



Rare Endocrine
Disorders

KNOW YOUR RIGHTS

*A resource for patients with
Rare Endocrine Disorders.*

 **AIMED ALLIANCE**

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DIAGNOSIS & TREATMENT

An endocrine disorder is a condition that affects the endocrine system, which is a network of glands that produce and release various hormones. Hormones regulate many important bodily functions, including metabolism (digestion, blood circulation, body temperature), sexual function, reproduction, and mood.¹

An individual can develop an endocrine disorder when their endocrine glands produce higher or lower than average levels of hormones or when their endocrine glands respond incorrectly to certain hormones.² Endocrine disorders can be caused by a genetic disorder, a disease, an injury to an endocrine gland, or as a result of nodules or tumors in the glands.³ Some rare endocrine disorders can also be caused as a result of a surgical procedure. For instance, hypoparathyroidism, a condition that causes lower blood calcium levels, high phosphate levels, and low parathyroid hormones, can be caused by an injury to the parathyroid gland during surgery or when the parathyroid gland requires removal.⁴

Some endocrine conditions, like Cushing's syndrome and familial chylomicronemia syndrome (FCS), affect fewer than 200,000

people each year.⁵ In the United States, diseases or conditions that affect fewer than 200,000 people are considered "rare diseases" under a federal law known as the Orphan Drug Act.⁶ There are a variety of rare endocrine disorders. Symptoms of rare endocrine disorders vary widely depending on the specific gland that is impacted by the disorder. Symptoms can include fatigue and weakness, mood swings, unintended weight fluctuations, irregular growth patterns, or changes in blood glucose or cholesterol levels.⁷

If you are experiencing symptoms of a rare endocrine disorder, you should discuss your symptoms with your health care provider. To help determine if you have a rare endocrine disorder, health care providers may use blood and urine tests to check hormone levels. Health care providers can also use imaging tests to determine if a gland has a nodule or tumor.⁸ Treatment of endocrine disorders can be complicated, as a change in one hormone level can cause an imbalance in another.⁹ Treatment options vary among disorders. In addition, some rare endocrine disorders may require surgery, or long-term treatments to manage symptoms.



BARRIERS TO CARE

According to the National Institutes of Health, there are approximately 7,000 rare diseases.¹⁰ Rare diseases affect one in ten Americans. While each person's experiences are unique, a 2019 survey by the National Organization for Rare Disorders (NORD) identified commonalities among individuals with rare diseases, including challenges with a timely and accurate diagnosis, and various other barriers to care.¹¹

More than a quarter of the NORD survey respondents reported waiting seven or more years before receiving a diagnosis, and nearly 40 percent received a misdiagnosis during their journey. Diagnostic challenges were attributed to, among other things, lack of disease awareness, limited medical specialization, long wait times for specialists, insufficient testing, diagnostic complexity due to shared symptoms with other conditions, and mischaracterizing symptoms in pediatric patients as developmental delays. Delays in diagnosis can have devastating impacts on individuals, including disease progression and mental, emotional, and financial stress.¹²

Additionally, many patients experience geographic barriers to care. Individuals with rare conditions often receive care from one or more specialists. Yet, for many rare diseases, there are a lack of specialists available. As a result, many NORD survey respondents reported having to frequently travel long distances to see a specialist, and some even permanently relocated to be closer to their specialists.¹³

Individuals with rare disorders also experience barriers to care due to limited treatment. The federal Orphan Drug Act, which created various incentives for drugmakers to invest in research and development for rare disease treatments, has helped accelerate the development of such treatments over the past 40 years.¹⁴ Only a fraction of known rare diseases currently have

FDA-approved treatments in the U.S., thus there continues to be a need for more research on rare diseases. As a result, many patients rely on "off-label" treatments, which are FDA-approved treatments that are not indicated for a patient's specific rare disorder.¹⁵

As explained in more detail below, insurers also create barriers to care. For example, 60 percent of NORD survey respondents reported being denied coverage or experiencing delays in accessing treatments that required insurer pre-approval, including FDA-approved treatments specifically indicated for their disorders. Nearly 20 percent of respondents were denied a referral to a specialist. Further, even when insurers do cover an FDA-approved medication for a rare disease, insurers often categorize the medication as a specialty drug and place it in a drug plan's highest cost-sharing tier, resulting in patients being forced to pay a high-cost sharing to access their treatment.¹⁶

These diagnostic challenges and other barriers to care create financial strain for many patients with rare endocrine disorders. Additionally, patients can experience stress from having to navigate complex insurance policies while continuing to learn how to manage their conditions.¹⁷ These challenges may be exacerbated by socioeconomic disparities in income, education, geography, and other measures that may influence access to care.¹⁸ For example, compared to non-Hispanic white patients, non-Hispanic Black patients have worse health outcomes and higher mortality rates from certain endocrine disorders despite having lower or similar incidence rates of these disorders.¹⁹ Differences in the incidence, prevalence, and mortality of these disorders among specific population groups emphasize the need for increased quality and access to care, and comprehensive, tailored approaches to addressing rare endocrine disorders.²⁰



Insurers use a variety of practices that may delay or deny access to your treatment for a rare endocrine disorder. To find out whether your insurer may have improperly delayed or denied access to your treatment, ask yourself the following questions:



STEP THERAPY

Did my insurer make me try a different treatment before covering the rare endocrine disorder medication or therapy that my care team originally prescribed?

This practice is called “step therapy” or “fail first” because it requires patients to try other treatments first and demonstrate that they do not work or are intolerable before the health plan will cover the originally prescribed treatment. Your insurer may require you to try and fail on multiple different treatments before covering the one prescribed by your care team.²¹ This can lead to delays in access to medically necessary treatment, which in turn, can result in disease progression. Step therapy policies may be against federal or state laws if your insurer treats you and others with similar diagnoses differently because of your health condition. In addition, some states have passed legislation to limit the type of step-therapy protocols that can be used. For instance, some state laws prohibit trying and failing on more than one treatment or prohibit failing on a treatment you have already tried and found ineffective. Additionally, you may also be entitled to an exception from the step therapy process.



NON-MEDICAL SWITCHING

Is my insurer forcing me to take a different medication, even though my current treatment is working, by refusing to cover it any longer or increasing my co-pay?

This practice is referred to as “nonmedical switching.” It occurs when your insurer (not your health care professional or pharmacist) forces you to switch from your current rare endocrine disorder treatment to a different (but not a generic equivalent) treatment by either refusing to cover your drug therapy any longer or increasing the out-of-pocket cost of your treatment.²² Non-medical switching can result in relapse and disease progression. One study found that when patients were non-medically switched, over 75 percent of them experienced side effects as a result of the switch.²³ In addition, nonmedical switching may violate certain states’ consumer protection laws.²⁴





PRIOR AUTHORIZATION

Do I need to get my insurer's approval before I can begin or continue my prescribed rare endocrine disorder treatment or therapy?

This practice is called "prior authorization." It happens when your insurer requires you or your doctor to get your insurer's approval before the treatment or therapy is covered. Approval is based on the insurer's standards, which may be inconsistent with medical standards of care and harmful to patients. For example, one survey found that 87 percent of physicians reported prior authorization policies had a negative impact on clinical outcomes.²⁵ For those living with a rare endocrine disorder, the results can be devastating. These policies can also violate state and federal laws if applied in a certain manner.



ADVERSE TIERING

Do I have to pay a high copay for certain medications that treat my condition?

This practice is called "adverse tiering." It can be used by insurers to shift much of the cost for newer or innovative therapies to patients by placing expensive drugs on what are called "specialty tiers". Certain tiering policies may also violate certain federal and state laws if used in a discriminatory way.²⁶



COPAYMENT ACCUMULATORS

If I receive coupons or discounts to help pay for my medication copays, does my insurer prohibit those coupons or discounts from counting toward my annual deductible?

This policy is known as a "copayment accumulator." Copayment accumulators force patients to pay more out-of-pocket when copayment assistance runs out and the insurance deductible has not been met. These policies are frequently buried in the fine print of insurance contracts and may violate state consumer protection laws.²⁷

My insurer refuses to cover a rare endocrine disorder treatment or therapy that my health care professional prescribed to me. **What can I do?**

If your insurer refuses to cover your treatment, here are three steps you can take to try to change your insurer's decision:

- Appeal the decision;
- Request an external review; or
- File a consumer complaint.



How do I appeal the decision?

If your insurer denies your claim, you have the legal right to an internal appeal.²⁸ This means you can ask your insurer to conduct a full and fair review of its decision. To appeal the denial, you should do the following:



Review the determination letter. Your insurer should have sent you a determination letter to tell you that it would not cover your claim. Review this document so you can understand why your insurer denied your claim and how you can appeal the denial.



Collect information. Collect the determination letter and all other documents the insurance company sent you. This includes your insurance policy and your insurer's medical necessity criteria. "Medical necessity criteria" refers to your insurer's policy for determining whether a treatment or service is necessary for your condition.²⁹



Request documents. If you did not receive the determination letter or do not have your policy information, the medical necessity criteria, or the instructions and forms for filing an appeal, call the insurer's customer service representative and ask for these documents. The company website will list the toll-free telephone number to call.



Call your health care professional's office.

The health care professional's office or clinic has people on staff to help with the appeal process. They will tell you how to fill out the forms to request an appeal, write an appeal letter on your behalf, or handle the appeal request for you.



Submit the appeal request. It is important for you or your health care professional's office to submit the appeal request as soon as possible along with the letter from the health care professional and all additional information the insurer requested.



Once you file an appeal request, expect to wait up to 30 days to hear back from the insurance company regarding a treatment you hope to receive.

It can take up to 60 days for a response if you received the treatment and are waiting for reimbursement.



Follow up. Follow up with your insurer regularly until you hear back. Be sure to keep a record of the name of any representative you speak with about the appeal, the date and time you spoke with that person, a confirmation number for the call, and a summary of your discussion.



What if my treatment has recently been approved by the Food and Drug Administration (FDA) and my insurer has denied coverage because it considers the treatment “experimental”?

You should ask your insurer for a written statement of the exact reasons for the denial (i.e., why the treatment is considered experimental), if that information has not already been provided. Review your plan documents carefully to see how the plan defines “experimental” (or “investigational” or “unproven”) treatment. These terms can vary from plan to plan.

In general, an insurer may deem a treatment experimental if it determines that the treatment is not the standard of care for a particular condition. Experimental treatments may include non-FDA-approved treatments, those that lack substantial evidence to support their medical effectiveness, and off-label treatments. The FDA will only approve a treatment if there is evidence that the treatment is safe and effective. Therefore, if your treatment has recently been approved by the FDA and is indicated for your condition, then you may be able to appeal the denial on the basis that it is no longer “experimental.”

If you are being prescribed an FDA-approved treatment off label (i.e., it is not indicated for your condition), review your plan documents closely. Some plans have exceptions for coverage of FDA-approved off-label treatments for rare disorders if certain conditions are met.

What if my insurer denies my appeal?

Under law, you are entitled to take your appeal to an independent third party for an “external review,” which means the insurance company no longer gets the final say over whether to approve a treatment or pay a claim. The situation applies if the insurer denies your appeal or if your medical situation is urgent and waiting would jeopardize your life or ability to function.

How do I request an external review?

To trigger an external review, file a written request with the independent organization within 60 days of the date your insurer sent you a final decision. The process should take no more than 60 days. However, in urgent situations requiring an expedited review, the process should take no longer than four business days. To find out whom to contact in your state to request an external review, please go to www.CoverageRights.org.



How do I file a complaint?

If your insurer denies your coverage after the external review process, you can file a complaint with the insurance commissioner or attorney general in your state. To determine whom to contact and how to submit the complaint, please go to www.CoverageRights.org

Your complaint should include the following information:

- The name, address, email address, and telephone number of the person filing the complaint (“Complainant”);
- The name of the insured individual, if different from the Complainant;
- The names of any other parties involved in the claim (for example, the plan administrator or pharmacy benefit manager);
- The name of the insurance company and the type of insurance;
- The state where the insurance plan was purchased;
- Claim information, including the policy number, certificate number, claim number, dates of denial, and amount in dispute;
- The reason for and details of the complaint; and
- What you consider to be a fair resolution.

You should also submit the following supporting documents with your complaint:

- A copy of your insurance card;
- Copies of coverage denials or adverse benefit determinations from your insurer;

- Copies of any determinations made by internal and external reviewers;
- Any materials submitted with prior appeals and complaints;
- Supporting documentation from your health care professional;
- A copy of your insurance policy; and
- All responses from your insurer.

What happens after the insurance commissioner or attorney general receives my complaint?

The insurance commissioner or attorney general will assign someone to research, investigate, and resolve your complaint. That person will examine your account, records, documents, and transactions. He or she may question witnesses, request additional documents from other parties, and hold a hearing. If the insurance commissioner or attorney general determines that the insurer violated laws or regulations, he or she may order the insurer to give you the requested coverage or compensate you.

Whom should I call if I have any questions about filing a complaint?

To determine whom to call in your state, please go to www.CoverageRights.org.



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1455 Pennsylvania Avenue NW, Suite 400 • Washington, DC 20004
202-349-4089 • AimedAlliance.org

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