Rob Waddell is a very lucky man, although he might not have thought so when his kidneys began to fail ten years ago.

The finance manager from the state of Kentucky in the US suffered from a life-threatening genetic condition called polycystic kidney disease. Although regular dialysis helped him manage his disease, Waddell knew he would eventually need a kidney transplant and face a life-long regimen of immunosuppressant drugs to prevent his body rejecting the new organ.

But Waddell feared side effects of these drugs could potentially leave him more vulnerable to infection and other diseases, including cancer, cardiovascular complications and diabetes.

With three young children and another on the way, Waddell was reluctant to go ahead.

Luck was on his side, however. Not only did he receive a new kidney, but Waddell became an early recipient of a novel stem cell-based therapy being studied by scientists as a possible alternative to immunosuppressant drugs after transplant surgery.

The experimental Facilitating Cell Therapy (FCR001) under evaluation at Novartis involves taking stem cells from the kidney donor and grafting them into the transplant recipient's bone marrow where the white blood cells that fight infections are produced.

If you can train a patient's immune system to tolerate the new organ, then they shouldn't need the medications

Mike Perry, Chief Scientific Officer of Cell & Gene Therapy Unit, Novartis.

This results in a genetically mixed immune system where recipients' own cells and those of the donor are both present. The combination tricks a recipient's immune system into accepting the donated kidney as its own.
“If you can train a patient’s immune system to tolerate the new organ, then they shouldn’t need the medications,” says Mike Perry, Chief Scientific Officer of Cell & Gene Therapy Unit, Novartis.

Although organ rejection was a serious problem in the early days of transplant surgery, immunosuppressant drugs have helped make the procedure far more commonplace. Every year more than 70,000 patients worldwide undergo kidney transplant surgery.

Conceived by American biopharmaceutical company Regenerex, the Facilitating Cell Therapy is being developed under license by Novartis. It is still early in the development process and much work remains to be done, but early research results show promise.

In a clinical trial involving 28 transplant recipients, nineteen subjects have reached two-year follow up and 12 have been successfully taken completely off immunosuppressant drugs for more than 18 months without suffering organ rejection.

Because immunosuppressants work by inhibiting patients' immune systems, they can leave some with an increased susceptibility to infection and other conditions, as well as affecting some internal organs.

“In the long term, not taking immunosuppressants means patients could avoid the frequent re-hospitalisation that often follows transplants,” says Sujata Vaidyanathan, Global Program Head responsible for the collaboration with Regenerex.

Avoiding immunosuppressants could also mean transplanted kidneys last years longer, possibly for life, making an immense difference to the patient’s quality of life.

For Waddell, the seven years since his operation have been busy and he enjoys spending time playing with his four children, trampolining, cycling and skateboarding.

“I have more energy now. I know I’m very fortunate,” says Waddell.

Tags:
- BioMedical Research [3]
- Cell and Gene Therapy [4]
- Emerging Technology [5]
- Medical Innovations [7]

Source URL: https://www.novartis.com/stories/global-impact/next-phase-transplant-medicine

Links