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Testimony Before the Senate Finance Committee
Hearing on the Medicare Prescription Drug, Improvement and Modernization Act of 2003 Regulations
September 14, 2004

I. Introduction

Mr. Chairman and members of the Committee, I am Michael Fitzpatrick, Executive Director of the National Alliance for the Mentally Ill (NAMI). I am here today on behalf of my organization and its 210,000 members and 1,200 affiliates, as well as:

- ALS Association,
- American Auto-Immune Related Diseases Association,
- Epilepsy Foundation of America,
- Huntington's Disease,
- The Latino Coalition,
- Lupus Foundation,
- Men's Health Network,
- National Alliance for Caregiving,
- National Adult Day Services Association,
- National Grange of the Order of Patrons of Husbandry,
- National Coalition for Women with Heart Disease,
- Prevent Blindness America, and
- RetireSafe.org.

Mr. Chairman, I want to thank you for convening this hearing and for providing us the opportunity to present to this Committee the concerns we have regarding patient protections under the new Medicare prescription drug benefit.

The organizations on whose behalf I am speaking today have long supported the principle of Medicare reform to include prescription drug coverage, and we applaud efforts in Congress to improve access to pharmaceuticals for our nation's most vulnerable citizens. We hope that that, when fully implemented, this new benefit will offer unprecedented – and long overdue – coverage of outpatient prescription drugs for our nation's seniors. Such coverage is critical to all Medicare beneficiaries, but it is especially important to those beneficiaries from vulnerable populations, such as those living with disabilities and chronic illnesses. We are committed to working with CMS and this Committee to ensure that the new law and its implementing regulations provide the intended coverage and protection to Medicare beneficiaries. In this regard, allow me to briefly highlight a few of our priority issues going forward.

II. Formularies Must Be Defined to Enable Access to Necessary Treatments

In treating Medicare beneficiaries, particularly vulnerable seniors and people with disabilities, physicians often must try many different drugs within the same pharmacological class before finding the one that is the safest and most effective for a specific individual. Physicians must consider a drug's side effects and efficacy, patient co-morbidities, and possible interactions with other drugs the beneficiary may be taking,

which, of course, can vary greatly from patient to patient. Further, it is not uncommon for a drug to be initially effective for a patient, but to subsequently lose efficacy, thus requiring the physician to begin the search again. Consequently, it is critical that physicians treating Medicare beneficiaries have access to a wide array of medications.

In this regard, we are concerned that the recently issued draft drug classification guidelines (the Guidelines) developed by the U.S. Pharmacopeia (USP), coupled with language in the recently issued prescription drug benefit Proposed Rule (Proposed Rule),[1] may not provide adequate access to all necessary medicines. Because the Proposed Rule only requires that two drugs be covered in each class,[2] the range of classes becomes a critical benchmark for the range of drugs that enrollees and their doctors will have access to. We believe the classifications set forth in the Guidelines may create confusion and could be used by prescription drug plans to discourage the enrollment of certain beneficiaries, e.g., Medicare beneficiaries with severe disabilities or chronic illnesses who have higher treatment costs.

For example, the Guidelines divide the category of antidepressants into three classes, one of which is reuptake inhibitors.[3] Selective serotonin reuptake inhibitors (SSRIs) and Selective Norepinephine Reuptake Inhibitors (SNRIs) are not segregated as distinct classes, but are instead collapsed into this single class with older tricyclic medications that are now widely recognized as outdated and antiquated treatment options. However, because of the way prescription drug plans can use the Guidelines to restrict access, it is reasonable to assume that many plans will choose to offer two older tricyclic medications as the only treatment option for depression for their enrollees. Likewise, in the case of anti-epileptic drugs used to treat seizures, USP failed to divide the category of anti-convulsants into any classes whatsoever. This means that USP's draft Model Guidelines would only require health plans to cover two drugs, or 10% of the currently available seizure medications.

We also are concerned that restrictions on the numbers of classes and categories of drugs, as proposed in the USP Guidelines, could discourage the development of new prescription medications. For example, manufactures would have little incentive to produce new drugs if formularies already satisfy the minimum two drugs per class requirement by providing access to older medications. There is little financial incentive to pursue these medications if there is a more than reasonable chance they would be excluded from formularies.

Consequently, CMS should require plans to provide clinical coverage for more than two drugs per class, if clinically appropriate. Since clinically inappropriate limits on certain classes could adversely impact the quality of care Medicare beneficiaries receive,

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[1] 69 Fed. Reg. 46,632 (Aug. 3. 2004).

[2] See 69 Fed. Reg. at 46,660.

[3] See Medicare Prescription Drug Benefit Draft Model Guidelines, Drug Categories and Classes in Part D, United States Pharmacopeia (Aug. 2004).

CMS should address the need for coverage of an appropriate range of therapeutic options in the final regulations. To its credit, CMS is seeking comments on ways to balance the needs of the mentally ill and other vulnerable patient populations with the need for flexibility by prescription drug plans.[4] We plan to respond to the agency's request with information demonstrating the importance of requiring broad access to medications so that physicians have the flexibility to prescribe any necessary drug. We urge the Committee to take an active role in overseeing the new benefit to ensure that CMS fulfills its obligations under the MMA.

We are also concerned that the process by which the USP Expert Committee developed the Guidelines was not open to groups representing Medicare beneficiaries living with severe disabilities and chronic illnesses. In June, eight patient groups, including NAMI, requested a meeting with USP staff to offer input into the process of developing the Guidelines. This request was made in writing through a formal letter that was signed by the groups. Unfortunately, we never received a formal response from the USP staff. After several follow-up phone calls, we were informed that neither the Expert Committee nor the USP staff would be meeting with patient organizations in advance of the August 27 public meeting.

At the same time, we understand through press reports that USP staff and the Expert Committee have been holding formal and informal meetings with other stakeholders. We are also frustrated that appointments to the Expert Committee that were to represent the interests of beneficiaries did not include organizations representing beneficiaries with chronic illnesses. While we respect the need for the Expert Committee and USP staff to develop these Guidelines free of influence from the diverse array of stakeholders in the new drug benefit, we feel strongly that this process has been tilted to restrict meaningful input from the stakeholder who is most at risk, i.e., the Medicare beneficiary who will depend on the new drug benefit for access to life-saving and life-sustaining treatment. We strongly urge this Committee to exercise its oversight authority to ensure that CMS does not adopt final Guidelines that fail to consider the needs of the most vulnerable beneficiaries.

Another potential problem faced by chronically ill, mentally ill, and other vulnerable Medicare beneficiaries under the new benefit is the provision of the MMA that allows prescription drug plans to change their formularies in the middle of the plan year.[5] Such a change is allowed so long as the plans provide "appropriate notice" to affected beneficiaries and other stakeholders prior to removing a covered drug from a formulary or changing its cost-sharing status.[6] "Appropriate" is defined as 30 days in the Proposed Rule.[7] We strongly believe that this is insufficient notice and does not recognize the real world, crucial nexus between drug plan choice and access to vital medicines for beneficiaries. Medicare beneficiaries are locked into one plan for an entire year and may

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[4] See id. at 46,661.

[5] See 42 U.S.C. § 1395w-104(b)(3)(E).

[6] See id.

[7] See id.

have specifically chosen the plan based on its formulary. Beneficiaries who cannot obtain the same treatment due to a formulary change may fail to complete their treatment regimens, thus increasing other Medicare costs if more expensive medical interventions are subsequently required.

If CMS believes that it cannot limit prescription drug plans in this manner, the agency should at a minimum require that plans "grandfather" coverage of chronic medications until the next open enrollment period. While this approach would still permit plans to use "bait and switch" marketing strategies involving popular medicines, it would provide the most vulnerable beneficiaries on established medicines the ability to continue their existing treatment regimen without having to pursue coverage through the plan's appeals process.

Relying on the most vulnerable populations – including those who are chronically ill, mentally ill, or enrolled in long-term care facilities – to successfully and in a timely way navigate the appeals process to obtain drugs that previously were covered as a matter of course is not a realistic option. Many may be unaware of, or not understand, the appeals process and instead turn to less effective therapies or stop their treatments altogether. For these populations, these concerns are exacerbated by their condition, making it critical that these beneficiaries be allowed to continue receiving their drugs under existing terms, without having to pursue an appeal.

III. Pharmacy and Therapeutic Committee Operations Should Be Transparent and Reflect an Independent Assessment of All Coverage Restrictions

As you know, the MMA outlines very basic standards for the development of formularies by prescription drug plan pharmacy and therapeutic (P&T) committees and for the composition of such committees, but grants CMS considerable latitude to establish guidelines to make this process sensitive to the specific needs of beneficiaries.

One important way to protect these interests is for CMS to require participating P&T committees to provide the public and their members with advance notice of their meeting agendas and to accept public input on drug coverage decisions. P&T committees should accommodate such input both during the development of policies, and after the policies have been finalized in draft form, but before implementation. Such a requirement would help CMS ensure that P&T committees comply with the MMA requirement that coverage decisions be based "on the strength of scientific evidence and standards of practice, including assessing peer-reviewed medical literature."[8]

Additionally, this process will ensure that beneficiary protections for coverage decisions under the new drug benefit parallel those protections provided by the public comment process in the traditional Medicare program for developing national and local coverage policies. P&T committees should also be required to document and explain the reasons for their formulary decisions and make these determinations public. This would ensure that the P&T Committee follows the intent of Congress and makes clinical, rather than financial, judgments when developing a formulary.

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[8] See 42 U.S.C. § 1394w-104(b)(3)(B).

To ensure that all coverage policies are based on objective, clinical rationales and are developed by clinical experts, CMS should also implement rules making explicit that P&T committee responsibilities extend beyond the development of simple formularies to include the development of all restrictive coverage policies. In the preamble to the Proposed Rule, CMS states that it interprets the MMA as "requiring that a P&T committee's decisions regarding the plan's formulary be binding on the plan."[9] In addition, CMS states that it expects "P&T committees will be involved in designing formulary tiers and any clinical programs implemented to encourage the use of preferred drugs (e.g., prior authorization, step therapy, generics programs)."[10] However, these provisions are not included in the actual regulations, but are only discussed in the preamble. We urge CMS to include these requirements in the regulations themselves to ensure that prescription drug plans understand their obligations. As noted above, the rationales and clinical justifications for these coverage policies should be subject to discussion and validation in an open forum with an appropriate opportunity for public input, including input from patient advocacy organizations.

CMS also is seeking additional public comment on the important issue of P&T committee independence.[11] In this regard, we strongly recommend limiting the number of voting P&T committee members with conflicts so as to avoid diluting the voices of independent members. The recent settlement of the government's investigation of Merck-Medco Managed Care provides guidance in this regard.[12] Pursuant to that agreement, a majority of P&T committee members must be actively practicing physicians, pharmacists, or health care professionals and not be employed by Medco,[13] thus limiting the risk that conflicted members will marginalize the input of independent members. This protection should be incorporated into the regulations.

IV. The Regulations Should Incorporate Patient Protections for Therapeutic Substitution

CMS should incorporate in the final regulations patient protections for therapeutic substitution and, in particular, a requirement that prescription drug plans not engage in such practices without the express consent of the prescribing physician. In the preamble to the Proposed Rule, CMS indicated that it supports such a requirement,[14] but neglected to include it in the regulations. We urge CMS to expressly include such a standard in the final regulations, or, alternatively, to expressly state that plans should defer to state laws

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[9] 69 Fed. Reg. at 46,659.

[10] Id.

[11] See id.

[12] See United States v. Merck-Medco Managed Care, Civil Action No. 00-737, Consent Order of Court for Permanent Injunction (E.D. Pa.).

[13] See id.

[14] 69 Fed. Reg. at 46,667 ("Therapeutic substitution would always require explicit prescriber notification and approval.").

on therapeutic substitution. As you may be aware, many states have laws requiring prescriber consent before plans may make a substitution.

Preserving the physician's role in the prescribing process is an important beneficiary protection, particularly for vulnerable Medicare populations who may be on multiple medications and living with many co-morbidities. We believe that the patient-physician relationship in these situations is sacrosanct and should not be undermined by any implication that therapeutic substitution can be executed without explicit physician consent.

V. CMS Should Provide Detailed Guidance for Alternative Benefit Designs to Ensure That Beneficiaries Receive Access to Needed Therapies

It is imperative that CMS vigorously enforce the requirement under the MMA that prescription drug plans not implement alternative plan designs – such as alternative tiered cost-sharing schemes – if "the design of the plan and its benefits . . . are likely to substantially discourage enrollment" by certain Medicare beneficiaries.[15] In this regard, we are very concerned that alternative schemes designed principally to reduce costs could impede patient access to medically optimal medicines and could be used by plans to "cherry-pick" only the healthiest enrollees. Medicare beneficiaries – particularly those living with chronic illness and severe disabilities – would be particularly at risk if plans engaged in such practices.

In the Proposed Rule, the Secretary states it will review "tiered cost-sharing, the use of categories and classes in a formulary, and the choice of drugs provided in each category," but does not state what the standard of review will be.[16] We plan to request that CMS further clarify the standards by which it will review benefit design for discriminatory effect, and that, in particular, it consider the following recommendations.

In general, we urge CMS to closely scrutinize applications to provide alternative benefit packages. To avoid the potential for favorable selection and ensure that patients and their providers can reasonably access different therapeutic choices – particularly, drug therapies that target vulnerable populations – we recommend that CMS place reasonable limits on the cost-sharing requirements a prescription drug plan could employ in alternative, tiered co-payment, benefit packages. Specifically, we recommend that the agency consider a maximum limit on cost-sharing differentials and that a beneficiary's co-payment never be allowed to be greater than one-half of the plan's cost for the drug. Further, to avoid adverse selection problems, we urge CMS to require plans to maintain consistent cost-sharing requirements across all therapeutic classes. By including these protections, CMS would help ensure that the most vulnerable beneficiaries do not face discriminatory co-payments that are markedly higher than those faced by individuals with other conditions and disease states.

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[15] See 42 U.S.C. § 1395w-111(e)(2)(D)(i).

[16] See 69 Fed. Reg. at 46,680.

Prescription drug plans should also be required to specifically address the issues of adverse selection and beneficiary access to care in their applications to provide alternative benefit packages. CMS should make public its analyses and an explanation of its final decisions to approve or disapprove these applications. These reports should specifically address the agency's findings on the issues of favorable selection and access. Publicizing the analyses and explanations regarding CMS' decisions will ensure public oversight of plan benefit designs and the ability of vulnerable populations to access the drug benefit prescribed by Congress.

The regulations must also ensure that Medicare beneficiaries have access to the latest treatments approved by the FDA. This is particularly important for vulnerable populations, such as people with ALS, for whom emerging treatments could significantly improve the treatment of their conditions. Standards must be established that recognize emerging medicines and provide an opportunity for these medications to be included in formularies in a timely manner. Additionally, patients should have access to these new therapies while they are being reviewed for inclusion on a formulary. In this way, formularies should be flexible, not only meeting the needs of the patients of today, but also those of tomorrow by providing timely access to new medications, while at the same time ensuring continued innovation.

Finally, we believe that the regulations must ensure access to "off-label" medications as necessary. Off-label use of prescription drugs is common practice in the care and treatment of patients with complex chronic conditions. For example, there are no medications approved by FDA with primary indications to treat lupus. Therefore, proper care of lupus patients requires physicians to prescribe multiple medications off-label. In its proposed rule (might only be in preamble…must check cite) CMS directs prescribers to clearly document and justify off-label use in their Part D enrollees' clinical records. Unintentional oversight of off-label treatments can lead to dire consequences in vulnerable populations, denying medications to frail patients or instituting additional barriers to access. Further, attention must be given to evidence-based off-label usage in formulary development. A plan may chose to cover more than two drugs in a given therapeutic class, but since the plan is not required to cover drugs per off-label use, essential medications could be omitted from the formulary.

VI. CMS Should Implement Special Protections for Dual Eligibles

We also believe the final regulations should address the unique problems faced by beneficiaries who qualify for both Medicare and Medicaid (so-called "dual eligibles"). These individuals are particularly vulnerable because of their low incomes. Significantly, a large percentage of dual eligibles (by some estimates as many as 40%) are living with severe mental illnesses and other disabilities.

Currently, these beneficiaries are receiving their drugs under Medicaid. To protect these and all low-income individuals, CMS should enforce a "continuity of care" requirement to ensure access to the same array of mental health and other medications that are available under Medicaid. At a minimum, dual eligibles should be allowed to

continue on the medications they are currently taking and not be required to switch to another drug.

In addition, under existing Medicaid law, dual eligibles cannot be denied access to their medications if they are unable to pay their co-payments. While the co-payment for any single drug may be nominal, beneficiaries taking multiple drugs may face multiple co-payments which in the aggregate can pose a substantial financial burden. Consequently, it is imperative that this Medicaid protection be included in the new Medicare drug benefit so that beneficiaries who cannot pay their co-payment are not denied access to necessary medications.

VII. Beneficiaries Should Have Meaningful Appeal Rights

To ensure that beneficiaries' rights are protected, the final regulations should provide meaningful grievance and appeal procedures for denials of coverage and improper conduct by prescription drug plans. We have a number of concerns with regard to these appeal procedures, not the least of which is their utter lack of clarity in establishing different processes and procedures for challenging different kinds of plan decisions. In general, we believe that CMS should endeavor to clarify these highly important procedures, so that beneficiaries and their families are fully aware of their rights under the new benefit.

We are also concerned that, under the Proposed Rule, it is unclear when a decision is considered to be a coverage determination that requires a specific written notice with appeal rights and, in particular, whether a denial of a drug as a non-formulary drug at the pharmacy counter would constitute such a coverage determination. Without a written notice of appeal rights, the beneficiary may never realize that an additional step is required to trigger the appeals process. Consequently, CMS should clarify the Rule to require that a notice of coverage determination be issued at the time the prescription is denied at the pharmacy and that such notice include an explanation of the beneficiary's appeal rights.

Next, CMS should clarify that beneficiaries have the right to de novo review of denials of coverage and exception requests before an independent review entity (IRE). Specifically, CMS seems to treat IRE reconsiderations arising from formulary exception requests differently from those arising from other coverage determinations. CMS states that an IRE, when reviewing an appeal of a denial of a formulary exceptions request, is limited to determining whether the prescription drug plan properly applied its own formulary exceptions criteria and that "the IRE would not have any discretion with respect to the validity of the plan's exception criteria or formulary."[17] This limited review is not supported by the MMA. CMS should clarify in the final rule that it does not intend to limit the scope of IRE review.

Third, beneficiaries with chronic, mental, and other debilitating illnesses must be able to obtain rapid responses to their appeals and not have to navigate multiple

[17] Id. at 46,721.

procedures. Under the MMA and Proposed Rule, to obtain a non-preferred drug on the same cost-sharing terms as a preferred drug, the prescribing physician must demonstrate that the preferred drug "either would not be as effective . . . or would have adverse effects."[18] Similarly, to receive coverage for a non-formulary drug, the prescribing physician must demonstrate that "all covered Part D drugs on any tier of the formulary . . . would not be as effective for the individual as the nonformulary drug [or] would have adverse effects for the individual."[19]

This second showing necessarily encompasses the determination that the preferred formulary drug is not as effective as the non-formulary drug or would have adverse effects on the individual. Therefore, it would not make sense to grant preferred cost-sharing status to a second or third tier drug for which the beneficiary had demonstrated medical necessity, but not grant similar treatment to a non-formulary drug for which the beneficiary had made a similar showing. Patients should be able to obtain both coverage and preferred status in one appeal.

Further, assuming a beneficiary is successful in an appeal to obtain coverage or preferred status for a drug, the plan appears to have complete discretion to determine the beneficiary's cost-sharing obligations.[20] A beneficiary who obtains coverage of a necessary drug but cannot afford the plan-established cost-sharing has wholly illusory appeal rights. We strongly urge CMS to establish reasonable parameters for the cost-sharing obligations of beneficiaries who file successful appeals.

Finally, CMS should clarify the scope of the plan decisions that are appealable. To ensure that appeal rights are meaningful, the appeal provisions should apply to the full scope of coverage denials – including denials of requests for prior authorization.

VIII. Conclusion

Mr. Chairman, we appreciate that Congress has taken an enormous first step toward providing a comprehensive outpatient prescription drug benefit to Medicare beneficiaries. CMS has built on that effort in its proposed regulations, but much work needs to be done to ensure that this benefit is robust and successful and that beneficiaries have the safeguards they need and deserve. We pledge to continue working with you and with CMS to provide assistance, feedback, and information in every way we can to develop a meaningful Medicare prescription drug benefit that protects the interests of all beneficiaries. Thank you again for allowing me the opportunity to present our views. I am, of course, prepared to answer any questions you may have.

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[18] 42 § 1395w-104(g)(2); see also 69 Fed. Reg. at 46,720.

[19] 42 § 1395w-104(h)(2) (emphasis added); see also 69 Fed. Reg. at 46,721.

[20] See 69 Fed. Reg. at 46,721, 46,844.