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# The Race To Commercialize Functional Precision Medicine And The Policy Battle Shaping It

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Early data from ex vivo drug sensitivity platforms suggest functional precision medicine could transform cancer treatment selection — but regulatory barriers are blocking access for the patients who need it most.

*This approach, called functional precision medicine (FPM), aims to eliminate the guesswork for doctors by directly exposing patient-derived tissue to a panel of possible treatments. (Shutterstock)*

When three-year-old Logan Jenner was diagnosed with acute myeloid leukemia in 2019, it seemed like all of the stars aligned to give him the best possible chance for a cure. He embarked upon a treatment regimen comprising standard chemotherapy and Bayer's targeted therapy Nexavar (sorafenib) to zero in on the FLT3-ITD mutation in his cancer cells. He entered remission in 150 days and, in a fairy-tale ending to his cancer journey, received a hematopoietic stem cell transplant (HSCT) using umbilical cord blood from his newborn sister, Mary Jane, who was a perfect 10-out-of-10 HLA match.

Thirteen months later, Logan relapsed.

Logan is not alone. Among children with AML, 30% to 40% ultimately relapse after their first round of therapy, and there is no universal consensus on a standard of care for subsequent treatment. The five-year survival rate after first recurrence hovers around 20%. Treatment in this setting typically involves chemotherapy and a repeat HSCT. However, due to the rarity of AML in children, little data exists to guide treatment selection, and therapy is often chosen by trial and error.

**Key Takeaways**

- In a prospective clinical trial, 87% of children with relapsed cancers who received treatments guided by a functional precision medicine test have shown improvement, and 55% were alive after one year.
- Diverse patient-derived ex vivo models are being used to develop commercial FPM tests including 2D cell cultures and tumor fragments or slices. These models are not yet standardized.
- FPM testing has potential to identify effective off-label therapies for patients who otherwise lack standard treatment options, but regulatory and policy barriers limit access.

A new option became available in time for Logan. He enrolled in a clinical trial through Florida International University combining genomic profiling with drug sensitivity testing (DST) across a panel of more than 100 FDA-approved drugs in the hope of finding the therapy most likely to return him to remission. Logan's mother, Diana Jenner, was hesitant, at first, not wanting her son to "be a lab rat." But when she learned it only required a few extra tubes of blood taken at the same time as a routine blood draw, and that it could help other children, she gave her consent. "It's very simple," she said. "Let's get a sample, let's take it to the lab, let's test everything under the sun that you possibly could and see what comes back from that.

This approach, called functional precision medicine (FPM), aims to eliminate the guesswork for doctors by directly exposing patient-derived tissue to a panel of possible treatments. The field is taking off with early successes in both solid tumors and hematologic cancers. Research groups around the world are advancing FPM platforms using diverse ex vivo models including 2D cell cultures, tumor organoids, tumor cell fragments, and more, often paired with artificial intelligence-driven analysis. Commercial startups are conducting clinical studies and in some cases have made the technology available as a laboratory-developed test. However, FPM methods are not standardized, and access remains difficult for patients who need it the most – those lacking standard treatment options.

In April 2024, researchers from First Ascent Biomedical, Florida International University, and other institutions published the results from Logan's clinical trial in Nature Medicine. Investigators enrolled 25 pediatric and adolescent patients with recurrent or refractory malignancies between February 2019 and December 2022 who had exhausted standard-of-care treatments to undergo genomic profiling and ex vivo DST using a panel of up to 125 FDA-approved drugs.

The study returned treatment recommendations for 21 of the 25 patients in a median nine days for hematological cancers and 10 days for solid tumors, achieving its main objective. Although patients did not receive treatment as part of the trial, their doctors could choose the FPM-guided therapy. The researchers then compared outcomes for patients on FPM-guided treatments with those on physician's choice of treatment. Seven patients did not go on to receive treatment. Out of 14 patients who did, six had FPM-guided treatments, and eight had a different treatment chosen by their doctor. All six on FPM-guided therapy had stable disease or better. Five out of the six patients (or 83%) achieved an objective response. In comparison, only one of the eight patients on physician's choice had an objective response, and six of those patients progressed.

Logan responded exceptionally well to an FPM-guided combination of chemotherapy and Novartis' FLT3 inhibitor Rydapt (midostaurin), which scored much higher against Logan's cancer cells than the Nexavar and Takeda Pharmaceuticals' Iclusig (ponatinib). The DST data also indicated that Logan would fare best on fludarabine and cytarabine chemotherapy without idarubicin, reducing toxicity for him. Another key finding was that Logan's cancer cells proliferated when exposed to steroids, which were removed from Logan's treatment plan.

Logan entered remission in 30 days, and received a second HSCT from a different donor. According to his mother, Logan will celebrate five years cancer-free in October of this year.

Diana Azzam, lead author on the study, said more than 80 patients are now enrolled in the trial. Investigators are in some cases conducting repeat testing and adjusting treatment based on new results.

"We're analyzing the data as we speak," Azzam said, noting that overall survival within the trial is increasing as time passes and 87% of patients have now shown improvement with FPM-guided therapy, and their one-year survival rate is 55%. Azzam and her collaborators are preparing these results for publication in the near future.

First Ascent is advancing its FPM platform, named xDRIVE, in multiple clinical trials. It also offers access to the technology through a (CLIA)- and College of American Pathologists (CAP)-certified lab in Miami. xDRIVE leverages artificial intelligence to analyze genomic, transcriptomic, and DST data and returns a ranked list of effective treatments for an individual patient. Patients currently pay $8000 out of pocket to access the test, but First Ascent has secured funding to offset some costs.

## COMING OF AGE FOR A NEW TECHNOLOGY

FPM has its roots in chemosensitivity assays used more than a half century ago to probe drug efficacy in cultured mouse cancer cells. However, these assays failed to demonstrate correlation between in vitro sensitivity and clinical outcomes. Translation of those methods to a test that can guide individualized therapy in cancer patients required developing techniques to grow patient-derived cells in vitro that faithfully recapitulate tumor heterogeneity, particularly in solid tumors.

A number of live cell models routinely used in research have shown potential for FPM including two-dimensional cell lines, patient-derived xenografts, and tumor organoids. Tumor organoids attracted early interest for translation as a clinical FPM test. They can be created from multiple sources including cell lines and tissue and have been shown to accurately model patient-specific therapeutic responses while preserving clonal heterogeneity. For example, in a case study reported in Nature Cancer in 2022, researchers from the University

of Utah showed that a patient-derived organoid could identify an FDA-approved drug for a patient with metastatic triple-negative breast cancer, leading to a complete response. In another study published in Cell in 2020, Dutch researchers identified high responsiveness to at least one drug for 22 out of 25 ovarian cancer patients.

However, culturing patient-derived organoids is complex. The process can take weeks to months, impeding commercial development for FPM testing.

"Every large academic center has at least one or two of those initiatives today," said Hinco Gierman, chief scientific officer at Elephas, in reference to FPM programs using patient-derived organoids. "Not a single one of them today is a commercially available test." He added that academic centers will sometimes screen a panel of drugs against patient-derived organoids for a particularly difficult-to-treat patient and present the results to a molecular tumor board.

Elephas is one of a handful of companies pursuing tumor fragments as a model for ex vivo drug sensitivity testing. Its Live Edge automated cutting instrument cuts samples from core needle biopsies while preserving large contiguous areas of the native tumor microenvironment. The resulting tumor fragments, approximately 300 µm thick, are encapsulated in a proprietary hydrogel and exposed to immune checkpoint inhibitors.

In a retrospective study of data from patients with solid tumors from three clinical trials presented at the American Society of Clinical Oncology (ASCO) 2026 annual meeting in June, Elephas' platform, elive, accurately predicted clinical response to immune checkpoint inhibitors in 93% of patients.

"The problem that Elephas is solving is the fact that today there is no good way to know which immunotherapy will work for cancer patients," Gierman said.

Gierman said Elephas has a CLIA-certified lab in Madison, Wisconsin and is launching the technology at other sites across the US.

Unlike First Ascent, Elephas does not currently use genomic screening in its analysis because commonly used biomarkers like PD-L1, microsatellite instability, and tumor mutational burden are not reliable. "Our test at the moment is so accurate that [genomic data] doesn't really add much," he said. "If you just measure the outcome, which is produced by a whole combination of factors, that's all you need to know."

Elephas has closed recruitment for its lung cancer trial (ELEPHAS-01) and basket trial, while it is still enrolling patients in a trial in solid tumors with Mayo Clinic (ELEPHAS-04) and a study of live tumor biopsies (ELEPHAS-05). "The long term goal is absolutely to make this available for any solid tumor where a clinician deems that this test could be useful," Gierman said.

## AN EMPIRIC APPROACH

Another approach to ex vivo FPM testing based on tumor fragments comes from Empiri, which takes thin slices from a core needle biopsy using a new method that the firm claims retains native tumor architecture, stroma, and endogenous immune cells. The platform then uses data from exposure of the slices to a panel of drugs to identify potential best therapies for the patient empirically—hence the company's name Empiri.

Empiri co-founder and CSO Kyuson Yun said Empiri's assay, named E-slice, differs significantly from other tumor slice technologies. While declining to share proprietary details, Yun said, "Basically what we do is take live patient tumor tissues and then we immediately make very thin precision slices from those and we empirically determine on that day, in that patient, which drug would work the best." The tissue can be from a local hospital or shipped overnight on ice. "We have a rigorous quality control step to make sure the slices don't contain necrotic lesions, for example, and then we immediately expose them to drugs." The final readout for the assay is viability.

Yun and colleagues from Empiri, Houston Methodist Research Institute and other institutions presented results from a prospective clinical study predicting response in patients with triple-negative breast cancer at ASCO last month. Patients in the trial received neoadjuvant therapy with Merck's PD-1 inhibitor Keytruda (pembrolizumab) plus chemotherapy comprising paclitaxel and carboplatin at MD Anderson Cancer Center. Yun's group collected samples from 10 patients prior to beginning therapy using the E-slice method and treated them with IgG control, Keytruda, or a combination of Keytruda and chemotherapy followed by doxorubicin and cyclophosphamide. The E-slice assay accurately predicted response in nine of 10 patients with 100% specificity and 88% sensitivity.

Empiri is developing the assay initially as a tool to choose among standard-of-care options for cancer patients in their specific indication, a divergence from First Ascent's strategy of screening a broad panel including potential off-label therapies. The company is working with some concierge medicine providers to provide custom tests using drug panels selected by physicians. Patients pay out of pocket for this service.

"Currently, we're restricted by what is FDA-approved," Yun said. "But there are about 30% of cancer patients who have what's called orphan cancer or they don't have standard-of-care [treatments] or have run through the standard of care. Initially, I wanted to help this population of patients. Then I found out there are all kinds of

regulatory hurdles."

## BREAKING DOWN BARRIERS

First Ascent has also encountered these hurdles in its path to market. "The FDA will only [accept] studies where we've used treatments that are on-label," Azzam said. "That could be okay in an adult cancer like lung and breast, but in children, you have very few drugs that are approved, especially in the exhausted standard-of-care setting."

The company has chosen to tackle the problem head-on. "There's a modernization effort that needs to happen," said Jim Foote, CEO of First Ascent, noting that the federal Right to Try Act, passed in 2017, allows patients with serious or life-threatening conditions to access eligible investigational drugs if they have exhausted approved treatment options and are unable to participate in a clinical trial.

"What Right to Try doesn't do is enable tools and technology such as software, advanced biology, and AI to help give the physician the data they need to make that [treatment] selection," Foote said.

First Ascent has proposed language to update Right to Try including tests like xDRIVE and is seeking sponsorship for it in Congress. "We believe this simple 'modernization' to Right to Try actually reduces risk," Foote said. "The update opens the door for non-invasive biology, technology and AI to provide data to physicians so they can make better decisions about patients in right-to-try situations."

The two Dianas, Azzam and Jenner, met at the annual Live Like Bella Pediatric Cancer Research Symposium in Doral, Florida in 2022. Jenner happened to be sitting in the audience when Azzam presented details of a case that sounded very much like Logan's. At this point, the patients were still anonymized and Azzam didn't know any of their names. At first, Jenner was surprised that there was another patient in the study with AML and a FLT3 mutation. "Then she talked about how steroids grew his cancer, and I was like, oh my god, she's talking about my son," Jenner said. During the break, she approached Azzam and told her son's story. Azzam confirmed that Logan was patient EV013-AML in the study.

"And he was her patient 13 that thrived, and he's here today because of this test," Jenner, who is now head of patient advocacy at First Ascent, said. "There's zero doubt in my mind."

Functional precision medicine tests using some form of ex vivo drug sensitivity assay are already available in academic centers and in commercial laboratories to help doctors choose among approved, standard-of-care treatment options for cancer patients. While no consensus has been reached on the optimal ex vivo model for testing patient-derived tissue, progress has been made in the use of tumor fragments or slices to preserve heterogeneity and immune cell components. The technology has potential to discover effective off-label treatments for patients who lack standard options or have exhausted all approved therapies for their cancers. However, it is difficult for patients to access this broader type of testing without regulatory and policy updates.


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