Nanoneedles for Cell and Gene Therapy

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Nanoneedle arrays, consisting of high-aspect-ratio nanostructures on nanotextured surfaces, have emerged as powerful tools for probing and manipulating the intracellular environment. Their unique design allows them to interact with cells with minimal perturbation, making them highly effective in delivering a variety of therapeutic agents—including small molecules, nucleic acids, proteins, and nanoparticles—into cells without inducing toxicity. Notably, nanoneedles have shown high efficiency in targeting cells that are traditionally difficult to transfect, such as primary human cells, stem cells, immune cells, and neurons. This capability is unlocking new possibilities for the ex vivo genetic engineering of primary cells with unprecedented efficiency. Moreover, the application of nanoneedles in vivo, particularly on the skin and eyes of live animals, is paving the way for novel topical therapies involving nucleic acids and biologics. These advances underscore the potential of nanoneedles in developing advanced therapy medicinal products (ATMPs), including cell and gene therapies, as well as CRISPR-based gene editing.

This talk outlines our progress in developing nanoneedles for topical gene therapy and for gene editing of cells used for autologous cell therapies. It will highlight fabrication methods for incorporating nanoneedles within a broad range of medical devices, including bandages, contact lenses, catheters and hydrogel. It will demonstrate the application of nanoneedles to skin wounds in epidermolysis bullosa for Collagen Type VII gene editing and gene therapy, and nucleic acid therapy for the topical treatment of corneal endothelial dysfunction. Nanoneedles also mediate the base editing of primary skin fibroblasts and the introduction of chimeric antigen receptor genes in regulatory T cells for cell-based therapies in epidermolysis bullosa and graft-vs-host disease.

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Figures

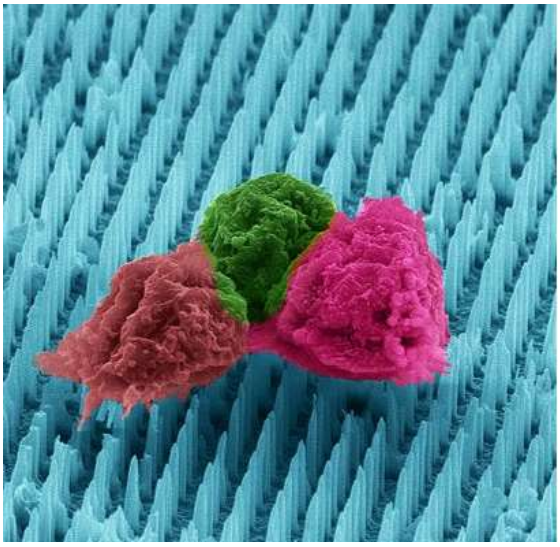

Figure 1: Scanning Electron Micrograph of three cells on nanoneedles.