NAMD
National Association of Medicaid Directors

April 8, 2015

The Honorable Fred Upton
Chairman
House Committee on Energy and Commerce
U.S. House of Representatives
Washington, D.C. 20515

The Honorable Frank Pallone
Ranking Member
House Committee on Energy and Commerce
U.S. House of Representatives
Washington, D.C. 20515

Dear Chairman Upton and Congressman Pallone:

On behalf of the nation's Medicaid Directors, we appreciate the opportunity to provide the Energy and Commerce Committee with comments on the 21st Century Cures Initiative and the January 27, 2015, discussion draft proposal.

NAMD is a bipartisan organization which represents Medicaid Directors in the fifty states, the District of Columbia and the territories. The Association was created in part to develop consensus among Directors on critical issues, specifically those that have national policy implications. In recent years, Directors have coalesced around emerging trends shaping access to and expenditures for outpatient prescription drugs, devices and related pharmaceutical therapies.

The 21st Century Cures and related Senate initiative could result in the delivery of meaningful access for all U.S. citizens over the longer term to high-quality, cutting-edge pharmaceuticals. We remain concerned, however, that the Committee has focused almost exclusively on the development and access components for stimulating innovation, and has not dedicated sufficient attention to equally important issues that impact payers and the safety of patients.

As you know, under federal statute pharmacy services are an optional benefit for most Medicaid-eligible populations. However, states have historically recognized that prescription drugs and devices are integral in prevention, treatment and maintenance of health and well-being for most individuals. Currently all states include pharmacy in their benefit Medicaid packages, and as of 2013, Medicaid expenditures on outpatient prescription drugs topped $40 billion.

Over two decades ago, as part of the Omnibus Reconciliation Act of 1990 (OBRA '90), Congress established the Medicaid Drug Rebate Program (MDRP) to provide expenditure offsets for covered outpatient drugs utilized by Medicaid populations. The MDRP requires that the Medicaid program cover all drugs approved by the Food and Drug Administration (FDA) where the manufacturer has signed a federal rebate agreement. In some situations, states have secured additional drug expenditure offsets by establishing state supplemental drug rebate programs.

As compared to new, innovative, "curative" drugs that have unit cost pricing in the thousands of dollars, the original MDRP was designed to provide offsets for drugs where the unit cost was several magnitudes lower. Additionally, state Medicaid programs are finding that legacy pharmacy market cost containment and utilization strategies are proving ineffective and potentially creating barriers or inequities or both for patient access to new novel "curative" therapies.

Modifications to the FDA's drug and device approval pathways and continued breakthroughs in science innovations and medical technologies coupled with the growth in the Medicaid enrollment, require a comprehensive review of the current MDRP incentives as they apply to Medicaid and drug manufacturers. This is particularly relevant to ensure states can advance value-based purchasing, risk-sharing and proven quality outcomes instead of the current no-risk sharing, discounted payment model which is driven by rebate agreements with fixed Average Manufacturer Price-based discounts.

Any meaningful discussion to improve process and incentives for the development and approval and access of pharmaceuticals for the U.S. market requires careful consideration by Congress. Federal policymakers must assess the impact to U.S. prescription drug budgets, insurance premiums and costs borne by the state Medicaid programs, taxpayers and patients.

Specifically, we believe Congress should carefully examine the existing legacy payment and reimbursement regulatory frameworks to ensure that the innovations proposed in the 21st Century Cures or similar proposals are appropriately balanced with equitable pharmaceutical pricing and payment strategies that create a sustainable and fiscally responsible competitive market. Further, Congress must assess the full spectrum of patient-related issues. New pathways and incentives should include appropriate protections for vulnerable patients, particularly those enrolled in the Medicaid program, to ensure they are not inadvertently subject to adverse consequences.

The key issue of U.S. pricing and expenditure offsets for new high-touch and high-cost curative pharmaceuticals remains a high priority for states. Enclosed we provide additional comments on the discussion draft. We also refer you to the NAMD letter transmitted to congressional

leaders on October 28, 2014, which discussed many of these issues in the context of hepatitis C therapies.

We appreciate your consideration of our comments. We remain committed to informing federal policy discussions and potential changes to federal statute that may impact the Medicaid program.

Sincerely,

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Vice-President, NAMD

Cc:
Congressman Tim Murphy, Chairman, Subcommittee on Oversight and Investigations, House Committee on Energy and Commerce
Congresswoman Diana DeGette, Ranking Member, Subcommittee on Oversight and Investigations, House Committee on Energy and Commerce

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Spurring Innovation in Pharmaceutical Development and Access: A Medicaid Perspective

The following comments from the National Association of Medicaid Directors (NAMD) address the January 27, 2015, discussion draft as posted on the House Energy and Commerce Committee website. Our overarching comments focus on the likely impact to state Medicaid program policies and budgets as well as safety considerations for the Medicaid-eligible population. We also offer comments on specific provisions of the discussion draft.

General Feedback

- The provisions of the discussion draft do not take into account ramifications for payers, both public and private, particularly with regards to the extended exclusivity periods for new or modified therapies.
- Congress should evaluate the Medicaid drug rebate program (MDRP) in the context of the emerging market for high-touch, high-cost curative pharmaceuticals. The MDRP was designed to provide offsets for drugs where the unit cost was several magnitudes lower than recent breakthrough therapies. Additionally, legacy pharmacy market cost containment and utilization strategies are proving ineffective and potentially creating barriers for patient access to novel curative therapies. Medicaid pharmacy program reforms will be required to address benefit design flexibility and value-based payment models to support Medicaid beneficiary access to these therapies.
- Lowering the evidentiary standard for drug and device approvals without granting coverage flexibility to the Medicaid program makes the Medicaid population a captive market for these products and potentially puts vulnerable Medicaid populations at risk. Other payers may choose not to cover these therapies, but current law requires Medicaid to do so.
- Medicare coverage and policy decisions will have downstream impacts and costs for the Medicaid program, both on the Medicaid-Medicaid dually eligible population and the potential need for states to support providers to comply with new Medicare policies.

Section-by-Section Feedback

Proposal Provisions	NAMD Comments/Questions

Sec. 1041 – Approval of Breakthrough Therapies
 	
• ***Allows the FDA to approve a drug, for a disease/condition with unmet medical need, that has received "breakthrough therapy" designation based on early stage clinical safety/effectiveness data that provides sufficient evidence under current safety and efficacy standards***
• ***Allows HHS to require post-market assessment of the drug, with ability to withdraw approval if assessment is not conducted, drug is found unsafe and/or ineffective, or manufacturer uses false or misleading marketing***
	• This provision appears to lower the evidentiary standards for this drug class.
	• Shorter FDA review times combined with increased FDA authority to require further studies *after* approval, rather than settling safety issues *before* approval, may contribute to increased rates of patient safety risks, drug withdrawals, and black box warnings.
	• Patient education is needed about any new approval pathway and the potential risks of fast-tracked therapies.
	• The Medicaid population is significantly different from the privately insured population. This raises concerns about the quality of evidence for drug approval as it pertains to a drug's effects on Medicaid's generally frailer, sicker population.
	• Medicaid is statutorily required to cover any FDA-approved drug in exchange for mandatory rebates. No other payer is under this same obligation. <u>In effect, the lowered evidentiary standards of this provision makes the Medicaid population a captive market for any potential adverse effects not discovered in the accelerated approval pathway. Further, the current framework would make the Medicaid program responsible for managing the short and long-term costs associated with such outcomes.</u>
	• If this provision is enacted, policymakers should consider a corresponding policy for periodic surveillance reports on adverse events, outcomes, etc. for Medicaid and other vulnerable populations.
	• <u>Policymakers should consider the benefits of providing new Medicaid flexibility in at least two ways:</u>
	  ○ Flexibility for state Medicaid programs to *not cover* drugs approved under this provision until sufficient post-approval studies have been conducted.
	  ○ Flexibility for state Medicaid programs to enter into value-based payment arrangements for drugs approved under this

	provision, with payment contingent on drug efficacy and safety.
Sec. 1063 – Election to Convey a Portion of Extended Exclusivity Period Applicable to Qualified Infectious Disease Products • ***Extends exclusivity periods for "qualified infectious disease products" by 5 years*** • ***Allows manufacturers to apply up to one year of this extended exclusivity to one or more drugs, in exchange for a commensurate reduction in exclusivity for the designated infectious disease product*** • ***Manufacturers are required to make a donation of profits to the NIH and a patient assistance programs.***	• This provision may delay the introduction of new generic antibiotics and delay generics for entirely separate drug categories. • As proposed, manufacturers could extend exclusivity for particularly high-cost drugs longer than they otherwise would. The variable exclusivity arrangement also removes predictability for state planning purposes, and could potentially impact Medicaid's ability to negotiate supplemental rebates for comparable treatment options. • Policymakers may wish to consider enhanced Medicaid rebates, value-based purchasing flexibility for Medicaid programs, or similar policies that reflect the burden of extended exclusivity periods to mitigate these concerns. The potential effect of high-cost therapies with extended exclusivity periods on state budgets, even if there were a potentially enhanced rebate, should be taken into consideration when crafting such a policy. • If policymakers were to allow a manufacturer to extend an exclusivity period for a "qualified infectious disease product," they may wish to consider tying this to the availability of a generic or lower-priced drug belonging to the same therapeutic category.
Sec. 1064 – Encouraging the Development and Use of New Antimicrobial Drugs • ***Adds supplemental Medicare payment to hospital discharges which use new antimicrobial drugs.***	• Further analysis is needed to ensure this provision does not create an incentive for overutilization of new antimicrobial drugs. • Policymakers should consider that, to the extent that such new drugs are utilized, Medicaid cost-sharing for Medicare-Medicaid enrollees will increase. Further analysis is needed to assess other potential impacts on the Medicaid program. • If policymakers include a supplemental Medicare payment they should also consider making this contingent upon other factors, such as the general ineffectiveness of previous antimicrobial drugs.

Sec. 1081 – Priority Review for Breakthrough Devices • ***Creates a priority review program for breakthrough devices which have no approved alternative or offer significant advances over existing devices*** ***Sec. 1082 – CMS Coverage of Breakthrough Devices [currently a placeholder]*** ***Sec. 1101 – Accelerated Approval for Breakthrough Devices*** • ***Allows HHS to approve breakthrough devices based on surrogate endpoints that are reasonably likely to predict clinical benefit*** • ***Such approval may be subject to post-approval studies***	• The forthcoming placeholder section could impact Medicaid coverage policies. Policymakers should consider the policy, clinical and budgetary impact to Medicaid. • The accelerated approval provision could lower the evidentiary standard for device approval. This reduction will make it more difficult for Medicaid programs to utilize evidence-based assessments for coverage decisions. • <u>The Medicaid population is significantly different from the privately insured population. This raises concerns about the quality of evidence for device approval as it pertains to the device's effects on Medicaid's generally frailer, sicker population.</u> If enacted, periodic surveillance reports on adverse events, outcomes, etc. for Medicaid and other vulnerable populations should be considered. • Post-marketing surveillance/reporting must be robust and timely, with swift action for any identified issues. • Further analysis is needed to determine what type of devices are permitted through this pathway. (Example: Implantable devices may need to be handled outside this process or have additional scrutiny before they are surgically implanted.)
Sec. 1121 – Expanded Access Policy as Condition of Expedited Approval • ***Requires manufacturers who receive a "covered investigational drug" designation to make their patient access policies to said investigational drug***	• A "covered investigational drug" should be treated as an "investigational drug" for purposes of the Medicaid outpatient drug exclusion for investigational drugs. <u>States should retain the flexibility to decide their Medicaid coverage policies for such drugs.</u> • If federal policymakers require states to cover a "covered investigational drug," policymakers should consider the budgetary impact to the Medicaid program. An enhanced rebate for coverage of such treatments or value-based purchasing flexibility are potential ways to address these

publicly available within 30 days of such designation. ***Sec. 1124 – Expanded Access Task Force*** • ***Establishes an Expanded Access Task Force to make one-time recommendations to Congress.***	concerns. Further, such a decision should consider the potential for states being held liable for punitive damages for adverse drug events on Medicaid beneficiaries. • Requiring Medicaid to cover these drugs may conflict with existing state laws, rules and policies on coverage of investigational drugs, requiring substantial time and resources at the state level to come into compliance. • The interaction of this provision with the ability for manufacturers to charge patients and payers for using investigational drugs must be considered. For example, policymakers should consider the potential impacts on patients and payers and limitations on these access costs.
Subtitle L, Sec. 1221 – Dormant Therapies • ***Creates a "Dormant Therapies" class with 15-year exclusivity for drugs that address one or more unmet medical needs, as determined by HHS***	• A 15-year exclusivity period for potentially high-cost drugs could disrupt pharmaceutical market dynamics and place significant strain on Medicaid programs. • Medicaid relies on competition in drug classes to secure supplemental rebates and ensure access to appropriate therapies. Lengthy exclusivity periods make these objectives more difficult to achieve. • Dormant therapies approved under this provision should either be considered "investigational drugs" for Medicaid purposes, or else be eligible for enhanced Medicaid rebates, value-based purchasing flexibility, or some other alternative payment model. • Policymakers need to clarify the types of therapies intended to be captured by this provision. It is not clear whether this provision precludes other competitor brands for the same indication or if this is meant to only prohibit generics for dormant brands. • It is not clear if it is possible for a manufacturer to provide a new application for a therapy that has been off the market for a period of time and receive dormant therapy approval. If so, this may have unintended consequences and requires further analysis. • It is not clear whether this provision includes traditional drugs through NDA approval and biologics assigned to CDER (Center

	for Drug Evaluation and Research) and CBER (Center for Biologics Evaluation and Research).
Sec. 1241 – Extended Exclusivity Period for Certain New Drug Applications and Abbreviated New Drug Applications • ***Extends exclusivity by 2 years for drugs which make "significant improvements" to existing molecules*** • ***Includes new indications, enhanced patient adherence, reduced public health risks, reduced side effects/adverse events***	• Allowing an additional 2 years of exclusivity for drugs may delay the introduction of generics into the market and reduce overall competition, which inhibits states' abilities to negotiate supplemental rebates. <u>This in turn has a direct impact on both federal and state Medicaid expenditures (within the pharmacy budget) and access to appropriate therapies.</u> • Consideration should be given to the number of continuous extensions granted for the same drug being manufactured with "significant improvements," such that these extensions do not create an excessive or monopolistic exclusivity period. • This provision appears to provide additional incentives for actions and practices already underway. Line extensions of existing drugs are already common occurrences in drug development. • It is not clear how this provision interacts with the additional rebates Medicaid receives for pharmaceutical line extensions under the ACA. • It is not clear who will determine whether the drug makes a "significant improvement"—the FDA or the manufacturer? The language of this section suggests it is the latter, which would require further analysis. • It is not clear whether a drug given this status would later lose the designation if post- marketing studies show that the drug does not represent a significant improvement. If so, policymakers should consider the implications for the Medicaid (required coverage, financial impact, etc.).
Sec. 1261 – Extension of Exclusivity Periods for a Drug Approved for a New Indication for a Rare Disease or Condition • ***Extends exclusivity by 6 months for orphan drugs***	• Allowing an additional 6 months of exclusivity for drugs may delay the introduction of generics into the market and reduce overall competition. This has a direct impact on federal and state Medicaid expenditures, as Medicaid disproportionately covers the sickest and frailest populations. • Congress may consider modifying the 340B Drug Discount Program as it relates to orphan drugs for the ACA's newly covered 340B entities. There is confusion as to whether 340B pricing applies to orphan drugs purchased by these entities for

	treating a non-orphan condition. The resulting confusion makes it difficult for Medicaid agencies to accurately determine which drugs are eligible for Medicaid drug rebates and which are not (due to receiving the 340B price, which makes a claim ineligible for Medicaid rebates – the "duplicate discounts" or "double-dipping" issue). • Any action taken on 340B must not further complicate the program's administration for state Medicaid agencies. We urge federal policymakers to refer to a forthcoming NAMD paper which details existing conflicts and challenges with the intersection of the Medicaid and 340B programs. This paper will also make recommendations to resolve or mitigate these issues.
Sec. 2001 – Innovative Cures Consortium • ***Creates a public-private partnership to accelerate drug discovery and development. Sunsets on September 30, 2021.*** • ***Membership includes NIH, FDA, CMS, 22 appointed members – 5 federal agency representatives; 8 biomedical representatives; 9 academia/research, patient, provider, health plan representatives*** • ***Consortium will award grants and contracts to small businesses and nonprofits to accelerate drug and device discovery, development, and delivery***	• Legislative language should provide for a state Medicaid representative on the consortium to ensure the entity considers issues of cost and access from the state perspective. • The consortium's grant and contract program should reflect considerations and issues unique to the Medicaid program, particularly in the delivery components of the grants and contracts. • The grant program should consider certain criteria, such as Good Manufacturing Practices (GMP), previous violations, and other factors when awarding grants to small businesses and nonprofits.

Sec. 2021 – Medical Product Innovation Commission • ***Creates a new Commission, structured similarly to MACPAC and MedPAC, to make recommendations to Congress on drug development.***	• Legislative language should provide for a state Medicaid representative as part of this body to address issues of cost and access from the state perspective.
Sec. 2085 – Expanding Availability of Medicare Data • ***Sec. 2085(b)(1)(B)(ii) grants the HHS Secretary discretion to share Medicaid and/or CHIP claims data (to supplement Medicare data) with clinical data registries to support outcomes and patient safety research.***	• Medicaid data can be variable and dependent on state program and population contexts. An insufficient understanding of the nuances of state Medicaid data can produce an inaccurate picture of a state's Medicaid program. • States should have the opportunity to provide context for data requests made through this provision. • CMS should share part of the data collection fees under this provision with the states to support any state administrative costs in fulfilling data requests.
Sec. 2121 – Authority for Coverage with Evidence Development for Medical Devices under the Medicare Program • ***Allows Medicare to pay for medical devices used by patients in clinical trials.***	• Policymakers should consider the budgetary impact of this policy on Medicaid cost sharing for dually eligible beneficiaries. • This provision could require Medicaid to pay, in part, for an intervention without sufficient evidence for normal coverage under the Medicaid program.
Sec. 2141 – Regulation of Combination Products by FDA	• Combination products potentially pose a difficult reimbursement issue for Medicaid programs. For example, the device component of the product may not need to be replaced as often as the drug component needs to be refilled, but a product which packages these components together requires

• ***Requires the FDA to issue additional guidance on the review process for products that combine drugs and devices.***	states to replenish both. This scenario does not comport with Medicaid's statutory mission to operate with efficiency and economy. • Combination products also pose potential challenges for coordination across Medicaid medical and pharmacy benefits. • It would be helpful to clarify whether FDA will approve combination drug/device products as a drug vs a device. These situations have different implications for Medicaid expenditures and state budgets. FDA's approval pathway may also impact coverage determinations for "drug-only" programs, such as AIDS Drug Assistance Programs (ADAP).
Sec. 4181 – Advancing Telehealth Opportunities in Medicare • ***Requires HHS to develop, within 4 years, a Medicare coverage and payment methodology for telemedicine services that is equivalent to face-to-face service coverage and reimbursement.*** • ***Applicable services will be selected by the HHS.*** • ***HHS may waive originating site, geographic, and/or health provider limitations in this methodology.***	• Policymakers should consider the impact on Medicaid's provision of cost-sharing for Medicare-Medicaid enrollees for these services.
Sec. 4281 – Establishing PDP Safety Program to Prevent Fraud and Abuse in Medicare Prescription Drug Plans • ***Creates a pharmacy lock-in program for Part D***	• States are supportive of the Medicare Part D lock-in provision. Most states already have some type of lock-in program for Medicaid beneficiaries prescribed controlled substances or where there may be other patient safety or program integrity concerns. A comparable requirement on the Part D side could help bring consistency across the programs particularly as it pertains to Medicare-Medicaid enrollees, improve patient care

beneficiaries prescribed controlled substances. • ***Allows Part D plans to suspend pharmacy payments pending investigation of credible allegations of fraud.*** ***Sec. 4284*** • ***Requires e-prescribing of covered controlled substances.***	and safety, and prevent inappropriate use. Lock-in programs are helpful for clinical coordination even in the absence of fraud and abuse. Provisions to enhance coordination between the proposed Part D lock-in program and existing Medicaid lock-in programs should be considered. • Policymakers should consider the distinction between the federal definitions of controlled substances versus state definitions, the latter of which may be stricter. • Provider and pharmacy readiness to meet the e-prescribing provision must be considered. As there is substantial overlap between Medicare and Medicaid providers and pharmacies, Medicaid will be impacted by this requirement and may have to provide education and support to comply with it. Policymakers should consider how to support states in this work, including incorporating state prescription drug monitoring programs (PDMPs) into the e-prescribing requirement. • Policymakers should consider what occurs if pharmacies are unable or unwilling to accept e-prescriptions. Pharmacies are the primary bearers of transaction costs in an e-prescribing environment. Non-participation could seriously disrupt access to medications for Medicare-Medicaid enrollees, which are among Medicaid's most vulnerable populations.
Sec. 5001 – Extension of Exclusivity Period for American-Manufactured Generic Drugs and Biosimilars • ***Placeholder section will define "American manufactured drug" for purposes of exclusivity*** • ***Provides designated "American-manufactured" generics or biosimilars an as-yet-unspecified exclusivity extension.***	• Though currently vague, this provision could delay introduction of additional generics and biosimilars to the market, which may otherwise help to maximize Medicaid expenditures. • Policymakers should consider additional Medicaid rebates, enhanced FMAP or other policy solutions to address the financial impact to the Medicaid program from extended exclusivity.