



## GUEST EDITOR

# Commentary: A Good Law is Always at Risk

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There can be no doubt whatsoever that the *Orphan Drug Act* has been a success. It has:

- Stimulated drug companies and researchers to discover and develop a significant number of valuable treatments for those with rare diseases,
- Led the FDA and NIH to a new understanding of the disease process and the importance of giving priority to life-threatening and severely debilitating diseases, regardless of population size, and
- Provided hope and a sense of community to millions of Americans with the double misfortune of being seriously ill and having a disease too small to have a support network.

Among those of us involved in the 1982 negotiations that led to the law's signing, our hopes for the Act were every bit this great, but our expectations were much lower. We thought we were opening a very small door for rare disease research, one that would stimulate a modest number of new orphan drugs by allowing a small profit through marketing exclusivity. The drugs produced were still likely to be the altruistic acts of large drug companies that could afford to carry a needed, but financially limited product.

To ensure this focused result, U.S. House and Senate tax counsels argued at length over how to narrow the definitions and lessen the potential for windfalls. Frustrated House and Senate health staffers (including myself) kept reminding tax counsel of the important public purposes we were trying to achieve. They were not impressed and we were not treated very sympathetically.

This is significant because the positive accomplishments of the Act have come about largely through means we never envisioned, but the tax counsels feared. More than anything, we underestimated the importance of substantial profit potential in stimulating valuable research and the ingenuity of individuals and companies in the highly

competitive pharmaceutical and biotechnology industries. An entire dissertation could be written (perhaps it has) on the ways that the narrowly drawn provisions of the Act have been used to produce a profitable drug for a rare disease.

I have no regrets. The Act we intended, especially after the tax counsels finished modifying it, would certainly have been much less successful than what ultimately occurred. And while I have occasionally wondered where incentives end and windfalls begin, I believe that prices paid and profits made from orphan drugs have served the public good by stimulating more interest and more research. This was even true for the ugly battle over which companies would find their pre-enactment, profit-motivated drug development efforts retroactively rewarded with marketing exclusivity. Whether deserved or not, this battle set the tone for how companies could “do good and do well” under the *Orphan Drug Act*.

This “do good and do well” philosophy was reinforced by two well-meaning and sensible amendments that were adopted early on: a 200,000 population limit was created to replace the initial unworkable, profit-based standard; inclusion of products that could also be patented. These important changes made it easier for the orphan drug process to be used to speed approvals for a broad array of therapeutic agents with clear moneymaking potential.

Later, we would see questions about the appropriateness of “salami-slicing” drug indications to steer new therapies toward orphan status and benefits. While I do not recollect any discussions regarding the inclusion of more familiar and widespread diseases like cancer under the Act, the “slicing” process has improved cancer therapies and yielded benefits for less common types of cancer.

The accomplishments of the *Orphan Drug Act* shine so brightly today that there is a look of great inevitability about the 1982 negotiations that led to the Act. Yet, it was hard fought then. An honest appraisal of the history of the Act, played out in the real world over the last 15 years, provides many reasons why the *Orphan Drug Act* might not become law today if someone proposed it anew. I doubt the tax counsels would be satisfied with the results.

So, as we celebrate the Orphan Drug Act’s anniversary, it is important to remember how fragile the compromises were that made the Act possible. This vulnerability is re-exposed every time a government official, a researcher or a company forgets that the underlying public policy of the Act is to help individuals with rare diseases (and little hope of rescue) through market-driven investment in research and development.

We should recognize that this “good” law, which has made such a critical difference for so many sufferers, is largely successful because it induces action through the lure of a profitable government monopoly in a specific product indication. Within fairly broad boundaries, the Act’s imperfections should be ignored or quietly dealt with, so that there is no opportunity to raise anew the question: where is the line between stimulating

critically important research and providing unjust enrichment? Those of us who are supporters of the *Orphan Drug Act* know this law is constantly at risk.

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