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The Terminally Ill Need More Than the 'Right to Try'

By Paul J. Marangos

After decades of medical research, why is chemotherapy still a mainstay for cancer patients? Why do many Alzheimer's patients still slide inexorably into helplessness? How did developing a new drug become a multibillion-dollar venture that can take a decade or more?

Government deserves a share of the blame. Regulators, including at the Food and Drug Administration, have shackled every aspect of drug development under the guise of doing no harm. But how exactly does it "harm" patients facing certain death to provide them with an experimental treatment? Congress took a small step in the right direction this May by passing a Right to Try law, which theoretically grants terminal patients the right to access investigational drugs that haven't shown clinical efficacy.

But Right to Try legislation gives only crumbs to these patients. Drug companies will not want to take on the liability of providing unapproved treatments. One wonders if Congress simply thought passing this bill would look good politically as a way of demonstrating concern for terminal patients.

I spent 13 years at the National Institutes of Health and co-founded five biotech companies in the ensuing 30 years, all focused on developing cures for terminal diseases. None of these prospects ever got to market, even though the early clinical trials were all positive. The path to commercialization was too long and expensive, with countless regulatory and procedural hurdles.

The most profound of these is the FDA's prolonged regulatory process. Phase III clinical trials, which involve many hundreds and sometimes thousands of patients, are by far the most costly and time-consuming requirement for FDA approval.

Another challenge are the strict stipulations for obtaining patents, which require demonstrating that the treatment works in the relevant laboratory experimental models and that it is not obvious to those in the industry. Getting patents can take five years or more, representing a substantial risk for the sponsoring company. Further, much existing scientific research is unpatentable. Very often biochemical data is published first, and then after a period it falls into the public domain.

Two hundred cancer drugs are in Phase III trials. Congress can put them into patients' hands today.

If Congress truly wants to help patients by passing new legislation—let's call it the Terminal Disease Act—it would include the following reforms:

- Reduce the FDA approval requirements so that treatments for terminal diseases no longer have to go through Phase III clinical trials. Instead, one Phase II clinical trial of at least 100 patients would suffice. This would dramatically reduce the time and cost of getting new treatments to patients.
- Make this change retroactive, so that all treatments currently in Phase III would be immediately approved. Cancer and Alzheimer's patients would suddenly gain access to scores of new drugs. According to the trade group PhRMA, there are more than 200 Phase III trials under way for cancer drugs. An academic survey of Alzheimer's drugs shows 26 currently in Phase III. All of these already have shown promise in Phase II.
- Mandate a 10-year period of marketing exclusivity on treatments for terminal diseases. This would obviate the need for patents and make a huge amount of unpatentable research available for commercialization. The model is the Orphan Drug Act of 1983, which granted exclusivity to treatments for rare diseases and gave rise to many new drugs.

This kind of legislation would offer credible short-term help to cancer and Alzheimer's patients. It would provide access to many new clinically tested treatments while making pharmaceutical companies likelier to provide them, since an FDA-approved drug is less of a liability risk. A faster development path and an exclusivity provision would give drug companies an incentive to focus on terminal diseases. Innovative startups would multiply to explore treatment ideas off the beaten path, as happened in response to the Orphan Drug Act.

When thousands march on Washington to push an issue, they can get Congress's attention. Lawmakers shouldn't need a protest on the National Mall to rouse themselves to act on behalf of cancer and Alzheimer's patients, but that may be what it will take. Millions of terminal patients are awaiting the inevitable without even the hope of a credible experimental treatment. A Right to Try law is a small victory, but it means little without broader reforms.

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