



# Translational Research in Cancer Complementary and Alternative Medicine: The National Cancer Institute's Best Case Series Program

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Translational research is an integral component in conventional medicine bringing bench research into clinical practice (Lehmann, Lacombe, Therasse, & Eggermont, 2003). The sequential methodology (in vitro experiments to animal models to human clinical trials) is an integral component in cancer care using conventional modalities. In complementary and alternative medicine (CAM), however, many modalities already in use have not undergone this sequential evaluation process. For safe and effective cancer CAM modalities to gain acceptance in the complex world of evidence-based conventional medicine, the translational research process must occur. Thus, CAM modalities presently in use for cancer symptom management or treatment must be studied in sequential order: generating in vitro data, animal model data, and then human clinical trial data.

For more than half a century, the National Institutes of Health (NIH) National Cancer Institute (NCI) has been assessing the value of various CAM modalities used to treat cancer. In September 1990, the U.S. Congress formally requested the Office of Technology Assessment (OTA), which is now defunct, to review the status of unconventional cancer treatments. Following OTA report recommendations, NCI identified two strategies to generate interpretable data: preparing a Best Case Series (BCS) and conducting a pilot clinical trial (Hawkins & Friedman, 1992).

The preparation of BCS in the 1990s involved the retrospective identification of patients who benefited from treatment with an alternative modality. Criteria for inclusion were: (a) documented diagnosis and stage of cancer with available pathologic slides for review, (b) documented tumor measurements

showing a reduction in tumor size, (c) documentation of no concurrent therapy with known antitumor effect, (d) listing of medications and previous therapies with responses, (e) documented course of unconventional therapy, and (f) therapy administration techniques that can be duplicated easily.

The NCI Division of Cancer Treatment (now known as the NCI Division of Cancer Treatment and Diagnosis [DCTD]) conducted the review of early case reports. Some modalities were Hoxsey therapy, Gerson therapy, vitamin C, laetrile, hydrazine sulfate, antineoplastons, and shark cartilage (White, 2002). Following a positive BCS review (i.e., the alternative modality is associated with tumor reduction), phase II trials were considered. If the alternative modality was associated with an improved quality of life or increased survival (versus tumor reduction), or if the modality could be given with standard therapies, a prospective pilot randomized trial was recommended. Clinical trials initiated as a result of BCS review include the use of antineoplastons and imagery.

In the late 1990s, the review of BCS submissions shifted from the NCI DCTD to the NCI Office of Cancer Complementary and Alternative Medicine (OCCAM). OCCAM was established in October 1998 to aid in the development of a prospective agenda in the research and use of CAM modalities for cancer symptom management and treatment. OCCAM coordinates NCI's CAM projects, serves as liaison to the National Center for Complementary and Alternative Medicine (NCCAM), and provides an interface with CAM practitioners, conventional practitioners, and the public (White, 2002).

## Criteria for Potentially Persuasive Cases

Many claims of the antitumor activity of alternative therapies are based on anecdotal case reports and patient testimonials. Critical review of such data frequently reveals a misinterpretation of the data in support of disease response and partial remission (PR) or complete remission (CR) confounded by other interventions such as conventional therapy or complementary modalities. Meaningful medical data support patient testimonials because patients do not always fully understand the disease process and interpretation of disease response.

OCCAM internally classifies cases according to the degree to which they fulfill BCS criteria. Potentially persuasive cases meet the following criteria: a pathologic diagnosis of cancer from a tissue specimen obtained prior to an alternative medicine intervention and after any conventional anticancer therapy, documentation of tumor regression appropriate for the disease type and location, absence of confounding or concurrent anticancer therapies, and documentation that a patient used the alternative medicine intervention under evaluation (see Figure 1).

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