in normal skin and absent in the affected skin. Source: Associate Prof. Dédée F. Murrell, Head of Dermatology St George Hospital University of NSW, Sydney.

Immunofluorescence mapping involves using a fresh skin sample and a panel of antibodies tagged with a fluorescent marker to detect the presence, or degree of binding, of a panel of antibodies to the skin to proteins affected in EB. This may provide an indication as to specific protein involved in the structural weakness of the skin.



Immunofluorescance mapping of normal skin (A) and affected (B). Source: Associate Prof. Dédée F. Murrell, Head of Dermatology St George Hospital University of NSW, Sydney.

Waiting for these results can be difficult, so it is helpful to locate measures of support. Support systems such as family, other parents with children affected by EB and organizations such as DebRA Australia can help during this trying time by providing support and information to families of affected individuals.

How is EB Inherited?

Autosomal Dominant Inheritance: An autosomal dominant disorder is one in which one gene for the condition expresses itself in an individual. A parent with an autosomal dominant form of EB has a 50:50 chance with each pregnancy of transmitting the abnormal gene. The chance is the same whether the child is a boy or a girl, and birth order does not make a difference. A child who does not inherit the gene for EB from an affected parent will not have the condition and cannot pass it on.

Autosomal Recessive Inheritance: An autosomal recessive disorder is one in which a recessive (unexpressed) gene for the disorder is passed from each parent and the two genes are paired together, causing the disorder to be expressed in the child. If a person has one recessive EB gene paired with a normal gene, the person is "a carrier", but does not have the disorder. If parents are each carriers of an autosomal recessive gene, there is a 25 percent chance with each pregnancy that the children will have the disorder. Again, the sex of the child and the birth order do not matter. An individual with a recessive form of EB will be at risk of having an affected child only if he or she has a child with a carrier or another person with recessive EB.

Once the genetic mutation is identified in a family, prenatal diagnosis of future pregnancies maybe possible.

Who gets EB?

It is estimated that there are around 1,000 people in Australia who have some form of EB and over 500,000 worldwide. It occurs in all racial and ethnic groups and affects males and females equally. At its worst EB can be fatal, but even in its mildest form it causes a life of pain and physical challenges. Some people spend hours every day dealing with blisters, wounds and destructive scarring.

The Impacts of EB

Children and adults with EB are commonly known as “cotton wool children” or “butterfly children”. Imagine your skin is as fragile as a butterfly’s wings, with painful blisters and wounds that form at the slightest touch. This requires constant care and attention that covers large areas of the body. EB does not just impact the Quality of Life of the individual but also the entire family. The severity of EB can range from mild re occurring blisters that require little support to all the impacts detailed below.

In the more severe types of EB, for those fortunate to make it to early adult hood, they have the added risk of skin cancers that can also cruelly cut their life short. Not to mention the years of wounds and scarring which can rob them of the use of their hands and feet leading to permanent disabilities and reduced mobility. Some people with EB require hand operations for continued limited use. Many other medical interventions such as feeding tubes to help maintain nutrition and to deliver medication. Problems with the esophagus may require regular hospital admission for dilatation. In a rare form of EB people may also be required to have a tracheostomy to breathe through because EB has affected their airway. Corneal abrasions can lead to people having to sit in the dark until the damage has passed, this can take several days. Constipation and bladder involvement are further complications that must be managed All this requires a team of medical specialists to supervise the care of these individuals.

Some people spend hours every day dealing with blisters, wounds and destructive scarring. Bathing and dressing changes can take 3– 4 hours and may at times involve pain management medication. The dressings have to be non-stick so that the new skin does not get torn off during dressing changes, and this type of dressing is expensive. If the dressings are not changed frequently, every couple of days, infections ensue that may require oral antibiotics or admission to hospital for intravenous antibiotics, further adding to the cost.

In Australia, in most cases, the only carers available are the parents. Children and adults with EB require 24 / 7 care and support, it mainly falls on the parents to be the carers on an ongoing basis. Parents who are the main carer are not able to work due to the intensive care routine, regular doctors and specialists visits and also being on call for mishaps and general support. After all this is taken into account, many families still need to buy top-of-the-line silicone dressings that are expensive and not generally available through the various health systems within Australia.

The families impacts include reduced income opportunities due to the need of a parent being required to be a carer. This would also include arranging appointments with many specialists, hospital admissions, training other support people, researching information sourcing equipment and dressings. This impacts on time dedicated to other siblings not to mention the financial, emotional, social and community burden for all the family.

How is EB Treated?

Because EB involves many parts of the body, parents and health professionals must take a multi disciplinary approach to the treatment of an EB patient. Total patient care often must be provided, particularly for young children. The severe forms of EB require meticulous nursing care in regards to dressings changes and wound care. Much of this care is often provided by the parents; however, the education of all people who have contact with the patient is essential, including all health professionals, as well as teachers, relatives and others.

Although there is no cure for EB, and gene therapy is still in the realm of experimental medicine, treatment of EB is directed towards the symptoms. Many persons with milder forms have minimal symptoms and may require less or no treatment. Management of the disorder is usually under the care of a Dermatologist as well as many other specialists as required. Focus should be on prevention of infection, protection of the skin against trauma, attention to nutritional deficiencies and dietary complications, minimization of deformities and contractures, and the need for support to the entire family.

The Role of the Multi Disciplinary Team

History of the EB Clinic

The Multidisciplinary EB Team was started with Dr Mark Eisenberg (GP and parent of a child with EB), Dr Brien Walder (dermatologist), Dr Kieran Moran (paediatrician) as a one hour monthly clinic at Sydney Children's Hospital in the 1990s. In 1996, Prof Dedee Murrell was invited to join the team as she had expertise running the National EB Registry facility at Rockefeller University, New York, and had recently moved to Sydney. She expanded the clinic to a full afternoon on the last Friday of each month and recruited the Head of Clinical Genetics, Dr Anne Turner, to the team, along with bringing her private practice nurse, and the hospital provided a physio, OT, and social worker. In 2000, Dr Walder passed away and his position was advertised, and A/Prof Orli Wargon, who had 17 years experience as a dermatologist at Children's Westmead Hospital, was selected to replace him. In 2005, funding was obtained for an EB clinical nurse consultant, part –time, to assist with the clinic (Louise Stevens). The supply of the non-adherent dressings for the EB patients has long been a source of difficulty. Initially it was via PADP in NSW but then dressings were deleted from their mandate, with no additional budget for that going to hospitals. Prof Murrell made an appeal regarding this and NSW donated a one-off amount of $100,000 to Sydney Children's for the dressings, which lasted 2 years.

Role of the Multi Disciplinary Team

Appropriate management of infants suffering from epidermolysis bullosa should begin as soon as possible after the birth of the affected infant and continue with transition into adult life. Specialist advice in handling, dressings, feeding and adaptation of equipment needs to be followed up with long term care provided by a multi-disciplinary healthcare team.

Contributions are made initially by the dermatologists regarding diagnosis confirmation, the paediatrician regarding the general health of the infant and by a specialist nurse regarding appropriate dressings. On going care involves plastic surgeons, gastroenterologists, endocrinologists, ophthalmologists, cardiologists, dental experts, dieticians, physiotherapists, occupation therapists and social workers. A specialised multidisciplinary team with expertise as to the specialised needs of EB patients is the most appropriate method of co-ordinating the many consultations needed.

Currently there are multi disciplinary EB Clinics held at the Sydney Children's Hospital Randwick and the Royal Children's Hospital in Melbourne. The adult patients with EB in NSW have been attending St George Hospital, Sydney. There is also an EB Clinic that is held in Queensland on an as needed basis as well as specialists in other States that are involved in specific EB care.

Who is DebRA Australia?

DebRA Australia (National Dystrophic Epidermolysis Bullosa Research Association of Australia) Incorporated.

For many years individuals and small DebRA State groups have been the primary source of support for people with EB. A collaborative effort has been made by the 4 State groups, Queensland, New South Wales, Victoria and South Australia and as a result DebRA Australia Inc was officially founded in February 2005.

In brief DebRA Australia is:

- Funded solely by voluntary donations.

No government funding is provided to any of the DebRA groups, including the national body. It is only through community awareness and support, as well as funding from corporate Australia and other charitable organisations, that DebRA can make a difference.

- Investigating and implementing ways to help people with EB and their families in their daily lives.

DebRA Australia has developed a new national newsletter that is produced twice a year along with a website www.debra.org.au This has lead to greater communication and network of support between member families nationally. In June 2007 the first professional conference on EB was held at St George Hospital in Sydney followed by the Second National Family conference held at Penrith in Sydney. The conferences included International Guest speakers from Great Ormond St Hospital in London and activities for children with EB and their siblings in a supportive environment.

During 2006 DebRA Australia funded $50,000 nationally in direct support to members for dressings and other vital equipment.

- Raise awareness and education of EB for the general public.

Our major activity for education and awareness is EB Butterfly Day which is held in November each year (Nov 23rd 2007). Along with presentations to schools, business and other community organisations.

- Provide support and information for health professionals working with EB.

The development of literature that includes “A Clinicians Overview” brochure and booklet “An Outline for Professionals.” The creation of a professionals registry in Australia who have an interest in EB. Along with funding for professional development.

- Raising funds for vital research into EB.

Refer to section Attachment 2 Research and Australasian EB Research Registry

Current Process for Access to Dressings

There is currently no national scheme to access dressings in Australia for people with EB, unlike New Zealand. Australian families have stated that they have to beg for dressings via the local hospital and if successful often have to settle for less suitable, cheaper products or even go without. For those who do have access to dressings, there are different processes for each state, within individual states themselves and even within the same hospital, which also leads to confusion. This makes it very difficult to get a clear understanding of how many families are accessing dressing supplies.

Some families rely on informal arrangements with their local health service that constantly change and are always under review to access some of their dressing needs. Others can not access top of the line dressings at all via their local or State health services and some rely on the support of community organisations such as DebRA, some families also rely on other organisations such as the Lions Clubs and Variety the Children's Charity to assist them cover the cost of these dressings.

For those who do have good access to dressing via a local hospital system there can be reluctance to discuss in detail how this works or give specifics for fear of losing what they have worked for so many years to have put in place.

A national dressing survey in 2005 conducted by DebRA found:

- *Only 35% of DebRA members who completed the survey are able to access dressings via the public system.*
- *40% of respondents were not aware there were other dressings available that were more suitable for EB dressings.*
- ***45% of members have problems accessing dressings because of expense or availability, of these dressings not accessible, 85% of them are silicone dressings now used for best practice techniques.***
- *In a perfect world the majority of members would like to access dressings via the public system and have them delivered, as occurs in the Netherlands for EB patients.*

Of those 35% of members who can access dressings from the public system:

- *65% of members get the types of dressings they require with the remaining 35% of members sometimes or always having to take alternative dressings.*
- *65% of members find the process confusing on a regular or sometimes basis.*
- *50% of members are always or sometimes made to feel guilty that they access dressings on an ongoing basis via the public system.*
- *71% of members have found that they regularly or sometimes experience that their dressings are on back order, causing them to be short of dressings.*

The health system at all levels currently recognize dressings as an ancillary item, therefore not currently funded like other medications. Government support such as the Medical Expense Offset scheme fall well short of meeting this need. This requires that a person with EB pay for their dressings which can be as high as $6,000 per month for the most severely effected. They are then able to claim a tax deduction at the end of the financial year which is only a partial rebate.

Current Process for Access to Dressings (continued)

Carer Allowance only covers some of the most basic of needs for individuals. With Carer Payment Child, although many families qualify medically, we have not been able to find a family currently receiving this benefit. We welcome the current review underway for this benefit and look forward to reviewing the changes when this report is available.

As previously stated the effects of EB can be mild to severe and the effects are very significantly from person to person, with some people requiring little support to others who require significant support in every aspect of their lives.

The Break up of Numbers

Nationally DebRA has 105 members registered who have some type of EB. The Australasian EB Registry has 350 people entered with 242 people listed with confirmed diagnosis of EB types. This is made up of people from Australia and New Zealand living and who have passed away with a median age of 17 years for all people currently registered. This database is administered by Professor Dedee Murrell head of Dermatology St George Hospital, Sydney.

Current Costing Estimates

Please find below a costing estimate table based on figures prepared for a dressing trial in Victoria early 2007. The costs are based on using Mepilex a dressing by Molnlycke Health Care and is a top of the line silicone based dressing that represents current best practice.
N.B. dressings costs based on Hepburn Health Service Price List

EB Type	Mepilex – use per month	Cost per box	Cost per month
Severe			
Patient 1	15 * 15cm – 52 boxes 20 * 10cm – 13 boxes 15 * 20cm – 1 box	$59 $49 $125	$3,068.00 $637.00 $125.00 Total: $3,830.00
Patient 2	20 * 20cm – 140 boxes	$91.00	$12,740.00
Patient 3	20 * 20cm – 140 boxes	$91.00	$12,740.00
Moderate			
Patient 4	20 * 20cm – 3 boxes	$91.00	$273.00
Patient 5	20 * 20cm – 12 boxes 15 * 15cm – 4 boxes 7.5 * 7.5cm – 4 boxes	$91.00 $48.50 $16.00	$1,092.00 $194.00 $64.00 Total: $1,350.00
Mild			
Patient 6	20 * 20cm – 1 box	$91.00	$91.00
Patient 7	20 * 20 cm – 1box	$91.00	$91.00
Patient 8	20 * 20cm – 1 box	$91.00	$91.00

Current Expenditure on Dressings

Sydney Children's Hospital currently supports 13 children via the EB clinic and Helpdesk with dressing requirements. Families are required to pay a co-payment of $100 per month to access this support. The average amount for these 13 patients is $1,024 per month plus the $100 co-payment. There are 3 patients averaging $3,176 per month in dressing requirements. There is no guarantee for ongoing future funding of this program.

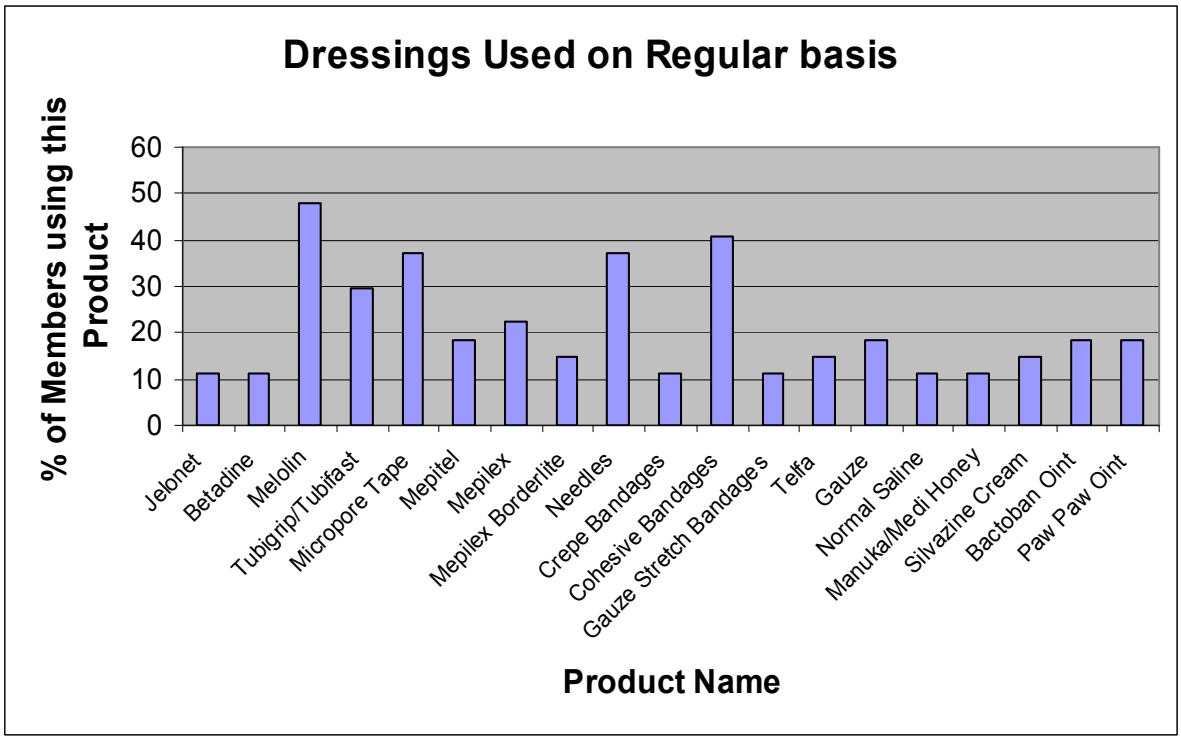
The above costing are based on children with EB and this should be taken into account when comparing to the large dressing needs of adults with EB.

DebRA Australia through its National and State groups spent the following in support of members dressing requirements alone during 2006-2007 financial year.

DebRA Australia and State group support of Dressings for EB Families		
Group	EB People Supported	Amount
National	14	$13,673
DebRA NSW	11	$17,036
DebRA Vic	10	$7,000
DebRA QLD	7	$5,200
DebRA SA	2	$1,500
DebRA Total Cost	**30**	**$44,409**

This is in addition to dressings that may have been supplied through local hospitals, other charities or funded directly by families themselves.

Dressings Currently in Use



Please note this data was collected in 2005, since then some members have started using the silicone dressings more intensively. (Eg. Mepilex and Mepitel)

Current New Zealand Dressing Supply Model
"Together we can make it happen !"

by Anna Kemble Welch, secretary/co-ordinator, DEBRA NZ
August 2003

All around the world things are moving to help find the best ways of meeting the challenges created by having EB, to find and be able to access the best treatments and to eventually find a cure. Here in New Zealand things are not perfect by a long way but they are far better than if you live almost anywhere else in the world. Here we at least have a philosophy of a free public health system.

About 10 years ago we managed to gain support from the government to cover a lot of the cost of dressings and wound care treatments for EB. People were previously having to buy these for themselves or rely on the cooperation and good will of their local hospital. After years of pressure from the NZ Society of Dermatologists and DEBRA members, a system was set up to allow dressings to be available for people with EB like prescription medicines are for other health problems.

The EB Bandage Supply System
The rarity of severe EB helped in the negotiations. A capped annual amount was allocated for bandage supplies for all NZ'ers with EB. Auckland Dermatologist Dr Nick Birchall has administered this since the government support was put in place . All those with EB who needed dressings were asked to provide a list of what and how much they used annually. They were then allocated an annual budget that was a slice of the pie to match their needs. Their local hospital or bandage supplier was now reimbursed for the supply of dressings on their list, up to the amount allocated to them each year. This has reduced the financial burden on EB families of buying essential supplies that the rest of the population doesn't have to consider.

At the time, this was a wonderful advance on begging local hospitals for access to good dressings or settling for less suitable, cheaper products, or going without. It meant some could now use sterile products previously unavailable to them and consequently their infections and pain have been reduced and their health, comfort and quality of life has greatly improved.

For a few with severe RDEB who needed the most dressings, the allocated amount has not been enough to cover all their costs, particularly the young children who have now grown and need more and bigger dressings. Their local hospitals have still needed to provide a top up, sometimes negotiating a separate "high use contract" with the Health Funding Authority for that individual's care costs. Unfortunately there were inequities in different parts of the country with some Health Districts being more supportive than others when the allocated budget was exceeded.

Updating the System
The system that has been in place for the last decade needs some minor tweaking. It hasn't included a mechanism to apply for more money to cover the needs of the new babies, growing children with EB, and people's changed needs.

The Minister of Health, Annette King, recently agreed that all NZ'ers with EB should have access to the most effective dressings, and it should be available nationally, not dependant on which area you live in. The funding is to be increased to cover the cost of dressings for everyone with EB each year, and will also allow for additional funding for new babies born within the calendar year so their needs can be immediately covered by the system.

Proposed Dressing supply Scheme

Assumptions

- There is currently no known data for exact numbers of people with EB in Australia.
- It is estimated that around 1,000 people in Australia have some form of EB based on International population estimates of 1 person per 20,000 to 30,000 people with some form of EB.
- Of this number there is around 100 people with EB that would require the most dressing support.
- This number is supported by the Australasian EB registry administered by Professor Dedee Murrell head of Dermatology St George Hospital, Sydney. Along with information from EB Clinics and Dermatology specialists.
- This registry currently has around 350 people entered with diagnosis available for some 216 people. This is made up of people from Australia and New Zealand living and passed with the average age of 24 years for all people in the database.
- DebRA Australia and its member States currently has 105 members listed with EB.

Costing

- Based on the previous information supplied in this submission we estimate that a National EB Dressing Supply scheme would cost approximately $5 million dollars for the first year and would support **ALL** people with EB in Australia.

Precedents of Other Schemes or Items Funded by the Federal Government

- Glucose Blood Indicator Strips for diabetics are listed as a "Device" under the Therapeutic Goods Administration (TGA). This device is included on the Pharmaceutical Benefits Scheme (PBS). Total cost to supply these consumable devices were over 24 million dollars in 2005/2006 for the 12 month period.
- Listed on the Repatriation Pharmaceutical Benefits Scheme (RPBS) are over 171 different types of dressings, bandages and tapes that are readily available to war veterans.
- Enteral Feeding Scheme
- Incontinence Aid Programs
- Stoma Therapy Appliance Scheme
- Factor 8 and 9 protein therapies are available free of charge as an "orphan drug" to people with haemophilia via a jointly funded state and federal government scheme under the National Blood Agreement. Devices such as needles, syringes and swabs are also included in the protein packs which are at no cost to the patient.

Proposed Dressing Supply Scheme

Medical and Quality of Life Improvements

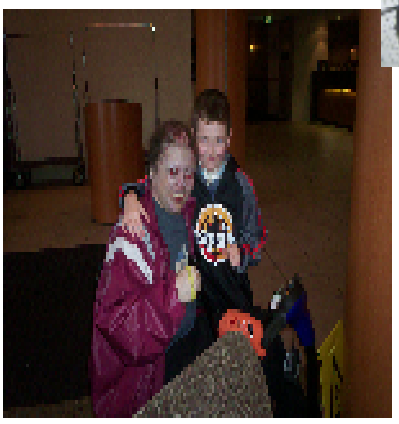
- Using best practice dressings would improve rates of healing and increase patient compliance.
- Reduce the risk of infection and reliance on other medications such as antibiotics and pain management drugs.
- Reduce the number and length of stay for hospital admissions.
- Improved quality of life, physical and mental wellbeing for people with EB and their families as well as reducing the financial burden.
- Reduce the amount of time patients and carers spend having to try and source adequate appropriate dressings.
- Increased attendance and participation in school, work and the wider community.

A Final Word about the Forgotten Patients with "Orphan Diseases"

- The "butterfly children" in the UK were not forgotten by the Patron of DebRA UK, the late Princess of Wales, Princess Diana.
- In wheelchairs, directly behind the cortege, the 4 princes and her brother were the patients with EB in their wheelchairs
- In the UK, patients with EB are supplied with all necessary special dressings and bandages by the cash-strapped National Health Service, and DebRA UK, thanks to the profile afforded by Princess Diana, has raised thousands of pounds for research into the causes and better, more advanced treatments for EB, rather than having to fund basic medical care, such as dressings and bandages.

THANK YOU

- DebRA Australia, their families and carers would like to thank YOU very much for taking the time to read this application and for your SUPPORT.



"Photos from the National EB Conference 2007"

Attachment 1 Proposed Dressing Supply Scheme

Outline for National EB Dressing Supply Scheme

Individuals and families living with EB would welcome the opportunity to implement an arrangement with the Federal Government similar to the model available in other countries. Individuals would need to meet the following requirements to be eligible for the scheme:

- Individuals would need to have a diagnosis of EB supported by a Dermatologist or a multidisciplinary team at an EB Clinic in order to access the scheme.

- A multidisciplinary team of EB specialists are available to see families with new babies born with EB, to provide support and adequate resources required in this very emotional and crucial time.

- Dressing requirements are reviewed on an annual basis to meet changes in age and care requirements. This would have to be approved by a Dermatologist or multidisciplinary EB Clinic.

- Dressings would include all types currently being used by people with EB as well as top of the line best practice dressings currently available in Australia.

- All dressings required are delivered via the local public hospital system on a regular basis to people with EB.

Cost Benefits and Offsets

- This scheme could replace current local and State arrangements that are not uniform or provide access to all people with EB. This scheme would provide fair and equitable access to dressings regardless of where people live.

- Consistent scheme across all of Australia would also deliver cost savings due to economy of scale.

- Reduce hospital admissions and length of stay.

- Reduced need for pain management and other medications.

- Allow DebRA Australia and member States to redirect financial support into research and other support for families.

Options for consideration

Option A
Required dressings to be available on the Pharmaceutical Benefit Scheme (PBS). However, this would require major changes in Government Policy.

Option B
Federal Government fund DebRA Australia to implement and run a national EB dressing scheme on an ongoing basis.

Option C
A customised national EB dressing scheme be implemented and co jointly run by the department of Health & Ageing and DebRA Australia. The National EB dressing scheme would be a customised efficient model to maximise access for dressings for all eligible people in Australia with EB.

Outline for Option C

Eligibility Criteria
People with EB must adhere to the two following criteria:

a). Patient must be registered on the Australasian EB Registry administered by Department of Dermatology, St George Clinical School, UNSW Faculty of Medicine.
b). Attend a consultation with recognized Dermatologist or EB clinic on an annual basis to review requirements of dressings (or more often if required)

Human Resources Required

Position	Full Time Equivalent (FTE)
EB Nurses	1.5
Secretariat – Dressing Coordinator	1
Australasian EB Registry Database Updating	.2
Total	2.7 FTE

Dressing Coordinator

The dressing coordinator position would be funded under the dressing scheme as a full time secretariat position. An online purchasing program could be purchased to improve access to the system and to maximise efficiencies. Main tasks would include:

- Maintain online purchasing program
- Order direct from supply companies
- Place 3 monthly orders for patients or more often if required
- Produce relevant reports and financials for DebRA and Government bodies
- Participate in negotiations for dressing supplies with companies

EB Nurses

EB Nurses would be employed via the Public Health system and funded by the National EB Dressing scheme. They would be responsible for the following tasks:

- Liaise with Medical Professionals, patients and organise annual reviews or more often if required.
- Review dressings required in conjunction with the medical team and patient.
- Update requirements with the Dressing Coordinator
- Establish new patients into the dressing system
- Forward on relevant data for updating of the Australasian EB Registry
- Dressing education for patient's
- Trial dressings
- Be a member of the clinical advisory panel and participate in meetings

1.5 full time equivalent (FTE) EB Nurses would be linked to the National EB dressing scheme via the clinics held in relevant States. As there are no clinics in some States, patients in those areas can be linked to the States, patients in those areas can be linked to the States with clinics to ensure there is national coverage as displayed below.

Australasian EB Registry Administration

States with EB Clinics	No Clinics available
Queensland	Northern Territory
New South Wales	Australian Capital Territory
Victoria	Tasmania
South Australia	Western Australia

This database is maintained by the Dept of Dermatology at St George Hospital, Kogarah Sydney. Funding from the scheme for .2 FTE would be required to update and maintain patient information.

EB Medical advisory board

This would be a body of professionals involved in the care and treatment of people. They would review the Dressing supply list on an annual basis and make recommendations for any changes. Trials of new dressings could be coordinated through this group to give a better cost benefit analysis and quality of life score to any new treatments or dressings. This would supply access to a larger pool of patients to give quality outcomes.

Currently it is difficult to perform effective cost benefit analysis due to the significant individual differences for people with EB and the small numbers involved.

DebRA Australia
EB Priority Dressing List 2008

The following dressing list has been identified as the main groups that include primary, hydrofiber, silver, foam, retention and hydrogels. They provide the basis of day to day dressings. Sterile needles have also been included as they are a vital part of wound management. What is not included is topical antiseptic, skin moisturisers, honey and exudate management. There are some excellent dressings that come under the later which are currently being TGA approved or are only at this stage available overseas.

The required dressings can be accessed from the following companies :

Molnlycke Health Care Pty Ltd
Convatec
Novartis
Hartmann
Smith & Nephew
Dermatech
Terumo

Products from various suppliers may also be sourced from companies already mentioned.

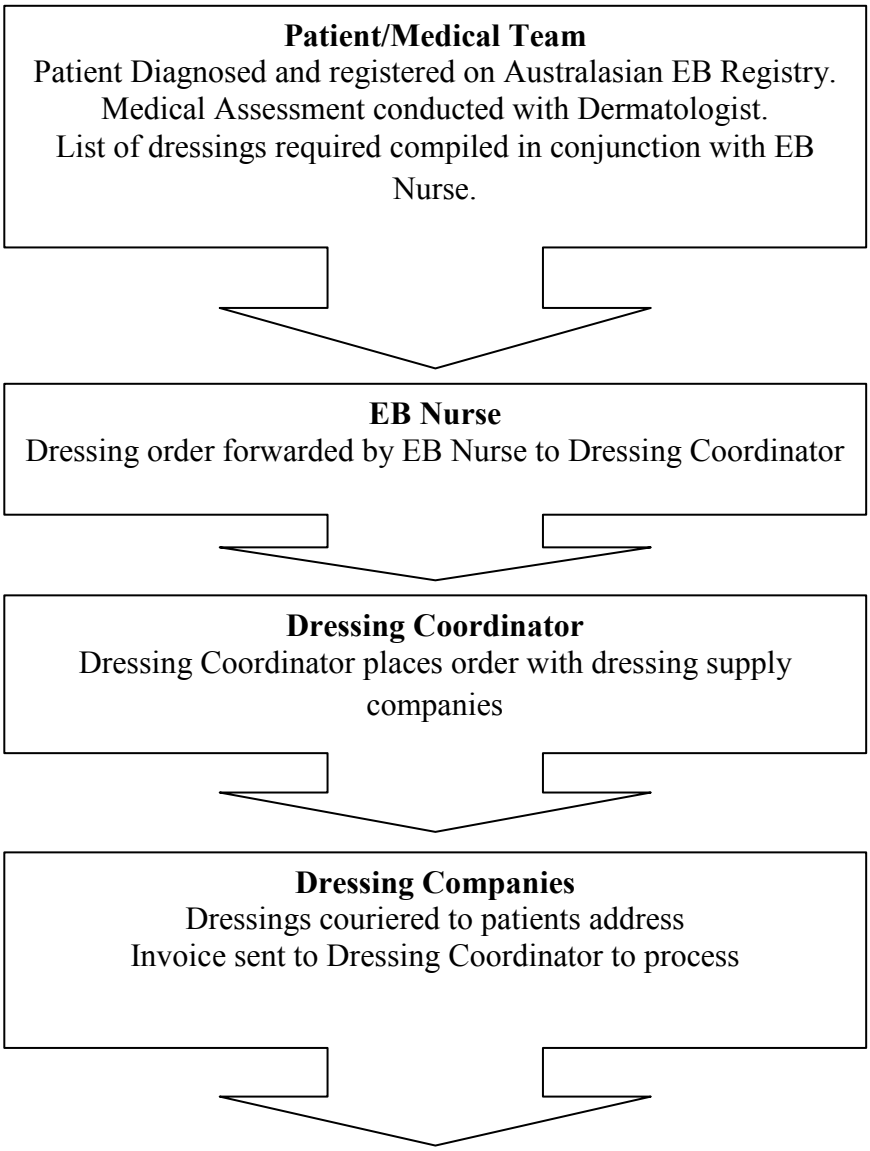
Estimated total cost for the scheme is 5 million per year and funding should be linked to CPI.

Dressing Name	Description	Manufacturer / Supplier
	PRIMARY DRESSINGS	
Aquacel AG Hydrofiber	Silver impregnated antimicrobial soft sterile, non woven dressing	Convatec
Aquacel Hydrofiber Dressing	Soft sterile absorbent non woven pad or ribbon dressing composed of hydrocolloid fibres – exudates management dressing.	Convatec
Bactigra	A cotton leno-weave fabric, impregnated with Soft Paraffin BP, containing Chlorhexidine Acetate BP	Smith & Nephew
Exudry	Absorbent antis sheer dressing	Smith & Nephew
Hydrogel	Have the ability to provide additional moisture to the wound which both rehydrates necrotic and sloughy tissue to effect debridement and to absorb certain amounts of wound fluid	Smith & Nephew
Intrasite Comformable	Gel impregnated wound contact layer dressing	Smith & Nephew
Jelonet	Soft paraffin dressing not medicated, contact layer dressing	Smith & Nephew
Lyafoam	Dual Layered Polyurethane foam dressing	Various Suppliers
Mepilex	Absorbent soft silicone dressing	Molnlycke Health Care Pty Ltd
**Mepilex AG *Presently with TGA and should be available soon*	Mepilex with Silver dressing More cost effective than Mepilex + Silver dressing	Molnlycke Health Care Pty Ltd
Mepilex Border	Self-adherent soft silicone dressing	Molnlycke Health Care Pty Ltd
Mepilex Border Lite	Thin self-adherent soft silicone dressing	Molnlycke Health Care Pty Ltd
Mepilex Heel	Absorbent soft silicone dressing for the heel	Molnlycke Health Care Pty Ltd
Mepilex Lite	Thin absorbent soft silicone dressing	Molnlycke Health Care Pty Ltd

Dressing Name	Description	Manufacturer / Supplier
Mepilex Transfer	Soft thin Silicone exudate transfer dressing	Molnlycke Health Care Pty Ltd
Mepitel	Soft silicone wound contact layer	Molnlycke Health Care Pty Ltd
Urgotul	Lipido-Colloid dressing	Pharamalink
Urgocell	Adhesive and noN adhesive	Pharamalink
Vaseline Gauze	Contact Layer dressing	Various Suppliers
	SECONDARY DRESSINGS	
Bandages – crepe	Protective dressing	Various suppliers
Combine	Aa highly absorbent thick layer of fleece enclosed in a soft and conformable non-woven fabric.	Smith & Nephew
Handyband	Soft, elastic bandage for dressing retention on high-body-movement areas such as joints. It is highly durable and washable	Smith & Nephew
Gauze Squares		Various Suppliers
Hypafix	Self-adhesive non woven fabric for dressing retention	Smith & Nephew
Hospilux	Cohesive elastic conforming bandage	Hartmann
Setopress	High Compression Bandage	Various Suppliers
Tubifast Bandages	lightweight tubular bandage	Various suppliers
Tubinet	Tubular retention bandage	Various Suppliers
Velband	Absorbant padding – hypoallergenic bandage	Smith & Nephew
Webril	Cotton undercast padding	Various suppliers
	AUXILARY ITEMS	
Non alcoholic wipes	Cleaning	Various Suplliers
Mepitac	Soft silicone tape	Molnlycke Health Care Pty Ltd
Needles 21 gauge	Sterile non toxic needle	Terumo
Welland adhesive remover (Apeel)	For gently removing adhesive dressings	Omnigon

Dressing Ordering Process

- Dressing requirements would be forwarded from the EB Nurse to the Dressing Coordinator on an annual basis or as required.
- Dressings would be purchased for each patient direct from the supplier and freighted to the patient's doorstep to streamline the process and improve turn around time.
- Dressing deliveries would be based on a 3 monthly basis per person.



Current Australian Research

Professor Dedee F. Murrell MA(Cambridge) BMBCh(Oxford) FAAD(USA) MD(UNSW)
Chair, Department of Dermatology, St George Clinical School, UNSW Faculty of Medicine.

Professor Murrell is one of the world's foremost authorities on EB.

Current EB Research Projects:
Clinical

-Australasian EB Registry
- Epidemiology of EB in Australasia
- Quality of Life in EB
- Cell therapy for dystrophic EB pilot study

Basic

Genotype to phenotype correlations in EB simplex (keratins 5 & 14 genes)
Genotype to phenotype correlations in dystrophic EB (collagen VII gene)
Genotype to phenotype correlations in Junctional EB (laminin 5 and integrin alpha 6 and beta 4 genes)
Immunofluorescence mapping in EB

Collaborative studies

Squamous cell carcinoma pathogenesis in RDEB –with St Thomas' London and Stanford University.
Genotype-phenotype correlation studies with Profs Jouni Uitto, Philadelphia, USA, John McGrath, London, UK & Hiroshi Shimizu, Sapporo, Japan.

Professor Murrell lectures on EB at local, national and international meetings regularly, including the World Congress of Dermatology, the American Academy of Dermatology, the European Academy of Dermatovenereology, the ASEAN regional conference, Australasian College of Dermatologists and New Zealand Dermatological Society. She has visited and given lectures to a number of the EB centres around Australia and abroad, including GOSH in London, OLMH in Dublin, when she was invited to the EB 2006 meeting for EB researchers in Dublin, and the wonderful EB Haus in Salzburg, and the EB centre in Groningen, Netherlands. She has many colleagues and friends in the worldwide EB care and research community.

In addition to attending the multidisciplinary EB clinic at Sydney Children's Hospital as a consultant dermatologist for (EB), Prof Murrell holds a dedicated bullous disease clinic every fortnight at her rooms in Kogarah where she sees adult patients with EB and at the Ambulatory Care unit at St George Hospital. Professor Murrell also hosted the first EB professionals conference in Australia at St George in June 2007. Prof Murrell also established a national diagnostic laboratory facility for EB, based at St George Hospital. In 2000.

For full details on previously completed projects, a selection of publications, awards and media coverage and other items related to her work on EB this can be found at the following website: www.debra.org.au select Research & Professionals. Her research into EB is partly funded by DebRA Australia, its member states as well as DebRA New Zealand.

The long term hope for an effective treatment or cure lies in Gene and Protein/Cell Therapy. A cell therapy trial is underway by Prof Murrell in collaboration with Australian of the Year 2005, Prof Fiona Wood.

**** Australasian Epidermolysis Bullosa Registry (statistical information)**

This registry is administered and maintained by the Dept of Dermatology, St George Hospital, Kogarah in Sydney. Overseen by the head of the Dept Professor Dedee Murrell, MA (Cambridge), BMBCh (Oxford), FAAD (USA), MD (UNSW).

- The registry currently contains confirmed diagnoses for 242 individuals.
- 49% of these being female, and 51% being male. They have an age range of 0 to 99 years with a median age of 17 years. Ages were found to be similar in EB Simplex and Dystrophic EB, but considerably lower in Junctional EB.
- 37.5% of our RDEB-HS cohort (16) have developed squamous cell carcinomas, with a 5-year survival rate of 40%.
- 19 individuals in the registry have so far passed away.
- 10 of these being JEB-H infants. 90% of the Registry cohort of JEB-H infants died within their first year of life.