The condition deteriorated severely six weeks before admission, indicating a homozygous acceptor splice site mutation (C1977-1G>A, IVS14-1G>A) within intron 14 of LAMB3. This resulted in blisters and impaired their quality of life and could lead to skin cancer. Unfortunately, there is no known cure for JEB and more than 40% of patients die before adolescence.

However, recent heroic work published in Nature (Hirsch et al, 2017) [3], showed that autologous transgenic keratinocyte cultures could regenerate an entire, fully functional epidermis on patients with JEB. The first patient, a seven-year-old child suffering from a devastating, life-threatening form of JEB [3], demonstrated life-saving regeneration. A major unaddressed question is related to the mechanisms that control the balance of epidermal growth and differentiation. A better understanding of the complex signals, cellular interactions, and mechanisms of tissue regeneration may open new avenues for treatment. However, it is notable that during this time, the epithelium undergoes continual remodeling. The challenge now is to exploit this newfound knowledge to harness the natural potential of stem cells for the desired task at hand. The successful outcome of this study paved the way for gene therapy to treat other types genetic disorders and provides a blueprint that can be applied to other stem cell-mediated combined ex vivo cell and gene therapies. Numerous unresolved biological problems will occupy researchers for the coming decade, but their solutions will provide the molecular soil that will nurture new and improved clinical applications in regenerative medicine.
Conflicts of Interest
All author declare that they have no conflicts of interest concerning on this manuscript.

References