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# Understanding Early Access Programs (EAPs)

## A Guide for the Duchenne Muscular Dystrophy (DMD) Community

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DMD Pathways

Navigating Duchenne Together

www.dmd-pathways.com

# Introduction

**Receiving a diagnosis such as Duchenne Muscular Dystrophy (DMD) can feel overwhelming. Families often learn about standard treatments and may hear about clinical trials, but there are additional pathways that are not always clearly explained.**

One of these pathways is Early Access Programs (EAPs) — a way for patients to access promising medicines before they receive full approval.

This guide aims to help you:

- Understand what early access is
- Learn how EAPs differ from clinical trials
- Explore when early access may be relevant
- Prepare questions to ask your healthcare team
- Feel more confident and informed when discussing treatment options

## What Are Early Access Programs?

**Early Access Programs (EAPs) allow patients with serious or rare conditions, like DMD, to access medicines that are not yet fully approved or available in their country.**

These medicines are usually:

- Still in development or awaiting regulatory approval
- Showing promising results in clinical trials
- Not yet licensed or available as a standard treatment option
- Supplied directly by the pharmaceutical company, usually under specific eligibility criteria

Doctors must apply on behalf of the patient, as these medicines cannot be accessed independently.

**Important:**
EAPs are not the same as clinical trials.

**Clinical trials focus on research** — helping to understand how a medicine works, how safe it is, and who it may benefit. In DMD, trials often have strict eligibility criteria (such as age, genetic mutation, or stage of disease), meaning not all patients will be able to take part. Some trials may also involve placebos or comparison treatments.

**Early Access Programs (EAPs) -** focus on treatment and access. They may provide an option for patients who are not eligible for a clinical trial, or when a trial has finished but the medicine is not yet approved. In these cases, EAPs can offer a way to access a potentially beneficial treatment earlier, under the supervision of a doctor.

## Why Early Access Matters in Rare Diseases

In rare diseases like DMD, time is critical.

There is often a gap between:

- A medicine showing positive results in trials
- And it becoming officially approved and available

This approval process can take several years.

Early Access Programs help bridge that gap, giving patients the opportunity to access treatment sooner — especially when:

- There are limited or no available treatment options
- A patient is not eligible for a clinical trial
- A trial has ended, but the medicine is not yet approved

**Did you know?**

It can take several years for a medicine to move from successful trials to being widely available. Early access is one of the ways to potentially bridge this gap and allow patients access to the treatment during this waiting period.

## Different Names for Early Access

Early access can be confusing because it is called different things depending on the country.

You might hear:

- Compassionate Use
- Expanded Access
- Managed Access
- Named Patient Program

Although the names differ, they all refer to similar pathways to access unapproved medicines for patients who need them most.

**Tip:**

Terms like “compassionate use” or “named patient” may describe similar access pathways, but the specific requirements, regulations, and costs can vary. It’s important to check whether the medicine is provided free of charge or if any costs may apply.

## Benefits and Considerations

**Early Access Programs can offer hope — but it's important to understand both the potential benefits and limitations.**

### Potential Benefits

- **Earlier access to treatment:** Access promising medicines before they are officially approved.
- **More options:** Especially important in DMD, where treatment choices may be limited.
- **Potential impact on progression:** Some therapies may help manage symptoms or slow disease progression.
- **Hope and empowerment:** Knowing there are additional options can help families feel more informed and in control.

### Things to Consider

- **Limited long-term data:** Not all side effects or outcomes may be fully known.
- **Access may change:** Program can stop or evolve over time.
- **Eligibility and regulations:** Availability depends on country rules and individual circumstances.
- **Practical and cost factors:** May involve hospital visits, monitoring, or additional expenses.

Your doctor will help assess whether this is an appropriate option for you or your loved one.

## Real-Life Perspective (DMD Community Insight)

**Many families within the DMD community have shared that, at the point of diagnosis, the information provided can feel limited — often focusing on standard care, with less awareness of other options such as clinical trials/early access program.**

Awareness of these pathways often comes later, through:

- Personal research
- Patient advocacy groups
- Connecting with other families

While your doctor must be the one to assess eligibility and apply for an Early Access Program, these options may not always be raised in early discussions.

This highlights how important it is for patients and families to feel informed and empowered to ask questions and explore all possible options.

Things to keep in mind:

- Don't hesitate to ask about all available treatment pathways, including those still in development
- Early conversations can help ensure no options are overlooked
- Being informed can support more open and productive discussions with your healthcare team

**Tip:**
Writing down your questions before appointments can help you feel more confident during conversations.

## Questions to Ask Your Doctor

**If you are exploring treatment options, here are some helpful questions you can bring to your next appointment:**

### Understanding the Options

- Are there any early access programs available for DMD?
- Are there treatments currently in development that may be relevant?

### Eligibility & Safety

- Would I or my loved one be eligible for an early access program?
- What are the potential benefits and risks?

### Practical Considerations

- How would treatment be given?
- Would there be any costs involved?
- How often would we need to attend hospital visits?

### Support & Monitoring

- How will progress be monitored?
- What happens if the treatment doesn't work or causes side effects?

**Tip:**
You don't need to have all the answers — starting the conversation is the first step.

## How Patients Can Learn More

**Finding information on early access can be challenging, but you are not alone.**

Here are some ways to learn more:

- Speak with your treating doctor or specialist
- Connect with for-profit organisations like DMD Pathways
- Look at clinical trial registries (to see what treatments are in development) e.g clinicaltrials.gov
- Visit pharmaceutical company websites for early access policies

**Important to know:**
Information shared online or in patient forums may not apply to everyone. Regulations and availability vary between countries, so it's always important to check with your doctor and understand what options are appropriate for your specific situation.

## How DMD Pathways Can Support You

DMD Pathways helps families understand what exists and how to navigate it — including clinical trials, early access programs, and global treatment options.

We don't provide medical advice, but we help you:

- Understand what options may exist globally
- Prepare for conversations with your healthcare team
- Connect to relevant organisations and information

**Our goal is simple:** to make sure no family is left trying to figure this out alone.

## Real-Life Navigation Insight

Many families only become aware of early access, trials, or global options after months or years.

This isn't because the options don't exist it's because they are fragmented.

DMD Pathways exists to help families see the full landscape earlier, so they can ask the right questions at the right time.

# Frequently Asked Questions

## Who can request an EAP?

**An Early Access Program is usually requested by the treating physician on behalf of the patient.**

These programs are typically for patients who have no suitable treatment options available. Each program has defined eligibility criteria set by the sponsor, and patients are asessed against these criteria by their healthcare team to determine suitability.

Although the request must be made by the physician, patients and caregivers can still play an active role by discussing the option with their healthcare team and bringing it forward for consideration.

## Will this affect future trial eligibility?

**In most cases, receiving treatment through an Early Access Program does not automatically prevent a patient from taking part in future clinical trials.**

However, eligibility for clinical trials is determined by the specific inclusion and exclusion criteria of each study, which can vary widely between trials.

Some trials may have restrictions related to previous or ongoing treatments, while others may still allow participation. This is assessed on a case-by-case basis by the trial sponsor and the clinical investigators.

For this reasons, patients should always discuss potential clinical trial opportunities with their healthcare team before starting or during an Early Access Program, where possible.

## Why might access be declined?

**Each Early Access Program has specific inclusion and exclusion criteria set by the sponsor (pharmaceuitcal company).**

Access may be declined if a patient does not meet these criteria. In some cases, a patient may meet the elgibility criteria, but the program may not be open in their country. This can be due to sponsor decisions (e.g. program availability or supply considerations) or local regulatory requirements not permitting the program in that country.

In some countries, access may also be subject to review or approval by the relevant health authority or regulatory body, depending on local regulations, even if the sponsor has approved the request.

## Final Thoughts

**Early Access Programs are not always widely discussed, but they can play an important role in the treatment landscape — particularly in rare diseases like DMD, where options may be limited and time is critical.**

Understanding what these programs are, how they work, and when they might be relevant can help families feel more informed and better prepared when speaking with their healthcare team.

While not every patient will be eligible, being aware of early access ensures that all potential options are considered. Asking questions, staying informed, and connecting with the wider DMD community can make a meaningful difference in navigating what can often feel like a complex and uncertain journey.

Ultimately, knowledge can help families feel more confident, more in control, and better supported when making decisions about care.

To learn more, please refer to the Managed Access Program  A practical guide — BAP Pharma developed by BAP Pharma.

For further information, resources and guidance relating to Duchenne muscular dystrophy. please visit DMD Pathways website

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