From the Editor's Desk

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Activism and Cancer Drug Approval

Death threats have no place in the drug approval process, nor in scientific debate in general for that matter. As a reminder of how violence can creep into our professional life, each day I walk past a memorial plaque to John L. Kemink, professor of otorhinolaryngology, who was shot to death in June 1992 by a patient. I was also in Los Angeles in February 1993, when a man carrying three firearms into the LA County USC emergency department shot physicians Richard May, Glen Rogers, and Paul Kaszubowski. Recently, two of our colleagues have reportedly received death threats after voting in opposition to a Food and Drug Administration (FDA) advisory committee's recommendation to delay approval of a cancer vaccine pending definitive trial results.

Patient activists and advocates play a valuable role in the federal drug approval and payment mechanism process. Partly through their efforts, we now have much more effective cancer treatments available, and rarely witness scenes similar to those I saw as a medical resident in San Diego in the late 1970s during my emergency department rotation. In what seemed like a weekly event, distraught families would wheel a cachectic, pain-racked loved one into the University Hospital with a plea to "Get them well enough for the flight to . . . [usually someplace in the Midwest or Northwest] so he [or she] can die at home." These patients were at the conclusion of a long, sad journey, seeking a magical treatment for their cancer in neighboring Mexico, after they had heard that medical science could do no more for them. In the ensuing 30 years, scientific inquiry; clinical and translational experiments; and patient involvement, advocacy, and activism have all expanded therapeutic and regulatory options.

I first became aware of activism in the oncology drug approval arena during the September 24, 2002, Oncologic Drugs Advisory Committee (ODAC) meeting, which deliberated and advised the US FDA regarding the approval of gefitinib for marketing. At the opening public forum, a patient advocate quoted several former ODAC members and chairs who had observed that patient presentations were "a federally mandated nuisance to be endured before real business got under way." The advocate went on to remind the assembled physicians and scientists that the advisory committee process is the only forum . . . [people have to] . . . participate in drug review." At that point, the committee members put down their newspapers and listened, as patient after patient told how gefitinib had benefited them (<http://www.fda.gov/ohrms/dockets/ac/02/transcripts/3894T1.pdf>). In a controversial decision (in which I participated), the committee recommended approval and the US FDA later accepted the recommendation.

Fast forward to early this year, when the Cellular, Tissue, and Gene Therapies Advisory Committee convened to review the evidence supporting the use of a prostate cancer vaccine. (I was a paid consultant to the sponsor and reviewed some of the data before its presentation.) The studies did not meet their primary end point. During the meeting, positive statements were followed by cheering from the observers, while negative statements were followed by a variety of audible, less than positive responses. To the disappointment of many, on May 9, 2007, the US FDA decided not to approve the vaccine, counter to its advisory committee's recommendation. The FDA apparently was convinced that the relative nontoxicity and novelty of the vaccine approach was insufficient to overcome the lack of demonstrable efficacy, and elected to await results of an already underway clinical trial. Others have commented on the seductive power of a harmless drug stimulating the natural immune system against cancer.¹ My former patients in San Diego were seduced by this promise.

Those in the role of patient advocates and disease related activists have increased the transparency of clinical research and have probably increased the participation in and the acceptance of, the clinical trial process—valuable outcomes all. Passion for a cause, and strongly held views are great and valuable motivators, but difficult decisions involving life-threatening diseases are best made based on evidence, not passion or threats. Death threats have no place in the helping professions, and their existence jeopardizes both the progress we have made and the transparency of scientific process from which we all benefit. Such threats are a tool of intimidation, not accountability, they have a chilling effect on candor, and they discourage voluntary public service. Let's agree when we can, agree to disagree when we must, and let the scientific process work.

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Reference

1. Devita VT: The Provenge decision. *Nat Clin Pract Oncol* 4:381, 2007