

## Sub-thematic group on Epidermolysis Bullosa

### Research Update

#### Currently recruiting clinical therapy trials for EB (October 2020)

- The following table gives an overview of clinical therapy trials for EB that are recruiting new patients
- If you would like to contact the trial leaders to see if your patient is eligible to participate, please contact an ERN Reference Center in your country
- Alternatively, you can find the contact information by entering the trial number on the trial registry website:
  - <https://www.clinicaltrialsregister.eu>
  - <https://clinicaltrials.gov>

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### Therapies with intent- to-cure

Gene therapy	Investigational Drug	EB Type	Trial Identification Nr.
1.	Transplantation surgery of genetically corrected cultured epidermal autograft	JEB <i>COL17A1</i> mutations	ClinicalTrials.gov NCT03490331 Salzburg
2.	Genetically corrected cultured epidermal autograft	RDEB	ClinicalTrials.gov NCT02984085 Salzburg
3.	EB-101 autologous, gene-corrected keratinocyte sheets	RDEB	ClinicalTrials.gov NCT04227106 USA, Stanford
4.	KB103, topically applied non-integrating, replication-incompetent herpes simplex virus vector expressing human collagen VII protein	DEB	ClinicalTrials.gov NCT03536143 USA, Stanford
5.	Genetically corrected skin equivalent	RDEB	EudraCT Number 2016-002790-35 Paris

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	Investigational Drug	EB Type	Trial Identification Nr.
<b>Antisense oligos</b>	QR-313 , topically applied antisense oligonucleotide	DEB with <i>COL7A1</i> exon 73 mutations	ClinicalTrials.gov NCT03605069 USA, multicenter
<b>PTC readthrough</b>	Gentamicin, intravenous	RDEB	ClinicalTrials.gov NCT03392909 USA, Los Angeles
	Gentamicin, topical	JEB	ClinicalTrials.gov NCT03526159 USA, Los Angeles
	Optimizing i.v. gentamicin in JEB	JEB	ClinicalTrials.gov NCT04140786 USA, Los Angeles
<b>Protein therapy</b>	PTR-01, recombinant human collagen VII	RDEB	ClinicalTrials.gov NCT03752905 USA, Stanford

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**Regenerative cell-based therapies**

	<b>Investigational Drug</b>	<b>EB Type</b>	<b>Trial Identification Nr.</b>
<b>Cell therapies</b>			
<b>1.</b>	Serial mesenchymal stem cell (MSC) infusions from a related donor	All EB types	ClinicalTrials.gov NCT02582775 USA, Minnesota
<b>2.</b>	Allogeneic stem cell transplantation and "off-the-shelf" mesenchymal stem cells	All EB types	ClinicalTrials.gov NCT01033552 USA, Minnesota
<b>3.</b>	Allogeneic ABCB5-positive Stem Cells	RDEB	ClinicalTrials.gov NCT03529877 Germany, international
<b>4.</b>	Epidermal grafts generated using the Cellutome System	EB after hematop. cell transplant.	ClinicalTrials.gov NCT02670837 USA, Minnesota
<b>5.</b>	Bone marrow derived MSC infusions (MisenSistem-EB)	RDEB	EudraCT Number 2017-000606-37 Spain

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Symptom-relief therapies - 1

	Investigational Drug	EB Type	Trial Identification Nr.
Anti-inflammatory	Oleogel-S-10, topical	All EB types	ClinicalTrials.gov NCT03068780 International
	Ixekizumab, subcutaneous	EBS severe	EudraCT Number: 2020-001542-19
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Accelerator of wound healing	RGN-137, a thymosin beta-4 gel, topical	JEB, DEB	. ClinicalTrials.gov NCT03578029 USA

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Symptom-relief therapies - 2

	Investigational Drug	EB Type	Trial Identification Nr.
<b>Analgesic</b>	Ropivacaine, topical .....	All EB types .....	ClinicalTrials.gov NCT03730584 Paris .....
<b>Anti-pruritic</b>	Neurokinin-1 Receptor Antagon., oral .....	All EB types .....	ClinicalTrials.gov NCT03836001 USA, Stanford .....
<b>Analgesic &amp; Anti-pruritic</b>	Pregabalin for itch and pain .....	RDEB .....	ClinicalTrials.gov NCT03928093 Canada, Toronto .....
<b>Anti-hydrotic</b>	Botulinic toxin .....	EBS .....	ClinicalTrials.gov NCT03453632 France, multicenter .....
<b>Anti-cancer</b>	Rigosertib for advanced EB cancer	RDEB	ClinicalTrials.gov NCT03786237 EudraCT Number: 2016-0036832- 19 Salzburg