

Foundation stirs controversy by charging cancer patients \$83,000 for unproven but promising experimental drug

By Jonathan Saltzman Globe Staff, Updated March 4, 2023, 4:07 p.m.



Julia Young, 62, has agreed to pay \$83,000 for an unproven cancer vaccine that is being offered outside a clinical trial by a nonprofit foundation with roots in Boston. She has had ovarian cancer for more than 11 years and began taking the shots in November. JAN STURMANN FOR THE BOSTON GLOBE

It has been more than 11 years since Julia Young was diagnosed with advanced ovarian cancer, and two years since it spread to her lymph system.

By her own account, she has already beaten the odds for how long most women survive the deadly disease.

Still, when doctors told her last year that the cancer was growing despite two operations, radiation therapy, and a fifth regimen of chemotherapy, the retired business-meeting facilitator decided to do something unorthodox: spend \$83,000 out of pocket on an unproven experimental cancer vaccine.

“I’m slowly running out of options,” said Young, 62, who in November began receiving a five-month series of shots at a clinic near her San Francisco home; the vaccine is designed to teach her immune cells to recognize and combat tumors.

She doesn’t know if the injections are working yet, she says, but feels fortunate to be able to afford the treatment, which isn’t covered by health insurance because it hasn’t been approved. And a little guilty. “I’m privileged because I have the money to pay for it,” she said.

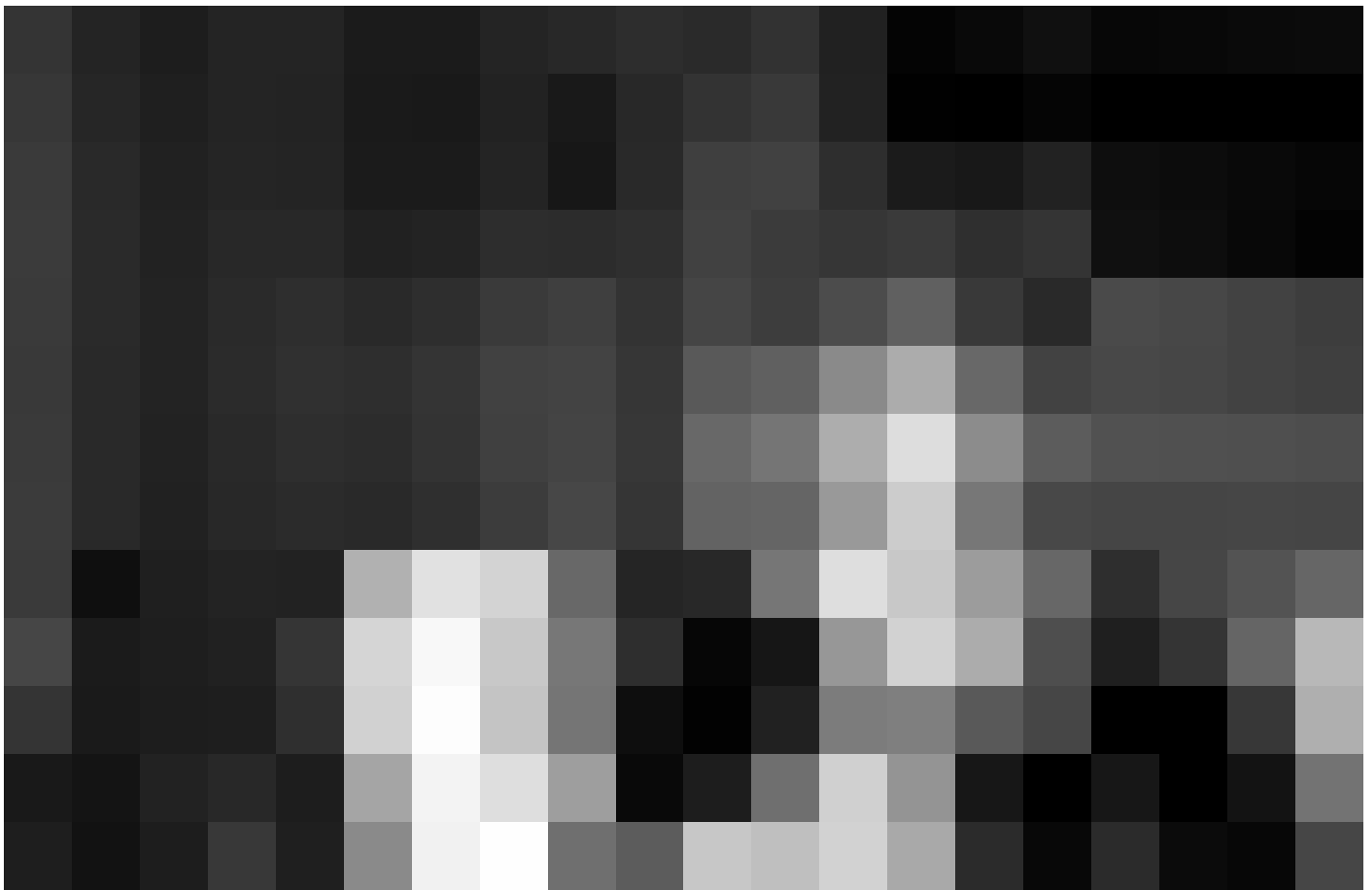
The vaccine was custom-made for her at the direction of a small, recently established nonprofit with roots in Massachusetts called the Jaime Leandro Foundation for Therapeutic Cancer Vaccines. It works with commercial partners and researchers at the Washington University School of Medicine in St. Louis to create a promising but yet-to-be-approved treatment known as a personalized neoantigen synthetic long peptide vaccine.

Therapeutic vaccines represent the next frontier in cancer medicine. Cambridge-based Moderna recently reported encouraging results from a study of another type of personalized cancer vaccine it developed with the pharmaceutical giant Merck; it uses messenger RNA technology to target advanced melanoma. But more work remains before personalized vaccines can be greenlighted.

So far, 26 cancer patients have ordered vaccines from the foundation, according to the nonprofit, also known as JLF, and it’s unclear whether the series of seven shots has been

beneficial. Young is one of six patients currently receiving injections. Another three received them but later died. Four died before shots could be administered, says JLF. Thirteen patients are waiting for their vaccine to be made.

Unlike most people who receive medications yet to be cleared by the Food and Drug Administration, none of these patients is getting the vaccines as part of a clinical trial. Those studies are usually free and rigorously test whether a medicine is safe and effective on volunteers — some of whom receive the drug and some of whom receive a placebo — in an effort to advance science.



Julia Young, 62, at her home in San Francisco. JAN STURMANN FOR THE BOSTON GLOBE

Instead, the 26 patients paid the foundation to create the vaccines and provide them through the FDA's expanded access program. That program, also known as compassionate use, makes experimental drugs available to patients with life-threatening illnesses outside of clinical trials when they have exhausted approved treatments. Typically, those patients pay nothing, in contrast to the foundation initiative.

Although the FDA allows drugmakers to charge patients for unproven medicines provided for compassionate use, it's uncommon and controversial. Four medical ethicists interviewed by the Globe said the foundation and its partners, however well intentioned, could exploit the desperation of cancer patients by charging for a treatment that may not help them.

"It's imprudent and unwise and not the way forward, even for people who are very ill," said Dr. Arthur Caplan, a professor of bioethics at the NYU Langone hospital system in New York who chaired a university panel that studied the FDA's compassionate use program. "People come to this thinking they will be cured."

Jonathan Kimmelman, a biomedical ethics professor at McGill University, was particularly troubled by the consent form that the foundation has patients sign. It says that "taking part in this treatment plan may or may not make your health better." It would be more honest to say that "taking part in this treatment plan is unlikely to make your health better," Kimmelman said, because no personalized neoantigen vaccine has yielded compelling enough results to win FDA approval.

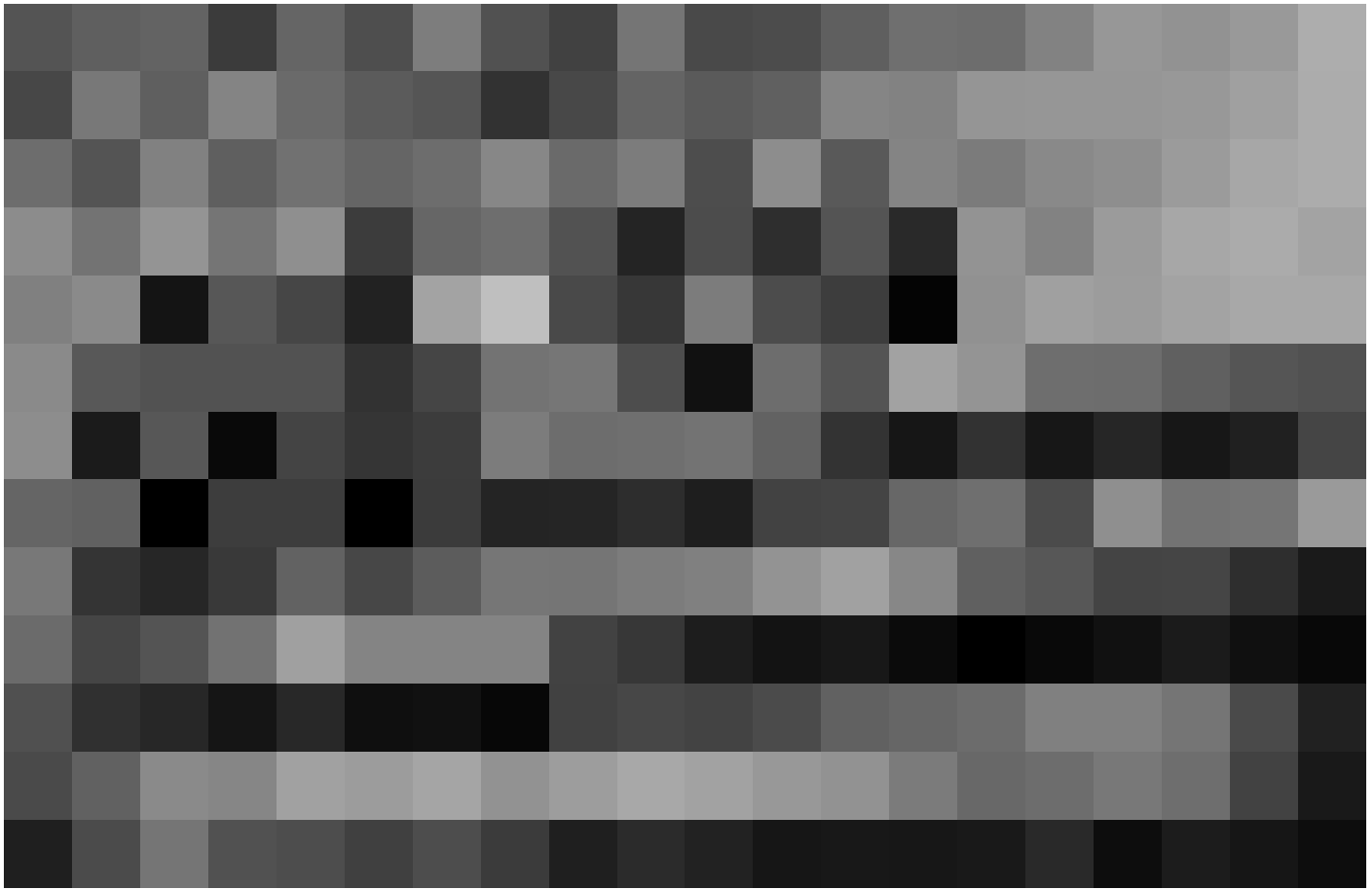
If JLF's vaccines do work, the ethicists said, there's another problem: The program threatens to widen health disparities between haves and have-nots. "It is an interesting paradox," said Marjorie Shaw, an associate professor of law and bioethics at the University of Rochester. "The impacted population is privileged through access to wealth and also vulnerable because of their disease state."

Leaders of the foundation, based in Chapel Hill, N.C., and established in 2020, acknowledged that the \$83,000 charge might raise eyebrows. But they said the sum covers only the direct costs of designing and manufacturing the vaccines — which are tailored to the unique genetic makeup of each patient's tumor — and that the FDA has approved the breakdown of expenses. It takes at least four months to create the vaccines, according to JLF.

Foundation officials said the nonprofit is filling a void for patients who have run out of standard cancer treatments and can't find a clinical trial to participate in. Unfortunately, they added, the foundation doesn't have money to pay for the vaccine and offer the treatment free. The nonprofit has a modest budget and ended 2021 with an \$80,550 deficit, according to a federal tax filing.

"There isn't a member of the board of JLF who wouldn't rather be able to do this without charging people," said Andrew Jacobs, who sits on the boards of the foundation and NorRD Bio, a small Cambridge bioinformatics firm that helps make the vaccines. "It really was a choice of this or nothing."

William Hoos, a self-described cancer crusader, venture capitalist, and president of JLF, said the foundation is providing a potentially beneficial treatment to patients whose cancer started in the breast, ovaries, pancreas, brain, and other organs. Although the treatment is costly and unproven, he said, other people with cancer are traveling to Germany and Mexico to buy experimental vaccines created and distributed without FDA oversight.



A sign for the Food and Drug Administration is displayed outside its offices in Silver Spring, Md., on Dec. 10, 2020. MANUEL BALCE CENETA/ASSOCIATED PRESS

“If you’re going to spend eighty or a hundred thousand dollars on a vaccine, it’s silly to spend thirty of that flying back and forth to Europe,” he said.

A spokeswoman for the FDA said federal law prohibits regulators from commenting on pending requests to make experimental drugs available, inside or outside a clinical trial.

As leaders of the foundation tell it, the program was the brainchild of Stephen Aldrich, a Harvard-educated entrepreneur who spent much of his life in Massachusetts. In 2017, he was diagnosed with advanced cancer of the esophagus and told he probably had only months to live.

Determined to prove his doctors wrong, Aldrich got his tumor genetically sequenced and arranged for a German biotech, CeGaT, to design an experimental neoantigen vaccine that he received in 2019, according to Jacobs and an account Aldrich gave to an online

cancer publication. Neoantigen vaccines are designed to target certain proteins called neoantigens on tumor cells.

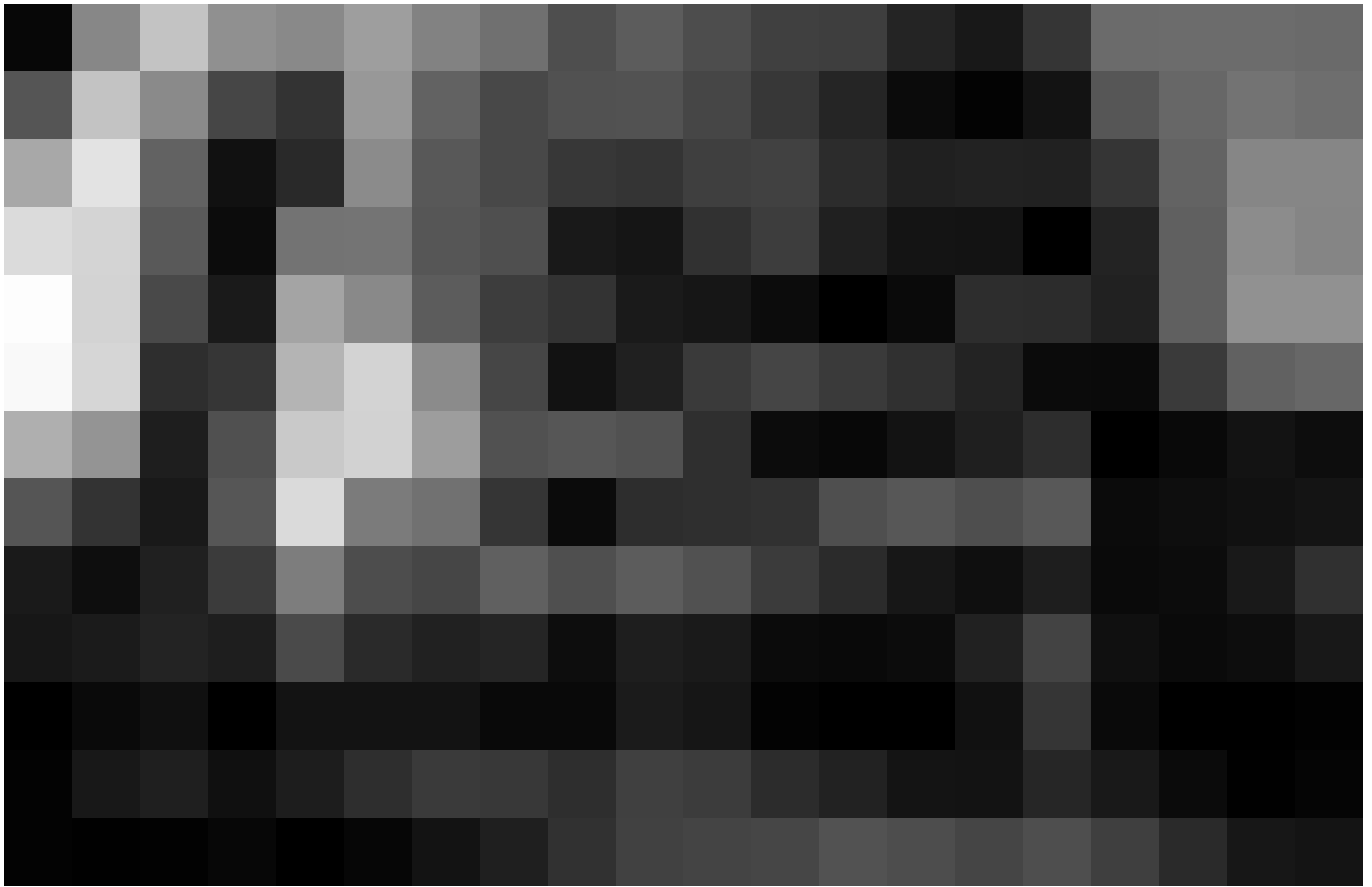
Before he died in 2020, Aldrich concluded that other patients might benefit from such vaccines and started the firm now called NoRD Bio. That business is one of three that help to design and manufacture vaccines for JLF, along with Washington University's medical school.

The foundation coordinates the efforts and files paperwork for each patient with the FDA, which must approve applications to distribute experimental medicines for compassionate use. (The agency approved 1,001 such requests – called individual non-emergency applications – nationwide in the fiscal year that ended on June 30, 2021, according to the most recent federal data.)

The Jaime Leandro Foundation was named for a Framingham mother of three and a friend of Aldrich. She had advanced pancreatic cancer and died at age 41 in 2020, three months before he did.

The class of neoantigen vaccines provided by JLF have been studied by academic labs and commercial drugmakers for more than a decade and yielded encouraging results in recent clinical trials, said Dr. Catherine J. Wu, a medical oncologist at the Dana-Farber Cancer Institute and an expert in the field.

A small study led by Wu found that eight patients with advanced melanoma who received this type of cancer vaccine were alive almost four years after treatment, including six without evidence of the disease, according to an influential 2021 paper published in the journal Nature.



The exterior of the Dana-Farber Cancer Institute in Boston. CRAIG F. WALKER/GLOBE STAFF

“The biology is pretty solid,” Wu said in a Globe interview, although she estimated that the treatment is still one to five years from FDA approval.

Unlike vaccines that the public is familiar with — mass-produced shots that aim to prevent people from catching infectious diseases like COVID-19 and the flu — neoantigen vaccines are personalized and meant to treat cancer by activating the immune system to control tumors.

Wu said she was unfamiliar with the foundation but knows and admires Dr. William Gillanders, a surgery professor at Washington University who helps make vaccines for JLF and serves on its leadership team.

Gillanders said he has been working on experimental neoantigen vaccines for a decade and tested them in multiple clinical trials, the FDA’s preferred method to give patients access to unapproved drugs. The problem, he said, is that many patients can’t get into a

trial. Some have rare forms of cancer for which pharmaceutical firms haven't developed a therapeutic vaccine. Other patients are excluded from trials because their disease is in remission or because they are on FDA-approved cancer drugs that could make it hard to interpret the results of vaccine studies.

"If you're a patient and you have cancer and you're not eligible to be in a trial, then it's extremely frustrating," said Gillanders. "It's kind of frustrating for both sides. We have these trials, people are contacting us all the time asking if they can participate, and a lot of times we have to tell them no."

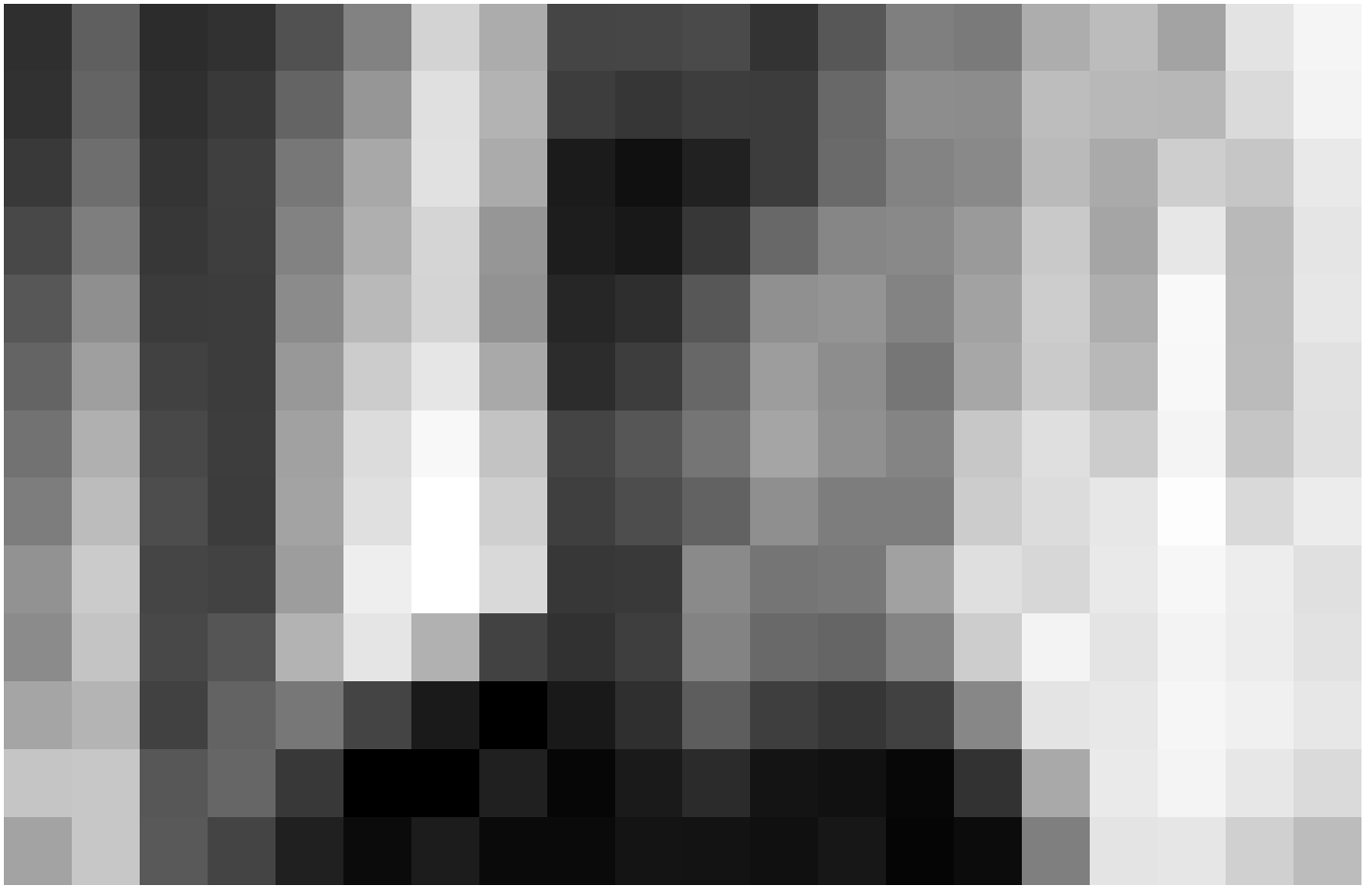
It's too soon to say whether the vaccines that Gillanders helps design for JLF fight off cancer, he said, but they appear to be safe and have generated encouraging immune responses in tests of blood drawn from patients.

The \$83,000 covers the expenses of NoRD Bio, Washington University, and two other collaborators, CSBio and xCures, both based in the San Francisco Bay area, according to JLF. It's up to patients to find and pay for a doctor willing to administer the shots, the foundation says.

Washington University said it provides its services at cost and that Gillanders and another researcher there serve on JLF's board without compensation. The school declined to say why it decided to participate in the project or to discuss potential liability if a patient alleges harm from injections.

Recipients of the vaccine include Mutsuyo Cox, a San Francisco mother of three who had late-stage pancreatic cancer. She started receiving shots in September 2021 after undergoing surgery, radiation therapy, and chemotherapy, according to her husband, Brad Cox. Despite the experimental treatment, she died last April at age 69. Her husband said he had no regrets.

"We knew going in that this was a long shot and there were no guarantees," he said. "JLF never gave any false hope."



University of Richmond visiting professor Colin Kielty will be taking an \$83,000 cancer vaccine outside of clinical trials in hopes it will cure a rare form of melanoma found in his eye. JAY PAUL FOR THE BOSTON GLOBE

Another who ordered a vaccine is Colin Kielty, a 37-year-old visiting professor of political theory at the University of Richmond. In 2021 he was diagnosed with uveal melanoma, a rare and aggressive ocular cancer and had to have his left eye surgically removed at Duke Eye Center in North Carolina.

Although Kielty has no evidence of cancer now, uveal melanoma has a high chance of spreading to other parts of the body, where it is invariably fatal, he said. He heard about JLF last year from another patient diagnosed with the same form of cancer, did some research on Gillanders, and decided to pay for a vaccine.

A doctor at Duke was skeptical and would not administer the shots, Kielty said. Kielty plans to travel soon to San Francisco to Quest Clinical Research, the same clinic where Young, the retired businesswoman with ovarian cancer, gets her shots.

“I owe it to myself and the people I love and the people who love me to do what I can,” he said.

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