

# ANSWERS TO FREQUENTLY ASKED QUESTIONS

## FAQs

SKYCLARYS<sup>®</sup>  
(omaveloxolone) 50 mg  
capsules

Patients featured are paid spokespersons for Biogen.

### What is Friedreich ataxia (FA)?

FA is a rare, genetic, progressive disease that damages the nervous system, causing people with FA to lose control of their muscles over time.

### How do I get diagnosed for FA?

People with FA may notice certain signs and symptoms that lead them to talk with a doctor. These can include frequent falls, feeling unbalanced, lack of feeling in the feet and lower legs, and extreme tiredness. For some patients, scoliosis can also be an early sign of FA.

If your doctor suspects FA, they may perform a genetic test to confirm a diagnosis.

### Is FA genetic?

Yes, FA is a genetic disease. It happens when a mutation in the frataxin gene (or *FXN*) causes the protein frataxin to be made incorrectly or causes the body to not make enough of it.

FA is what's called an "autosomal recessive" disease. This means that you need to inherit a mutated *FXN* gene from each of your parents in order to have FA.

Because FA is a genetic disease, genetic testing is the best way to confirm a diagnosis.

### What is disease progression?

"Disease progression" is a term that describes how FA affects your body over time. While FA affects everyone differently, people with FA can expect to progress toward loss of function. This can include loss of coordination in the hands, feet, arms, and legs. It can also include slurred speech and difficulty swallowing. Most people with FA will need to use a wheelchair within 10 to 15 years after being diagnosed.

It's important to know that, while SKYCLARYS has been shown to slow down disease progression in a clinical trial, it is not a cure for FA.\*

\*In a 48-week clinical trial, treatment with SKYCLARYS resulted in less physical impairment over time versus placebo, as measured by the modified Friedreich Ataxia Rating Scale (mFARS).



## How is disease progression measured?

There are many ways to measure FA disease progression. Your doctor will decide which way is best for measuring your specific disease progression.

In the clinical trial for SKYCLARYS, researchers used a neurological exam called the modified Friedreich Ataxia Rating Scale (mFARS) to measure disease progression and to assess the effectiveness of SKYCLARYS.

The mFARS exam evaluates 4 areas related to everyday activities:

- ▶ Mouth and throat function (bulbar function)
- ▶ Upper limb coordination
- ▶ Lower limb coordination
- ▶ Upright stability

## How does SKYCLARYS work?

The exact way that SKYCLARYS works is unknown. In some tests of both animals and humans, SKYCLARYS was shown to activate a certain protein, called Nrf2, which is involved in reducing oxidative stress inside of cells.

## How do I take SKYCLARYS?

SKYCLARYS is a daily oral medication for FA. SKYCLARYS is taken as 3 capsules of 50 mg each.

- ▶ Take SKYCLARYS exactly as your healthcare provider tells you to take it
- ▶ Take SKYCLARYS capsules on an empty stomach, at least 1 hour before or 2 hours after eating
- ▶ Swallow SKYCLARYS capsules whole. Do not crush or chew
- ▶ If SKYCLARYS capsules cannot be swallowed whole, the capsules may be opened and the entire contents of both halves sprinkled onto 2 tablespoons (30 mL) of applesauce
  - Stir the mixture
  - Swallow all the mixture of medicine and applesauce right away. Do not store the mixture of medicine and applesauce to use at a later time
  - The contents of the SKYCLARYS capsules should not be mixed with milk or orange juice
  - Do not administer SKYCLARYS by an enteral feeding tube
- ▶ If you miss a dose, then you should skip the missed dose and take the next dose at the regular time the next day. Do not double your next dose or take more than the prescribed dose
- ▶ Do not drink grapefruit juice or eat grapefruit. These may change the amount of SKYCLARYS in your blood

## Is SKYCLARYS a good fit for me?

SKYCLARYS is the first and only FDA-approved prescription medication for the treatment of FA in adults and adolescents aged 16 years and older. Talk with your doctor about all the risks and benefits of SKYCLARYS. Your doctor will order tests of your liver function, BNP (a protein that tells how well your heart is working), and cholesterol before and throughout your treatment with SKYCLARYS.

## What were the results of the clinical trial?

In a 48-week clinical trial, treatment with SKYCLARYS resulted in less physical impairment over time versus placebo, as measured by the modified Friedreich Ataxia Rating Scale (mFARS).

## What are the possible side effects?

### Some serious side effects:

Some patients taking SKYCLARYS in the clinical trial saw an increase in liver enzymes, an increase in B-type natriuretic peptide (BNP, a protein that tells how well your heart is working), and changes in cholesterol levels. Your doctor will monitor you for changes in these areas periodically throughout your treatment.

### Most common side effects:

The most common side effects from the clinical trial include elevated liver enzymes, headache, nausea, stomach pain, and tiredness. Some people also experienced diarrhea and muscle aches and pain. These are not all the possible side effects of treatment with SKYCLARYS.

Talk to your doctor about all the possible risks and benefits of SKYCLARYS before you start treatment.

## How long do side effects last?

In the MOXle clinical trial, patients experienced side effects for different amounts of time. Your experience with side effects may be different than those of patients in the clinical trial. Talk to your doctor about any side effects you may be experiencing with SKYCLARYS.

## What do I need to get started with SKYCLARYS?

Before starting treatment with SKYCLARYS, talk to your healthcare provider about all the possible risks and benefits. Your doctor will order tests of your liver function, BNP (a protein that tells how well your heart is working), and cholesterol before and throughout your treatment with SKYCLARYS.

Tell your healthcare provider about all of your medical conditions, including if you:

- ▶ Have liver problems
- ▶ Have a history of heart problems, including heart failure
- ▶ Have a high level of fat in your blood (high cholesterol)
- ▶ Are pregnant or plan to become pregnant
  - There is a pregnancy registry for women who are pregnant and are taking SKYCLARYS. The purpose of this registry is to collect information about the health of you and your baby. Your healthcare provider can enroll you or you may enroll yourself by calling 1-866-609-1785 or by sending an email to [SkyclarysPregnancySurveillance@ppd.com](mailto:SkyclarysPregnancySurveillance@ppd.com)
- ▶ Are breastfeeding or plan to breastfeed

Tell your healthcare provider about all the medications you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements such as St John's wort.

Taking SKYCLARYS with other medicines can cause serious side effects.



## What do I need to get started with SKYCLARYS? (cont'd)

SKYCLARYS may affect the way other medicines work, and other medicines may affect the way SKYCLARYS works.

Know the medicines you take. Keep a list of them to show to your healthcare provider and pharmacist when you get a new medicine.

## How much will SKYCLARYS cost me?

Financial support options may be available to you through Biogen REACH.\*

Whether or not you are insured, if SKYCLARYS is right for you, the REACH program may be able to help.

Through the Biogen REACH copay program, 95% of commercially insured patients pay \$0 in out-of-pocket costs, and 94% of Medicare patients pay \$15 or less in out-of-pocket costs.<sup>†</sup>

If you have limited income and resources, you may qualify for the Medicare Part D Low-Income Subsidy Program, also known as Extra Help.

If you are uninsured, please contact the REACH program at 1-844-98-REACH (1-844-981-3224) to determine financial assistance options.

\*You are not required to enroll in Biogen REACH to receive a prescription for SKYCLARYS.

<sup>†</sup>As of July 2024.

## How do I get SKYCLARYS?

SKYCLARYS is not available at retail pharmacies. Your SKYCLARYS prescription will be delivered to you by Biologics, the Biogen REACH exclusive specialty pharmacy.

When you enroll in Biogen REACH, you'll be assigned a Family Access Manager (FAM) who can help you work through the logistics associated with getting your SKYCLARYS prescription.\* This can include specialty pharmacy support.

\*You are not required to enroll in Biogen REACH to receive a prescription for SKYCLARYS.

### WHAT IS SKYCLARYS?

- ▶ SKYCLARYS<sup>®</sup> (omaveloxolone) is used for the treatment of Friedreich ataxia in adults and children aged 16 years and older. It is not known if SKYCLARYS is safe and effective for use in children younger than 16 years of age

### IMPORTANT SAFETY INFORMATION

#### What are the possible side effects of SKYCLARYS?

#### SKYCLARYS may cause serious side effects, including:

- ▶ **Increase in blood liver enzymes:** Some people taking SKYCLARYS have had an increase in the level of liver enzymes in their blood. Your healthcare provider will do liver function tests
  - before you start taking SKYCLARYS
  - every month for the first 3 months after starting your treatment with SKYCLARYS
  - during certain times as needed while taking SKYCLARYS

If your liver enzymes increase, your healthcare provider may change your dose, stop treatment for some time or completely stop treatment with SKYCLARYS.

## IMPORTANT SAFETY INFORMATION (cont'd)

### What are the possible side effects of SKYCLARYS? (cont'd)

#### SKYCLARYS may cause serious side effects, including (cont'd):

- ▶ **Increase in a blood protein called B-Type Natriuretic Peptide (BNP).** BNP tells how well your heart is working. Your healthcare provider will check your BNP levels before your treatment with SKYCLARYS. Tell your healthcare provider if you have signs and symptoms of your heart not working well such as too much fluid in your body (fluid overload). Signs and symptoms may include:
  - sudden weight gain (3 pounds or more of weight gain in 1 day, or 5 pounds or more of weight gain in 1 week)
  - swelling in your arms, hands, legs, or feet (peripheral edema)
  - fast heartbeat (palpitations)
  - shortness of breath

If you have symptoms of fluid overload that is considered a side effect of SKYCLARYS, your healthcare provider may stop treatment with SKYCLARYS

- ▶ **Changes in cholesterol levels.** Increases in low density lipoprotein cholesterol (LDL-C) or bad cholesterol and decreases in high density lipoprotein cholesterol (HDL-C) or good cholesterol have happened during treatment with SKYCLARYS. Your healthcare provider will check your cholesterol levels before and during your treatment with SKYCLARYS

**The most common side effects of SKYCLARYS include:** increased liver enzymes (ALT/AST), headache, nausea, stomach pain, tiredness, diarrhea, and muscle pain.

#### Before taking SKYCLARYS, tell your healthcare provider about all of your medical conditions, including if you:

- ▶ have liver problems
- ▶ have a history of heart problems, including heart failure
- ▶ have a high level of fat in your blood (high blood cholesterol)
- ▶ are pregnant or plan to become pregnant. It is not known if SKYCLARYS will harm your unborn baby. Women who use hormonal birth control should use another form of birth control such as a non-hormonal intrauterine system or an extra non-hormonal birth control such as condoms while using SKYCLARYS and for 28 days after stopping SKYCLARYS
- ▶ Pregnancy exposure registry: There is a pregnancy registry for women who are pregnant and are taking SKYCLARYS. The purpose of this registry is to collect information about the health of you and your baby. Your healthcare provider can enroll you or you may enroll yourself by calling 1-866-609-1785 or by sending an email to [SkyclarysPregnancySurveillance@ppd.com](mailto:SkyclarysPregnancySurveillance@ppd.com)
- ▶ are breastfeeding or plan to breastfeed. It is not known if SKYCLARYS passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby if you take SKYCLARYS

**Tell your healthcare provider about all the medicines you take,** including prescription and over-the-counter medicines, vitamins, and herbal supplements such as St. John's Wort.

- ▶ Taking SKYCLARYS with other medicines can cause serious side effects
- ▶ SKYCLARYS may affect the way other medicines work, and other medicines may affect how SKYCLARYS works
- ▶ Know the medicines you take. Keep a list of them to show to your healthcare provider and pharmacist when you get a new medicine

### What should I avoid while taking SKYCLARYS?

- ▶ Do not drink grapefruit juice or eat grapefruit. These may change the amount of SKYCLARYS in your blood
- These are not all the possible side effects of SKYCLARYS. For more information, ask your healthcare provider or pharmacist.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

**Please see full Prescribing Information, including Patient Information.**