RDAC
Utah Rare Disease Advisory Council

Improving the Lives

OF RARE DISEASE PATIENTS

POLICY GUIDE

Above: Utah siblings Matt and Alexandra Pearl both have Fanconi Anemia, a very rare genetic condition.

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RDAC
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INTRODUCTION

Welcome to the 2025 Utah Rare Disease Advisory Council (RDAC) Policy Guide. The Guide is a compilation of policy statements about issues of critical importance to Utah's rare disease patients, their families and caregivers. Those who live with rare diseases may face reduced quality of life due to the burdens of their diseases as well as a lack of autonomy caused by chronic, progressive, degenerative, and/or life-threatening aspects of many rare diseases. Patients, as well as their families and caregivers may face burdens, including a lack of financial and other resources, along with disability resulting from the diseases and their treatments.

The RDAC was established by the Utah State Legislature with a mission to provide an in-depth understanding about rare disease for government officials and policymakers. The goal is to address barriers to resources, proper treatment and care.

The Guide's policy statements support the mission of the RDAC and provide a roadmap for taking steps that will help Utahns with rare diseases lead full and healthy lives. The Guide is a dynamic document. Policies will be modified or added as appropriate.

ABOUT THE RARE DISEASE COMMUNITY

Rare diseases are rare individually, but as a group they are more common than many people think. Approximately 7,000 rare diseases affect more than 30 million people in the U.S. An estimated one in ten Utahns, or an estimated 350,000 women, men and children are suffering from a rare disease.

Whether it is an immune deficiency, a rare cancer, or a genetic disorder, medical care for a rare disease is chronic and costly. Moreover, although the FDA has approved hundreds of drugs for rare diseases, most conditions do not have FDA-approved treatments. Drug, biologic and device development in rare diseases is challenging for many reasons, including the complex biology, the lack of understanding of many rare disorders and the absence of financial motivation due to small market sizes.

ABOUT THE RDAC

The RDAC was formed under HB 106 during the 2020 legislative session of the Utah Legislature (Utah Code Annotated Section 26-1-41) "to advise the state Legislature and state agencies on improving access to treatment and services provided to individuals with a rare disease; make recommendations to the Legislature and state agencies on improving access to treatment and services provided to individuals with a rare disease; and identify best practices to improve the care and treatment of individuals in the state with a rare disease."

The RDAC includes a representative from the Utah Department of Health and Human Services, researchers and physicians who specialize in rare diseases, rare disease patients and caregivers, and representatives from rare disease organizations.

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Approximately 7,000 rare diseases affect more than 30 million people in the U.S.

BAN ON COPAY ACCUMULATOR PROGRAMS

RDAC POSITION

Copay accumulator programs impose a considerable burden on the community of people with rare diseases, adversely impacting their health and well-being. In addition to increasing out-of- pocket costs, the resulting skipped treatments can cause adverse health outcomes for patients, including rare disease patients. These adverse health outcomes may increase overall costs to other payers, such as state-sponsored health plans, insurers and pharmacy benefit managers. The RDAC recommends banning copay accumulator programs in the State of Utah.

ISSUE

For medical insurance coverage, many families and individuals have deductibles of thousands of dollars and may also have significant copays or coinsurance. Many patients, including patients with rare diseases, receive financial assistance from drug manufacturers and other third parties to help pay for their medications, which frequently include high-cost specialty medications that are used long-term. This financial assistance significantly helps these people meet out-of-pocket costs, including deductibles, copays and coinsurance, so patients can access proven interventions.

Some insurance companies have instituted copay accumulator programs that typically target specialty drugs for which third-party copay assistance is available. The accumulators prevent copay assistance from applying to a patient's out-of-pocket costs for health insurance deductible, copays and coinsurance. Consequently, patients who depend on third-party support must still pay the full copay, coinsurance, and/or deductible, despite a third-party having already paid it on their behalf. The result is unnecessary financial hardship and increased likelihood of treatment non-adherence, which can lead to a more advanced disease state and/or increased disability for patients.

Families and individuals, especially those who have rare diseases with high treatment costs, face decisions about whether to delay or postpone visits and care based on achieving their respective out-of-pocket costs. According to Doug Long, vice president of industry relations for IQVIA, prescription abandonment at pharmacies by patients has been fueled by cost shifting to patients, including copay accumulators. In 2021, 60% of prescriptions with patient monthly out-of-pocket costs of $125 or more were abandoned at pharmacies. Skipped medical visits, treatments and prescription pickups may result in deterioration of the patient's condition and lead to significantly increased costs for hospitalizations, office visits and treatments that otherwise would likely not have been incurred. In addition to increasing costs for additional care, deterioration of the patient's health may interfere with the ability of patients and caregivers to be contributing members of society.

The burden of copay accumulator programs is clear. Applying payments derived from all sources, including out-of-pocket and copay assistance program payments, to insurance deductible, copays and coinsurance would enable families and individuals to meet their financial obligations with minimal interruption of care.

COVID-19: PUBLIC HEALTH GUIDELINES, ACCESS TO VACCINES AND TREATMENTS

RDAC POSITION

There are many rare diseases that put Utahns at high-risk of severe disease from COVID-19. The lack of recognition of some rare diseases as a high-risk category for vaccination and treatment eligibility has had unintended, but serious, consequences. Delays in vaccination have impacted patient safety for many that were unable to get early vaccination. The RDAC asks that in future policy decisions and public messaging for COVID-19 (and for other pandemic preparedness and response), "rare diseases that may cause severe risks" be clearly considered a formal category of high-risk.

ISSUE

Throughout the COVID-19 pandemic, many members of Utah's rare disease communities have faced extreme disruption to their lives and medical care. For most rare diseases, there is no information that could inform risk of severe illness and complications from COVID-19 and patients have to rely on their healthcare provider's recommendations and on common sense to determine their risk. Utah public health messaging and policies regarding vaccination eligibility for those at high-risk has consistently been silent regarding the rare disease population, including those who may be at highest risk for severe disease and death.

The lack of recognition of rare disease as a high-risk category from vaccination and treatment eligibility has had unintended, but serious consequences.

For many, this lack of attention in messaging and eligibility requirements has resulted in confusion, hesitancy and significant delays in getting vaccinated. Because it wasn't clear that rare disease patients were identified as high-risk, this population faced a quandary - risk getting turned away at vaccination sites or delay vaccination and potentially endanger their health.

Subsequently, many of these patients remained unvaccinated and unprotected despite a physician recommendation that they receive a timely vaccine, even as other patients that were at less risk, but had a more common and specific condition were eligible.

The Utah State Department of Health and Human Services made recommendations based on the best available information at hand about how to quickly protect the patient populations at highest risk. However, while it may be not feasible to ask for familiarity of the approximately 7,000 rare diseases in the midst of a public health crisis, such conditions should be considered when decisions to prioritize patient groups are being made.

ACCESS TO GENE THERAPY

RDAC POSITION

Gene therapy holds tremendous potential to improve treatment and potentially cure many rare genetic diseases. The RDAC supports access to and coverage for gene therapy for Utah's rare disease community and our healthcare system at large. Engagement with key stakeholders—including patients, providers, payers, manufacturers, and policymakers—is vital to equipping Utah's healthcare system, and the many children and families it serves, to realize the tremendous potential of gene therapies for rare diseases.

The RDAC further encourages Utah's Medicaid Program to explore innovative payment approaches, such as value-based payment arrangements, that could facilitate availability and utilization of life-changing treatments and, in some cases, cures for those with rare diseases.

ISSUE

Gene therapy is a new generation of medicine where a functioning gene is delivered to a patient to treat and potentially cure a genetic disease. Through gene therapy, the underlying cause of a genetic disease can be targeted and corrected, potentially with a one-time treatment.

1 in 10 Utahns, or an estimated 350,000 women, men and children in the state, are afflicted by a rare disease.

Gene therapy holds tremendous promise for the **estimated 1 in 10 Utahns, or an estimated 350,000 women, men and children in the state, who are afflicted by a rare disease**[1]. If you don't yourself have a rare disease, you likely know a family member or friend with a rare disease—whether it is an immune deficiency, a rare cancer, or a genetic disorder. Rare diseases are often debilitating, life-threatening and lack alternative treatment options.

Unlike traditional medicines or therapies, which require frequent administration and focus on managing symptoms, gene therapy is designed as a potential one-time treatment aimed at targeting the underlying cause of a disease at the cellular level.

Gene therapy could deliver transformational improvement in health and quality of life for Utahns and their families and caregivers, as well as provide substantial value for Utah's healthcare system and communities.

- **Utahns Living with Rare Diseases**: Eliminate or reduce clinical burden of disease, improve quality of life and increase life expectancy.
- **Utah's Families and Caregivers**: Alleviate the emotional and physical burden of care, enabling work and socialization and facilitating family relationships.
- **Utah's Healthcare System**: Reduce the need for chronic treatment, lower occurrence of disease-related complications and decrease healthcare utilization.
- **Utah's Communities**: Contribute to

1 For more information about PBM reforms implemented in other states, see: https://insidesources.com/rein-in-profiteering-by-drug-industry-middlemen/ article by David Balto, April 11, 2022. https://nhjournal.com/pezzillo-the-real-cause-of-the-high-price-of-prescription-drugs-pbms/ article by Rich Pezzillo, March 24, 2022. https://www.pbmaccountability.org/ accessed on September 6, 2022.

longer, healthier and more productive lives, with increased contributions to schools, workplaces, our economy and other aspects of Utah's local communities.

- **Utah's Economy**: Reduce direct and indirect costs of rare disease to Utah's economy, including by decreasing chronic care costs and disability support, while increasing economic contributions from rare disease patients who are freed from symptoms. **It's estimated that the total economic burden of rare disease in the U.S. is over $966 billion**[2].

While Utah may only comprise a portion of that economic burden, **each year an estimated 150 Utahns born are diagnosed with a rare disease**[3], resulting in significant healthcare utilization and economic impact. Gene therapies could benefit Utah's healthcare system in transformative and sustainable ways, offering potential to avoid years—and potentially lifetimes—of burden associated with chronic disease management for severely debilitating and life-threatening diseases.

To illustrate, consider **Duchenne muscular dystrophy** (DMD) and **hemophilia**. In the U.S., the total annual burden of illness per DMD patient is approximately $121,000[4]. Twenty-seven percent of caregivers said they reduced working hours or stopped working completely to care for a relative with DMD[5]. For hemophilia, the overall lifetime cost of treating moderately severe to severe hemophilia ranges from $19 million to $23 million per patient[6]. Underemployment or part-time employment as a result of American's living with hemophilia is estimated to cause almost $4 million in societal losses per year[7].

Gene therapies that treat the underlying cause of DMD, hemophilia and other rare diseases could help Utah avoid these significant costs. Importantly, despite the upfront costs of gene therapies, **projections indicate that they will not threaten health system financial sustainability**. Despite an expected increase in approved therapies, projected 2030 gene therapy spending in the U.S. is only **0.2% of total healthcare spending**[8].

SUPPORTING ACCESS TO GENE THERAPY

There are approximately **7,000 known rare diseases and 95% of them have limited or no treatment options. Fifty percent of people affected by rare diseases are children. With more than 500 gene therapies in clinical development**, all Utahns could benefit from improved access to these potentially life-saving cures and treatments for their children and families.

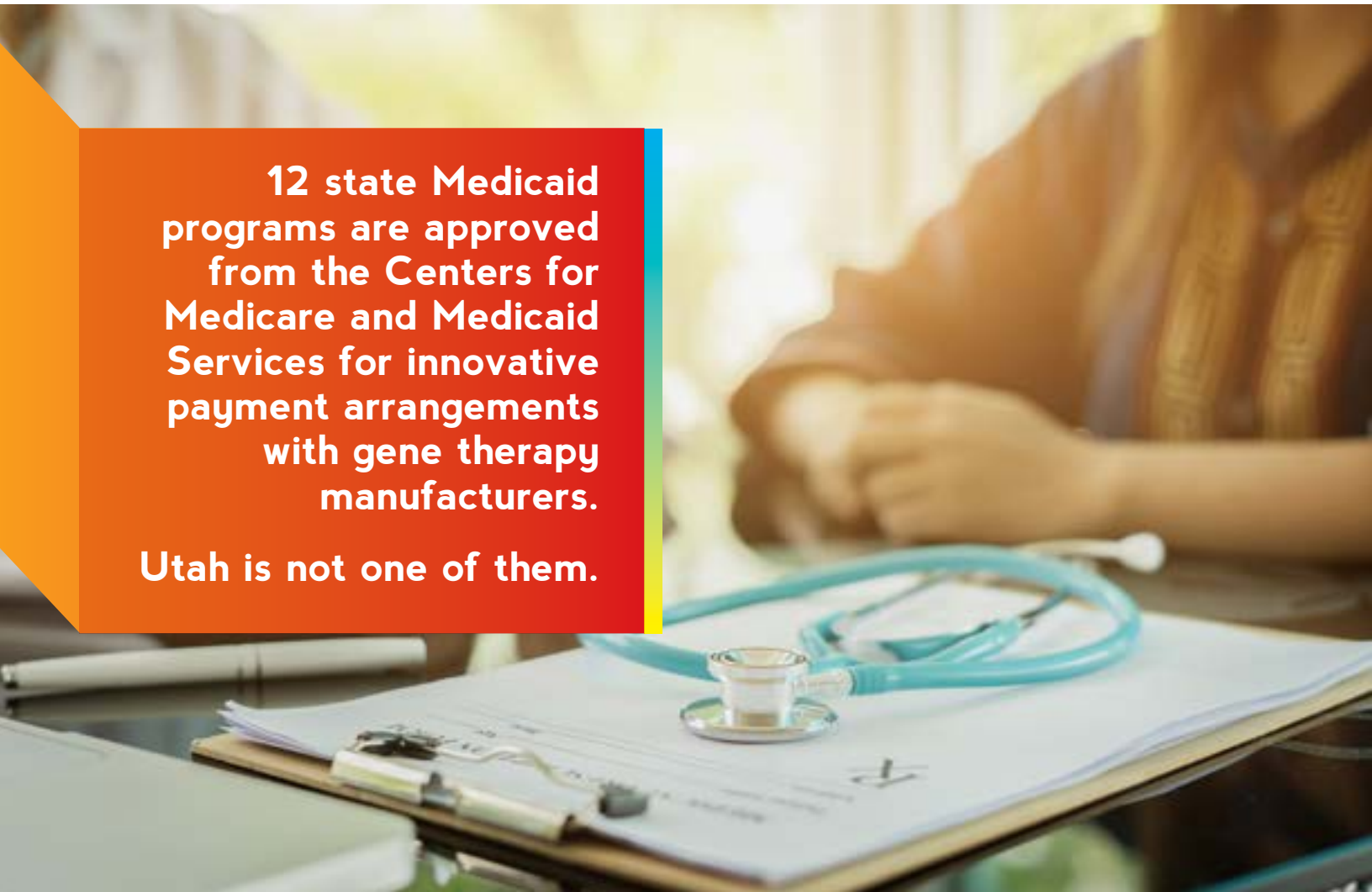
Because Medicaid and Medicare cover a significant share of those with disability status, these payers are expected to be the prominent payers for gene therapies. As more and more innovative gene therapies are discovered for rare diseases, Utah's Medicaid Program, along with Medicare, has

2 EveryLife Foundation. The National Burden of Rare Disease Study. February 25, 2021. https://everylifefoundation.org/burden-study/.
3 EveryLife Foundation for Rare Diseases. Newborn Screening – Utah. https://everylifefoundation.org/states/utah/.
4 Landfeldt, Erik, et al. The burden of Duchenne muscular dystrophy: an international, cross-sectional study. Neurology 2014;83(6):529-536.
5 Ibid.
6 Rind et al. Valoctocogene Roxaparvovec and Emicizumab for Hemophilia A: Effectiveness and Value; Evidence Report. Institute for Clinical and Economic Review, October 16, 2020. https://icer.org/wp-content/uploads/2020/10/ICER_Hemophilia-A_Evidence-Report_102620.pdf. Accessed May 2022. Li et al. Adult lifetime cost of hemophilia B management in the US: payer and societal perspectives from a decision analytic model. J Med Econ. 2021;24(1):363-72.
7 Chen, S. Economic Costs of Hemophilia and the Impact of Prophylactic Treatment on Patient Management. American Journal of Managed Care. Am J Manage Care. 2016;22:S126-S133.
8 Young, M., et all. Durable cell and gene therapy potential patient and financial impact: US projections of product approvals, patients treated, and product revenues. https://doi.org/10.1016/j.drudis.2021.09.001.

an opportunity to develop innovative policy to ensure children with debilitating and life-threatening conditions can access these potentially transformative therapies.

One barrier for State Medicaid Programs, in particular, is the actuarial risk of paying for gene therapy treatments. Compared to traditional chronic therapies, gene therapy treatment comes with large upfront costs, which are counterbalanced by significant, potentially life-long reductions in healthcare utilization and increases in productivity. Anticipating the potential budget impact of such new therapies on Medicaid programs is therefore of utmost importance. Consideration should be given to the need for sufficient annual appropriations and the use of innovative payment approaches, such as annuity or pay for performance contracts with gene therapy manufacturers.

Stakeholders from across the healthcare continuum are exploring alternative, innovative approaches to paying for these upfront costs while realizing the long-term benefits of treatment with gene therapies. Currently, 12 state Medicaid programs have sought and gained approval from the Centers for Medicare and Medicaid Services to enter into innovative payment arrangements with gene therapy manufacturers. Utah is not one of them.



ACCESS TO GENETIC TESTING

RDAC POSITION

Early and accurate diagnosis through genetic testing is crucial to improving outcomes for children and adults with rare diseases. The RDAC supports expanded access, funding and healthcare coverage of genetic testing for all Utah patients that could benefit from it.

ISSUE

Rare diseases represent an underserved and undercounted public health crisis. Most rare diseases have a genetic basis, and genetic testing, such as exome sequencing, whole genome sequencing and disorder-specific genetic panels, play an increasingly important role in the diagnosis and treatment of rare diseases.

Despite the importance of genetic diagnostics, there are still significant barriers for many rare disease patients that limit or prevent access to appropriate testing. The average length of time from the onset of recognizable symptoms to diagnosis of a rare disease in the U.S. is eight years. Misdiagnosis and delays in diagnosis carry high costs, including financial costs in terms of healthcare spending, and the human costs of avoidable disease progression and disability. Early and accurate diagnosis through genetic testing is crucial to improving outcomes for those with rare diseases.

MEDICAID EXPANSION

RDAC POSITION

The RDAC supports reasonable efforts to expand Medicaid coverage in Utah, including expansion through Utah state plan amendments and Medicaid waivers from the Centers for Medicare and Medicaid Services.

ISSUE

One of the greatest concerns for rare disease patients and their caregivers is obtaining health insurance coverage that provides access to the health care system for diagnosis and treatment, and many rare disease patients rely on Medicaid for this access. Rare diseases create health care problems that are not faced by the general public, but patients and their caregivers also must deal with the more routine health care needs faced by the general population. Therefore, any expansion of Medicaid coverage that benefits Medicaid beneficiaries in general also benefits the rare disease community. Any expansion of Medicaid benefits that is specific for rare diseases also is obviously beneficial to the community.

MEDICAL NUTRITION AND NEWBORN SCREENING

RDAC POSITION

The RDAC supports access to medically necessary nutrition for all individuals diagnosed with phenylketonuria (PKU) and other genetic metabolic disorders diagnosed on the basis of newborn screening, as recommended by physicians, the National Institutes of Health, Utah Department of Health & Human Services, the American College of Medical Genetic and Genomics and Genetic Metabolic Dietitians International. In addition, the RDAC supports the requirement that every baby born in Utah hospitals should receive a routine newborn screening test around 24 hours after birth. Every individual diagnosed with PKU, or other Inborn Errors of Metabolism requiring medical nutrition should be provided the appropriate coverage without unnecessary denials and appeals, regardless of insurance status.

ISSUE

Phenylketonuria (PKU) is a rare inherited inborn error of metabolism in which the amino acid phenylalanine is not broken down in the body properly, resulting in harmful build up of phenylalanine in the blood. In the United States, PKU occurs in about 1 in 10,000-15,000 newborns. Untreated PKU results in severe neurological complications and irreversible brain damage, including permanent intellectual disability, seizures, delayed development, behavioral problems, and other psychiatric disorders. Most cases are detected shortly after birth by newborn screening, and treatment is started promptly. Treatment consists of a life-long, carefully controlled, phenylananine-restricted diet beginning during the first days or weeks of life. Newer FDA-approved medications may allow a few people with PKU to follow a diet that has a higher amount or unrestricted amount of protein.[1] [2] [3]

Recognition of the critical importance of newborn screening is imperative. Newborn screening saves lives. Early diagnosis by newborn screening, along with medical treatment of PKU, and other inborn errors of metabolism, is critical for maintaining the health of infants born with these conditions. When an infant is diagnosed with PKU or another inborn error of metabolism, it is essential to begin treatment immediately with a protocol that includes medically necessary food and formula to maintain good health. The need for treatment is lifelong, and with strict adherence to treatment, patients may be able to lead normal lives.

1 https://medlineplus.gov/genetics/condition/phenylketonuria/
2 https://rarediseases.org/rare-diseases/phenylketonuria/?filter=ovr-ds-resources
3 https://www.mayoclinic.org/diseases-conditions/phenylketonuria/symptoms-causes/syc 20376302#:~:text=Phenylketonuria%20(fen%2Dul%2Dkey,needed%20to%20break%20down%20phenylalanine

In the United States, Phenylketonuria PKU occurs in about 1 in 10,000-15,000 newborns.

The standard treatment for PKU is a low protein diet, severely restricting the offending amino acid, phenylalanine (phe). This diet requires collaboration with a medical team to regulate the person's protein intake and determine specific daily intake of medical foods for optimal nutrition and development. This is accomplished by consuming medical foods and formula modified to be low in protein for the provision of required calories.

The PKU diet is costly and unaffordable for most people. Utah has mandated insurance coverage for medical nutrition for the treatment of inborn metabolic errors (Utah Code Annotated Section 31A-22-623), yet 72% of insured Utah residents are on federally regulated plans that pre-empt state legislation on medical nutrition. Many Utah families do not have coverage for low protein formula or foods and thus cannot adequately follow treatment protocol or recommendations due to access issues.

In 2021, the Utah legislature funded the PKU Formula Program for children through an ongoing appropriation to the Department of Health. This restored a former program to cover formula for all Utah children with PKU through age 18. From 2009-2021, the program was restricted to children with PKU under the age of 6 due to budget restraints. However, the need for medical formula and medical foods is life-long and continues through adulthood. All Utahns who require medical nutrition for the treatment of inborn metabolic errors should have access to lifesaving medical nutrition regardless of insurance coverage.

ACCESS TO MEDICAL RECORDS

RDAC POSITION

The RDAC supports improvements to medical records systems to provide easier access to records from different facilities for patients and providers.

ISSUE

Many Utahns receive medical care from multiple providers in different hospital or health care systems. In general, each system has set up its own medical record database. Thus, it is not always straightforward for providers and patients to access patient records, and the formats of the records also vary and make it more difficult to compare information from various dates and facilities.

One frequently encountered situation is that provider notes, lab results, imaging results, and other records are not always easily shared between systems. As a result, tests may be repeated unnecessarily, provider and patient time may be used inefficiently, appropriate care may be delayed or skipped entirely, and inappropriate care may be delivered.

Rare disease patients tend to have complex medical histories and often see many health care providers, making the lack of consistent access to their records even more of a problem than for other patients and providers.

Facilitating information sharing between hospital and health care systems would increase efficiency in providing care and simplify the burdens that both providers and patients face in managing care. It would be preferable to have a single user interface, data presentation format, and login arrangement that providers and patients can use to access records ordered by different providers and obtained at different facilities. Of course, protections need to be included to guarantee patient privacy and compliance with HIPAA requirements.

PHARMACY BENEFIT MANAGERS

RDAC POSITION

Utah should implement Pharmacy Benefit Manager (PBM) reforms to decrease drug costs for patients, ensure access to local pharmacies, and improve pharmacy reimbursement practices. A multi-pronged approach is necessary to address:

- Higher drug costs driven by PBM rebate policies that don't pass discounts on to patients.
- PBM lack of transparency.
- PBM discrimination against non-network and independent pharmacies.
- Cost pressures that burden state-run insurance programs as a result of the PBM business model.

Individuals in the rare disease community tend to use multiple medications and relatively expensive medications to manage their health conditions. Thus, the abuses of PBMs weigh especially heavily on this population.

ISSUE

PBMs are middleman companies that interface with pharmaceutical manufacturers, health insurers, and pharmacies. Often, they are part of large conglomerates that own or are owned by insurers and/or pharmacy chains with vertically and horizontally integrated businesses. PBMs develop drug formularies with and for insurers. This process is informed by factors such as negotiated reimbursement rates with pharmacies and negotiated rebates with drug manufacturers. The process includes discounts and other fees paid by manufacturers as well as drug placement in formularies.

Rare disease individuals often rely on multiple and more expensive medications to manage their health conditions.

The largest PBMs are interconnected with other behind-the-scenes affiliates, such as pharmacy payment processors, group purchase organizations (rebate aggregators), specialty drug labelers (especially for biosimilars), and healthcare providers. These affiliates may be headquartered outside the U.S., removing transparency in their operations. Acquisitions and mergers during the last few years have resulted in three large PBMs controlling about 80% of the prescriptions filled in the U.S. Another three control an additional 10%.

The Federal Trade Commission issued a report in July, 2024, describing the detrimental effects of PBM structures and operations. The agency has since sued CVS Health's Caremark, Cigna's Express Scripts, and UnitedHealth's Optum Rx for anticompetitive supply chain practices, resulting in artificially inflated insulin prices for some patients. The U.S. House Oversight Committee also issued a report in July, 2024, about PBM practices increasing drug costs for payers and consumers.

DRUG FORMULARY DESIGN

Formularies may discourage or encourage the use of generic and biosimilar medications through placement in pricing tiers or excluding them from a formulary altogether. Drug formularies may be designed on the basis of contracts with drug manufacturers for rebates and often implement utilization management provisions such as prior authorizations, step therapy, and quantity limits. Utilization management may function in keeping drug costs lower, but it is being misused to create obstacles for timely patient access to medically necessary drugs.

SPECIALTY DRUGS

There is no uniform definition of "specialty drugs," which are usually handled by specialty

pharmacies. The designation may be based on price alone or on a need for special handling and administration. Specialty drugs account for 40-50% of pharmacy dispensing revenue, but about 2% of total Rx volume. PBMs sometimes classify drugs as "specialty" to limit patient access at pharmacies not owned by or associated with the PBM.

PATIENT COST SHARING

PBM formularies and/or the associated health insurance plans are set up to share drug costs with patients. The cost used to calculate the patient's share is generally the manufacturer's list price, which may be significantly higher than the price negotiated with the drug manufacturer (see examples in Appendix A of the House Oversight Report), and patients typically don't receive the benefit of the manufacturer's price concessions to PBMs. In addition, third-party patient assistance programs for some expensive medications are intended to lessen the cost burden for patients, but PBMs and insurers are increasingly using practices such as copay accumulators and copay maximizers to pocket the financial assistance payments without applying them toward patients' out of pocket expenses.

PBM RELATIONSHIPS WITH PHARMACIES

PBMs have contractual relationships with pharmacies in their networks and may have common ownership with those pharmacies. Pharmacies may be retail, where patients pick up their prescriptions; mail order, where prescription medications are shipped to the patient's designated address; or specialty, where drugs designated as specialty may be delivered to the patient or to a clinic or care provider.

Often, reimbursement rates for payments to pharmacies for dispensing drugs discriminate against out-of-network and independent pharmacies. Inflated reimbursement rates may be paid to affiliated pharmacies. The reimbursement rates may change frequently and be difficult for pharmacists to understand. Post-sale adjustments, or clawbacks, have been assessed to independent and out-of-network pharmacies, based on designated pharmacy performance criteria that are outside of the pharmacy's control, such as patient compliance evidenced by repeat prescription refills. Reimbursement rates may be independent of reasonable costs for pharmacies to acquire the drugs. PBMs may set maximum allowable costs (MACs) for pharmacy reimbursement, which may be less than what the pharmacy pays. These practices may have serious adverse effects on the ability of non-network and independent pharmacies to operate profitably and may decrease market stability. Further, pharmacies have little bargaining power in setting contractual terms.

PBMs may engage in practices that steer patient specialty drug purchases to specialty pharmacies controlled by the PBM. Pharmacy purchases of generic and biosimilar drugs may be reimbursed only if they are obtained through PBM-affiliated labelers, with exclusivity requirements that prohibit pharmacies from obtaining specialty drugs from other sources. For example, a PBM may implement "white bagging" and designate a required pharmacy for purchase of a drug, or it may implement "brown bagging" and require a patient to purchase a drug at a designated pharmacy and bring it to their health care provider for administration.

Brown bagging is prohibited in Utah under SB 193, enacted in 2023.

Ways PBMs are Being Bypassed Currently
A limited number of pharmacies are offering mail order availability for some generic medications, which provides lower-cost access without insurance. These pharmacies may be in network for a few insurance plans. Mark Cuban's Cost Plus offers selected medications at prices that include the pharmacy's acquisition cost for the medication, a 15% fee to Cost Plus, a pharmacy fee for filling the Rx, and the shipping cost. CivicaRx is a nonprofit company that works to reduce hospital drug shortages and make lower-cost generic and biosimilar outpatient medications available,

including by manufacturing medications.

Companies such as GoodRx and SingleCare offer coupons or discount cards to consumers for many medications at their participating pharmacies. RX Partner offers prescription discounts to PBMs and health insurance plans. RX Savings Solutions provides services to pharmacists and health plans to offer drugs at lower prices to consumers. These two programs appear to work outside of PBM/insurer formularies.

AbbVie, the manufacturer of Synthroid, name brand levothyroxine, has a program that provides medication by mail and outside of insurance coverage, for $25/month, considerably less expensive than what many insurers pass on to patients as copays or coinsurance, if their formularies even include Synthroid.

There are several problems with bypassing insurance in these ways for rare disease patients and others who take multiple medications and receive them from multiple pharmacies. These options are only available for the limited selection of drugs available from the entity that is offering alternative pricing. Although using these pharmacies can reduce costs significantly for patients, patient payments for drugs acquired outside of insurance coverage do not count toward the insurance out of pocket costs.

Dealing with multiple pharmacies can be complicated for patients and their caregivers. In addition, mail order purchase from pharmacies may not be appropriate when the medication is needed immediately, such as antibiotics. One often-provided pharmacy service when new prescriptions are filled is to check whether concurrent use of the new prescription is contraindicated with other medications the patient takes, either because one medication interferes with the efficacy of another, or because serious adverse events can occur with concurrent use. A pharmacy that provides only one medication to a patient may not have access to a list of other medications the patient uses, and a pharmacy that provides the patient's other medications likely doesn't have a record of the medication from the other supplier.

PBM REFORMS UTAH CAN IMPLEMENT

Utah has already implemented some measures to improve accountability and transparency of PBMs. The state can implement additional legislation and take other appropriate steps to address PBM-related issues listed below.

TRANSPARENCY OF PBM OPERATIONS

- Reporting requirements for total drug spend, manufacturer rebates, and other discounts received.
- Total pharmacy compensation (see SB 208, 2018).
- Point-of-sale pharmacy price concessions.
- Patient cost-sharing.
- Establishing maximum allowable cost list requirements.
- PBMs to report conflicts of interest.
- Manufacturer reporting.

PBM INCOME

- Prohibitions against spread pricing, where insurers are charged more than is reimbursed to pharmacies.
- Requirements for PBMs to pass rebates to the health plan.
- Requirements for PBMs to apply rebates at the point of sale or use rebates to lower insurance premiums to reduce enrollee costs.
- Increasing Medicaid control over pharmacy reimbursement rates.
- Prohibiting a PBM from reimbursing a pharmacy less than the national average drug acquisition cost (NADAC), a metric used by Medicare.
- Prohibiting a PBM from reimbursing an unaffiliated pharmacy less than an affiliated pharmacy.
- Setting standards for pharmacy audits and recoupments.

DRUG COST SHARING

- Copay caps, already implemented for insulin and considered for albuterol inhalers and epinephrine injectors.
- Requiring third party payments to be applied toward out-of-pocket costs (co-pay accumulator and copay maximizer bans).
- Controlling State Costs (Medicaid and other State-Run Programs)
- Utilization management, which can include lifestyle changes.
- Limitations on step therapy.

EXAMPLES OF RECENTLY PASSED LEGISLATION IN OTHER STATES

Idaho HB 96 (2024)
Strong focus on transparency and reporting, pass through pricing to consumers, prevents steering, pharmacy adequacy standards, defines specialty drug.

Massachusetts Senate No. 2520
Passed both Houses and in conference committee (7/25/24). The bill includes PBM licensing with the Department of Insurance, periodic auditing of PBMs by health insurers, bans spread pricing, bans point of sale and retroactive fees with assessment to PBM for violation, requires at least 80% of rebates to be passed on to consumers at point of sale, requires pharmacies to charge customers the lesser of applicable cost-sharing or pharmacy retail price, requires copay assistance to be included in enrollee cost sharing, imposes duty of good faith and fair dealing on PBMs when dealing with all parties they interact with in performing PBM services, requires PBMs to provide an adequate and accessible network for prescription drugs, requires PBMs to maintain a Maximum Allowable Cost list for generic prescription drugs and must reimburse an independent pharmacy for drugs at the same amount that the PBM reimburses PBM affiliates for providing the same pharmaceutical services, makes permanent the ability of consumers to use drug manufacturer coupons to pay for prescription drugs, procedure for notice and hearing if planned closure of a pharmacy will create pharmacy desert.

Pennsylvania PA HB 1993 (2024)
Amounts of rebates and payments received from drug manufacturers and how those payments were distributed by the PBM.

ABOUT THE RARE DISEASE COMMUNITY

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Whether it is an immune deficiency, a rare cancer or a genetic disorder, medical care for a rare disease is chronic and costly. Moreover, although the FDA has approved hundreds of drugs for rare diseases, most conditions do not have FDA-approved treatments. Drug, biologic and device development in rare diseases is challenging for many reasons, including the complex biology, the lack of understanding of many rare disorders and the absence of financial motivation due to small market sizes.

ABOUT THE RDAC

The RDAC was formed under Utah Code Annotated Section 26-1-41, enacted during the 2020 Utah Legislative session to advise the state Legislature and state agencies on improving access to treatment and services provided to individuals with a rare disease; make recommendations to the Legislature and state agencies on improving access to treatment and services provided to individuals with a rare disease; and identify best practices to improve the care and treatment of individuals in the state with a rare disease.

The RDAC includes a representative from the Utah Department of Health and Human Services, researchers and physicians who specialize in rare diseases; rare disease patients and caregivers; and representatives from rare disease organizations.

For additional information, please contact Lorenzo Botto, M.D., RDAC Chair, at lorenzo.Botto@hsc.utah.edu.

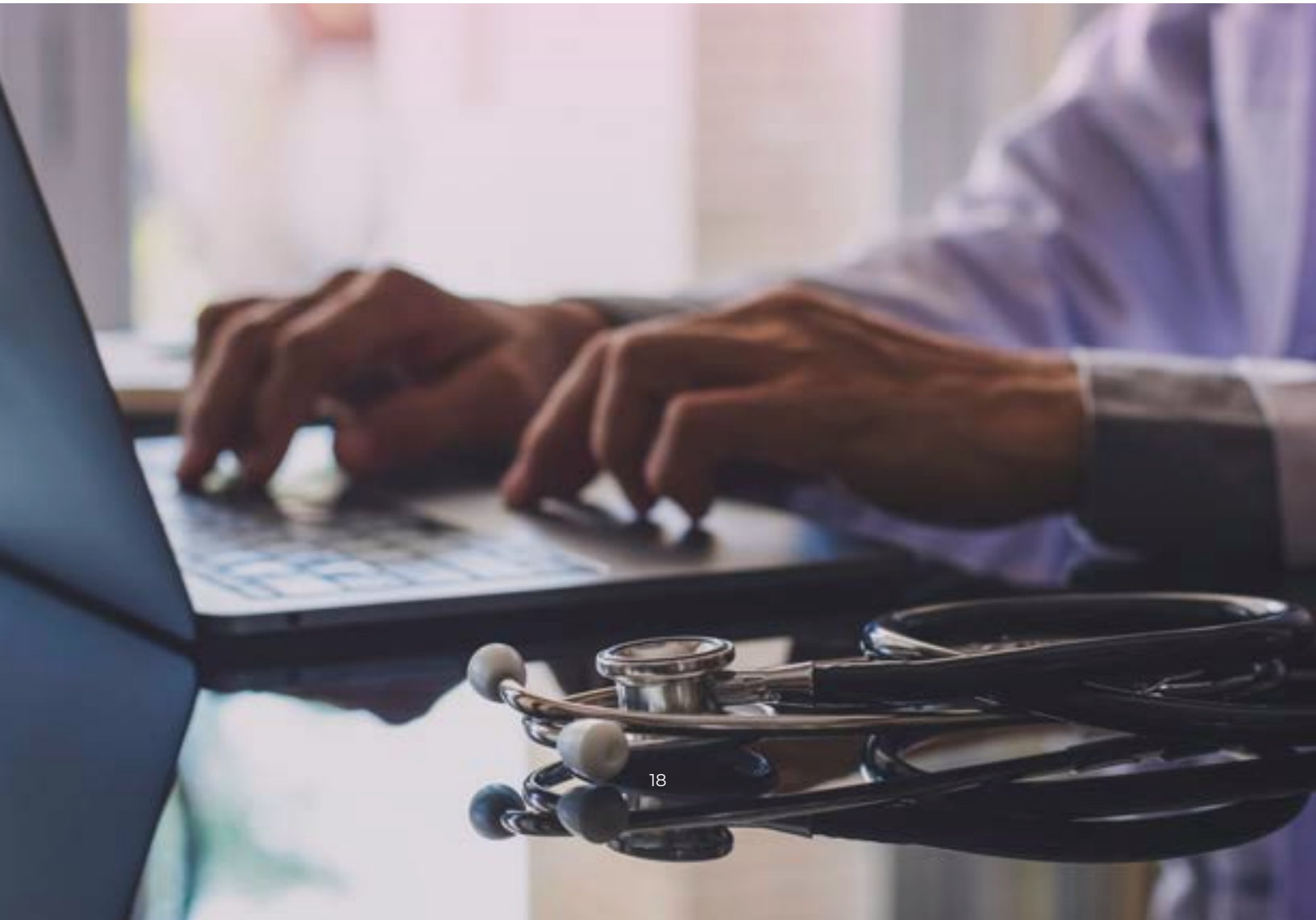
ACCESS TO TELEHEALTH SERVICES

RDAC POSITION

The RDAC supports the continuation of accessible telehealth services in Utah.

ISSUE

Telehealth services, provided in accordance with accepted standards of care and in appropriate situations, should continue to be available to all Utahns. Many rare disease patients have limited mobility, making it difficult for them to travel to medical appointments. Many rare disease patients are immunocompromised, which means they may face additional risks at medical facilities due to exposure to infectious diseases that they can't fight effectively. Thus, it is particularly important for rare disease patients to have continued access to telehealth services as appropriate.



VOTER BALLOT ACCESS

RDAC POSITION

The RDAC supports changes to the Election Code that would facilitate voting by people with disabilities, including changes that provide for alternative means of identity verification for people who cannot sign their names consistently due to a disability.

ISSUE

In Utah, most elections are conducted primarily by mail, with options available for in-person ballot submission and voting. The current system requires each voter to sign an affidavit that is included with the mail-in ballot, whether the ballot is returned by mail or dropped in a collection box. Poll workers compare the signature on the ballot to a previously obtained signature to verify the identity of the voter prior to counting each ballot. However, some disabled voters are unable to provide signatures or unable to provide reproducible signatures that a poll worker will find acceptable. Some rare disease patients are among these disabled voters. Disabilities that preclude use of signatures for ballot verification should not prevent the disabled voters from casting ballots.

It is also important to provide easy access to information about how people who are unable to provide consistent signatures can vote.

Perseverance

UTAH'S RARE DISEASE PATIENTS, INCLUDING THESE TEENS, DESERVE A CHANCE TO LIVE HEALTHY AND PRODUCTIVE LIVES.

RDAC
Utah Rare Disease Advisory Council

Utahns with rare diseases who participated in the Utah Rare and Undiagnosed Network teen campaign in 2016.