



## INTRODUCTION

### *Our Mission*

*The mission of NCCAM is to define, through rigorous scientific investigation, the usefulness and safety of complementary and alternative medicine interventions and their roles in improving health and health care.*

### *Our Vision*

*Scientific evidence informs decisionmaking by the public, by health care professionals, and by health policymakers regarding use and integration of complementary and alternative medicine.*

**T**he National Center for Complementary and Alternative Medicine (NCCAM) is the Federal Government's lead agency for scientific research on complementary and alternative medicine (CAM). There are many definitions of CAM, none of them perfect. NCCAM defines CAM simply as a group of diverse medical and health care interventions, practices, products, or disciplines that are not generally considered part of conventional medicine. Clearly the boundaries between CAM and conventional medicine (also called Western or allopathic medicine) are not absolute. For example, CAM interventions are often incorporated into integrative medicine practices located in conventional medical care settings, and data from national surveys suggest that CAM is most often used by the general public as a complement or adjunct to conventional medical care. In addition, the boundaries are constantly evolving: interventions such as hospice care or relaxation and breathing techniques in childbirth that were once considered unconventional are now widely accepted.

*The scope, self-care nature, and associated costs of CAM use in the United States reinforce the need to develop scientific evidence concerning the usefulness and safety—or lack thereof—of CAM interventions.*

The most current and comprehensive picture of Americans' use of CAM has been developed under NCCAM leadership through two National Health Interview Surveys (NHIS), conducted by the National Center for Health Statistics at the Centers for Disease Control and Prevention in 2002 and 2007. Both surveys showed that nearly 40 percent of adult Americans reported using some form of CAM. The 2007 survey showed that 12 percent of children are using some form of CAM.

These data also show that Americans spent \$33.9 billion out-of-pocket for CAM in 2007. This accounted for approximately 1.5 percent of total health care expenditures, but more than 11 percent of total out-of-pocket health care expenditures. Moreover, a large fraction of total out-of-pocket spending was self-care—i.e., various products, classes, and materials not specifically recommended by a health care provider or CAM practitioner.

The scope, self-care nature, and associated costs of CAM use in the United States reinforce the need to develop scientific evidence concerning the usefulness and safety—or lack thereof—of CAM interventions, and to ensure the public has access to accurate and timely evidence-based information. Since its creation as an independent Center at the National Institutes of Health (NIH) in 1998, NCCAM has twice developed strategic plans to help guide the implementation of its legislative mandate to address these needs. Building on a decade of scientific progress, a robust research enterprise, and strong collaborations across NIH, NCCAM is now shaping its future through this third comprehensive strategic plan, developed with considerable input from its diverse stakeholder community.



# The Use and Cost of CAM in the United States

## Use

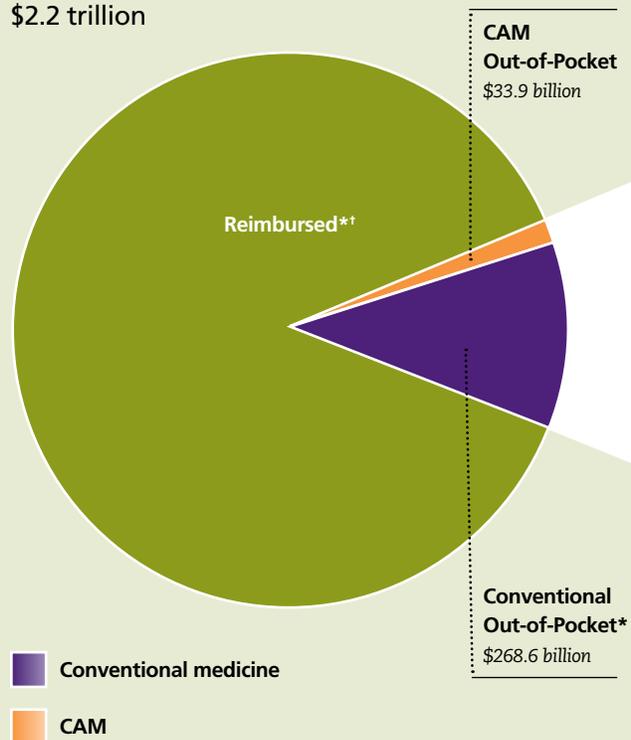
According to the 2007 National Health Interview Survey, which gathered information on more than 32,800 Americans, 38.2 percent of adults in the United States aged 18 years and over and nearly 12 percent of children aged 17 years and under used some form of CAM within the previous 12 months. Use among adults remained relatively constant from previous surveys. The 2007 survey provided the first population-based estimate of children's use of CAM.

## Costs

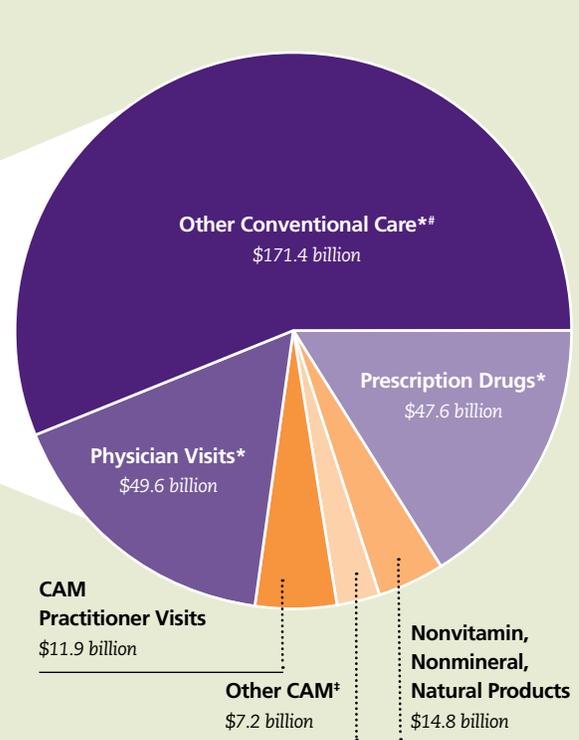
Americans spent \$33.9 billion out-of-pocket on CAM during the 12 months prior to the survey. This accounts for approximately 1.5 percent of total United States health care expenditures, but 11.2 percent of total out-of-pocket expenditures. A substantial portion of this expenditure is self-care (i.e., does not include the guidance of a health care provider or CAM practitioner).

### Total Health Care Spending, 2007

\$2.2 trillion



### Out-of-Pocket Spending



\* National Health Expenditure Data for 2007. U.S. Department of Health and Human Services, Centers for Medicare and Medicaid Services Web site. Accessed at: [http://www.cms.hhs.gov/NationalHealthExpendData/02\\_NationalHealthAccountsHistorical.asp#TopOfPage](http://www.cms.hhs.gov/NationalHealthExpendData/02_NationalHealthAccountsHistorical.asp#TopOfPage) on June 25, 2009.

† Reimbursed spending includes all employer and individual private insurance, Medicare, Medicaid, State Children's Health Insurance Program, and other private and public spending.

‡ Other CAM includes, for example, yoga, tai chi, qi gong classes; homeopathic medicine; and relaxation techniques.

# Other conventional care includes dental care, nursing homes, home health care, nondrug medical products, hospital care, and other professional services.

Source: Nahin RL, Barnes PM, Stussman BJ, and Bloom B. Costs of Complementary and Alternative Medicine (CAM) and Frequency of Visits to CAM Practitioners: United States, 2007. National Health Statistics Reports; no. 18. Hyattsville, MD: National Center for Health Statistics. 2009.



## Goals of the Strategic Plan

NCCAM enters its second decade at a time of both exciting scientific opportunity and heightened potential for making valuable contributions to health care practice and health promotion. This strategic plan is built around three long-range goals aimed at improving the state and use of scientific evidence regarding the two major reasons for use of CAM in the United States—treating health problems and supporting or promoting better health and well-being.

### **GOAL 1: Advance the science and practice of symptom management.**

CAM approaches are most often used to manage symptoms of underlying diseases and conditions, including back or neck pain, arthritic or other musculoskeletal pain, and insomnia, usually in conjunction with conventional medical strategies. Furthermore, evidence suggests that some CAM approaches may be helpful in managing these symptoms and that, in some cases, they engage innate biological processes involved in pain and emotion. Research to understand more clearly whether and how such interventions add value to existing approaches and to identify the biological mechanisms by which they exert beneficial effects will advance the science and practice of symptom management.



## **GOAL 2: Develop effective, practical, personalized strategies for promoting health and well-being.**

It is generally accepted and well established that sustaining healthy behaviors (e.g., good eating habits and regular physical exercise) and modifying unhealthy behaviors (e.g., smoking) reduce risks of major chronic diseases. Many CAM and integrative medicine practitioners and disciplines employ various CAM-based interventions (e.g., meditation or yoga) to help motivate people to adopt and sustain health-seeking behaviors, or they encourage dietary practices (sometimes grounded in traditional medical systems) that incorporate a healthy food philosophy. Newly emerging evidence suggests that CAM use may be associated with greater degrees of health-seeking behavior. While causal relationships between CAM use and healthy behavior have not been established, the claims and preliminary data deserve investigation given the formidable public health challenges in motivating behavior change. Research is needed to explore, clarify, and examine the hypothesis that certain CAM approaches or practices can, in fact, be useful in encouraging better self-care, an improved personal sense of well-being, and a greater commitment to a healthy lifestyle.

## **GOAL 3: Enable better evidence-based decisionmaking regarding CAM use and its integration into health care and health promotion.**

The needs of the public and health care providers (both conventional and CAM) for reliable, objective, evidence-based information regarding CAM remains compelling. Addressing that need remains central to NCCAM's success in fulfilling its legislative mandate. Importantly, evidence gathered during the past several years shows that CAM research findings have influenced CAM use and practice. NCCAM not only will continue to support research that addresses this need but also will continue to provide world-class, evidence-based information on the CAM practices used by millions of Americans.

## **A Decade of Investment in CAM Research**

### ***Growth of the Evidence Base***

Two overarching research priorities of NCCAM's first decade addressed (1) the relative paucity of foundational scientific information on the biological properties, safety, and efficacy of most CAM modalities and (2) the need for clinical trials testing the efficacy and safety of selected CAM interventions frequently used by the public. The products of this investment include, first



and foremost, an emerging evidence base—which has grown substantially in both quality and quantity. Basic research and clinical trials, large and small, have yielded results—both “positive” and “negative”—regarding the effects, efficacy, safety, and in some cases, promise regarding CAM. Formal evidence-based analyses and systematic reviews by independent organizations (e.g., the Cochrane Collaboration) point increasingly toward helpful conclusions regarding safety and efficacy—or lack thereof—of specific CAM interventions and practices. These conclusions are influencing the practice guidelines of professional medical societies.

## Impact of Research on CAM Natural Product Use and Spending

### Major Changes in Frequency of Use Track Research Results

Both the 2002 and the 2007 National Health Interview Surveys examined the use of a number of CAM natural products. Direct comparisons of the extent of use of specific products cannot be made because of differences in the questions asked in the two surveys. However, changes in the relative ranking of the 10 most commonly used products suggest that results from CAM research do influence consumers’ decisions regarding CAM use.

In 2002, fish oils/omega-3 fatty acids ranked eighth, while in 2007 they ranked first. This change correlates with a growing body of evidence suggesting benefit of these products in preventing complications of cardiovascular disease and other significant health problems.

In 2002, the herb St. John’s wort (*Hypericum perforatum*) ranked sixth in use, while in 2007 it ranked twenty-first. This change correlates with publication of research documenting potentially harmful herb-drug interactions involving antidepressants, birth control pills, antiretrovirals used to treat HIV infection, Dilantin or other antiseizure drugs, and warfarin. It also correlates with the publication of several studies that did not find efficacy in major depression or attention deficit hyperactivity disorder (ADHD).

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Source: Barnes PM, Bloom B, Nahin R. CDC National Health Statistics Reports #12. *Complementary and Alternative Medicine Use Among Adults and Children: United States, 2007*. December 2008.

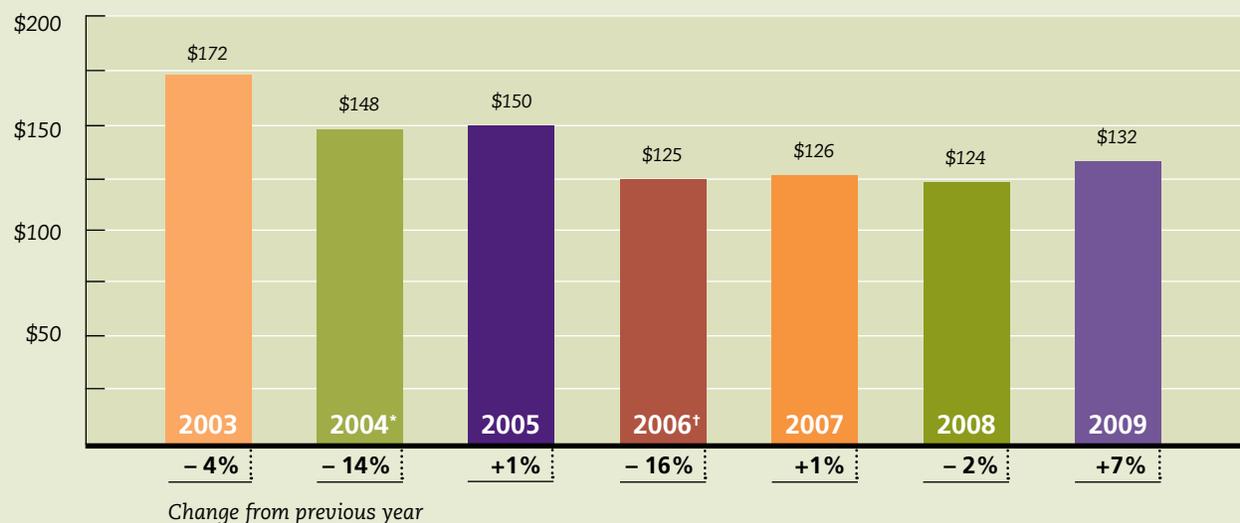
## CAM Research Influences Consumer Use and Spending

Two independent lines of evidence—survey data from the NHIS regarding use of dietary supplements and industry sales data—suggest that results of CAM research do influence consumers’ decisions regarding CAM use. Both show changes over time that track with research findings. Specifically, the publication of “negative” results from clinical trials preceded declines in both the frequency of use and the sales of several nonvitamin/nonmineral dietary supplements. Similarly, publication of evidence pointing toward the potential value of omega-3 fatty acids/fish oil preceded increased use and sales. It is also notable that in direct response to the results of NCCAM-funded research, the U.S. Food and Drug Administration has taken action to address concerns about the safety of several specific CAM products.

Basic research and clinical trials, large and small, have yielded results—both “positive” and “negative”—regarding the effects, efficacy, safety, and in some cases, promise regarding CAM.

## U.S. Echinacea Sales, 2003–2009

Sales estimates (in millions)



\*In a 2003 study, published in the *Journal of the American Medical Association*, researchers evaluated echinacea for efficacy and safety in children with upper respiratory tract infections. In this study, echinacea did not reduce the severity or duration of symptoms.

†In a 2005 study published in *The New England Journal of Medicine*, researchers examined *Echinacea angustifolia* root extracts for effects against rhinovirus (a virus that causes the common cold). None of the echinacea preparations in this study reduced the rate of infection, severity of symptoms, or inflammation. According to the *Nutrition Business Journal*, echinacea sales were negatively affected specifically by the 2005 study.

Source: *Nutrition Business Journal. Supplement Business Report 2010*. Boulder, CO: New Hope Natural Media, Penton Media, Inc. 2010.

Dietary supplement sales data also suggest that new research findings affect use. For example, sales of echinacea have fallen as research findings have indicated that certain preparations do not prevent or reduce the severity of the common cold.



### *Areas of Scientific Promise*

Most importantly, the expanding evidence base includes a large body of intriguing, albeit preliminary, evidence that points toward specific opportunities to improve health care and health promotion using CAM-inclusive strategies. Some examples of these promising leads are included in the table below.

## Examples of Important Clinical Research Opportunities and Needs

### **Mind and Body**

- Developing better, comprehensive strategies for management of chronic back pain and defining the roles of acupuncture, spinal manipulation, and massage in those strategies
- Exploring the role of specific promising CAM practices or disciplines (e.g., meditation, yoga, or acupuncture) in developing better strategies for alleviating symptoms (e.g., chronic pain, stress) or in promoting healthier lifestyles
- Exploring the associations between well-characterized pathways of pain processing and acupuncture analgesia or the placebo response
- Exploring the associations of major pathways of cognitive processing and emotion regulation by meditative practices
- Studying the influence of the provider-patient/client interaction, context effects, and the placebo response on outcomes of CAM interventions

### **Natural Products**

- Studying the molecular targets and biological effects of potentially beneficial small molecules that are constituents of natural products or diet (e.g., quercetin, curcumin, or other polyphenols and flavonoids)
- Defining the anti-inflammatory actions of omega-3 fatty acids
- Employing state-of-the-art tools and technologies to study the effects of probiotics on the human microbiome
- Developing evidence regarding the safety profile of certain widely used natural products, including interactions with drugs and other herbals or dietary supplements

## Mapping the Path Forward

Over a year-long planning process, NCCAM carefully assessed how recent developments in science, medicine, and health care have affected the Center's strategic approaches in the diverse arena of health care and health promotion interventions. With broad public input, the Center took stock of its existing programs and priorities, the growing evidence base, research capacity, and scientific and public health needs going forward. This assessment led to the following conclusions.

### **It is important that a portion of the Center's research efforts be specifically targeted toward carefully selected areas of particular public health promise.**

Two overarching goals of NCCAM's early history included developing foundational scientific evidence regarding most CAM interventions and building multidisciplinary CAM research capacity where little existed. A relatively broad-based, nontargeted, investigator-initiated research project grant approach was adopted to address these goals. This approach over the past 10 years has served the field very well. Notably, NCCAM's investigator-initiated research project grant stream is increasingly robust. More importantly, it has yielded a substantial body of basic, translational, and clinical research evidence that points toward the potential of a number of CAM interventions to contribute to important public health needs. It is essential that similar opportunities for investigator-initiated research involving less well-studied or -characterized CAM interventions and disciplines be preserved.

At the same time it has become clear that this approach cannot solely be relied upon to ensure that major gaps in knowledge are filled, or that development of a definitive clinical evidence base regarding the most promising research leads are pursued with goal-oriented clarity, timeliness, and efficiency. Therefore, there is a need, particularly given finite resources, to target a portion of NCCAM's investment in research toward development of definitive evidence regarding carefully selected opportunities that offer the most potential for adding significant value to public health.

**Framework for Priority Setting.** Establishing priorities across the entire field of CAM research is particularly challenging. For a small number of opportunities, available evidence will support a targeted, intensive approach toward development of definitive clinical evidence. For many others, mechanistic or exploratory basic or clinical research data, or the development of translational tools necessary for rigorous clinical investigation, are the most immediate research priorities.

*The expanding evidence base includes a large body of intriguing, albeit preliminary, evidence that points toward specific opportunities to improve health care and health promotion using CAM-inclusive strategies.*



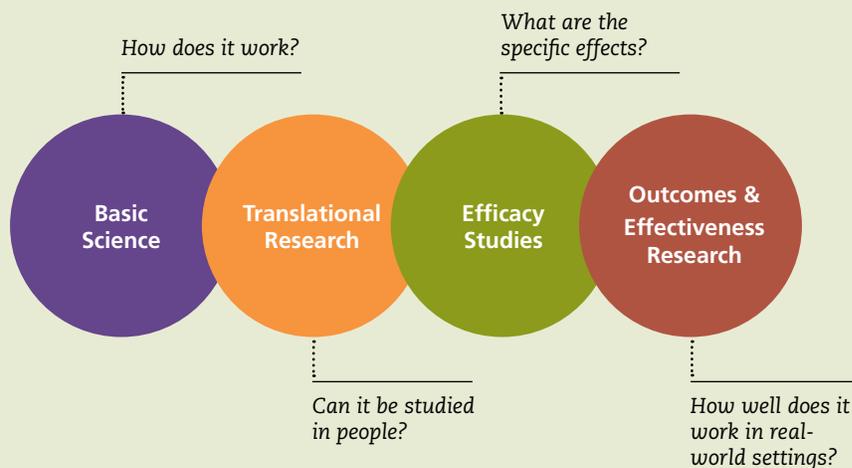


A framework of four factors (as outlined below) will be used by NCCAM in its interactions with investigators and its National Advisory Council to assist in (1) identifying and shaping targeted research initiatives, (2) identifying the most critical knowledge gaps in advancing research on particular CAM interventions, practices, or disciplines, and (3) striking the proper balance between targeted research initiatives and investigator-initiated creativity. The specific weight of these factors will vary, depending on the particular focus of the research.

- **Scientific Promise:** Does a reasonable body of evidence support the potential of the proposed research to lead to improved (1) options or strategies to treat troubling or prevalent health conditions or symptoms or (2) approaches to promote better health and well-being? Is evidence sufficient to support the scope and direction of the proposed research? If not, what research is needed to establish such evidence?
  
- **Amenability to Rigorous Scientific Inquiry:** Are the key research goals achievable, and are the key research questions amenable to rigorous scientific investigation, given needed and available methods for measurement, translational tools, and technologies? Are potential approaches feasible and scientifically plausible? Do they lend themselves to rigorous quality control? If not, does the proposed research focus appropriately on developing needed methods, tools, and technologies?
  
- **Potential To Change Health Practices:** Is it reasonably likely that the results of the research or program could lead to changes in the health practices of consumers or health care providers or in the decisions of health policymakers?
  
- **Relationship to Use and Practice:** Does the proposed project address an important public health concern or scientific information need regarding efficacy, safety, or public use of CAM?

**CAM interventions, approaches, and disciplines can and must be studied across the continuum of basic, translational, efficacy, and effectiveness research.** Continued emphasis on both *basic research* to understand biological effects and mechanisms of action and on *efficacy studies* to determine specific clinical effects of interventions is essential in developing the scientific evidence base. This plan also articulates the need to strengthen *translational research* required to design and implement definitive clinical research and “real world” *outcomes and effectiveness research* that capitalizes on the reality that many CAM interventions are in widespread public use.

## The Range of Research Questions



While the need for clinical research evidence is at the heart of NCCAM’s mission, developing that evidence requires support across the continuum of basic, translational, and clinical research.

- **Basic science:** defining biological effects and mechanisms of action; clarifying scientific hypotheses; supporting development of translational tools
- **Translational research:** identifying and validating biomarkers or other signatures of biological effect; developing and validating measures of outcome; validating treatment algorithms and measures of quality control; developing preliminary clinical evidence regarding efficacy and safety; establishing feasibility or estimates of sample size for future studies
- **Efficacy studies:** determining the specific effects of an intervention under carefully controlled conditions that minimize nonspecific and contextual effects
- **Outcomes and effectiveness research:** studying usefulness and safety in general populations or health care settings

**Large clinical trials on the efficacy and effectiveness of CAM interventions require a solid foundation of basic and translational research.** Large clinical trials studying efficacy and effectiveness will remain a cornerstone of the CAM clinical research enterprise. Because they are operationally and ethically complex, expensive, and time-consuming, it is imperative that they be designed in ways that maximize the likelihood of clear and unambiguous results (a defining factor of a successful clinical trial). NCCAM's previous strategic plan articulated the importance of understanding mechanisms and biological effects in designing definitive clinical trials. This plan reaffirms that conclusion and further stresses the central importance of translational research in creating a foundation for definitive clinical investigation.

## Outcome Measures—Symptoms Matter

Randomized clinical trials are powerful tools for investigating the effects of interventions. The success of a clinical trial in yielding clear and unambiguous evidence depends, among other things, upon the validity and reliability of the techniques used to measure response to the intervention. This presents a particular challenge in designing studies of interventions on troubling symptoms such as pain, nausea, anxiety, or depression, relief of which may be foremost in the minds of patients or clients.



Clinical investigators often rely upon measures of functionality, which can be observed or measured by others, or utilize objective biomarkers that assess physiological variables. While such measures are invaluable and an important part of clinical trial design, they often do not capture the complexity of symptom states or track closely with clinical responses most important to patients. For example, depression may accompany chronic pain, and in some individuals alleviating depression may have a greater impact on overall sense of well-being than pain relief *per se*. In addition, some interventions, particularly mind and body interventions, may affect many aspects of health, so it is often important in a clinical trial to measure the effect of the intervention on a number of health domains and symptoms.

These and similar challenges are the focus of the science of patient-reported outcomes (PROs), a discipline that is critical to NCCAM's research portfolio. New electronic tools are being developed to permit more accurate and frequent assessment of symptoms. NCCAM is taking a leadership role in PROMIS, a trans-NIH Common Fund program that is developing computer-assisted methods to strengthen the assessment of patient-reported outcomes. These issues will continue to be of central importance in designing clinical research studies to test the effects of CAM interventions in alleviating symptoms or improving health and well-being.



**Given the widespread use of CAM, opportunities exist to employ clinical outcomes and effectiveness research methodologies in developing useful “real world” evidence about application of specific CAM approaches to health care and health promotion.** Most CAM interventions are readily available to the public, and many are used regularly in the health care and health promotion practices of individuals and professions. There are increasingly viable opportunities to take advantage of this fact by employing the methods and tools of clinical outcomes and effectiveness research to develop (1) evidence, based in real-world practices and use, about the potential of CAM interventions, modalities, and disciplines to contribute to better treatment and health promotion and (2) data needed to design maximally informative clinical trials. Pursuing such research will require creative collaboration with experts who confront similar challenges and opportunities in studying outcomes of procedures or multicomponent interventions introduced into and adapted in clinical practice (e.g., surgery, psychotherapy, and behavioral change).

**Better strategies for management of chronic pain are an area of public health need and scientific promise in CAM research.** National survey data show that the majority of Americans’ use of CAM for treating specific health problems is aimed at ameliorating symptoms, particularly for chronic pain. Furthermore, emerging data from the past 10 years point toward specific scientific opportunities for research with the potential to contribute to better integrative approaches for care and treatment of chronic pain.

**Research on the contributions of CAM interventions, practices, and disciplines in promoting or supporting health-seeking behavior is another area of special public health need and scientific opportunity.** Although much of CAM use by Americans is aimed at improving general health and well-being, most CAM research to date has focused on the application of CAM practices to the treatment or prevention of various diseases and conditions. While scientific and operational challenges are significant in pursuing a health-promotion research



agenda, compelling opportunities exist to explore the potential role of CAM practices, interventions, and disciplines in promoting or supporting health-seeking behavior.

**NCCAM must continue to play a central role in addressing the need for reliable, objective information based on scientific evidence so that consumers and health care providers can make well-informed decisions.** Although a vast amount of information about CAM is available in the public domain, much of it is incomplete, misleading, inaccurate, or based on scientifically unproven claims. Much of the public's use of CAM occurs in the absence of advice or guidance from health care providers (conventional or CAM). These facts reinforce the need for reliable, objective, evidence-based information regarding the usefulness and safety—or lack thereof—of CAM.

## Strategic Objectives

This plan seeks to address three long-range goals, discussed earlier and summarized here:

**GOAL 1: Advance the science and practice of symptom management.**

**GOAL 2: Develop effective, practical, personalized strategies for promoting health and well-being.**

**GOAL 3: Enable better evidence-based decisionmaking regarding CAM use and its integration into health care and health promotion.**

The plan is organized around the following five strategic objectives, which are summarized below and discussed in greater detail in the ensuing five chapters. Each strategic objective serves, to varying and often overlapping degrees, the above three long-range goals.

**Strategic Objective 1:** Advance research on mind and body interventions, practices, and disciplines.

**Strategic Objective 2:** Advance research on CAM natural products.

**Strategic Objective 3:** Increase understanding of “real world” patterns and outcomes of CAM use and its integration into health care and health promotion.

**Strategic Objective 4:** Improve the capacity of the field to carry out rigorous research.

**Strategic Objective 5:** Develop and disseminate objective, evidence-based information on CAM interventions.