Abstract citation ID: ljaf465.004

O04 Mission EB -mesenchymal intravenous stromal cell infusions in children with recessive dystrophic epidermolysis bullosa: a randomised, double-blinded, placebo controlled, crossover trial

Maria L. Bageta,[1] Pablo López Balboa,[1] Matt Bursnall,[2] Isabelle Wilson,[2] Katie Biggs,[2] Rachel Glover,[2] Katie Hutchence,[2] Shamila Ditta,[2] Steven Julious,[2] Pratima Poudel,[3] Muna Nadeem,[3] Diana Papaioannou,[2] Malobi Ogboli,[3] Marie-Louise Lovgren,[3] John McGrath,[4] Cindy Cooper,[2] Gabriela Petrof,[1] and Anna E. Martinez[1]

[1]Dermatology Department, Great Ormond Street Hospital NHS Foundation Trust, London, UK; [2]Sheffield Centre for Health and Related Research, Clinical Trials Research Unit, Sheffield, UK; [3]Dermatology Department, Birmingham Women´s and Children´s Hospital NHS Foundation Trust, Birmingham, UK; and [4]St John's Institute of Dermatology, King's College London, London, UK

Abstract

Recessive Dystrophic Epidermolysis Bullosa (RDEB) is a severe type of epidermolysis bullosa resulting from mutations in *COL7A1* gene. Patients experience various degrees of blistering following minor trauma which can over time lead to fibrosis, limb contractures and an increased risk of developing squamous cell carcinoma. The aim was to assess if repeated infusions of allogeneic umbilical cord derived mesenchymal stromal cells (UC-MSCs), CORDStrom^{TM}, were safe and could benefit children with RDEB.

This was a prospective, double-blinded, randomised, placebo-controlled crossover trial with an internal dose de-escalation trial (for safety) conducted at the two National Paediatric UK EB centres in RDEB children aged >6months and <16years.

Participants received IV infusions of $2–3 \times 10^6$ cells/kg of UC-MSCs or placebo at day 0 and 14 days later with a 9-month wash out period between the opposite.

Main outcomes were change in disease severity as measured by the Epidermolysis Bullosa Disease Activity and Scarring index (EBDASI) and the Instrument for Scoring Clinical Outcomes of Research for Epidermolysis Bullosa (ISCOREB), wound clinical appearance, pain, itch, and quality of life at 3-months from infusion. Results will be discussed at the meeting.

This is the largest study worldwide in children with RDEB. Administering cell therapy early and at regular intervals has the potential to reduce inflammation, effectively modulate disease activity, and lead to clinically meaningful and sustained improvements in disease progression and quality of life. An open-label study is planned and will help us further evaluate the long-term safety and outcomes of CORDStrom^{TM} infusions in children with RDEB.

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of patients with PsO achieved **PASI 75 at Week 4**

(vs 1.2% placebo [n=1/86], p<0.0001)*** [2]

76.9% (N=52)†

of patients with PsO achieved **PASI 100 at 5 years**[3]

51.5% (n=222/431) and **50.6%** (n=135/267)

of biologic-naïve and TNFi-IR PsA patients achieved **ACR 50 at Week 104/100,** respectively‡ [1,4–6]

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These data are from different clinical trials and cannot be directly compared.

Co-primary endpoints PASI 90 and IGA 0/1 at Week 16 were met.**Secondary endpoints. †N= mNRI, missing data were imputed with mNRI (patients with missing data following treatment discontinuation due to lack of efficacy or a TRAE were counted as non-responders; multiple imputation methodology was used for other missing data). ‡43.9% (n=189/431), and 43.4% (n=116/267) of biologic-naïve and TNFi-IR PsA patients achieved the primary endpoint of ACR 50 at Week 16 in BE OPTIMAL and BE COMPLETE, respectively (vs 10.0% [n=28/281] and 6.8% [n=9/133] placebo, p<0.0001); 54.5% (n=235/431) and 51.7% (n=138/267) maintained it at Week 52 (NRI).[4–6]

ACR 50, ≥50% response in the American College of Rheumatology criteria; **AS**, ankylosing spondylitis; **CRP**, C-reactive protein; **DMARD**, disease-modifying antirheumatic drug; **HS**, hidradenitis suppurativa; **IGA**, Investigator's Global Assessment; **(m)NRI**, (modified) non-responder imputation; **MRI**, magnetic resonance imaging; **nr-axSpA**, non-radiographic axial spondyloarthritis; **NSAID**, non-steroidal anti-inflammatory drug; **PASI 75/90/100**, ≥75/90/100% improvement from baseline in Psoriasis Area and Severity Index; **PsA**, psoriatic arthritis; **PsD**, psoriatic disease; **PsO**, psoriasis; **TNFi-IR**, tumour necrosis factor-α inhibitor – inadequate responder; **TRAE**, treatment-related adverse event.

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GB-BK-2500315 | July 2025

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