

## GENE THERAPY CREATES NEW SKIN FOR A DYING BOY

The details of the following medical case were published in the November 2017 journal Nature. It is amazing what Italian doctors did for a dying boy.



Dr. Michele de Luca

Italian doctors treating a critically ill boy with a devastating skin disease used experimental gene therapy to create an entirely new skin for most of his body in a desperate attempt to save his life. The boy had a rare, incurable skin disease caused by genetic mutations. People with the disease lack critical proteins that attach the outer layer of the skin to the inner layer, resulting in

fragile skin with almost constant blisters and open sores.

The German boy, then 7, was hospitalized in June 2015 with blisters on his limbs, back and elsewhere. He quickly lost about 60 percent of the outer layer of his skin and was put into an induced coma to spare him further suffering. The doctors in Germany tried skin grafts from his father and donor skin but all failed.

The boy's parents asked about experimental treatments. Dr. Michele de Luca and his colleagues of the University of Modena, Italy were contacted by the German doctors treating the boy. Dr. de Luca told the family that the boy's precarious state meant that he might not survive the complicated surgeries needed to save him. "We were forced to do something dramatic because this kid was dying," said Dr. Michele de Luca.

Dr. de Luca and his colleagues had previously used gene therapy to produce a small piece of skin in a similar case. In this boy's case to fix his gene mutations, the Italian doctors took a small piece of the boy's skin from an area that was OK. In the lab, they added a normal version of his bad gene to his skin cells. They grew sheets of the boy's skin in much the same way skin grafts are grown for burn victims.

In total, they grew close to 3 square feet of skin. The lab-grown skin was then transplanted onto the boy in 3 operations, ultimately covering 80 percent of his body. Ten days later, the new skin was already beginning to grow, Dr. De Luca said. After eight months, the doctors said that nearly all of the boy's skin had been generated by the modified stem cells.

Two years later, the boy is doing so well that he doesn't need any medication, is back in school and even playing soccer. So far, no problems have detected. Dr. De Luca said the boy will be monitored closely for skin cancer and other potential issues.

The boy and his family later visited Dr. De Luca and the other Italian doctors involved in his treatment. Dr. De Luca said he recalled how the boy spontaneously began taking off his clothes. "The boy was so happy with his new skin that he wanted to show off."

"This takes us a huge step forward," said Dr. Peter Marinkovich of Stanford University of Medicine, who has done related work. He said it was impressive that Dr. De Luca and his colleagues were able to make such large amount of viable skin after correcting the genetic deficit. Dr. Marinkovich said many patients don't survive beyond the age of 2.



### Sakura Blossom (Cherry Tree Blossom)

The snow gently falls down from the blue sky,  
And down, towards south migrating birds go.  
Strongly and harshly does the North Wind blow,  
Causing stones, twigs to willy-nilly fly.  
As I sit here, I watch these scenes and sigh,  
When I see the river no longer flows,  
And to the ground, wilted flowers bow low,  
This makes me ask, why we have winter; why?  
But I too know the answer: to give rest,  
To the birds and the animals and trees.  
To show that pure white things can be awesome.  
But when I touch snow, I feel hope at best:  
If I wait, be patient, then I may seize,  
A glimpse of the first Sakura blossom.

By Francesca Mia Tortoza, 2016

Prepared by Jennifer Murphy

- [https://www.poetrysoup.com/poems/about/sonnet\\_winter\\_poems](https://www.poetrysoup.com/poems/about/sonnet_winter_poems)
- "Gene therapy creates new skin for dying boy" by Marcia Cheng Albuquerque Journal, Thursday, November 9, 2017