

May 5, 2004

Dockets Management Branch (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, Maryland 20852

RE: Document number 2004S-0170 (Suggested Priority Topics for Research)

The Academy of Managed Care Pharmacy (AMCP) is pleased to provide suggestions to the Department of Health and Human Services regarding the highest priorities for research, demonstration, and evaluation projects to support and improve the Medicare, Medicaid, and State Children's Health Insurance (SCHIP) programs. The Academy strongly supports the Department's decision to ask stakeholders responding to this Notice to focus their priorities on "evaluating existing evidence regarding the comparative clinical effectiveness of prescription drugs in anticipation of the Medicare prescription drug benefit."

AMCP is a professional association of pharmacists and associates who serve patients and the public through the promotion of wellness and rational drug therapy through the application of managed care principles. The Academy has more than 4,800 members nationally who provide comprehensive coverage and services to over 200 million Americans served by managed care. Many of the Academy's members are involved in decisions in their daily practice that require the evaluation of outcomes and appropriateness of health care items and services, especially prescription drugs.

The Academy supports research on the comparative clinical and cost effectiveness of prescription drugs. Such research is a fundamentally necessary component of any rational approach to determining the value and usefulness of prescription drugs. Currently, only limited authoritative research exists that distinguishes the effectiveness and safety profile offered by any particular drug as compared to other drugs in the same or a similar treatment class. Physicians, pharmacists, other health professionals, patients and purchasers of health care need objective, easily-accessible evidence-based information regarding the comparative clinical and cost effectiveness of prescription drugs in order to make knowledgeable and informed decisions.

Some privately-sponsored research evaluating and comparing prescription drugs does exist, but it is extremely limited. Almost all of these efforts have been funded by drug manufacturers; some have proven to be valuable, but in general the extent, scope and quality of these efforts have not provided the type of independent assessment that is needed. While the Academy recognizes the potential deficiencies inherent in private sector comparative effectiveness research, it will continue

to support and encourage an expansion of such efforts when it can be demonstrated that the research methodology is scientifically sound, and the resulting information is reliable and will be presented objectively. The Academy believes that the federal government must assume principal responsibility for sponsoring this type of research. More information on the importance of comparative effectiveness research can be found in the Academy's position statement on "Comparative Effectiveness of Prescription Drugs" at:

<http://www.amcp.org/amcp.ark?c=legislative&sc=position&id=22>

AMCP understands that the steering committee staff will prepare a preliminary ranking of suggested topics for study taking into consideration factors suggested by the terms of Section 1013(a)(2)(c) of the Medicare Prescription Drug, Improvement and Modernization Act (DIMA) of 2003:

Health care items or services that impose high costs on Medicare, Medicaid, or SCHIP programs; those which may be underutilized or overutilized; and those which may significantly improve the prevention, treatment, or cure of diseases and conditions which impose high direct or indirect costs on patients or society.

As the steering committee works to identify the priorities for federally funded comparative effectiveness studies, the Academy recommends that several principles guide the decision-making process.

- ?? Medications consumed by seniors should drive the priorities.
- ?? Studies should be focused on those areas in which prescribing decisions may be influenced by the outcomes of the comparative effectiveness research. Research priorities should be in areas in which results are actionable.
- ?? The priority setting process should emphasize the value of studies in the senior population. Studies that measure the efficacy, effectiveness and side effects of medications are not often conducted in seniors and thus do not provide the data necessary to make decisions for this important population. (They have guidelines about who can participate. Guidelines are based on such factors as age, type of disease, medical history, and current medical condition. Seniors are often excluded from clinical studies).
- ?? Studies need to address both efficacy and effectiveness of medications. Because efficacy is measured in many pharmaceutical manufacturer conducted clinical trials, the unmet need is in studies that address effectiveness.

Efficacy measures patient outcomes when medications are taken exactly as prescribed and are studied in the type of clinical trials performed as a part of manufacturer-funded research to obtain data for the Food and Drug Administration's review of products seeking market status. Clinical trials routinely have guidelines that attempt to control variables so that the effect of the drug being tested can easily be evaluated.

Effectiveness can only be evaluated once a drug is being used in a broad-based population. Effectiveness studies will show practitioners what effects a drug can have when it is prescribed for patients with a variety of characteristics, e.g. patients being treated for more than one disease, patients with differing physiologies, and/or patients with differing

physical capabilities and demands. Effectiveness includes an examination of practical issues such as patient compliance with directions on how to use the drug which has a significant influence on health outcomes.

Both efficacy and effectiveness need to be addressed in a comparative evaluation. Because drug manufacturers must perform efficacy studies to gain approval to market their products, these studies are generally in existence. Frequently effectiveness studies are not performed. Therefore, comparative studies need to emphasize the valuable data that can be gained from effectiveness research and then evaluate it in conjunction with efficacy results.

?? We especially recommend that studies measure the impact of medications on overall health outcomes of patients.

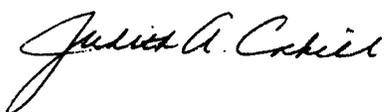
In developing recommended research priorities, AMCP based the list below on input received from Academy members involved in decisions in their daily practice that require the evaluation of outcomes and appropriateness of prescription drugs. Members evaluated the input received and arrived a consensus priority list. The Academy recommends that these studies initially focus on the following priority areas (in order).

- ?? Congestive heart failure (CHF)
- ?? Atherosclerotic cardiovascular disease (including hyperlipidemia/statin medications)
- ?? Osteoporosis
- ?? Alzheimer's disease
- ?? Visudyne (verteporfin)/macular degeneration
- ?? Chronic obstructive pulmonary disease (COPD)
- ?? Diabetes
- ?? NSAIDs (nonsteroidal anti-inflammatory drugs) vs. Cox-2s (COX-2 selective NSAIDs)
- ?? Rheumatoid arthritis medications (oral and injectable, including disease-modifying antirheumatic drugs [DMARDs])
- ?? Anticonvulsants (for example, Topamax [topiramate] and Neurontin [gabapentin])

Although randomized controlled trials that provide direct comparison between medications would be the ideal types of studies for comparative effectiveness research, AMCP realizes that these studies are expensive and not always feasible. Before incurring these costly undertakings, the government should first conduct a systematic evaluation of the currently available research on the medications selected, utilizing both published literature and internal studies conducted by pharmaceutical manufacturers.

The Academy thanks HHS for the opportunity to assist with suggestions regarding the highest priorities for research, demonstration, and evaluation projects. If you have any questions, please contact me at (703) 683-8416 or at jcahill@amcp.org.

Sincerely,



Judith A. Cahill, CEBS
Executive Director