

What do we do best and what do we still need to develop ?

Ben Merrett CEO DEBRA UK

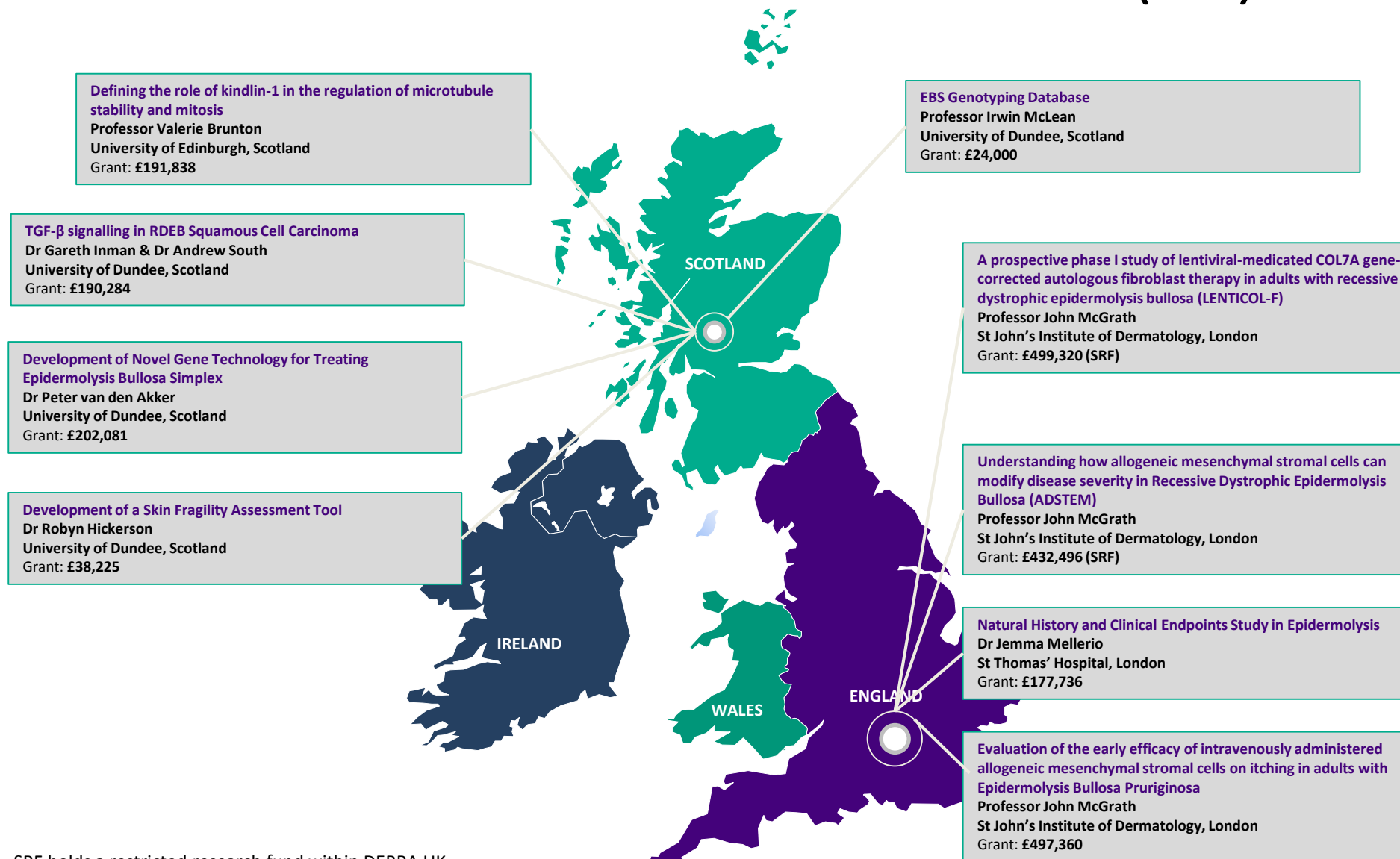
#Fighteb



EB care and research is
worldwide

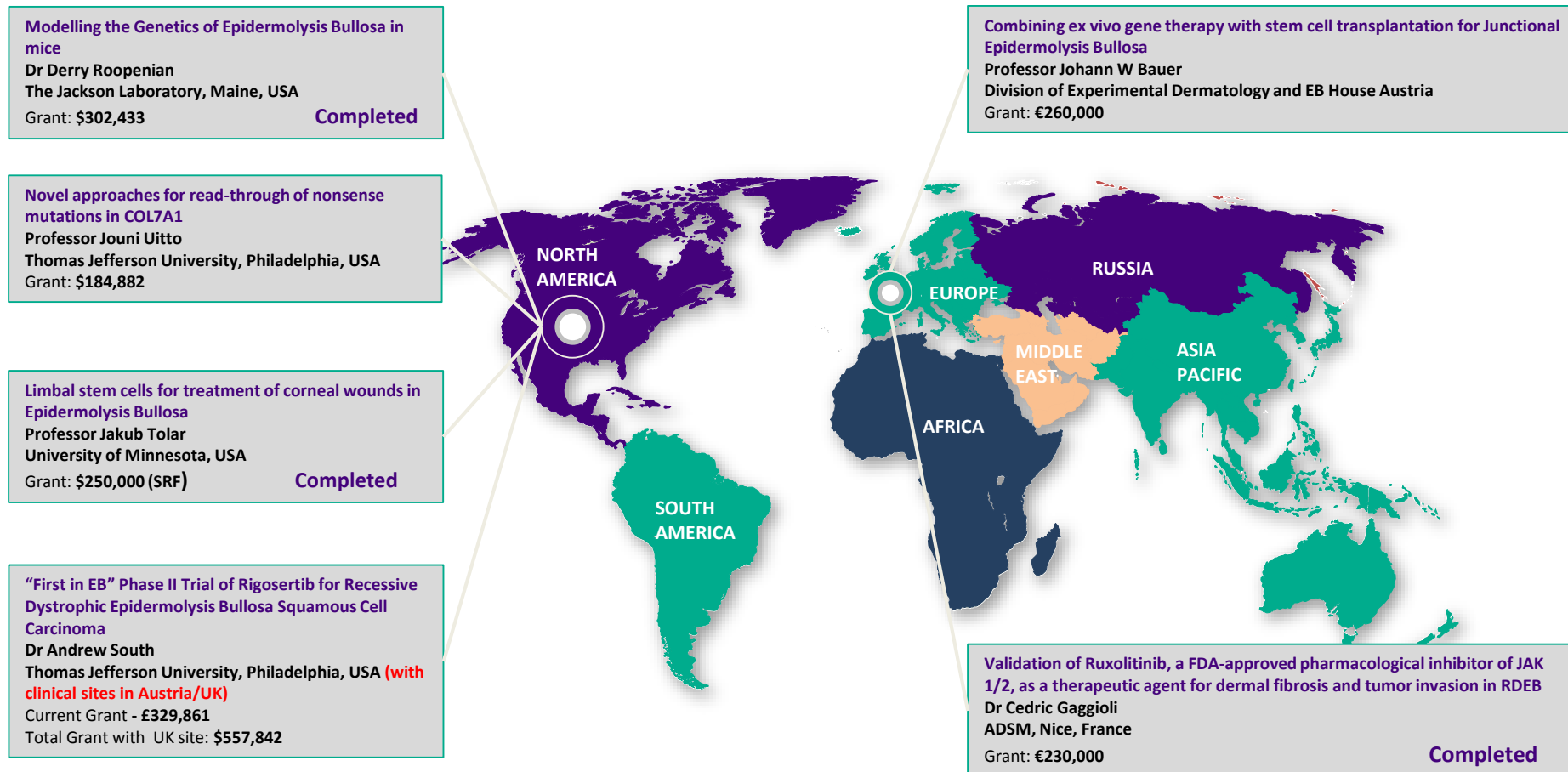


DEBRA UK funded research 2017 (UK)



SRF holds a restricted research fund within DEBRA UK and now is also a UK registered charity no 1158672

DEBRA UK funded research 2017 (Global)



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For more information visit:
<http://www.debra.org.uk/uk-funded-projects/current-uk-funded-projects>

● ● | EB research is accelerating



● DEBRA UK, Austria, USA primary funders (DEBRA International)

● Sohana Research Fund UK

● EB Research Partnership EB Medical Research Foundation USA

● Other government and agency funding

● Pharmaceutical and biotechnology companies

● ● | Patient perspectives – Top 10



- 1 Which wound care method gets better outcomes
- 2 What is the best treatment to control itch
- 3 What is the best pain control strategy
- 4 How much does management in reference centres help
- 5 How effective is tumour early diagnosis
- 6 What are the long term effects of syndactyly surgery
- 7 Which is the most important method to prevent or delay syndactyly
- 8 What role might tissue engineering have in treating wounds
- 9 What role might stem cell therapy and bone marrow transplantation play
- 10 What role might growth hormone play

Wound Healing Priority Setting Meeting London December 2016



● ● | Grant Rounds 2017

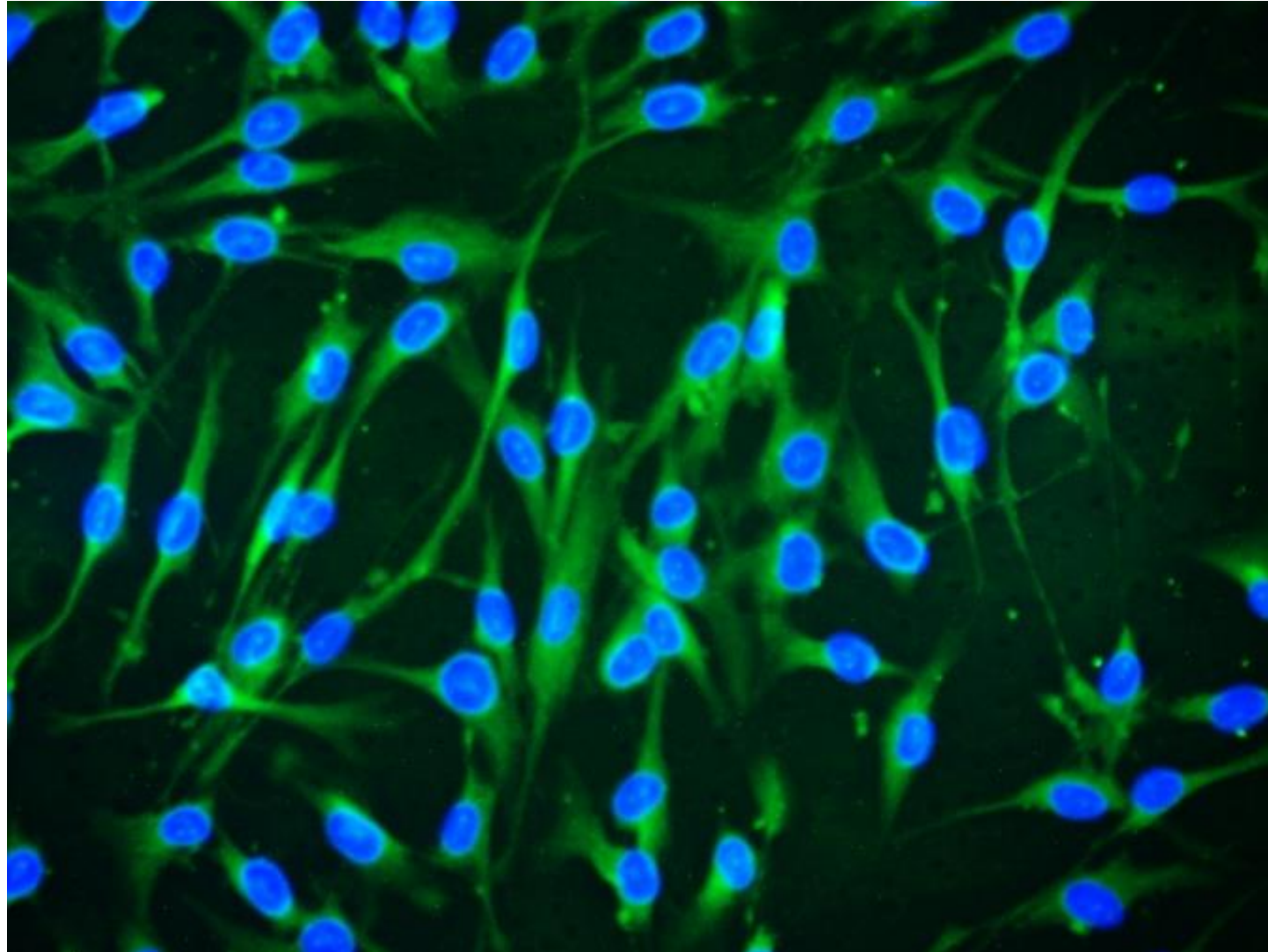


- Wound healing projects generated from priority setting meeting Dec 2016 (18 at stage 2 applications – Euro 6m)
- All projects peer reviewed – organised by UK and Austria
- Scientifically robust proposals approved for funding
- 3 projects costing about £1m

● ● | What about pruritus/itch?



- ● | Mesenchymal stem cells
(human – bone marrow)



● ● | MSC infusions

- Study to evaluate if donor MSCs can produce benefit in the skin of 10 children with RDEB - 3 intravenous MSC infusions + 2 year follow up
 - **Improvement in Quality of Life** Score and Birmingham EB severity score (BEBSS) [decrease in mean parent-reported pain score, disease severity, total body surface area affected]
 - Telephone interviews at 9 months revealed subjective reports of **better wound healing** in all 10 children and **less skin redness** in 9/10; clinical benefit lasted around 4-6 months. **Better sleep and improved family life** was reported

● ● | PRUSTEM - £500k



- Follows successful and informative studies EBSTEM (published) and ADSTEM (ongoing) which looked at MSCs in children and adults
 - Positive effect seen in itch
- This study will
 - Add to the safety and efficacy data (increasing patient numbers) for MSCs vital for the regulatory environment
 - Importantly continue the use of MSCs in the clinical setting
 - Group of patients treated over 3 years



Transduced epidermal sheets for DEB (Stanford)



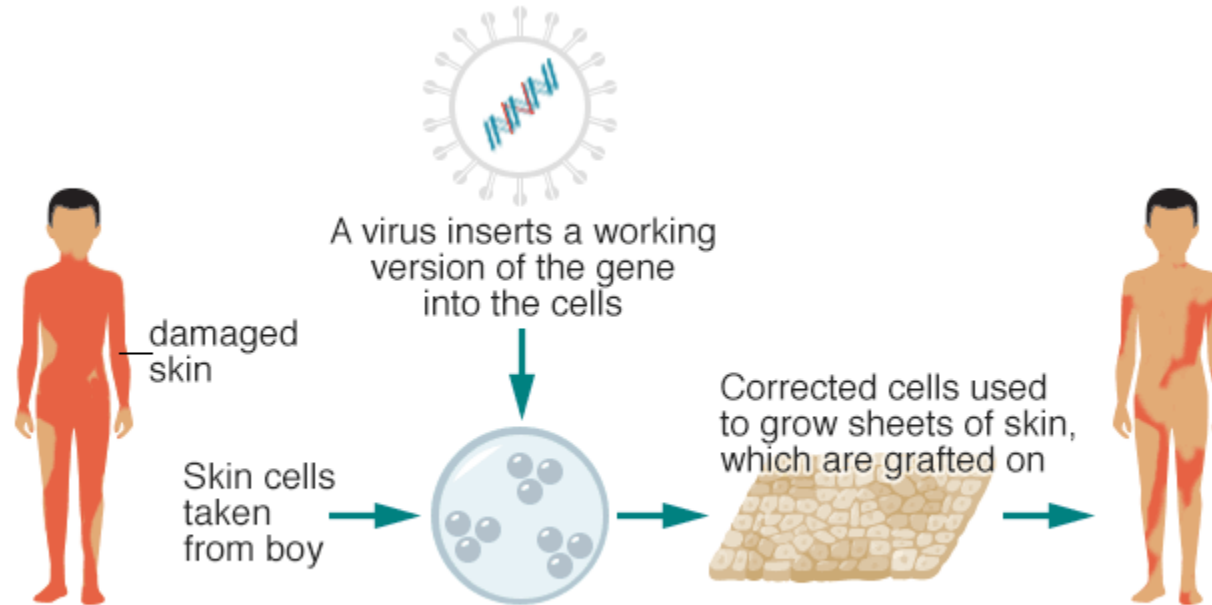
Phase I Clinical trial in 4 adult males

- 6 wounds per patient
- 24 wounds total
- Safety evaluation
- Efficacy
 - Wound healing
 - Itch/Pain
 - Biopsy for collagen 7



Results: 21 out of 24 wounds healed after 6 months

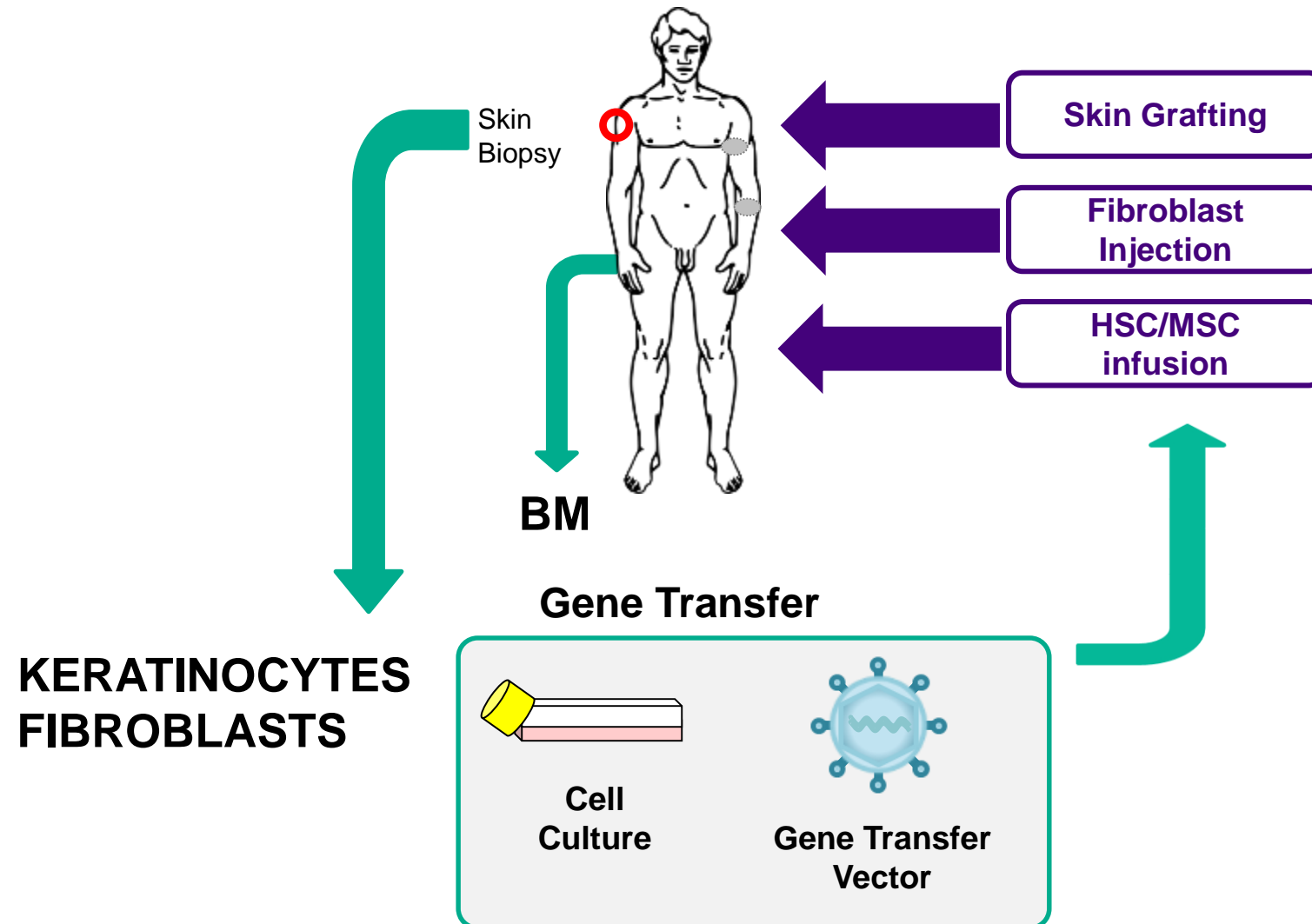
● ● | German boy Skin Graft



Source: Nature

BBC

● ● | The future...?



● ● | "First in EB" Phase II Trial of Rigosertib
for Recessive Dystrophic Epidermolysis Bullosa Squamous Cell
Carcinoma



- Test of a new drug in development
- Clinical study in a small number of patients
- Scientific research on samples from the patients
- \$557,842 [£377,508]
- Up to 60 months

Funding DI activities about £200k each year

- Staffing support
- DI Research conference and Clinet 2019 - London
- Matched funding for
 - Registry
 - web site development
 - clinical best practice
 - clinical fellow (Robin Eady memorial)