TECHNICAL BRIEF

AB 1887

Prescription Drug Coverage for Rare Diseases

CHBRP

About the Technical Brief

This document provides details on the analytical foundation for CHBRP's analysis of AB 1887. While the main report synthesizes key findings for immediate policy consideration, this document is designed to support a deeper understanding of the background of the topic of the legislation and CHBRP's methodology and research in conducting its analysis. It contains the data sources, methods, assumptions, and other bill-specific considerations necessary for legislative staff, fiscal analysts, and other stakeholders to fully understand the scope and impact of the proposed measure.

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Acronyms and Terminology

Acronyms

AB – Assembly Bill

CA – California

CalPERS – California Public Employees' Retirement System

CDI – California Department of Insurance

CHBRP – California Health Benefits Review Program

COHS – County Organized Health System

DMHC – Department of Managed Health Care

EHBs – essential health benefits

FDA – U.S. Food and Drug Administration

SB – Senate Bill

Terminology

CHBRP uses the following terminology for this analysis:

Orphan drug – a pharmaceutical product developed to treat rare diseases or conditions.

Rare disease – a condition affecting fewer than 200,000 persons in the United States.

Utilization management: Utilization management techniques are used by health plans and insurers to control costs, ensure medication compatibility, and manage safety. Examples include benefit coverage requirements related to prior authorization, step therapy, quantity limits, and limits related to the age or sex of the enrollee (such as prescription-only infant formula or prostate cancer screening for men).

Prior authorization:[1] Also known as precertification, prior approval, or prospective review, prior authorization is a utilization management technique commonly used by health insurance carriers to ensure that a given medical intervention meets the insurance plan or policy's criteria for coverage (Newcomer et al., 2017). Prior authorization was developed as a tool for insurers to assess the appropriateness of treatment that would result in a hospital admission or a high-cost procedure (Resneck, 2020). The primary uses of prior authorization include:

- **Coverage evaluation:** Allows evaluation of whether a test, treatment, or service is medically necessary and otherwise covered.
- **Safety:** Acts as a safeguard to confirm that a patient's medications are compatible and provides an opportunity to check that proper diagnostic testing has been completed to ensure patient safety prior to use of a requested treatment. Prior authorization also reduces inappropriate patient care by stopping unsafe or low-value care that is inconsistent with the most recent clinical evidence.
- **Cost control:** Imposition of prior authorization for nonpreferred medications can encourage the use of preferred medications that can be procured at lower price.

Step therapy: Also known as "fail-first" protocols, step therapy may be applied to prescription medications by health plans and insurers to control costs, ensure medication compatibility, and manage safety. Health plans/insurers may use step therapy protocols to apply clinical guidelines established by professional societies and other recognized organizations to treatment plans. They require an enrollee to try and fail one or more medications prior to receiving coverage for the initially prescribed medication. Step therapy protocols usually recommend starting with a medication that is less expensive (generics) and/or has more "post-marketing safety experience" (PBMI, 2015).

[1] More information about prior authorization is available in CHBRP's 2023 analysis *Prior Authorization in California*.

Legislative Text Analyzed

CHBRP analyzed AB 1887 Prescription Drug Coverage for Rare Diseases, as amended on March 26, 2026 per the request of the California Assembly Committee on Health. The text analyzed is copied below.

SECTION 1. Section 1342.76 is added to the Health and Safety Code, to read:

1342.76 (a) Notwithstanding any other law, a health care service plan contract issued, amended, or renewed on or after January 1, 2027, shall not impose prior authorization, step therapy, or other utilization review for a prescription drug approved by the United States Food and Drug Administration that is ~~prescribed~~ *approved* for the treatment of a rare disease if the ~~prescribing health care professional~~ *drug is prescribed by a specialist with expertise in the condition or disease being treated and the specialist* has determined the drug is medically necessary, unless a biosimilar, interchangeable biologic, or generic version of the drug is available.

(b) For purposes of this section, "rare disease" means a disease that affects fewer than 200,000 people in the United States.

(c) This section does not apply to Medi-Cal managed care contracts with the State Department of Health Care Services entered into pursuant to Chapter 7 (commencing with Section 14000) of, or Chapter 8 (commencing with Section 14200) of, Part 3 of Division 9 of the Welfare and Institutions Code.

SEC. 2. Section 10123.1936 is added to the Insurance Code, to read:

10123.1936. (a) Notwithstanding any other law, a health care service plan contract issued, amended, or renewed on or after January 1, 2027, shall not impose prior authorization, step therapy, or other utilization review for a prescription drug approved by the United States Food and Drug Administration that is ~~prescribed~~ *approved* for the treatment of a rare disease if the ~~prescribing health care professional~~ *drug is prescribed by a specialist with expertise in the condition or disease being treated and the specialist* has determined the drug is medically necessary, unless a biosimilar, interchangeable biologic, or generic version of the drug is available.

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SEC. 3. No reimbursement is required by this act pursuant to Section 6 of Article XIII B of the California Constitution because the only costs that may be incurred by a local agency or school district will be incurred because this act creates a new crime or infraction, eliminates a crime or infraction, or changes the penalty for a crime or infraction, within the meaning of Section 17556 of the Government Code, or changes the definition of a crime within the meaning of Section 6 of Article XIII B of the California Constitution.

Policy Framework

This brief provides additional material to support the findings and recommendations presented in CHBRP's Analysis of Assembly Bill 1887, Prescription Drug Coverage for Rare Diseases.[2] The following sections contain details on the California and federal landscape. While this information is essential to the completeness of the analysis, it has been placed in this separate brief to maintain the flow of the main report. Readers are encouraged to consult this material for deeper insights into existing laws and technical details that informed the analysis and conclusions of the main report.

California Policy Landscape

Previous California Legislation and Relevant Programs

Since 2019, multiple bills have been introduced to establish the Rare Disease Advisory Council (RDAC) in California.[3] The RDAC would be responsible for coordinating statewide efforts for studying the incidence of rare diseases within California, act as the advisory body on rare diseases to the Legislature and state agencies, and other related duties. In 2024, AB 2613 was chaptered to establish the Jacqueline Marie Zbur RDAC, making California the 29th state to establish an RDAC (NORD, 2024).

Housed under the California Health and Human Services Agency until January 1, 2029, the Jacqueline Marie Zbur RDAC is intended to act as the advisory body on rare diseases to the Legislature and consult with experts on rare diseases to develop recommendations to improve patient access to needed services, among other duties. To date, California's RDAC has not been funded and therefore remains inactive.

Federal Policy Landscape

Federal Laws

In 1983, Congress passed the Orphan Drug Act (ODA), which created financial incentives for drug and biologics manufacturers to research and develop treatments for orphan diseases. These include tax credits for the costs of clinical research, government grant funding, assistance for clinical research and a seven-year exclusive marketing period for the first sponsor of an orphan-designated product that obtains market approval from the U.S. Food and Drug Administration (FDA). Since the ODA passed, more than 250 orphan drugs have been developed and made available to more than 13 million Americans.

Essential Health Benefits

In California, nongrandfathered[4] individual and small-group health insurance is generally required to cover essential health benefits (EHBs) as mandated under the Affordable Care Act.[5] In 2027, approximately 11.5% of all Californians will be enrolled in a plan or policy that must cover EHBs.[6] States may require state-regulated health insurance to offer

[2] California Health Benefits Review Program (CHBRP). (2026). *Analysis of California Assembly Bill 1887 Prescription Drug Coverage for Rare Diseases*. Berkeley, CA.
[3] AB 1016 (2019); AB 2283 (2020); SB 247 (2021).
[4] A grandfathered health plan is "a group health plan that was created – or an individual health insurance policy that was purchased – on or before March 23, 2010. Plans or policies may lose their 'grandfathered' status if they make certain significant changes that reduce benefits or increase costs to consumers."
[5] For more detail, see CHBRP's issue brief, *Essential Health Benefits: An Overview of Benefits, Benchmark Plan Options, and EHBs in California*.
[6] See CHBRP's resource, *Sources of Health Insurance in California*.

benefits that exceed EHBs.[7,8,9,10] Should California do so, the state could be required to defray the cost of additionally mandated benefits for enrollees in health plans or policies purchased through Covered California, the state's health insurance marketplace. Because AB 1887 does not require coverage for any additional prescription drugs, the proposed mandate would not exceed the current definition of EHBs in California.

Federal Programs

The U.S. Food and Drug Administration (FDA) administers two programs to incentivize research and development of pharmaceuticals, biologics, and medical equipment to treat rare diseases.

- **The Rare Pediatric Disease Designation and Voucher Program:** aims to incentivize drug development for rare pediatric diseases. Sponsors who obtain FDA approval for a rare pediatric disease drug may be eligible to receive a priority review voucher. These vouchers can be redeemed to receive a priority review for a different product and can be transferred or sold to another sponsor (FDA, 2026d).
- **The Humanitarian Use Device Designation Program:** creates an alternative pathway for obtaining market approval of medical devices intended to treat or diagnose people with rare diseases or conditions (FDA, 2023). Notably, designation is limited to those devices that would impact diseases or conditions manifested in less than 8,000 individuals annually in the United States.

The FDA has also launched the Rare Disease Innovation Hub to accelerate treatment development (FDA, 2026b). It is a cross-center FDA initiative between the Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research designed to streamline the development of treatments for rare diseases by serving as a single point of engagement for the scientific community, and aligning regulatory science across different federal agencies, Within this hub is the Plausible Mechanism Framework, with draft guidance released in February 2026, to provide a set of recommendations to help entities developing individualized therapies generate sufficient clinical safety and efficacy data to demonstrate that a drug or biological products are safe and effective for their intended use (FDA, 2026a).

[7] ACA Section 1311(d)(3).
[8] State benefit mandates enacted on or before December 31, 2011, may be included in a state's EHBs, according to the U.S. Department of Health and Human Services (HHS). Patient Protection and Affordable Care Act; Standards Related to Essential Health Benefits, Actuarial Value, and Accreditation. Final Rule. Federal Register, Vol. 78, No. 37. February 25, 2013.
[9] However, as laid out in the Final Rule on EHBs U.S. Department of Health and Human Services (HHS) released in February 2013, state benefit mandates enacted on or before December 31, 2011, would be included in the state's EHBs, and there would be no requirement that the state defray the costs of those state-mandated benefits. For state benefit mandates enacted after December 31, 2011, that are identified as exceeding EHBs, the state would be required to defray the cost.
[10] In February 2026, HHS released a proposed rule that would alter what benefits would be determined to exceed EHBs. The conclusions in this analysis of AB 1887 are subject to change based on the final language of the regulations. U.S. Department of Health and Human Services (HHS). Patient Protection and Affordable Care Act, HHS Notice of Benefit and Payment Parameters for 2027; and Basic Health Program. Proposed Rule. Federal Register, Vol. 91, No. 28. February 11, 2026.

Background on Rare Diseases

This section provides context for the potential impacts of AB 1887, including an overview of rare diseases, prevalence, diagnosis, treatment, disparities and differences among rare diseases, barriers to diagnosis and treatment, and the overall societal impact of rare diseases.

Rare diseases (sometimes referred to as "orphan diseases") are medical disorders, illnesses, or conditions that affect a relatively small number of individuals. According to the National Institutes of Health and the Orphan Durg Act, rare diseases are those that affect fewer than 200,000 people in the United States, which is the definition used in this report (Congress.gov, 2024).[11] Rare diseases are often chronic, serious, and progressive in nature and can be life-threatening or life-limiting (NLM, 2017; Zhao et al., 2023).

Signs of rare diseases are often present at birth or in childhood, although there is a subset of rare diseases which do not appear until adulthood (NIH, 2024; NLM, 2017). Rare diseases can affect any body system and might affect multiple body systems. Approximately 80% of rare diseases are caused by genetic mutations (referred to as "Mendelian disorders") and may be inherited (passed down through families) but can also be new mutations (de novo) (NIH, 2024a). Rare diseases may also be caused by infections or environmental factors (NIH, 2024).

Examples of Rare Diseases

There are an estimated 5,000 to over 10,000 rare diseases in the United States (FDA, 2026c; NIH, 2024b). This variation is due to the lack of consistent definitions and categorization of rare diseases. Some diseases are considered "rare" in certain geographic regions but not in others. For example, tuberculosis and sickle cell disease are considered rare in the United States, but worldwide they are major global health burdens causing high morbidity and mortality (Colombatti et al., 2023; Williams et al., 2024).

Collectively, it is estimated that there are close to 4 million people in California with a rare disease, based on estimates that 25 million to 30 million Americans, or 1 in 10 Americans, have a rare disease (NIH, 2024).

Table 1 shows examples of rare diseases and their estimated prevalence in California.

[11] While rare diseases are defined the in United States by the 1983 Orphan Drug Act as a condition that affects fewer than 200,000 people, there are global differences in the definition of rare diseases. In Europe, rare diseases are defined as a condition that affects fewer than 1 in 2,000 people (Rath et al., 2017).

Table 1. Examples of Rare Diseases and Estimated Prevalence

Disease	Description	Estimated Prevalence	FDA-Approved Medications Indicated for Rare Disease
Cystic fibrosis	Caused by a genetic mutation that affects the lungs, gastrointestinal tract, and other body systems, causing severe respiratory issues	1 in 6,899 newborns in California	• Aztreonam (Azactam, Cayston) • Ceftazidime (Fortaz) • Chloramphenicol sodium succinate (Chloromycetin Sodium Succinate) • Dornase alfa (Pulmozyme) • Elexacaftor/tezacaftor/ivacaftor; ivacaftor (Trikafta) • Mannitol (Osmitro) • Tobramycin (AKTob, Tobi, Tobrasol, Tobrex, Bethnkis, Podhaler, Kitabis Pak) • Tobramycin sulfate (Nebcin, Tobi) • Vanzacaftor/tezacaftor, deutivacaftor (Alyfretek)
Duchenne muscular dystrophy	Caused by a genetic mutation that leads to progressive loss of muscle function and weakness	1 in 5,000 male children in the United States (approximately 7,800 male children in California) (a)	Vitolarsen (Viltepso)
Fabry disease	Caused by a genetic mutation that leads to a harmful buildup of fat, which affects the heart, kidneys, central nervous system, and skin	Ranges from 1 in 40,000 to 170,000 depending on form of disease and gender (b) (approximately 200 to 1,000 Californians) (a)	Pegunigalsidase alfa-lwxj (Elfabrio)
Huntington's disease	Caused by a genetic mutation that causes nerve cells in the brain to break down, severely affecting movement and balance	1 in 20,000 people in the United States (approximately 2,000 Californians) (a)	• Tetrabenzine (Xenazine) • Deutetrabenazine (Austedo, Astedo XR)
Phenylketonuria	Caused by a genetic mutation that prevents the body from processing phenylalanine (an amino acid), which can lead to brain damage or severe intellectual disabilities if untreated by medications and a strict low-protein diet	1 in 25,000 people in the United States (approximately 1,500 Californians) (a)	• Pegvaliase-pqpz (Palynziq) • Sapropterin dihydrochloride (Kuvan, Javygtor) • Sepiapterin (Sephience)
Pompe disease	Caused by a genetic mutation that leads to the buildup of glycogen in the body's cells and muscle weakness, which affects the heart and liver, leading to severe respiratory movement issues	1 in 40,000 people in the United States (approximately 1,000 Californians) (a)	Avaglucosidase alfa-ngpt (Nexviazyme)

Disease	Description	Estimated Prevalence	FDA-Approved Medications Indicated for Rare Disease
Sickle cell disease	Caused by a genetic mutation affecting red blood cells, which leads to damage in the bones, spleen, and other organs as well as severe pain crises	Approximately 7,000 Californians	• Hydroxyurea (Siklos, Hydrea) • L-glutamine (Endari) • Crizanlizumab (Adakveo)

Source: California Health Benefits Review Program, 2026, based on CDC, 2025; FDA 2026e; Hillert et al., 2020; Kharrazi et al., 2015; Lerario et al., 2024; Medina et al., 2022; NIH, 2013; NLM, 2016, 2021, 2022, 2024a, 2024b, 2025a, 2025b; Reeves et al., 2024.
Notes: (a) Calculated estimate based on California population of 38 million people.
(b) Fabry disease is X-linked (see the *Disparities* section for more details) and has multiple forms and more severely affects male children and adults. Female children and adults usually have milder symptoms, and a small percentage could have no symptoms.

Diagnosis of Rare Diseases

Because approximately 80% of rare diseases are genetic in origin, genetic testing can diagnose many rare diseases. Exome sequencing[12] and genome sequencing[13] of individuals and their family members can also help expedite the diagnostic process (Marwaha et al., 2022). Many rare diseases are included in the California Newborn Screening Program, a mandatory state-run initiative that tests a baby's blood within 12 to 48 hours of birth for over 25 severe conditions (CDPH, 2024).

The growing use of electronic health records has also led to the development of decision support systems to assist clinicians in diagnosis. Some tools may be focused on a specific disease or group of diseases, while other tools are aimed at providing general diagnosis support for all rare diseases (Faviez et al., 2020; Garcelon et al., 2020). Newer research is also exploring the use of artificial intelligence, such as language learning models, to support diagnosis (Hirsch et al., 2020; Wojtara et al., 2023).

Despite these advances, the rarity of these conditions can lead to a longer time to diagnose them and often require ultra-specialized clinicians. Delays in the diagnosis of rare diseases and other barriers to obtaining a rare disease diagnosis are discussed further in the *Barriers* section below.

Medication Treatments for Rare Diseases

The diversity of the conditions and the symptoms the diseases cause mean that there is not one treatment or set of treatments for all rare diseases. Each rare disease needs its own treatment or set of treatments and appropriate treatments are determined by specialists with expertise in the condition or disease being treated (Han et al., 2022). Vitamins and supplements are also a major component of treatments for rare diseases.[14] Non-medication treatments (e.g., low-protein formulas and foods for people with PKU) and other medical assistive equipment (e.g., wheelchairs and feeding tubes) may be necessary for certain diseases as well.

Medications for rare diseases can be split into three categories, only the first of which is included in the scope of AB 1887:

- **On-label usage:** An orphan drug is used on-label when it is administered to treat an indication (e.g., a disease or symptom) according to the FDA-approved labeling. Table 1 shows examples of rare diseases and the number of FDA-approved medications to treat the disease. In 2023, a study estimated around 5% of rare diseases have a disease-specific FDA-approved medication (Fermaglich and Miller, 2023).

[12] Test where DNA is extracted to analyze the protein-coding regions of a patient's genes to identify genetic variation.
[13] Test where DNA is extracted to analyze the entire genetic makeup to find changes in areas of the genome to identify genetic variation.
[14] Patients with cystic fibrosis are commonly prescribed multivitamins to prevent deficiencies in vitamins A, D, E, and K (Siwamogsatham et al., 2014).

The other two categories of medications which are not included in the scope of AB 1887 are:

- **Symptom management:** Some orphan drugs are FDA-approved medications that treat symptoms that are present in patients with a rare disease, but the medication is not specific for treatment of that rare disease. For example, anti-seizure medication to manage symptoms of Dravet syndrome, which causes seizures (Strzelczyk and Schubert-Bast, 2022).

- **Off-label usage:** An orphan drug is used off-label when it is administered for an indication not listed on its FDA-approved labeling (Adachi et al., 2023). For example, prednisone is often used off-label for Duchenne muscular dystrophy to slow progressive muscle weakness (Kourakis et al., 2021).

Utilization Management

As described in *Policy Context* section of CHBRP's Analysis of Assembly Bill 1887, utilization management techniques are used by health plans and insurers to control costs, ensure medication compatibility, and manage safety.

Medication Treatments for Rare Diseases

Because rare diseases usually have limited treatment options, step therapy is not typically used for utilization management for treatments of rare diseases.[15] Prior authorization is the utilization management technique that is commonly used for treatments for rare diseases. A study analyzing reimbursement for orphan drugs found there was a moderate use of prior authorization for orphan drugs, with the median payer using prior authorization for 45% of orphan drugs, which was higher than the use of prior authorization for non-orphan drugs (Cohen and Awatin, 2017). None of the payers in this study reported the use of step therapy for orphan drugs (Cohen and Awatin, 2017).

Health Care Professional Burden

A literature review of 164 articles examined spending related to drug utilization management (for non-rare diseases) generally estimated that payers, manufacturers, physicians, and patients collectively incur approximately $93.3 billion in costs annually from utilization management in 2019. Of this estimate, payers spend approximately $6.0 billion annually to implement and follow utilization management procedures. Utilization management is costly in time for clinicians as well, who devote an estimated $26.7 billion in time and effort navigating utilization management (Howell et al., 2021).

Several studies have indicated that utilization management could lead to health care provider burden. A survey of physicians found that 77% of physicians reported that prior authorization is the biggest utilization management barrier in their clinic and 58% of physicians hired someone to help with the administrative burden associated with utilization management. Sixty-four percent of physicians reported that utilization management contributed to feelings of burnout and 8% of physicians reported it was a major factor (Struthers et al., 2024). A cross-sectional study also found that 42% of health care professionals reported that prior authorization was a high contributor to burnout, and the follow-up process with private payers was the most reported step in the prior authorization process that led to burnout (Sahni et al., 2024).

Although these studies were for non-rare diseases, utilization management for rare disease medications could also have an impact on health care professionals and lead to administrative and financial burden on health care systems. Additional research is necessary to understand the impacts of utilization management on administrative burden and burnout of health care professionals.

[15] Per discussion with content experts, Drs. V. Ma and M. Martin. March 2026.

Disparities[16] in Rare Diseases

Disparities are noticeable and preventable or modifiable differences between groups of people. Health insurance benefit mandates or related legislation may impact disparities. Where intersections between health insurance benefit mandates and social drivers or systemic factors exist, CHBRP describes relevant literature.

CHBRP found literature identifying disparities in treatment access and treatment development by race or ethnicity, sex or gender, age, and geography. However, there are general challenges with obtaining a large, representative sample due to the small number of people who have rare diseases (see *Barriers* section below).

For many inherited rare diseases, there are also differences due to how the diseases genetically arise, which are not considered preventable nor modifiable disparities. These differences include:

- **Diseases linked to genetic lineage:** For example, cystic fibrosis and sickle cell disease are linked to genetic lineage. In the United States, cystic fibrosis is more common among non-Hispanic White individuals compared to Black or Asian American individuals, and sickle cell disease is more common among Black newborns (NLM, 2021, 2024b).
- **X-linked diseases**: when the female parent carries the genetic mutation that causes the disease and passes it down to their male newborn, who are typically more affected than a female newborn (NIH, 2025). Examples of X-linked diseases include Duchenne muscular dystrophy, which most often affects male children, and Fabry disease, which more severely affects male children but can also affect female children (NIH, 2013; NLM, 2022, 2024a).

Race or Ethnicity

Several studies observed that clinical trials for rare diseases are predominantly made up of non-Hispanic White groups, lacking representation from non-White patients, although recruitment of participants generally is a challenge for rare disease research (Goel et al., 2021; Serrano et al., 2023). The lack of racial and ethnicity representation in the development of new rare disease treatment could lead to unequal access or medical impacts.

Sex or Gender[17]

One cross-sectional study found that women with rare diseases experienced poorer health-related quality of life than men (Bogart and Irvin, 2017). Poorer health-related quality of life was defined as an individual's perceived physical and mental well-being.

Age

Many rare diseases present symptoms at birth or during early childhood, however there are challenges with researching and conducting clinical trials on children with rare diseases, as well as obtaining FDA approval for treatments for children.[18] There are greater restrictions on research involving children, demonstrated by a widening gap on clinical trials on adults with rare diseases as compared to children with rare diseases (Jacobson et al., 2024). Therefore, there can be disparities in treatment availability for younger or smaller (by body weight) persons with rare diseases.

[16] Several competing definitions of "health disparities" exist. CHBRP relies on the following definition: Health disparity is defined as the differences, whether unjust or not, in health status or outcomes within a population (Wyatt et al., 2016).
[17] CHBRP uses the National Academies of Sciences, Engineering, and Medicine distinction between "sex" and "gender": "'Sex' refers to biological differences between females and males, including chromosomes, sex organs, and endogenous hormonal profiles. 'Gender' refers to a multidimensional construct that links gender identity, a core element of a person's individual identity; gender expression, which is how a person communicates their gender to others; and social and cultural expectations about status, characteristics, and behavior that are associated with sex traits (NASEM, 2022).
[18] Per discussion with content expert.

Income

Rare diseases are costly to diagnose and treat. People with rare diseases have more out-of-pocket costs than people without rare diseases, so it can be challenging for people with rare diseases and their families to afford treatment (Adachi et al., 2023). Patients may have nonmedical costs such as with travel and lodging to receive specialty care or unpaid caregiving (Larkindale et al., 2014). In general, income is strongly associated with morbidity and mortality across the income distribution. Lower incomes are associated with lower life expectancy, higher rates of chronic disease and physical limitations, and worse self-reported health status (Khullar and Chokshi, 2018). A cross-sectional study of people with rare diseases in the United States found that people with lower incomes also experienced poorer quality of life . Generally, poor health contributes to reduced income, creating a negative feedback loop (Khullar and Chokshi, 2018).

Geography

There are a limited number of rare disease trained clinicians and specialty clinics in the United States, making it challenging for people with rare diseases to access specialized care. See the following section for more details.

Barriers to Accessing Timely Diagnoses and Treatment of Rare Diseases

Diagnostic Delay

Patients with rare diseases frequently experience a “diagnostic odyssey,” a prolonged period characterized by long delays and multiple incorrect diagnoses caused by the limited volume of existing medical research (Pavisich et al., 2024). Diagnostic testing can be extensive and expensive, requiring multiple specialist physicians and genetic counselor visits. In the United States, it is estimated that patients with rare diseases may need 17 interactions on average with the health care system to reach a diagnosis (Adachi et al., 2023).

Many patients also experience challenges with finding a clinician who has experience with recognizing or managing a variety of rare diseases (Adachi et al., 2023). Rare disease symptoms may be misattributed to more common conditions due to lack of awareness among clinicians and the clinical heterogeneity of rare diseases (Chaudhary and Kumar, 2025). It can be challenging for clinicians to gain knowledge and experience in caring for patients with rare diseases, and there is limited availability of clinical practice guidelines for treating rare diseases (Gittus et al., 2023).

It can take a range of months to over a decade to obtain a rare disease diagnosis, depending on the particular disease and age group. On average, it takes 4 to 5 years to get a rare disease diagnosis (Marwaha et al., 2022). California’s Newborn Screening Program has been shown to reduce delays in diagnosis and treatment by testing newborns for many conditions, including rare diseases, shortly after birth. An analysis of California’s newborn screening for cystic fibrosis found that the screening process had high detection rates of cystic fibrosis and that newborns with positive test results were evaluated by cystic fibrosis specialty centers at around 60 days of age and on average, were diagnosed within 6 months of age (Kharrazi et al., 2015). Phenylketonuria is another example of a rare disease which is tested at and diagnosed shortly after birth (NLM, 2025c).

Yet, for some patients, it could take over a decade to be diagnosed (Marwaha et al., 2022). For example, Fabry disease can manifest in different ways, with symptoms ranging from mild to severe, appearing as early as infancy but also could appear in adulthood (NLM, 2022). A study on delays in diagnosis of Fabry disease found that delays ranged from 13.7 years for males and 16.3 years for females (Reisin et al., 2017).

Medication Treatment Options

It is estimated that 5% of rare diseases have a disease-specific FDA-approved medication despite incentives to boost the development of therapies for rare diseases in the United States (Fermaglich and Miller, 2023; Rath et al., 2017). As a result, patients may need to rely on symptom management and supportive care which may not address the underlying disease (Chaudhary and Kumar, 2025). New research and development of medication treatments is a challenge due the

rarity and heterogeneity of rare diseases (Chaudhary and Kumar, 2025; Rath et al., 2017). For clinical trials for rare diseases, it is a challenge to identify participants because rare diseases are underrepresented in medical coding systems (ICD,[19] SNOMED,[20] etc.) and due to limited ability of registries to track patients with rare diseases (Rath et al., 2017). It is also challenging to recruit patients for clinical trials due to the small number of people impacted by each disease and who may need to travel long distances to research centers in order to participate (Bell and Tudur Smith, 2014; Rath et al., 2017). Studies have also observed the lack of ethnic and racial minority representation in clinical trials for rare diseases, and fewer clinical trials involving children with rare diseases (Goel et al., 2021; Jacobson et al., 2024; Serrano et al., 2023). The landscape for funding rare disease research is also complex and challenging due to prioritization of research with broader public health implications or greater potential for higher return on investment (Chaudhary and Kumar, 2025; Monaco et al., 2024).

Medication Access

Utilization management and medication access

A qualitative study that interviewed 15 parents of children with rare diseases found that parents had difficulty accessing needed health care services for their children and needed to interact repeatedly with insurance representatives during these processes. The authors conclude: "...patients with rare diseases may benefit from time limits for processing coverage decisions, increasing transparency in the claims and preauthorization processes, and more expansive authorizations for on-going needs" (Pasquini et al., 2021). Families may also need to pay out of pocket to avoid missed prescription drugs due to delays in treatment related to prior authorizations, as delays in treatment can exacerbate disease symptoms (Gotlieb et al., 2026).

Out-of-pocket costs [21]

Costs of treatment for rare diseases can be a large burden on individuals with rare diseases and their families, although costs of treatment can vary for each disease. Medications to treat rare diseases can be expensive and unaffordable for some patients (Adachi et al., 2023; Chaudhary and Kumar, 2025). In 2020, it was estimated that the annual out-of-pocket rare disease treatment costs were estimated to be over $20,000 more than people without a rare disease (Adachi et al., 2023).

Geography and travel costs

There are a small number of rare disease specialists, requiring families to travel or relocate to be able to access specialty care for rare conditions. People with rare diseases may also have to navigate different doctors, specialties, and health systems, which can also be time-consuming (Adachi et al., 2023). In California, the genetics workforce is geographically concentrated within metropolitan areas (Wojcik et al., 2023). One study on the genetics workforce found that people with rare diseases in California may need to travel 75 miles on average (and in some cases up to 300 miles) to access genetics care (Penon-Portmann et al., 2020). Reaching these specialty centers can require travel time and costs, missed work, and possibly relocation to be closer to specialty care for frequent, ongoing treatments.

Because some medications are physician-administered, some patients with rare diseases need to receive treatment at a specialized infusion center and require several months of appointments. For example, there are two gene therapies approved for people with sickle cell disease. These therapies involve drawing blood from a patient and collecting stem cells, which may require multiple appointments over several months to collect enough stem cells (FDA, 2023). These stem cells are then modified and delivered back to the patient as a one-time, single dose infusion (FDA, 2023).

[19] Coding system by the Whole Health Organization (WHO) for recording, reporting, and monitoring diseases.
[20] Systematically organized collection of medical terms, including codes, terms, and definitions, which is used for clinical documentation and reporting.
[21] There are also life-sustaining treatments with vitamin supplements (for conditions that drastically disrupt vitamin pathways) or food treatments (such as low protein foods for patients with PKU) that may not be covered by insurance, and cost of these treatments can be prohibitive to people with rare diseases and their families (NIH, 2024).

Societal Impact of Rare Diseases in the United States

The presence of rare diseases in the United States has direct and indirect economic and societal costs, which can vary for different rare diseases. Since rare diseases are often life-limiting and have significant medical needs, individuals with rare diseases or guardians of children with rare diseases may need to miss work, retire early, or require constant caregiving (Yang et al., 2022). The time needed to care for individuals with rare diseases often impacts a care givers' ability to work, especially if travel is necessary to access specialty care. Caregiver stress, isolation, and burnout, can also have an impact of caregiver well-being and productivity (Adams et al., 2016).

Indirect costs can include both patient and caregiver productivity loss, absenteeism, and losses due to early retirement. A 2022 assessment of the United States estimated that the total economic burden of rare diseases was $997 billion and found substantial indirect costs for patients with rare diseases (Yang et al., 2022). Indirect medical costs were estimated to be 44% ($437 billion) of the total economic burden, which was comparable to direct medical costs (45% of total economic burden or $449 billion) (Yang et al., 2022). The study also found that the per-person indirect costs for children with rare diseases were greater than indirect costs for adults with rare diseases (Yang et al., 2022).

Please note, the societal impact discussed here is relevant to a broader population than the one AB 1887 impacts, which would affect the health insurance of a subset of Californians (see the *Policy Context* section). See the *Benefit Coverage, Utilization, and Cost Impacts* section for estimates of direct cost impacts for the specific population targeted by AB 1887.

Medical Effectiveness Review

The medical effectiveness review related to AB 1887 summarizes evidence on the potential impact of utilization management on prescription drug treatments for rare diseases. For the purposes of this review, utilization management refers to prior authorization, step therapy, and utilization review techniques, as outlined in the bill text of AB 1887. See the *Legislative Text Analyzed* section for more details.

Research Approach and Methods

The literature search was limited to studies published from the last 10 years to include the most recent and relevant literature. A total of 13 studies were included in the medical effectiveness review for this report. The other articles were eliminated because they did not focus on utilization management and prescription drugs or rare diseases, were of poor quality,[22] or did not report findings from clinical research studies. A more thorough description of the methods used to conduct the medical effectiveness review and the process used to grade the evidence for each outcome measure is presented in CHBRP's Medical Effectiveness Analysis and Research Approach document.

The conclusions below are based on the latest available evidence from peer-reviewed and grey literature.[1] Unpublished studies are not reviewed because the results of such studies, if they exist, cannot be obtained within the 60-day working timeframe for producing CHBRP reports.

Key Questions

1. For persons with rare diseases, what is the impact of utilization management on access to FDA-approved orphan drugs?
   a. For persons with rare diseases, does utilization management *delay treatment* to prescription drugs?
   b. For persons with rare diseases, does utilization management for prescription drugs *impact clinical outcomes*?
2. For persons with conditions that are not rare diseases that require prescription drug treatments, does utilization management impact health care use and clinical outcomes (e.g., delays in care, morbidity, hospitalizations)?

Methodological Considerations

Due to the large number of rare diseases (~5,000–10,000 as outlined in the *Background on Rare Diseases* section) and CHBRP's 60-day timeframe, the medical effectiveness review does not include information about specific prescription drugs indicated for the treatment of specific rare diseases. CHBRP assumes that FDA-approved drugs indicated for the treatment of rare diseases are medically effective. Instead, the scope of the medical effectiveness review is limited to the impact of utilization management on access to prescription drugs indicated for the treatment of rare diseases and other chronic conditions, delays in treatment, clinical outcomes related to delays or denials of treatment, and the impact on clinicians and health care professionals.

CHBRP did not identify any studies that directly assessed the impact of utilization management on prescription drugs for rare diseases. In light of this, CHBRP included studies on utilization management for prescription drugs for the treatment of any chronic condition/disease. Although these studies assess the impact of utilization management on prescription drug treatments for non-rare diseases, they are the closest evidence available to assess the impact of utilization management for rare diseases. However, prescription drug treatments for rare diseases may differ from prescription drugs for other chronic diseases as there is typically one treatment available for rare diseases compared to multiple generic and/or non-

[22] For a detailed explanation of how CHBRP defines high-quality research, see the "Selecting Studies for Inclusion in the Literature Review" section of CHBRP's Medical Effectiveness Analysis and Research Approach document.

generic treatments for non-rare diseases. Utilization management may impact prescription drugs for rare diseases differently than non-rare diseases, but CHBRP found no evidence to distinguish the differences.

In addition, as discussed with the content experts CHBRP consulted with for this analysis, prior authorization is the primary utilization management technique used with prescriptions drugs for rare diseases; step therapy does not typically apply since the number of prescription drug options are limited for rare diseases. Out of the 5% of diseases for which there is an FDA-approved treatment for the condition, there is generally only one prescription drug treatment available for each rare disease.[23] Thus, CHBRP does not include step therapy in this review. Furthermore, **CHBRP found no studies that directly assessed rare diseases and delays in prescription drug treatments. Thus, CHBRP's analytic approach relied on information from content experts instead of findings for outcomes related to prescription drugs for non-rare diseases.**

The conclusions of the medical effectiveness review are limited due to differences in generalizability between prescription drug treatments for chronic non-rare diseases and rare diseases. CHBRP did not identify any randomized controlled trials or studies with true comparison groups,[24] and the evidence used for this analysis covers a broad range of diseases and prescription drugs. The research studies included in the medical effectiveness are retrospective and prospective study designs with mostly claims-based, electronic health record review, or survey data. Five of the retrospective studies included comparison groups (prior authorization vs no prior authorization). As these studies are observational, the generalizability of the evidence is limited.

Outcomes Assessed

The outcomes of interest in the medical effectiveness review related to utilization management include:

- Delays or denials of prescription drugs for the treatment of rare diseases;
- Delays or denials of prescription drugs for non-rare diseases; and
- Health care use and clinical outcomes due to delays in prescription drugs.

Study Findings Related to Utilization Management

This following section summarizes CHBRP's findings regarding the strength of evidence for the impact of utilization management on prescription drug treatments for rare diseases outlined by AB 1887. Each section is accompanied by a corresponding figure. The title of the figure indicates the test, treatment, or service for which evidence is summarized. The statement in the box above the figure provides CHBRP's conclusion regarding the strength of evidence about the effect of a particular test, treatment, or service based on a specific relevant outcome and the number of studies it is based on. Definitions of CHBRP's grading scale terms are included in the box below.

The following terms are used to characterize the body of evidence regarding an outcome:

Very strong evidence (formerly called *clear and convincing evidence*) indicates that there are multiple studies of a treatment and the large majority of studies are of high quality and consistently find that the treatment is either effective or not effective. Conclusions are unlikely to be altered by additional evidence.

Strong evidence (formerly called *preponderance of evidence*) indicates that the majority of the studies reviewed are consistent in their findings that treatment is either effective or not effective. Conclusions could be altered with additional strong evidence.

[23] Discussion with content expert, M. Martin.
[24] CHBRP identifies level of evidence for the medical effectiveness review based on research study design with or without comparison groups. True comparison groups are historical groups or before-and-after groups. The comparison groups used in the studies for this review were observational.

Some evidence (formerly called *limited evidence*) indicates that a small number of studies have limited generalizability to the population of interest and/or the studies have a serious methodological concern in research design or implementation. Conclusions could be altered with additional evidence.

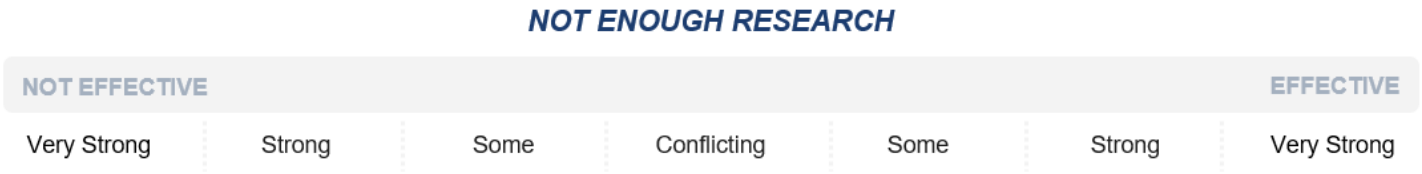
Conflicting evidence (formerly called *inconclusive evidence*) indicates that of the studies of equal quality, the number suggesting the treatment is effective is similar to the number of those suggesting the treatment is not effective.

Not enough research (formerly called *insufficient evidence*) indicates that (1) there are no studies of the treatment **or** (2) the available studies are not of high quality, meaning there is not enough evidence available to know whether or not a treatment is effective. *Not enough research* does not indicate that a treatment is not effective.

Utilization Management and Prescription Drugs for Rare Diseases

CHBRP found no studies that directly evaluated utilization management and prescription drug treatment for rare diseases.

Summary of findings regarding utilization management and access to prescription drugs for rare disease: CHBRP did not find any studies the assessed the impact of utilization management on access to prescription drugs for the treatment of rare diseases.



Utilization Management and Specialty and Common Prescription Drugs

CHBRP identified retrospective and prospective studies that assessed utilization management techniques on prescription drugs and other health care services not specific to rare diseases. The studies indicate that prior authorization requirements lead to delays in treatment, with median wait times ranging from several days to over a month. This utilization management tool affects a range of treatments, including biologic, antiepileptic, and oncology medications, resulting in treatment lapses or delayed initiation of care.

Delay to treatment initiation due to utilization management (delays to medication access)

Nguyen and colleagues (2023) conducted a retrospective chart review (N=35 patients) with an accompanied questionnaire (N=15) to assess the average time to initiation of dupilumab for the treatment of Eosinophilic esophagitis, a condition that was previously considered a rare disease, but has become increasingly more commonly diagnosed over the last 2 decades (NORD, 2025). The median time to starting dupilumab was 37 days (98% of prescriptions required prior authorization, 17% required letter of appeal, 2% required peer-to-peer, and 100% were eventually approved) (Nguyen et al., 2023).

Wirrell and colleagues (2018) conducted a survey among parents of children with epilepsy treated in the clinic (N=164) to evaluate how prior authorization resulted in delays to starting new antiepileptic drugs or a lapse in coverage for current antiepileptic drugs. Thirty-eight percent of parents reported that prior authorizations were required for new antiepileptic drugs. Forty-one (65%) children experienced delays in starting a new antiepileptic medication, 23 (36,5%) children experienced delays ≥7 days (1–4 weeks), and 24 (38%) children experienced a lapse in coverage for currently prescribed antiepileptic medications (Wirrell et al., 2018).

Dickens and colleagues (2025) conducted a study among pediatric oncology patients (N=68) to examine the occurrence of medication prior authorizations and resulting treatment delays. Among the sample, 38 patients required 69 medication prior authorizations during a 90-day study period. The study reported that medication prior authorizations delayed care in 22% of cases with a median delay of 4 days (range: 1–21 days) with most medications considered essential medication for cancer treatment (Dickens et al., 2025).

Two studies found that prior authorization delayed initiation of biologic treatments an average of 25 days up to 44 days (including time for approval and specialty pharmacy fill) for index prescriptions (Constant et al., 2022; Dudiak et al., 2021). Wallace et al. (2020) found that prior authorization for infusible medications prescribed in a rheumatology clinic was associated with significantly greater delays (median 31 days [IQR 15–60 days] vs median 27 days [IQR 13–41 days], p=0.045) compared to infusible medications with no prior authorization.

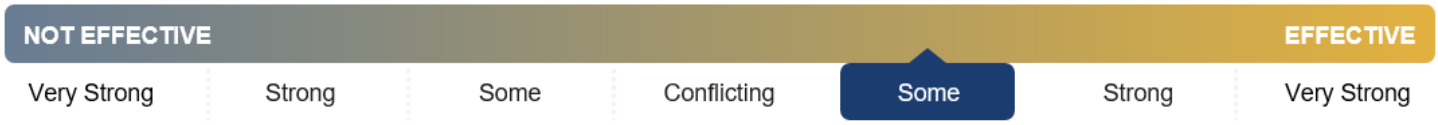
Table 2. Summary of Findings for Utilization Management and Delays in Prescription Drugs for Non-Rare Diseases

Study Authors	Study Design	Sample Size	Outcomes	Findings
Constant et al. (2022)	Retrospective cohort study, comparison groups PA vs no PA	N=190 pediatric patients with IBD with new biologic prescription	Impact of PA on biologic initiation time and IBD-related health care use	Median biologic initiation time for patients with PA was 25 days [IQR 16–38 days]; uncomplicated PA was associated with a 10.2-day (95% CI, 8.2- to 12.3-day) increase and complicated PA* was associated with a 24.6-day (95% CI, 16.4- to 32.8-day) increase in biologic initiation time compared with no PA
Dickens et al. (2025)	Prospective, multi-institutional cohort study, no comparison groups	N=68 pediatric patients with cancer	Medication PA during study and delays in care	56% of enrolled patients subject to at least one PA over 90 days; PA delayed care in 22% of cases with median of 4 days (range, 1–21 days)
Dudiak et al. (2021)	Retrospective cohort study, electronic health records review, no comparison groups	N=60 patients with severe asthma, N=20 patients with urticaria	Impact of clinical features, insurance, specialty pharmacy fill times, and quantified exacerbations and prednisone use while waiting biologic initiation	The mean time to receive the first dose of prescription biologic was 44 days (SD ±23.2) and includes (insurance approval (mean 21.5 SD ±19.6 days) and time for specialty pharmacy to fill prescription (mean 22.8 SD ±14.1 days)
Nguyen et al. (2023)	Retrospective chart review with survey questionnaire, no comparison groups	N=42 patients prescribed dupilumab for EoE	Prescribing practices of dupilumab, patient experiences with dupilumab	Median initiation time of 37 days (range, 5 to 154 days) with 98% required PA
Wallace et al. (2020)	Retrospective chart review, comparison groups PA vs No PA	N=225 patients with prescribed infusible prescription drugs ordered by provider in the rheumatology clinic	Time between the index date and infusion of prescription drug for patients with PA and without PA, approvals and denials of prescription drugs, glucocorticoid exposure within 90 days from index date	PA associated with significantly greater number of days to infusion compared to no PA (median 31 days [IQR 15–60 days] vs median 27 days [IQR 13–41 days], p=0.045)

Study Authors	Study Design	Sample Size	Outcomes	Findings
Wirrell et al. (2018)	Retrospective chart review and survey, comparison groups PA vs no PA	N=164 pediatric patients with epilepsy and prescribed AED	Survey data; patients with PA required vs PA not required, the PA process as smooth or not smooth	38.4% required PA; 53.7% did not require PA; 7.9% unsure of PA; 65% of patients with PA experienced delays in starting new AED (36.5% were greater than 7 days)

Source: California Health Benefits Review Program, 2026.
Note: * Complicated PA is defined as requiring step therapy, peer-to-peer review, or letter of appeal.
Key: AED = antiepileptic drug; EoE = eosinophilic esophagitis; IBD=inflammatory bowel disease; PA = prior authorization.

Summary of findings regarding prior authorization and delays to initiation of prescription drug treatments: Based on 5 retrospective studies (3 studies with comparison groups prior authorization vs no prior authorization) and 1 prospective cohort study for a range of medical conditions and pharmaceutical classes, there is *some* evidence that prior authorization for prescription drug treatments of chronic (non-rare) diseases could cause delays in the initiation of prescription drug treatment ranging from an average of 4 to 44 days.



Utilization management and denial of prescription drugs

Denial rates

Doshi et al. (2020) examined PCSK9i[25] prescription drug approval rates and reasons for rejection related to prior authorization for patients with high cholesterol from 2025 to 2017 (N=12,309). The study found initial approval rates for index prescriptions of 13% to 23% (initial denial rate of 77%–85%). Prior authorization–related rejections increased over the study period from 22% to 48%. Patients who had plans with six or more prior authorization criteria had lower odds of index and 90-day approval compared to patients with five or fewer prior authorization criteria (6 to 10 criteria: OR 0.63; 95% CI 0.44–0.92; greater than 11 criteria: OR 0.52; 95% CI 0.33–0.83) (Doshi et al., 2020).

For pediatric oncology patients, 61 of 69 medications with prior authorization requirements were approved, and the remaining 8 medications with prior authorization received a change in prescription for supportive care drugs (e.g., antiemetics, pain medications, antibiotics, antifungals, proton pump inhibitors, colony-stimulating factors, intravenous immune globulin) (Dickens et al., 2025). Among adult oncology patients, 97.5% of prior authorizations received initial approval and among patients seen in rheumatology clinic, 79% of prior authorizations for infusible prescription drugs received initial approval (Agarwal et al., 2017; Wallace et al., 2020). All patients prescribed dupilumab for the treatment of EoE eventually received insurance approval; 98% required prior authorization, 17% required a letter of appeal, and 2% were required peer-to-peer review (Nguyen et al., 2023).

Mizell (2024) assessed the relationship between disease-modifying therapies for patients with multiple sclerosis (N=52) and prior authorizations including the duration of the prior authorization processes, factors that determine prior authorization approval, and disease activity related to insurance restrictions for newly diagnosed patients with multiple sclerosis. The results found that 26 patients received initial approval and 26 received initial denial. The overall median approval time was 14 days (range, 1–79 days), median approval time for initial approvals was 4 days (range, 1–15 days), and median approval time for initial denials was 37 days (range, 8–79 days). Ninety percent of all prior authorizations

[25] Proprotein convertase subtilisin/kexin 9 inhibitors (PCSK9i) are cholesterol-lowering drugs.

were approved and 10% abandoned. The reasons for denial included step therapy (54%), criteria not met (27%), and nonformulary (19%) (Mizell, 2024).

Table 3. Summary of Findings for Utilization Management and Denials for Prescription Drugs for Non-Rare Diseases

Study Authors	Study Design	Sample Size	Outcomes	Findings
Agarwal et al. (2017)	Retrospective chart review, quality improvement study, no comparison groups	N=270 patients with breast cancer	Process for PAs, type of medications that require PA, outcomes of PA request, and time and resources involved with PA process	97.5% of initial PAs approved
Dickens et al. (2025)	Prospective, multi-institutional cohort study, no comparison groups	N=68 pediatric patients with cancer	Medication PA during study and delays in care	12% of prescriptions for supportive care for pediatric oncology patients were changed due to denial
Doshi et al. (2020)	Retrospective cohort study, medical and claims-based data, no comparison group	N=12,309 patients with PCSK9i prescriptions	Approval/denial of PCSK9i prescriptions, associations between plan-related factors and patient-level approval outcomes	Initial PA approval rates for index prescriptions of 13%–23%; PA-related rejections increased over the study period from 22% to 48%; 6 or more PA criteria had lower odds of index and 90-day approval compared to patients with 5 or fewer PA criteria
Mizell (2024)	Retrospective cohort study, no comparison groups	N=52 patients with multiple sclerosis with PA requirement for DMT	Duration of PA for disease-modifying therapies for patients with multiple sclerosis, factors that determine PA approval, disease activity and insurance restrictions	50% of patients received initial denial, 90% of all PA were approved
Nguyen et al. (2023)	Retrospective chart review with survey questionnaire, no comparison group	N=42 patients prescribed dupilumab for EoE	Prescribing practices of dupilumab, patient experiences with dupilumab	98% with PA, 17% letter of appeal, 2% peer-to-peer review, all patients received prescription
Wallace et al. (2020)	Retrospective electronic health records review, comparison groups PA vs no PA	N=225 patients with prescribed infusible prescription drugs ordered by provider in the rheumatology clinic	Time between the index date and infusion of prescription drug for patients with PA and without PA, approvals and denials of prescription drugs, glucocorticoid exposure within 90 days from index date	79% of PAs approved, 21% denied initially; 82% of PAs originally denied were approved after appeal; in total, 96% of prescriptions were approved

Source: California Health Benefits Review Program, 2026.
Key: DMT = disease-modifying therapies; PA = prior authorization; PCSK9i = Proprotein convertase subtilisin/kexin 9 inhibitors.

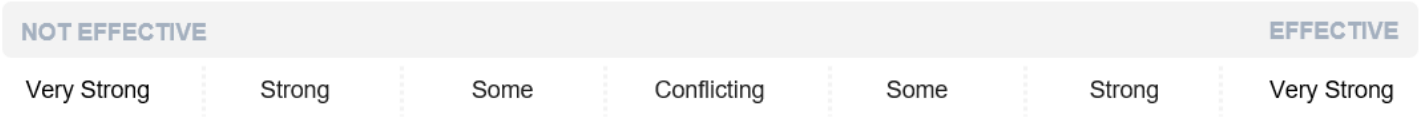
Summary of findings regarding prior authorization and prescription drug denials: Based on five retrospective claim-based/chart-review studies (one study with comparison groups prior authorization vs no prior authorization) and one prospective cohort study, there is conflicting evidence related to the impact of prior authorizations for prescription drugs denial rates, ranging from 0% denial (after peer-to-peer review) to 10% overall denial rate and differences in initial denial rates (50% to 87% compared to overall denial rates [0% to 10%]).

Denials and patient safety

One retrospective claims-based study with comparison groups (Gleason et al., 2013) assessed the implementation of a prior authorization program for dalfampridine as indicated for patients with multiple sclerosis and compared outcomes for patients in the prior authorization plan (N=60 patients) and a plan that did not require prior authorization (N=20). The outcomes assessed was dalfampridine utilization and its costs. The results of the study showed that for the patients with prior authorization requirements, 53.3% received initial approval and 38.3% reached final approval. Furthermore, it resulted in an average of 2.1 (P<0.0001) fewer claims per member in the prior authorization group versus the comparison group. The authors concluded that the 2-phase prior authorization process could potentially improve patient safety as the prior authorization process identified patients that did not meet clinical criteria for prescription drug treatment (e.g., safety concerns for renal impairment and increased risk of seizures) (Gleason et al., 2013).

Summary of findings regarding prior authorization, prescription drug denials, and patient safety: Based on one retrospective claims-based study with comparison group (prior authorization vs no prior authorization), there is not enough evidence that the prior authorization process impacts patient safety.

NOT ENOUGH RESEARCH



Utilization management and health care use

Prior authorization and unnecessary hospitalization and emergency department visits

Constant et al. (2022) found a 13% increased likelihood (95% CI, 2.5 to 3.4) of hospitalizations and emergency department use within 180 days of physician biologic recommendations associated with prior authorization for biologics among patients with inflammatory bowel disease. Patients with rheumatoid arthritis who experienced access restrictions (prior authorization and step therapy) for biologics were three times (2.3% vs. 0.8%; p<0.001) more likely to be hospitalized for infection compared to those without access restrictions (Boytsov et al., 2020). In addition, patients who experienced prior authorization delays for outpatient parenteral antimicrobial therapy[26] had significantly more days of inpatient treatment (7 days [IQR, 4–13 days] vs 3 days [IQR, 1–5 days], p<0.001) and longer duration of hospitalization (13 days [IQR, 8–25 days] vs 7 days [IQR, 5–10 days], p<0.001) compared to patients with no prior authorization delays (Bianchini et al., 2020).

Wirrell et al. (2018) found that patients with epilepsy and prior authorization experienced higher number of emergency department visits (*p*<0.001, mean number visits 5.3 vs 1.8) and hospitalizations (p<0.001, mean number of hospitalizations 3.6 vs 1.3) compared to patients without prior authorization.

[26] Outpatient parenteral antimicrobial therapy allows patients to receive intravenous antibiotics or antimicrobials in the community setting such as in the clinic or at home (Barr and Seaton, 2013).

Table 4. Summary of Findings for Utilization Management and Health Care Use for Non-Rare Diseases

Study Authors	Study Design	Sample Size	Outcomes	Findings
Hospitalizations and emergency department visits				
Bianchini et al. (2020)	Retrospective cohort, electronic health record review, comparison groups PA delay vs no PA delay	N=200 patients prescribed OPAT	Direct hospital costs, unplanned 30-day readmissions, discharge delays, patient safety outcomes	PA delays for OPAT significantly more days of inpatient treatment (7 days [IQR, 4–13 days] vs 3 days [IQR, 1–5 days], p<0.001) and longer duration of hospitalization (13 days [IQR, 8–25 days] vs 7 days [IQR, 5–10 days], p<0.001) compared to patients with no prior authorization delays
Boytsov et al. (2020)	Retrospective, claims-based data, with health plan restrictions vs without health plan restrictions	N=3,993 patients with RA; N=1,713 patients with PsA	Compare treatment effectiveness bDMARD or tsDMARD with and without health plan restrictions	Patients with RA and access restrictions were 3 times more likely to be hospitalized for infection compared to RA patients without access restrictions
Constant et al. (2022)	Retrospective cohort study, comparison groups PA vs no PA	N=190 pediatric patients with IBD with new biologic prescription	Impact of PA on biologic initiation time and IBD-related health care use	13% increased likelihood of IBD-related health care within 180 days of physician biologic recommendation associated with PA
Wirrell et al. (2018)	Retrospective chart review and survey, comparison groups PA vs no PA	N=164 pediatric patients with epilepsy and prescribed AED	Survey data; patients with PA required vs PA not required, the PA process as smooth or not smooth	Significantly increased hospitalization and emergency use among patients with PA compared to patients without PA (p<0.001)

Source: California Health Benefits Review Program, 2026.
Key: AED = antiepileptic drug; bDMARD = biologic disease modifying antirheumatic drugs; IBD = inflammatory bowel disease; OPAT = outpatient parenteral antimicrobial therapy; PA = prior authorization; RA = rheumatoid arthritis; PsA = psoriatic arthritis; tsDMARD = targeted synthetic disease-modifying antirheumatic drugs.

Summary of findings regarding utilization management and health care use: Based on four retrospective studies with comparison groups (prior authorization vs no prior authorization; prior authorization delays vs no delays; health plan restrictions vs no restrictions), there is *some evidence* that utilization management for prescription drugs for **non-rare diseases** are related to unnecessary hospitalizations and emergency department visits that result from delays in prescription drug treatments.

Utilization management and clinical outcomes

Prior authorization and increased morbidity

Constant et al. (2022) reported increased use of symptom management prescription drugs such as corticosteroids (14% increased likelihood, 95% CI 3.3 to 24.8) related to delays in prescription drug treatments that result from utilization management (Constant et al., 2022). Patients at the rheumatology clinic who received initial denial of prior authorization for infusible prescription drugs, experienced greater exposure to glucocorticoids compared to patients without prior authorization requirements (median 605 mg [IQR 0.0–1,575 mg] vs median 160 mg [0.0–675 mg], p=0.01) (Wallace et al., 2020).

Wirrell et al. (2018) found that patients with epilepsy who had prior authorization requirements experienced increased seizure frequency (p<0.001), greater use of rescue medications (p<0.001), higher likelihood of dietary restrictions (e.g., ketogenic diet) (p=0.003) and/or vagus nerve stimulation therapies (p<0.001) compared to patients without prior authorization.

Mizell (2024) reported a significantly increased likelihood of disease activity[27] (OR, 6.18; 95% CI, 1.33–44.86; p=0.03) among patients with multiple sclerosis who received an initial prior authorization denial for disease-modifying therapies compared to patients who had an initial approval.

Table 5. Summary of Findings for Utilization Management and Clinical Outcomes for Non-Rare Diseases

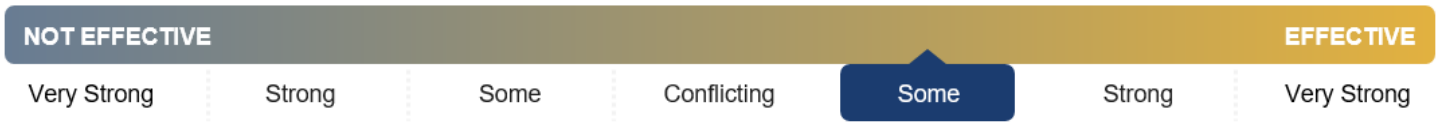
Study Authors	Study Design	Sample Size	Outcomes	Findings
Increased morbidity				
Constant et al. (2022)	Retrospective cohort study, comparison groups PA vs no PA	N=190 pediatric patients with IBD with new biologic prescription	Impact of PA on biologic initiation time and IBD-related health care use	14% increased likelihood of corticosteroid use at 90 days associated with PA
Mizell (2024)	Retrospective cohort study, no comparison groups	N=52 patients with multiple sclerosis with PA requirement for DMT	Assess duration of PA for disease-modifying therapies for patients with multiple sclerosis, factors that determine PA approval, disease activity and insurance restrictions	Initial PA denial associated with increased disease activity compared to initial approval (OR, 6.18; 95% CI, 1.33–44.86; p=0.03)
Wallace et al. (2020)	Retrospective electronic health records review, comparison groups PA vs No PA	N=225 patients with prescribed infusible prescription drugs ordered by provider in the rheumatology clinic	Time between the index date and infusion of prescription drug for patients with PA and without PA, approvals and denials of prescription drugs, glucocorticoid exposure within 90 days from index date	Initial PA denial was associated with significantly greater glucocorticoid exposure compared to no PA (median 605 mg [IQR 0.0–1,575 mg] vs median 160 mg [0.0–675 mg], p=0.01)

[27] Disease activity was defined as “a relapse or a new or enhancing lesion on imaging that occurred after the prior authorization was initiated within 6 months of approval” (Mizell, 2024).

Study Authors	Study Design	Sample Size	Outcomes	Findings
Wirrell et al. (2018)	Retrospective chart review and survey, comparison groups PA vs no PA	N=164 pediatric patients with epilepsy and prescribed AED	Survey data; patients with PA required vs PA not required, the PA process as smooth or not smooth	Significantly increased seizure frequency and rescue medication use, vagus nerve stimulation (p<0.001), and likelihood of dietary restriction (p=0.003) for PA compared to no PA

Source: California Health Benefits Review Program, 2026.
Key: AED = antiepileptic drug; bDMARD = biologic disease modifying antirheumatic drugs; IBD = inflammatory bowel disease; OPAT = outpatient parenteral antimicrobial therapy; PA = prior authorization; PsA = psoriatic arthritis; RA = rheumatoid arthritis; tsDMARD = targeted synthetic disease-modifying antirheumatic drugs.

Summary of findings regarding utilization management and clinical outcomes: Based on four retrospective studies (three studies with comparison groups of prior authorization vs no prior authorization), there is *some evidence* that utilization management for prescription drugs for **non-rare diseases** are related to increased morbidity that results from delays in prescription drug treatments.



Summary of Findings

CHBRP found there is *not enough research* about the impact of utilization management on access to prescription drug treatments for rare diseases. Lack of evidence of an effect does not mean there is a lack of effect, but rather that there are no studies on this topic. CHBRP found some evidence that prior authorization results in delays for initiation of prescription drug treatments for non-rare diseases from an average of 4 to of 44 days. There was conflicting evidence on the impact of prior authorization for eventual prescription drug access as most, but not all studies reported initial denials of coverage through the prior authorization process with eventual approval after appeals or peer-to-peer review. CHBRP found *some evidence* that prior authorization causes delays for initiation of prescription drugs which may contribute to unnecessary hospitalizations and emergency departments visits and *some evidence* that delays for initiation of prescription drugs due to prior authorization impact clinical outcomes such as increased disease morbidity. Lastly, CHBRP found *there is not enough research* that prior authorization for prescription drugs for non-rare diseases impacts patient safety.

Utilization management may impact prescription drugs for rare diseases differently than non-rare diseases. Thus, CHBRP's analytic approach relied on information from content experts instead of findings for outcomes related to prescription drugs for non-rare diseases.

Additional Analytical Assumptions and Other Considerations for Policymakers

Analytical Assumptions

In addition to the assumptions described in the *Analytical Approach and Assumptions* section of CHBRP's Analysis of California Assembly Bill 1887, CHBRP made the following assumptions:

Pharmacy Benefit Coverage

CHBRP has assumed that plans and policies that do not have coverage for outpatient prescription drugs or brand-name outpatient prescription drugs would not be required to do so for FDA-approved orphan drugs for rare diseases. Almost all (95.3%) commercial/CalPERS enrollees in plans and policies regulated by Department of Managed Health Care (DMHC) or California Department of Insurance (CDI) have an outpatient pharmacy benefit regulated by DMHC or CDI that covers both generic and brand-name outpatient prescription medications.[28] Of the remaining commercial/California Public Employees' Retirement System (CalPERS) enrollees, 0.99% do not have a pharmacy benefit and 3.7% have a pharmacy benefit that is not regulated by DMHC or CDI. In other words, CHBRP assumes AB 1887 would have no impact for plans without a regulated pharmacy benefit except for CalPERS.

Additional Assumptions on Baseline and Postmandate Coverage and Utilization

CHBRP made the following assumptions:

- All enrollees with state-regulated commercial insurance coverage face utilization management for FDA-approved medications that are indicated for the specific rare disease on the FDA label without a generic or biosimilar available.
- Approximately 5% of rare diseases have an available FDA-approved medication indicated for the specific rare disease. As a result, the population of enrollees subject to the utilization management requirements addressed by AB 1887 represents a subset of all enrollees with rare diseases.
    - Utilization management leads to 60-day delays for prescriptions in new users, i.e., individuals with rare diseases starting FDA-approved drugs specific to rare diseases.[29] The 60-day delay estimate reflects clinical expert opinion from a specialist in rare metabolic diseases with extensive experience managing utilization management requirements for this patient population. Evidence from the literature suggests that utilization management in the form of prior authorization and step therapy functions primarily as an administrative barrier that delays care rather than reducing long-term utilization. Studies of prior authorization for single-indication orphan drugs found no meaningful difference in utilization between those subject to prior authorization and those who were not, with average fills per beneficiary per year nearly identical across groups. High approval rates further support this conclusion: one prospective study found that 96.2% of prior authorization requests were ultimately approved, suggesting that the administrative process rarely results in outright denial, though 59.6% of approvals still resulted in delays greater than 24 hours. For non-rare conditions such as juvenile rheumatoid arthritis, Crohn's disease, and psoriasis, prior authorization had insignificant effects on drug use, likely due to the lack of therapeutic alternatives. Where utilization was initially reduced following prior authorization implementation, utilization eventually returned to levels comparable to states without prior authorization requirements. However, these delays carry clinical consequences, an American Medical Association survey found that 94% of physicians reported prior authorization-related delays in necessary care, with delays linked to disease exacerbation and preventable hospitalizations (AMA, 2025).

[28] For more detail, please see CHBRP's resource *Pharmacy Benefit Coverage in State-Regulated Health Insurance*.
[29] Per discussion with content experts, Drs. V. Ma and M. Martin.

Quantifiable delays in treatment initiation ranged from 10 to 46 days across conditions including pediatric inflammatory bowel disease and severe asthma.
    - Unlike the studies reviewed, which examined utilization management delays in more common conditions over shorter timeframes, rare disease prior authorization processes are typically more burdensome, often requiring detailed diagnostic documentation, genetic testing results, and specialist attestation, justifying a longer estimated delay than those observed in the published literature.
- 10% of enrollees with a rare disease who are eligible to take orphan drugs start a new drug each year.[30]
    - For these new starters, removal of utilization management requirements is assumed to reduce delays in accessing their medication by 60 days, based on clinical expert opinion reflecting the typically burdensome prior authorization process for rare disease medications.
    - This reduction in delay means that new starters would receive a full year of fills in their first year of treatment rather than approximately 10 months. In other words, eliminating utilization management would result in those users receiving two additional 30-day prescriptions per year.
    - The resulting increase in prescriptions varies by drug type, as injectables and infusions are typically administered less frequently (e.g., quarterly) than oral medications, meaning the 60-day delay displaces fewer administrations and produces a smaller percentage increase in fills for those drug types.
    - Some enrollees with rare diseases may be taking more than one FDA-approved drug specifically indicated for the rare disease.
- Utilization management does not impact existing users, i.e., individuals already taking FDA-approved medications indicated for rare diseases. While it is possible that utilization management could impact a small proportion of individuals who may experience gaps or delays during the beginning of plan years,[31] CHBRP was unable to quantify this number. The cost impact of AB 1887 reflects an acceleration effect rather than induced demand, that is, costs increase because patients access medications sooner than they otherwise would, not because new patients begin treatments they would not have otherwise received.
- The new prescription replaces a delayed start to the same medication, not a switch from an alternative therapy or the addition of a new drug on top of an existing regimen.
- Gene therapies for rare diseases are covered under reinsurance programs[32] and therefore excluded them from the utilization and cost estimates, as increases in the use of these treatments would not affect expenditures.
- AB 1887 specifies that the prohibition on utilization management applies only when a drug is prescribed by a specialist with expertise in the condition or disease being treated. CHBRP assumes that all FDA-approved drugs for rare diseases are prescribed by specialists with relevant expertise, as the complexity of rare disease diagnosis and management typically requires specialist involvement.

Other Considerations for Policymakers

In addition to the impacts a bill may have on benefit coverage, utilization, and cost, related considerations for policymakers are discussed below.

Postmandate Administrative and Other Expenses

CHBRP estimates that the increase in administrative costs of DMHC-regulated plans and/or CDI-regulated policies will remain proportional to the increase in premiums. CHBRP assumes that if health care costs increase as a result of increased utilization or changes in unit costs, there is a corresponding proportional increase in administrative costs.

[30] Per discussion with content experts, Drs. V. Ma and M. Martin.
[31] Per discussion with content experts, Drs. V. Ma and M. Martin.
[32] Reinsurance programs provide financial protection to health plans against high-cost claims by transferring a portion of the risk to a secondary insurer. Reinsurance purchased by an insurance company or health maintenance organization allows the company to pass all or part of its risk to another insurance company, either on a per-person basis or on a pooled basis. Typical reinsurance policies cover medical expenses surpassing $250,000–$500,000 per individual annually, a threshold into which all currently approved cell and gene therapies would fall. Given the high cost of gene therapies for rare diseases, with many therapies priced between $2 million and $4 million per treatment, health plans rely on reinsurance or stop-loss coverage specifically designed for gene therapies.

CHBRP assumes that the administrative cost portion of premiums is unchanged. All health plans and insurers include a component for administration and profit in their premiums.

State Health Care Spending Target

In 2024, in an effort to slow health care spending growth and improve health care affordability for California families, California's Office of Health Care Affordability (OHCA) under the Department of Health Care Access and Information approved a statewide target for maximum annual growth in health care spending for certain health care entities. The targets apply to per capita spending to specific entities, including health plans and insurers, provider organizations with at least 25 physicians, and hospitals (HCAI, 2022). The state is implementing this target with a phased-in approach, with a spending target of 3.5% for 2026, lowered to 3.2% in 2027 and 2028, and will be at 3% for 2029 and beyond (HCAI, 2025). Since health insurance benefit mandates may increase health care spending, such as increases to insurance premiums, administrative costs, and out-of-pocket costs, OHCA spending targets may be relevant considerations in benefit mandate policy decisions.

Postmandate Changes in the Number of Uninsured Persons

CHBRP assumes that if premiums increase by more than 1.7% in the small- or large-group market segments or 0.6% in the individual market, some enrollees will lapse their coverage. Because the change in average premiums do not exceed either of these thresholds (see Table 5, Table 9, and Table 10 in CHBRP's Analysis of California Assembly Bill 1887), CHBRP would expect no measurable change in the number of uninsured persons due to the enactment of AB 1887.

Changes in Public Program Enrollment

CHBRP estimates that the mandate would produce no measurable impact on enrollment in publicly funded insurance programs due to the enactment of AB 1887.

How Lack of Benefit Coverage Results in Cost Shifts to Other Payers

Without the mandate, enrollees with rare diseases who face utilization management delays may bear out-of-pocket costs for FDA-approved orphan drugs during the delay period or may go without treatment. Given the high cost of FDA-approved orphan drugs, particularly biologics, with an average cost per physician-administered drug of $17,800, it is unlikely that most enrollees would be able to self-pay for these treatments during a 60-day delay period.

Enrollees unable to access FDA-approved orphan drugs through their commercial insurance may seek assistance through pharmaceutical manufacturer patient assistance programs, which provide drugs at reduced or no cost to eligible patients. Nonprofit organizations and rare disease advocacy groups may also provide financial assistance to enrollees facing coverage delays, though the extent to which this occurs is unknown. Several nonprofit organizations provide supplemental coverage or financial assistance to enrollees with rare diseases in California. California Medicaid's Genetically Handicapped Persons Program (GHPP) provides health care coverage and case management services for adults with specific genetic diseases, helping beneficiaries with health care costs (DHCS, 2026). At the national level, the National Organization for Rare Disorders (NORD) has provided patient assistance programs since 1987, offering help with medication costs, insurance premiums, co-pays, diagnostic testing, and travel assistance for clinical trials.[33] The Assistance Fund (TAF), an independent nonprofit, provides financial assistance for copayments, coinsurance, and deductibles for patients with over 100 disease states who meet income and insurance eligibility requirements.[34]

The administrative burden of utilization management for FDA-approved drugs for rare diseases falls primarily on physician offices, which are responsible for submitting and managing prior authorization requests on behalf of their patients. For physician-administered drugs in particular, which have an average unit cost of $17,800 for biologics, the prior

[33] For more information, see NORD.
[34] For more information, see The Assistance Fund.

authorization process requires significant staff time and resources to navigate, and delays in approval directly delay the administration of treatment. Evidence suggests that prior authorization-related administrative costs are substantial, with physician practices spending considerable time and resources on the submission, follow-up, and appeals processes associated with prior authorization requests. Utilization management is estimated to cost payers, manufacturers, physicians, and patients a combined $93.3 billion annually (2019 dollars), including $6.0 billion for payers to implement utilization management procedures and $26.7 billion in clinician time navigating them (Howell et al., 2021). A cross-sectional study across 11 dermatology clinic locations found that prior authorization imposed substantial administrative burden, with costs per prior authorization of $6.72 overall and $15.80 specifically for biologics, requiring 170 staff hours over a single 30-day period (Carlisle et al., 2020). These administrative costs are not captured in CHBRP's utilization and premium estimates but represent a real burden on providers serving enrollees with rare diseases.

CHBRP was unable to provide a quantifiable estimate of cost shifts to other payers resulting from utilization management delays for FDA-approved orphan drugs.

Cost Impact Analysis: Data Sources, Caveats, and Assumptions

With the assistance of CHBRP's contracted actuarial firm, Milliman, Inc., the cost analysis presented in this report was prepared by the faculty and researchers connected to CHBRP's Task Force with expertise in health economics.[35] Information on the generally used data sources and estimation methods, as well as caveats and assumptions generally applicable to CHBRP's cost impact analyses are available at CHBRP's website.[36]

Current step therapy requirements were determined by a survey of the largest (by enrollment) providers of health insurance in California. Responses to this survey represented 72% of commercial enrollees with health insurance that can be subject to state benefit mandates. In addition, CalPERS and DHCS were queried regarding related benefit coverage.

For this analysis, CHBRP relied on CPT codes to identify services related to AB 1887.

Health Cost Guidelines

The Health Cost Guidelines (HCGs) are a health care pricing tool used by actuaries in many of the major health plans in the United States. The guidelines provide a flexible but consistent basis for estimating health care costs for a wide variety of commercial health insurance plans. It is likely that these organizations use the HCGs, among other tools, to determine the initial premium impact of any new mandate. Thus, in addition to producing accurate estimates of the costs of a mandate, we believe the HCG-based values are also good estimates of the premium impact as estimated by the HMOs and insurance companies.

The highlights of the commercial HCGs include:

- Specific major medical, managed care, and prescription drug rating sections and guidance with step-by-step rating instructions.
- Other helpful analysis resources, such as inpatient length of stay distribution tables, Medicare Severity-Adjusted Diagnosis Related Group (MS-DRG) models, and supplementary sections addressing EHBs and mandated benefits, experience rating, and individual and small group rating considerations.

[35] CHBRP's authorizing statute, available at https://chbrp.org/about_chbrp/index.php, requires that CHBRP use a certified actuary or "other person with relevant knowledge and expertise" to determine financial impact.
[36] See method documents posted at https://www.chbrp.org/about/analysis-methodology/cost-impact-analysis.php; in particular, see Cost Analyses: Data Sources, Caveats, and Assumptions.

- Presentation of loosely and well-managed nationwide utilization and cost information by Milliman benefit-aligned service categories used throughout the Rating Structures – inpatient hospital services for both loosely and well-managed are also supported by DRG-level utilization and cost benchmarks.
- Annual updates address emerging regulatory considerations such as health care reform and mental health parity requirements.
- Annually updated benefit descriptions used in the HCG service categories.
- Annually updated medical trend assumptions and considerations.
- Presentation of two sets of nationwide area factors to facilitate development of area-specific claim costs, including separate utilization and charge level factors by type of benefit, state and Metropolitan Statistical Area for first-dollar coverage, and composite factors by deductible amount.
- Claim Probability Distributions (CPDs) by type of coverage that contain distributions of claim severity patterns for unique combinations of benefits and member types (adult, child, composite member).
- The Prescription Drug Rating Model (RXRM), an automated rating tool that provides a detailed analysis of prescription drug costs and benefits.

Consolidated Health Cost Guidelines Database

Milliman maintains benchmarking and analytic databases that include health care claims data for nearly 60 million commercial lives and over 3 million lives of Medicaid managed care data. This dataset, Milliman's Consolidated Health Cost Guidelines™ Sources Database (CHSD), is routinely used to evaluate program impacts on cost and other outcomes.

Analysis-Specific Caveats and Assumptions

The population subject to AB 1887 includes DMHC-regulated commercial insurance plans, CDI-regulated policies, and CalPERS plans subject to the requirements of the Knox-Keene Health Care Service Plan Act that offer outpatient pharmacy coverage and injections through the medical benefit.

Methodology and Assumptions for Baseline Utilization

- CHBRP identified FDA-designated orphan small-molecule and biologic drugs without a generic alternative. Drugs obtained through the pharmacy benefit were identified using the brand name of the drug, whereas drugs obtained through the medical benefit were identified using Level II Healthcare Common Procedure Coding System (HCPCS) codes. Uncommon drugs attributed to claim identifiers that do not provide a specific designation to a single treatment were excluded from the analysis.
- CHBRP identified FDA-designated gene therapies.[37] Drugs classified as gene therapies were excluded from the analysis.
- CHBRP calculated the total 30-day scripts or injections per 1,000 enrollees of rare disease drugs for commercial populations using Milliman's proprietary 2024 CHSD. The rare disease users were trended from 2024 to 2027 using a 0% utilizer trend due to the rare nature of the diseases these drugs treat.

Methodology and Assumptions for Baseline Cost

- CHBRP summarized the average commercial cost per 30-day script or injection for rare disease drugs using CHSD. This average commercial cost per 30-day script or injection was trended from 2024 to 2027 using a 9.75% pharmacy cost trend based on the 2025 Milliman Health Cost Guidelines.

[37] FDA approved cellular and gene therapy products: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products.

Methodology and Assumptions for Baseline Cost Sharing

- CHBRP summarized the average commercial cost sharing per 30-day script or injection for rare disease drugs using CHSD. This average commercial cost sharing per 30-day script or injection was trended from 2024 to 2027 using a 9.75% pharmacy cost trend based on the 2025 Milliman Health Cost Guidelines.

Methodology and Assumptions for Postmandate Utilization

- CHBRP assumed 10% of users of rare disease drugs would receive two additional 30-day scripts or injections as a result of AB 1887.

Methodology and Assumptions for Postmandate Cost

- CHBRP assumed the average cost per script would not change as a result of AB 1887.

Methodology and Assumptions for Postmandate Cost Sharing

- CHBRP assumed the average cost sharing per script would not change as a result of AB 1887. The composite average cost sharing across a population would change due to a projected change in the mix of drugs being prescribed.

Determining Public Demand for the Proposed Mandate

CHBRP reviews public demand for benefits by comparing the benefits provided by self-insured health plans or policies (which are not regulated by the DMHC or CDI and therefore not subject to state-level mandates) with the benefits that are provided by plans or policies that would be subject to the mandate.

Among publicly funded self-insured health insurance policies, the preferred provider organization (PPO) plans offered by CalPERS have the largest number of enrollees. The CalPERS PPOs currently provide benefit coverage similar to what is available through group health insurance plans and policies that would be subject to the mandate.

To further investigate public demand, CHBRP used the bill-specific coverage survey to ask plans and insurers who act as third-party administrators for (non-CalPERS) self-insured group health insurance programs whether the relevant benefit coverage differed from what is offered in group market plans or policies that would be subject to the mandate. The responses indicated that there were no substantive differences.

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CHBRP is an independent program administered and housed by the University of California, Berkeley, under the Office of the Vice Chancellor for Research. A group of faculty, researchers, and staff complete the analysis that informs CHBRP reports. The CHBRP **Faculty Task Force** comprises rotating senior faculty from University of California (UC) campuses. In addition to these representatives, there are other ongoing researchers and analysts who are **Task Force Contributors** to CHBRP from UC that conduct much of the analysis. The **CHBRP staff** works with Task Force members in preparing parts of the analysis, and manages external communications, including those with the California Legislature. As required by CHBRP's authorizing legislation, UC contracts with an independent actuarial firm, **Milliman, Inc.**, to assist in assessing the financial impact of each legislative proposal mandating or repealing a health insurance benefit. The **National Advisory Council** provides expert reviews of draft analyses and offers general guidance on the program to CHBRP staff and the Faculty Task Force. Information on CHBRP's analysis methodology, authorizing statute, as well as all CHBRP reports and other publications, are available at chbrp.org.

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CHBRP assumes full responsibility for the report and the accuracy of its contents. All CHBRP bill analyses and other publications are available at chbrp.org.

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Please direct any questions concerning this document to: California Health Benefits Review Program, MC 3116, Berkeley, CA 94720-3116; info@chbrp.org; or chbrp.org.

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The California Health Benefits Review Program (CHBRP) was established in 2002. CHBRP's mission is to inform and support policymaking in California through the creation of impartial, evidence-based resources. As per its authorizing statute, CHBRP provides the California Legislature with independent analysis of the medical, financial, and public health impacts of proposed health insurance benefit-related legislation. CHBRP is dedicated to providing academic rigor on a Legislature's timeline.

The state funds CHBRP through an annual assessment on health plans and insurers in California.

An analytic staff based at the University of California, Berkeley, supports a task force of faculty and research staff from multiple University of California campuses to complete each CHBRP analysis. A strict conflict-of-interest policy ensures that the analyses are undertaken without bias. An independent actuarial firm helps to estimate the financial impact. Content experts with comprehensive subject-matter expertise are consulted to provide essential background and input on the analytic approach for each report.

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This analysis is based on existing literature and public sources identified through systematic search methods. This evidence informs the California Legislature about potential impacts of proposed health benefit legislation and does not constitute a policy recommendation from CHBRP.

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