Supplemental Comments of Pfizer, Inc.:
Medicare Prescription Drug Improvement and Modernization Act Section 1013:
Priority Topics for Research on Outcomes of Health Care Items and Services

[Docket No. 2004S-0170]

Pfizer, Inc. (Pfizer) respectfully submits these supplemental comments in response to the Agency for Healthcare Research and Quality's (AHRQ) request for comments on its longer-term priorities under Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA).

Pfizer is a research-based pharmaceutical company with enormous experience applying objective data to clinical problems. We maintain the largest privately funded biomedical research establishment in the world, and have a global enterprise dedicated to obtaining, understanding, and applying the very best evidence from basic and clinical sciences to human health. This experience in the discovery and development of pharmaceuticals, and our keen understanding of the clinical environment in which our products are used, provides a unique perspective on the value and role of AHRQ's activities under MMA Section 1013.

Executive Summary

Pfizer recognizes that policy makers must make health care resource allocation decisions, and that purchasers and consumers of health services are increasingly concerned with the value of the care being provided. That said, Pfizer believes clinical judgment and patient choice, within the bounds of accepted medical practice, must always be the overriding force in decisions about individual care. To provide quality, individualized care, physicians must have the opportunity for multiple treatment options. Dosing, packaging and different metabolic pathways may make particular compounds in a therapeutic class suitable for one person but not for another with the same condition.[1] As Pfizer Chairman and CEO Dr. Henry McKinnell told the World Health Care Congress on January 28, 2004, Pfizer believes our health care system "should allow doctors and patients to choose the best courses of care…rather than settling for the lowest common denominator of 'average care for the average person.'"

Unfortunately, numerous articles have shown that aggregating and analyzing comparative clinical effectiveness studies is difficult because the methods used can differ widely.[2] Methodologies currently relied upon by policymakers often lack rigor, and in some cases, the underlying studies are ill conceived or undertaken by a researcher with a clear agenda or insufficient qualifications. More consistent and transparent methods and reporting must be achieved for comparative clinical effectiveness analysis to become useful.[3]

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[1] Gorman, L. Medicaid Drug Formularies. Independence Institute, Issue Paper Number 2-2002. April 2002.
[2] P.J. Neumann et al., "A Formal Audit of 228 Published Cost-Utility Analyses" (Working paper, Harvard School of Public Health, 1999).
[3] I.S. Udvarhelyi et al., "Cost-Effectiveness and Cost-Benefit Analyses in the Medical Literature: Are the Methods Being Used Correctly?" *Annals of Internal Medicine* 116, no. 3 (1992): 238–244; Neumann et al., "A Formal Audit of 228 Published

Given these limitations, Pfizer strongly believes that the resources provided under MMA Section 1013 should be employed in an open, ongoing process for obtaining input from stakeholders, and AHRQ to consider all stakeholder objections as the agency develops and employs any standards for comparative clinical effectiveness analysis. The application of such standards and methodologies in specific areas should be reviewed regularly via a notice and comment process.

Moreover, any studies undertaken should apply equally to all health care interventions, both diagnostic and therapeutic, and should be applied in the context of disease states, rather than for product- or product class-specific cost-cutting objectives. It is critical that such standards include measures to ensure that all costs are included in comparative cost effectiveness analyses, including the harm that would be caused to certain populations by excluding or disfavoring treatments. Additionally, all health care stakeholders should be held to the same level of rigor in analysis and transparency in presentation, particularly when the outcomes research and comparative effectiveness studies are done for comparison purposes.

Pfizer strongly believes the agency should research the underutilization of appropriate treatment as well as the comparative costs and outcomes from all available treatments for the most burdensome health conditions. Based on this belief and a literature review to determine the most costly, most treatable health conditions or those with the greatest disparities in care, Pfizer recommends that AHRQ study the following seven categories of health care:

1. Heart disease
2. Cancer
3. Chronic kidney disease
4. Diabetes
5. Maternal and child health
6. Respiratory diseases
7. Long term care

8. Mental health

Pfizer also believes it would be advisable for AHRQ to further research the benefits of disease management and other methods of improving coordination of care, including case management and improved information systems. Generally, disease management studies conducted around the country indicate that closely managing patients with chronic diseases can reduce the cost of physician and hospital services these patients require and at the same time improve quality of life for the patient.[4] Disease management can prevent or delay the onset of the more severe and costly stages of a disease and in many cases may be the most cost-effective method of treatment.

Cost-Utility Analyses"; and K. Gerard, I. Smoker, and J. Seymour,"Raising the Quality of Cost-Utility Analyses: Lessons Learnt and Still to Learn," *Health Policy* 46, no. 3 (1999): 217–238.

[4] Teutsch, S. M., presentation at third meeting of the Cost-Effectiveness Panel on Clinical Preventive Services, Office of Disease Prevention and Health Promotion, Office of the Assistant Secretary for Health, Public Health Service, U.S. Department of Health and Human Services, Washington, DC, Sept. 28, 1993.

Finally, Pfizer believes AHRQ must be vigilant in ensuring that its studies are not misapplied to support cost containment strategies. Only after patients and physicians are aware of clinical options and available outcome data is it proper for issues of cost and coverage to enter into the decision. Restricting access to important medicines not only harms patients, but also inflates overall health care costs as a greater number of patients frequently require costly hospital care and other medical services as a result of untreated and worsening conditions. MMA Section 1013 was intended to facilitate studies of various therapeutic areas: it does not mandate a narrow focus on the comparative clinical effectiveness of pharmaceutical treatments, and certainly not for facilitating or influencing drug payment decisions.

AHRQ's overall mission is to improve the quality, safety, efficiency and effectiveness of health care for all Americans. Given the agency's objectives, it is important that it supports research designed to improve the outcomes and quality of health care, address patient safety and medical errors, and broaden access to effective services, not to develop cost containment strategies that limit patient access to life-saving therapies.[5] In particular, while Pfizer recognizes the substantial cost of many novel pharmaceutical treatments, we also recognize the impact these treatments have on the health and lives of the patients who take them. To that end, Pfizer believes that AHRQ should recognize that the 10.5% of health care spending used to pay for prescription pharmaceuticals helps reduces spending on other, more expensive interventions.[6] It is critical that AHRQ not allow the resources provided under MMA Section 1013 to be used to develop cost containment strategies that limit access to new therapies.

With all of theses issues in mind, the following comments are intended to:

- **Outline the purpose and scope of MMA Section 1013 –** Specifically, Pfizer believes that Section 1013 of the Medicare Prescription Drug Improvement and Modernization Act of 2003 was intended to facilitate studies of various therapeutic areas, not just focus on the comparative clinical effectiveness of pharmaceutical treatments.

- **Discuss the purpose of AHRQ's research –** Pfizer believes comparative clinical effectiveness and outcomes research cannot replace the patient-physician relationship. The primary decision as to treatment should be made by the physician and patient, and only after patients and physicians are aware of all clinical options and available outcomes data is it proper for issues of cost to enter into the decision making process.

- **Propose basic standards for evidence -** Pfizer believes all outcomes research and comparative effectiveness research must be (1) transparent, (2) objective, and (3) consistent. The best research, no matter its use, must be easily shared, dissected and understood.

[5] See [http://www.ahrq.gov/about/profile.htm]

[6] Frank R. Lichtenburg, "Benefits and Costs of Newer Drugs: An Update" (Cambridge. MA: National Bureau of Economic Research, June 2002.

?? **Recommend disease states for study -** Pfizer believes additional research must be done on the comparative clinical effectiveness of treatments for the most costly disease states. Additionally, analysis must be undertaken to understand the underutilization of appropriate treatments by the patient populations most susceptible to these diseases.

?? **Discuss the benefits of disease management -** Pfizer believes disease management systems should be in place for each disease state as a context for pharmaceutical outcomes research or comparative effectiveness analysis. This will enable policy makers to make rational economic and clinical choices between therapeutic alternatives.

?? **Warn against aligning payment policies with quality improvement goals -** Pfizer believes that payment for health care technologies must encourage the development of novel methods to improve the value obtained for the resources expended.

I. Introduction

Applying scientific evidence, generally gained by studying groups of patients, to individuals, is perilous. That experience has given us enormous respect for the importance of clinical judgment and the need for flexibility in addressing individual patient circumstances and values. We recognize that the practice of medicine requires that each patient is cared for individually, meaning that appropriate evidence needs to be applied to reflect the clinical situation and needs of the patients who may differ in their genetics, co-morbidities, medications, metabolic status or preferences. Among the core strengths of American medicine is the ability of practitioners to interpret and apply evidence in individual clinical settings. Therapeutic discretion allows for improvements in care, but requires continuous review with dissemination of outcomes to foster recognition and elimination of ineffective or dangerous practices.

Unfortunately, in today's health care marketplace, outcomes research and comparative effectiveness studies are frequently undertaken with a cost-cutting rather than quality agenda. In other words, cost savings in disconnected areas of treatment are emphasized over systemic cost-consciousness. Pfizer is quite concerned that state, federal and private payers will design drug-oriented cost-containment processes around AHRQ's studies without regard to quality patient care. While the concept of assembling comparative clinical information on products within a class is inherently attractive and for some very limited purposes may be sound, using such analyses as a cost-saving measure may endanger patients. Indeed, just as Pfizer regularly must prove that its drugs are both safe and effective, proponents of this cost-driven approach should be forced to ensure that the restrictions placed on patient access do not in fact cause patient harm.

Pfizer believes it is inconsistent with the legitimate goals and values of comparative clinical effectiveness and outcomes research to permit such studies to be used by health care payers to make product-specific decisions with the goal of controlling costs. Recognizing that cost-efficiency must be achieved within the health care system, Pfizer believes financial decisions

must be kept separate from clinical decisions in order to preserve the quality of care achieved by respecting the core physician-patient relationship in medicine.

Pfizer holds itself to the highest standards of excellence and believes we, as a company, have a responsibility to patients and the physicians that care for them to ensure that our medicines are analyzed in a thorough, fair, and objective manner.

II. Purpose and Scope of MMA Section 1013

Section 1013 of the Medicare Prescription Drug Improvement and Modernization Act of 2003 (MMA) was intended to facilitate studies of various therapeutic areas. The provision does not mandate a narrow focus on the comparative clinical effectiveness of pharmaceutical treatments, and certainly not for facilitating or influencing drug payment decisions.

Specifically, Section 1013 calls on AHRQ to conduct studies to improve the quality, effectiveness, and efficiency in the Medicare, Medicaid, and State Children Health Insurance (SCHIP) programs. Under the statute, the studies must focus on (1) the outcomes, comparative clinical effectiveness, and appropriateness of health care items and services (including prescription drugs); and (2) strategies for improving the efficiency and effectiveness of such programs, including the ways in which such items and services are organized, managed, and delivered under such programs. Notably, as stated by AHRQ in its request for comments, "the statute does not limit the scope of the initial priority list". Therefore, as discussed throughout these comments, Pfizer believes AHRQ should look to improving the clinical return on overall money spent by examining disease management, prevention programs, and various other long-term strategies for treating chronic conditions, rather than short-term cost containment.

In addition to conducting studies, the statute charges the Secretary of HHS with: (1) ensuring that there is broad and ongoing consultation with relevant stakeholders in identifying the highest priorities for research, demonstrations, and evaluations; (2) including health care items and services which impose a high cost on such programs, as well as those which may be underutilized or overutilized and which may significantly improve the prevention, treatment, or cure of diseases and conditions (including chronic conditions) which impose high direct or indirect costs on patients or society; and (3) ensuring that the research and activities undertaken pursuant to this section are responsive to the specified priorities and are conducted in a timely manner.

Once the studies are complete, the statute requires that the Secretary: (1) evaluate and synthesize available scientific evidence related to health care items and services (including prescription drugs) with respect to the comparative clinical effectiveness, outcomes, appropriateness, and provision of such items and services; (2) identify issues for which existing scientific evidence is insufficient with respect to such health care items and services (including prescription drugs); (3) work in voluntary collaboration with public and private sector entities to facilitate the development of new scientific knowledge regarding health care items and services (including prescription drugs).

Notably, Congress expressly required that "research, evaluation, and communication activities performed pursuant to this section shall reflect the principle that clinicians and

patients should have the best available evidence upon which to make choices in health care items and services, in providers, and in health care delivery systems, recognizing that patient subpopulations and patient and physician preferences may vary." Thus, the Secretary must be very careful to make certain that these studies are not used to facilitate cost-driven approaches aimed at restricting the pharmaceutical care available to patients.

III. Purpose of Research

As noted, the primary decision as to treatment should be made by the physician and patient, and only after patients and physicians are aware of all clinical options and available outcomes data is it proper for issues of cost to enter into the decision making process. Moreover, when costs are considered, all relevant costs should be included across the system of care and over the length of a treatment for the patient. Simply focusing on short-term financial savings or a single treatment option may result in increased long-term costs to the overall health care system, and a disparate impact on vulnerable patients, such as the poor and those on multiple medications. Assessing interventions in isolation risks overlooking the main drivers of health care costs.

This approach was mistakenly taken in New Hampshire. There, to reduce state drug spend for a burgeoning budget, the Medicaid Prescription Drug List limited the number of psychiatric drugs available for patients. Unfortunately, unilaterally restricting access to these important medicines not only harmed patients, but also inflated overall health care costs as more patients were admitted to psychiatric hospitals as a result of untreated and worsening conditions.[7] The result was a large increase in overall costs to the New Hampshire Medicaid system.

Pfizer believes AHRQ should explicitly acknowledge that conducting comparative clinical effectiveness and outcomes research and analyses will not remove the need for difficult resource allocation decisions, it will only help inform policymakers and caregivers of the benefits of new technologies in the overall context of care. Comparative clinical effectiveness analysis cannot resolve the question of whether society should pay for a new technology. To determine whether a technology should be covered, it will be necessary to determine how much society is willing to pay for the health benefits derived from the technology. This decision cannot be resolved by looking exclusively at the benefits of one new technology as compared with another.

Finally, AHRQ must address the fact that the applicability of most comparative clinical effectiveness reviews will be extremely short-lived due to the rapidly evolving nature of clinical evidence and technology development. As new treatments emerge, comparative clinical effectiveness analyses must be constantly updated. Moreover, research done on prior incarnations of technologies may not be applicable. Pfizer recognizes, and AHRQ should acknowledge, that medical treatment is dynamic, and therefore static comparative clinical effectiveness analysis will be of no help – and may in fact be detrimental – to health care policy decision-makers.

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[7] Huskamp, HA, et al., "The Effect of Incentive-Based Formularies on Prescription-Drug Utilization and Spending," *New England Journal of Medicine*, Dec. 4, 2003

IV. Appropriate Standards for Evidence

Pfizer believes all outcomes research and comparative effectiveness research must be (1) transparent, (2) objective, and (3) consistent. The best research, no matter its use, must be easily shared, dissected and understood.

Transparency

AHRQ outcomes research and comparative effectiveness studies will only be beneficial to health care policy-makers if the studies are conducted and produced in a transparent manner. The best way to accomplish this goal is to require all AHRQ-funded researchers to publish the design of their studies, prior to implementation, for a public comment period.

Pfizer believes the recent interest of state, federal and private payers in processes that are not transparent and position comparative clinical effectiveness analysis as quality improvements highlights the need for AHRQ to have a transparent process for reviewing outcomes research and comparative effectiveness data. These entities are employing narrowly focused clinical trials to compare medicines and inappropriately conclude, absent direct contrary evidence, that the medicines are equivalent for entire populations and that restricted access to certain medicines is justified. The motives of those drawing these conclusions are often not acknowledged.

The need for transparency is further highlighted by the drug formulary experience. Managed care organizations have been using drug formularies for many years. However, the processes by which these organizations have made formulary decisions frequently lack transparency and scientific rigor. Generally, the pharmacy and therapeutics committees overseeing formulary decisions for a managed care organization determines which drugs should be covered based on scattered literature reviews, anecdotal information from physicians, and the price of the product. Frequently the broader health and economic consequences to the health plan or hospital and individual patient needs are not heavily weighted in these considerations.

While the concept of assembling comparative clinical information on products within a class is inherently attractive and for some purposes may be sound, designing the analysis as a cost-saving device introduces insurmountable bias and may be harmful to patients. Scientific evidence is routinely being misapplied by these entities to support elevating cost containment over appropriate care for individual patients. Adding such transparency will give a voice to physicians, patients, and other interested parties, and add to the ultimate credibility of legitimate cost and efficiency measures.

Objectivity

Pfizer believes AHRQ should establish guidelines to make certain all of the outcomes research and comparative effectiveness research it sponsors is objective. Specifically, to ensure objectivity, Pfizer believes it is imperative that: (1) AHRQ make certain that standards of evidence that impact access to care are defined independently of, and prior to, consideration of costs; (2) the interpretation and analysis of evidence is conducted by objective specialists and open to review (this also addresses the transparency issue above);

and (3) manuscripts should disclose funding sources. Pfizer believes allowing a period for review of studies by all stakeholders and establishing a process under which stakeholders could request a third party review would go a long way to ensuring objectivity.

Consistency

Unfortunately, studies on comparative clinical effectiveness and patient outcomes frequently contradict one another with regard to the economic benefits of a product or therapy. Studies have shown that one reason for this inconsistency is that the methods used to estimate comparative clinical effectiveness could differ widely based on the use of varying methodologies to complete the analysis.[8] Health care decision makers need to be assured that the information they are reviewing has been produced using an appropriate methodology and that the results are valid and consistent. No good is served by generating information if the validity of the information is called into question because the results of various studies are inconsistent due to varying methodologies or study techniques.

In particular, AHRQ must ensure that for all such research a consistent mechanism is used for capturing indirect costs. Indirect costs include hard to quantify items like change in productivity, increased quality of life and fewer side effects brought about by the intervention.[9] When comparing outcomes and comparative clinical effectiveness, inconsistent and/or non-transparent mechanisms for capturing these costs can easily skew results and preclude accurate and fair comparisons.

Finally, the impact of underutilization must always be considered. As a component of such efforts, AHRQ should look to the RAND/UCLA Appropriateness Method (RAM) when considering how to analyze the underutilization of treatments. The rationale behind the RAM method is that randomized clinical trials often are either not available or cannot provide evidence at a level of detail sufficient to apply to the wide range of patients seen in everyday clinical practice. Consequently, the RAM method combines the best available scientific evidence with the collective judgment of experts to yield a statement regarding the appropriateness of performing a procedure at the level of patient-specific symptoms, medical history and test results.

V. Targeting High Impact Areas

Pfizer believes additional research must be done on the comparative clinical effectiveness of treatments for the most costly disease states. We also believe it is essential to analyze and understand the underutilization of appropriate treatments by the patient populations most

[8] I.S. Udvarhelyi et al., "Cost-Effectiveness and Cost-Benefit Analyses in the Medical Literature: Are the Methods Being Used Correctly?" Annals of Internal Medicine 116, no. 3 (1992): 238–244; Neumann et al., "A Formal Audit of 228 Published Cost-Utility Analyses"; and K. Gerard, I. Smoker, and J. Seymour, "Raising the Quality of Cost-Utility Analyses: Lessons Learnt and Still to Learn," Health Policy 46, no. 3 (1999): 217–238.

[9] Koopmanschap MA, Rutten FF. A practical guide for calculating indirect cost of disease. Pharmacoeconomics. 1996; 10: 460-6.

susceptible to these diseases.[10] To that end, for each disease state studied by AHRQ, Pfizer believes it is essential to study the disparate treatment of the disease in minority populations.

Health care spending is highly concentrated. Between 1995 and 1999 the most costly 5 percent of Medicare beneficiaries accounted for 47 percent of total Medicare spending.[11] During the same time period, the most costly 20 percent of beneficiaries accounted for 84 percent of spending. By contrast, the least costly 40 percent of beneficiaries accounted for 1 percent of spending.[12] For this reason, Pfizer believes it is essential that AHRQ focus its studies on the most costly diseases as well as on populations that tend to not receive appropriate care and therefore live less healthy lives. Therefore, the following areas are recommended for study, in that almost 90 percent of beneficiaries in the top 5 percent of annual Medicare spending had at least one of the following:

1. Heart disease
2. Cancer
3. Chronic kidney disease
4. Diabetes
5. Maternal and child health
6. Respiratory diseases
7. Long term care
8. Mental Health

Heart Disease

The tremendous impact heart disease has on the U.S. health care system is well documented. The leading cause of death for men and for women in the United States, heart disease was responsible for over 700,000 deaths in 2000.[13] It was also the third leading cause of activity limitation. About 4.8 million Americans have heart failure, and 550,000 Americans develop heart disease each year.[14] The economic cost of heart disease is estimated to be $214 billion, including $115 billion in health care expenditures. Two of the most common heart diseases are coronary heart disease and heart failure.[15] About 12.6 million persons have coronary heart disease and over 1 million heart attacks occur each year.[16]

Cancer

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[10] Please note, per AHRQ's request, these comments focus on pharmaceutical utilization/underutilization. Pfizer does, however, believe that in addition to these areas, AHRQ should utilize MMA Section 1013 to study other areas critical to improving quality and efficiency, such as coordination of care, information technology and health care delivery processes.

[11] Pfizer believes AHRQ should devote more resources to the study of waste in the Medicare system. For example, a recently published article by Fisher, et. al, *The Implications of Regional Variations in Medicare Spending*, notes that the overutilization of certain services, caused by the skewed apportionment of physicians throughout the United States is causing a drain on the Medicare system.

[12] See "Congressional Report on National and Medicare Spending" at: http://www.medpac.gov/publications/congressional_reports/ Jun03DataBookSec5.pdf

13 National Heart Lung and Blood Institute. Morbidity and Mortality: 2002 Chartbook on Cardiovascular, Lung, and Blood Diseases. Bethesda, MD: NIH, 2002.

14 Id.

15 Id.

16 Id.

The second leading cause of death in the United States, cancer causes one in four deaths.[17] In 2003, approximately 1.5 million persons in the United States were diagnosed with cancer.[18] More than half of new cancer cases and cancer deaths can be attributed to four cancers: lung, colorectal, breast and prostate.[19] While cancer incidence rates have increased gradually in recent years, cancer death rates have declined because the medical community has made great strides in treating the disease. Perhaps most importantly to AHRQ, the economic costs of cancer are high: in 2002, total costs exceeded $171 billion, and direct costs for physicians, hospitals, and drugs exceeded $60 billion.[20]

Kidney Disease

Chronic kidney disease has been defined as structural or functional damage to the kidney with or without impairment of the kidney's ability to filter water and waste from the body.[21] Using this definition, 11% of the U.S. adult population has chronic kidney disease.[22] Of the nearly 19.2 million persons with kidney disease, 8.3 million have moderate impairment of the kidney function and almost 400,000 have End Stage Renal Disease (ESRD) requiring renal replacement therapy to sustain life.[23] Each year, almost 100,000 new ESRD patients begin treatment with either dialysis or renal transplantation, and about 70,000 ESRD patients, 19% of the total ESRD population, die. Expenditures of the ESRD program totaled over $19 billion in 2000, of which the Medicare program paid $14 billion.[24]

Diabetes

Diabetes afflicts over 17 million people in the United States, including 20% of persons over age 65. One million new cases of diabetes are diagnosed annually. [25] Diabetes was the sixth leading cause of death in 2003, when about 200,000 death certificates listed diabetes as an underlying or contributing cause of death.[26] Diabetes is also the leading cause of many other diseases including: blindness, non-traumatic lower extremity amputation and it increases the risk of heart disease, stroke, neuropathy, and complication of pregnancy. The costs of diabetes total about $132 billion, including over $90 billion in direct medical expenditures and about $40 billion due to lost productivity and premature death.[27]

Maternal and child health

17 American Cancer Society, Cancer Facts and Figures, 2003. Department of Health and Human Services, Centers for Disease Control and Prevention, National Center for Health Statistics, National Vital Statistics Report, Vol. 50, No. 16.
18 Jemal A, Murray T, Samuels A, Ghafoor A, Ward E, Thun MJ. Cancer Statistics, 2003. CA Cancer J Clin. 2003; 53:5-26.
19 Id.
20 National Cancer Institute. Bethesda, MD. http://seer.cancer.gov/csr/1973_1999/, 2002
21 National Kidney Foundation. Chronic Kidney Disease: Evaluation, Classification and Stratification. From NKF website: http://www.kidney.org/professionals/doqi/kdoqi/p2_background.htm
22 Coresh J, Astor BC, Greene T, Eknoyan G, Levey AS. Prevalence of chronic kidney disease and decreased kidney function in the adult U.S. population: Third National Health and Nutrition Examination Survey. Am J Kidney Dis. 2003; 41:1-12.
23 Obrador GT, Pereira BJ, Kausz AT. Chronic kidney disease in the United States: An underrecognized problem. Semin Nephrol. 2002; 22:441-448.
24 U.S. Renal Data System, USRDS 2002 Annual Data Report: Atlas of End-Stage Renal Disease in the United States, National Institutes of Health, National Institute of Diabetes and Digestive and Kidney Diseases, Bethesda, MD, 2002.
25 American Diabetes Foundation. Diabetes Fact Sheet: http://www.diabetes.org/main/info/facts/facts_natl.jsp.
26 Institute for Health care Improvement and the Health Services Resources Bureau. Health Disparities Collaboratives: Diabetes Training Manual. (Boston: IHI). 2002.
27 American Diabetes Association. Economic Costs of Diabetes in the U.S. in 2002. Diabetes Care. 2003; 26:917-932.

Childbirth and reproductive care are the most common reasons for women of childbearing age to use health care. With more than 11,000 births each day in the United States, childbirth is the most common reason for hospital admission.[28] In 2001, 11.9% of infants were born preterm, 7.7% were born with low birth-weight, including 1.4% with very low birth-weight.[29] Over time, rates of preterm birth and low and very low birth-weight have increased, although rates of infant mortality have decreased.[30] Comprehensive prenatal care can prevent complications of pregnancy and reduce neonatal mortality. Given that birth outcomes have effects that accrue over a lifetime, prenatal care is highly cost-effective.[31]

Respiratory diseases

Respiratory diseases cause activity limitation in 2.6 million persons. Annual costs of respiratory diseases exceed $116 billion, including $65 billion in health care expenditures.[32] Asthma, perhaps the most well known respiratory disease, affects about 15 million persons.[33] Each year, about 11 million persons experience asthma attacks and 5,500 persons die of the disease. Pneumonia, another common respiratory disease, is a leading cause of hospitalization among children and the elderly. Treatment costs for pneumonia in the United States exceed $9.7 billion.[34]

Long-term care

As the number of elderly Americans increases from 35 million in 2000 to an estimated 71 million in 2020, the need for long-term care is expected to increase.[35] Long-term care includes the provision of services at home, in the community, and in special facilities. In 1999, about 12,000 home health care agencies provided care to 8.8 million persons, about two-thirds of whom were aged 65 and above.[36] Nursing home care costs on average, $56,000 per person per year, and expenditures total almost $90 billion, about half of which is paid by Medicaid and Medicare.[37] It is estimated that in 2020 approximately 80% of nursing home residents are supported in part by Medicaid.[38]

Mental Health

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28 Institute of Medicine. Calling the Shots: Immunization Finance Policies and Practices. Washington, DC. National Academy Press. 2000.
29 Vintzileos, A.M., C.V. Ananth, J.C. Smulian, W.E. Scorza and R.A. Knuppel. 2002. The impact of prenatal care on neonatal deaths in the presence and absence of antenatal high-risk conditions. American Journal of Obstetric Gynecology. 186 (5): 1011-6.
30 Vintzileos, A.M., C.V. Ananth, J.C. Smulian, W.E. Scorza and R.A. Knuppel. 2002. The impact of prenatal care on neonatal deaths in the presence and absence of antenatal high-risk conditions. American Journal of Obstetric Gynecology. 186 (5): 1011-6.
31 Huntington J, Connell FA. For every dollar spent the cost-savings argument for prenatal care. N Engl J Med. 1994; 331:1303-1307.
32 Bell PD, Huson S. Equity in the diagnosis of chest pain: Race and gender. Am J Health Behavior. 2001; 25:60-71.
33 Rhew, D. Quality indicators for the management of pneumonia in vulnerable elders. Annals of Internal Medicine. 2001; 135: 736-743.
34 Rhew, D. Quality indicators for the management of pneumonia in vulnerable elders. Annals of Internal Medicine. 2001; 135: 736-743.
35 Goulding MR, Rogers ME, Smith SM. Public health and aging: Trends in aging – United States and worldwide. MMWR. 2003; 52:101-106.
36 Haupt BJ, Jones A. National Home and Hospice Care Survey: Annual summary, 1996. National Center for Health Statistics. Vital Health Stat 13(141). 1999.
37 AARP. Nursing Homes. Washington, DC: AARP, 2001.
38 Id.

The cost of untreated and mistreated mental illness is estimated at $113 billion annually.[39] The Journal of the American Medical Association in July 2003 reported that lost productivity stemming from depression alone totals $44 billion annually. However, the National Institute of Mental Health has shown that success rates of treatment for disorders such as schizophrenia (60%), depression (70-80%) and panic disorder (70-90%) surpass those of other medical conditions (heart disease, for example, has a treatment success rate of 45-50%).

VI. Health Care Disparities

As recently noted by AHRQ in its "National Health Care Disparities Report," health care disparities are costly.[40] Poorly managed care or missed diagnoses, which more regularly occur in certain populations result in expensive and avoidable complications. As discussed in a recent Institutes of Medicine Report, "to the extent that minority beneficiaries of publicly funded health programs are less likely to receive high quality care, these beneficiaries—as well as the taxpayers that support public health care programs—may face higher future health care costs."[41]

In addition to costs directly borne by taxpayers, unfortunate outcomes resulting from health care disparities may contribute to higher health insurance and malpractice premiums, both of which threaten the viability of our health care system. While difficult to quantify precisely, the costs of lost productivity and the indirect costs to families are probably very high. Thus, Pfizer believes the elimination of health care disparities, specifically the impact of underutilization of appropriate medications in certain populations and the comparative clinical effectiveness of appropriately treating these populations should be studied by AHRQ and should become a top public policy priority.

The following statistics outline the health care disparities issue for each of the disease states Pfizer believes should be studied by AHRQ:

[39] The National Mental Health Association, August 2003

40 http://qualitytools.ahrq.gov/disparitiesReport/documents/Report%207.pdf

[41] Institute of Medicine. Unequal Treatment: Confronting Racial and Ethnic Disparities in Health care. (2003) National Academies Press.

Heart Disease

Racial, ethnic, and socioeconomic disparities in cardiovascular care have been extensively reviewed and documented.[42] Specifically: (1) heart disease deaths are higher among African Americans compared with non-Hispanic whites; and (2) coronary heart disease is more prevalent among African Americans compared with whites and the prevalence among African Americans is rising while the prevalence among whites is falling.[43] In addition, coronary heart disease mortality is higher among African Americans compared with whites.[44]

Cancer

Cancer incidence and death rates vary by race and ethnicity. African Americans have a 10% higher cancer incidence rate and a 30% higher cancer death rate compared with whites.[45] Compared with whites Hispanics have higher rates of cervical, esophageal, gallbladder, and stomach cancer and Asians have higher rates of stomach and liver cancer.[46]

Cancer care varies by race, ethnicity, and socioeconomic status.[47] Differences in screening for and primary treatment of breast cancer, cervical cancer, colorectal cancer, early stage lung cancer and prostate cancer exist.[48] Research also indicates that there are both differences in follow-up care after diagnosis of breast cancer.[49]

Kidney Disease

Racial and ethnic minorities develop ESRD at a younger age and have rates of ESRD that are several-fold higher than whites.[50] Research has also indicated that racial and ethnic disparities in care for chronic kidney disease exist.[51] There are significant differences in the rate of referral to renal transplant centers, placement on a waiting list, timing of placement on a transplant waiting list, and receipt of a kidney transplant.[52]

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42 Okelo S, Taylor AL, Wright, Jr. JT, Gordon N, Mohan G, Lesnefsky E. Race and the decision to refer for coronary revascularization: The effect of physician awareness of patient ethnicity. J Am Coll Cardiology. 2001; 38:698-704.
43 National Heart Lung and Blood Institute. Morbidity and Mortality: 2002 Chartbook on Cardiovascular, Lung, and Blood DiseasesBethesda, MD: NIH, 2002.
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Diabetes

Significant racial, ethnic, and socioeconomic differences in diabetes have been observed. The prevalence of diabetes is higher among African Americans and Hispanics and among less educated persons.[53] African Americans, American Indians and Hispanics have higher diabetes death rates. African Americans also have higher rates of serious complications from diabetes, including higher rates of ESRD due to diabetes and higher rates of lower extremity amputation.[54] African American diabetics are less likely than white diabetics to receive patient education and more likely to be treated with insulin.[55]

Maternal and child health

There are significant racial and ethnic differences in birth rates. Non-Hispanic African Americans and Hispanics, have higher birth defect rates than non-Hispanic whites.[56] African American, American Indian, and Hawaiian mothers are more likely to have preterm, and low birth-weight compared to white mothers. Similarly, Hispanic mothers are more likely to have preterm infants but less likely to have low birth-weight compared with non-Hispanic white mothers.[57] During the first year of life, black infants are more likely to die than non-Hispanic white infants.[58]

Respiratory diseases

There are racial and socioeconomic differences in respiratory disease prevalence. For example, asthma is more prevalent among minorities and low-income persons, and asthma attack rates and mortality are higher among African Americans compared with whites.[59] Tuberculosis is highly concentrated in two populations: foreign-born persons and U.S.-born non-Hispanic African Americans.[60] Non-Hispanic African Americans account for almost half of all cases among U.S.-born persons. In addition, there are differences in influenza vaccination among Medicare beneficiaries and in management of asthma among managed care enrollees.[61]

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Long-term care

Studies indicate that racial, ethnic, and socioeconomic disparities in nursing home care exist, particularly with respect to differences in the management of pain and the receipt of rehabilitative services.[62]

Mental Health

Racial and ethnic minority groups are generally considered to be underserved by the mental health services system.[63] Despite the lack of care, the prevalence of mental disorders is estimated to be higher among African Americans, Hispanics and Native Americans than among whites.[64] Additionally, among adults, the evidence is considerable that persons from minority backgrounds are less likely to seek outpatient treatment in the specialty mental health.[65] Thus, AHRQ should examine the underutilization of mental health treatment by minority populations.

VII. Disease Management

Pfizer believes it would be advisable for AHRQ to further research the benefits of disease management. Disease management is "a system of viewing health care disease by disease and examining the interrelated elements in the treatment process with outcomes research to improve quality and lower costs."[66] We believe disease management systems should be in place for each disease state and should serve as the context for pharmaceutical outcomes research or comparative effectiveness analysis. This will enable policymakers to make economic and clinical choices between therapeutic alternatives.

Generally, the purpose of a disease management program is to: (1) establish best practices in care delivery for a condition; (2) improve outcomes (clinical, utilization, perceived well-being and satisfaction); (3) promote involvement between patients, practitioners and the health plan (e.g., case management); (4) implement interventions for common, costly, complex and chronic conditions; (5) promote early disease detection and diagnosis; (6) integrate prevention, wellness and disease management strategies across the continuum of care; and (7) minimize or forestall inpatient hospitalizations or emergency room visits.[67]

In order to accomplish the goals of disease management programs, the following components are frequently included as part of the program:

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[62] Gabrel CS. Characteristics of elderly nursing home current residents and discharges: Data from the 1997 National Nursing Home Survey. Advance data from vital and health statistics; No. 312. Hyattsville, Maryland: National Center for Health Statistics. 2000. Bernabei R, Gambassi G, Lapane K, Landi F, Gatsonis C, Dunlop R, Lipstiz L, Steel K, Mor V. Management of pain in elderly patients with cancer. JAMA. 1998; 279:1877-1882. Harada ND, Chun A, Chiu V, Pakalniskis A. Patterns of rehabilitation utilization after hip fracture in acute hospitals and skilled nursing facilities. Med Care. 2000; 38:1119-1130.

[63] Neighbors et al., 1992; Takeuchi & Uehara, 1996; Center for Mental Health Services [CMHS], 1998.

[64] Regier et al., 1993a

[65] Sussman et al., 1987; Gallo et al., 1995; Leong & Lau, 1998; Snowden, 1998; Vega et al., 1998a, 1998b; Zhang et al., 1998.

[66] See Castagnoli (1995).

[67] Burns, 1998

1. Systematic population identification - defining criteria for patient identification and implementing systematic processes for ongoing patient identification is essential for successful disease management.

2. Evidence-based practice guidelines - guidelines provide practitioners and patients with access to the most current accepted recommendations for clinical care.

3. Integrated, collaborative practice model - each member of the health care team is responsible for defining and delivering the best care in his/her area of expertise and for communicating and collaborating with the patient, family and all other health care team members.

4. Patient education - patient education must be ongoing, and educational information must be current and evidence-based.

5. Performance measurement - disease management programs should measure: (1) clinical data (e.g., mortality, morbidity and complications); (2) utilization; and (3) functional status.[68]

Pfizer believes that the success of the Pfizer/Florida-sponsored disease management program "Florida: A Healthy State" demonstrates the need for further study of disease management programs. Under the program, Pfizer has paired more than 16,000 of the highest risk; highest cost chronically ill patients with 50 specially trained care managers. These patients have received one-on-one attention and more than 28,000 home health aids, such as peak flow meters for asthmatics and blood pressure cuffs for hypertensives, to reinforce physician treatment and care recommendations and help better monitor their conditions. The demonstrated program success and behavioral and clinical results have lead to lower utilization of high cost inpatient services and emergency department visits for acute medical events. Overall hospital days of those under care management provided through this program declined 12% compared to those who were not under care management. For those who joined the program at inception, hospital days have declined over twice the rate as shown among all patients (27%).

Pfizer believes that only when a coordinated system of preventive, diagnostic and therapeutic measures is put in place can cost-effective, quality health care be provided to a patient population. Currently, organizational barriers obstruct the disease management perspective on treatment. Notably, coordination between health care entities (like a pharmaceutical company and HMO) about patient experiences is heavily regulated by the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), thereby making coordination of care between and among various health care entities more complex. However, ongoing industry changes, particularly the forthcoming entrance of pharmaceutical companies into the Medicare Discount Drug Card Program and the growing use of PBM services by HMOs, could help facilitate the development and use of disease management programs.

[68] Nelson, Mohr, Batalden & Plume, 1996.

VIII. Aligning Payment Policies With Quality Improvement Goals

Payment policies have a tremendous influence on how health care organizations and health care professionals deliver care and how patients select and use that care. These goals are relevant regardless of whether the ultimate purchaser of the care is an employer, insurer or patient/consumer. However, AHRQ should recognize that payers might misuse the agency's comparative clinical effectiveness and outcomes research to limit patient access to life saving drugs. The result is payment policies that may be at odds with quality goals.

Managed care organizations and pharmacy benefit managers, purporting to rely upon comparative clinical effectiveness research, have been developing methods for controlling prescription drug expenditures rather than achieving quality care and good patient outcomes. While the use of such data may, in fact, lower cost for the insurer, it also limits the therapeutic alternatives available to physicians treating patients. This blurring of the boundary between clinically and economically driven decisions poses measurable risks to patients.

The growing popularity of tiered formularies presents an example. Tiered processes act as a mechanism to shift cost from payers to patients but are often justified on clinical grounds, implicitly conveying to patients and physicians that the more expensive medicine is unjustified. These formularies, "regardless of the particular details…lead to higher out-of-pocket costs to consumers and to some patients' going without prescription drugs."[69] As noted, related concerns about opaque economic conflicts apply to various cost control strategies including cost driven treatment guidelines and prior authorization policies.

While it is understood that payers desire to contain costs, these policies can result in inappropriate medication usage patterns, which can be associated with higher total health care costs and poorer clinical outcomes. These practices beg the question, "What is the evidence that restricted drug access policies do not harm patients?" Healthcare expert Dr. S. B. Soumerai has recently attempted to answer:

> Given the rapid increase in the use of PA [prior authorization] policies and other cost-control mechanisms in Medicaid, the relative lack of data on their risks and benefits is cause for concern. It is sobering to realize that if such policies were considered for a clinical study, the possible risks of reduced access to essential medicines would likely result in failure to obtain human-subject approval from most institutional review boards (IRBs). These policies can be viewed as massive experiments on vulnerable populations.[70]

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[69] Thomas, CP, Incentive-Based Formularies, *NEJM*. 349:23, December, 4 2003; 2186.
Huskamp H, Deverka PA, Epstein RS, McGuigan KA, Frank RG (2003). The effect of incentive based formularies on prescription drug utilization and spending. *NEJM*, 349(23): 2224-2232.
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[70] Soumerai SB, "Benefits and risks of increasing restrictions on access to costly drugs in Medicaid," Health Affairs. 2004; 23: 141

Indeed, the risks of bad decisions in restricting pharmaceutical access threatens to exacerbate what is already a one of the most serious problems in achieving quality in health care: drug underutilization. The most recent report of the RAND group's comprehensive Assessing the Care of Vulnerable Elders (ACOVE) project, *The Quality of Pharmacologic Care for Vulnerable Older Patients*, suggests that underutilization of medications is one of the most important factors in addressing quality care in the older population.[71] ACOVE presents in-depth findings pertaining to the 43 quality indicators that address medication treatment. Among other important findings:

- ?? Vulnerable elders with chronic conditions are not prescribed recommended medications 50% of the time.
- ?? Education about prescribed medications occurred only 81% of the time.
- ?? Medication monitoring occurred only 64% of the time.

Clearly these results indicate a gap in quality when it comes to quality medication care, which would only be worsened by inappropriate reliance on cost shifting to patients. The lesson is not that cost containment strategies are necessarily bad, but instead that they need to be explicitly identified with concurrent processes instituted to detect and avoid negative outcomes, and should not be weighted against pharmaceuticals, which are often the best choice in terms of quality care.

Moreover, Pfizer believes that payment for health care technologies must encourage the development of novel methods to improve the value obtained for the resources expended. The research and development of new prescription drugs is extremely costly because most new drug candidates fail to reach the market.[72] Typically, less than 1 percent of the compounds examined in the pre-clinical period make it into human testing.[73] Only 20 percent of the compounds entering clinical trials survive the development process and gain FDA approval.[74] Furthermore, the full research and development process from synthesis to FDA approval involves undertaking successive trials of increasing size and complexity. The pre-clinical and clinical testing phases generally take more than a decade to complete and cost more then $800 million.[75] In order to encourage companies to undertake this long and costly process, it is essential that payers not limit patient access to new and novel drugs, which often provide substantial leaps in terms of quality patient care.

IX. Conclusions and Recommendation

Pfizer believes all AHRQ research should work towards the goals of making healthcare available to everyone, affordable to all tailored to the individual information driven and oriented towards prevention. Additionally, Pfizer believes the patient's health must come first, the physician-patient relationship is essential and would not be compromised, and health care financing and payment levels must ensure quality and innovation. As noted, to ensure quality care for American patients, Pfizer believes AHRQ research must be highly

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[71] Higash, T., *et. al, The Quality of Pharmacologic Care for Vulnerable Older Patients,* Ann. Intern. Med. 2004: 140:714-720.
[72] Kenneth I. Kaitin and Joseph A. DiMasi, "Measuring the Pace of New Drug Development in the User Fee Era" 34 *Drug Information Journal* (2000) 673–80.
[73] Id.
[74] Id.
[75] Id.

transparent, objective, and consistent. Moreover, the agency should focus its research on the analysis of all available treatments for the most costly health conditions as well as the underutilization of appropriate treatments by patient populations most susceptible to these diseases. Specifically, Pfizer recommends that AHRQ study the following eight categories of health care: (1) heart disease; (2) cancer; (3) chronic kidney disease; (4) diabetes; (5) maternal and child health; (6) respiratory diseases; (7) long term care; and (8) mental health. Pfizer also believes AHRQ should research the affect disease management programs could have on each of these conditions.

Finally, to enhance the quality of care, AHRQ must ensure that its studies are not misapplied to support inappropriate cost-containment strategies, which undermine patient care and beneficial innovation.

Pfizer thanks AHRQ for this opportunity to provide comments, and looks forward to working with the AHRQ as it implements MMA Section 1013.

Respectfully submitted,

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