

Brazilian law grants patients right to use untested cancer ‘drug’

But researchers warn that there is no real evidence that it actually works.

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For years, a chemistry lab at the University of São Paulo supplied cancer patients with synthetic phosphoethanolamine.

Brazilian President Dilma Rousseff has sidestepped the country's drug regulators, signing into law a bill that guarantees people with cancer access to an unapproved 'miracle drug' that has never been tested in clinical trials.

The law went into effect on 14 April and came just weeks after Brazil's science ministry reported that the compound, called synthetic phosphoethanolamine, had failed to fight cancer in its laboratory tests. "It's very sad," says Paulo Hoff, an oncologist at the University of São Paulo. "It undermines the regulatory agency and it legalized something which at this point we don't know if it helps, or it has even some unknown side effects.

Rousseff was under significant political pressure to wave the bill through: she is facing possible impeachment for financial impropriety, and lawmakers and their constituents have been [clamouring for access to the compound](#). The law allows anyone with a medical report that certifies their cancer diagnosis to acquire the drug, and does not require a prescription.

Limited access

For now, patients may have difficulty getting their hands on the pills, says Jailson de Andrade, secretary for research-and-development policy at Brazil's science and technology ministry. The law also demands that the compound be made at a facility that is approved for making human drugs. But no such facility is currently manufacturing phosphoethanolamine in Brazil, de Andrade says.

Even so, Hoff says that patients regularly arrive at his hospital saying that they have taken the drug, even though he is still waiting for a pharmaceutical company to produce just enough of the compound to treat the ten patients he wants to enrol in an initial clinical trial. "I don't know where they are getting it, but the reality is they are," he says.

For years, patients got the compound from a chemistry lab at the University of São Paulo's campus in São Carlos. When the university attempted to halt distribution, thousands of patients sued. In the past, some judges have ordered the university to give patients phosphoethanolamine made in the chemistry laboratory, but it is not authorized to produce medicines.

In response to the debacle, the science and technology ministry committed about 10 million reais (US\$2.9 million) to study the compound. Early results from the study show that it is not toxic to cells grown in culture — but it is equally harmless to cancer cells. Animal studies are ongoing, says de Andrade.

Pushing on

Hoff hopes that his trial, which is sponsored by the state of São Paulo, will get under way in a few weeks. He and his colleagues are not waiting on the animal studies because the compound has already been taken by thousands of people, he says, with no obvious signs of toxicity. His team plans to first test whether the dose that patients are currently taking is safe in ten people. After that, the trial will expand and will test whether the drug works against cancer.

He anticipates results in about six months, but it is likely that some patients will refuse to accept any data that question the effectiveness of phosphoethanolamine. Hoff says that some of his patients who took the compound had a hard time believing it when their cancer progressed. “A patient told me, ‘No, there is a mistake. I want to keep taking this,’” he says. “It has become a matter of faith.”

Meanwhile, de Andrade is worried about reports of patients — even those with early, potentially treatable disease — who decline standard treatment with chemotherapy or surgery in favour of taking the untested compound. “We are urging everyone: do not stop the formal treatment,” he says. “Do not stop.”

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