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Kara Karlson

American University Washington College of Law

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THE COST OF LIVING: ADAPTING THE COMPARATIVE EFFECTIVENESS APPROACH TO HEALTH CARE COVERAGE FOR TERMINAL PATIENTS

Kara Karlson*

I. Introduction

“Comparative effectiveness” is a relatively new buzzword in health care,¹ gaining national attention from the recently enacted health care reforms and provisions in the American Recovery and Reinvestment Act of 2009 (ARRA). The more traditional pricing model in the United States calculates charges on an objective fee-for-service model.² Comparative effectiveness, however, relies on qualitative assessments of the patient’s quality of life, probability of a positive outcome, and the general burden on society. Although qualitative comparative effectiveness models are a reasonable way to control costs at some level (e.g., using physician assistants to perform annual physicals), once the care or the treatment becomes more advanced and specialized, the model begins to crack. Furthermore, the model that relied on more cost effective alternatives and standardized care falters if more lethal conditions are considered and patients are simply more likely to die than survive because of the serious nature of their diseases. The recent emergence of a subjective standard to evaluating health care coverage based on “quality of the outcome” begs the question—whose opinions will determine a treatment’s “effectiveness” and what are the desired results of the treatment?

Comparative effectiveness under the new health care reform legislation factors cost into health care decisions in a way that could be inimical to patient care under bleak circumstances. Systems similar to the British National Health Service, which narrow access to medicines by denying drugs to entire populations of disease sufferers or by limiting the amount of medication available for specific diseases,³ will have a disparate impact on the elderly and the poor, particularly if applied in a system that stills prefers private insurance. For example, the United Kingdom denies a treatment for macular degeneration (a condition that ultimately leads to blindness if untreated) to most patients. When the health system’s regulatory body approves the treatment’s usage, it will only pay for

enough medication to treat one eye.⁴ Independently wealthy persons who can afford the cost of treatment at their own expense and those who can afford to invest in private insurance will most likely maintain their sight, whereas those without access to this medication, due to the health care system’s cost containment biases, are left blind.⁵ This disparity is even more pronounced with terminal diseases, as families lose precious time with their loved ones when the government will not cover life-extending treatments, such as cancer drugs, due to cost.

This article will explore how using comparative effectiveness and disease management as cost containment measures poses an especially egregious threat to patients with extremely aggressive diseases, and can tie the hands of their health care providers. Part II will provide a basic background and definition of comparative effectiveness, as well as examples of international implementation of comparative effectiveness models. The article present the difficulties inherent in equitably applying a comparative effectiveness model to the current health insurance and Food and Drug Administration drug approval regimes. Part III will address how the potential conflict between cost containment and quality care has the propensity to harm some of the most disadvantaged members of our society, particularly those with terminal diseases. Specifically, the article argues that comparative effectiveness should balance the potential harms with the possible benefits of treatment, similar to the current expedited approval process for drugs, as opposed to some of the more aggressive comparative effectiveness cost containment schemes enacted in other countries. Part III.B analyzes the exacerbating impact of cost containment measures on the pre-existing funding biases, including lack of access to specialized care for the extremely ill and the poor. Finally, this Article concludes that, although expensive, treatment for terminal patients is necessary and denying viable alternatives due to inadequate funding violates the Patient Protection and Affordable Care Act (PPACA).

II. Background

While recent health care developments have thrust discussions of comparative effectiveness into the spotlight, comparative effectiveness actually is as old

* J.D. Candidate, May 2011, American University, Washington College of Law; M.B.A. Candidate, May 2012, American University, Kogod School of Business; B.A., Journalism, 2007, University of Arizona.

“PPACA must mitigate its emphasis on cost containment in extreme cases, specifically rare and orphan diseases, in favor of providing treatment.”

as the study of medicine. Comparative effectiveness now is defined as:

Comparison of the *effectiveness* of the risks and benefits of two or more health care services or treatments used to treat a specific disease or condition (e.g. pharmaceuticals, medical devices, medical procedures and other treatment modalities) in approximate real-world settings.⁶

Even a small town family doctor is in a position to make these types of judgments during the day-to-day workings of his practice. However, the primary concern about comparative effectiveness is not when science indicates that a certain treatment produces the best result, which is obviously helpful to aid patients and their families in making the best decisions and reining in the runaway costs of health care. Rather, the most pressing danger comes when these efficiencies determine coverage specifically to control costs.⁷

The comparative effectiveness directive in the health care reform package, including the funding provision in ARRA creating an institute to study systematically comparative effectiveness, strongly indicates that cost containment will be a consideration in determining funding and coverage, despite statutory requirements to the contrary.⁸ The Congressional Budget Office’s (CBO) study on comparative effectiveness stated that one of its objectives was to link the information from the study to financial incentives as the appropriate method to change the behavior of health care providers and consumers.⁹ Although the focus on research to determine the best treatments for patients is laudable,¹⁰ the possibility of being denied life-prolonging treatment due to cost is disconcerting.

The reality of comparative effectiveness as a cost containment measure instead of a process to develop more efficient treatment, evolved in countries where the government is the primary third-party payer. For instance, the British government created the National Institute for Clinical Excellence (NICE) in 1998 after a lawsuit filed by Pfizer in response to the government limiting the use of Viagra over cost concerns.¹¹ Sometimes the U.K. taxpayer is able to challenge the denial of coverage successfully,¹² but often the determination refusing coverage is final.¹³ For example, NICE refused to cover the breast cancer drug lapatinib in certain cases of metastatic breast cancer.¹⁴ NICE predicted the treatment would give the particular patients an average of an additional ten weeks of life.¹⁵ NICE, however, does not pay for it because the pill costs about £25,000 per year, despite the fact that manufacturer GlaxoSmithKline agreed to pay for the first twelve weeks of each treatment.¹⁶ More than 3,000

cancer patients in the United Kingdom had to request money from their local primary care trusts (PCT) as an alternative funding method to NICE; more than 1,000 of those patients with breast cancer had their claims rejected by the PCT as well.¹⁷ As of December 2008, the life of a person in the United Kingdom was rarely worth more than £22,750 semi-annually.¹⁸

If similar cost containment procedures are enacted in the United States to control government health care spending, millions of people—particularly the elderly and the underserved—could face strict bureaucratic obstacles when attempting to obtain treatment for terminal diseases. The FDA has defined several groups of diseases that typically result in significant disability or death for more aggressive approval procedures, including “rare diseases” and “orphan diseases.” Rare diseases are diseases that affect fewer than 200,000 people in the United States, or one in fifteen hundred people.¹⁹ Orphan diseases include diseases for which there is no reasonable expectation that the cost of developing the drug will be recouped through sales in the United States. Orphan diseases also encompass most rare diseases, and others that are aggressive and have stymied efforts to find a cure.²⁰ These diseases, which comprise many forms of highly lethal cancers, would be at the bottom of the funding list under the plain language of the comparative effectiveness regime in the statute, and might not be covered at all under the more stringent NICE provisions.²¹

Comparative effectiveness addresses essentially two issues: cost and treatment outcomes. The goal of comparative effectiveness is to use science to determine the best treatment for a patient or groups of patients. One of the ways to determine which treatment is “best” is to compare the cost and outcome of one treatment to another.²² The better the outcome and the lower the cost, the better the treatment or professional service. Similarly, there are two primary types of costs: professional services including the technical services that assist the provision of professional care, and prescription medicine. Both types of costs have increased considerably over the last few decades. Third-party payers, both public and private, have implemented a number of techniques to try to minimize costs.²³

Third-party payers in the private sector use a variety of methods to control costs, like precertification or preauthorization and utilization review. To promote or deter the use of certain drugs, insurance companies provide different levels of financial incentives on multi-level formularies.²⁴ Private insurance cost containment measures, however, are different from

government-run cost containment measures for two reasons: 1) although an insurer may make a treatment more expensive, it is unlikely to disallow completely treatments deemed necessary by a doctor; and 2) patients have a choice of insurance providers, albeit a choice limited by a number of factors. Finally, private companies generally respond more quickly and more forcefully in response to campaigns against the company.²⁵ These differences between private insurance and the new government-created health care program lessen the finality of the decision to cover a drug, treatment, or physician, thereby mitigating the harm to terminal patients by providing alternatives.

A final consideration when implementing a cost effectiveness structure for the health care system in the United States is that the FDA does not consider cost when deciding whether to approve a drug.²⁶ A direct correlation, however, exists between federal funding for research and positive outcomes for fatal disease. HIV has evolved in the last few decades from a death sentence to a more manageable, chronic condition.²⁷ Even metastatic breast cancer survival rates have improved dramatically, from a ten-percent five-year survival rate in 1974 to forty percent in 2000.²⁸ Causative factors, genetic predisposition, disease mechanisms, and possible curative methods are unlikely to be discovered without the lifeline of federal research dollars.²⁹ Unfortunately, the health reform law's focus on treatments that present "the potential for new evidence to improve patient health" and bias towards research that will have an "effect on national expenditures associated with a health care treatment" is likely to have a negative effect on federal funding of clinical trials for the most desperately needed therapies.³⁰ The FDA has acknowledged that more aggressive diseases might require more aggressive treatment,³¹ and that implementation of PPACA should include guidelines to ensure that extremely lethal conditions receive adequate funding in a non-arbitrary manner.³²

III. Analysis

A. Health Care Providers Should Use a Sliding Scale to Balance Seriousness of Disease, Alternative Treatments, and Cost

PPACA's health care provisions apply the concept of 'burden to society'—both in terms of disease prevalence and economic cost—to determine which treatments are more effective.³³ This application presumably will adversely affect coverage for drugs that are not "comparatively effective" from a prevalence and cost standpoint, and will most likely lead to a chilling reduction of research funds. Research

funds will be jeopardized at both the public and private level. Private companies will not want to invest time and money creating a product that third-party payers will not cover. Federal funds will also be limited. Although the comparative effectiveness model, in its current form under PPACA, seeks to help a large amount of people and not strain the budget,³⁴ the proposed system will limit coverage of therapies and stymie research and development, with potentially devastating effects to patients.

In contrast, over the past fifty years, the FDA has balanced varying interests from public safety to industry concerns.³⁵ The system is far from perfect; it takes a new drug, on average, more than ten years and hundreds of millions of dollars to get to market.³⁶ After decades of being at one extreme or the other, however, the FDA's dogged pursuit of the optimal balance of safety, effectiveness, and in extreme cases, *need*, is bringing the regulatory framework to an appropriate equilibrium.³⁷ The FDA's attempt to reach out and include typically underrepresented patient groups through programs like the Rare Diseases Initiative advances patient care by ensuring that rare but devastating diseases are not ignored. Expedited approval for medication to treat diseases that are highly lethal and for which no alternative treatments are available provides a flexible regulatory framework and offers a glimmer of hope for patients devastated by disease. The FDA also developed a process to provide individuals or groups of individuals access to a drug in the early testing phase in extremely grim cases.³⁸ These alternative initiatives are useful examples of federal bureaucracy working with patients and their doctors to provide better health care while still adhering to the principles of general safety and effectiveness that are at the core of the regulatory system.

The approximately thirty-two million people who are projected to be covered under PPACA's health care expansions will most likely be predominantly Medicaid recipients and the already uninsured.³⁹ These people are less likely to have the formal training necessary to dispute a claim denial effectively and will generally not have the option of obtaining better insurance through an employer. Further, the elderly are much more likely to have poor health, and aggressive forms of cancer in particular, that will make them more reliant on the quality of their health care program than the average middle-aged health care consumer.⁴⁰ Although this coverage will benefit the traditionally under-served when it comes to basic, traditional medical care, these patients, if diagnosed with a terminal illness, are also the most likely to be uninformed of the coverage limitations and have the fewest opportunities to argue successfully for appropriate care and services

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These limitations have similar effects to some of the behaviors criticized as predatory lending during the recent subprime mortgage crisis. In an effort to get people who could not afford a home into one, mortgage brokers would arrange for interest only, low or no down payments, or adjustable rate mortgages.⁴¹ Many people had no problems paying their monthly mortgage bills, as long as the stock market was performing well.⁴² Analogous, most people would benefit from the expansion of health care coverage, as long as their health status remained in good condition or there were standard treatments for their condition. In the subprime mortgage crisis, once the system came under tremendous pressure from either rising interest rates or a balloon payment coming due, the asset of home ownership became a liability, and the effort to provide the “American dream” to everyone attacked home prices and caused the entire economy to falter.⁴³

The systemic dangers faced in the home loan market potentially augur what could happen if PPACA adopts a more stringent cost containment stance, similar to NICE, instead of adopting a more flexible regulatory framework, like the FDA. The provision of routine health care to the elderly and the poor, like the goal of home ownership, is a laudable one. Cost containment measures work readily with routine care. Utilization of physician assistants (PA) is one cost saving measure that can provide high quality patient care by developing stronger relationships with patients due to the lower cost of a PA’s time, while maintaining the more specialized oversight of a physician.⁴⁴ Most patients in good health can benefit from the same general advice—eat healthy foods, exercise, get regularly screened, monitor blood pressure, and see a physician annually.⁴⁵ Once the “balloon payment” of a serious diagnosis enters the equation, however, the standard treatment regimen is no longer sufficient. Not only will standard cost containment measures no longer provide an adequate guide for the necessary specialized care, but expert physicians are also harder to find, and treatments are fewer and much more expensive.⁴⁶

Generally, the young, healthy or rich are not the ones who suffer under any third-party payer system due to lack of access or extreme cost. Accordingly, PPACA health care envisions supporting the elderly, ill, unemployed and impoverished. PPACA must mitigate its emphasis on cost containment in extreme cases, specifically rare and orphan diseases, in favor of providing treatment.⁴⁷ Regulations should be enacted now to ensure that PPACA’s vision of quality, efficient health care is realized. Lessons from the FDA’s attempts to provide access to experimental medication, and the inequity of access which resulted from those measures,

are instructive to the PPACA framework, specifically in balancing comparatively effective treatment and optimal patient care.

Some of the provisions of the FDA rules implementing expedited approval are not directly applicable. For example, since PPACA should only cover treatments that are operating under the auspices of the NIH, no additional safety protocols or studies are necessary.⁴⁸ The requirement that the treatment show at least preliminary clinical effectiveness or therapeutic benefits, however, should be rated using a balancing scale to ensure that only appropriate treatments and drugs are covered.⁴⁹ The FDA’s definition of life-threatening disease—one where the likelihood of death is high unless the course of the disease is interrupted⁵⁰—could readily be adopted to determine when comparative effectiveness measures should appropriately be relaxed. The strict focus on survival as the only outcome worth funding should be avoided.⁵¹ At this point in health care technology development, many diseases and conditions result in fatality. Several factors should be considered when determining whether the cost is worth the treatment, including survivability and the impact on the day-to-day quality of life of the patient. The latter is particularly important in terminal cases.

This balanced approach would help realize the vision of access to affordable health care, while minimizing some of the most widely criticized pitfalls of other systems. For example, if NICE implemented a more flexible comparative effectiveness framework, women with terminal, metastatic breast cancer would have access to lapatinib, a better therapy that would provide women with an average of ten more weeks with their families and reduce their cancer-associated pain.⁵² This result is especially effective and equitable considering that the manufacturer is willing to pay for twelve weeks of treatment per patient.⁵³ Furthermore, NICE coverage limitations were originally intended to cap the runaway costs of non-life saving drugs like Viagra, not life-extending chemotherapy.⁵⁴ At the same time that this proposed regulatory framework would ensure lifesaving treatments to terminal patients, it could also provide comparatively effective options for more people. To continue with the breast cancer example, some studies indicate that mammograms should be given at a later age and at a lower frequency in patients without elevated risk factors.⁵⁵ Providing flexibility by adopting some of the progressive standards promulgated by the FDA under PPACA could enforce the cost saving measures of fewer mammograms for the general population, while giving the handful

of women with terminal metastatic breast cancer additional valuable time with their loved ones.

B. The Lessons Learned by the FDA in its Attempts to Provide Equal Access to Investigational Drug Studies Should be Used by Other Agencies to Ensure that Pre-existing Biases Against Rare and Orphan Drugs are not Exacerbated Under PPACA

One of the primary concerns the FDA addressed when altering the rules to the “fast track” program was the unequal distribution of access to investigational drugs to rural areas and other underserved populations.⁵⁶ Most of the investigational trials were conducted at large, research-based, academically-related hospitals, not accessible by rural and poor patients.⁵⁷ Serious illness can transform a straightforward fifty-mile trip into a treacherous and expensive affair. A high percentage of terminally ill patients are unable to transport themselves to specialized care centers.⁵⁸

In addition to the inequities the FDA tackled while implementing the fast-track process, the NICE program illustrates the likely inequities that would result from implementing the comparative effectiveness model. Approval for federal funding and patient care coverage is more likely for prevalent diseases and diseases with organized advocacy groups than smaller or unorganized lobbying groups. NICE has spent £21 billion on outreach and other attempts to equalize treatment, while denying tens of thousands of U.K. patients treatments considered the standard care in the United States and continental Europe.⁵⁹ Despite the creation of Life Sustaining Protocols to provide more options for terminal patients under NICE, thousands of patients are denied life-extending coverage every year.⁶⁰ For example, NICE determined that a drug called Sutent would double the life expectancy of kidney cancer patients compared to the alternative treatments, but average was denied. After an uproar over the denial of coverage of four kidney cancer drugs, including Sutent—all denied after the Life Sustaining Protocols were implemented—NICE approved the drug.⁶¹ The manufacturer of one of the denied drugs, Bayer, even offered to provide the treatment for free to British patients, but NICE refused to approve it.⁶² In contrast, most private insurers in the United States cover these treatments.⁶³

These inequities will lead to longer survival rates for some diseases, and shorter survival rates for others. The five-year survival rate for breast cancer in the United Kingdom is seventy-seven percent, while the five-year survival rate for prostate cancer is only seventy percent.⁶⁴ In comparison, the five-year survival rate

for breast cancer in the United States is ninety percent and more than ninety-percent for prostate cancer.⁶⁵ Approximately two hundred thousand people are diagnosed with breast or prostate cancer in the United States each year,⁶⁶ and survivability for both these diseases is similar despite a large disparity in research funding in the United States, due primarily to the size and organization of the different advocacy groups.⁶⁷

The primary reason for the more favorable coverage and treatment alternatives for breast cancer is the organized lobbying effort, and that is unlikely to change if a comparative effectiveness model is implemented in the United States. Breast cancer already has much higher survivability rates, even in the metastatic stages, than some of the worst cancers in the early stages.⁶⁸ Under the plain reading of the PPACA statute, this would tilt the bias in favor of more funding toward clinical research for breast cancer because it affects a larger number of people and has better chances for treatment. That bias is further exacerbated by the huge lobbying efforts promoting breast cancer awareness and research. In 2007 alone, the National Cancer Institute dedicated \$572.4 million to breast cancer research, the National Institutes of Health gave another \$705 million, and the Department of Defense set up its own breast cancer research facility with another \$138 million.⁶⁹ Pancreatic cancer, which is diagnosed in about forty-thousand people annually, received a mere \$73.3 million in federal funding despite killing almost the same number of people per year as breast cancer.⁷⁰

A rigid comparative effectiveness regulatory regime will intensify pre-existing funding biases at work against the elderly and the very ill by determining what coverage millions of Americans will be able to afford. Removing thirty-two million people, particularly those more likely to suffer from terminal diseases, from the pool of people who can access certain types of treatment will have a chilling effect on the research and development of new therapies for orphan diseases. Obtaining a sponsor and approval for orphan drugs is already very difficult, despite programs developed by the FDA specifically to encourage the development of tools to fight these devastating conditions.⁷¹ Implementing a system of cost-conscious comparative effectiveness militates against treatments that will help a smaller percentage of the population, which will amplify the pre-existing market biases against the development of orphan drugs.

It is contradictory to reimburse federally the costs associated with a clinical trial (excepting the cost of the drug and administrative fees), while thwarting the potential for federal research funding during the pre-trial and post-approval phases. Medicare currently

“Federal agencies can ensure enhanced equity of access both by pursuing the standards already used by the FDA to encourage orphan drug development and by setting aside comparative effectiveness funding specifically for terminal and difficult-to-treat diseases.”

reimburses for clinical trials and investigative studies of cancer treatments and diagnostic tools that are in the early stages of the testing process to obtain FDA approval.⁷² Given that Medicare/Medicaid spending currently accounts more than forty percent of all health care spending and that PPACA will add to that number, the potential to profit from drugs that only extend life to some degree or only work for certain patients would significantly decline under a NICE-style framework.⁷³ Instead, implementing agencies should recognize that the current system attempts to incentivize work on orphan drugs and should promulgate regulations that further this aim instead of hindering it.

The goal of incentivizing the creation of drugs and therapies to treat more rare and fatal conditions can be realized by adopting a view of equal access that recognizes the value of treatments for terminal diseases. Under the standard comparative effectiveness model, rules that guarantee funding for rare and orphan drugs and acknowledge that the results of the study (e.g. providing ten more weeks of life) might not ultimately result in a cure, would spur research and development in those areas. The regulatory framework developed by the FDA, such as the definitions for orphan drugs and rare diseases, should be applied to research funding decisions to provide a regulatory exception to spur development for treatment of orphan drugs and rare diseases.⁷⁴

Absent these safeguards, access will depend on the strength of a particular disease's lobbying and organizational efforts, and the potential for positive, cost effective outcomes. This result would increase the bias in the health care industry, where the patients most in need of treatment that could bring more comfortable final days receive less funding than the patients with a disease that has a strong pro-research lobbying effort and existing effective treatments. Equal access is an important goal that should be fostered by PPACA, not hindered by it. Federal agencies can ensure enhanced equity of access both by pursuing the standards already used by the FDA to encourage orphan drug development and by setting aside comparative effectiveness funding specifically for terminal and difficult-to-treat diseases.

IV. Conclusion

PPACA will require massive resources to implement, and regulatory provisions are still forthcoming.⁷⁵ It remains unclear how regulators will determine which treatments are covered and how much of a role containment cost will play. Statutory language emphasizes that cost is at least one consideration for the provision of treatment and research funding.⁷⁶ The British experience indicates that the dedication to more efficient patient care can evolve into a discrete dollar value on the lives of people.⁷⁷ The rhetoric used to dismiss PPACA's comparative effectiveness provisions as "death panels" not only refused to acknowledge the usefulness and dire need for cost containment measures in general care, but also did nothing to advance the needs of patients in the most desperate circumstances.⁷⁸ PPACA did not establish death panels,⁷⁹ but regulatory agencies will be tasked with some very difficult decisions, including how to distinguish between standard care, where more rigid cost containment measures are appropriate, and when cost concerns must give way to the value of life.

The FDA has evolved from a small agency focused solely on the safety of so-called medications to a health care behemoth that has ensured the

safety and efficiency of drugs through many political administrations, emerging diseases, and technologies. The fairly recent developments of balancing safety and efficacy with patient autonomy has, for the most part, enriched those patient's lives and scientific understanding of different disease processes and treatments. The measured approach that attempts to balance competing factors like the harshness of a disease, while ensuring that patients are not buying an unknown quantity, is a model on how to reconcile the competing aims presented by comparative effectiveness.

Creating a more inclusive framework will also alleviate the biases already present in the system, which incentivize research in fields where the hope of a good outcome is more realistic and limit the access of the poor and ill. Devastating illness, poverty, and old age are often linked.⁸⁰

The expanded PPACA programs—intending to cover the thirty-two million people who are uninsured due to unemployment, lack of employer-provided coverage, and a number of other reasons—should consider the failed attempts in the United Kingdom to ensure equitable coverage.⁸¹ Also instructive are the processes developed by the FDA in the United States to minimize disparities in care that can result after a rare disease diagnosis.⁸²

The elimination of waste in government spending is important, but the government should not alleviate that concern at the expense of lives. Patients and their families should not have to endure anguish in addition to a devastating diagnosis because better therapies are available but too expensive to cover. The state has always asserted an interest in protecting human life at all stages, from fetal development to end of life decisions.⁸³ This assertion of an interest in human life does not come without a cost, but for a dying person, an extra six months of life is priceless.

¹ *Buzzword: Comparative Effectiveness*, CONSUMER REPORTS HEALTH BLOG (Feb. 10, 2009), <http://blogs.consumerreports.org/health/2009/02/comparative-effectiveness.html> (last visited July 13, 2010).

² *InvestorWords.com*, http://www.investorwords.com/5569/fee_for_service.html (last visited July 13, 2010). See also Anthony L. Schlaff, *A must for health care reform: End fee-for-service medicine*, CHRISTIAN SCI. MONITOR (Sept. 10, 2009), <http://www.csmonitor.com/Commentary/Opinion/2009/0910/p09s01-coop.html> (opining that health care costs can only be limited by revising the traditional fee-for-service model that encourages more tests).

³ *Of NICE and Men*, WALL ST. J., at A14 (July 7, 2009), <http://online.wsj.com/article/SB124692973435303415.html> (discussing NICE's policy of severely restricting access to macular degeneration drugs).

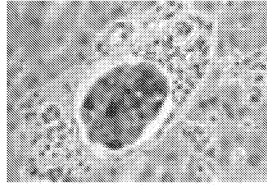
⁴ *Id.*

⁵ *Id.*

⁶ ACADEMY HEALTH, *A FIRST LOOK AT THE VOLUME AND COST OF COMPARATIVE EFFECTIVENESS RESEARCH IN THE UNITED STATES*, 3 (June 2009), http://www.academyhealth.org/files/FileDownloads/AH_Monograph_09FINAL7.pdf.

⁷ See *id.* (listing a number of reasons, including cost containment, that the effectiveness studies can be used). See also CONGRESSIONAL BUDGET OFFICE, *RESEARCH ON COMPARATIVE EFFECTIVENESS OF MEDICAL TREATMENTS: ISSUES AND OPTIONS FOR AN EXPANDED FEDERAL ROLE*, 28 (Dec. 2007), <http://www.cbo.gov/ftpdocs/88xx/doc8891/12-18-ComparativeEffectiveness.pdf>. (focusing solely on the potential cost savings through comparative effectiveness).

⁸ *Compare Patient Protection and Affordable Care Act of 2010*, Pub. L. No. 111-148, § 1181(a)(2)(A), 124 Stat. 727, (defining comparative effectiveness as comparing cost and health outcomes), with *Of NICE and Men*, *supra* note 3 (contrasting the original aims of NICE as "ensur[ing] that every treatment, operation, or medicine used is the proven best" with the board's tendency to limit care based on cost and probable outcome).



⁹ See CONGRESSIONAL BUDGET OFFICE, *supra* note 7.

¹⁰ Judith Graham, *Effective medical treatment: \$1.1 billion to study "comparative effectiveness" research and evaluate therapies*, CHIC. TRIB. (Apr. 24, 2009), <http://www.chicagotribune.com/news/local/chi-health-what-works-intro-24-apr24,0,3312703.story>.

¹¹ Gardiner Harris, *British Balance Benefit vs. Cost of Latest Drugs*, N.Y. TIMES (Dec. 2, 2008), http://www.nytimes.com/2008/12/03/health/03nice.html?_r=1.

¹² Jenny Hope, *Cameron vows Tories will give cancer patients drugs rejected by rationing body*, THE DAILY MAIL (U.K.) (Apr. 3, 2010), <http://www.dailymail.co.uk/health/article-1263223/NHS-rationing-body-denies-15-life-saving-drugs-cancer-patients.html#ixzz0thIhRDpT> ("The watchdog totally blocked a quarter of the cancer drugs made available since 2008 and heavily restricted others—despite Government promises to make more treatments available.").

¹³ See, e.g., NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE, BEVACIZUMAB (FIRST-LINE), SORAFENIB (FIRST- AND SECOND-LINE), SUNITINIB (SECOND-LINE) AND TEMSIROLIMUS (FIRST-LINE) FOR THE TREATMENT OF ADVANCED AND/OR METASTATIC RENAL CELL CARCINOMA 5, 20 (Aug. 2009), <http://www.nice.org.uk/nicemedia/live/12220/45232/45232.pdf> (refusing to cover a number of different therapies for kidney cancer, a disease with a five-year survival rate of approximately ten percent and median overall survival rate 11.4 months for those who receive treatment versus 7.6 months with control treatments). Patients receiving these drugs in the NICE trials saw increased life expectancy, decreased tumor size (which increases overall comfort and minimizes symptoms), and increased time before an event marking a progression of their degeneration. *Id.* See also Sibohan Gleeson, *Cancer Treatment Offers Hope, But Not in Australia*, TODAY TONIGHT (Austral.) (May 23, 2007), <http://au.todaytonight.yahoo.com/article/39425/health/cancer-treatment-offers-hope-australia> (recounting the story of a man who received CT-delivered radiation to destroy a tumor in his neck with technology that is available in many states in the U.S., but not available in Australia due to the high cost of the machine).

¹⁴ Mark Pownhall, *NICE rejects drug for metastatic breast cancer because of cost and poor efficacy*, BRIT. MED. J. (June 11, 2010), http://www.bmj.com/cgi/content/extract/340/jun11_2/c3145.

¹⁵ *Id.*

¹⁶ *Id.*

¹⁷ Rarer Cancers Forum, *Towards the best in Europe: transforming rarer cancer care*, 1, available at http://www.rarercancers.org.uk/news/current/the_rarer_cancers_forum_publishes_its_new_manifesto/rcfinanifes.pdf (last visited July 13, 2010).

¹⁸ Harris, *supra* note 11.

¹⁹ Rare Disease Act of 2002, PUB. L. NO. 107-280, § 404F(c), 116 Stat. 1990.

²⁰ Orphan Drug Act of 1983, PUB. L. NO. 97-414 § 526(a)(2).

²¹ Patient Protection and Affordable Care Act of 2010, Pub. L. No. 111-148, sec. 6301, § 1181(a)(2)(A), 124 Stat. 119, 727.

²² *Id.*

²³ TONI JOHNSON, HEALTH CARE COSTS AND U.S. COMPETITIVENESS (Council on Foreign Relations, Mar. 23, 2010), http://www.cfr.org/publication/13325/health_care_costs_and_us_competitiveness.html (noting that health care premiums have risen 114% over the past decade).

²⁴ *Id.* at 75.

²⁵ See, e.g., Julianne Pepitone, *YouTube credit card rant gets results*, CNN MONEY, Sept. 29, 2009, http://money.cnn.com/2009/09/29/news/companies/youtube_bank_of_america/index.htm (last visited July 13, 2010) (highlighting Bank of America's response to a customer who posted a YouTube video after direct negotiations with the company did not provide relief from a rate increase on her credit card).

²⁶ See 21 C.F.R. § 312.32 (2010) (requiring that the drug be proven safe and effective). For example, the FDA website touts multiple drugs, all derived from the same compound found in Salmon but delivered in different forms, to fight osteoporosis. See also FOOD AND DRUG ADMINISTRATION, DRUGS, <http://www.fda.gov/drugs/developmentapprovalprocess/howdrugsaredevelopedandapproved/default.htm> (last visited July 13, 2010) ("FDA approved the first drug based on salmon calcitonin in an injectable. Since then, two more drugs, one injectable and one administered through a nasal spray were approved. An oral version of salmon calcitonin is in clinical

trials now"); FOOD AND DRUG ADMINISTRATION, GUIDANCE FOR INDUSTRY NON-INFERIORITY CLINICAL TRIALS 5 (March 2010), <http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM202140.pdf> (highlighting that the new drug is not required to be better than the current drug, just "effective").

²⁷ Mark A. Wainberg & Kuan-Teh Jeang, *25 years of HIV-1 research—progress and perspectives*, BMC MEDICINE (Oct. 2008).

²⁸ Sharon H. Giordano et al, *Improvement in breast cancer survival: results from M.D. Anderson Cancer Center protocols from 1975-2000*, 21 PROC. AM. SOC. CLINICAL ONCOLOGY 212 (2002).

²⁹ See, e.g., Pancreatic Cancer Action Network, Learn the Facts, http://www.knowitfightitendit.org/learn_the_facts (last visited July 13, 2010) (highlighting the difference in federal funding for a number of different cancers and the fact that increased federal funding directly correlates with increased survival rates).

³⁰ Patient Protection and Affordable Care Act of 2010, Pub. L. No. 111-148, sec. 6301, § 1181(a)(2)(A), 124 Stat. 119, 727; cf. Kate Kelland, *Interview—UK watchdog offers health rationing tips to world*, REUTERS, Oct. 9, 2009, available at <http://www.reuters.com/article/idUSL9535043> (explaining that health care rationing is a fact for rich and poor countries).

³¹ Press Release, Food and Drug Administration, *FDA Issues Final Rules to Help Patients Gain Access to Investigational Drugs*, Aug. 12, 2009, <http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/2009/ucm176526.htm>.

³² See "Give impotent men Viagra," BBC NEWS, Mar. 26, 1999, http://news.bbc.co.uk/2/hi/special_report/1998/viagra/302614.stm (noting that NICE was started to help handle the Viagra coverage scandal after the NHS approved Viagra for men with impotence from spinal injuries and Multiple Sclerosis, but denied coverage if the impotence was due to psychological issues).

³³ Patient Protection and Affordable Care Act of 2010, Pub. L. No. 111-148, sec. 6301, § 1181(a)(2)(A), 124 Stat. 119, 727.

³⁴ See *id.* (establishing research agendas that address relevance to patients and expenditures associated with treatment).

³⁵ FOOD AND DRUG ADMINISTRATION, HISTORY, <http://www.fda.gov/AboutFDA/WhatWeDo/History/default.htm> (last visited July 13, 2010).

³⁶ See Charles P. Wallace, *Who'll Swallow Bayer*, TIME, Jan. 5, 2003, <http://www.time.com/time/magazine/article/0,9171,404241,00.html> (outlining the rise in price and time between the idea for a new drug and its emergence on the market).

³⁷ Kate Traynor, *For Opiate Management, FDA Pledges to Balance Enforcement, Palliative Care Needs*, AJHP NEWS, June 1, 2009, <http://www.ashp.org/import/news/HealthSystemPharmacyNews/newsarticle.aspx?id=3088> (quoting Douglas Throckmorton, FDA Deputy Director) ("We missed the uses of these concentrated morphine sulfate solutions in palliative and cancer pain treatment. We underestimated the market for these products . . ."). This story highlights that the FDA was threatening enforcement action of the makers of concentrated morphine sulphate, a pain medication used for hospice and oncology patients, because there was limited need for these medications in hospital treatment. *Id.* The FDA quickly changed its policy when the dire need of home care terminal patients became apparent. *Id.*

³⁸ See Robert J. Temple, M.D., Assoc. Dir. for the Center for Drug Evaluation and Research, FDA, *Availability of Investigational Drugs for Compassionate Use*, June 20, 2001 (testimony before the House Committee on Government Reform), <http://www.fda.gov/NewsEvents/Testimony/ucm115209.htm> (outlining the process for obtaining "compassionate use" drugs as a single-person IND and highlighting the other projects undertaken by the FDA in an attempt to provide access to therapies for patients who had serious diseases with no treatment alternatives).

³⁹ CENTERS FOR MEDICARE AND MEDICAID, ESTIMATED FINANCIAL AFFECTS OF THE "PATIENT PROTECTION AND AFFORDABLE CARE ACT" AS AMENDED, Apr. 22, 2010, at 3-4, [hereinafter *Financial Affects of PPACA*], <http://graphics8.nytimes.com/packages/pdf/health/oactmemo1.pdf>.

⁴⁰ See Cancer Research U.K., *Cancer incidence by age*, <http://info.cancerresearchuk.org/cancerstats/incidence/age/> (last visited July 14, 2010) (showing a steep increase in cancer diagnoses from a young age to seventy-five years and older).

⁴¹ U.S. DEPARTMENT OF HOUSING AND URBAN DEVELOPMENT, PREDATORY LENDING, <http://www.hud.gov/offices/hsg/sfh/pred/predlend.cfm> (last visited July 13, 2010).

- ⁴² *Id.*
- ⁴³ See U.S. DEP'T OF TREASURY, MAKING HOME AFFORDABLE: UPDATED DETAILED DESCRIPTION (Mar. 2009), 3, http://www.treas.gov/press/releases/reports/housing_fact_sheet.pdf (explaining how the loss of millions of jobs and predatory lending practices have forced millions of hard-working Americans to the brink of foreclosure).
- ⁴⁴ See, e.g., Pres. Richard Nixon, Special Message to the Congress Proposing a National Health Strategy (Feb. 18, 1971), <http://www.presidency.ucsb.edu/ws/index.php?pid=3311> (expressing support for the expansion of physician assistants and other "allied health professionals" as a way to address the ballooning costs of health care).
- ⁴⁵ *Id.*
- ⁴⁶ Tom Kisten, *Specialists earn double what primary care doctors get, study says*, VENTURA VALLEY STAR (May 17, 2010), <http://www.vcstar.com/news/2010/may/17/specialists-earn-double-what-primary-care-get/>.
- ⁴⁷ See, e.g., *Ruth Bader Ginsburg Diagnosis Puts Spotlight on Pancreatic Cancer*, FOX NEWS (Feb. 5, 2009), <http://www.foxnews.com/story/0,2933,488626,00.html> (reporting that Justice Ginsburg's cancer was caught at an early stage during an annual CT exam); Lauren Cox, *Steve Jobs' Reported Liver Transplant Stirs Debate*, ABC NEWS (June 28, 2009), <http://abcnews.go.com/Health/Economy/story?id=7902416&page=1> (summarizing the "multi-list" option that people with the ability and means to travel can use to increase their chances of receiving an organ transplant).
- ⁴⁸ Any treatment, investigational study, or clinical study is required by rule to have established safety protocols. See, e.g., 21 C.F.R. § 312.32 (2010) (mandating reports on drug safety and adverse effects to the FDA, even at the earliest point of development).
- ⁴⁹ *Id.* at § 312.315(b)(2).
- ⁵⁰ *Id.* at § 312.81(a).
- ⁵¹ *Id.*
- ⁵² Pownhall, *supra* note 14.
- ⁵³ *Id.*
- ⁵⁴ See Harris, *supra* note 11.
- ⁵⁵ Gina Kolatta, *In Reversal, Panel Urges Mammograms at 50, not 40*, N.Y. TIMES (Nov. 16, 2009), <http://www.nytimes.com/2009/11/17/health/17cancer.html>.
- ⁵⁶ Expanded Access to Investigational Drugs and Treatment Use, 74 Fed. Reg. 40932-33 (Aug. 13, 2009), <http://edocket.access.gpo.gov/2009/pdf/E9-19005.pdf> (seeking more alternatives to improve access to treatment in underserved communities).
- ⁵⁷ *Id.*
- ⁵⁸ See, e.g., Air Charity Network, <http://aircharitynetwork.org/AboutUs/tabid/183/Default.aspx> (last visited July 13, 2010) (highlighting the groups that work together to provide air and ground transportation for the seriously ill).
- ⁵⁹ Daniel Martin, *Betrayal of 20,000 cancer patients: Rationing body rejects ten drugs (allowed in Europe) that could have extended lives*, THE DAILY MAIL (U.K.) (Mar. 16, 2010), <http://www.dailymail.co.uk/health/article-1257944/NICE-rejects-cancer-drugs-extended-patients-lives.html>.
- ⁶⁰ *Id.*
- ⁶¹ Sarah Bosley, *U-turn as NICE Approves NHS Kidney Cancer Drug*, THE GUARDIAN (U.K.), (Feb. 4, 2009), <http://www.guardian.co.uk/society/2009/feb/04/kidney-cancer-drug-nhs>.
- ⁶² *Id.*
- ⁶³ *Of NICE and Men*, *supra* note 3.
- ⁶⁴ See Matthew Weaver, *Cancer survival rates have doubled since 1970s, research shows*, THE GUARDIAN (U.K.), July 12, 2010, <http://www.guardian.co.uk/science/2010/jul/12/cancer-survival-rates-doubled> (explaining that the higher survival rates are attributable to better treatments and earlier diagnosis).
- ⁶⁵ See *Cancer Facts and Figures*, AM. CANCER SOC. (2010), <http://www.cancer.org/aes/groups/content/@epidemiology-surveillance/documents/document/acspc-026238.pdf> (providing statistics on cancer incidence and survival by state and cancer type).
- ⁶⁶ *Id.* at 5.
- ⁶⁷ See NATIONAL CANCER INSTITUTE, NATIONAL CANCER INSTITUTE ANNUAL FACT BOOKS AND NCI FUNDED RESEARCH PORTFOLIO, <http://fundedresearch.cancer.gov> (last visited July 15, 2010) (providing survival and fund statistics by cancer type). Since 2005, prostate cancer research has received approximately half as much funding as breast cancer research from the National Cancer Institute. *Id.* In 2009, breast cancer research received \$600 million in funding, prostate cancer research received just under \$300 million in funding, while pancreatic cancer research received less than \$100 million in funding. *Id.*
- ⁶⁸ AM. CANCER SOC., *supra* note 65, at 17.
- ⁶⁹ Brian Alexander, *The Politics Behind the Pink Ribbon*, MSNBC.COM, Oct. 22, 2008, <http://www.msnbc.msn.com/id/27283197/ns/health-cancer>.
- ⁷⁰ *Id.*
- ⁷¹ See generally FDA, Developing Products for Rare Diseases and Conditions, FDA LAW BLOG, <http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm> (last visited July 15, 2010) (summarizing the different incentives, including grants and expanded clinical testing parameters, that the FDA uses to incentivize orphan drug development). But see Kurt R. Karst, *Nord Chair/HP&M Director Presents Opening Testimony at First Ever FDA Orphan Drug Hearing*, June 30, 2010, http://www.fdalawblog.net/fda_law_blog_hyman_phelps/2010/06/nord-asks-for-statement-of-policy-and-reduced-regulatory-uncertainty-during-fdas-first-public-hearin.html ("Although the ODA has been hailed as a resounding success, there remain approximately 5,800 rare diseases/conditions for which there are no FDA-approved therapies").
- ⁷² See generally CENTERS FOR MEDICARE AND MEDICAID SERVICES, NATIONAL COVERAGE DETERMINATION, July 2000 <http://www.cms.gov/ClinicalTrialPolicies/Downloads/finalnationalcoverage.pdf> (last visited July 15, 2010) (explaining the various treatments and procedures covered under the federal programs).
- ⁷³ See CENTERS FOR MEDICARE AND MEDICAID SERVICES, NHE FACT SHEET, https://www.cms.gov/NationalHealthExpendData/25_NHE_Fact_Sheet.asp (last visited July 15, 2010) (summarizing federal program health care expenditures and projecting costs for future years).
- ⁷⁴ See generally 21 C.F.R. § 316.20 (1992) (limiting incentives to products designed to treat rare or orphan diseases).
- ⁷⁵ E.g., Press Release, *Administration Announces Regulations Requiring New Health Insurance Plans to Provide Free Preventative Care*, Dep't of Health & Human Svcs., July 14, 2010, <http://www.hhs.gov/news/press/2010pres/07/20100714a.html>.
- ⁷⁶ Patient Protection and Affordable Care Act of 2010, § 1181(a)(2)(A), PUB. L. NO. 111-148, 124 Stat. 727.
- ⁷⁷ See *supra* note 3 (explaining the limitations on patient coverage under NICE).
- ⁷⁸ See generally Elizabeth J. Clark, Ph.D., "Death Panel" Rhetoric Sets Us Back, NAT'L ASSOC. OF SOC. WORKERS, Oct. 2009, <http://www.socialworkers.org/pubs/news/2009/10/clark.asp>.
- ⁷⁹ See *id.*
- ⁸⁰ Antonella Surbone, M.D., Ph.D., *Old Age and Frailty: Biological and Socio-cultural Constructs*, <http://www.clinicalcorrelations.org/?p=830> (last visited July 14, 2010) ("Poverty is especially common in the elderly, and socioeconomic, combined with cultural, factors in ethnic minorities in western countries limit their access to medical care and research."); Michael Schwartz, *et al.*, *Catastrophic Illness Expense: Implications for national health policy in the United States*, 12 J. SOC. SCIENCE & MEDICINE 20 (1978).
- ⁸¹ See generally Martin Powell & Mark Exworthy, *Equal Access to Health Care and the British National Health Service*, 24 POL. STUDIES 51, 59-60 (2003).
- ⁸² E.g. Rare Disease Act of 2002, PUB. L. NO. 107-280, § 404F(c), 116 Stat. 1990; Orphan Drug Act of 1983, PUB. L. NO. 97-414 § 526(a)(2).
- ⁸³ See, e.g., *Cruzan v. Missouri Dept. of Health*, 497 US 261, 282 (1990) ("Finally, we think a State may properly decline to make judgments about the 'quality' of life that a particular individual may enjoy, and simply assert an unqualified interest in the preservation of human life to be weighed against the constitutionally protected interests of the individual."); *Colautti v. Franklin*, 439 U.S. 379, 387 (1979) ("This right, we said, although fundamental, is not absolute or unqualified, and must be considered against important state interests in the health of the pregnant woman and in the potential life of the fetus.").