BIOTECHNOLOGY PATENT POLICY

A Consumer's Perspective and Recommendations

Testimony before the Commissioners of the U.S. Patent & Trademark Office

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By way of introduction, I am a kidney cancer patient. Chemotherapy and radiation are not effective in treating kidney cancer. Biological response modifiers created through biotechnology are helpful, but these agents are far from perfect and most patients are not cured despite rigorous therapy.

I am also President and Chief Executive Officer of the National Kidney Cancer Association, a non-profit charity which provides information to patients and physicians, sponsors biomedical research, and acts as an advocate on behalf of the nation's 75,000 kidney cancer patients.

I hold a Ph.D. in Management from the J. L. Kellogg Graduate School of Management at Northwestern. I have also worked as a new product consultant in the Advanced Methods Group of N.W. Ayer, a major advertising agency. In addition, I have started five high tech computer-related companies, including one that has been publishing economic information on research and development expenditures for 15 years.

I have no financial interest in any drug, biotech or health care company. However, I am a kidney cancer patient whose life depends upon private industry efforts to find a cure for my disease.

General Perspective

Patent policy in biotechnology is extremely relevant to the well-being of millions of Americans who suffer from cancer, AIDS, Alzheimers, and other diseases for which there are no effective treatments. Patent policy can speed scientific progress or retard it, accelerate products to patients or delay cures. Therefore, as health care consumers, patients cannot be indifferent to the work of the Patent and Trademark Office.

If patent policy creates high hurdles, companies will be granted few patents or have to expend extraordinary resources to get a patent. If too few patents are granted, incentives for invention will be diminished and the public will get fewer medical advances. Similarly, if tremendous resources are consumed obtaining a few patents, the public will get fewer medical advances as dollars are shifted from laboratories to legal offices.

Similarly, a patent policy which is too lax would generate too many patents. These patents would represent a cheap currency. It would create intellectual assets devoid of real economic value with limited protections. It would also water down the incentives for legitimate discovery. The public would enjoy fewer advances.

When patent policy is either too restrictive or too lax, corporate management and outside investors would be less willing to commit capital to research intensive ventures. No manger or investor wants capital consumed by an overly complicated patent process that adds little value to products delivered to the public. No manger or investor wants capital committed to creating intellectual property which has no value and limited protection even though it is patented.

From this perspective, the Patent & Trademark Office and the patent process itself should add economic value to inventions. An optimal patent policy should maximize this value. The public will be served because maximized value will stimulate investment and the development of more life saving inventions.

Making Life Saving Inventions Special

In addition to maximizing value, the Patent & Trademark Office should speed the processing of patent applications so life saving advances reach the public more quickly. As I understand current operations of PTO, patent applications are supposed to be processed within eighteen months. However, this goal is not always achieved.

The public has a unique and special interest in life saving inventions in contrast to inventions which are primarily commercial. Therefore, as a matter of public policy, the Patent & Trademark Office should automatically make "Special" all patent applications for inventions which diagnose and treat life threatening illnesses.

There is significant precedent for accelerating the processing of important patent applications. During the energy crisis of the 1970's, the PTO embraced a policy of making "Special" all patent applications for energy conservation inventions. Public health and the lives of the nation's cancer, Alzheimers, and AIDS patients are no less important than energy conservation.

Many life saving inventions also reduce the cost of health care. Too often, I see cancer patients go from therapy to therapy in search of a drug which will stop their disease. Great sums of money are expended on treatment after treatment which does not work. Accelerating the patent process for life saving inventions will help control health care costs by bringing new, more effective treatments to market faster.

To implement the policy which I have recommended, the Patent & Trademark Office should expand its core of biotechnology patent examiners through new hiring and retention of its existing staff. Congress should appropriate the funds to support the needed staffing.

The eighteen month goal should be a "hard target" for life saving inventions both on the part of the PTO and the applicant. Additional computerized systems and other resources may be needed to expedite and support the processing of patents. Investment in PTO infrastructure is probably money well spent and Congress should be urged to make the required investment.

Practical Utility and Clinical Trials

As we all know, Practical Utility is an essential criteria in patent decision making. While human clinical trial data are valuable for documenting Practical Utility of a new invention, the standard for determining Practical Utility should not be a human clinical trial.

In clinical care, many intervening factors may determine clinical efficacy or safety. It is often impossible for an inventor to control or even anticipate these factors, such as:

1. The characteristics of an affliction such as a tumor, bacteria, genetic damage, or injury. For example, you and I can both have the same type of cancer, but biologically yours is different from mine because genetically our tumors are different even though we share the same diagnosis.

2. The characteristics of the host which bears the affliction. For example, biologically you and I are unique and different from other individuals suffering from the same illness.

3. The effect of a drug on both the affliction and the host may depend upon unique characteristics of the drug or agent, the amount of substance administered, the route of administration, the method of administration, the timing of administration, and where it is administered. For example, it is recognized that many living organisms, including people, have circadian biological rhythms which are extremely important to health, yet we know little about these rhythms except that we know that the same drug can produce different side effects and different benefits depending upon the timing of its adminstration.

From this perspective, there is a biological and clinical gestalt which must be understood in order for the utility doctrine to be meaningfully and reliably implemented from the perspective of human clinical trials. Unfortunately, it is often impossible to forecast the gestalt itself let alone control it. The development and clinical use of Interleukin-2 provides interesting proof of this point.

Interleukin-2: A Clinical Case History

IL-2 was first identified as an anti-cancer agent in 1976. It was first given to humans about 1984. It came up for review before the FDA in July of 1990 and was not approved even though it had been approved in nine European countries and had been shown to be safe and effective when tested in thousands of patients with advanced kidney cancer.

The reason that it was turned down was that it produced only a 15 percent response rate and the side effects of IL-2 were so severe, patients were put in intensive care when they received the drug. Eventually, with more research and a push from the National Kidney Cancer Association, IL-2 was approved by the FDA in early 1992.

During its review, the FDA focused on IL-2 as a single agent given in high doses by IV. However, that is not the way the drug is used today.

One of the most effective ways for a cancer patient to get IL-2 today is to inhale the drug using an inhalator like asthmatics use. Since metastatic kidney cancer occurs most frequently in the lungs, inhalation of the drug delivers high concentrations of the drug where it is needed while avoiding the side effects of systematic therapy.

In German clinical trials for metastatic kidney cancer in the lungs, inhalation therapy has produced a 65 percent response rate and is an outpatient therapy with almost no side effects. Many clinicians now believe that the human body produces and uses IL-2 locally rather than systemically. Inhalation therapy may be effective because it more closely approximates what the body itself is doing.

The lesson in this case history is that nobody ever envisioned that IL-2 would be inhaled when it was invented, or when it went through U.S. clinical trials, or when it came before the FDA. In fact, initial clinical trials shed little light on Practical Utility and the initial trial data almost led the FDA not to approve the drug at all.

Accelerated Patent Processing and Human Clinical Trials

In addition, requiring lengthy human clinical trials is completely at odds with a policy of accelerating the patent process for life saving inventions. Animal experiments are one substitute for human clinical trials, but even more is possible.

Surrogate end points are often needed and used as precursors to human clinical end points. In fact, modern clinical practice itself is moving away from a blind reliance on average response rates derived from clinical trials. For example, in vitro drug tests using a patient's living tumor tissue are now being used to determine a specific individual's drug sensitivity and resistance, and to design "patient specific" therapies.

The FDA has adopted a policy of using surrogate end points in its evaluation of AIDS drugs. T-cell counts and other markers have been used by the FDA as a basis for the approval of new drugs.

Many of the same scientific advances which allow gene fragments and other tiny biological components to be evaluated, also enable the Patent Office to adopt surrogate end points for patent decision making. What is required is the motivation by the Patent Office to develop and use an evaluation system composed of valid surrogates.

In this regard, it may be wise for the Patent & Trademark Office to develop "advisory boards" as the FDA has. These advisory boards, however, would not advise on any specific patent applications. Their responsibility would be confined only to the system of evaluation and assist the Patent Office in the selection of appropriate surrogate markers used in evaluating biotechnology patent applications.

Summary

To sum up my recommendations:

1. Develop a patent policy which maximizes the value of patented inventions, a policy which is neither too restrictive nor too lax.

2. Adopt a policy of making "Special" all patent applications which involve life saving inventions, and in so doing, accelerate the patent process for these inventions.

3. Beef up the corp of biotechnology patent examiners.

4. Do not rely on human clinical trial data for decision making. It is helpful but is not the proverbial "gold standard" for decision making for clinical practice or for the FDA.

5. Develop a system of surrogate end points for use in evaluating new life saving inventions and in evaluating biotechnology patents.

6. Build an advisory board to help the Patent & Trademark Office develop and update its system of surrogates and decision making criteria.

I urge you to consider these recommendations because, if adopted, they will enable the Patent & Trademark Office to better serve the public, particularly those of us who are suffering from life threatening illnesses. Thank you.