

Could These Tiny Organs Help Scientists Beat Tumors and Save Millions of Lives?

Scientists test hundreds of drugs on organoids—tiny versions of a patient’s tumor grown in a lab.

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Research is underway to test the effectiveness of different chemotherapies, trace the influence of the environment on disease, and compare healthy brain cells to those with disorders. What’s ahead for organoids — regrowing organs?

If you’re a woman newly diagnosed with breast cancer, treatment can feel ... well, scary. Will chemotherapy work? Or will it become a cycle of educated guesswork: trying one drug combination, waiting, scanning? If the tumor doesn’t respond, trying something else. In the meantime, the cancer doesn’t wait.

But in the not-too-distant future, patients may begin with a head start. In this future, doctors grow a tiny version of a patient’s tumor in a lab — a living replica carrying the same mutations and quirks as the original cancer. Before treatment begins, doctors test drugs on the replica, watching how it responds. Decisions begin with evidence tailored to the individual patient.

“If we can predict how a tumor will respond before starting down a therapeutic path, it would save time, money, and a lot of anxiety,” said [Jennifer Rosenbluth](#), MD, PhD, an associate professor of Medicine and the Sulochana Pradhan, MD Distinguished Professor in Breast Cancer.

That’s one of many promises of organoids — tiny, three-dimensional clusters of cells that can be grown from a patient’s own tissue. Given the right conditions, the cells self-organize into “mini organs” that capture some of the complexity of how tumors grow, intestines heal, and brains develop.

What makes human organoids especially powerful is how closely they reflect human biology, often capturing features difficult to reproduce in mice and other animal models. Over the past decade,

advances in stem cell technology have made organoids easier to grow and sustain, shifting the focus from how to make them to how to use them, thanks to funding from the NIH.

Organoids, once seen mainly as research tools, are now emerging as promising platforms for developing drugs and tailoring treatments without relying first on animal testing. At the same time, they are opening a window into disease itself — helping scientists trace how disease risk develops before birth, evolves across a lifetime, and responds to the environment.

Tiny models that predict a particular tumor's resistance

The Dutch scientist Hans Clevers grew the first human organoids in 2009, miniature models of the intestine. He laid the foundation for researchers around the world, including Rosenbluth, to use organoids to study disease biology and test potential treatments.

Seventeen years later, that vision is finally coming into focus.

In Rosenbluth's lab, researchers embed tumor cells donated by patients into a gel that mimics conditions in the body. Bathed in a carefully calibrated nutrient solution, the cells begin to grow and organize. Over days and weeks, they cluster into tiny spheres that reflect the structure and biology of the original tumor.

By the time they are ready to test drugs, each cluster contains thousands of cells — too small to see clearly without a microscope, though the largest appear as faint, translucent clusters in the gel.

These organoids allow Rosenbluth's team to test how a patient's breast tumor responds to chemotherapy, particularly when standard treatments fail. The ultimate goal is to use these results to guide treatment decisions for future patients. The work is closely tied to the [I-SPY clinical trial](#), a UCSF-led study that tests multiple cancer therapies simultaneously.

In the lab, Rosenbluth's team details the organoids' responses to standard chemotherapy and experimental drug combinations, connecting it with clinical data from the patient each tumor comes from — including genetic mutations and response to treatment.

"This connection is critical for taking organoids to the next level, which means being able to use them to identify which therapies may be most effective for a specific patient," she said.

Behind this work is a growing biobank — a frozen library of tumor tissue and organoids derived from hundreds of patients. Each represents a patient's tumor, allowing researchers to study cancer diversity and test multiple treatments in weeks rather than months.

See how disease develops over years or decades

Organoids are also helping scientists understand diseases that develop over years or decades through the ongoing interaction between cells and their environment.

In the intestine, that interaction is constant. The intestinal lining faces daily exposure to food, microbes, and toxins while continually defending and repairing itself. For decades, researchers studied this process using flat, two-dimensional cell cultures that lacked complexity, said [James Bayrer](#), MD, PhD, professor of Pediatric Gastroenterology and Nutrition and vice chair of laboratory research in Pediatrics.

Today, Bayrer grows intestinal organoids from patient biopsies to study inflammatory bowel disease (IBD). His lab's biobank includes samples from pediatric and young adult patients, allowing comparisons across life stages. Pediatric organoids highlight genetic predisposition, while adult organoids reflect years of environmental influence.

His models have revealed a surprising repair mechanism: When chronic inflammation destroys intestinal stem cells, some mature cells can “rewind” to a stem-like state and help rebuild damaged tissue. The discovery points to new ways to restore the gut's natural healing ability.

“What we thought was a one-way street, going from a stem cell to a mature cell, can actually go backwards,” he said.

Can organoids reveal the foundations of psychiatric disorders?

The human brain may be the hardest organ to study. It is wildly complex, and much of its development occurs before birth, when the foundations of disorders like autism and schizophrenia are laid.

[Arnold Kriegstein](#), MD, PhD, has spent decades working to understand those early stages, when brain cells rapidly appear, migrate, form temporary networks, and disappear as the brain organizes itself.

In 2013, a new technology changed everything. Induced pluripotent stem cells — adult cells reprogrammed in the lab into a flexible state that can become almost any cell type — enabled neuroscientists to grow brain organoids and observe early human brain development in the lab.

“This is one of the most exciting times of my career,” said Kriegstein, who has worked with neural stem cells since the 1990s. “We’re able to do things that were unimaginable just five or 10 years ago.”

Kriegstein compares organoids from healthy individuals with those from patients with psychiatric disorders to identify where brain development first diverges. He can then test interventions that

may prevent those early disruptions and, potentially, the psychiatric conditions themselves.

Achieving that goal is still a long way off, and Kriegstein emphasizes that brain organoids are nowhere near as complex as the human brain.

From organoids to organs

At the moment, organoids face two major limitations: They lack a blood supply, and they often grow in random shapes. Once these are overcome, scientists might be able to use organoids to grow replacement tissues and even whole organs.

UCSF researchers are at the forefront of science trying to overcome these limitations.

Vascular neurosurgeon [Ethan Winkler](#), MD, PhD, has taken a big step toward giving organoids a blood supply. By combining cells that form brain tissue with those that form blood vessels, he's created vascularized brain organoids. The result is a more lifelike system, where cells receive oxygen and nutrients and form stronger connections.

To tackle the inconsistent shapes, [Zev Gartner](#), PhD, developed [new materials that help guide organoid growth into more consistent shapes](#), while [Wendell Lim](#), PhD, is engineering cells that communicate and organize in predictable ways — moving the field from growing organoids to designing them.

These mini-organs are still evolving. They won't replace clinical trials or real organs anytime soon. But they are already transforming research — bringing experiments closer to the biology of real patients and hinting at the promise of lab-grown tissues for transplant.

For now, they are small, microscopic, even. But in those tiny clusters of cells, researchers are building a new way of understanding and ultimately treating human disease and injury.

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