Medical Xpress

Cell therapy, now artisanal and costly, heads for mass production

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Not so long ago, manipulating living cells to serve as therapies was a difficult and mysterious art. Only a few biomedical companies and academic labs could claim proficiency.

But in recent years, there's been an explosion of knowledge about how cells work, how they can become diseased and how they can be cured. Three groundbreaking cell and gene therapy products have been approved since 2017; two for relapsing blood cancers, and one for a genetic disease that leads to blindness.

This transformation attracted nearly 70 presenting companies to La Jolla, Calif., for this year's Cell & Gene Meeting on the Mesa, an annual conference in the field.

These treatments are now costly and performed for a small number of patients. But scaling up this artisanal industry through mass production is expected to reduce costs and bring the benefits to many more people.

To prepare for this wave of cell therapies, the U.S. Food and Drug Administration has been updating its standards for assessing therapies, standards developed for pills and injections of drugs, not of living cells.

"It's the nature of this whole meeting that the process of characterizing something as squishy and variable as human biology, packaging it and heading toward the clinic, is cutting-edge for the FDA," said Taylor Crouch, CEO of San Diego's Organovo.

Organovo made a name for itself by selling "bioprinted" human liver cells for toxicology screening of potential drugs. The San Diego company is preparing to take its technology to patients with end-stage liver disease, Crouch said.

The company collects cells from donor livers, and prints them with a device that arrays the cells into a three-dimensional form, with various cells that support each other, Crouch said.

Transplanted liver cells could supplement function of a diseased liver until a donor liver can be found or perhaps even help the patient's own liver recover, Crouch said.

The company plans to file in 2020 to begin clinical testing, Crouch said.

Another presenting company, Athersys, is in late-stage or Phase 3 clinical testing of its cell therapy for strokes caused by blood clots. The product, called MultiStem, consists of cells taken from a donor's bone marrow, grown in the lab and frozen. When needed, they are thawed and then infused into a patient, said Gil Van Bokkelen, chairman and CEO of the Cleveland-based company.

MultiStem consists of a class of cells that reproduce prolifically in the lab, so the cells from one donor can yield "millions of clinical doses," he said. These cells reduce inflammation, promote regeneration, and are tolerated by the patient's immune system, Van Bokkelen said.

"We can administer them just like Type O blood," he said.

San Diego's ViaCyte is clinically testing a cell therapy for type 1 diabetes. The San Diego company's product replaces insulin-producing pancreatic "beta" cells, which are destroyed in the disease.

ViaCyte turns embryonic stem cells into precursors of these beta cells. These cells are then encapsulated in a device that is implanted into patients. The company is testing two variants of this approach.

Early human testing of one approach, using cells shielded by a semi-permeable membrane, hit a roadblock when the devices caused growth of fibrous tissue. This blocked diffusion of insulin into the patient. Testing has been suspended while ViaCyte reworks the encapsulating material, with help from W.L. Gore & Associates, the makers of Gore-Tex.

That trial is expected to be restarted next year once approval is granted, said Paul Laikind, ViaCyte's CEO.

Meanwhile, ViaCyte has advanced development of a somewhat different device, which allows direct contact between the cells and patient tissue. This risks an immune reaction, so patients receiving the devices must take immune-suppressing drugs.

So far, side effects have been like those expected from taking immune-suppressing drugs, and appear controllable, Laikind said. But it's too early to know if the cells can produce therapeutic quantities of insulin.

Patients and others looking for more information on clinical trials from these companies can search for the name of the company on www.clinicaltrials.gov .