Some patients with a rare disease face hurdles getting the only treatment

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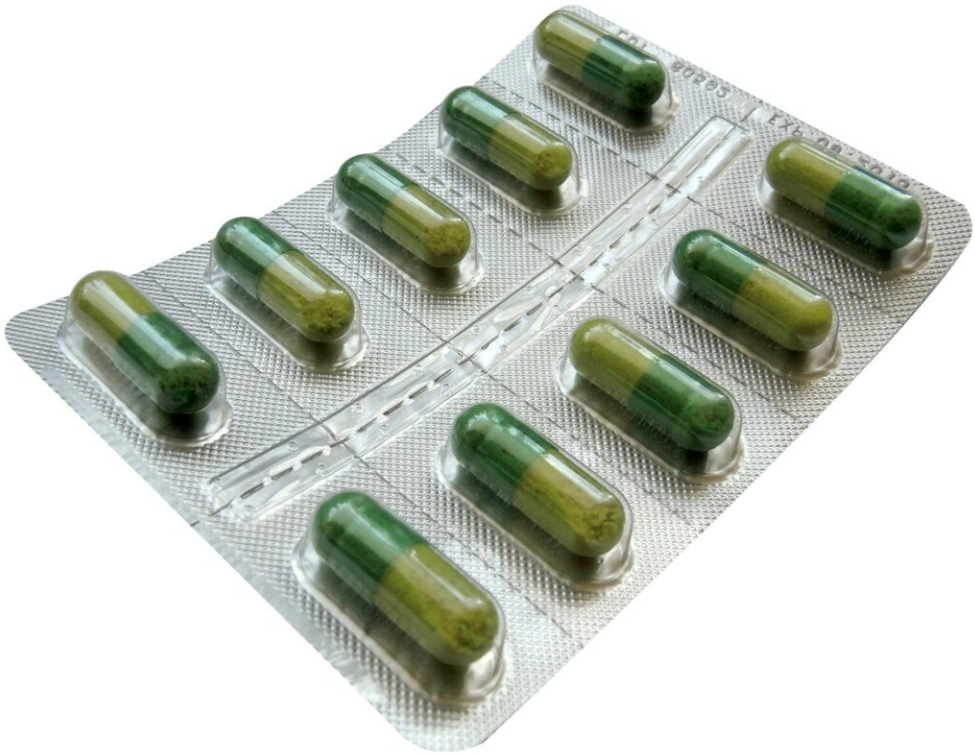

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Victor A. Mejias had been taking a drug to treat a rare skin disease for about two years when its manufacturer effectively cut off his supply.

Mejias was switching dermatologists and needed to find someone approved to administer the treatment by Clinuvel Pharmaceuticals Ltd, which makes the drug. But he says the company wouldn't help him find a new doctor. Mejias was enrolled in a study for another treatment and the drugmaker told him it would give him the name of a center only after he was done with the trial.

"I was so flabbergasted," Mejias said. "This deep depression sets in. What am I going to do?"

Clinuvel's drug, known as Scenesse, is the only US-approved drug for erythropoietic protoporphyria, or EPP, a genetic disorder that causes intense pain when sunlight hits a person's skin. It's been on the market since 2020.

The Melbourne-based company is an outlier in the pharmaceutical industry in that it keeps tight control over who can administer its drug. It doesn't publish a list of the doctors it's trained and approved and declined to give one to Bloomberg.

It also discourages patients like Mejias from participating in trials of other treatments, and has without warning barred a hospital from administering Scenesse.

Clinuvel's business practices are highly unusual. In the American heath care system, drugmakers generally don't get to choose which doctors can provide their medicines.

"Pharmaceutical companies don't practice medicine," said Ryan Nash, the director of the bioethics center at the Ohio State University. "It would be inappropriate if they tried to move into the space of taking away a clinician's ability to make clinical judgment."

Steven Joffe, chair of medical ethics and health policy at the University of Pennsylvania's medical school, said it could be an intrusion into the doctor-patient relationship. "When my doctor and me go into the clinic room together and we close the door, a company's commercial interests don't belong in that room with us," he said.

Mejias was participating in a trial of cimetidine, which has been sold as the cheap over-the-counter acid reflux medication Tagamet for decades. The sticker price of Scenesse, administered as an implant, is about $57,000, according to the consultancy 3 Axis Advisors. Depending on need, patients can get a new implant every two months.

Scenesse is Clinuvel's only approved drug. Overall the business had A$65.7 million in revenue in fiscal year 2022, which ended in June, or about $45 million at the time.

"This is perhaps an unorthodox approach in the industry, but we strongly believe that you need to have a high level of trust and confidence in the professionals with whom you work," Lachlan Hay, director of global operations for Clinuvel Group, said in April in a video posted on the company's LinkedIn and Facebook pages.

In March, the company abruptly cut off Massachusetts General Hospital, the Harvard-affiliated institution where Mejias was doing his cimetidine trial, according to patients and a doctor at that hospital.

Three patients said they received calls from Clinuvel representatives informing them of a "conflict of interest" at Mass General, and were told they couldn't get their treatment at that hospital anymore. Amy Dickey, co-director of the Mass General center that cares for patients with this condition, denied any such conflicts, and a hospital spokesperson said they didn't receive an explanation from the company. Clinuvel said last year that it didn't want patients taking Scenesse to also be in the

cimetidine trial, Dickey said.

Clinuvel didn't comment when asked about this.

Discouraging patients from taking multiple medicines is not normally how American health care works. Because people often take several drugs for the same illness, drugmakers are required to include information on the label about safety concerns related to other medications.

But Scenesse's label doesn't mention potential dangers from the clinical trial drug cimetidine, which has been available for decades without a prescription. Any patient taking Scenesse could have used cimetidine without Clinuvel ever finding out, Dickey said. She said patients have taken both drugs with no ill effects.

Clinuvel didn't respond to a question about what the risks are of taking Scenesse with the antacid in the trial. On its website, it says Scenesse is "not suitable for patients involved in clinical trials for other EPP treatments." Clinuvel declined to explain what this means.

"The company should be clear about the scientific basis of its concern," said Mark Aulisio, chair of bioethics at Case Western Reserve University's medical school. "An unwillingness to provide a rationale is a bit of a red flag. It makes you worry that it's more about competition."

Clinuvel didn't respond to a request for comment about this characterization.

Dickey said she has been unable to order Scenesse for her patients since March. Patients in Boston can get the drug at a different hospital in the city that isn't involved in the antacid trial.

EPP affects thousands of people in the US, forcing many to avoid or limit time in the sun without covering their skin. It turns basics of everyday life—grocery shopping, taking out the trash, picnics, watching the kids play sports—into painful challenges.

Scenesse stimulates cells that make melanin, a pigment that protects skin from light.

Doctors who've treated the condition for years sometimes can't get the drug. Manish Thapar at Einstein Health Network in Philadelphia has been treating porphyria for over 15 years but couldn't get approved to prescribe Scenesse, so one of his patients had to go to Virginia.

"Traveling 100, 200 miles just to get a medication?" Thapar said. "I always thought, how are they getting away with it?"

Mejias, who lives in a Chicago suburb, was finally able to get Scenesse again. After Clinuvel denied him the name of a new doctor to provide it, he pointed out that he wasn't actually taking the antacid—he was at a stage of the trial where he wasn't receiving any pills. At that point, he says, Clinuvel sent him the name of a doctor in Wisconsin.