NAMD
National Association of Medicaid Directors

March 4, 2016

The Honorable Ron Wyden
Ranking Member
Committee on Finance
United States Senate

The Honorable Charles Grassley
Member
Committee on Finance
United States Senate

Dear Senator Wyden and Senator Grassley,

We applaud your ongoing, bipartisan work to investigate and understand the totality of issues driving prescription drug pricing and expenditures. In particular, we appreciated the opportunity to help inform your joint investigation into the pricing strategies for new hepatitis C therapies. The investigation findings demonstrate the challenges that such pricing strategies present, in terms of both budgetary impact on payers and access for patients.

The National Association of Medicaid Directors (NAMD) supports and shares your goals as well as those of the Congress and Administration to address the challenging, but critically important, public policy questions that new drug innovations pose. NAMD is a bipartisan organization which represents Medicaid Directors in the fifty states, the District of Columbia, and the territories. Medicaid programs are often the largest insurers in a state, with responsibility to provide coverage for the sickest, frailest and most complex and costly patients in the country. NAMD and its members are well-positioned to identify the policy, fiscal and operational challenges posed by new pharmaceutical price benchmarks.

State Medicaid agencies are accustomed to financing high-cost drug treatments for highly specialized populations. However, recent trends in the pricing of innovator drugs and the product pipeline challenge the carefully constructed coverage and reimbursement parameters for Medicaid's optional prescription drug benefit. We continue to believe that the policy levers available to state Medicaid programs are not designed to address fundamental sustainability issues posed by high-cost, high-impact products.

There is a clear need for a public policy intervention to address issues unique to the Medicaid program, including to ensure that Medicaid enrollees have appropriate access to breakthrough drug therapies. Our response to your questions regarding the value of breakthrough

prescription drugs is enclosed. We wish to emphasize for you the following four main considerations.

First, there is a clear need for federal policymakers to revisit the Medicaid Drug Rebate Program (MDRP) in light of the sustainability issues posed by record-high drug therapy prices. As demonstrated in the case of hepatitis C therapies, without competitor products Medicaid programs are unable to exert significant leverage for negotiating supplemental rebates due to the MDRP's coverage requirements. While the introduction of competitor products has provided some relief for state Medicaid agencies, the prices net of rebate are comparatively higher than traditional drug therapies. Still, we note the utility of the MDRP in helping to lower federal and state Medicaid expenditures for outpatient prescription drug treatments that are priced consistent with historical norms.

Additionally, states must have different tools to address high-cost, high-impact drug therapies and other concerning trends that are emerging with prescription drug pricing more broadly. The traditional drug management tools available to state Medicaid agencies, such as prior authorization and generic substitution, are ineffective in addressing similarly situated drug therapies. Outside of Medicaid managed care programs, states are generally not allowed to use cost containment tools (for example, closed drug formularies, tiered copayments, mandatory mail order) used by private purchasers to control drug spending. However, it is notable that recent guidance from CMS regarding Medicaid hepatitis C coverage policies calls this existing leeway in managed care into question.

States must have the flexibility to incorporate pharmaceuticals into coverage and reimbursement models which encompass the whole person and the total cost of care. States have taken up the call for payment and delivery system reform. Pharmaceuticals, however, are noticeably absent from state Medicaid initiatives around value-based purchasing (VBP) and related work to encompass "whole person care." This situation is increasingly at odds with VBP pursued with other Medicaid providers, suppliers and vendors.

States require access to the underlying clinical data in order to design the most appropriate coverage benefit for pharmaceutical therapies. Data transparency is necessary if states are to engage in risk-based arrangements with pharmaceutical manufacturers. Pricing transparency must be paired with more relevant Medicaid population data collection in clinical trials, both to assist states in estimating potentially eligible patient populations and ensuring a drug will be effective for Medicaid's complex beneficiary population.

We believe that this complex and politically fraught issue will require a multi-pronged strategy that is applicable to the Medicaid program and the broader health care system. NAMD stands

ready to work with federal policymakers to bring the pharmaceutical space into the value conversation and to balance the myriad factors influencing this conversation.

Sincerely,

Matt Salo
Executive Director

ENCLOSURE: NAMD Responses to Senate Finance Committee Questions on Drug Policy

NAMD Responses to Senate Finance Committee Questions on Drug Policy

1. What are the effects of a breakthrough, single source innovator drug on the marketplace?

Medicaid Directors are supportive of the incredible innovations produced by the pharmaceutical sector over the past several years. The ability to cure previously chronic diseases, such as hepatitis C, is of great benefit to the health and well-being of the nation, particularly the vulnerable and medically complex Medicaid population. That being said, the pricing strategies accompanying these revolutionary therapies pose significant challenges for the Medicaid program as currently structured.

Prescription drug coverage is an optional benefit under Medicaid statute, though every state provides such coverage. Medicaid does so via the Medicaid Drug Rebate Program (MDRP), an agreement between states, the federal government, and pharmaceutical manufacturers under which Medicaid receives a mandatory rebate of 23.1% for brand drugs and 13% for generic drugs in exchange for covering all Food and Drug Administration (FDA)-approved drugs from a manufacturer. States have authority to impose limited utilization controls on these drugs via a prior authorization (PA) process, though they may not exclude coverage of a given product altogether. This is true even if a product lacks relevant evidence of efficacy among the Medicaid population, is comparatively effective or cost effective compared to currently available treatments, or is priced in a manner that results in budgetary pressures that impact other aspects of a state's Medicaid program.

Medicaid's overall financing structure, which relies on significant contributions from states on an annual or biannual basis depending on the frequency of state legislative sessions, produces unique budgetary challenges in the prescription drug space. States must prospectively estimate their pharmacy spending over their next Medicaid budget window and allocate resources accordingly, and in all but one state must do so within an overall balanced state budget.

The introduction of a high-cost, breakthrough single source innovator drug can pose unanticipated high pharmacy expenditures, inserting a significant level of uncertainty in the overall Medicaid budget. States do not have unlimited resources to finance the non-federal share; therefore, increased costs in one program area can lead to unintended and sometimes undesirable trade-offs in other program areas within Medicaid or other core areas of state government. Such measures may include reduced or eliminated optional program benefits, tightened eligibility standards, provider rate reductions or reduced agency capacity, each of which may undermine program goals, invite additional stakeholder scrutiny, and pose other significant difficulties for states. Alternatively, Medicaid programs may seek supplemental

funding from their legislatures, though in the challenging fiscal environments faced by most states such requests are difficult to secure.

One avenue to alleviate these pressures is for states to pursue supplemental rebate negotiations with manufacturers of single-source innovator drugs. However, the mandatory coverage requirements associated with the MDRP often leave states with insufficient negotiating power to secure meaningful rebates, and such rebates are generally contingent on unrestricted access to the new therapy. Absent a competitor product to leverage in negotiations, states simply do not have the flexibility under the MDRP to secure sufficiently large supplemental rebates to offset costs associated with unrestricted access.

In light of this, states generally respond to single source innovator drugs by developing clinically appropriate utilization controls and prior authorization criteria. States rely on their Pharmacy and Therapy Committees and/or Drug Utilization Review Boards to ensure such criteria reflect appropriate clinical evidence, patient characteristics, and other relevant information. However, these processes take significant amounts of time and may result in PA criteria that certain stakeholders consider unduly restrictive. This leaves states open to legal action.

Medicaid's experience with breakthrough hepatitis C therapies over the past two years is illustrative of the above points. The price point of Sovaldi and, later, Harvoni, combined with higher-than-anticipated utilization, put significant pressure on Medicaid budgets. States worked to develop appropriate PA criteria to ensure these products were being utilized appropriately, but received significant scrutiny from stakeholders. This scrutiny resulted in the unprecedented step of CMS issuing a letter to states in November 2015 which, in effect, stipulated the types of evidence and criteria states could and could not take into consideration in developing their hepatitis C coverage policies. This letter runs counter to the traditional federal-state Medicaid partnership, in which states have the flexibility to determine coverage policies consistent with the Medicaid statute. The CMS letter has been discussed in the context of legal action brought against some states.

While we recognize that CMS has an obligation to oversee state Medicaid programs to ensure their policies comport with statute and must do so in a manner which strikes a balance between competing stakeholder interests, we wish to underscore that states are not equivalent to other stakeholders. States contribute financially to the Medicaid program and are responsible for program administration, structure, coverage, reimbursement, and other critical areas.

2. Do the payers in the programs have adequate information to know the cost, patient volume, and increases in efficacy of a new treatment regimen?

Currently it is difficult to speak with certainty to the availability of Medicaid data to inform utilization projections of new treatment regiments and therapies. Such data is highly condition-specific and generally dependent on current claims by beneficiaries already seeking treatment. However, depending on the nature of the breakthrough therapy and the extent to which it makes previously untreatable conditions treatable, such data may be scarce or not available.

Again, the hepatitis C case is illustrative. Prior to the introduction of Sovaldi, hepatitis C treatments were prolonged, had only moderate success rates, and came with severe side effects which made completing a course of treatment difficult. These factors discouraged patients from seeking early treatment when they were aware of their hepatitis C status in favor of awaiting more effective treatments with fewer side effects. Additionally, the specific clinical factors of hepatitis C's disease progression mean it can take many years before treatment becomes necessary, with many persons infected not being aware of their status – making accurate estimates of the potentially-treatable patient population difficult. These considerations limited the available claims data states had to estimate hepatitis C patient volume and Sovaldi's potential impact. As a result, Medicaid programs had great difficulty budgeting for hepatitis C in their pharmacy budgets in advance of Sovaldi's release.

It is also important to note here that the populations studied in the course of clinical trials for FDA approval of a drug are often significantly different from the Medicaid population. In general, Medicaid's beneficiaries are frailer, more medically complex, and face larger health challenges than clinical trial patients. These differences mean that treatment outcomes documented in clinical trials do not necessarily reflect the real-world experience of the Medicaid program and its enrollees. This fact makes the task of assessing the comparative effectiveness of a new treatment for the Medicaid population even more difficult.

There are potential policy options which would improve states' ability to better estimate the impact of new drugs on their Medicaid programs and manage that impact:

- *Require clinical trials for breakthrough innovator drugs to reflect the Medicaid population:* Federal policymakers could require clinical trials to include patients that are representative of the Medicaid beneficiary population, ideally by having the clinical trial patient group include a number of Medicaid patients that is roughly proportional to the percentage of Medicaid enrollees nationwide. This would guarantee the generation of useful data for Medicaid

programs to estimate baseline efficacy of the new therapy for their populations, which would assist in coverage policy development. Currently, the MDRP requires states to cover therapies even in the absence of clinical trial data that reflects the Medicaid population. Until states have the flexibility to make drug coverage dependent upon demonstrated effectiveness in the Medicaid population, we believe it is appropriate to require clinical trials to incorporate patients who are representative of the Medicaid population.

3. What role does the concept of "value" play in this debate, and how should an innovative therapy's value be represented in its price?

Throughout most areas of the healthcare system, the pursuit of value is driving innovations in delivery system and payment reform. State Medicaid programs are among the leaders of this innovation, pioneering efforts such as the integration of physical and behavioral health services, making flat payments for entire episodes of care or bundling several services together in one payment, rewarding high-quality providers and managed care plans with incentive payments, designing targeted Health Home programs to provide coordinated care for the most vulnerable and challenging patients with complex care needs, and addressing social determinants of health affecting entire populations.

What is missing, thus far, from this exciting work is prescription drugs. While major public and private payers, providers, and managed care plans are increasingly moving towards incentivizing value, legal and regulatory frameworks impede similar progress in the prescription drug space.

As recent developments demonstrate, this treatment of prescription drugs as somehow distinct from other clinical interventions can and should be rethought. The contribution prescription drug therapies make to reducing the overall cost of care must be thoughtfully considered and reflected in overall value-based reimbursement strategies. There are a number of factors that should be considered in bringing prescription drugs up to speed with the rest of the healthcare system in regards to value, including:

- The comparative and cost effectiveness of the new treatment compared to existing treatments, based on long-term or tangible clinical outcomes rather than biomarkers;
- The expected timeline for accrual of savings associated with a treatment compared to its upfront costs;
    - This includes accounting for which payer makes the initial investment in the treatment versus which payer accrues the associated savings, and how to properly allocate the return on investment. For example, often Medicaid

resources are used to treat a person, but once the individual becomes eligible for another program the savings are more likely to accrue to that program.

- Accounting for treatment supports and care coordination to ensure success of the overall drug therapy; and
- Incorporating outcomes-based thinking into the value conversation.

In the Medicaid context, the requirements of the MDRP as currently structured pose barriers to value-based prescription drug purchasing. The MDRP guarantees Medicaid a "best price" that in reality functions as a price floor for a given product – yet there is no commensurate price ceiling. Manufacturers can, and do, price new treatments at ever-increasing price points and are not subject to value or performance metrics for their products. While the MDRP's mandatory rebates proved useful for alleviating pressures on Medicaid pharmacy spending in the past, the recent hepatitis C experience demonstrates the gaps of the MDRP in the face of unprecedented price points. States remain concerned by the lack of effective tools to address the growing number of highly effective, specialized therapies that will enter the market.

Potential policy options for addressing the value proposition of prescription drugs in the Medicaid context are provided in our response to question five below.

4. What measures might improve price transparency for new higher-cost therapies while maintaining incentives for manufacturers to invest in new drug development?

Currently, there is little insight into how pharmaceutical manufacturers price new therapies entering the market and, more specifically, the potential value for Medicaid populations. Providing access to relevant data would be a necessary step for aligning reimbursement strategies for prescription drugs with most other health care services. This could assist manufacturers in making the case to patients and payers, including Medicaid, that their therapies represent a true step forward, justifying their prices in terms of increased effectiveness over prior therapies or other relevant factors.

Specific steps policymakers could take to facilitate this pricing transparency include, but may not be limited to, the following:

- *Improve communication between manufacturers and payers, including Medicaid:* Similar to the option outlined above, by requiring Medicaid populations be reflected in clinical trial data federal policymakers could ensure that clinical trial findings are reflective of actual Medicaid patient experience. Further, by facilitating enhanced communication between manufacturers and payers, this data could be analyzed within a price and value context and compared to outcomes of current therapies to assist in assessing the overall value of the new drug's price point upon entry into the market. Policymakers could also consider

allowing or requiring drug manufacturers to release estimated product costs and utilization prior to a drug's FDA approval to allow Medicaid programs sufficient time to estimate the fiscal impact of the therapy.

- *Require CMS to partner with independent researchers to conduct cost effectiveness and outcomes-based research:* Many organizations conduct valuable cost effectiveness and comparative effectiveness research for new pharmaceutical therapies; several states have established relationships with such entities. This research provides articulate, quantifiable parameters to assess the relative value of a therapy at a given price point. Medicaid programs utilize this research to assist in setting their coverage policies for such therapies. Other groups can play a role by supporting comparative effectiveness research and other valuable clinical research. By creating a formal relationship between CMS and research entities, policymakers would bring clarity and transparency to the realm of pharmacy prices. Such partnerships should be structured to conduct their work in a manner that does not pose additional administrative or reporting burdens on the states and is respectful of their existing relationships with these research entities.
- *Establish Transparency and Reporting Requirements in Drug Pricing:* The President's fiscal year (FY) 2017 budget proposes requiring pharmaceutical manufacturers to publicly disclose drug production costs, including research and development investments and discounts to various payers for certain high-cost drugs. By disclosing research and development costs in particular, the reasonableness of a drug's price point as a means of bringing return on development cost investment could be independently assessed. Policymakers may also consider the utility of having manufacturers disclose spending on advertising associated with particular products to provide a full picture of costs associated with a product's launch. Such pricing transparency could also be required for generic drugs which experience sharp price increases, an issue which significantly impacts Medicaid and other payers. Further, we recommend policymakers consider the value of transparency around manufacturers' discounts to entities receiving government-identified or mandated discounts, such as 340B covered entities, to streamline the administration of other aspects of the Medicaid drug rebate program.

5. What tools exist, or should exist, to address the impact of high cost drugs and corresponding access restrictions, particularly on low-income populations and state Medicaid programs?

States are interested in designing value-based reimbursement strategies for prescription drugs provided by the Medicaid program. To do so, state Medicaid agencies require new flexibility and tools to pioneer innovative solutions and, if possible, bring these solutions to scale.

The MDRP construct is not designed to support an all-encompassing vision of value for prescription drugs, for the reasons we have discussed above. To reiterate, the MDRP does not create meaningful incentives or parameters for pricing new therapies. It also leaves states without the tools necessary to negotiate sufficient supplemental rebates to offset the high prices of new therapies appropriate for substantial segments of the Medicaid population in instances where these therapies have no competitors in class. We are concerned that these pressures will only increase with time, as more manufacturers treat the price of single-source innovator drugs as a reference point for pricing their own products. As a result, states will be forced to implement other strategies to manage these costs, which could include but are not limited to, parameters around access to specific high-cost therapies or reductions in other program areas.

States share federal policymakers' goals to make innovative therapies accessible in a fiscally responsible manner to the patients that need them. This requires a multipronged strategy to address the distinct but related issues around pricing and access. To that end, we encourage policymakers to continue to explore and develop a range of policy options, which include, but may not be limited to, the following options.

We organize these options here as those which could function under the MDRP as it currently exists; options which would require a new approach to the MDRP; and options which are agnostic to MDRP policy changes.

Options Enhancing the MDRP:

States find significant value in the MDRP, despite the challenges posed by high-priced breakthrough therapies. While the following options work within the MDRP's current structure, policymakers must be cognizant of how modifications to one aspect of mandatory rebates could invite a corresponding change to rebates in other ways.

- *Enhance FMAP for high-impact innovator drugs:* Innovator drugs meeting a certain price threshold could trigger an automatic enhanced federal medical assistance percentage (FMAP) for state Medicaid programs. This measure would ease budgetary pressures on states, but would increase federal Medicaid costs. A potential consideration could be to make this FMAP adjustment available for a set number of quarters or the time period during which there is no innovator drug in its class, or whichever is longer. Some mechanism would be needed to ensure that the enhanced federal support remains available until states are able to secure meaningful supplemental rebates, once competitor products are introduced. Policymakers may also consider linking the length of the enhanced match to utilization trends and budgetary impacts associated with the

new therapy, and be structured in a manner which strikes a balance between state Medicaid programs' stability and underlying market dynamics.

- *Require additional manufacturer rebates for high-impact innovator drugs:* Innovator drugs meeting a certain price threshold could trigger an automatic enhanced manufacturer rebate under the MDRP. This measure would provide savings to both states and the federal government. We recommend that this enhanced rebate be shared jointly between the states and the federal government, per the state's FMAP.
- *Create MDRP waivers for the purposes of value-based purchasing:* Every state operates at least one waiver under the Medicaid program. Waivers are a key tool for providing states with flexibility to innovate, and have been critical to the early successes states are already seeing in value-based purchasing. However, it is currently unclear to what extent states may enter into value-based purchasing arrangements with manufacturers under the MDRP. States need clarity as to whether such arrangements are possible under existing program authorities, such as waiver vehicles. If such authority is not available, policymakers should consider modifying Medicaid statute to allow states to bring prescription drugs into the value-based purchasing arena, via a waiver vehicle or other mechanism.

Options Significantly Altering the MDRP:

- *Permanently modify the MDRP:* Should a waiver or demonstration program prove the viability of state value-based purchasing strategies, a permanent modification to the MDRP to allow such payments without seeking waiver authority would be warranted. One potential modification would be to grant states the authority to conduct data-driven, public development of cost effectiveness coverage criteria, which would dictate the drugs states would cover in their Medicaid programs. This would give states the same authority to exclude drugs from coverage currently possessed by private payers and Medicare.
- *Create a federal-state Medicaid negotiating pool:* The President's FY 2017 budget proposes that CMS and states jointly partner with a private sector contractor to negotiate supplemental rebates from manufacturers. While the effectiveness of such a negotiating pool is highly dependent on how each of its participants views the program, it could provide states with significant negotiating power that they currently do not possess. Further, a negotiating pool should include the option to incorporate the principles of value-based design, and states should have the option to leverage the pool on a per-drug basis rather than for all drug negotiations. We note that further consideration is needed as to how this concept might be applicable in states where outpatient drug coverage is part of a capitated arrangement with managed care entities.

- *Require coverage with evidence development in Medicaid:* The President's FY 2017 budget proposes that Medicare Part A and B's coverage with evidence development process apply to Medicare Part D. Policymakers may wish to explore extension of this policy to the Medicaid program. This would require that for certain high-impact drugs, manufactures be required to undertake further clinical trials and data collection to support the use of the drug in the Medicaid population. CMS should, in consultation with states, also identify relevant Medicaid subpopulations for this additional data collection.
- *Enhance state flexibility to develop prior authorization requirements for drugs approved by the FDA with "Breakthrough" status:* Drugs approved by the FDA through the "Breakthrough" designation's expedited process have lower thresholds to reach regarding evidence of safety and effectiveness. Currently, states are required to cover such drugs for the vulnerable Medicaid population despite these lower evidentiary thresholds. States should be allowed flexibility to restrict coverage of breakthrough drugs until a more complete body of clinical evidence exists.

Options Independent of the MDRP:

- *Create a Medicaid risk corridor program for high-impact innovator drugs:* Risk corridors in health insurance markets provide a safeguard against unanticipated service utilization patterns, while maintaining an element of risk to incentivize appropriate service delivery. A similar approach could be utilized in the Medicaid prescription drug space, by creating a risk corridor under which state Medicaid programs are guaranteed enhanced federal assistance for innovator therapy utilization which exceeds utilization estimates developed by states and approved by CMS.
- *Enhance federal support for Medicaid case management services:* As more innovative breakthrough therapies enter the market at high price points, states will likely increase their investments in case management services to provide clinical supports that ensure patients successfully complete treatment courses. We recommend that federal policymakers consider avenues for supporting these case management efforts, as unsuccessful treatment courses represent poor outcomes for Medicaid beneficiaries and inefficient use of scarce Medicaid resources.