

# Value-Based Insurance Design Pharmacy Benefits for Children and Youth With Special Health Care Needs: Principles and Opportunities

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Value-based insurance design (VBID) represents an innovative approach to health insurance coverage. In the context of pharmacy benefits, the goal of VBID is to minimize access barriers to the most effective and appropriate treatments for specific medical conditions. Both private and public insurance programs have explored VBID pharmacy projects primarily for medical conditions affecting adults. To date, evidence for VBID pharmacy programs for children and youth with special health care needs (CYSHCN) appears lacking. There appears to be potential for VBID concepts to be applied to pharmacy coverage benefiting CYSHCN. An overview of VBID pharmacy principles and guiding principles are presented. Opportunities for the creation of pharmacy programs with a value-based orientation and challenges to the redesign of pharmacy benefits are identified. VBID pharmacy coverage principles may be helpful to improve medication use and important clinical outcomes while lowering barriers to medication use for the population of CYSHCN. Pilot projects of VBID pharmacy benefits for children and youth should be explored. However, many questions remain.

abstract

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## OVERVIEW OF PHARMACY VALUE-BASED INSURANCE DESIGN CONCEPTS

Pharmacotherapy has the potential to provide tremendous advances for human health. Insulin, antibiotics (judiciously used), treatments for asthma, and other medications for a host of other previously untreatable ills have reduced mortality and morbidity. At the same time, the history of pharmacotherapy provides many examples of missteps or irrational exuberance. Whether we consider new uses of older medications or the evolution of understanding the safety and effectiveness of medications, treatment approaches evolve over time.

Pharmacotherapy can also be a costly component of total health care spending. An analysis by the US Department of Health and Human Services revealed that prescription medication spending has grown and continues to grow more rapidly than other medical costs.<sup>1</sup> In the 1990s, during the early years of the pharmacy benefit management industry, retail prescription medications accounted for 7% of total spending on personal health care services. By 2013, the percent of total personal health care spending devoted to all prescription medication was 15.5%. Medication costs as a percent of total personal health care spending are expected to increase to nearly 17% by 2018. The rate of the cost growth of prescription medication projected from 2013 to 2018 is 7.3% annually, fully 2 percentage points higher than total health care spending.

As evidence-based medicine concepts have entered the mainstream, greater concern has been focused on relevant long-term health outcomes. This focus pushes the question of medication use beyond narrow questions of specific treatment effects versus placebo. Comparative effectiveness research expands knowledge of which treatments

provide improved outcomes and greatest patient safety. By using data from clinical trials, population studies, meta-analyses, and expert treatment guidelines, the merits of various treatment approaches are more objectively defined for policy-makers, clinicians, and patient advocates.

Where a body of evidence relating to health outcomes exists, value-based insurance design (VBID) principles can be applied. Fundamentally, VBID coverage policies are intended to guide consumers and clinicians toward the “things that work” while steering clear of the “things that don’t.” The goals in VBID include: health improvement, reduction of potential harms, and management of total health care costs.

VBID can be viewed as a utilization management tool for shaping health care consumer and provider behaviors. Barriers and consumer costs for safe, effective, and preferred health interventions are lowered. These changes are intended to increase uptake of the preferred health interventions, which should result in health gains and cost savings. The other side of the VBID coin is that consumer costs for treatments with less proven benefits may be relatively increased. VBID focuses on patient and population health outcomes, rather than costs or pricing.

Applied to pharmacy benefits, VBID can encourage the selection of medications with greater long-term outcomes and/or lower risks. Applying VBID concepts to pharmacy benefits has the potential to affect consumer medication adherence behaviors. Promoting medication adherence to effective treatments through a VBID pharmacy benefit design is possible.

Experimental VBID pharmacy benefit programs have been attempted in both public (Medicare) and private employer sponsored health plans.

**TABLE 1** Value-Based Pharmacy Benefit Tactical Components

Lowered patient/family responsibility for “valuable” medications
Medication therapy management/counseling
Case management/pharmacist engagement
Employee/member wellness programs with or without health-risk assessments
Disease management programs
Provider education/academic detailing
Financial penalties for undesirable behaviors or failure to participate

VBID pharmacy efforts to date have focused on adult diseases, including heart disease and diabetes, but there is recognition that chronic respiratory diseases and other conditions are appropriate targets.

In pilot programs, VBID pharmacy plans have generally had a neutral impact on total costs of care.<sup>2</sup> VBID pharmacy programs commonly reduce copayments and patient costs for specific medications. Consequently, VBID pharmacy plan sponsors incurred a larger proportion of pharmacy costs for the treatment areas targeted. Nonetheless, Geisinger Health Systems recognized a \$144 per member per month reduction in expected total health costs with a comprehensive VBID program that included a “zero-dollar” copayment<sup>3</sup> for certain medications. In a large private insurance plan, the expected rate of total pharmacy costs actually slowed to 3% rather than an expected 11% rise over a single year.<sup>4</sup> Tactics deployed in VBID pharmacy programs are outlined in Table 1.

Cost savings are only 1 consideration of VBID pharmacy programs. VBID pharmacy experiments have attempted to alter not only treatment preferences, but also patient medication adherence. Even the most appropriate and cost-effective medication will fail to yield optimal health outcomes if the medicine remains in the bottle. Improved medication adherence to beneficial treatments is expected to improve

long-term patient outcomes or reduce disease burdens.

Traditionally, an 80% adherence rate was considered a minimum to generate a benefit from use of a medication. This 80% adherence rate expectation has been validated across a range of adult disease states from cardiovascular disease and diabetes to treatment of schizophrenia.<sup>5</sup>

Elimination of copayments on specific medications aimed at preventing cardiovascular disease progression for Pitney Bowes employees resulted in a modest improvement in medication adherence.<sup>6</sup> Experience in a commercial insurance plan in North Carolina showed that elimination or reduction of copayments for select medications was associated with increased medication adherence of 2% to 4%.<sup>7</sup>

Successful approaches to increasing medication adherence are known. The “Asheville Project,” incorporating pharmacist-based counseling on medication and disease state education, led to improved medication use, lower total health care costs, and improvements on measures of cardiovascular disease progression for those who received the intervention.<sup>8</sup> Similar positive results were achieved by this same approach with diabetic patients. Another approach trialed in Philadelphia increased adherence to warfarin through the use of lottery-based financial incentives. Opportunities to win awards equivalent to \$3 or \$5 per day were earned by participants who maintained consistent medication use and monitoring visits. Although participants’ adherence (and laboratory-monitored medication effect) improved during the intervention period, most returned to a pattern of poor adherence when the incentives were discontinued.<sup>9</sup>

Unfortunately, for all VBID pharmacy approaches, there are gaps between ideal and actual use patterns. Likely,

the secret to successfully impacting patient treatment behaviors is not only the combination of coverage policy tactics, but also the commitment to educating patients and clinicians on the new approach.

### **VBID CORE PRINCIPLES**

Considering the potential application of VBID principles to pharmacy benefits for children and youth with special health care needs (CYSHCN), several concepts appear obvious.

First, use of medications supported with evidence of good outcomes should be encouraged. Second, the “value” of a medication depends on the condition being treated. A large proportion of chronic medication use in pediatrics, particularly in the area of behavioral health, is off-label. An anticonvulsant medication used to prevent seizures may have a higher value than when the same medication is used as a preventive medication for migraines or as a mood stabilizer. Third, the “outcomes” of medication treatment need to be relevant and important health benefits.

A thoughtful approach to VBID pharmacy benefits for CYSHCN should apply. Considering the relative lack of long-term data on outcomes for medication treatments for pediatric conditions, the opportunities for successful application of VBID principles to pharmacy benefits for CYSHCN may be limited.

Nevertheless, there are likely opportunities for VBID pharmacy programs that could benefit the CYSHCN population. Considering previous VBID pharmacy efforts suggests that potential pediatric pharmacy programs should involve:

- Common conditions,
- Clinical consensus, and
- Costly consequences.

Common conditions resulting in medication use are significantly

more likely to generate a return on the investment for the design and communication of VBID pharmacy policies. Although costly treatments of rare conditions cannot be ignored, these conditions may be better managed through intensive case management or other mechanisms. Often, plan sponsors have insufficient pediatric expertise to evaluate specific treatment evidence for rare pediatric conditions. More often, there may be insufficient information on long-term outcomes of some of the newest treatments for rare conditions.

Emphasis should be placed where clear clinical consensus on appropriate medication treatment exists. Ideally, the evidence supporting a VBID pharmacy program should be robust and demonstrate improvements in both short- or long-term costs and health outcomes. There are many conditions affecting CYSHCN where evidence and treatment guidelines are sufficiently robust to support specific VBID pharmacy benefits. In addition, through a VBID pharmacy benefit paradigm, increasing adherence to first-line medications may be an achievable goal.

### **VBID PHARMACY BENEFITS FOR CYSHCN**

Medication is the mainstay of treatment of various conditions affecting children. For some conditions, such as insulin-dependent diabetes, pharmacotherapy is essential. In others, appropriate medication use can improve patient outcomes. VBID pharmacy benefit principles may apply to many conditions affecting CYSHCN. Some common medical conditions among children and young people where there is strong clinical consensus, and where failure to adequately treat the condition has potential for costly health or financial consequences, are listed in Table 2.

VBID is focused on creating behavior changes by individual plan members and/or their families as well as the health care team. Increased communication is needed to provide prescribers, pharmacists, and patient families with information on the appropriateness and consistency of medication use.

Pharmacist counseling and/or care coordination should be incorporated for a VBID pharmacy program to succeed. Pharmacists are needed to more effectively support families' knowledge about the medications used and to monitor and encourage appropriate use. Families appear more likely to discuss medication concerns with pharmacists or care coordinators. Although there are wide variations in the retail costs of medications, family cost responsibility at the pharmacy should be tied to appropriate use of the medications rather than acquisition costs.

Implications for prescribers may include insurance plans assessing prescribers based on the cost-effectiveness of their treatment choices or adherence to accepted treatment guidelines. However, with effective alignment of payment policies and support from pharmacists, prescribers may find patient families more engaged in ensuring that preferred treatment options are maintained. Prescribers could also be impacted where compensation is tied to medication, laboratory testing, imaging, and completion of other treatment-monitoring activities relating to the patients they manage.

Patient and patient family engagement in a treatment plan is crucial in managing chronic conditions. Lowering barriers to preferred treatments (which are often in the form of patient family costs) and increasing adherence to prescribed treatments are aims of VBID principles applied to pharmacy benefits.

**TABLE 2** Conditions Among Children and Youth with Potential for Value-Based Pharmacy Benefits

Common Condition, Costly Consequence, Clinical Consensus	Costly Consequence, Clinical Consensus, but Less Common
Asthma	Insulin-dependent diabetes
Anxiety	Cystic fibrosis
Attention-deficit/hyperactivity disorder	Hemophilia
Depression	Inflammatory bowel disease
Seizure disorders	Juvenile idiopathic arthritis
Other mood and behavior disorders	Chronic infectious disease
	Select, specific metabolic conditions

In chronic illnesses, adherence to medication should be considered of paramount importance to maintain control of chronic conditions. Even when the consequences of medication failure are highly visible, adherence or persistence of treatment can be poor. A recent study of children in their first year of taking anticonvulsant medication for seizures revealed that 14.5% were considered to have discontinued treatment at the end of 6 months, and by the end of 1 year, 26.6% were no longer receiving their treatment consistently.<sup>10</sup> This study found that socioeconomic status was helpful in predicting persisting (or nonpersisting) medication use. "Breakthrough" seizures, seizure type, child age and sex, as well as parent marital status did not predict which children would continue treatment. When there is low adherence to an anticonvulsant, there can be frequent breakthrough seizures.

Missed doses or early discontinuation of treatment should be considered a significant risk for recurrence, complications, and added total health costs. Barriers to continuing treatment, such as high out-of-pocket costs, inconvenient dosing, and unanswered concerns about medications may be associated with poor adherence. When poor adherence to a treatment plan is not detected by the prescriber, a recurrence of symptoms will likely result in escalation of care and continued poor adherence. Unfortunately, prescribers are in a poor position to evaluate adherence,

most often relying on patient or parent report of adherence.

Patient/family cost responsibility should be linked not only to the appropriateness of the medication selected for the condition treated, but also to patient adherence to the treatment. VBID pharmacy coverage could be designed so that patients using medications outside of expected maximal dosing may bear greater family financial responsibility. This would serve as a signal to encourage reassessment of the condition and/or treatment.

Among the common conditions affecting the CYSHCN population are those where medication therapy may be helpful, but where medication alone is not the most effective treatment. Many mental or behavioral health conditions in children lack specific pharmacologic treatments known to be safe and effective in isolation. These conditions are recognized with increasing frequency in children and adolescents. Yet, there exist good treatment guidelines and an evidence base that may include the use of medication for anxiety,<sup>11</sup> attention-deficit/hyperactivity disorder,<sup>12</sup> and depression.<sup>13</sup> Any of these conditions may exist in a child with other special health care needs. Alternatively, any of these conditions may be present alone, but of sufficient impact to qualify the child as one with special health care needs.

At least for anxiety and depression, current evidence supports that behavioral health interventions, such as counseling, cognitive

behavioral therapy, and behavioral therapy approaches, are important. Medication effects are less predictable and reliable in children and adolescents, but outcome measures may be improved when medication is combined with therapy. To the extent that counseling interventions result in insurance claims, monitoring for these events could help steer treatment toward a comprehensive approach and away from a “medication only” approach. For behavioral or mental health diagnoses, the linkage between medical and/or behavioral health and pharmacy claims could be designed to alter family cost responsibility for medications (and/or counseling interventions) and to encourage continuation of comprehensive treatment.

### **AN EXPLORATION OF VBID PHARMACY BENEFITS APPLIED TO ASTHMA**

Asthma is common among children and carries the potential for significant health consequences. Fortunately, there is good evidence regarding treatment and prevention, including national treatment guidance that applies to children and youth.<sup>14</sup> Asthma appears to support a VBID pharmacy program.

Although avoidance of triggers, environmental modification, and control of allergic disease are helpful, medication is the mainstay of treatment of asthma. Medication treatment of asthma falls into 2 categories: “rescue” medications and “controller” medications. VBID concepts can be applied to both medication categories to encourage appropriate medication use.

### **ASTHMA RESCUE MEDICATIONS**

Inhaled albuterol is the mainstay “rescue” medication. Albuterol metered-dose inhalers (MDIs) are the most frequently prescribed asthma rescue medicine for children. With

the many advantages of MDIs<sup>15</sup> comes a price. Although the original albuterol MDI was inexpensive, mandatory reformulation to change the propellants had the effect of raising costs of these medications significantly. In 2016, the cost of MDI albuterol brands is in the realm of \$50 to \$60 per month without insurance coverage. In some insurance plans, the copay costs are almost as costly as purchasing the medication without prescription drug coverage. Adding to the challenges for prescribers and consumers, the MDI albuterol formulations are not generically interchangeable,<sup>16</sup> although most prescribers accept that there is no clinically discernible difference among the brands.

Any asthma patient should have ready access to a rescue treatment. Children with asthma or wheezing can be expected to use 1 or 2 rescue inhalers in the course of a year, even if the disease is well controlled. Use of a low number of asthma rescue inhalers in the course of a year suggests reasonable disease control, regardless of how the asthma is categorized (intermittent or persistent; mild, moderate, or severe).

Medications inhaled via MDI can only provide benefits if the medication reaches the lungs. Valved holding chambers are recommended for use with MDIs for children that need inhaled medications. Unfortunately, these Food and Drug Administration-approved devices also carry high costs. In many instances, families find that insurance coverage for these devices is limited.

### **VBID APPLICATION: RESCUE MDIS**

MDI rescue inhalers should carry low to no copayment when a low number of inhalers are used during a 12-month period. This coverage approach can be expected to encourage the availability of this essential medication. Typically,

school-aged children may need a rescue inhaler to be available both at home and at school. Plan sponsors should consider that an inhaler used at school may be an additional cost, but may impact other important measures and avoid other costs.

MDI rescue inhalers should have a gradually increasing copayment relating to a patient’s increasing use. Frequent use of albuterol is a marker for poorly controlled persistent asthma or a reflection of a need for education on appropriate use of albuterol. When the only known respiratory condition is asthma, families should experience increasing cost responsibility as the frequency of albuterol refills increases. Increased family out-of-pocket costs for excessive reliance on a rescue inhaler should signal a need to seek care. Seeking care may lead to education on appropriate times/situations to use albuterol, or possibly a recognition by all that a controller medication or other treatment intervention is needed.

Use of inhaled controller medications should also be considered in calculating patient responsibility for rescue medication. For patients with ongoing consistent use of an inhaled controller, the patient family responsibility for costs of additional “rescue” inhalers should be less than the costs of the same quantity of rescue inhalers for families not using appropriate asthma controlling treatments.

Finally, value-based pharmacy plans should provide complete coverage through the pharmacy benefit for valved holding chamber/spacer devices. These devices are proven to be useful in enhancing the delivery of inhaled medications to the lungs and are essential for younger patients. Even if the coverage is quantity limited, for example, 1 device in 24 months, relieving the cost burden would remove a potential barrier to effective medication use.

## INHALED ASTHMA CONTROLLERS

Inhaled steroid or combination products are considerably more costly than rescue inhalers. Fortunately, only a minority of patients with wheezing or asthma have persistent disease that requires daily treatment. Inhaled steroid controllers are the most effective and appropriate first-line controller medications.<sup>14</sup> Current guidelines emphasize that controller medications must be used consistently to be effective. Although the guidelines recommend that clinicians check with patients to assess medication use when patient reports of compliance are checked against pharmacy records, there are shockingly consistent differences.

When inhaled controllers are used, clinicians have difficulty determining whether an inhaled steroid or a combination controller is needed. Treatment failures of inhaled controllers are frequently observed as patients with known persistent asthma return to the office, urgent care, or emergency department with repeated exacerbations. Pharmacists are aware that adherence to inhaled controller medication is poor, but prescribers do not tend to have ready access to this information. A recent study of children and adolescents in the Texas Medicaid population revealed that average adherence to all types of controller strategies was only 32%.<sup>17</sup> In that study, only 4% of patients achieved an adherence rate of 80% based on the proportion of days covered. Clinicians, particularly in the context of a busy day of seeing patients, rarely have time or systems in place to reliably check a patient's inhaled controller refill history. The result is that patient failure to take an inhaled steroid controller as recommended is frequently misinterpreted as a need to "step-up" therapy, typically involving the prescribing of a more expensive product or regimen.

In the author's experience working with health plans in Arkansas and Oregon, use can be shifted from more expensive combination controllers toward inhaled steroid medications with appropriate and evidence-based coverage policies. In the Arkansas Medicaid Program, "market share" rates of combination controllers fell from 50% before an evidence-based approach to coverage policy to ~20% afterward. Analysis of claims data revealed that this market shift reduced medication costs, but had no negative impact on any measure of asthma exacerbations, including hospitalization or emergency department visits.

The evidence-based changes in policy reduced apparently unneeded combination controller use, and increased the measured adherence to combination controller medications in the patients who met criteria for coverage. However, these changes did little to increase adherence to inhaled steroid controllers. The vast majority of patients treated with inhaled steroids failed to fill them on a monthly basis after 3 months of use. Although this identifies a limit of 1 innovative approach to the pharmacy benefit, it identifies an opportunity.

Studies of VBID pharmacy projects have generally focused on adult populations. A study relating to asthma controllers in the HEB grocery chain self-insured plan included both adults and dependent children.<sup>18</sup> The intervention lowered asthma controller copayments from \$20 to \$30 per prescription to \$5 and provided patients with 3 education mailings. This small VBID experiment yielded lower medical costs for the patients in the intervention group, which were offset by higher medications costs. However, there was an observed 10% increase in medication adherence between those participating in the intervention (54% adherence) and the control cohort (44% adherence).

## VBID APPLICATIONS: ASTHMA CONTROLLERS

Patient family cost responsibility for daily asthma controller medicines should be linked to medication adherence and/or the duration of time since the last fill of the inhaled controller. With greater patient adherence, patient families should pay lower out-of-pocket costs.

Coverage policies for inhaled steroid and combination controllers can incorporate guidelines to encourage appropriate "stepwise" treatment. Determination of coverage for a prescribed controller may incorporate automated, point-of-purchase review of claims for other medications. Other relevant medications include inhaled controller medicines, rescue inhalers, and oral steroids.

Linkages between medical claims and pharmacy claims could be used to encourage appropriate monitoring. Patients with high use of albuterol and frequent exacerbations or care-seeking behaviors, particularly emergency department/urgent care visits, would be good candidates for an intervention. Interventions may include patient education or assessment of the need to start or step-up a controller medication. Patients with inconsistent use of controller medications or low use of albuterol may be candidates to reconsider the severity of their persistent asthma.

## VBID APPLICATIONS IN LOW INCIDENCE/HIGH MEDICATION COST CONDITIONS

CYSHCN are affected by diverse health conditions. Many conditions are amenable to medication treatments, but as the rarity of the disease and specificity (and novelty) of the treatment increases, so too do the medications costs. For some conditions, the costs of medications may be the largest category of total health care costs for a child.

Historically, health plans and pharmacy benefit managers have actively managed the use of costly medications. Often, this management involved creating barriers to access for costly therapies, instituting previous authorization schemes, and/or by assessing high patient family copayments for medications with high prices. Where previously there were 3 tiers of copayments for pharmacy benefits, “specialty” medications are now subject to a fourth or fifth “benefit” tier.

Increased patient cost burdens are justified by pharmacy benefit management and health plans in the name of “controlling costs,” or ensuring patients have “skin in the game.” However, in the case of CYSHCN, these historical tactics may simply be barriers to effective use of the medications. The tactics of increasing copayment or coinsurance demands or creating laborious previous authorization processes for important medications may serve only to increase administrative costs and burdens for care providers and health plans. Given that a large percentage of CYSHCN will qualify for insurance coverage through government-sponsored health plans, the efforts of private insurers to shift cost burdens to consumers may merely shift costs to government-sponsored plans, essentially subsidizing the private insurance market players.

Yet, could there be a role for VBID pharmacy principles to apply to these conditions? Lowering the cost barrier for medications with established clinical outcome benefits could be helpful to ensure adherence. Increases in patient cost responsibility could be used to signal a departure from treatment recommendations with proven benefits. In this situation, higher costs for families deviating from recommendations may help to maintain CYSHCN on the best proven and established treatments for their

condition. Integrating laboratory monitoring and other patient behaviors into the calculus for patient family medication cost responsibility may help with early detection of complications or problems with treatment. Enhancing communication between the pharmacy, prescriber, and the patient family could improve care.

General concepts that may be useful in low incidence conditions include:

- Ongoing reinforcement of education on monitoring and treatment;
- Preferential patient/family costs for medications and medication delivery and monitoring systems that are well-studied in children to treat specific conditions;
- The use of medications or devices that have not been well studied in children could be expected to be more costly, even if it is used consistently;
- Involvement of research-based patient advocacy groups (ie, the Cystic Fibrosis Foundation and others) in the assessment of the current “state of the art” for treatment considerations to help establish coverage policies;
- Flexibility in medication insurance coverage policies, particularly in light of the high costs of medication and equipment and limitations on coverage; and
- The need to recognize differences between treatments of flares and treatments aimed at maintenance of remission/disease control.

#### **CHALLENGES FOR VBID PHARMACY INITIATIVES FOR CYSHCN**

Multiple factors impact the potential application of VBID principles to pharmacy benefits for the CYSHCN population. There is enthusiasm at the federal level for experimentation with VBID pharmacy programs in the Medicare Part D population.

However, even in pilot studies involving common adult diseases, there is not consistent evidence of beneficial health or fiscal outcomes. Although the Medicare population is large and costly, CYSHCN are less visible contributors to health care costs.

VBID pharmacy benefits for children and youth with chronic illness would represent a significant departure from the status quo. Efforts to educate patient families and care teams would be needed. Pharmacists would have the opportunity to play a more significant role in patients’ care teams, coordinating medication therapy management in conjunction with VBID pharmacy benefits. This opportunity creates the challenge of how to compensate pharmacists for their cognitive services.

Challenges for the creation of VBID pharmacy programs for children include:

- A disconnect between cost and clinical value. Products with the greatest evidence supporting effectiveness and safety should be the “preferred” medications. These are not generally the newest and often are not the most expensive medications available for a particular condition. Neither are they always the oldest and least expensive. Unfortunately, the US regulatory environment can create circumstances that result in the withdrawal of an inexpensive alternative to be replaced by essentially the same medication in a new, more costly incarnation, or where generic competition becomes effectively impossible.
- Provider adherence to treatment guidance. Adherence to clinical practice guidelines is frequently noted to be less than ideal. Dissemination of guidelines, slow adoption/resistance to change previous habits, and imperfect implementation are all barriers that may create challenges for a

VBID pharmacy program. On the other hand, a VBID pharmacy program may increase patient and provider awareness that a patient's treatment is or is not consistent with national treatment guidelines. Such awareness and a linkage to specific behavior expectations may help to improve uptake of specific treatment guidelines.

- Provider brand/generic biases. In some conditions, a combination of factors contributes to a persisting bias against generic anticonvulsant medications. Prescriber concerns have led to studies examining variability between brand and generic formulations of anticonvulsants in patients with seizure disorders. A published review of 16 of these studies in 2010 found no evidence that generic substitutions for brand name medications had any effect on the odds of loss of seizure control<sup>19</sup>; nevertheless, there continue to be prescribers who specify that brands are necessary, regardless of the higher costs without proven benefits.
- Marketing influences on prescriber behavior and comparative effectiveness/safety data. The purpose of pharmaceutical marketing is to cause providers to write more prescriptions for the brands a company is promoting. Pharmaceutical marketers have little interest in funding research to determine if 1 treatment has any advantage against the "standard" treatment. The Food and Drug Administration approval process generally requires comparison only with placebo, although in some instances, ethical considerations require some active treatment. Pharmaceutical marketers are not required to include children in studies, but have incentives to do so. Comparative effectiveness studies are generally rare, and if they are sponsored or influenced by pharmaceutical marketers, then

they may be poorly designed or focused on surrogate measures rather than patient outcomes. Generally, but not always, publicly funded comparative effectiveness studies are more free of the limitations of industry sponsored studies.

- Patient/family issues. With greater scrutiny of the use of medications for chronic conditions and with potential complexity in a VBID pharmacy plan, it is predictable that not all patient families will understand or welcome a change in a familiar benefit. Although there is potential for a reduction in out-of-pocket expenses, for patients with coverage through Medicaid or the Children's Health Insurance Plan, this may not be a relevant benefit. In some cases, family choices drive treatment toward over- or underuse of some treatments. These less than ideal patterns of medication use may be resistant to education or other interventions.
- Plan sponsor commitment. So-called "specialty" medications are the most costly pharmaceuticals for plans. Although only a small percentage of patients need these treatments, they represent a substantial portion of total medication spending. Significant focus is directed to these medications due to their cost impact. The CYSHCN population may be the only group of children generating significant pharmacy costs. One component of the VBID pharmacy approach has been reducing or eliminating costs for patients for consistent use of the "high value" medications. To make VBID pharmacy programs work for the CYSHCN population, plan sponsors will likely need to increase plan contributions toward patient medications. This is a reversal of the advice to increase patient cost responsibility previously given by health insurance and pharmacy

benefit consultants. Conceptually, increased medication adherence (and appropriate medication selection) should help to reduce costs of medical complications, but this concept needs validation with more robust real-world experience.

- Changes in the role of the pharmacy. A VBID approach to pharmacy benefits creates opportunities for value-added services and elevates the role of the pharmacist in patient care teams. Pharmacists are likely in the best position to educate patient families on medication issues and to access and understand previous medication use patterns. Consumer and prescriber communication from the pharmacy of choice or the health plan would be important to ensure that the care team is aware of medication use issues. Increased involvement of a pharmacist may be an important addition to the care team of many CYHSN families. However, to the extent that a pharmacist's involvement is tied to the use of a specific pharmacy, there may be complications in implementation and payment for a pharmacist's services with the VBID pharmacy program. Patient families can be fairly mobile and may use multiple pharmacies during the course of a year.
- Operational choices. Successful implementation of VBID concepts applied to pharmacy benefits will depend on the specifics of implementation. There are multiple resources for comparative effectiveness evaluations, although all suffer from limited large-scale studies. Similarly, treatment guidelines for some conditions affecting CYHSN may be based as much on expert opinion as on carefully controlled trials. The tactics selected to shift prescriber and patient behaviors relating to medications are also important for a VBID pharmacy program. Fortunately, there are a number

of studies that have demonstrated effective methods to shift both provider and patient behaviors.

## SUMMARY

VBID principles have potential application to pharmacy benefits for CYHSN and may offer a powerful tool to reduce total costs of care and/or improve patient health. The rationale for value-based pharmacy benefits is particularly strong for certain chronic diseases. There is good evidence that correct selection and use of appropriate medications can improve disease outcome and/or reduce other costs and complications that exist for some conditions. VBID pharmacy benefit programs may be helpful in these conditions, but to explore this potential requires significant reexamination of pharmacy coverage approaches. There is a need to fill in knowledge gaps regarding the outcomes of medication treatments for children and youth. Failure to carefully consider, plan, and execute a VBID pharmacy program risks higher costs for insurance plans without relevant health gains.

The foundation of VBID is an evidence-base that leads to the identification of strategies to achieve the best outcomes for specific conditions. In many cases, treatment guidelines are based as much on expert opinion as on clear, definitive evidence. Gaps in knowledge and/or incorrect expert opinion included in treatment protocols can be barriers to achieving gains within a VBID pharmacy program. There is a clear need for more robust comparative effectiveness research on treatments used in pediatric care.

Evidence for VBID principles applied to pharmacy benefits in adult conditions is mixed. The variability in the results of VBID pharmacy trials may relate to:

- the components and tools used in each program;

- the outcomes being measured (medication adherence, achievement of short-term clinically measurable goals, medium-/long-term treatment outcomes, or total costs of care are all relevant measures);
- the degree of integration/awareness of and participation with the VBID pharmacy program among primary care providers.

Although some research in adult conditions appears promising, there are virtually no studies of VBID pharmacy programs specifically in the pediatric population. Yet, in some regards, a pharmacy program aimed at a pediatric population may have greater opportunities for success. Typically, parents can be significant allies in ensuring that their children receive appropriate treatments, and they may be more engaged in providing care for others than adults are in providing care for themselves.

There are clearly opportunities that fit the “common condition, clinical consensus, and costly consequences” test. However, many questions remain regarding the potential for VBID pharmacy policies to benefit CYSHCN families. Within the context of accountable care organizations, and other health care reforms, carefully conceived VBID pharmacy pilot projects are worthy of study.

## ABBREVIATIONS

CYSHCN: children and youth with special health care needs

MDI: metered-dose inhaler

VBID: value-based insurance design

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