ELLEN SCHUTT
STATE REPRESENTATIVE • 31ST ASSEMBLY DISTRICT

Testimony in Support of Assembly Bill 687

Assembly Committee on Health, Aging and Long-Term Care

January 10, 2024

Thank you Chairman Moses and committee members for hearing Assembly Bill 687 today. This legislation would allow the Department of Health Services (DHS) to enter into a value-based purchasing agreement with a drug manufacturer for purposes of the Medical Assistance program.

As advancements in medical treatments progress, we are presented with the chance to address the healthcare needs of Wisconsinites more effectively, particularly concerning rare diseases. A major challenge in developing new treatments for these conditions lies in the limited number of individuals who would benefit from them. These smaller populations lead to increased expenses and place a financial strain on the taxpayer-supported Medical Assistance program.

This legislation would allow DHS to enter into agreements with manufacturers to ensure the cost of treatment is based on the value it provides to the patient. This is determined based on specific metrics outlined in the value-based purchasing agreement between DHS and the drug manufacturer. These agreements will make sure that patients receive the expected results from expensive treatments. Although the bill grants DHS the option to enter into a value-based purchasing agreement, it does not mandate it.

Thank you for your consideration of Assembly Bill 687. I am happy to answer any questions you may have.

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Rep.Schutt@legis.wi.gov

MARY FELZKOWSKI
STATE SENATOR • 12TH SENATE DISTRICT

Testimony on AB 687
January 10, 2024
Assembly Committee on Health, Aging and Long-Term Care

Chair Moses and Members of the Assembly Committee on Health, Aging and Long-Term Care:

Thank you for this opportunity to provide testimony on Assembly Bill 687, which would allow the Wisconsin Department of Health Services (DHS) the opportunity to enter into value-based purchasing agreements with drug manufacturers for purposes relating to the Medicaid program.

As our medical treatments continue to advance, it's opened up the door to a wide array of medical solutions – especially when dealing with rare diseases. Unfortunately, due to such small rare disease patient populations, the cost for these treatments are extremely high.

This bill would allow DHS to enter into value-based purchasing agreements with drug manufacturers, ensuring that the cost of the treatment is based on the value it provides to the patient. This is done through agreed upon metrics between DHS and the manufacturer, stating what bench marks need to be met in order to receive full payment.

These value-based purchasing agreements will add a reasonable and responsible assurance that patients will see the results expected from high-cost treatments.

It is extremely important to note that this bill allows DHS to enter into a value-based purchasing agreement, but does not require them to. Likewise, manufacturers have the option to enter into a value-based purchasing agreement, but are not required to.

Thank you again for this opportunity to provide testimony on AB 687.

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State of Wisconsin
Department of Health Services
Tony Evers, Governor
Kirsten L. Johnson, Secretary

TO: Members of the Assembly Committee on Health, Aging, and Long-Term Care

FROM: HJ Waukau, Legislative Director

DATE: January 10, 2024

RE: AB 687 relating to: value-based purchasing arrangements under the Medical Assistance program

The Wisconsin Department of Health Services (DHS) would like to submit written testimony for information only for Assembly Bill 687 (AB 687), relating to value-based purchasing arrangements (VBPA) under the Medical Assistance program. AB 687 would allow, but not require, DHS to enter into a VBPA with a drug manufacturer for the purposes of the Medical Assistance program. It would also authorize DHS to submit a state plan amendment (SPA), waiver, or any other federal approval necessary to the Centers for Medicare & Medicaid Services (CMS) to support implementation of a VBPA.

Wis. Stat. § 49.45 (49m) was created to authorize the creation of Medicaid's Preferred Drug List (PDL) and provides authority for prescription drug cost controls, which currently includes supplemental rebate agreements for prescription drugs. Current statute could be interpreted to already allow authority for additional contracting efforts through VBPAs. However, legislation specific to outcomes-based VBPAs would provide explicit authority to begin the first steps in evaluating specific needs for this this type of new contracting. Additionally, successful implementation of a VBPA may reduce program costs for Medicaid. Further, CMS has approved SPAs for VBPAs for other states.

It is worth noting VBPAs are more difficult to set up and administer than traditional supplemental rebate agreements. DHS also does not currently have existing resources to establish and maintain a VBPA program. States that have implemented VBPAs have established new teams to stand up and maintain VBPAs, negotiate contract terms including patient outcome measures, collect and monitor patient outcomes data, and enforce contractual provisions related to agreed upon patient outcomes. Were DHS to pursue a VBPA as allowed under AB 687 it would have to evaluate its resource capacities.

DHS is happy to offer itself as a resource for the Committee and address any questions it may have.

Dr. Sheldon Garrison, Ph.D.
102 N Water St, Unit 612
Milwaukee, WI 53202
608-358-3372

Re: Testimony in Support of Assembly Bill 687 - Authorization of Value-Based Arrangements for Medicaid Programs

I am a Neuroscientist at Rogers Behavioral Health with more than 15 years of experience working in rare disease within health care, pharmaceutical and academic institutes. I am well published in the areas of rare disease, genetic testing, orphan drugs/gene therapies, patient-centered medicine, and the health care economics in this space. I have also raised nearly $30 million in NIH, foundation and venture investment to support these efforts, and have a strong understanding of this space. On a personal level, I have lost to immediate family members to a rare blood cancer and rare metastasis of melanoma, both of which are future candidate conditions for the types of potentially lifesaving or extending therapies addressed in this bill.

I am here today to express my endorsement for Assembly Bill 687. The introduction of Value-Based Arrangements, as proposed by this non-partisan bill, marks a positive step toward a patient-centered and cost-effective healthcare system. The intersection of patient-centered medicine and cost-effective will require multiple approaches, and value-Based Arrangement is an important first step.

PATIENT PROBLEM

Innovative therapies, including gene therapies and orphan drugs are There are about 10,000 unique rare, genetic diseases that affect an estimated **10% of people** in the United States (U.S.). Individual rare diseases are legally defined in the U.S. as conditions that have a prevalence of <200,000 people. Collectively, these conditions account for an estimated $800 billion annually in direct patient care in the U.S., and $550 billion in indirect/non-medical costs (e.g., loss of work, family caregiver time).

Wisconsin Patient Impact: Rare disease has a massive footprint in Wisconsin with 60,000 babies are born annually, 10% of whom will add to the estimated 600,000 people living with a rare disease.

Diagnostic delay: A key clinical challenge for those affected by rare disease is the delay to diagnosis (5-8 years), resulting in poorer patient outcomes and substantial economic burden. The goal is to rapidly detect rare genetic conditions, saving clinicians and patients precious time, and increase the quality of life for those suffering from rare disease. Moreover, access to advanced therapeutics and diagnostics once a diagnosis is made is critical to reduce disease severity, improve quality of life, reduce caregiver burden and reduce economic burden. A recent study by the EveryLife Foundation demonstrated this concept by providing key evidence that financial burden is ultimately reduced when patients get optimal treatments earlier in disease course.

WHAT IS THE COST OF RARE DISEASE IN U.S.?

- Rare disease patient hospitalizations: $768B; All other patients: $880B
- Prolonged inpatient hospitalizations
- Likelihood of dying for rare disease: 4x inpatient, 12x emergency department
- 70% of rare disease adults use public payer (Medicare, Medicaid)
- 95% of births are complicated when newborn has a rare disease
- Everylife Foundation estimated $1 trillion annually for both direct and indirect costs

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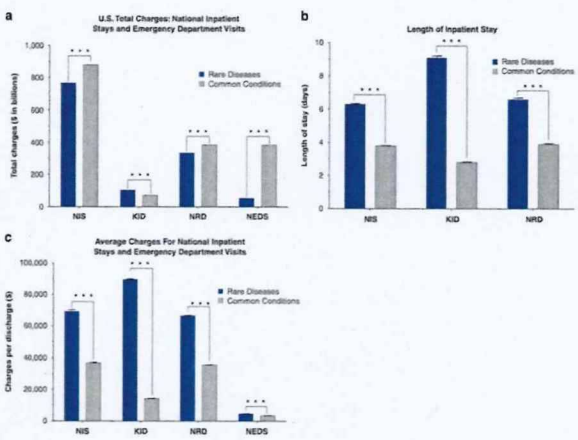
BENEFITS OF VALUE-BASED ARRANGEMENTS



- Gene and cell therapies are continuing to receive FDA approval and it is important to allow access to these transformative therapies to all patients.
- 30-45% of FDA-approved medications are orphan drugs, which tend to be costly.
- Aligning reimbursement incentives with patient outcomes utilizing value-based agreements, helps mitigate financial risk for Medicaid and ensures that individuals with rare diseases have access to the most advanced and effective treatments available.
- The authorization of Value-Based Arrangements reflects a commitment of collaboration among healthcare stakeholders, and ultimately, ensures that Medicaid beneficiaries receive the highest standard of care.
- The goal is to create a more sustainable, patient-centric healthcare system that not only improves health outcomes but also ensures responsible allocation of resources

SOURCES

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2. Everylife Foundation for Rare Diseases. The National Economic Burden of Rare Diseases Study. https://everylifefoundation.org/burden-study/.
3. Tisdale A, Cutillo CM, Nathan R, Russo P, Laraway B, Haendel M, Nowak D, Hasche C, Chan C, Griese E, Dawkins H, Shukla O, Pearce DA, Rutter JL, Pariser AR. The IDeaS initiative: pilot study to assess the impact of rare diseases on patients and healthcare systems. Orphanet Rare Dis 2021;16:429. https://doi.org/10.1186/s13023-021-02061-3
4. US Government Accountability Office (GAO). Rare Diseases: Although limited, available evidence suggests medical and other costs can be substantial. October 18, 2021. https://www.gao.gov/products/gao-22-104235.
5. Parker E, Myers E, Ume N, et al. *The Cost of Delayed Diagnosis in Rare Disease: A Health Economic Study.* EveryLife Foundation for Rare Diseases: The Lewin Group, Inc.;2023. https://everylifefoundation.org/delayed-diagnosis-study/

Katie K. Moureau
319 Southing Grange
Cottage Grove, WI 53527

Re: Testimony in Support of Assembly Bill 687 - Authorization of Value-Based Arrangements for Medicaid Programs

I am here today to express my support for Assembly Bill 687, which proposes the authorization of value-based arrangements. As a parent of a child living with a rare genetic disorder, this legislation is an extremely important step forward in ensuring access to transformative therapies, like gene therapy.

I am a mom to 5 boys. My 8-year-old, Cade, was born with a rare disorder Prader Willi Syndrome (PWS). PWS affects approximately 1 in 20,000 people. In Wisconsin there are approximately 80 individuals living with PWS. While there are many characteristics of PWS, one of the hallmark ones is hyperphagia. Hyperphagia is an uncontrolled desire to eat. Those living with hyperphagia constantly feel hungry, even if they just finished a large meal, their brain is making them feel as if they are starving. If left unsupervised, a person with hyperphagia could literally eat themselves to death.

In addition, those with PWS may suffer from debilitating anxiety, compulsive, and aggressive behaviors, struggles with sleep, and difficulty in school. It is typically unsafe for those with PWS to live in an unsupervised environment even once they have reached adulthood.

All of these aspects create heavy burdens on those with PWS and those who care for them. Cade receives a nightly growth hormone shot which is currently the ONLY FDA approved drug to help treat some of the symptoms surrounding PWS.

But there are no FDA approved treatments for hyperphagia. That is why the arrival of gene and cell therapies has brought hope to parents like me. However, the rapid pace of innovation in medicine has outpaced our state's capacity to finance these groundbreaking therapies.

Assembly Bill 687addresses this challenge by creating a platform for the Department of Health Services to enter into value-based arrangements. The legislation does not mandate such agreements but provides the flexibility between the state and manufacturers.

Value-based arrangements are a solution to mitigate the potential high costs associated with these transformative therapies. By measuring the outcomes of treatments and compensating Medicaid programs accordingly, we introduce a new level of accountability.

For our family living with PWS, we know the importance of timely access to new and advanced treatments. This legislation provides a forward-looking approach to help the ever-changing landscape of gene and cell therapy. It is our responsibility to ensure that individuals with rare diseases, such as PWS, have access to the most advanced and effective treatments available. It is a matter of life and death for many!

I urge you to support Assembly Bill 687 and advocate for its passage. By doing so, you contribute to the well-being of individuals like me who are eagerly anticipating the benefits of these groundbreaking therapies. Thank you for your time.

RARE READY

A GENETIC CONDITION COALITION

January 4, 2024

Dear Representative Moses,

The 38 organizations above offer enthusiastic support for A.B. 687, introduced by Rep. Schutt and Rep. Dittrich. The bill allows the state of Wisconsin, through its Department of Health Services, to enter into value-based purchasing (VBP) agreements with drug manufacturers to improve patients' access to innovative therapies. We urge you to lend your support by co-sponsoring A.B. 687, supporting it in committee or voting in favor when it comes to the floor.

Under VBP agreements, payment for innovative treatments, like gene therapies, is based on patient outcomes. If a patient does not respond or stops responding to the therapy, the drug manufacturer will provide a full or partial refund to the state payer. This payment system improves patients' access to innovative therapies and saves the state money by only paying for drugs that actually work.

VBP agreements hold drug manufacturers accountable for the efficacy of their drugs and support patient access to innovative therapies without restrictions or delays.

Thank you for your consideration. Your support will help patients living with rare or complex diseases get access to the innovative care they need and deserve.

Sincerely,

Members of the Rare & Ready Coalition