

**Table S1. Active Clinical Trials for Epidermolysis Bullosa (June 2015)**

This Table provides a status update of current clinical trials in EB. The authors have attempted to compile a comprehensive list but apologise to investigators and participants for any additional clinical trials that are not shown (for updated website, see <http://www.badgem.org.uk/active-clinical-trials>).

Disease under investigation	Epidermolysis bullosa
Study title	A phase I/II study evaluating allogeneic mesenchymal stromal cells in adults with recessive dystrophic epidermolysis bullosa.
Study synopsis	A phase I/II study evaluating allogeneic mesenchymal stromal cells in adults with recessive dystrophic epidermolysis bullosa.
Clinical trial registration no.	EudraCT Number: 2014-004500-30
Country	United Kingdom
Date Registered	May 2015
Current Status	Ongoing
Disease under investigation	Epidermolysis bullosa
Study title	Phase I study of COL7A1 gene-modified autologous fibroblasts in adults with recessive dystrophic epidermolysis bullosa.
Study synopsis	A study to evaluate whether intradermal injections of COL7A1 gene-modified autologous fibroblasts are safe in adults with recessive dystrophic epidermolysis bullosa.
Clinical trial registration no.	EudraCT Number: 2014-004884-19
Country	United Kingdom
Date Registered	May 2015
Current Status	Ongoing
Disease under investigation	Epidermolysis bullosa
Study title	An open label, multi-center, extension study to evaluate the long-term safety of Zorblisa (SD-101-6.0) in patients with epidermolysis bullosa
Study synopsis	This study aims to evaluate the long-term safety of Zorblisa in patients with simplex, recessive dystrophic, and junctional non-herlitz epidermolysis bullosa
Clinical trial registration no.	EudraCT Number: 2014-005679-96
Country	Austria
Date Registered	February 2015
Current Status	Ongoing

Disease under investigation	Epidermolysis bullosa simplex
Study title	Diacerein for the treatment of epidermolysis bullosa simplex
Study synopsis	A placebo-controlled study to evaluate diacerein as treatment of epidermolysis bullosa simplex
Clinical trial registration no.	EudraCT Number: 2013-002034-21
Country	Austria
Date Registered	1 August 2013
Current Status	Ongoing
Disease under investigation	Epidermolysis bullosa
Study title	Comparative clinical trial, open label, multicenter, to evaluate the effectiveness and safety of anew therapy for grown chimeric skin treatment of cutaneous lesions of patients with epidermolysis bullosa (Translated from Spanish)
Study synopsis	To assess the efficacy of treatment by local application of chimeric cultured skin patches, versus the use of a non-adherent occlusive dressing. (Translated from Spanish)
Clinical trial registration no.	EudraCT Number: 2005-002329-30
Country	Spain
Date Registered	15 March 2010
Current Status	Ongoing
Disease under investigation	Recessive dystrophic epidermolysis bullosa
Study title	Bicentric, open and pilot study evaluating the efficiency and the tolerance of the photodynamic therapy in the treatment of epidermal dysplasia for patients affected by hereditary dystrophic epidermolysis bullosa
Study synopsis	The main objective of this study is to determine the efficiency of photodynamic therapy in the treatment of epidermal dysplasia for patients affected by dystrophic epidermolysis bullosa
Clinical trial registration no.	NCT02004600
Country	France
Date Registered	26 November 2013
Current Status	Ongoing
Disease under investigation	Epidermolysis bullosa simplex and pachyonychia congenita
Study title	Botulinum toxin A treatment in epidermolysis bullosa simplex and pachyonychia congenita-a double-blind placebo-controlled phase II proof of

Study synopsis	concept study This is a phase II study to evaluate the use of Botulinum toxin A as a treatment of children and adult patients with epidermolysis bullosa simplex and pachyonychia congenita.
Clinical trial registration no.	EudraCT Number: 2009-010763-17
Country	Sweden
Date registered	20 March 2009
Current status	Ongoing
Disease under investigation	Epidermolysis bullosa (simplex, recessive dystrophic, junctional non Herlitz)
Study title	A Phase 3, multi-center, randomized, double-blind, placebo controlled study of the efficacy and safety of SD-101 cream in patients with epidermolysis bullosa
Study synopsis	This is a phase III study to investigate the efficacy and safety of topical SD-101 cream in patients with epidermolysis bullosa.
Clinical trial registration no.	EudraCT Number: 2014-002288-14 NCT02090283
Country	Austria; United States
Date registered	12 March 2014
Current status	Ongoing, but not recruiting patient
Disease under investigation	Recessive dystrophic epidermolysis bullosa
Study title	Unrelated cord blood transplantation after reduced toxicity conditioning with mesenchymal stromal cell co-infusion in patients with severe epidermolysis bullosa
Study synopsis	This is a phase II study to evaluate stem cell transplantation with cord blood and mesenchymal stem cells after reduced intensity conditioning for patients with severe forms of epidermolysis bullosa.
Clinical trial registration no.	EudraCT Number: 2012-000605-72
Country	Netherlands
Date registered	12 August 2013
Current status	Ongoing
Disease under investigation	Recessive dystrophic epidermolysis bullosa
Study title	Traitement des épidermolyses bulleuses dystrophiques héréditaires par l'épigallocatechine-3-gallate oral (Polyphenon E®)
Study synopsis	The primary purpose of this study is to assess the efficacy of oral Polyphenon E in decreasing the

Clinical trial registration no.	number of cutaneous bullosa after four month of treatment.
Country	EudraCT Number: 2009-012750-21
Date registered	France
Current status	27 November 2009
	Ongoing
Disease under investigation	Recessive dystrophic epidermolysis bullosa
Study title	A phase I single center trial of gene transfer for recessive dystrophic epidermolysis bullosa using the drug LZRSE-Col7A1 engineered autologous epidermal sheets (LEAES)
Study synopsis	The purpose of this study is to achieve proof-of-concept for this general approach to cell-based gene therapy in humans with recessive dystrophic epidermolysis bullosa.
Clinical trial registration no.	NCT01263379
Country	United States
Date registered	15 December 2010
Current status	Ongoing, recruiting
Disease under investigation	Epidermolysis bullosa
Study title	Biochemical correction of severe epidermolysis bullosa by allogeneic stem cell transplantation
Study synopsis	The primary objective of this phase II study is to estimate the event-free survival rate by 1 year post-transplant with an event defined as a death or failure to have a demonstrable increase in collagen, laminin, integrin, keratin or plakin deposition by 1 year post-transplant or other biochemical, structural or physical measure of improvement.
Clinical trial registration no.	NCT01033552
Country	United States
Date registered	14 December 2009
Current status	Ongoing, recruiting
Disease under investigation	Epidermolysis bullosa
Study title	A phase 3, multi-center, randomized, double-blind, placebo controlled study of the efficacy and safety of SD-101 cream in patients with epidermolysis bullosa
Study synopsis	To assess the efficacy and safety of SD-101-6.0 cream versus SD-101-0.0 (placebo) in the treatment of patients with epidermolysis bullosa.
Clinical trial registration no.	NCT02384460

Country	United States
Date registered	13 February 2015
Current status	Ongoing, recruiting
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Disease under investigation	Epidermolysis bullosa
Study title	A comparative study of the healing of chronic skin ulcers of recessive dystrophic epidermolysis bullosa: Standard dressing versus amniotic membrane.
Study synopsis	The aim of this study is to evaluate the efficacy of the amniotic membrane on the healing of chronic ulcers REBD on the percentage ulcerated surface re-epithelialised at 12 weeks (M3) from the start of treatment.
Clinical trial registration no.	NCT02286427
Country	France
Date registered	5 November 2014
Current status	Ongoing, recruiting
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Disease under investigation	Epidermolysis bullosa
Study title	Pilot study evaluating the efficiency and the tolerance of the PDT in the treatment of epidermal dysplasia for patients affected by hereditary DEB
Study synopsis	The main objective of this study is to determine the efficiency of the photodynamic therapy in the treatment of epidermic dysplasias for patients affected by dystrophic epidermolysis bullosa (DEB).
Clinical trial registration no.	NCT02004600
Date registered	26 November 2013
Current status	Ongoing, not recruiting yet
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Disease under investigation	Epidermolysis bullosa
Study title	Treatment of Dowling Meara Type of Epidermolysis Bullosa Simplex by Oral Erythromycin (Phase 3)
Study synopsis	The aim of this study is the evaluation of the efficiency of oral erythromycin to decrease the number of cutaneous blisters in severe EBS-DM patients from 6 months to 8 years old after 3 months of treatment.
Clinical trial registration no.	<u>NCT01340235</u>
Date registered	11 April 2011
Country	France
Current status	Ongoing

Disease under investigation	Recessive dystrophic epidermolysis bullosa
Study title	Clinical research of bone marrow-derived mesenchymal stem cell transplantation for the patients with epidermolysis bullosa.
Study synopsis	The aim of this study is the evaluation of the safety and therapeutic potential of intradermal injections of allogeneic mesenchymal stromal cells on wound healing
Clinical trial registration no.	UMIN000006723
Date registered	14 November 2011
Country	Japan
Current status	Ongoing