Give your child with DMD...

the strength to be a kid

Tough on Duchenne.
So it's easier to be him.

LEARN MORE AT **AGAMREE.COM**

FDA approved to treat Duchenne muscular dystrophy (DMD) in patients 2 years of age and older

IMPORTANT SAFETY INFORMATION

Patients should not take AGAMREE if they are allergic to vamorolone or any of the inactive ingredients in AGAMREE.

aGamree® (vamorolone) oral suspension 40mg/mL

Please see Important Safety Information on pages 16-17 and accompanying full Prescribing Information.

What Is Duchenne Muscular Dystrophy (DMD)?

DMD is a progressive disorder of muscle weakness, which means muscles get weaker over time

DMD is a rare disease, estimated to occur in up to 1 in 3500 live male births in the United States

It is the most common form of muscular dystrophy in children

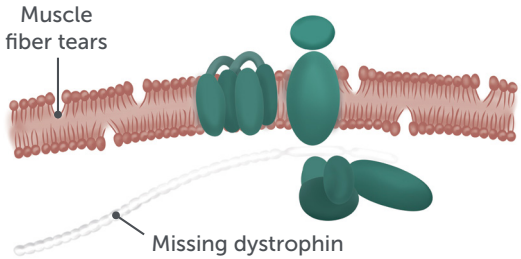
DMD is caused by a mutation in the gene responsible for producing a protein called dystrophin

How Does DMD Cause Muscle Weakness?

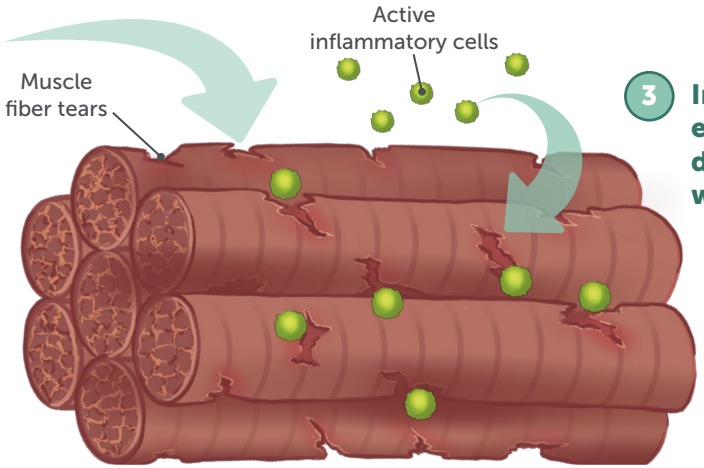
People living with DMD do not have enough dystrophin protein

- Dystrophin is necessary to strengthen and stabilize muscles to prevent damage during muscle contraction
- In people living with DMD, muscle damage also causes chronic inflammation, where inflammatory cells remain active in damaged muscles, causing even more muscle damage and muscle weakness over time

1 **People living with DMD do not have enough dystrophin protein**



2 **Without dystrophin, muscle contraction causes muscle damage and muscle weakness**



3 **Inflammation causes even more muscle damage and muscle weakness over time**

What Are the Symptoms of DMD?

All children living with DMD have muscle damage and muscle weakness that is progressive (gets worse over time)

Initially, DMD impacts the muscles that control bones in the hips and legs

This can impact the ability to get around, play, and participate in normal activities

Over time, weakness can advance to other muscles

Muscles of the heart and diaphragm (important for controlling breathing) are eventually affected

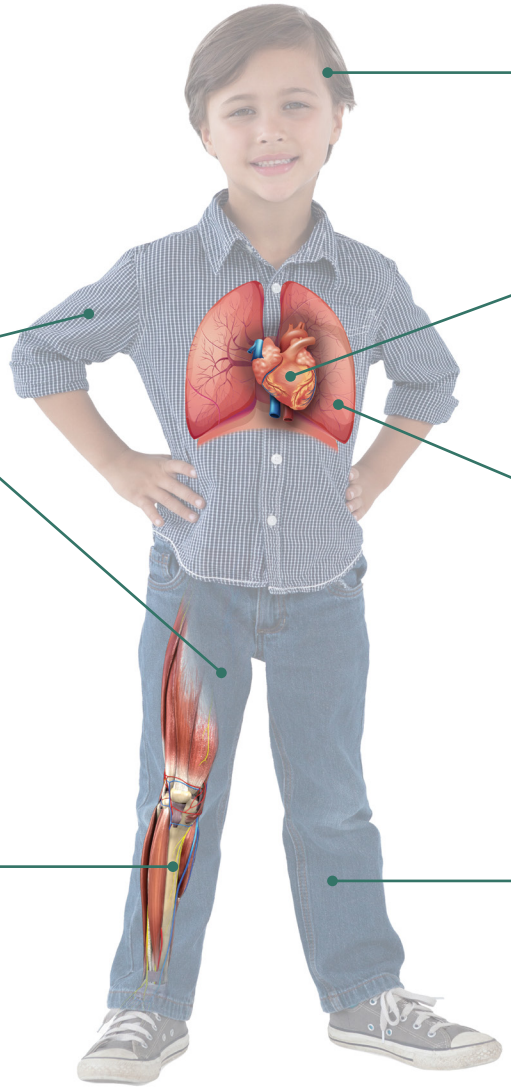
DMD increases the risk for osteoporosis (weakening of bones) and fractures

Bone fractures can impact mobility, which can also contribute to worsening muscle weakness over time

Behavior and learning problems may also occur

The lack of healthy dystrophin in the brain may result in social, behavioral, and emotional problems, as well as cognitive and learning impairments

What Are the Symptoms of DMD? (cont'd)



Behavior and learning problems

Gradual progression to heart muscle

- Irregular heartbeat
- Difficulty pumping enough blood to meet the body's needs

Gradual progression to breathing muscles

Weakened diaphragm, leading to difficulty breathing and need for assisted breathing

Weakened skeletal muscles

- Delayed milestones
- Difficulty keeping up with peers
- Frequent falls
- Waddling gait
- Difficulty rising from lying or sitting position (need to use arms to stand when leg muscles are too weak)

Weak, brittle bones with increasing risk of fractures

Large calf muscles due to buildup of fat and scar tissue

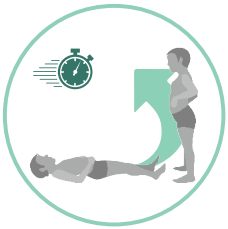
How Is DMD Evaluated?

Your healthcare provider may perform several tests to assess motor function

Many tests to assess DMD are timed function tests, which evaluate how long it takes to perform specific physical tasks.

Common DMD Assessments

Time to Stand (TTSTAND)



- Time required for the patient to stand from lying on their back

6-Minute Walk Test (6MWT)

- How far the patient can walk on a flat, hard surface in 6 minutes

Time to Run/Walk 10 Meters (TTRW)

- Time required for the patient to run or walk 10 meters

Additional tests commonly used to evaluate functional status in people with DMD include the North Star Ambulatory Assessment and the Time to Climb 4 Stairs (TTCLIMB).

The Role of Corticosteroids in DMD Treatment

Corticosteroids slow disease progression by reducing inflammation and maintaining muscle strength and control

However, corticosteroids also have unwanted side effects that can limit their use in treatment.

- These side effects may include behavior changes, growth delay, bone changes or fractures, cushingoid appearance (facial puffiness), weight gain, excessive hair growth, and cataracts
- Many of these side effects may make it necessary to modify or stop corticosteroid therapy
  - 6 of 10 people with DMD who stop treatment with corticosteroids do so because of side effects

Side Effects of Long-Term Corticosteroid Use in People Living With DMD

Behavioral Health

Nearly **4** in **10** have **behavior changes**

May make it necessary to modify or stop treatment

Bone Health

Up to **7** in **10** may have **growth delays**

Can negatively affect well-being and adherence to treatment

More than **7** in **10** experience **bone changes** or **fractures**

Increases risk for premature, permanent loss of ambulation

Talk with your healthcare provider about any side effects experienced while taking corticosteroids

Please see Important Safety Information on pages 16-17 and accompanying full Prescribing Information.

aGamree®
(vamorolone) oral suspension 40mg/mL

FDA Approved for the Treatment of Duchenne Muscular Dystrophy (DMD) in Patients 2 Years of Age and Older

Whether your child was recently diagnosed with DMD or has been diagnosed for years and is ready for a change, talk with your healthcare provider about how AGAMREE® is different and why it might be the right choice for your child.

IMPORTANT SAFETY INFORMATION

Patients should not take AGAMREE if they are allergic to vamorolone or any of the inactive ingredients in AGAMREE.

Please see Important Safety Information on pages 16-17 and accompanying full Prescribing Information.

Why AGAMREE®?

Designed to uncouple its anti-inflammatory effects from certain side effects

Clinically proven to improve muscle strength and function in boys with DMD and was well tolerated in clinical studies

Available as a once-daily, orange-flavored oral suspension

aGamree® (vamorolone) oral suspension 40mg/mL

Please see Important Safety Information on pages 16-17 and accompanying full Prescribing Information.

About AGAMREE®

AGAMREE was developed to uncouple the anti-inflammatory effects of corticosteroids commonly used in DMD from certain side effects

AGAMREE is...

A Corticosteroid With a Novel Structure

AGAMREE is structurally unique compared with corticosteroids commonly used to treat DMD

Designed to Retain Strong Anti-Inflammatory Action

Like corticosteroids commonly used to treat DMD, AGAMREE inhibits the activity of a protein complex that is an important factor in the inflammatory process

While the way AGAMREE works to treat DMD is not fully understood, it is thought that AGAMREE treats DMD through its strong anti-inflammatory action.

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How Was AGAMREE® Studied?

AGAMREE was studied in a multicenter, 24-week clinical trial

Included Patients

121 boys with confirmed DMD
Age range: 4 to less than 7 years
Average age: 5.4 years

Treatments

Patients were randomly assigned to receive 1 of 4 treatments:

- AGAMREE 6 mg/kg/day (**30** boys)
- AGAMREE 2 mg/kg/day (**30** boys)
- Prednisone 0.75 mg/kg/day (**31** boys)
- Placebo* (**30** boys)

Treatment was blinded, meaning that study physicians did not know which patients were taking which medication.

*Non-active substance that looks the same and is administered in the same way as the medications being studied.

Settings/Locations

33 Centers
11 Countries

Measures of Effectiveness

Effectiveness of AGAMREE was evaluated based on the average change from the start of the study to Week 24 in the results of timed function tests compared with placebo.

- The **primary measure** of effectiveness was the average change in **Time to Stand (TTSTAND)** velocity for AGAMREE 6 mg/kg/day versus placebo

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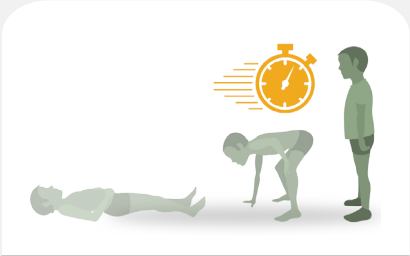
Muscle Strength and Function at the Start of the Study

At the start of the study, boys taking AGAMREE® 6 mg/kg/day and boys taking placebo had similar performance on the timed function tests

Timed Function Tests at the Start of the Study



AGAMREE 6 mg/kg/day
0.19 rises/second

Placebo
0.20 rises/second

Time to Stand Test

How fast a patient can stand from lying on their back

AGAMREE 6 mg/kg/day
313 meters

Placebo
355 meters

6-Minute Walk Test

Distance a patient can walk in 6 minutes

AGAMREE 6 mg/kg/day
1.6 meters/second

Placebo
1.7 meters/second

Time to Run/Walk 10 Meters

How fast a patient can run or walk 10 meters

aGamree® (vamorolone) oral suspension 40mg/mL

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AGAMREE® Improved Muscle Strength and Function

At Week 24, boys treated with AGAMREE 6 mg/kg/day...

Stood up **faster**

0.06 rises/second faster*

Time to Stand Test

How fast a patient can stand from lying on their back

Walked **farther**

42 meters farther*

6-Minute Walk Test

Distance a patient can walk in 6 minutes

Walked/ran **faster**

0.24 meters/second faster*

Time to Run/Walk 10 Meters

How fast a patient can run or walk 10 meters

*Compared with placebo.

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Side Effects With AGAMREE® in Clinical Studies

What are the most common side effects associated with AGAMREE?

In clinical studies in boys with DMD, the most common side effects with AGAMREE treatment included facial puffiness (cushingoid features), psychiatric disorders, vomiting, weight gain, and vitamin D deficiency.

Side Effects That Occurred in at Least 5% of Patients Treated With AGAMREE and More Frequently Than Placebo

Adverse Reactions, %	Placebo (n=29)	AGAMREE 2 mg/kg/day (n=30)	AGAMREE 6 mg/kg/day (n=28)
Cushingoid features	0	7	29
Psychiatric disorders[†]	14	7	21
Vomiting	7	17	14
Weight increased	3	0	11
Vitamin D deficiency	0	7	11
Cough	3	10	7
Headache	3	7	7
Diarrhea	3	3	7
Increased appetite	3	3	7
Rhinitis	3	3	7

[†]Includes the following adverse reactions that occurred more frequently in the AGAMREE group than in placebo: abnormal behavior, aggression, agitation, anxiety, irritability, mood altered, sleep disorder, and stereotypy.

These are not all the possible side effects of AGAMREE.
Call your healthcare provider for medical advice about side effects.

aGamree® (vamorolone) oral suspension 40mg/mL

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aGamree® (vamorolone) oral suspension 40mg/mL

What is AGAMREE®?

AGAMREE is a prescription medicine used to treat Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

IMPORTANT SAFETY INFORMATION

Patients should not take AGAMREE if they are allergic to vamorolone or any of the inactive ingredients in AGAMREE.

What is the most important information I should know about AGAMREE?

- **Do not stop AGAMREE, or change the amount taken, without first checking with your healthcare provider.** There may be a need for gradual dose reductions to decrease the risk of adrenal insufficiency crisis, which can be fatal.
- **There is an increased risk of infection when taking corticosteroids like AGAMREE.** Tell your healthcare provider if the patient has had recent or ongoing infections or has recently received a vaccine. Seek immediate medical advice in the case of fever or other signs of infection. Some infections can be severe, and sometimes fatal. Patients should avoid exposure to chickenpox or measles; alert your healthcare provider immediately if exposure occurs.
- **Corticosteroids, including AGAMREE, can cause an increase in blood pressure and water retention.** Your healthcare provider may monitor for these increases during treatment.
- **There is an increased risk of developing a hole in the stomach or intestines** in patients with certain gastrointestinal disorders when taking corticosteroids like AGAMREE.
- **Corticosteroids, including AGAMREE, can cause severe behavioral and mood changes.** Seek medical attention if behavioral or mood changes develop.
- **There is a risk of osteoporosis with prolonged use of corticosteroids like AGAMREE,** which can lead to vertebral and long bone fractures.
- **Corticosteroids like AGAMREE may cause cataracts or glaucoma.** Your healthcare provider should monitor for these conditions if AGAMREE treatment continues for more than 6 weeks.
- Immunizations should be up to date according to immunization guidelines prior to starting therapy with AGAMREE. **Live-attenuated or live vaccines should be administered at least 4 to 6 weeks prior to starting AGAMREE. Live-attenuated or live vaccines should not be administered in patients taking AGAMREE.**
- **Rare instances of severe allergic reaction have occurred** in patients receiving corticosteroid therapy.

IMPORTANT SAFETY INFORMATION (cont'd)

Before taking AGAMREE®, tell your healthcare provider about all medical conditions, including if the patient:

- has decreased liver function
- is pregnant or planning to become pregnant. AGAMREE can harm an unborn baby.
- is breastfeeding or planning to breastfeed. AGAMREE may appear in breastmilk and could affect a nursing child.

Certain medications can cause an interaction with AGAMREE. Tell your healthcare provider about all the medicines the patient takes, including prescription and over-the-counter medicines, dietary supplements, and herbal products.

What are the possible side effects of AGAMREE?

The most common side effects with AGAMREE include facial puffiness (cushingoid features), psychiatric disorders, vomiting, weight gain, and vitamin D deficiency. These are not all the possible side effects of AGAMREE.

Call your doctor for medical advice about side effects.

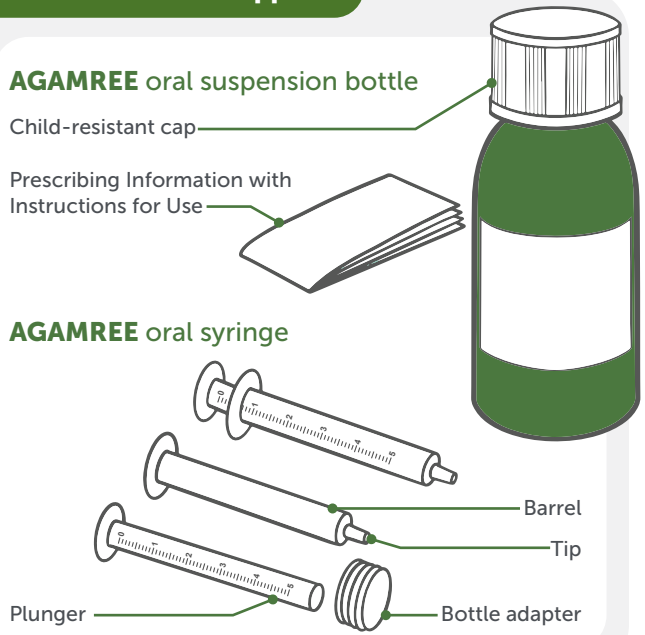
To report SUSPECTED ADVERSE REACTIONS, contact Catalyst Pharmaceuticals, Inc. at 1-844-347-3277 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

See the full Prescribing Information for AGAMREE at AGAMREE.com

aGamree® (vamorolone) oral suspension 40mg/mL

Getting Started With AGAMREE®

How AGAMREE Is Supplied



- AGAMREE is an **orange-flavored** oral suspension (liquid) that contains 40 mg/mL of vamorolone (the active ingredient of AGAMREE)
- AGAMREE is supplied as 100 mL of AGAMREE in a 125-mL bottle
- The bottle comes with one bottle adapter, two 5-mL oral syringes, and an Instructions for Use document shipped to your home in the AGAMREE carton

Read the Instructions for Use before you start using AGAMREE oral suspension and each time you get a new bottle

aGamree® (vamorolone) oral suspension 40mg/mL

Please see Important Safety Information on pages 16-17 and accompanying full Prescribing Information.

The Proper Daily Dose of AGAMREE®

The recommended dose of AGAMREE is 6 mg/kg taken orally once daily, preferably with a meal, up to a maximum daily dosage of 300 mg for patients weighing more than 50 kg (110 lb)

AGAMREE Dosage

6
mg/kg

Up to 300 mg/d for patients weighing >50 kg (110 lb)

- Doses may be gradually reduced to 2 mg/kg/day as needed, based on side effects
  - Call your healthcare provider for medical advice about side effects
- The dosage of AGAMREE should be reduced in patients with mild to moderate liver disease or if the patient is taking certain medications
  - Tell your healthcare provider about all medical conditions, and about all medications the patient takes

Patients may switch to AGAMREE from another oral corticosteroid (like prednisone or deflazacort) without treatment interruption or period of prior corticosteroid dosage reduction

aGamree® (vamorolone) oral suspension 40mg/mL

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How to Take or Administer AGAMREE®

Ask your healthcare provider or pharmacist to show you how to measure your prescribed daily dose using the oral syringe

Verify the dose in milliliters (mL) as prescribed by your healthcare provider or noted by the pharmacist.

Example of How to Verify the Proper Daily Dose

Noah weighs...

and is prescribed 6 mg/kg/day

6 mg/kg/day x 20 kg = 120 mg/day

Each mL of liquid contains 40 mg of active ingredient (40 mg/mL)

Divide the total mg dose by 40 mg to get the correct mL amount

$$\frac{120 \text{ mg/day}}{40 \text{ mg/mL}} = 3 \text{ mL/day}$$

Follow detailed guidance for administration described in the Instructions for Use

Withdraw the amount in mL using the oral syringe

Remember to shake well for about 30 seconds before withdrawing

Withdraw 3 mL

Administer the entire syringe into Noah's mouth

Administer 3 mL

Example dose calculation. Dose and milliliters (mL) should be calculated by your healthcare provider or pharmacist.

aGamree® (vamorolone) oral suspension 40mg/mL

Please see Important Safety Information on pages 16-17 and accompanying full Prescribing Information.

Important Administration Instructions

AGAMREE® is for oral use only

Read the Instructions for Use before you start using AGAMREE and each time you receive a new bottle.

Place the oral syringe tip in the mouth toward the cheek and slowly push the plunger down until the oral syringe is empty

✘ Do not forcefully push on the plunger or give AGAMREE too fast to the back of the mouth or throat

Administer once daily, preferably with a meal

✘ Do not mix with any type of liquids before administering the prescribed daily dose

Administer exactly as prescribed by your healthcare provider

✘ Do not administer more than the prescribed daily dose

✘ Do not stop AGAMREE suddenly without first speaking with your healthcare provider

Read the Instructions for Use

aGamree® (vamorolone) oral suspension 40mg/mL

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What Should I Tell My Healthcare Provider Before Starting AGAMREE®?

Before Starting AGAMREE

Tell your healthcare provider about...

All medical conditions

Including if the patient:

- Has decreased liver function
- Is pregnant or planning to become pregnant
- Is breastfeeding or planning to breastfeed

All medications the patient takes

- These may include prescription and over-the-counter medicines, dietary supplements, and herbal products
- Certain medications may cause an interaction with AGAMREE

aGamree® (vamorolone) oral suspension 40mg/mL

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What Else Should I Know About AGAMREE®?

Immunizations should be up to date according to immunization guidelines prior to starting therapy with AGAMREE

- Live-attenuated or live vaccines should be administered at least 4 to 6 weeks prior to starting AGAMREE
- Live-attenuated or live vaccines should not be administered in patients taking AGAMREE

Do not stop AGAMREE, or change the amount taken, without first checking with your healthcare provider

- There may be a need for gradual dose reductions to decrease the risk of adrenal insufficiency crisis, which can be fatal

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AGAMREE® Access and Financial Support

Catalyst PATHWAYS®

A clear path to prompt, affordable treatment

People living with DMD and their families face a variety of challenges every day. The cost of treatment should NOT be one of them. That's why we created Catalyst Pathways®—a free program that offers personalized support and assistance to people living with DMD and their families.

Enroll Now in Catalyst Pathways
Complete the enrollment form with your healthcare provider or call 1-833-4-CATALYST (1-833-422-8259)

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Please see Important Safety Information on pages 16-17 and accompanying full Prescribing Information.

Help With Access

Catalyst Bridge program helps cover access delays

While your insurance coverage is being reviewed, Catalyst Pathways® will provide qualifying patients with "Bridge" medicine (free medicine to carry eligible patients over the gap between insurance investigation and confirmation of coverage).*

*Available for new patients with DMD who are enrolled in the Catalyst Pathways program and have been prescribed AGAMREE®. Some restrictions apply.

Financial Assistance Programs

Copay Assistance

For patients with commercial insurance, Catalyst Pathways lowers out-of-pocket costs to $0/month

Patient Assistance Program

If you do not have insurance or are denied coverage, Catalyst Pathways provides free medicine to qualifying patients for as long as it's needed

Foundation Assistance

If you're having trouble paying your out-of-pocket costs, Catalyst Pathways can direct you to nonprofit organizations that can help you pay for your medicine

Visit **YourCatalystPathways.com** for a full list of support programs

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Catalyst PATHWAYS®

Personalized Support for Patients and Families

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Catalyst Pathways® Support Specialists

Support specialists help you manage the challenges of living with DMD

Catalyst Pathways features a dedicated team of support specialists who have the answers you're looking for and are trained to troubleshoot any issues that may arise.

Care Coordinator

Provides a warm welcome, passes along need-to-know information, and connects you to your dedicated team

Insurance Navigator

Handles complicated coverage and reimbursement issues and works to resolve them

Patient Access Liaison (PAL)

Your go-to, local resource for education and treatment support when and where you need it

Contact a Care Coordinator
Call 1-833-4-CATALYST (1-833-422-8259)
Monday–Friday, 7AM–7PM Central

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(vamorolone) oral suspension 40mg/mL

Talk to Your Healthcare Provider

The information in this brochure does not take the place of talking to your healthcare provider about your medical condition or treatment

- Preparation is the key to having a productive conversation with your healthcare provider
- Consider taking notes on how your child is doing to help inform your healthcare provider during your office visit
- Be sure to include questions you may have for your healthcare provider

Get tips on how to talk with your healthcare provider

Duchenne Communities

There are several groups and organizations that can provide information, support, and more for people living with DMD and their families

Connect with organizations created to help families living with DMD

Catalyst pharmaceuticals

AGA-0152 March 2024