Delaware Youth Rare Disease Initiative

RareDiseasesDE.org

Rare Diseases in Delaware:

An Overview

Delaware Youth

RARE DISEASE INITIATIVE

December 2025

A rare disease is defined as a condition affecting fewer than 200,000 people in the United States, but over 10,000 known disorders affect roughly 30 million Americans nationwide[1]--more than every American cancer patient or survivor combined[2]. Studies[3] place the total economic impact of these conditions at 1 trillion dollars per annum, yet ninety-five percent of these diseases lack an FDA-approved treatment.

Broad estimates suggest that nearly 10% of Americans live with rare diseases. Many of these patients face difficulties finding resources, obtaining diagnoses, and planning treatment due to a lack of infrastructure for their conditions.

Despite the severity of this issue, statistics do not exist to define the exact prevalence or socioeconomic impact of these disorders in Delaware. A lack of epidemiological knowledge prevents policymakers from taking effective, well-informed action to support their constituents.

Rare Diseases in Delaware: An Overview seeks to accurately portray current challenges, illustrate potential policy actions, and inform attempts to address issues encountered by affected individuals. While the authors recognize this challenge's difficult nature, they hope that their dedication contributes to an improved understanding of rare disease in their community.

- **Delaware Youth Rare Disease Initiative**

[1] (National Organization for Rare Disorders)
[2] The American Cancer Society suggests that 18 million Americans with a history of cancer were alive in 2022.
[3] (Yang 2020)

Defining Rare Disease

The National Organization of Rare Disorders defines rare diseases as conditions that affect less than 200,000 individuals in the United States. By this standard, the organization estimates that seven thousand rare diseases affect 25 to 30 million Americans across the nation. These conditions affect the body in a vast variety of ways, but patients' experiences share many similarities, including diagnostic difficulties, uncoordinated access to medical care, and barriers to effective treatment.

Delaware's Baseline Assessment

The NIH estimates that 350 million people suffer from rare disorders worldwide, including as many as 30 million in the United States alone (NIH). Other statistics suggest that they affect a lower range of 3.5% to 5.9% of humans (Rare Diseases International), but no reliable figure exists for rare disease prevalence in Delaware, and diagnostic barriers hinder the accurate collection of data.

Statistics from NORD indicate that 28% of affected patients receive incorrect diagnoses for more than seven years after symptom onset, part of a diagnostic odyssey responsible for considerable emotional and economic strain.

Diagnosis poses a formidable barrier to Delawareans with rare disease; the state ranks last in the U.S. for primary physician coverage (Becker's Hospital Review) per capita, straining healthcare systems statewide, and appropriate medical intervention poses an equally significant challenge.

The FDA has approved treatments for less than 5% of rare diseases (U.S. Government Accountability Office), and clinical expertise for these disorders is rare, especially in Delaware; NORD lists zero centers for rare disease expertise in the state, and there exists no statewide compendium of experts for these disorders.

Significantly, the state also lacks exact statistics to quantify rare disease incidence and prevalence within its borders; there are no published statistics for these conditions as a group,

and knowledge of temporal or geographic tendencies does not exist, preventing the effective allocation of resources.

Delaware lacks a system to identify and communicate directly with affected patients or families, and it lacks patient-driven data to understand the challenges faced by its citizens. The average time and barriers to diagnosis, transportation issues for patients, access to care, impacts on quality of life, economic hardships, and caregiver strain remain unmeasured in Delaware, precluding any attempt at facts-based, community-oriented action. Without statistics to quantify rare diseases' effects, addressing patients' struggles becomes an even greater challenge. The state also lacks reliable statistics to assess how rare disease patients receive at-home care, and no data exists to measure the toll on caregivers' mental, social, and economic wellbeing.

Rare disease patients face significant obstacles to care in Delaware, beginning with basic access to information. Due to the vast variety of relevant disorders, a site compiling Delaware-specific resources for rare disease patients, caregivers, and healthcare providers does not exist, a service that the Delaware Rare Diseases Youth Initiative hopes to provide with RareDiseasesDE.org. This gap in online information exacerbates existing concerns about barriers to diagnosis and treatment.

Healthcare Insurance

Access to healthcare through insurance is one of the most challenging issues for patients, as their medical costs may be five times higher than their unaffected peers (Tisdale et al.). Research suggests that rare diseases' total economic burden approached one trillion dollars in 2019 (Yang et al.), a number that has likely grown in the ensuing years.

Data suggest the following insurance breakdown for rare insurance patients nationwide in 2019: 7 million using commercial policies; 1.5 million on Medicaid; and almost 7 million on Medicare (Yang et al.).

Significantly, 24% of Delawareans rely on Medicaid for healthcare coverage (Biden School of Public Policy and Administration), and the state government plays a significant role in determining individual eligibility, treatment plans, and access to prescription drugs.

Delaware is one of 40 states that has expanded Medicaid access to incomes below 138% of the federal poverty line (Biden School of Public Policy and Administration); Medicaid expansion allows programs to spread the cost of high-cost prescriptions (including gene therapy and drugs for rare diseases) among a larger population of enrollees. Medicaid is especially important for rare disease patients because many conditions present challenges to steady employment.

Encouragingly, Delaware received an A for Medicaid eligibility on the National Organization of Rare Disorders's annual report card; however, it did receive a B for its policies on genetic testing for rare disease (NORD).

The state covers newborn genetic testing through its Newborn Screening Program, which facilitates early diagnoses that reduce overall care costs and mitigate negative outcomes. However, existing Delaware statutes do not include all conditions in the U.S. Department of Health and Human Services' recommended uniform screening panel (RUSP), including Krabbe disease, Hunter Syndrome, and guanidoacetate methyltransferase deficiency (Delaware Health and Social Services, "Delaware Newborn Screening"). Both Krabbe and Hunter are life-threatening illnesses that require early treatment for the best prognosis. Delaware has yet to pass legislation that aligns its screening with RUSP as recommended by the National Organization of Rare Disorders, and control over the screening program's scope lies with the Director of the Division of Public Health (16 Del. C. §.804); no specific criteria appears to exist for including specific panels in the program.

With more than 6% of live births affected daily by genetic disorders and congenital anomalies (End The Odyssey), many of these conditions would benefit from earlier detection through expanded newborn screening, which could significantly improve health outcomes, reduce long-term healthcare costs, and prevent avoidable morbidity and mortality.

Beyond newborns, Delaware Medicaid through MCOs requires prior-authorization for most other pediatric or adult genetic testing (Genetics Policy Hub). Pre-authorization processes can cause delays that frustrate diagnostic processes and delay essential symptom-alleviating care. Lengthy application processes, complex criteria, and problems with paperwork can result in claim denials for testing that families may be unable to pay for themselves. Analysis shows that Medicaid denies about 15% of genetic testing claims in some regions, while private insurance denies around 25% (Zion et al.), including patients that were eventually diagnosed.

Delaware's failure to require that all plans cover medically-necessary biomarker testing, an action recommended by the National Organization of Rare Disorders, aggravates these gaps in coverage for private insurance. Legislation to resolve this issue would encompass single-analyte tests, multi-plex panels, protein expression, whole exome, whole genome, and whole transcriptome sequencing for patients using evidence-based guidelines. Senate Bill 120, introduced in May of 2025, would effect this change and currently awaits consideration by the Banking, Business, Insurance, and Technology Committee. Though biomarker testing can provide conclusive diagnoses, insurance restrictions present barriers to coverage that lengthen costly diagnostic journeys, which already last seven years on average for rare conditions nationwide (NORD). Estimates suggest that delays to treatment may cost up to half a million dollars in medical costs and productivity loss for patients by the time they receive a diagnosis.

Though access to genetic testing remains an issue, Delaware has fortunately made some progress to address this issue in recent years. Senate Bill 70, signed in August of 2025, allows genetic counselors to order genetic testing, and the Ericka Byler act, signed in 2024, prevents life insurance from discriminating based on genomic information. Delaware's most recent Pre-Authorization Act amendment also created stricter standards for insurers reviewing treatment requests and patient appeals.

Cellular Gene Therapies

Besides gene testing, Delaware Medicaid faces the challenge of providing access to revolutionary cellular gene therapy (CGT) through its current system. Though these one-time treatments reduce lifetime insurance expenditures and produce better health outcomes, their high

up-front costs pose challenges to existing systems. The FDA has currently approved more than 40 CGTs as of December 2025 (FDA), with more than 100 in developmental pipelines; also significant are hundreds of high-cost “orphan drugs” for rare disease.

The majority of FDA-approved drugs are covered through Medicaid in exchange for manufacturing rebates, though individual states determine how inpatient and outpatient drugs are administered.

By law, Medicaid must cover all FDA approved drugs included in its drug rebate program. These drugs--including Lenmeldy, retailed at 4.25 million--have the potential to place significant strains on state Medicaid programs in the future, especially those with managed care organizations (like Delaware). Under the Diamond State Health Plan, Delaware’s three MCOs--Highmark Health Options, AmeriHealth Caritas, and Delaware First Health--serve almost all enrollees, with small fee for service exceptions. The state pays these organizations per beneficiary and promotes high quality of care with penalties for failing to meet certain benchmarks. MCOs must meet standards in 7 general health attributes while dedicating a minimum proportion of their budgets to value-based agreements with providers (Medicaid).

Under this agreement, cellular gene therapy and specialty drugs can challenge budgets due to high pharmaceutical costs--CGT is expected to incur 20 billion in annual national spending by 2034 (Cervicorn Consulting). Delaware has not adopted a risk corridor to mitigate costs, but the state is a member of the Sovereign States Drug Consortium pool (Sovereign States Drug Consortium), which negotiates supplemental rebates with drug manufacturers. The Centers for Medicare and Medicaid Services (CMS) authorized Delaware to enter value-based purchasing agreements with individual manufacturers on a discretionary basis starting in 2024. These contracts protect MCOs from risk by tying pharmaceutical payments to treatment success.

Delaware Medicaid reimburses providers for pharmaceuticals (including CGT) under its Medicaid State Attachment Plan 4.19-B, using the lower of several measures like national average drug acquisition cost (NADAC), Delaware maximum allowable cost (DMAC), and wholesale acquisition cost (WAC).

Delaware has also entered the federal government's Cell and Gene therapy access model, which brokered outcomes-based purchasing agreements for the hemophilia therapies Casgevy and Lyfgenia. Under this model, the federal government negotiates contracts with manufacturers in lieu of individual states, lowering costs and creating a more cohesive nationwide model through carve-outs (which send the responsibility for drug payments to states instead of MCOs).

As of July 1st, 2025, Delaware reported that it had carved both Casgevy and Lyfenia out from MCO agreements (Hinton et al.), transferring responsibility for payment from MCOs directly to the state; the state reported no carve-outs for any other treatments. However, the state recently approved a mechanism to carve out "high-investment medications" under its 4.19B Medicaid attachment, though the criteria for inclusion remains unclear; the authors were unable to access the relevant list of drugs using the link provided by the state HHS (Delaware Medical Assistance Portal for Providers). Studies associate carve-out policies with a 54% higher usage of CGTs without increasing net Medicaid spending per-capita for MCO systems (Odouard et al.). Furthermore, claims for newer CGTs and specialist drugs can cause significant budgetary stress for MCOs compensated using older pricing logic. As this issue evolves, creating systems to designate pharmaceuticals as high-investment drugs would facilitate access to life-changing treatment and create a more sustainable economic model for state MCOs.

The Delaware Preferred Drug List & Prior Authorization

Delaware controls Medicaid drug administration through its Preferred Drug List (PDL), a list of curated treatments which its Pharmaceutical and Therapeutics committee edits four times a year. Delaware's three MCOs--AmeriHealth Caritas, Hallmark Health Options, and Delaware First Health--all use this list as a basis for their drug formularies, which list the prescriptions covered by insurance under specific usage criteria. Hallmark Health Options and Delaware First Health both require patients to submit prior authorization requests for items omitted from their formularies, which MCOs must rule on within two calendar days (18 Del. C. §.3373). This complex process requires patients to demonstrate medical necessity for non-preferred or expensive drugs and procedures before they receive treatment.

The state Pharmaceutical and Therapeutics Committee selects drugs for the Delaware PDL, while the Drug Utilization Review board creates prior authorization requirements applicable to a minority of fee for service (FFS) beneficiaries.

All three MCO formularies in Delaware have adopted the state PDL and are barred from denying treatment with requirements more restrictive than those outlined by the DUR board. Notably, the drug utilization review board has only set prior authorization criteria for three CGTs, (Zolgensma, Casgevy, and Lyfgenia), leaving the rest to MCO discretion. Hallmark Health Options provides prior authorization criteria for about a dozen therapies on its website (Medication Information). Delaware First Health's website includes one gene therapy (Adstiladrin) on its formulary, and AmeriHealth Caritas lists specific criteria to qualify for sickle cell, hepatitis B, and RBC transfusion-dependent beta-thassalemia treatment. Explicit, easily-accessible prior authorization requirements facilitate responsible access to CGT coverage while maintaining transparency for appeals.

However, MCO approval criteria for CGTs are sometimes more restrictive than their FDA label requirements. Updating the Delaware PDL for gene therapies in a timely manner sets statewide standards for MCOs and allows community input through P&T and DUR meetings that are open to the public. Frequent PDL updates for CGTs also ensures that authorization criteria take effect before the first claims for novel treatments, ensuring that decisions are made with clear, publicly available standards based on best medical practice. Meetings for the P&T committee are open to the public, with agendas and details available on the state medical assistance document repository.

Non-Medicaid Coverage

Beyond Medicaid, Delaware's Group Health Insurance Plan for state employees, retirees, and dependents covers treatment for select gene therapies through Aetna and Highmark, affecting more than 100,000 citizens statewide (WTW). GHIP members can receive additional specialty medications with no co-pays if they participate in CVS Caremark's PrudentRx solution (Delaware Department of Human Resources), but those who do not opt in are charged a 30% coinsurance fee.

Otherwise, the 41% of Delawareans insured through private employers or unions (Delaware Health and Social Services) must check their plans to see what they cover. These individual policies contain significant differences, and further data collection remains necessary to determine the extent of coverage for specialty drugs and gene therapy in Delaware. Most private insurance plans cover "medically necessary" action, but cellular gene therapies represent a novel challenge to existing paradigms for treatment.

The recently-passed Prior Authorization Reform Act helped democratize access to treatment for both public and private insurance by clarifying the process for treatment authorization: it implemented stricter timeframes for rulings, qualification requirements for reviewers, and an electronic system to mediate requests. However, several changes have the potential to further increase transparency in Delaware. Some states have adopted requirements to disclose AI usage by review boards. Others have guaranteed patients continued care for a set period of time after changing insurers without requiring an updated prior-authorization (Sullivan et al.). While Delaware requires carriers to submit claims and appeals data to a statewide database, some states also require insurers to report significant increases in denials for specific services, including gene therapies, which ensures oversight for rare disease patients (Sullivan et al.). Concerningly, fair hearings for Medicaid appeals through the Delaware Department of Health and Social Services do not require the presence of a qualified medical expert, though hearing officers may request their input on a discretionary basis (title 16 of the Delaware administrative code). The prior authorization appeal system's complex nature places significant burdens on healthcare stakeholders and creates administrative hurdles that delay life-saving treatment, necessitating transparent, timely, and responsible prior authorization reform for the rare disease population.

Though the summary above provides an overview of Delaware's rare disease landscape, further data collection and patient testimony remains necessary to ascertain the current situation and policy impacts on providers, insurers, and patients.

As of now, Delaware's greatest challenge with regard to rare disease is a lack of consolidation and data. The state does not have a coordinated system for determining rare disease patients'

needs, and policymakers lack crucial information about the prevalence and incidence of these illnesses and their fiscal impact. Concerningly, there is no central information source for those affected by rare disorders in Delaware, and there is no central compendium of specialist providers, researchers, or clinical trials in the state, reflecting a lack of resources to promote rare disease expertise within Delaware's borders. The prevalence and life-changing impact of these conditions demands that insurers, providers, and legislators take immediate action.

Current Action

As many as 30 million Americans, or almost 10% of the nation's population, live with a rare disease, although accurate statistics do not exist for these conditions in Delaware.

While addressing rare diseases in the Diamond State presents a challenging issue, the state possesses several resources to provide expertise in relevant fields.

NORD does not recognize any clinical centers for excellence in Delaware, but several facilities have the experience or capabilities to treat rare disorders. Christiana Care recently announced an initiative to treat rare pediatric conditions through the Children's Hospital of Pennsylvania, and Aetna recognizes Nemours Children's Hospital as a center for three gene therapies. The state also hosts significant biomedical research capabilities through the Delaware Network for Biomedical Research Excellence, the University of Delaware, and organizations like Nemours' Children Health, which is currently conducting several clinical studies for rare pediatric illnesses.

From a legislative standpoint, Senate Bill 55 from the 152nd General Assembly recently established Delaware's Rare Disease Advisory Council, creating a central authority for these conditions statewide. The council's responsibilities include convening public hearings, producing policy recommendations, disseminating relevant resources, and identifying best practices for research, diagnosis, and treatment of rare diseases in Delaware.

The RDAC is also tasked with establishing best protocols for rare disease patients, creating a survey of patient needs, and producing its first annual report by 2026. These important

responsibilities highlight the council's role as a central stakeholder in Delaware's rare disease landscape.

Beyond the RDAC, Delaware has also passed significant changes to improve insurance coverage for patients and offset financial risks. These actions include the Pre Authorization Reform Act, value-based purchasing agreements, and carve-outs for cellular gene therapies. The state legislature recently named February 28th as "Rare Disease Day" in Delaware.

Future Perspectives and Action

Addressing rare disease in Delaware will require coordinated, data-driven action through sustained efforts by government, advocacy organizations, and affected communities. Recent progress demonstrates meaningful gains, but the current landscape contains critical gaps that must be addressed to ensure equitable treatment for patients.

First, the state must endeavor to collect high-quality data from patients, providers, and insurance to clearly evaluate rare disease issues in Delaware. Although each of the approximately 7000 rare diseases is relatively uncommon, as a group they affect 7 to 8 percent of the population. There are no reliable statistics to determine rare diseases' impact on patient, insurance, and healthcare systems in Delaware beyond what can be inferred through reasonable estimation. To address this issue, policy and healthcare stakeholders should develop an epidemiological structure to assess disease incidence and its economic cost in Delaware. Such a program's success would be measured against accuracy, sustained participation, and fiscal sustainability.

Delaware must also develop a framework to identify rare disease patients' needs with a focus on establishing priorities. Many common areas of concern are well documented and known, but it is critical to evaluate patient input and avoid making undue assumptions.

Affected populations with rare conditions face long delays to diagnostic attention, and fragmented or confusing information contributes to emotional, physical, and economic distress. Prohibitive treatment and medication costs, compounded by geographic barriers to specialized care, lead to difficulties for many patients and caregivers along with complex insurance

processes. These problems' acute nature and the long-term prognosis for care necessitate an accurate assessment of informational, physical, financial, psychological, and practical population needs.

Both Pennsylvania and New Jersey have pursued this goal through online surveys, producing valuable data for consideration by policymakers and legislators. Though these efforts may raise privacy concerns, voluntary participation can circumvent such issues along with proper institutional review and HIPAA-compliant survey platforms (like Survey Monkey). Strategic social media marketing, stakeholder partnerships, and support from legislators, advocacy groups, and the media can aid effective data collection.

Pursuant to this goal, the Delaware Youth Rare Disease Initiative is developing a preliminary survey for online distribution (available upon completion on its website). Although organizational infrastructure exists to facilitate survey completion, the most significant barriers include marketing, requirements for institutional review, and the availability of personnel to develop the survey and compile information. The Delaware Youth Rare Disease Initiative hopes to circumvent these barriers by collaborating with groups like the Rare Disease Advisory Council to benefit from existing organizational infrastructure along with proper institutional review.

Beyond data collection, Delaware must continue reviewing its insurance landscape to ensure financial stability and equitable access to innovative therapies. The state has made substantial progress toward this goal through mechanisms like value-based agreements, Medicaid carve-outs, and prior authorization reform statewide. These advancements, among many others, create a positive outlook for the continued innovation that is necessary in this field.

However, several legislative and regulatory improvements remain to be made.

Currently, Delaware's Newborn Genetic Screening Panel does not contain three disorders in the National Department of Health and Human Services' recommended universal screening program (RUSP) that cause disability or death without early detection. Legislation aligning Delaware's

program with the RUSP, as passed in 13 states, would cover these conditions and allow the state to take advantage of existing medical expertise without excessive burdens on government officials. At minimum, adding Krabbe disease, Hunter Syndrome, and guanidoacetate methyltransferase deficiency to the panel appears to be an appropriate course of action.

The Delaware Division of Medicaid and Medical Assistance should also provide clear criteria for the "high-investment medications" that it carves out from MCO payment using Medicaid State Plan Attachment 4.19B for the federal CGT program. While the regulation specifies that high investment medications are listed on the state website, the authors failed to find the relevant document using the link provided, and a search returned no results. Introducing a specific mechanism to designate treatments under this category would provide clarity and flexibility to control pharmaceutical payments through Medicaid. Studies suggest that including gene therapies in carveouts significantly increases access to treatment.

In alignment with the state's most recent Pre-Authorization Act Amendment, legislators should consider further prior authorization reform to increase accountability and transparency for those affected by rare disease. Further legislation could include mandatory disclaimers for AI-influenced coverage decisions, continuity of care guarantees for patients changing carriers, and mandatory reports to the state when denials rise significantly for existing treatments or novel pharmaceuticals.

To ensure unbiased Medicaid access to lifesaving therapy, the state's P&T committee, drug utilization review board, and contracted MCOs should create prior authorization criteria for gene therapies on a more timely basis. The FDA has approved more than 40 cellular gene therapies to date, but the state's PDL currently lists only three. The establishment of CGT authorization standards before patient filings ensures that coverage decisions are made using clear, publically available standards based on best medical practice, and with more than a hundred gene therapies in development, publically transparent action by the state P&T committee and drug utilization review board remains paramount. Specific prior-authorization criteria on the state PDL limits the restrictions that MCOs can place on treatments for Medicaid participants.

The state should also consider passing Senate Bill 120, introduced in May of 2025, to require that insurers cover medically-necessary biomarker tests. These panels, which include genetic and metabolic testing, reduce diagnostic difficulties for rare disease patients and provide guidance for effective treatment. Similar legislation has been introduced in several other states with the support of advocacy groups like the National Organization of Rare Disorders.

Finally, rare disease stakeholders should strive to increase informational access in Delaware through educational programs and resource gathering. The state lacks a central website for affected populations, and fragmented resources exacerbate a growing gap in rare disease expertise. In order to address this issue, the Delaware Youth Rare Disease Initiative has created and maintains its web page as a central hub for relevant information, including the content contained in this report.

Conclusion

Rare diseases affect tens of thousands of Delawareans, and the issues faced by patients demand action from healthcare stakeholders throughout the state. While significant work remains to produce measurable outcomes, recent legislative changes, Medicaid reforms, and developmental treatments offer a promising outlook for improved access to care and an equitable healthcare model in Delaware.

Pursuant to its mission of raising rare disease awareness and creating meaningful change, the Delaware Youth Rare Disease Initiative hopes to advance five main goals in the 2026 calendar year.

First, it will perform in-person outreach and provide patient and family resources through its website to address information gaps, increase quality of life, and empower affected populations statewide. The Initiative will contact advocacy organizations and rare disease patients to produce multi-media material highlighting rare conditions' impact on Delaware communities. The Initiative will also conduct research and contact experts to create prior-authorization resources for rare-disease patients.

Second, it will seek to partner with groups such as the Delaware Rare Disease Advisory Council to develop and assist with a statewide survey to assess patient needs, set policy priorities, and clarify the rare disease landscape in Delaware.

Third, the DYRI will advocate for evidence-based legislation and regulatory action by contacting legislators and government officials. Biomarker insurance coverage, RUSP newborn screening alignment, further prior authorization reform, and clearer criteria to initiate Medicaid pharmaceutical carveouts are among the Initiative's key commitments, which it will continue to develop through cooperation with local and national advocacy groups.

As part of its advocacy, the Initiative will also petition the state P&T board and other relevant authorities to review prior-authorization criteria for novel gene therapies on a more timely basis.

Finally, the Initiative will raise awareness about rare disease and create opportunities for youth to engage, learn, and contribute meaningfully to this issue in partnership with adult experts and established organizations. Estimates suggest that 75% of rare disease cases manifest in children nationwide, and it is appropriate that youth take interest in issues that disproportionately affect their peers. While the Delaware Youth Rare Disease Initiative recognizes the difficult nature of addressing this challenge, it hopes that its members' time, energy, and dedication will result in a stronger, more informed, and more equitable healthcare system for individuals and families living with rare diseases in Delaware. The undeniable severity and impact of these conditions demand increased awareness and actionable change starting now and into the future..

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