

Did you know?

# RARE DISEASE MEDICATION FAQs:

As programs advance through the Friedreich's Ataxia Treatment Pipeline, FARA has been researching how other rare disease patients access their approved medications.

During this research, we learned some new vocabulary along with processes and programs. Of note, it is common for pharmaceutical companies to employ case managers who assist physicians and patients in navigating this process.

We would like to share some of our learnings with the community in this Frequently Asked Questions (FAQ) document.

1

## If the Food and Drug Administration (FDA) approves a treatment, is it approved worldwide?

The FDA makes decisions about new medications that are prescribed in the United States. Other countries have their own regulatory agencies that make decisions on products for their countries.

2

## When a new medication or device is approved, is it approved for specific conditions or are there limitations or restrictions on the approval?

Based on the clinical trial, the FDA works with the company to decide to whom and how the medication should be prescribed. The approved label on the medicine contains this important information.

3

## What is the label for a medication and why is it important?

The label for a medication is determined by the FDA as part of the approval process and is used by prescribing physicians and insurers. The label typically contains all of the information about that medicine including [1]:

- Who is eligible to be prescribed the medicine (ie individuals with a specific condition or diagnosis, age).
- The product's active ingredients, including the amount in each dosage unit.
- The specific indications (or symptoms) the medication is intended to treat.
- Specific warnings, including when the medication should not be used, and when it is appropriate to consult with a doctor or pharmacist.
- Possible side effects and substances or activities to avoid.
- Dosage instructions - when, how, and how often to take the medicine.
- The product's inactive ingredients, important information to avoid ingredients that may cause an allergic reaction. [2]

[1]: <https://www.ecfr.gov/current/title-21/chapter-I/subchapter-C/part-201/subpart-B/section-201.56>

[2]: OTC Drug Facts Label | FDA

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4

## What is a formulary and how are new medications added to it?

A formulary is a list of medications that are covered by a particular insurance plan, including Medicare Part D plans. Although the process for adding new medications to a formulary can vary, the following steps are typically involved:

- The manufacturer submits a request to have the new medication added to the formulary.
- The insurance company reviews the request and considers factors such as the medication's effectiveness, safety, and price.
- If the insurance company decides to cover the medication, it may negotiate with the manufacturer to get a lower price.
- The insurance plan updates its formulary to include the new medication.

It is important to note that not all new medications will be added to a formulary. Some insurance plans may decide not to cover a particular medication if they do not believe it is cost-effective or if there are other medications that are similarly effective but less expensive.

5

## What is Medicaid's process for covering new medications?

The process for adding new medications to Medicaid formularies can vary by state, as Medicaid is a joint federal and state program. In general, however, the process is similar to the steps for private insurance companies that is outlined above.

6

## Are medications for rare diseases available at local pharmacies?

Medications that are only approved for a rare disease and not for other more common conditions are not typically available through a local pharmacy. Most rare disease medications are accessed through a specialty pharmacy.

7

## What is a specialty pharmacy?

A specialty pharmacy is not defined by location but rather by the type of service provided. The medications to treat many rare diseases are dispensed through specialty pharmacies. They are trained to deliver medicines that might require special handling or storage and can deliver to a patient's door. In addition, a specialty pharmacy might offer access to disease management services, education, or other support resources relevant to managing the specific rare disease or condition.

An insurance plan may specify which specialty pharmacy must be used. If that specific specialty pharmacy is not dispensing the medication, the patient or prescriber may need to request a pharmacy override to dispense through the "non-preferred" pharmacy.

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8

## What is a Prior Authorization (PA)?

A prior authorization may be required before insurance will cover the cost of a patient's prescription, even if the medication is on the formulary. The process for obtaining prior authorization can vary depending on the insurance plan, but generally it is facilitated by a third-party Pharmacy Benefits Manager (PBM) and the following steps are involved:

1. The healthcare provider writes a prescription for the medication.
2. The patient, physician, or pharmacy submits a request for prior authorization to the insurance plan.
3. The insurance plan reviews the request and decides if they will pay for the medication.
  - a. The decision is based on factors such as the patient's medical history, the medication's effectiveness, and the availability of other treatment options.
  - b. Additional documentation (for example, the genetic diagnosis report for the rare disease) may be required

If the insurance plan approves the request, the patient can fill the prescription and the insurance plan will cover the cost of the medication. If the request is denied, the patient may have to pay for the medication out-of-pocket or try to appeal the decision. It is important that patients understand their insurance company's prior authorization process and follow the necessary steps to get coverage for the medication.

9

## How does someone with a rare disease typically gain access to an approved medicine?

This is often a multi-step process with varying time frames and success rates, from submission of the initial form to medicine delivery at home. Steps might include:

1. Patient makes an appointment with a physician who is knowledgeable about prescribing the medication.
2. Additional tests or exams may be required prior to prescribing, starting, or refilling the medication.
3. If required by insurance, a Prior Authorization is obtained. The PA is submitted to the insurance company to verify coverage and out-of-pocket costs.
4. Once insurance coverage has been established, the prescription can be submitted to a specialty pharmacy to dispense the medicine.
5. The medicine is then shipped or delivered directly to the patient's home.
6. To obtain refills of the prescription, these steps may need to be repeated.

It is common for the pharmaceutical company to create a branded program with access to case managers to assist physicians and patients in navigating this process.

10

## What if someone doesn't have insurance, their insurance denies coverage, or they can't afford their rare disease medication?

Patient assistance programs (PAPs) help people afford their medications. PAPs are for people who don't have health insurance and those who are underinsured. They are managed by pharmaceutical companies, nonprofits, and government agencies, and they may cover the full cost of medications or provide a discount.

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## AFTER THE FDA APPROVES A MEDICATION, WHAT HAPPENS NEXT?



### PHARMACEUTICAL MANUFACTURING

During the FDA approval process, the FDA inspects the manufacturing facility where the medication will be made. After approval, the pharmaceutical company begins manufacturing the medication in commercial quantities.



### INSURANCE FORMULARY

The medication manufacturer requests to have the new medication added to the formulary of each insurance plan, including Medicaid and Medicare Part D. If approved, each insurance plan updates their formulary to include the new medication.



### PHYSICIAN PRESCRIPTION / PRIOR AUTHORIZATION

Knowledgeable physicians prescribe the medication. The patient verifies if their insurance requires a prior authorization and works with the physician and pharmacy to provide any information that is required. If patients don't have insurance or are under-insured, there may be Patient Assistance Programs (PAPs) to help patients afford their medication.



### SPECIALTY PHARMACY

The specialty pharmacy - which is usually selected by the insurance plan - dispenses the medication directly to the patient.



### FOLLOW-UP

Patients visit their physician regularly to monitor for side effects. Refills of the prescription may be dependent on monitoring, and prior authorizations may need to be reviewed and renewed regularly.



### PROCESS

This is a multi-step process, with varying time frames and success rates, from submission of the initial form to medicine delivery at home.