

## Quality control for adult stem cell treatment

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A team of European researchers has devised a strategy to ensure that adult epidermal stem cells are safe before they are used as treatments for patients. The approach involves a clonal strategy where stem cells are collected and cultivated, genetically modified and single cells isolated before being rigorously tested to make sure they meet the highest possible safety criteria. The strategy, which is published online in *EMBO Molecular Medicine*, is inspired by the approaches the biotechnology industry and regulatory affairs authorities have adopted for medicinal proteins produced from genetically engineered mammalian cells.

"Until now there has not been a systematic way to ensure that adult epidermal <u>stem cells</u> meet all the necessary requirements for safety before use as treatments for disease," says EMBO Member Yann Barrandon, Professor at Lausanne University Hospital, the Swiss Federal Institute of Technology in Lausanne and the lead author of the study. "We have devised a single cell strategy that is sufficiently scalable to assess the viability and safety of adult epidermal stem cells using an array of cell and molecular assays before the cells are used directly for the treatment of patients. We have used this strategy in a proof-ofconcept study that involves treatment of a patient suffering from recessive dystrophic epidermolysis bullosa, a hereditary condition defined by the absence of type VII collagen which leads to severe blistering of the skin."

The researchers cultivated <u>epidermal cells</u> from the patient that can be used to regenerate skin. The scientists used their array of tests to determine which of the transduced cells met the necessary requirements



for stemness – the characteristics of a stem cell that distinguish it from a regular cells – and safety. Clonal analysis revealed that the transduced stem cells varied in their ability to produce functional type VII collagen. When the most viable, modified stem cells were selected, transplantation onto immunodeficient mice regenerated skin that did not blister in the mouse model system for recessive dystrophic epidermolysis bullosa and produced functional type VII collagen. Safety was assessed by determining the sites of integration of the viral vector, looking for rearrangements and hit genes, as well as whole genome sequencing.

"Our work shows that at least for adult epidermal stem cells it is possible to use a clonal strategy to deliver a level of safety that cannot be obtained by other gene therapy approaches. A clonal strategy should make it possible to integrate some of the more recent technologies for targeted genome editing that offer more precise ways to change genes in ways that may further benefit the treatment of disease. Further work is in progress in this direction."

**More information:** *EMBO Molecular Medicine*, "A single epidermal stem cell strategy for safe ex vivo gene therapy," Stéphanie Droz-Georget Lathion, Ariane Rochat, Graham Knott, Alessandra Recchia, Danielle Martinet, Sara Benmohammed, Nicolas Grasset, Andrea Zaf-falon, Nathalie Besuchet Schmutz, Emmanuelle Savioz-Dayer, Jacques S. Beckmann, Jacques Rougemont, Fulvio Mavilio and Yann Barrandon, DOI: 10.15252/emmm.201404353

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