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Re: Potential approaches to address the challenges posed to Medicaid capitation rates by Breakthrough Therapy Designation medications, including Sovaldi for Hepatitis C

Deputy Administrator Mann and Director Edwards,

I am writing to you on behalf of the American Academy of Actuaries¹ Medicaid Work Group regarding an emerging high-cost issue affecting Medicaid programs. The rapid introduction and associated uncertainties of new therapies, such as Breakthrough Therapy Designation (BTD)² medications, have made it more difficult for state operational and budget staff, and actuaries, to accurately project future Medicaid expenses based on historical experience. Although this uncertainty affects both fee-for-service-based Medicaid and risk-based Medicaid managed care, this letter focuses primarily on issues for risk-based Medicaid programs. In particular, it outlines various options for mitigating the uncertainty regarding future expenses, within the requirement of developing actuarially sound³ Medicaid capitation rates. Given the cost uncertainties associated with BTD medications such as the Sovaldi Hepatitis C drug, as well as other emerging

¹ The American Academy of Actuaries is an 18,000+-member professional association whose mission is to serve the public and the U.S. actuarial profession. The Academy assists public policymakers on all levels by providing leadership, objective expertise, and actuarial advice on risk and financial security issues. The Academy also sets qualification, practice, and professionalism standards for actuaries in the United States.

² The Food and Drug Administration designates a breakthrough therapy as a drug: intended alone or in combination with one or more other drugs to treat a serious or life threatening disease or condition; and, preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.

³ For more information on actuarial soundness, see the Academy's 2005 practice note, "Actuarial Certification of Rates for Medicaid Managed Care Programs."

http://actuary.org/files/publications/Practice_Note_Actuarial_Certification_Rates_for_Medicaid_Managed_Care_Programs_aug2005.pdf.

high-cost medical therapies, a key factor in ensuring that capitation rates are actuarially sound will be to give states and their actuaries flexibility in the funding approaches they may use. The following chart summarizes a range of potential pricing methodologies and risk-mitigation strategies compiled by this work group that states could consider using to help achieve the requirement of actuarially sound Medicaid capitation rates. When assessing each option, states should consider how it would affect administrative expenses, state budget predictability/neutral, payment accuracy implications, incentives for health plans to manage costs, equitability of premium allocation among plans, and whether it would be appropriate to implement the option on a permanent basis, or only on a temporary basis until sufficient experience has emerged and stabilized.

Table 1. Potential pricing methodologies and risk-mitigation strategies for addressing cost uncertainties associated with Breakthrough Therapy Designation medications

Pricing Methodology / Risk Mitigation Strategy	Considerations
Prospective trend with or without risk adjustment	<ul style="list-style-type: none"> - No additional administrative complexity - State budget predictability - Exposes states and plans to over- and under-payment risk - Maintains plan incentives to manage cost and utilization - Doesn't allow emerging experience before including in rates - Risk adjustment may enhance allocation of premium revenue among plans
Prospective trend with risk pool, reinsurance, and/or risk corridor	<ul style="list-style-type: none"> - Administratively more complex - May or may not be state budget neutral - Reduces but does not eliminate plan incentives to manage cost and utilization - Doesn't allow emerging experience before including in rates - Enhances allocation of premium revenue among plans - Introduces cash flow issues
Supplemental payment	<ul style="list-style-type: none"> - Administratively more complex - May or may not be state budget neutral - Mitigates the risk of over- or under-payment - Maintains plan incentive to manage cost per treatment; significantly diminishes incentive to manage utilization - Allows experience to emerge before including in rates - Enhances allocation of premium revenue among plans
Pass-through payment, reconciliation, or carve-out	<ul style="list-style-type: none"> - Administratively more complex - Creates uncertainty in state budget - If fully/100% employed, removes plan incentive to manage cost and utilization - Allows experience to emerge before including in rates

Each of these options is discussed in more detail later in this document.

Of course, ensuring that capitation rates are actuarially sound is not the only challenge presented by BTD medications and emerging high-cost medical technologies. Managing the cost of care,

while maintaining appropriate access to care and quality of care, is equally vital. Some state Medicaid agencies have already taken the important step of developing clinical guidelines, protocols, and policies for BTD medications that aim to balance access, quality, and cost goals. Consistent with Academy initiatives⁴ around positively impacting the health care spending curve, the work group encourages the Centers for Medicare and Medicaid Services (CMS), states, health plans, and other stakeholders to continue working collaboratively to explore options around pricing and contracting, care management techniques, and other available tools to ensure the affordability of Medicaid. The National Association of Medicaid Directors' Oct.28, 2014, letter to Congress contains several important policy strategies (pages 6-7) for consideration.⁵

Background

For over 20 years, the Food and Drug Administration (FDA) has utilized an expedited review and approval process for certain qualifying drugs. Through mid-2012, in addition to the non-expedited standard process, expedited review and approval included Fast Track Designation, Accelerated Approval, and Priority Approval. On July 9, 2012, a fourth expedited program was added, the BTD. BTD accelerates the development of medications that are intended to treat serious diseases or conditions. The BTD is granted when preliminary clinical evidence demonstrates that the medication is substantially better than existing therapies. There is considerable overlap in the expedited programs, and hence it is impossible to strictly categorize a particular drug into one program or another. But the concern around accelerating FDA expedited review and approval is clear. From calendar year (CY) 2008 to CY 2013, the FDA's Center for Drug Evaluation and Research's (CDER) drug and biologic approvals (including standard) increased approximately 15 percent.⁶ While the annualized rate of growth (almost 3 percent) is material, it was only minimally impacted by BTD drugs. In CY 2013, the number of BTD drug approvals was only three (including Sovaldi). However, in the first 9.5 months of CY 2014 (through mid-October, 2014), 10 BTD drugs had received approval.⁷

Challenges for Actuarially Sound Capitation Rate Development

Capitation rates paid to risk-based Medicaid health plans must be certified as actuarially sound by a qualified actuary and approved by CMS. One of the primary components of the rate development process is the projection of historical expenses to a future rate period, using adjustments for expected changes in those expenses between the periods. The actuary may use a range of assumptions to develop an actuarially sound rate range. The reasonableness, appropriateness, and attainability of the actuarial rate range is dependent, in part, upon corresponding estimates of future expenses. A new therapy, such as a BTD medication, has the potential to diminish the ability of the actuary to accurately project future expenses from historical experience because of the significant change in practice patterns and costs that such new treatments can create.

⁴ <http://www.actuary.org/healthcosts>

⁵ http://medicaiaddirectors.org/sites/medicaiaddirectors.org/files/public/namd_sovaldi_letter_to_congress_10-28-14.pdf

⁶ <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/DrugandBiologicApprovalReports/NDAandBLAApprovalReports/ucm373413.htm> (CDER Drug and Biologic approvals by year)

⁷ <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/DrugandBiologicApprovalReports/NDAandBLAApprovalReports/ucm373418.htm> (CDER BTD approvals by year)

Medicaid actuaries typically develop capitation rates for one-year periods and the rates are often finalized several months prior to the effective date. An actuary developing assumptions six months prior to the rate period will be estimating expenses as far as 18 months into the future. With base data sources often being 24 months or more from the rate period, projections of new treatments can be particularly challenging. The speed of the approval process for a BTD medication could mean that the actuary is not able to completely and accurately incorporate the BTD medication expenses in the rate-setting process.

The aggregate costs of BTD medications are expected not only to be high, but also highly variable, for the following reasons:

1. The addition of the BTD to the FDA's expedited review and approval process appears to have accelerated the number and timing of higher cost medications into the health care system.
2. The timing of FDA approval for a BTD medication is often uncertain.
3. The cost per treatment is expected to be very high for BTD medications.
4. The utilization (both number of individuals impacted, as well as prescriptions per individual) of the current therapies are likely poorly approximates the future utilization of BTD medications.

Case Study – Sovaldi

The recent introduction of Sovaldi for the treatment of Hepatitis C illustrates the challenge in developing actuarially sound capitation rates that include new BTD medications. Before the release of Sovaldi™ in December 2013, Hepatitis C treatments required longer therapies with multiple pills per day, had significant side effects and lower efficacy rates. Sovaldi is currently viewed as the most effective Hepatitis C treatment, and has fewer side effects than alternative treatments. By definition, a BTD medication (like Sovaldi) should be superior to the current medication treatments. Therefore, there is less pressure for the BTD medication to compete on price. A challenge facing actuaries is forecasting the price of a BTD medication. Currently, Sovaldi's wholesale acquisition cost (WAC) is \$1,000 per day (a 27 percent increase over the prior treatment's daily cost), and treatment typically lasts 12 weeks, but may run to 24 or even 48 weeks. Actual total costs could also vary depending on such factors as rebate and discount negotiations and the availability of 340b pricing.⁸

A second challenge actuaries face is projecting utilization, which involves estimating not only disease prevalence but also the share of the afflicted population undergoing treatment. Several chronic diseases historically have only a small share of the afflicted population undergoing treatment. Treatment may have been forgone due to being ineffective, unpleasant, inconvenient, or for a host of other reasons. As such, the utilization of existing therapies is likely to be a poor predictor of utilization for a new BTD medication. In addition, some of the afflicted population may be unaware of their condition. As of mid-year 2014, Sovaldi's utilization may still have been on the increase, but it is likely that only a relatively small fraction of the known infected

⁸ The 340B Drug Pricing Program requires drug manufacturers to provide outpatient drugs to eligible health care organizations/covered entities at significantly reduced prices. For more information on the 340b Drug Pricing Program, please see the following Health Resources and Services Administration (HRSA) web page (<http://www.hrsa.gov/opa/>).

Hepatitis C population is undergoing treatment. The future consumption of Sovaldi and other Hepatitis C medications by those not yet receiving treatment is largely uncertain. With BTDs, the expectation of new, even more effective treatments on the horizon may cause physicians to wait for the new treatment and further distort utilization patterns. Specifically in the case of Hepatitis C, there is a new medication, Ledipasvir, which was released in October 2014 that will also be used in the treatment of Hepatitis C. The projection of cost and utilization for this drug is also complex as it treats orally, without injections of interferon, thus likely increasing treatment compliance.

Medicaid Pricing Considerations for BTD Medications

Actuaries are having to address the rate-setting complexities brought about by high-cost BTD medications in a manner that maintains their ability to certify capitation rates as actuarially sound. The certification is generally performed in the aggregate for all Medicaid risk-based health plans in a state. Actuaries consider state mechanisms in place, or to be put in place, that reduce costs or risks associated with BTD medications, such as: 1) availability of 340b pricing, or rebates; 2) state-specific drug formularies; and 3) treatment protocols and coverage criteria (such as in the case of Sovaldi, applying to those with moderate or severe liver disease; or as labeled advanced liver disease). Actuaries are having to consider the degree to which they are confident they can estimate the new BTD medicines and their potential volatility within total pharmacy expenditures. Actuaries may also consider the appropriate spreading of risk in and among the health plans as well as using a combination of pricing methodologies.

Described below are potential pricing methodologies and risk-mitigation strategies that states might consider using to help achieve the goal of actuarially sound Medicaid capitation rates. For additional detail on several of the concepts described below such as risk adjustment, risk pools, reinsurance, risk corridors, supplemental payments, pass-throughs, or carve-outs, please see the Work Group's June 16, 2011, presentation to CMS on Medicaid "Capitation Rate Development Process and Considerations."⁹

Prospective trend with or without risk adjustment

Under general rate-setting methodologies, medical and pharmacy spending for the effective rate period is projected by applying specific medical and pharmacy adjustments and trend factors to base period spending data. Estimating trends are often done using several sources of information and assumptions for the changes in utilization and unit cost by categories of service. In the case of a newly introduced BTD medication, the methodology will rely more heavily upon assumptions than actual historical experience. This increases the risk of estimation error for this component of the capitation rate.

Many states have risk-adjustment programs that mitigate health plan risk due to selection among plans. However, risk adjustment does not reduce the overall uncertainty of aggregate cost and utilization of BTD medications. Additionally, risk adjustment is calibrated using historical medical and drug expenses, which might not reflect treatment costs for particular conditions after the introduction of a BTD medication. It would be a significant challenge to recalibrate the risk-

⁹ http://www.actuary.org/files/publications/Medicaid_Work_Group_CMS_Presentation_Final.pdf

adjustment cost weights in a timely fashion to accurately reflect the BTD medication expenses with a disease state due to the uncertainty of the utilization rates.

Prospective trend with risk pool, reinsurance, or risk corridor

The addition of a risk pool, reinsurance, or risk corridor program would help reduce the volatility among health plans for risks such as adverse selection and small sample sizes. A risk pool typically is budget neutral so it would not reduce aggregate health plan risk around the overall uncertainty of the cost and utilization of BTD medications, but would re-allocate funds to plans disproportionately impacted. A reinsurance mechanism can limit excess risk to health plans, but would not necessarily maintain budget neutrality. A risk corridor program would add additional administrative complexity, but if implemented as a two-sided risk corridor it would protect not only health plans from under-payment risk, but also the state from over-payment risk. These strategies would at least partially preserve health plan incentives to manage BTD expenses, while also providing pricing protection. Each can be designed to maintain the benefit of state value-based purchasing.

Supplemental payments

A supplemental payment is generally a payment, outside of the normal capitation rate, that is paid once a predefined trigger has occurred. The payment is a per-occurrence payment as opposed to an amount included in the capitation rate. This approach is used extensively for payments related to maternity delivery and neonatal care in Medicaid managed care.

Supplemental payments also have been used when a new treatment has been added to Medicaid managed care but there was not sufficient experience to determine the expected utilization of the treatment. For example, the state of Texas implemented a supplemental payment to reimburse plans for the expected costs of bariatric surgery when the procedure was first introduced as a Medicaid-covered service. Using a similar approach for BTD medications would have the benefit of mitigating health plan utilization risk. The supplemental payment could be structured to cover most – but not all – of the expected treatment cost, leaving the health plans continued incentive to manage unit-cost spending. This approach might introduce additional administrative complexities and create some budget uncertainty for the state, but it would effectively eliminate the state's risk of over- or under-paying plans in the aggregate and addresses issues related to properly allocating premium revenue across plans.

Pass-through payment or reconciliation

Several states have used a pass-through approach for risks that are uncertain and may not be subject to direct management by the health plans. Examples of expenditures subject to pass-through payments include HIV/AIDS medications and hospital bed day taxes. Under reconciliation, plans would be paid capitated rates, with subsequent cost reconciliation. This methodology removes the pricing risk for the health plans, but creates budget uncertainty for the state. Additionally, this methodology removes incentives for plans to manage expenses and utilization. At least one state uses a reconciliation approach with separate capitation rates for HIV/AIDS medications rather than including those expenses in the base pricing or in risk adjustment.

Carve-out

A carve-out is generally defined as a service that is not part of the health plan benefit requirements, but is covered through Medicaid fee-for-service or a separate managed care contract. Common examples of carve-outs in Medicaid managed care include: non-emergency transportation, dental care, long-term care, and behavioral health care. In addition, several states currently carve out specific classes of pharmacy benefits such as HIV/AIDS or hemophilia medications for reasons similar to the risks presented by some BTD medications. This approach removes the pricing risk for the health plans but creates budget uncertainty for the state. This approach has no incentives for health plans to manage costs and utilization for these treatments as they are not part of the health plan contract.

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As evidenced by the recent introduction of Sovaldi, new BTD medications and other emerging medical technologies pose challenges to state Medicaid managed care programs. Because of the uniqueness of each state's Medicaid program, its philosophy, and available resources, it would be helpful for CMS to give states flexibility when exploring and incorporating new pricing approaches and risk-mitigation strategies to address these challenges. When assessing alternative approaches, CMS and states should consider various factors, including the potential impact on administrative burden, state budget predictability/neutralilty, payment accuracy implications, and incentives for health plans to manage costs. In addition, CMS, states, health plans, and other stakeholders should continue working collaboratively to explore options around pricing and contracting, care management techniques, and other available tools to ensure the affordability of Medicaid.

We welcome the opportunity to discuss any of the potential approaches presented in this letter with you at your convenience. If you have any questions or would like to discuss these items further, please contact Tim Mahony, the Academy's state health policy analyst at 202.223.8196, or Mahony@actuary.org.

Sincerely,

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Cc: / Matt Salo, Executive Director, National Association of Medicaid Directors