



OUTCOMES-BASED ARRANGEMENTS

*Sustainable Financing for
Transformative Therapies
and a Review of
State Activity*

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Executive Summary

As newly developed, innovative therapies come to market, policymakers continue to explore sustainable financing solutions to pay for these life-changing medicines. Outcomes-based arrangements (OBAs) - which base reimbursement on whether or not a therapy worked- are one tool to help manage costs. This is particularly applicable to gene therapies for rare diseases, where the pipeline is robust, and the need is great. The U.S. federal government has taken steps through rulemaking to enable these arrangements. Some states are leading the way through Medicaid State Plan Amendments (SPAs), with Oklahoma Medicaid implementing some of the earliest OBAs. As more states investigate financing options for gene therapies, this paper reviews example OBAs and key lessons learned from states with existing contracts.

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Introduction

The United States spends more on healthcare than any other country.¹ Yet despite this spending, life expectancy in the United States trails behind other developed countries.² To alter this trajectory, we must re-frame how care is reimbursed and build a more sustainable financing system that pays for value.

A major contributor to the current state of the U.S. healthcare system is the widespread use of traditional managed care models that restrict access to costly treatments through utilization management and other techniques. These models use cost as the key metric to control access. As systems and technology have evolved, alternative systems that encourage innovation by paying for value rather than restricting access based on cost alone, are emerging.

One such method is the implementation of outcomes-based arrangements (OBAs), where payments are tied to the results - or value - of a therapy or procedure rather than volume. These OBAs can be applied to various healthcare models and prescription drugs - in particular, gene therapies are ripe for changes in reimbursement. This is due to multiple factors: the rare nature or unmet need of some of the conditions treated by these gene therapies, the relatively high cost of such treatments, and the need to collect and analyze clinical data regarding outcomes and durability.

ALSO KNOWN AS (AKA)

VALUE-BASED ARRANGEMENT (VBA): Catch-all term for any type of innovative contracting where price is based on some definition of value or quality; often used for provider payments

OUTCOMES-BASED ARRANGEMENT (OBA): Generally used for prescription drugs, OBAs base payment on patient outcomes

VALUE-BASED PURCHASING ARRANGEMENT (VBP): Official U.S. government (CMS) definition targeted to prescription drugs; agreement that aligns payment to an observed or expected therapeutic or clinical value (outcomes relative to costs) in a population

¹ In 2019, the United States spent nearly 18 percent of its Gross Domestic Product (GDP) on national health expenditures, totaling \$3.8 trillion dollars; available: <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/NHE-Fact-Sheet>.

² In 2019, life expectancy at birth in the U.S. was 78.9 years, which is the lowest among countries with high GDP per capita; from Kaiser Family Foundation's Health System Tracker; available: <https://www.healthsystemtracker.org/chart-collection/u-s-life-expectancy-compare-countries/>

Gene therapies are already transforming care delivery and improving patient lives in ways previously thought to be beyond our capabilities. By addressing non-functioning genes (often in a single prescribed course of treatment), gene therapies target the underlying cause of disease and can potentially make long-lasting changes that transform patients' lives. Patients may be able to engage in activities they never thought possible, often achieving basic functions that most of us take for granted in our everyday lives.

Given the significant value these therapies can offer patients, caregivers, and the healthcare system, coupled with the realities of complex research and development programs and relatively small patient populations,³ the list price can also be significant. With ten gene therapies currently⁴ on the market in the U.S. and more in development,⁵ these transformative therapies may quickly become commonplace for certain disease states. To deliver on the promise to help patients while recognizing the financial constraints of many payers, including state Medicaid programs, the time to develop public policy to apply sustainable financing models - like OBAs - is now. Conversely, to ignore the pipeline of costly life-changing treatments that are on the horizon will result in fragmented policies that will likely restrict patient access and fail to build the concept of value into financing mechanisms.

How a particular state Medicaid program moves forward to implement OBAs can take different paths, which are outlined in greater detail below. These steps include the process laid out in the Centers for Medicare & Medicaid Services (CMS) Medicaid VBP Rule; submission of a state plan amendment (SPA) requesting authority to enter into OBAs; and/or passage of state legislation, where required.

The goal of this paper is to provide greater understanding of the current landscape of OBAs among public payers, focusing on lessons learned from existing agreements within state Medicaid programs and exploring the regulatory environment for such arrangements.

³ Recent projections indicate an average of 93,000 patients will be treated by cell and gene therapies by 2030, which is a fraction of one percent of the population; Young, CM, et al. Durable cell and gene therapy potential patient and financial impact: U.S. projections of product approvals, patients treated, and product revenues; Drug Discovery Today; 27(1)17-30; 2020; available: <https://www.sciencedirect.com/science/article/pii/S1359644621003901?via%3Dihub>

⁴ As of Q3 2023, the following gene therapies have been approved in the U.S.: ADSTILADRIN®, ELEVIDYS®, HEMGENIX®, IMLYGIC®, LUXTURNA®, ROCTAVIAN®, SKYSONA®, VYJUVEK®, ZOLGENSMA®; and ZYNTEGLO®; available: <https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products>

⁵ There are currently over 2,000 gene therapies in clinical development; available from the American Society of Gene and Cell Therapies Q2 2022 Quarterly Data Report; available: <https://asgct.org/global/documents/asgct-pharma-intelligence-quarterly-report-draft-q.aspx>

Policy Environment

Value-based reimbursement achieves cost savings from improved disease management that targets results (e.g., did the patient improve?) rather than procedures (e.g., did the patient receive the service as prescribed?). Conceptually, this means that payers would only reimburse for treatments that work in the manner expected. Even in today's polarized political environment, many policymakers and thought leaders have expressed the need to better incentivize effective care for patients. To that end, several strategies have emerged to transition the current system toward value-based care - such as the Center for Medicare and Medicaid Innovation (the Innovation Center) Enhanced Oncology Care Model⁶, Bundled Payments for Care Improvement Initiative⁷, and Next Generation Accountable Care Organizations⁸. In addition, Medicaid programs are increasingly implementing value-based strategies with the Centers for Medicare & Medicaid Services (CMS) offering technical assistance to states for value-based payment approaches through their Innovation Accelerator Program.⁹

Until recently¹⁰, prescription drugs have been left out of this transition to value-based care as government-initiated reforms have focused on services, with drugs being an afterthought or specifically carved out.¹¹ This has left pharmaceutical manufacturers (manufacturers) and payers (both public - i.e., state Medicaid programs - and private - i.e., commercial plans) on their own to develop OBA models.¹²

These models have had some success in the private sector with calls for expansion into federal programs (i.e., Medicare and Medicaid). However, barriers still exist and the resulting advocacy around prescription drug OBAs encouraged the federal government- under two different Administrations- into acting. CMS, under the Trump Administration, promulgated a Medicaid VBP Rule at the end of 2020,¹³ which went into effect in July 2022. That rule is intended to facilitate value-based payments for prescription drugs (discussed

⁶ <https://innovation.cms.gov/innovation-models/enhancing-oncology-model>

⁷ <https://innovation.cms.gov/innovation-models/bundled-payments>

⁸ <https://innovation.cms.gov/innovation-models/next-generation-aco-model>

⁹ <https://www.medicaid.gov/resources-for-states/innovation-accelerator-program/functional-areas/value-based-payment/value-based-payment-financial-simulations/index.html>

¹⁰ February 2023 CMMI Announcement here: <https://innovation.cms.gov/data-and-reports/2023/eo-rx-drug-cost-response-report> ; and July 2022 implementation of 2020 CMS VBP Rule here: <https://www.medicaid.gov/prescription-drugs/downloads/mfr-rel-116-vbp.pdf>

¹¹ The Office of the Inspector General (OIG) and Centers for Medicare and Medicaid Services (CMS) finalized regulations that created new safe harbors under the Anti-Kickback Statute (AKS) and Stark Laws for certain value-based arrangements but specifically carved out pharmaceutical manufacturers and pharmacy benefit managers from participating in these arrangements, thereby omitting prescription drug OBAs; OIG rule accessed here: <https://www.federalregister.gov/documents/2020/12/02/2020-26072/medicare-and-state-health-care-programs-fraud-and-abuse-revisions-to-safe-harbors-under-the>; CMS rule accessed here: <https://www.federalregister.gov/documents/2020/12/02/2020-26140/medicare-program-modernizing-and-clarifying-the-physician-self-referral-regulations#sectno-reference-411.351>

¹² https://phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/P-R/PhRMA_InnovativeContracts_Sep2020.pdf

¹³ <https://www.federalregister.gov/documents/2020/12/31/2020-28567/medicaid-program-establishing-minimum-standards-in-medicaid-state-drug-utilization-review-dur-and>

in more detail on page 9). Then in October 2022, President Biden released an Executive Order¹⁴ that directed CMMI to develop and test models for reducing prescription drug costs that also improve access, specifically referencing value-based payments. As a result of that Executive Order, in January 2024, CMMI announced the Cell and Gene Therapy Access Model¹⁵ would initially focus on sickle cell disease (SCD) gene therapies, where CMMI would coordinate and administer multi-state OBAs as voluntarily requested by state Medicaid agencies.

While the federal government is implementing its various policies, state Medicaid programs have taken charge. Medicaid spending on molecular targeted therapies doubled between 2015 and 2019 and accounted for the fifth most costly drug group in 2019.¹⁶ This trend is expected to continue if not addressed due to the robust pipeline of transformative therapies currently in development. To this end, more than twenty state Medicaid programs have already received approval from CMS for State Plan Amendments (SPAs)¹⁷ that enable them to enter into OBAs with manufacturers.

Outcomes-Based Arrangements 101

What is an OBA?

Outcomes-based arrangements (OBAs) are agreements between payers and manufacturers that tie reimbursement to a drug's effectiveness. Should it be determined the drug is not successful because the patient did not meet predetermined outcomes, the manufacturer rebates, refunds, or repays money back to the payer. Payers would only be responsible for paying for therapies that improve patient outcomes, which would reduce wasteful spending in the system by delivering the right care to the right patient at the right time.

How do OBAs Work?

Feasibility and implementation are centered on a contractual arrangement between the manufacturer and the payer¹⁸ where a patient's outcome determines the ultimate reimbursement rate. According to the contract, should the drug prove successful for the patient, the manufacturer retains the original payment. Conversely, should the drug fail to achieve the agreed-upon outcomes, the manufacturer would provide additional rebates, refunds, or repayments to the payer (potentially amounting to a significant portion of the Wholesale Acquisition Cost or WAC), depending on the terms of the contract.

¹⁴ <https://www.whitehouse.gov/briefing-room/presidential-actions/2022/10/14/executive-order-on-lowering-prescription-drug-costs-for-americans/>

¹⁵ <https://www.cms.gov/priorities/innovation/innovation-models/cgt>

¹⁶ <https://www.kff.org/medicaid/issue-brief/utilization-and-spending-trends-in-medicaid-outpatient-prescription-drugs-2015-2019/>

¹⁷ <https://www.medicaid.gov/medicaid/medicaid-state-plan-amendments/index.html>

¹⁸ OBAs can also be contractual agreements between providers and manufacturers or between providers and payers.

This model stands in direct contrast to the traditional approach for pharmaceutical reimbursement, which incentivizes prescription volume rather than patient outcomes; manufacturers and prescribers are reimbursed per pill regardless of the results. Such practices lack focus on coordination of care and, therefore risk truncated and inefficient patient care, suboptimal clinical results, and large bills for payers and patients. Conversely, OBAs incentivize delivery of the right treatment to the right patient population (where the treatment is most likely to be effective), thus minimizing payments for ineffective treatments.

This is especially noteworthy due to the recent trend toward the development and prescribing of more targeted drug therapies for smaller patient populations, which tend to be more expensive than traditional therapies for larger populations. Under the current system, even a potentially high value drug can be labeled “low value” due to its high price tag and limited number of impacted patients (often those with rare medical conditions), and consequently can lead insurers to implement measures that restrict access to those products. Since OBAs incentivize the right drug for the right patient, there is a higher likelihood of lower downstream costs by minimizing exacerbated medical conditions, which mitigates the perceived need for access restrictions payers may implement.

How are Outcomes Tracked?

To ensure value for patients, clearly defined and measurable metrics can be used to judge the performance of a particular drug or treatment. As referenced, OBAs can condition payment on a variety of outcomes such as medication adherence, reduced rates of hospitalization, or certain biomarkers such as reduction of tumor size for specific cancers or the amount of clotting factor used for some bleeding disorders.

Claims data are the most common method for tracking outcomes today. However, using only claims data limits the type of outcome information that can be collected. De-identified patient-level clinical data captured in electronic health records (EHR) or submitted by a provider to a third party can also be used, but the barriers around data sharing, administrative burden, and privacy concerns make this more difficult. However, as technology and regulations around interoperability and data sharing evolve, the promise for OBAs to yield even greater savings based on more specific patient outcomes is significant.

Regulatory Barriers to Outcomes-Based Arrangements

As transformative therapies continue to receive approval from the Food & Drug Administration (FDA) and as states further explore alternative payment mechanisms, both public and private payers are increasingly interested in OBAs and similar models that incorporate value in payments to drug manufacturers. However, one of the largest and most often cited deterrents to the widespread use of OBAs is the best price¹⁹ provision of the Medicaid Drug Rebate Program (MDRP). This provision stipulates that Medicaid should pay no more than the lowest price a manufacturer offers to any other provider, payer, or retailer (with some caveats). If a manufacturer enters into an OBA and provides a rebate, refund, or repayment to a private payer for just one non-responding patient, that would lower the best price of the drug and require the manufacturer to provide the drug at the new low price to the entire Medicaid population, regardless of patient outcomes. This discourages manufacturers from offering large rebates, refunds, or repayment terms as part of their OBAs.

Similarly, both the Average Manufacturer Price (AMP)²⁰ and Average Sales Price (ASP)²¹ must also be addressed regarding how they are calculated for drugs that have an OBA. The AMP is the average price paid to a manufacturer by wholesalers for drugs distributed to retail pharmacies. The ASP is calculated based off the sales from manufacturers to all purchasers, including discounts. The federal government uses AMP to determine how manufacturers rebate drugs in Medicaid²² and ASP for Medicare Part B (physician-administered) drugs. Unless OBAs are exempted from both price reporting calculations (AMP and ASP), the potential rebates, refunds, or repayments due for non-responding patients could artificially skew how AMP and ASP are reported.

Lastly, the fraud and abuse laws including Stark²³ and the Anti-Kickback Statute (AKS)²⁴ may present barriers for OBAs. Stark prohibits a physician from making referrals for certain designated health services, which include prescription drugs, that are reimbursed by CMS. The AKS prohibits providing anything of value to induce the purchase of items or services reimbursed by federal healthcare programs. Since both laws are fairly broad in their application, there is some concern OBAs could trigger them regarding the refunds, rebates, or repayments provided (i.e., the Office of the Inspector General [OIG] could view the refunds as "something of value" and all parties participating in the OBA could be subject to criminal prosecution unless OBAs are clearly defined in a safe harbor).

¹⁹ 42 U.S.C. § 1396r-8(c)(1)(C)

²⁰ 42 U.S.C. § 1396r-8(k)(1)(A)

²¹ 42 U.S.C. § 1395w-3(a)

²² AMP also serves as the basis for 340B pricing.

²³ 42 U.S.C. § 1395nn

²⁴ 42 U.S.C. § 1320a-7b

CMS Efforts to Promote Adoption of Value-Based Payments for Drugs

CMS Medicaid VBP Rule

In response to the regulatory and statutory obstacles that undermined the agency's support for more widespread adoption of value-based payments, CMS promulgated regulations²⁵ at the end of 2020, which went into effect in July 2022, to provide flexibility to manufacturers and payers, including state Medicaid programs, to enter into OBAs without triggering the best price provision (note: CMS defines OBAs as value-based purchasing agreements or VBPs). There are ongoing federal efforts to seek additional clarification, either through additional guidance, rulemaking, or legislation, about the CMS VBP rule to support this avenue for implementing OBAs.

MULTIPLE BEST PRICE EXAMPLE

Drug X treats cancer, and the OBA is based on tumor size. If X does not shrink a patient's tumor by 100% in 1 year, manufacturer rebates payer 100%.

- **BEST PRICE #1:** \$1000 for patients with 100% tumor reduction
- **BEST PRICE #2:** \$0 for patients with <100% tumor reduction
- **BEST PRICE #3:** \$800* for non-VBP patients

*The non-VBP price is hypothetically calculated based on the existing formula that takes into account the price concessions available anywhere in the marketplace.

The CMS rule enables manufacturers to report multiple best prices for prescription drugs that have a VBP in place: one or more²⁶ best price(s) for patients when the drug successfully achieved the desired outcome(s) (called "responders"); another best price for cases where the drug did not work as intended ("non-responders"); and another best price for drugs not part of a VBP. These multiple best prices would be made available to states that are willing to adhere to the same VBP arrangement terms as are in place in the commercial sector, which would provide those states with the option to receive the benefits of the arrangements already available in the marketplace. States are not required to go through the SPA process or obtain CMS approval to enter into VBP arrangements with manufacturers under this multiple best price approach.

²⁵ Medicaid Program: Establishing Minimum Standards in Medicaid State Drug Utilization Review and Supporting Value-Based Purchasing for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability Requirements, 85 Fed. Reg. 87000 (December 31, 2020).

²⁶ Hypothetically, one OBA could have a tiered rebate system with multiple best prices depending on a range of effectiveness observed in the patient post-treatment.

It is estimated that the uptake of the new VBP arrangements will save states and the federal government \$228 million over three years.²⁷ But these arrangements deliver value beyond taxpayer savings, including reduced overall patient medical spending, reduction in disease progression and/or improvement of symptoms, or improvement in reported quality of life. Most importantly, for patients living with serious medical conditions, access to new therapies can be lifesaving and life changing.

Regarding AMP, the VBP rule does not directly address how it is calculated when a prescription drug has a VBP in place. In the final rule, CMS specifically discusses AMP with respect to payments made over time²⁸ as they relate to VBPs but not for VBP upfront payments. Since many rare disease gene therapies will be sold with federally-mandated rebates or discounts, accuracy in calculating these payments is paramount. This lack of clarity around AMP and implementation timelines, combined with the uncertainty around how different administrations may interpret these regulations, has resulted in a federal advocacy effort where interested parties are advocating for legislation and regulations that provide additional clarity.

CMMI CGT Demonstration

In October 2022, President Biden issued an Executive Order²⁹ directing CMMI to develop and submit a report describing potential models for lowering drug costs and promoting access to innovative drug therapies for Medicare and Medicaid beneficiaries. HHS then released a report in February 2023 selecting three new payment models for testing through CMMI, one of which is the Cell and Gene Therapy Access Model (CGT Model).³⁰

The Medicaid-focused CGT Model would establish a partnership among CMS, manufacturers, and state Medicaid agencies that tests a new, centralized approach to facilitate OBAs for certain cell and gene therapies where CMS would act as the administrator of the program.

In January 2024, the Biden Administration announced³¹ that sickle cell disease (SCD) would be the first focus of the CGT Model. CMMI will facilitate OBAs for SCD gene therapies by negotiating both the front-end financial and clinical outcomes measures to be tracked and the back-end data analysis, which includes monitoring results, evaluating outcomes, and reconciling results with the manufacturers. States and manufacturers can voluntarily participate in the model. This demonstration is another indication that CMS and the Biden Administration are interested in pursuing policies that promote OBAs, but there is still work to be done regarding the ongoing federal barriers these new payment models face.

²⁷ <https://www.cms.gov/newsroom/press-releases/cms-issues-final-rule-empower-states-manufacturers-and-private-payers-create-new-payment-methods>

²⁸ <https://www.federalregister.gov/d/2020-28567/p-246>

²⁹ <https://www.whitehouse.gov/briefing-room/presidential-actions/2022/10/14/executive-order-on-lowering-prescription-drug-costs-for-americans/>

³⁰ <https://innovation.cms.gov/data-and-reports/2023/eo-rx-drug-cost-response-report>

³¹ <https://www.cms.gov/priorities/innovation/innovation-models/cgt>

STATE MEDICAID OPTIONS FOR ESTABLISHING Rx VBAs

CMS VBP RULE*: States would review available VBP arrangements on the CMS Medicaid Drug Programs (MDP) system and work with individual manufacturers on implementation. This does not require a state plan amendment.

STATE PLAN AMENDMENT: States would submit a SPA to CMS requesting authority to adopt alternative payment methods for prescription drugs. State Medicaid programs would then individually reach out and negotiate with interested manufacturers over specific prescription drugs.

STATE LEGISLATION: Some states require legislation to be passed prior to pursuing a SPA. See Appendix A for examples.

CMMI DEMONSTRATION:** CMMI is testing a pathway for states to work with CMS and manufacturers to establish and implement VBPs for SCD gene therapies.

* <https://www.medicaid.gov/prescription-drugs/downloads/state-rel-189-vbp.pdf>

** <https://www.cms.gov/priorities/innovation/innovation-models/cgt>

State Actions to Leverage OBAs in Medicaid

While covering prescription drugs is optional under Medicaid, all 50 states have opted to provide this benefit.³² As part of the benefit, states must cover all drugs from manufacturers participating in the MDRP. Therefore, states may face increasing financial pressure as they seek to cover new, potentially high-cost, and life-altering therapies. This challenge will only become more acute as the availability of new therapies grows over the next five to ten years. Given the atmosphere of rising prescription drug costs and the pipeline of new transformative therapies, states are seeking new ways to mitigate the budgetary impact.

Within this broader policy context, each state has unique approaches to its Medicaid system. Some states have relied on managed care organizations (MCOs) to make decisions on the medical effectiveness of a particular drug for a particular patient, leading to potentially inconsistent treatment for clinically similar patients. In other cases, the managed care decisions are made inside the state Medicaid department, which adds consistency but increases complexity. This means the system can be particularly complicated for high-risk patients with high-cost medical conditions because their treatment options may vary significantly depending on the structure of the Medicaid program and the managed care criteria used to evaluate treatment options. Layered on top of this are the different ways Medicaid programs purchase prescription drugs and specialty therapies. Such methods add further complexity as each state evaluates access to such therapies.

Since limiting the scope of covered drugs to control costs is prohibited under the federal statutory rebate agreement outlined in the MDRP, and undesirable from a patient access perspective, states are increasingly considering value-based approaches to both manage costs and improve patient health outcomes.

³² <https://www.ncsl.org/research/health/medicaid-pharmaceutical-laws-and-policies.aspx>

TYPES OF STATE R_x VALUE-BASED ARRANGEMENTS

SUBSCRIPTION-BASED MODELS: State pays a flat fee to a drug manufacturer for unlimited access to a specific drug. In turn, the state must agree to let that manufacturer be the sole provider of said drug.

OUTCOMES-BASED AGREEMENT MODELS: Manufacturers provide the state supplemental rebates, if agreed upon clinical outcomes are not met.

WARRANTY AGREEMENTS*: Some manufacturers are developing alternative models like warranty agreements, whereby third parties other than the manufacturer may make payments to states when patients do not meet predetermined outcomes.

* No state warranty agreements are in place as of Q1 2024.

For example, ZOLGENSMA® is a gene therapy that targets the genetic root cause of spinal muscular atrophy (SMA), a devastating disease that results in severe and often deadly muscle weakness that makes breathing, eating, and moving extremely difficult. At early-onset, SMA is the most common genetic cause of infant mortality.³³ ZOLGENSMA is a one-time infusion into a vein targeted to treat children less than two years old with severe SMA by replacing the faulty gene with a new working copy, stopping progression of the disease.³⁴ Most state Medicaid programs cover ZOLGENSMA; however, with a price tag of \$2.1 million³⁵ per dose, states generally require several steps before authorizing use. Therefore, some states are turning to OBAs as a method to ensure the therapy is worth the investment. As noted by then-Massachusetts Medicaid Director Dan Tsai, "We think it makes sense to pay for innovation for a drug that could really work. And we think [Massachusetts], with public dollars, should not pay for something if it doesn't do what it's advertised to do."³⁶

As previously discussed, some state Medicaid agencies have already submitted proposals to the federal government in the form of State Plan Amendments (SPAs) to CMS to adopt alternative payment methods through OBAs. Generally speaking, if the OBA state proposal is approved by CMS, "the state and each manufacturer are able to jointly agree on benchmarks based on health outcomes and the specific populations for which these outcomes-based benchmarks will be measured and evaluated."³⁷

Visit <https://cahc.net/policy-priorities/campaign-for-transformative-therapies/> for an updated map of states with approved SPAs for Medicaid OBAs.

³³ <https://www.zolgensma.com/how-zolgensma-works>

³⁴ Ibid.

³⁵ <https://www.novartis.com/news/media-releases/avexis-announces-innovative-zolgensma-gene-therapy-access-programs-us-payers-and-families>

³⁶ <https://www.wbur.org/news/2020/02/07/massachusetts-masshealth-zolgensma-cost-control>

³⁷ <https://www.cms.gov/newsroom/press-releases/cms-approves-state-proposal-advance-specific-medicaid-value-based-arrangements-drug-makers>

Thus far, for the approved SPAs, two primary payment models have been proposed: (1) subscription-based purchasing, and (2) outcomes-based supplemental rebate agreements. Although states received approval from CMS to begin negotiations of VBPs in their drug purchasing programs, only a few states have publicly announced such contracts with drug manufacturers. Examples of state OBAs are detailed below:

COLORADO: Colorado utilizes supplemental rebates to design and implement outcomes-based agreements.

- **AveXis (Novartis)³⁸:** Contracted with AveXis (Novartis Gene Therapies) for its gene therapy drug ZOLGENSMA® (onasemnogene abeparvovec-xioi), used to treat spinal muscular atrophy (SMA) in children under 2 years old. This is a one-time treatment with a list price of \$2.1 million per treatment. Under this model, the Colorado Department of Health Care will be able to receive back a significant portion of the price if the therapy is not successful in delivering the expected clinical health outcomes for a five-year period following its use.

OKLAHOMA: Oklahoma currently leads as the state with the most existing OBA contracts with drug manufacturers.

- **Melinta³⁹:** Contracted with Melinta Therapeutics for the antibiotic ORBACTIV® (oritavancin), an antibacterial treatment for skin infections. Historically, ORBACTIV is more expensive than other treatments and the state used prior authorization as a cost management tool, slowing access to the drug. However, despite the higher cost, if used as a first-line treatment, ORBACTIV promises lower overall costs by avoiding hospitalizations. Under the terms of the OBA, the state will no longer use prior authorization for ORBACTIV and Melinta is responsible (via higher rebates) if patients incur higher costs due to hospitalizations.
- **Alkermes⁴⁰:** Contracted with Alkermes for ARISTADA® (aripiprazole lauroxil), an injectable treatment for schizophrenia. Oklahoma's OBA for ARISTADA aims to improve patient adherence to the treatment plan by decreasing the monthly prescription cost.

³⁸ Williams, M. (2022) Colorado Medicaid Executes Its First Pharmaceutical Value-Based Contracts. Colorado Department of Health Care Policy & Financing. <https://hcpf.colorado.gov/colorado-medicare-executes-its-first-pharmaceutical-value-based-contracts>

³⁹ Beck, J. (2018). Oklahoma Signs the Nation's First State Medicaid Value-Based Contracts for Rx Drugs. National Academy for State Health Policy. <https://www.nashp.org/oklahoma-signs-first-medicare-value-based-contracts-for-rx-drugs/>

⁴⁰ <https://www.reuters.com/article/us-health-usa-medicare/oklahoma-medicare-tests-new-tactic-to-curb-u-s-drug-costs-idUSKCN1L81L6>

LOUISIANA: Louisiana's arrangements with pharmaceutical manufacturers are volume-based rather than value-based, utilizing a subscription-based model.

- **Asegua (Gilead)⁴¹:** In 2019, contracted with Asegua Therapeutics, a subsidiary of Gilead Sciences Inc., for a hepatitis C treatment, the authorized generic of EPCLUSA® (sofosbuvir/velpatasvir), via a subscription-based model. Under this model, the state and manufacturer agree to an aggregate cap on the costs of this medication regardless of volume. This provides the state with predictable budgetary impacts while ensuring patient access.

WASHINGTON: Similar to the Louisiana model, Washington also employs a subscription-based model.

- **AbbVie⁴²:** Engaged with AbbVie in a public-private partnership that relies on a modified subscription model to help the state control costs related to hepatitis C. Similar to Louisiana, the state receives unlimited access to AbbVie's hepatitis C drug, MAVYRET® (glecaprevir/pibrentasvir), for a capped cost. Unique to Washington, however, the public-private partnership takes the subscription model further by coordinating broader public health efforts around eliminating hepatitis C among Washington's Medicaid program (Washington State Health Care Authority), Washington State Department of Health, AbbVie, and community leaders.⁴³

ARIZONA: Arizona utilizes supplemental rebates to design and implement outcomes-based agreements.

- **AveXis (Novartis)⁴⁴:** Engaged with AveXis, a Novartis company, to implement a value-based contract for ZOLGENSMA, an innovative gene therapy for pediatric patients with spinal muscular atrophy. The model is based on rebates paid back to the state if certain agreed-upon patient outcomes are not met.

⁴¹ Louisiana Department of Health (2019). State's innovative payment agreement with Asegua Therapeutics for hepatitis C medication allows more Louisianans to receive life-saving treatment. <https://ldh.la.gov/index.cfm/newsroom/detail/5357>

⁴² <https://stories.abbvie.com/stories/for-hepatitis-c-seeking-end-road-in-washington-state.htm>

⁴³ <https://www.spokesman.com/stories/2021/mar/05/michael-ninburg-and-sue-birch-a-public-private-par/>

⁴⁴ Arizona Bioindustry Association (2020). Curing the Incurable – This Gene Therapy Delivers. <https://www.azbio.org/curing-the-incurable-this-gene-therapy-delivers>

Lessons Learned from Existing State OBAs

In researching state initiatives around managing prescription drug costs through OBAs, the Campaign for Transformative Therapies (CTT) interviewed a subset of states regarding their OBA contracts, lessons learned, and wish lists for future potential contracts. Below, recurring themes and observations are listed to better inform future contracts between payers (e.g., additional states) and manufacturers.

1. Data collection and reporting are challenging.

The most common avenue for analyzing the results of OBAs is through claims data. Clinical data at the individual patient level are difficult to collect, and there is no common method for collecting these data among states - some states are using third parties for the tracking and reporting of data; some states are allowing the pharmaceutical manufacturers to handle the data collection; and some states are performing these functions on their own. Claims data are the easiest data to analyze because they are widely available, objective, and standardized; however, most states agree that using claims data often fails to accurately reflect patient outcomes and there may be a significant time lag in the claims submission process that limits analysis. The more robust clinical data collected in electronic health records by providers is clearly preferable; however, this requires a practical mechanism to collect and share the data. Interestingly, there was no consensus regarding control over the data: some states prefer internally maintaining such control while others see value in the manufacturers retaining this function. This could be due to operational components of each state's Medicaid program, as well as the varying relationships between Medicaid programs and manufacturers. A third option also emerged, where the manufacturers pay for a third party to collect the data. However, in the event manufacturers are compelled to pay for the collection of data, they must consider compliance with the AKS in addition to privacy laws.

2. States want more meaningful outcomes to be part of OBAs.

Some states want to see measured outcomes that reflect functional outcomes. For example, for ZOLGENSMA, rather than measuring survival (i.e., did the child live past two, three, four years?), some states expressed the desire to know if a child was able to sit, walk, or eat by themselves when they were previously unable to achieve such milestones. Another example is LUXTURNA® (voretigene neparvovec-rzyl), which treats vision loss. Commercial market OBAs for LUXTURNA are often based on light sensitivity tests.⁴⁵ However, states have suggested that knowing/reporting whether the patient is able to navigate better in daily life would be an improved incentive for states to invest time and effort into developing OBAs for this therapy.⁴⁶ On the other hand, manufacturers may be reluctant to enter into arrangements where the reported outcomes were not studied extensively in clinical trials or in subsequent real-world evidence studies post-FDA approval. This could hypothetically be addressed through supplemental contractual language or reports that include mutually agreed upon metrics regarding patient outcomes.

⁴⁵ https://www.harvardpilgrim.org/public/news-detail?nt=HPH_News_C&nid=1471914707173

⁴⁶ 2021 CTT interviews with state Medicaid programs

3. States are interested in pursuing additional OBAs, with caveats.

Generally, states expressed interest in pursuing OBAs with additional manufacturers. At the same time, concerns over the significant time and effort required by state Medicaid programs to invest and build an arm of their department aimed at negotiating with manufacturers over OBAs, as well as the additional work and functional expertise necessary to maintain and track ongoing OBAs were recurring themes. Some states with approved SPAs have not yet executed an OBA because of these issues, yet they maintain a willingness to continue engaging with manufacturers on finalizing future contracts. Other states that may have only one OBA in place and are satisfied with the results thus far are finding it difficult to increase the utilization due to a lack of manufacturers willing to engage, in part because of existing legal and regulatory concerns. Still, other states that do not have OBAs in place are interested in the process but want manufacturers to provide them with unique terms that fit their populations rather than a “one-size-fits-all-states” approach. A minority of states claim that they would be interested in pursuing additional OBAs if manufacturers’ risks were greater.

Despite the hurdles for implementing OBAs, the general trend indicates that more states are interested in pursuing OBAs as one method for controlling high drug costs. Since each state Medicaid program is unique in how they design the drug benefit (e.g., managed care vs. fee-for-service, various models for dealing with high-cost specialty drugs, etc.) and have different budget cycles (e.g., two years vs. one year), each state OBA is unique and must be approached as such. This theme produced another suggestion: creation of a public database maintained by CMS that lists the various OBAs states have in place. The information would need to exclude proprietary details. This could provide states with a menu of potential approaches to utilize depending on their patient populations and programmatic structures. CMMI might consider this approach as it further develops its CGT Model.

Appendix A: Example State Legislation

Some states may find it necessary or preferable to adopt legislation which would permit implementation of OBAs prior to submitting a State Plan Amendment (SPA) to CMS. Such legislation has been adopted in Texas and Wisconsin. Below is model bill language “relating to value-based arrangements in the Medicaid vendor drug program.”

Texas S.B.1780 (86th Legislature); Passed on 5/28/2019⁴⁷

*AN ACT
relating to value-based arrangements in the Medicaid vendor drug program.
BE IT ENACTED BY THE LEGISLATURE OF THE STATE OF TEXAS:*

SECTION 1. Subchapter B, Chapter 531, Government Code, is amended by adding Section 531.0701 to read as follows:

Sec. 531.0701. VALUE-BASED ARRANGEMENTS. (a) In this section, “manufacturer” has the meaning assigned by Section 531.070.

(b) Subject to Section 531.071, the commission may enter into a value-based arrangement for the Medicaid vendor drug program by written agreement with a manufacturer based on outcome data or other metrics to which this state and the manufacturer agree in writing. The value-based arrangement may include a rebate, a discount, a price reduction, a contribution, risk sharing, a reimbursement, payment deferral or installment payments, a guarantee, patient care, shared savings payments, withholds, a bonus, or any other thing of value.

SECTION 2. If before implementing any provision of this Act a state agency determines that a waiver or authorization from a federal agency is necessary for implementation of that provision, the agency affected by the provision shall request the waiver or authorization and may delay implementing that provision until the waiver or authorization is granted.

SECTION 3. The Health and Human Services Commission is required to implement a provision of this Act only if the legislature appropriates money specifically for that purpose. If the legislature does not appropriate money specifically for that purpose, the commission may, but is not required to, implement a provision of this Act using other appropriations available for that purpose.

SECTION 4. This Act takes effect September 1, 2019.

⁴⁷ <https://legiscan.com/TX/text/SB1780/2019>

Wisconsin 2023 Act 184; Passed on 3/33/2024⁴⁸

AN ACT

to create 49.45 (49r) of the statutes; relating to: value-based purchasing arrangements under the Medical Assistance program.

The people of the state of Wisconsin, represented in senate and assembly, do enact as follows:

*SECTION 1. 49.45 (49r) of the statutes is created to read:
49.45 (49r) VALUE-BASED PURCHASING.*

(a) In this subsection, "manufacturer" has the meaning given in s. 450.01 (12).

(b) Subject to par. (e), the department may enter into a value-based purchasing arrangement for the Medical Assistance program by written agreement with a manufacturer based on agreed upon metrics to which the department and the manufacturer agree in writing. A value-based purchasing arrangement under this paragraph may include any of the following:

- 1. Rebates.*
- 2. Discounts.*
- 3. Price reductions.*
- 4. Risk sharing.*
- 5. Reimbursements.*
- 6. Payment deferrals or installment payments.*
- 7. Guarantees.*
- 8. Shared savings payments.*
- 9. Withholds.*
- 10. Bonuses.*
- 11. Any other thing of value.*

(c) Nothing in this subsection may be interpreted to require a manufacturer or the department to enter into an arrangement described under par. (b).

(d) Nothing in this subsection may be construed to alter or modify coverage requirements under the Medical Assistance program.

(e) If the department determines it is unable to implement this subsection without a waiver of federal law, state plan amendment, or other federal approval, the department shall request from the secretary of the federal department of health and human services any waiver of federal law, state plan amendment, or other federal approval necessary to implement this subsection. If the federal department of health and human services does not approve a waiver of federal law, state plan amendment, or other federal approval under this paragraph, the department is not required to implement this subsection.

⁴⁸ <https://docs.legis.wisconsin.gov/2023/related/acts/184.pdf>

About The Campaign for Transformative Therapies

The Campaign for Transformative Therapies (CTT) is an issue-driven campaign of the Council for Affordable Health Coverage (CAHC) that brings together diverse interests - including organizations representing insurers, drug manufacturers, and patient groups. We support policies that encourage outcomes-based arrangements for gene therapies to ensure patient access.



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