Medicare Rights Center

Off-Base:

The Exclusion of Off-label Prescriptions from Medicare Part D Coverage

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Introduction

The establishment of a Medicare prescription drug benefit was intended to bring improved health care and increased financial security for older adults and people with disabilities. For years, many people with Medicare—increasingly reliant on prescription drugs—tried to cobble together coverage for their medications through an unwieldy and often inadequate combination of employer insurance, retiree insurance, state pharmaceutical assistance programs (SPAPs), Medicaid and assistance offered by drug manufacturers. Often too many men and women were left with the choice of paying for their prescription medicines at the expense of other basic needs.

The Medicare prescription drug benefit (Part D), implemented in 2006, has left many people who enrolled in the benefit without coverage for the prescriptions they need. Indeed, many of these individuals were actually better off before the Medicare drug benefit came along.

Among those in this situation are the many people with Medicare whose doctors have prescribed medically necessary medications for "off-label" uses. Drugmakers seek Food and Drug Administration (FDA) approval for specific uses of their products and conduct trials to test their drugs' safety and effectiveness in patients with specific conditions. If the FDA approves the drug for those conditions, the drug manufacturer has to sell the medications with a label that lists the FDA-approved uses for that drug. Any use outside of that is referred to as off-label.[1]

Regulations issued last year by the Bush administration prohibit Part D coverage of off-label prescriptions, unless the prescribed use is supported in one of three specific medical compendia, which print overviews of new uses of medications supported by surveys of clinical studies and peer-reviewed medical literature. If it is not, Part D plans cannot cover the drug regardless of the extent of medical necessity and proof showing the effectiveness of the drug for that use, including peer-reviewed clinical research published in medical journals.[2]

Often individuals taking off-label prescriptions have tried treatment after treatment to no avail—sometimes experiencing debilitating side effects over a period of years. They, along with their doctors, are thrilled to finally find a medication that eases their discomfort—or, in some cases, preserves their lives—only to discover that even though the drug is on their Medicare Part D plan's formulary, it cannot be covered for them. When coverage of such medicines stops, people face increased suffering that frequently also increases the costs to Medicare because of their resulting need for more drastic medical care, such as emergency hospitalizations.

Over 20 percent of prescriptions written for the 500 most commonly used drugs were for off-label uses.[3] The exclusion of off-label prescriptions from Medicare Part D coverage hurts the most vulnerable people with Medicare and is in conflict with standard medical practice. The Centers for Medicare & Medicaid Services (CMS), the federal agency that administers the Medicare program, should change its regulations to allow coverage of off-label prescriptions under Part D. That would more closely follow the letter and intent of the Medicare Modernization Act of 2003 that established the Medicare drug benefit.

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[1] "Off-Label Use, Prescription, and Marketing of FDA-Approved Drugs: An Assessment of Legislative and Regulatory Policy," Steven R. Salbu, *Florida Law Review*,, 1999.
[2] 42 C.F.R. § 423.100 (2007).
[3] "Off-label prescribing among office-based physicians," David C. Radley, Stan N. Finkelstein, and Randall S. Stafford, *Archives of Internal Medicine*, 2006.

If CMS does not act quickly, then Congress must clarify that medically necessary off-label prescriptions without citations in one of the three approved compendia are not excluded from Part D coverage. The lives and health of people with Medicare depend on prompt action.

True Story

Ms. Q, who has both Medicare and Medicaid, lives in Oregon. She has been diagnosed with diabetes and a digestive disorder that causes frequent nausea and vomiting. After she tried several medications with no success, Ms. Q's doctor prescribed an antiemetic drug that is used to prevent nausea in chemotherapy and/or postoperative patients. This new drug has stabilized Ms. Q. and eliminated her need for frequent hospitalizations for dehydration and malnutrition.

However, because this medication is not FDA-approved or compendia-supported for Ms. Q's diagnosis, her Part D plan disregarded her doctor's statement that this drug is medically necessary and denied coverage for this "off-label" treatment. While Oregon Medicaid will cover drugs excluded from Medicare coverage by law, it does not consider this drug to be excluded from Medicare—even though it is effectively excluded for Ms. Q—and therefore will not continue covering it. On her limited income, Ms. Q cannot afford to pay for it herself because it costs more than $7,500 a month. With help from NorthWest Senior & Disability Services, Ms. Q accessed temporary, emergency coverage under Medicaid—but this coverage is currently set to expire when she completes the appeals process, even if she is unable to get coverage from her Part D plan.

Off-Label Prescriptions Are Common Medical Practice

While drug companies and manufacturers are prohibited from marketing drugs for uses for which the drug has not received FDA approval, off-label prescribing is widespread in the medical community as an essential means of providing patients with optimal medical care. A former FDA deputy commissioner for policy stated as long ago as 1996 that "under certain circumstances, off-label uses of approved products are appropriate, rational and accepted medical practice."[4]

Doctors routinely prescribe drugs for off-label purposes to treat a disease that is not listed on the label based on their professional medical judgment. In 2001, the last year for which data is readily available, 21 percent of 725 million prescriptions written for the 500 most commonly used drugs were for off-label uses.[5] In addition, the FDA has acknowledged that its labels often are not updated in a timely manner—particularly for cancer treatments.[6] And a recent article in the *Journal of Clinical Oncology* recommends requiring Medicare [Part D] contractors to consider peer-reviewed literature from "reliable sources" when making coverage determinations, noting "concerns…about

[4] Statement by William B. Schultz, before the Committee on Labor and Human Resources, U.S. Senate, February 22, 2006.
[5] "Off-Label prescribing among office-based physicians," David C. Radley, Stan N. Finkelstein, and Randall S. Stafford, *Archives of Internal Medicine*, 2006.
[6] Statement of Sarah F. Jagger, director of Health Services Quality and Public Health Issues, Health, Education, and Human Services Division, U.S. General Accounting Office, before the Subcommittee on Human Resources and Intergovernmental Relations, Committee on Government Reform and Oversight, U.S. House of Representatives, September 12, 1996.

the speed with which the [Medicare-approved] compendia review the available evidence and issue their conclusions about off-label uses…"[7]

Off-label prescriptions are particularly prevalent among cancer and human immunodeficiency virus (HIV) patients. A 1991 study by the U.S. General Accounting Office (GAO) revealed that one-third of drug treatments prescribed by cancer specialists were off-label, and more than 50 percent of cancer patients were prescribed at least one drug for an off-label indication.[8] Similarly, in a 1997 survey of cancer doctors by the American Cancer Society and the American Enterprise Institute, 60 percent said they prescribed medications for off-label uses.[9] A 1993 University of California survey of doctors who treated patients with HIV indicated that 42 percent of all such drug treatment was off-label, and that 90 percent of patients were prescribed at least one off-label medication. [10]

The same trend is seen in the treatment of pain and mental health conditions. According to the Pain and Policy Studies Group at the University of Wisconsin, "Literally thousands of medications are appropriately used by physicians for therapeutic purposes, including pain treatment, even though the U.S. Food and Drug Administration (FDA) has not specifically approved them for a particular use."[11] Likewise, a recent article in the journal *Pain Medicine* examining the benefits and dangers of using Actiq (a drug indicated to treat cancer-related pain) for the treatment of non-cancer related pain states, "We believe it is nonsensical for an opioid analgesic to carry an indication specifically for cancer pain. Period….The physicians of the world are prescribing one such labeled opioid, Actiq, 'off-label' in well over 90% of prescriptions written. Why? Because it works."[12]

An article in the trade journal *Drug Benefits Trends* explains why off-label prescriptions are necessary in the treatment of mental health conditions: "Clinical trials do not depict the conditions of real-world practice. Actual patients typically do not present with an isolated behavioral problem; they present with multiple problems. Therefore, physicians need to extrapolate specific findings from evidence-based research to manage multiple problems. The result is often off-label use of mental health medications, including anticonvulsants."[13]

The rate of off-label prescribing is also extremely high among individuals with rare conditions, with estimates that as many as 90 percent of such patients use off-label prescriptions.[14] In MRC's experience, some people with Medicare may have conditions so rare that there are literally no medications FDA-approved to treat them, nor any support in the Medicare compendia for the use of any drug for their condition.

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[7] "Reimbursement for Cancer Treatment: Coverage of Off-Label Drug Indications," *Journal of Clinical Oncology*, 2006..

[8] "Off-Label Drugs: Reimbursement Policies Constrain Physicians in Their Choice of Cancer Therapies," U.S. General Accounting Office, GAO/PEMD-91-14, 1991.

[9] "Off-label passes muster," American Cancer Society News Center, 1998.

[10] "Off-Label Drug Use in HIV Disease," Brosgart, C. et al., *Abstr Intersci Conf Antimicrob Agents Chemother Intersci Conf Antimicrob Agents Chemother.* 1994.

[11] "Off-Label Uses of Prescription Drugs in Pain Management," Angarola, Robert T., Pain and Policy Studies Group, University of Wisconsin Paul P. Carbone Comprehensive Cancer Center, 1995.

[12] "Weighing in on the Off-Label Use of Actiq for Noncancer Related Pain: A Recipe for Success or a Recipe for Disaster?" *Pain Medicine*, Passik, Steven D., and Kenneth L. Kirsh, 8 (2), 2007.

[13] "Rising Mental Health Drug Costs: How Should Managed Care Respond?," Sheila Fifer, PhD; Patricia Marken, PharmD; Joyce Kamanitz, MD; Antohony Kotin, MD; Norrie Thomas, PhD, MS, RPh, *Drug Benefit Trends*, 2005.

[14] "Off-Label or Out of Bounds? Prescriber and Marketer Liability for Unapproved Uses of FDA Approved Drugs," O'Reilly, James, and Amy Dalal, *Annals of Health Law*, 2003.

Consequences of this Exclusion for People with Medicare

The Centers for Medicare & Medicaid Services (CMS) has advised Medicare private drug plans (both stand-alone drug plans and health plans that offer drug coverage) to list the consumer hotline of the Medicare Rights Center (MRC) as a consumer resource on all drug coverage denial notices. In the ensuing deluge of calls from people with Medicare desperate to get their prescriptions covered, MRC has found that many rely on off-label medications for pain relief or to treat rare and serious medical conditions.

People with Medicare who cannot access their medications suffer adverse health effects. Off-label prescriptions are used in many circumstances, including controlling pain, easing nausea, preventing the growth of cancerous tumors and retarding the progression of terminal or degenerative illnesses. For this reason, when people with Medicare cannot take their medications, they may suffer a recurrence of their debilitating pain and nausea, experience acceleration of their illnesses and risk serious bodily harm or even death. Many require more drastic medical care—including frequent admission to the hospital—costing Medicare significantly more than it would cost to cover their prescriptions.

True Stories

Mr. U, who lives in Michigan, has a rare type of blood disorder for which there is no known cure. When he was first diagnosed, Mr. U had to undergo monthly blood transfusions to maintain an adequate blood supply. Then his doctor prescribed an anticancer medication that worked so well it eliminated Mr. U's need for regular transfusions. However, Mr. U's new medication was FDA-approved and compendia-supported only for patients with both his diagnosis **and** a chromosomal abnormality that Mr. U does not have. As a result, Mr. U's Part D plan denied coverage for this "off-label" use despite his doctor's testimony that the drug was medically necessary for him and had eliminated his need for painful and dangerous transfusions.

Mr. U does not qualify for Medicaid, and Michigan terminated its state pharmaceutical assistance program (SPAP) with the introduction of Part D. Even the lowest dose of this medication costs more than $9,000 per month, which Mr. U cannot afford. Fortunately, with help from his doctor, Mr. U was able to enroll in the drug manufacturer's patient assistance program (PAP) and is currently receiving his medication free of charge. However, most manufacturer assistance programs have been changing their rules to exclude people with Medicare. And Mr. U's PAP will only guarantee him a six-month supply of his medication.

Ms. L has been diagnosed with a rare form of ovarian cancer. In order to prevent excessive bleeding and tumor growth, she was being kept alive with a drug that is FDA-approved to treat infertility. Before the start of Medicare Part D, Ms. L paid $40 a month through her retiree insurance for this drug, which has a retail cost of over $30,000 a year. When Part D was implemented, Ms. L's retiree insurance eliminated its prescription coverage and required all of its members to enroll in Part D. Ms. L's Part D plan proceeded to deny coverage for her prescription, deeming it "off-label." Despite its medical necessity—attested to by her doctor—in ensuring Ms. L's health, and despite medical literature overwhelmingly supporting Ms. L's use of the drug, she was unable to obtain coverage through either of her health plans.

Recognizing her dire need to control her cancer, Ms. L's retiree insurance agreed to cover 75 percent of the cost of the drug. However, even with this assistance, for several months, Ms. L, who does not qualify for Medicaid, an SPAP or a PAP, remained responsible for a copayment of 25 percent, totaling over $7,300 per month. Ultimately, Ms. L paid over

$108,000 for her prescription before she was eventually able to obtain more comprehensive coverage for this drug—with help from an MRC caseworker—through insurance from her deceased husband's employer. However, this coverage is temporary. And there is no guarantee that, when this coverage ends, Ms. L's retiree insurance will continue contributing to the cost of this life-saving medication.

Mr. F has a rare degenerative disease. He has sought treatment for this condition for years, as his muscle tremors and progressive weakening have prevented him from performing tasks of daily living. In recent years, Mr. F has experienced further deterioration, including weakness in his knee and hip, reaching a point where he requires significant help to stand up from a tip-up easy chair and cannot raise his arms above his head, put on a jacket unaided or shave both sides of his face.

Mr. F eventually reached out to a university medical center and received information about a possibly life-changing drug. Since beginning treatment with this new medication approximately one year ago, Mr. F's condition has improved. The medication has halted the degeneration of his muscles, and he has regained enough muscle strength to enable him to live independently. Nevertheless, Mr. F's Part D plan has repeatedly denied coverage for this prescription because the drug is not indicated for his condition. Mr. F's drug costs nearly $4,000 per month—an amount that Mr. F is being forced to pay out of pocket.

Ms. C experiences persistent and widespread muscle and joint pain as a result of fibromyalgia and rheumatoid arthritis. She also suffers from chronic intractable pain associated with spinal nerve dysfunction and knee replacement therapy. These conditions, which affect her muscles, nerves and tissues, impact every aspect of her daily life. The pain is so overwhelming that it limits her ability to move, making it extremely difficult for her to leave her home. On several occasions, the pain has been so acute that it caused Ms. C to lose her balance and fall. Since the onset of arthritis over eight years ago, Ms. C's doctors tried a multitude of therapeutic interventions and medications to no avail.

Then one of Ms. C's doctors prescribed a local anesthetic indicated for the treatment of shingles, which has managed to control her pain. Nevertheless, Ms. C cannot get coverage for this medication under her Medicare Part D plan because she does not have shingles. Although she has a low income, she does not qualify for coverage under her state's Medicaid program because the program only covers medications categorically excluded under Medicare. Further, Ms. C is not eligible for her state's pharmaceutical assistance program because she is younger than 65. For these reasons, and because this medication costs $125 each month without coverage, Ms. C is currently going without this medically necessary treatment and enduring debilitating—and avoidable—pain on a daily basis.

Current Regulations on Part D (Non)Coverage of Off-label Prescriptions

The Medicare Part D statute requires drug plans to cover drugs that are reasonable and necessary for the treatment of an illness, with the only exceptions being exclusions explicitly listed in the statute (such as prescription vitamins and minerals, over-the-counter drugs and drugs for fertility or the treatment of anorexia, weight loss or weight gain).[15]

[15] 42 U.S.C. § 1395w-102(e) (2007); 42 U.S.C. § 1396r-8(d)(2) (2007).

Nevertheless, the Centers for Medicare & Medicaid Services (CMS) regulations exclude coverage of drugs that are not prescribed for a "medically accepted indication" as defined by the Medicaid statute.[16] MRC's legal counsel has concluded that this regulation violates the purpose of the statute and, as a matter of law, should be modified by CMS.

Under the Medicare Part D statute, a "covered Part D drug" is defined by referencing specific portions of the Medicaid statute.[17] The Medicaid statute has a definition of the term "medically accepted indication" that requires off-label prescriptions to be referenced in one of three compendia.[18] The Medicare statute, however, does not explicitly reference that definition.[19] Instead, the Medicare statute references only specific subparagraphs in the Medicaid statute that, in part, limit coverage of medications to only those that are FDA-approved to be sold in the United States.[20] Therefore, Congress must have meant to incorporate those parts only.

If Congress had intended the definition of "covered part D drug" to track exactly the Medicaid definition of "covered outpatient drug," then Congress would have expressed this intent by referring to the Medicaid definition of "covered outpatient drug" as a whole. It did not do so. In addition, Congress could have included off-label prescriptions in the list of excluded drugs enumerated in the Medicare Part D statute. It did not do so.

Despite the many ways in which Congress avoided specifically excluding off-label prescriptions from Medicare coverage, CMS wrote its regulations to limit coverage of off-label prescriptions to "medically accepted indications" as defined in the Medicaid statute. Because of that interpretation of the statute, many people with Medicare now lack access to life-sustaining off-label prescriptions.

Similarly, the structure of the Medicare statute demonstrates congressional intent to provide coverage for prescriptions that are medically necessary unless explicitly excluded. In developing their formularies, Part D plans are required by statute to base clinical decisions on the strength of scientific evidence and standards of practice, including an assessment of peer-reviewed medical literature.[21] Under the statute, people enrolled in Medicare Part D plans are entitled to coverage if their prescribing doctor indicates that none of the drugs on their plan's formulary would be as effective as the prescribed drug.[22]

Part D plans are also required to implement an appeals process that allows enrollees to secure coverage of drugs that are not included in plans' formularies but that are medically necessary.[23] Against this backdrop, it is evident that Congress did not intend, as the Bush administration has done, to create an absolute bar to coverage of medically necessary drugs. Instead, Congress repeatedly expressed an intention to ensure the health and safety of people enrolled in the Medicare drug benefit.

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[16] 42 C.F.R. § 423.100 (2007).
[17] 42 U.S.C. § 1395w-102(e) (2007).
[18] 42 U.S.C. § 1396r-8(k)(6); 42 U.S.C. § 1396r-8(g)(1)(B)(i) (2007).
[19] 42 U.S.C. §1395w-102(e)(1)(A) (2007).
[20] 42 U.S.C. § 1396r-8(k)(2)(A) (2007).
[21] 42 U.S.C. § 1395w-104(b)(3)(B)(i) (2007).
[22] *Id.* at (h)(2) (2007).
[23] *Id.*

In addition, other parts of the Medicare statute are inconsistent with the CMS interpretation of the Part D definition of a Part D-covered drug. Specifically the Medicare Part B statute allows for more comprehensive coverage of off-label cancer drugs. For these drugs, "medically accepted indication" has been defined broadly, permitting the use of peer-reviewed medical literature published in medical journals as well as compendia listings to evaluate the efficacy and safety of an off-label use.[24] Medicare Part B also provides coverage of drugs administered incident to doctors' services that are "reasonable and necessary."[25] Accordingly, CMS guidance grants Part B contractors considerable deference in deciding whether off-label prescriptions are "reasonable and necessary."[26]

The Medicare regulation defining a "Part D drug" to include only those prescribed for a "medically accepted indication" as defined in the Medicaid statute cannot be given controlling weight because it is manifestly contrary to the broad remedial purpose of the Medicare statute.[27] The limitations imposed by the Medicare Part D regulations are excessively restrictive.[28] This definition does not give plans an opportunity to even consider the medical necessity of off-label prescriptions, as intended by Congress. Because this definition goes against the purpose of the Part D statute, which is to ensure that enrollees have access to medically necessary medications, it should not be allowed to persist.

Ironically, Medicaid programs—whose federal statute is the source of Medicare's restrictive definition of a "medically accepted indication"—have the flexibility to cover off-label prescriptions, and many states do so. The federal Medicaid law that addresses off-label use provides that a "state may exclude or otherwise restrict coverage of a **covered outpatient drug** if . . . the prescribed use is not for a medically accepted indication [emphasis added]."[29] In this provision, Congress has merely set the floor on the coverage states can offer. If states offer prescription drug coverage, they must not put restrictions on off-label use beyond those stipulated in the federal law—but they may grant *more generous* coverage than the federal statute requires. Forty-two state Medicaid programs cover off-label, non-compendia prescriptions.[30]

CMS officials themselves have indicated that the purpose of the Medicare statute is to provide coverage of medically necessary medications. In 2005, Dr. Jeffrey Kelman, chief medical officer of CMS, testified before the Practicing Physicians Advisory Council, stating,

> **"The question comes up often, will part D only pay for FDA-approved indications? And the answer is no**. It will clearly pay for a drug that has an FDA-approved indication. If an indication for that drug is found on [sic] any of the major compendia: USPDI, AHFS, Drugdex and Drug Evaluations, it also has to be available. And last, **if there's published evidence for a drug use, that can be used as part of the appeals and exceptions process** [emphasis added]."[31]

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[24] 42 U.S.C. § 1395x(t)(2)(B) (2007).
[25] 42 U.S.C. § 1395y(a)(1) (2007).
[26] CMS *Carriers Manual*, Pub. 14, § 2049.4.
[27] *See* 42 C.F.R. § 423.100 (2007).
[28] 42 C.F.R. § 423.100 (2007).
[29] 42 U.S.C. §1396r-8(d)(1)(B)(i) (2007).
[30] U.S. ex rel. Franklin v. Parke-Davis, 2003 U.S. Dist. LEXIS 15754.
[31] "Practicing Physicians Advisory Counsel Meeting Transcription," Centers for Medicare & Medicaid Services, pages 69-72 (May 23, 2005).

Further, the introduction in *Medicare & You 2007*, CMS' annual consumer guide to Medicare, clearly states that "Medically necessary drugs must be covered" under Medicare Part D.[32] At no point does the document qualify that drugs must be prescribed for medically accepted indications or that coverage is limited by FDA indications or support in any compendia.

Thus, pursuant to the purpose of the Part D statute and the guidance issued by CMS officials, Part D plans are responsible for ensuring the safety of enrollees by providing coverage—or at least the opportunity to prove medical necessity for coverage—of drugs that are deemed by the prescribing doctor to be essential for the person's well-being, even in the absence of FDA indications or compendia support.

Few Alternatives for People Denied Off-Label Prescription Coverage

Individuals denied Part D coverage for their off-label prescriptions often cannot obtain coverage elsewhere. Options for drug coverage have shrunk or disappeared in the wake of Part D.

Many SPAPs have been limited or discontinued since Medicare Part D was instituted. Virtually all states shrunk or eliminated benefits previously provided by their SPAPs after the implementation of Part D. Although the National Conference of State Legislatures reports that about 27 states currently offer SPAPs that supplement Part D coverage, closer examination reveals that only a handful of these programs provide relief to people with Medicare who have off-label prescriptions. Many states—Kansas and Nebraska, for example—limit eligibility of their SPAPs to people who have both Medicare and Medicaid, while others, like New York and South Carolina, exclude people under 65 who qualify for Medicare because of a disability. Some states only offer benefits to people with specific conditions, such as Virginia, whose SPAP only serves individuals diagnosed with HIV/AIDS. Finally, not all of these programs cover drugs that are excluded from Part D. North Carolina's program, for example, only offers assistance with Part D premiums, and Nevada's program only covers drug costs during the doughnut hole, and, further, requires members to follow their Medicare drug plan's formulary and comply with all prior authorization and step therapy requirements.[33] As a result, in nearly every state, SPAPs provide little to no relief for individuals who cannot access coverage of off-label prescriptions under Medicare Part D.

In most states, Medicaid will only cover prescriptions for drugs that are explicitly and categorically excluded from Medicare Part D coverage. Before Part D, individuals who were eligible for both Medicare and Medicaid received comprehensive prescription coverage under state Medicaid programs. In 2006, the federal Medicare benefit mandated that all of these individuals begin receiving their prescription drug coverage under Medicare Part D. Medicaid can still continue to cover drugs that are explicitly excluded from Medicare coverage and that are on the Medicaid formulary or are optionally covered by Medicaid. However, as off-label prescriptions often involve drugs that are not categorically excluded from Medicare coverage, Medicaid programs frequently do not consider these drugs to be excluded from Medicare Part D. As a result, Medicaid programs do not consider themselves obligated to cover these prescriptions—even though these drugs are effectively excluded from coverage for people who rely on them for off-label, non-compendia

[32] *Medicare & You 2007*, Centers for Medicare & Medicaid Services (2007).
[33] "State Pharmaceutical Assistance Programs in 2006-07: Helping to Make Medicare Part D Easier and More Affordable," National Conference of State Legislatures, June 4, 2007.

supported needs. To make matters worse, some state Medicaid programs do not provide any pharmaceutical coverage for individuals who are also enrolled in Medicare Part D. These exclusions put the poorest and sickest people with Medicare at risk, as most cannot afford their prescriptions without coverage.

While some manufacturer patient assistance programs (PAPs) provide limited assistance for Medicare Part D enrollees, most drug companies do not offer help to individuals who are eligible for Medicare Part D coverage. Most PAPs are intended to provide access to medications for those who do not have insurance coverage or cannot otherwise afford their prescriptions. Since many PAPs are predicated on income, these programs have been a vital source of drug coverage for many low-income individuals. Most PAPs limit eligibility to individuals with incomes under 200 percent of the Federal Poverty Level, and assistance is not usually provided to those who receive or are eligible for prescription coverage from any other public or private source. Some companies even require that the individual have no health insurance at all. This is the case even if coverage for the company's drug is not available under the individual's policy—such as, for example, Medicare enrollees who are prescribed the drug for an off-label use without support in the Medicare compendia. Ultimately, enrollment in a Medicare prescription drug plan often prevents these individuals from accessing their medically necessary medications through PAPs.

With the introduction of Medicare Part D, individuals with retiree insurance have experienced a sharp reduction in their prescription drug coverage. The trend over the last two decades has been a reduction in retiree coverage as employers try to rein in their health care costs. According to the 2006 annual Kaiser/Hewitt Survey on Retiree Health Benefits, "Between 1988 and 2006, the share of large employers (200 or more employees) offering retiree health benefits declined from 66% to 35%."[34] The introduction of Part D has created more disruption in retiree coverage. The Kaiser/Hewitt survey found that five percent of surveyed employers started offering prescription drug coverage as a supplement to the Medicare drug benefit; three percent contracted with a Medicare prescription drug plan or a Medicare Advantage plan to offer additional prescription drug coverage; two percent have become a Medicare prescription drug plan; and eight percent have discontinued drug coverage. That means 18 percent of people with Medicare who previously had retiree drug coverage now have to get their drug coverage in one form or another from Medicare Part D. The survey also found that number is expected to rise to 22 percent in 2007.

The cost of prescription drugs is rising rapidly. The escalating cost of prescription drugs leaves most people with Medicare unable to afford their prescription drugs if they are not covered by their Part D plan. According to an *AARP Rx Watchdog Report,* "The cumulative effect of continued increases in the manufacturers' price of brand name drugs is substantial. Of the drugs in the sample, 153 have been on the market since December 1999. On average, manufacturers' prices for these drugs increased almost 54 percent during that time [December 1999 to December 2006], more than two-and-a-half times the general inflation rate of about 20 percent."[35] In addition, prices for medications have actually increased under Part D.

[34] "Retiree Health Benefits Examined: Findings from the Kaiser/Hewitt 2006 Survey on Retiree Health Benefits," Kaiser Family Foundation and Hewitt Associates, 2006.
[35] "Brand-Name Drug Prices Climb Again in 2006," *AARP Rx Watchdog Report*, 2007.

Conclusion

An ever-growing number of people with Medicare now rely on off-label prescriptions for uses that are not included in the Medicare compendia to treat serious conditions, relieve extreme pain, or, in some cases, to survive. CMS' exclusion of these prescriptions from coverage effectively deprives these individuals of the most basic care to which they are entitled. These are medications that the individuals' doctors have prescribed—often after years of unsuccessful experimentation with more traditional treatments—and attested to as medically necessary for their patients.

CMS' regulation excluding these off-label drugs from Part D coverage conflicts with the language and intent of the Medicare statutes. By failing to cover off-label, non-compendia uses of FDA-approved medications, the program is not meeting its intended goal of providing a "comprehensive prescription drug benefit."[36] CMS should revise its current Medicare Part D regulations so as to prevent an absolute bar against coverage of medically necessary off-label prescriptions.

If CMS fails to adjust the current regulations appropriately, Congress should revise the language of the Part D statute to more clearly allow for the consideration of other evidence of medical necessity in Medicare Part D coverage determinations and appeals, including peer-reviewed medical literature and the individual's medical history.

Otherwise, for many people, Medicare drug coverage will remain nothing but an unfulfilled promise. Human suffering will be needlessly prolonged. Lives will be lost. And, most likely, substantial avoidable health care costs will be paid by Medicare.

[36] "Prescription Drug Coverage - General Information," Centers for Medicare & Medicaid Services web site, (www.cms.hhs.gov/PrescriptionDrugCovGenIn/).