ISRCTN67757229 https://doi.org/10.1186/ISRCTN67757229

# Allogeneic fibroblast cell therapy for dystrophic epidermolysis bullosa

**Submission date**
09/08/2011

**Recruitment status**
No longer recruiting

**Registration date**
29/09/2011

**Overall study status**
Completed

**Last Edited**
24/08/2016

**Condition category**
Skin and Connective Tissue Diseases

[_] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

## Plain English summary of protocol

Background and study aims
This research study concerns individuals with the inherited blistering skin disease known as dystrophic epidermolysis bullosa (DEB). This a very severe condition in which skin injury leads to blisters and chronic wounds that remain inflamed and take longer to heal and sometimes never heal. The entire skin and internal organs can be affected. The increased skin fragility leads to scarring and an increased risk of skin cancer at a young age. Currently we have no effective treatments for DEB and we are unable to prevent the disease complications. Our aim is to show that skin cells called fibroblasts can improve wound healing in people with DEB. We know that these cells make collagen VII, the protein that is genetically abnormal in these people. We have shown in previous studies that skin fibroblasts from other healthy people are able to compensate for this and stimulate production of some of the collagen VII that is missing in your skin.

Who can participate?
Patients aged 16-70 diagnosed with dominant or recessive DEB.

What does the study involve?
If you take part, first of all, you will have several of your skin wounds assessed and photographed. You will have a physical examination of your skin and you will be asked to donate a small blood sample. The sample will be taken in the same way as any standard blood test. A urine test to exclude pregnancy will be needed for women. On your next visit, if your wounds are not showing signs of spontaneous healing, you will be invited to participate in the next part of the trial which involves injections into your skin. We would like to inject two preparations into your skin. One will be a solution containing the fibroblast cells, the other will be the solution but without the fibroblast cells. The solution lacking the fibroblasts is called a placebo. We feel it is important to compare the effects of fibroblasts and placebo on wound healing in your skin so that we can learn more about how your skin heals.
Up to 10 skin wounds may be treated and we plan to do this on one visit to the hospital. This may require up to 60 injections in your skin. This may sound like a lot of injections but they should not cause you much discomfort. Each injection may cause brief stinging for a few seconds, similar to having a blood test. The size of the needle used for injecting the fibroblast cells or placebo solution is in fact slightly thinner than most blood test needles. If you find the

injection process at all uncomfortable, we can interrupt the injections and numb the skin with a topical anaesthetic cream. After the injections you will be able to go home immediately.
The choice of either fibroblasts or placebo will be selected in a random manner. On average, half the wounds will be treated with cells and half with placebo, but you will not be told how each individual wound has been treated. This step will then allow us to follow up the healing process in a blinded manner. Only at the end of the trial you will learn how each wound was treated.
After the initial injection day, you will be asked to attend for six further clinic visits over the subsequent six months. On these visits, the wounds selected during your first visit will be photographed and their size measured. We will ask you to keep a wound diary and take weekly photos of the selected wounds with cameras provided by us. Training and technical support will also be provided. The measurements will be done with a computer software programme and this will be a painless procedure. We will also take a skin swab from the wounds to test for any infection; this is also a painless procedure. On two of these visits you will be asked to give two further small blood samples. We are also keen to assess the impact of the cell injections on your skin and we will ask you to complete a questionnaire about this.

What are the possible benefits and risks of participating?
It is likely that participation in this research project will not be of immediate clinical benefit to you. However, the results should be able to tell us whether skin fibroblasts have the potential to speed up wound healing and improve skin function as well as the quality of your life by reducing the wound pain. At the same time by following non-treated wounds with serial photographs we will be able to assess and document their healing process. The final results of the clinical trial will be discussed with you in detail by a member of the research team.
The injection of fibroblasts or inactive solution into the skin could cause pain for a few seconds. We plan to minimise the pain by anaesthetising the skin using a topical anaesthetic cream if you find the pain of the injections too uncomfortable. Several studies have shown that injected skin fibroblasts are well tolerated but there is the potential to cause minor redness and irritation that should resolve spontaneously over a few days. Venous sampling for blood tests could result in pain, bruising and/or infection at the injection site. Infection can be treated with a short course of oral antibiotics.

Where is the study run from?
It is being organized by Guys and St Thomas NHS Trust in London (UK).

When is the study starting and how long is it expected to run for?
The study began in January 2011 is open to participants until November 2011.

Who is funding the study?
The study is being funded by the Dystrophic Epidermolysis Bullosa Research Association (DEBRA) (UK), Technology and Strategy Board (UK) and Intercytex Ltd (UK) - investigational product manufacturer and supplier.

Who is the main contact?
Dr Gabriela Petrof
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# Contact information

**Type(s)**
Scientific

### Contact name
Prof John McGrath

### Contact details
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## Additional identifiers

### EudraCT/CTIS number

### IRAS number

### ClinicalTrials.gov number

### Secondary identifying numbers
ICX-DEBRHY-2

## Study information

### Scientific Title
A prospective placebo controlled phase II study to evaluate ICX-RHY-013 for the treatment of skin erosions in dystrophic epidermolysis bullosa patients

### Study objectives
To evaluate the efficacy of ICX-RHY-013 compared with placebo when injected intradermally around open skin erosions of patients with dystrophic epidermolysis bullosa.

### Ethics approval required
Old ethics approval format

### Ethics approval(s)
South East London Research Ethics Committee 3, 17 January 2011, ref: 10/H0808/146

### Study design
Single centre randomised placebo controlled phase II study

### Primary study design
Interventional

### Secondary study design
Randomised controlled trial

## Study setting(s)
Hospital

## Study type(s)
Treatment

## Participant information sheet
Not available in web format, please use the contact details below to request a patient information sheet

## Health condition(s) or problem(s) studied
Dystrophic epidermolysis bullosa (DEB)

## Interventions
Up to ten selected lesions per subject will be randomised to receive ICX-RHY-013 or placebo. Randomisation will be performed within each subject, using a computer generated randomisation scheme. A single sealed envelope for each subject will show the treatment to be allocated to each of the (up to 10) numbered lesions for that subject.

1. Each subject will undergo an initial health screen and baseline assessment of DEB erosions.
2. Subjects will undergo a single treatment with a series of injections around the margin of the erosions selected for the study.
3. Subjects will be evaluated for erosion healing (area measurements), erosion pain and erosion recurrence throughout the 26-week study period.
4. Subjects to be evaluated for study treatment safety and tolerability for 26 weeks.

## Intervention Type
Drug

## Phase
Phase II

## Drug/device/biological/vaccine name(s)
ICX-RHY-013

## Primary outcome measure
To compare the time to wound closure of skin erosions treated with ICX-RHY-013 with those treated with placebo.

## Secondary outcome measures
1. Wound area over treated areas using standard methodology (wound surface area measurements)
1.1. Wound surface area measurements at every study visit using photography and digital planimetry software will be used to measure change in wound surface area.
2. Erosion pain - study subjects will undertake pain and quality of life assessments by completing a quality of life questionnaire.
3. Wound recurrence
4. Adverse events for 12 weeks
5. Clinical laboratory safety assessment at 4 and 12 weeks
6. Clinical assessment of change in the treated area (photographic evidence)

### Overall study start date
31/01/2011

### Completion date
30/11/2011

## Eligibility

### Key inclusion criteria
1. Subjects who have a clinical diagnosis of dystrophic epidermolysis bullosa, either autosomal recessive or autosomal dominant
2. Subjects who are ≥16 and ≤70 years of age
3. Subjects with at least 5 open skin erosions which are located on the limbs or the trunk, each with a surface area between 5cm sq and 50cm sq. The size of these erosions must not exceed 50cm^2 at baseline
4. Subjects who have voluntarily signed and dated an Informed Consent Form (ICF) prior to the first study intervention
5. Subjects, who are, in the opinion of the Investigator, able to understand the study, co-operate with the study procedures and are willing to return to the clinic for all the required follow-up visits

### Participant type(s)
Patient

### Age group
Adult

### Sex
Both

### Target number of participants
Planned sample size 20-25

### Key exclusion criteria
1. Subjects who have received immunotherapy or chemotherapy within 60 days of enrolment into this study
2. Subjects with a known allergy to any of the constituents of the product
3. Subjects with known or suspected malignancy
4. Subjects with intolerance or allergy to additional study-associated drugs / therapies (e.g. anaesthetic etc)
5. Subjects who have taken systemic antibiotics within 7 days
6. Subjects taking immunosuppressive therapy including systemic steroids (i.e., oral prednisolone > 40mg for more than 1 week, intranasal / inhaled steroids are acceptable) within the 30 days of the first treatment or planning immunosuppressive therapy at any time during the study
7. Subjects who have taken any other investigational product within 90 days prior to screening or planned use of any other investigational product during the study period
8. Subjects who are pregnant, planning pregnancy and women of child-bearing potential who are not abstinent or practicing an acceptable means of contraception, as determined by the Investigator, for the duration of the treatment phase
9. Subjects with abnormal laboratory findings considered clinically significant

10. Subjects with a known history of poor adherence/compliance with medical treatment or follow up
11. Subjects who are unable to understand the aims, objectives and follow-up treatment
12. Subjects with known alcohol or narcotic drug dependence
13. Subjects who have previously been screened on more than two occasions, or who have previously been treated under this protocol
14. Subjects who have previously been treated with ICX-RHY-013 and who have an immune response for collagen VII antibody detected by immunofluorescence

### Date of first enrolment
31/01/2011

### Date of final enrolment
30/11/2011

## Locations

### Countries of recruitment
England

United Kingdom

### Study participating centre
**St John's Institute of Dermatology**
London
United Kingdom
SE1 9RT

## Sponsor information

### Organisation
Intercytex Ltd (UK)

### Sponsor details
Core Technology Facility
46 Grafton Street
Manchester
United Kingdom
M13 9NT

### Sponsor type
Industry

### Website
http://www.intercytex.com/

**ROR**
https://ror.org/04svmrs70

# Funder(s)

**Funder type**
Research organisation

**Funder Name**
Dystrophic Epidermolysis Bullosa Research Association (DEBRA) (UK)

**Funder Name**
Technology and Strategy Board (UK)

**Funder Name**
Intercytex Ltd (UK) - Investigational product manufacturer and supplier

# Results and Publications

**Publication and dissemination plan**
Not provided at time of registration

**Intention to publish date**

**Individual participant data (IPD) sharing plan**

**IPD sharing plan summary**
Not provided at time of registration

**Study outputs**

| Output type | Details | Date created | Date added | Peer reviewed? | Patient-facing? |
|---|---|---|---|---|---|
| Results article | results: | 01/11/2013 | | Yes | No |
| HRA research summary | | | 28/06/2023 | No | No |