Should the FDA provide "fast track" approval for curing edge drugs and devices that may help terminal patients?

Citing concerns that the current FDA approval processes may be too slow to help dying patients, " Representative Diane Watson (D-CA) introduced H.R. 4732, the Compassionate Access Act of 2010, recently The bill would amend the federal Food Drug and Cosmetic Act ("FDCA") to create a new expedited review system for patients in need of compassionate access to tests that have not otherwise completed the FDA review process.

The proposed bill amends FDCA, Section 561, to create new subsection (d) – "Compassionate Investigational Access." Here, if a sponsor of an application wanting to provide widespread access to an investigational drug, biological product, or device for eligible patients, Secretary shall permit such investigational drug, biological product, or device, to be made available for expanded access under a treatment investigational new drug application or treatment investigational device exemption if the Secretary determines that [certain] requirements . . . are met with respect to Compassionate Investigational Access.

The requirements to be presented for approval are:

- Data and information from completed Phase I clinical investigations and any other nonclinical or clinical investigations;

- Preliminary evidence (that may be based on uncontrolled data and on a small number of patients or a subset of a patient population) that the product may be effective in humans against a serious or life-threatening condition or disease; and

- Evidence that the product is safe at the dose and duration proposed consistent with the level of information needed to initiate a Phase II clinical trial.

- A sponsor statement that it is actively pursuing marketing approval with due diligence.

In making an approval decision, FDA must "consider whether the totality of the information available to the Secretary regarding the safety and effectiveness of an investigational drug, biological product, or device, as compared to the risk of morbidity or death from a condition or disease, indicates that a patient (who may be representative of a small patient subpopulation) may obtain more benefit than risk if treated with the drug, biological product, or device." And "[i]f the potential risk to a patient of the condition or disease outweighs the potential risk of the product, and the product may possibly provide benefit to the patient, the Secretary shall provide Compassionate Investigational Access approval of the application. The FDA could also refer the application to a new "Accelerated Approval Advisory Committee" for further review and recommendation.

The bill also creates a new section to the FDCA, Section 561B – "Expanded Access to Investigational Drugs and Devices" – requiring the FDA to "establish a new program to expand access to investigational treatments for individuals with serious or life threatening conditions and diseases." The bill would amend the FDCA to provide a new section 568 – "Policies Related to Study Evaluation Information" – requiring FDA to "give consideration to clinical judgment and risks to the patient from the disease or condition involved in the evaluation of the safety and effectiveness of drugs, biological products, and devices that treat serious or life-threatening diseases or conditions" (i.e., non-statistical measures).

If this bill were to pass it would provide an additional fast track approval channel for new medical devices that are cutting edge and have at least some limited evidence of efficacy and safety for use by patients who are in serious danger of dying in the near term and who might benefit from the use of the product. If the product is successful for the very sick, the performance data should help speed up the FDA approval for the approved product generally. This kind of process is very appealing to those of us who believe that terminal patients should have the right to access drugs, treatments and devices that hold some potential for helping them without the exhaustive process of FDA approval. The likelihood of this bill passing is problematic.

While, hope can be a formidable force for survival. Fast track approval bills have been tried before, I think most recently by Sen. Brownback of Kansas who was himself a cancer victim. The major impediments seem to be that it will cost money to set up an alternative program for approval, it might actually slow down the regular approval route for other drugs and products because of the necessity of diverting assets and efforts to meet emergency situations, bureaucratic reluctance to make decisions, particularly quick ones, and paternalistic fear of patients being taken advantage of or abused in their vulnerable condition. Another related issue is that the devices thus approved would probably not be covered under many insurance policies which would exclude them as being "experimental."