



一種基因或其他生物分子的轉殖方法

提案人： 徐善慧 教授

單位： 臺灣大學高分子科學與工程學研究所

簡歷： <http://homepage.ntu.edu.tw/~shhsu/index.htm>

市場及需求： 基因治療是指將外源正常基因導入靶細胞，以糾正或補償因基因缺陷和異常引起的疾病，以達到治療目的。根據 GlobalData 的研究數據，全球基因治療市場的總交易數從 2013 年的 16 例增長到 2014 年的 36 例，同一時期，交易的綜合價值從 1.228 億美元猛增到 49 億美元，這代表 40 倍的增長。由此可知基因治療於全球市場的潛力。而目前基因治療的範疇也由原始的遺傳性疾病不斷擴增至癌症、感染性疾病、心臟血管疾病等。除此之外，人類基因組計畫的進行，已讓我們對更多疾病的成因有更多了解，其中許多遺傳疾病的成因也在此計畫進行期間被揭開，如乳癌基因與杭丁頓舞蹈症基因等。這些致病基因的揭開，可使我們更了解如何去設計治療基因以治療這些基因缺陷所造成的疾病。然而目前將外源正常基因導入靶細胞的方法皆有生物安全的問題，易產生極大的副作用，容易造成患者死亡。有鑑於此，本發明提出一種新的轉殖技術應用於基因治療。本發明的亮點優勢為不具有生物毒性、不具有外來物免疫反應、可結合生醫材料同時進行基因治療與客製化組織修復。

技術摘要(含成果)： 本技術將特定物料、基因(或其他生物分子)與細胞混合，以同時達到該患部基因治療與組織修復。

優勢： 不具有生物毒性、不具有外來物免疫反應、可結合生醫材料同時進行基因治療與組織修復、客製化。

競爭產品： 反轉錄病毒載體 (retroviral vector)，腺病毒載體 (adenoviral vector)，腺衛星病毒載體 (adeno-associated viral vector)，或微脂粒 (liposome)。以上產品為目前市面上所常用之基因治療載體，具有外來物免疫反應與細胞毒性，故易造成副作用，更甚者易導致患者死亡。

專利現況： 徐善慧教授研發生醫療材料與組織工程已有數十年之經驗。在本發明中所揭露的技術對於組織修復(體內與體外)皆具有令人鼓舞的成效。本研究結果，目前已準備投稿於高影響因子之國際期刊並準備專利申請。

聯絡方式(請不用填)： 臺大產學合作總中心 Tel: 02-3366-9945, E-mail: ntuciac@ntu.edu.tw

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In situ transfection

PI : Prof. Shan-hui Hsu; Institute of Polymer Science and Engineering

Experience: <http://homepage.ntu.edu.tw/~shhsu/index.htm>

Market Needs: Gene therapy refers to the therapeutic introduction of normal exogenous genes into target cells to correct or compensate for diseases triggered by genetic defects and disorders. According to data from Global Data, the total number of transactions in the global gene therapy market increased from 16 in 2013 to 36 in 2014. During this period, the total value of the transactions increased from \$122.8 million to \$4.9 billion, representing a 40-fold growth and indicating a strong potential for gene therapy in the global market. The current scope of gene therapy has been continuously expanded from original genetic diseases to other diseases such as cancers, infectious diseases, and cardiovascular diseases. Additionally, the Human Genome Project allowed for an increased understanding of genes relating to disease etiology, such as those for breast cancer and Huntington's disease. The revelation of these genes allows us to design therapeutic DNA treatments to treat diseases that are caused by genetic mutations. However, there is a concern for biosafety with the current methods of introducing normal exogenous genes into target cells, which can readily lead to serious side effects, resulting in patient mortality. Thus, the present invention proposes a novel transduction technique for gene therapy. The key advantages of this invention include a lack of biological toxicity and immune reactions to foreign substances, and the ability to be implemented with biomaterials for simultaneous gene therapy and customized tissue repair.

Our Technology: We developed a novel transfection method based on cells encapsulated within a material containing gene or biomolecules.

Strength: Low cytotoxicity, high efficiency, customized tissue repair.

Competing Products: Retroviral vector, adenoviral vector, adeno-associated viral vector) ' and liposome.

Intellectual Properties: Prof. Shan-hui Hsu has a deep experience in biomaterials, biodegradable materials, and biomimetic green materials, as well as the study of interface between materials and cells/tissues. We showed that the novel *in situ* transfection offers the possibility to repair damage tissue in the future. We will contribute to a high impact factor journal for *in situ* transfection.

Contact (do not need to fill out):

Center for Industry-Academia Cooperation, NTU

Tel: 02-3366-9945, E-mail: ntuciac@ntu.edu.tw

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