

## **Skin cell ‘factories’ could provide cure for deadly skin disease**

For immediate release

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A group of scientists in London are using a pioneering technique to grow sheets of genetically engineered skin to be grafted on and replace defective skin affected by the skin disease Netherton’s Syndrome.

The year-long study, funded by the skin disease research charity the British Skin Foundation, is being headed by Dr Wei-Li Di at University College London’s Institute of Child Health. She and her team are focusing their efforts on the rare and potentially life-threatening form of inherited ichthyosis which is believed to affect around one person in every 200,000 births in the UK.

Typical symptoms of Netherton’s Syndrome include severe scaling and cracking of the skin, thin fragile scalp hair and frequent infections due to the cracked and open wounds on the skin. Babies born with the disease are often underweight and grow slower than usual for their first few of years of life. Although the skin tends to improve as the child gets older, symptoms can repeatedly flare up without warning.

There are currently no cures for this disease with treatment limited to use of emollients to maintain the skin’s moisture levels and barrier function. It is believed that Netherton’s is caused by changes in a gene called *SPINK5*, resulting in defective production and function of a particular skin protein needed for healthy skin known as LEKTI.

The team at University College London are proposing to treat patients with the disease by engineering skin stem cells obtained from patients to carry a functional copy of the *SPINK5* gene – in essence correcting the faulty gene. Corrected cells will then be grown as skin-like sheets and grafted back onto patients. Initial tests so far have shown that this works when even using a small number of gene corrected cells.

Dr Di says: “We’re hoping to show that genetically engineered skin grafts can provide an immediate local protective barrier much like normal skin. The grafts can also act as ‘protein factories’, secreting LEKTI which will then provide wider benefits to the skin, capable of sustaining therapeutic effects for the previously affected areas.”

The idea to grow new skin cells was a result of advancements in the delivery of foreign DNA into stem cells, as well as improvements in the ability to culture skin cells as sheets. A previous study showed that a patient with another severe skin disease, Epidermolysis Bullosa (EB), who also underwent similar skin graft treatment, suffered no known side effects with no blistering, infections, inflammation or adverse immune

response even after a year. Epidermolysis Bullosa (EB) received widespread attention in the documentary 'The Boy Whose Skin Fell Off' which aired in the UK in 2004.

"This study was important as it showed that such a technique – where skin stem cells are corrected - was possible. We believe that the genetically modified skin grafts, even if applied in relatively small patches will act as protein factories and hopefully prove beneficial to sufferers of Netherton's Syndrome across the UK," says Dr Di.

Matthew Patey, Chief Executive of the British Skin Foundation, says: "The implications of what Dr Di and the team are doing are huge. Although these are very early stages of the study, if proven to work for people with Netherton's Syndrome, this form of gene therapy may have potential benefits for skin disease sufferers with other conditions caused by genetic defects."

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The British Skin Foundation (BSF) is a charity committed to raising funds for skin disease research. 100% of the money raised for the charity goes back into funding vital research. Over the last three years alone, the BSF has awarded in excess of £2.2 million to a number of studies that aim to find new treatments and eventually cures for the many skin diseases in the UK.