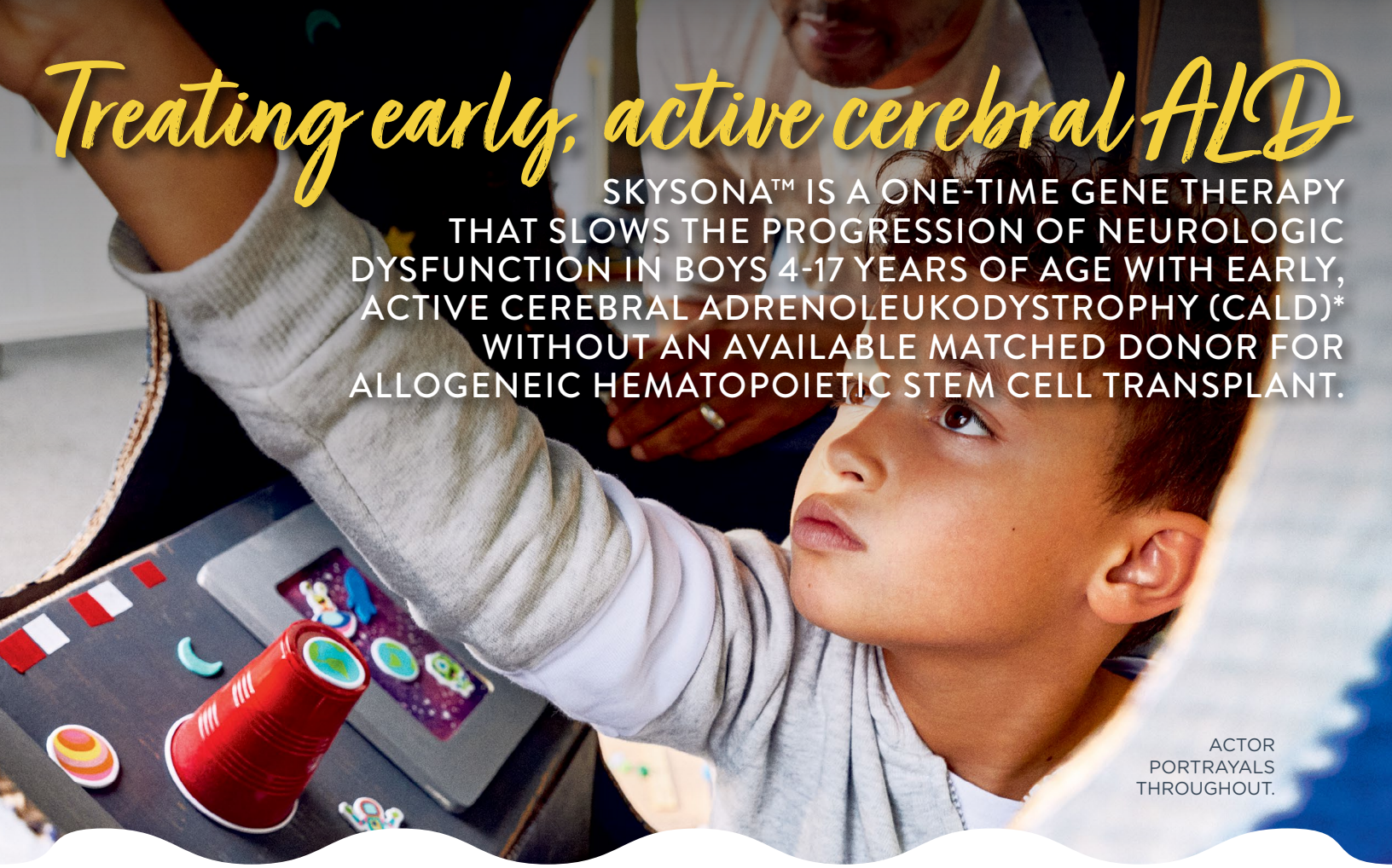

Treating early, active cerebral ALD

SKYSONA™ IS A ONE-TIME GENE THERAPY THAT SLOWS THE PROGRESSION OF NEUROLOGIC DYSFUNCTION IN BOYS 4-17 YEARS OF AGE WITH EARLY, ACTIVE CEREBRAL ADRENOLEUKODYSTROPHY (CALD)* WITHOUT AN AVAILABLE MATCHED DONOR FOR ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANT.

ACTOR PORTRAYALS THROUGHOUT.

As a caregiver, you can use this guide to help you understand how SKYSONA uses your child's own cells to treat cerebral ALD.

*Early, active CALD refers to asymptomatic or mildly symptomatic (NFS ≤1) boys who have gadolinium enhancement on brain MRI and Loes scores of 0.5-9.

What is SKYSONA?

SKYSONA is a one-time gene therapy to treat boys with early, active cerebral adrenoleukodystrophy (CALD). CALD is a genetic disease caused by mutations in the *ABCD1* gene that lead to the buildup of very long chain fatty acids (VLCFAs) in the brain. These VLCFAs may destroy the protective covering around nerve cells and cause damage to the brain. Once this occurs, this damage can be seen on magnetic resonance imaging (MRI) of the brain, which is when the cerebral form of adrenoleukodystrophy (CALD) is diagnosed. SKYSONA may be recommended if this damage is determined to be early (based on a lesser degree of the damage on MRI) and if cerebral disease is active (based on presence of contrast enhancement on MRI that indicates this damage is ongoing). SKYSONA is made specifically for each patient, using the patient's own blood stem cells and adds functional copies of the *ABCD1* gene to your cells. This may help your body to break down the VLCFAs to slow the progression of damage to the brain and slow the decline in neurologic function.

IMPORTANT SAFETY INFORMATION

What is the most important information I or my caregiver should know about SKYSONA?

SKYSONA may cause cancer of the blood and bone marrow, which can be life-threatening and lead to death. Blood cancer has resulted from treatment with SKYSONA because cancer-causing genes have been turned on by the gene therapy. Patients have developed cancer as early as 1 year to more than 9 years after SKYSONA administration. The percent of patients who will develop cancer and the maximum timeframe when cancer caused by SKYSONA could develop is unknown. Blood cancer is usually treated with chemotherapy and may require stem cell transplant. Stem cell transplant using cells from a donor have been used to treat children diagnosed with blood cancer that was caused by SKYSONA. Cancer may recur and require further treatment. Treatment of blood cancer has led to death.

Please see Important Safety Information on pages 10-11, and full Prescribing Information, including Boxed WARNING and Medication Guide.

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how TO USE THIS GUIDE

From disease education to understanding the SKYSONA treatment journey, there's a lot of information available to you in this guide. To get started, pick a topic you're ready to learn more about.

To make learning about SKYSONA and cerebral ALD a little easier, we've added a glossary of clinical terms to the back of this guide. If you ever see a word that's **BOLD, BLUE, and UNDERLINED**, that means there's a definition in the glossary you can refer to.

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IMPORTANT SAFETY INFORMATION (cont'd)

What is the most important information I or my caregiver should know about SKYSONA? (cont'd)

Before SKYSONA treatment, discuss the risks and benefits of SKYSONA and alternative treatment options with your doctor. Because of the risk of cancer caused by SKYSONA, your doctor may recommend that you are evaluated by a hematologist to determine if you are at an increased risk for cancer and change whether SKYSONA is appropriate for you.

Because of the risk of cancer, it is important for you to be monitored lifelong. At a minimum we recommend blood tests every 3 months for 15 years. Blood tests will look at your blood cell counts and the locations in your blood cells where the gene therapy is inserted. If your blood counts are too low or too high, or if you have lots of cells with the same gene therapy insertion sites, additional testing may be recommended. Additional testing might include more frequent blood tests and a bone marrow evaluation, which can tell your doctor more than blood tests about the health of your bone marrow and if there is cancer forming.

Please see Important Safety Information on pages 10-11, and full Prescribing Information, including Boxed WARNING and Medication Guide.

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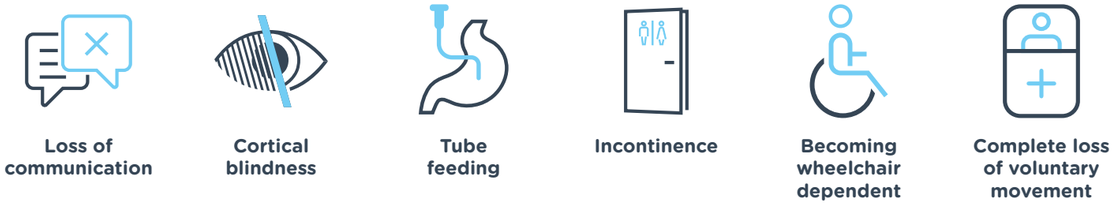
UNDERSTANDING CEREBRAL ALD

Cerebral adrenoleukodystrophy (ALD) is a genetic disease that progresses from ALD and affects many important functions of a person's brain. If left untreated, cerebral ALD can result in **major functional disabilities (MFDs)** and early death. MFDs include:



Loss of communication | **Cortical blindness** | **Tube feeding** | **Incontinence** | **Becoming wheelchair dependent** | **Complete loss of voluntary movement**

ABOUT CEREBRAL ALD

To understand cerebral ALD, let's dive into the disease at the genetic level.

ALD is caused by a **genetic mutation** in a specific gene labeled ***ABCD1***. This mutation prevents a person's body from producing a specific protein that's needed to break down **very long-chain fatty acids (VLCFAs)**.

VLCFAs BUILDING UP IN THE BRAIN

VLCFAs are an important part of our bodies. They are the building blocks of fat and contribute to important body functions like forming the skin barrier or assisting how our kidneys operate. But when VLCFAs are not able to be broken down, a buildup occurs in the brain, which destroys the protective covering around nerve cells and causes damage to the brain.

When a person progresses from ALD to cerebral ALD, it's caused by a buildup of VLCFAs in the brain.

Left untreated, MFDs can develop.

CAREFULLY MONITOR CEREBRAL ALD

An important goal in successfully managing cerebral ALD is to identify it as early as possible. This is done by continuous monitoring for it through **magnetic resonance imaging (MRI)**.

Based on a 2021 review of multiple scientific studies to establish guidelines in monitoring children with cerebral ALD, it's recommended that children diagnosed with ALD (the non-cerebral kind) should receive regular MRI screenings. Screenings should start when a child is between 12 and 18 months of age. The second screening should occur a year after the first. Afterwards, regular screenings should be done every 6 months from ages 3 to 12.

Doctors use these screenings to spot any early signs of cerebral ALD and rely on changes on MRI. With the goal of identifying active cerebral ALD early on, it may create the opportunity to slow its progression by acting fast.

AROUND

40%

of children with ALD will progress to cerebral ALD **between the ages of 3 and 12**

CEREBRAL ALD HAS HISTORICALLY BEEN TREATED WITH DONOR CELLS

Cerebral ALD treatments have relied on the transplantation of **donor blood stem cells**. The idea is that the donor cells give a body functioning copies of the *ABCD1* gene that will allow the body to produce **ALD protein (ALDP)**, the protein needed to prevent the buildup of VLCFAs. However, donor blood stem cell transplants require something very important—the availability and willingness of a donor.

The best option for a stem cell donor is a child's sibling, called a **matched sibling donor (MSD)**. The better the match from a sibling donor, the less risk of immunologic complications (infection, graft-versus-host disease). However, MSDs are only available for about 11% of children, leaving many people having to find a donor through the **bone marrow registry**.

Is there a way to treat early, active cerebral ALD?

discover SKYSONA

SKYSONA is the first and only **gene therapy** for:

- Boys 4-17 years of age without an available matched donor for allogeneic hematopoietic stem cell transplant
- Early, active CALD refers to asymptomatic or mildly symptomatic (NFS ≤1) boys who have gadolinium enhancement on brain MRI and Loes scores of 0.5-9

Before being treated with SKYSONA, you should have a detailed discussion with your child's doctor about the risks and benefits of SKYSONA and alternative treatment options. Alternative treatment may include an allogeneic hematopoietic stem cell transplant—discuss with your child's physician the possibility of a stem cell transplant using cells from a suitable donor, if available.

YOUR CHILD'S BLOOD STEM CELLS ARE COLLECTED

SKYSONA is made specifically by using your child's own blood stem cells

SKYSONA IS MANUFACTURED

Once a child's blood stem cells have been collected, working copies of the *ABCD1* gene are added to these cells through the aid of a **viral vector**

SKYSONA CELLS REPRODUCE

After SKYSONA is administered, it makes its way to the child's bone marrow, where it goes through **engraftment** (making other healthy blood cells)

FUNCTIONAL ALDP IS PRODUCED

Genetically modified cells allow the child's body to make ALDP

ALDP BREAKS DOWN VLCFAs IN THE BRAIN

With functional ALDP, the child is able to effectively break down VLCFAs from their brain—slowing the progression of damage to the brain and slowing the decline in neurologic function

IMPORTANT SAFETY INFORMATION (cont'd)

What is the most important information I or my caregiver should know about SKYSONA? (cont'd)

If blood cancer develops quickly or if you have not been having the recommended blood or bone marrow tests, you might experience symptoms of cancer before it is diagnosed. You or your caregiver should call your healthcare provider right away for any of these signs or symptoms:

- Abnormal bruising or bleeding (including nosebleed)
- Severe headache
- Unusual stomach or back pain
- Fever (100.4ºF/38ºC or higher)
- Blood in urine, stool, or vomit
- Coughing up blood
- Swollen glands
- Abnormal tiredness

If you are diagnosed with a cancer, have your treating physician contact Genetix Biotherapeutics at 1-833-999-6378.

how SKYSONA WORKS

SKYSONA gives a child with cerebral ALD the genetic instructions to be able to produce ALD protein (ALDP)—the protein needed to break down very long-chain fatty acids (VLCFAs) in the brain. Below is a detailed description of what SKYSONA looks like in action once it is infused.

Curious about the SKYSONA process?

Pages 16–19 offer information on the SKYSONA treatment journey to help you stay informed when discussing treatment plans with your child's doctor.

Please see Important Safety Information on pages 10-11, and full Prescribing Information, including Boxed WARNING and Medication Guide.

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SKYSONA *study information*

Before SKYSONA was made available, two clinical studies (Studies 1 and 2) were conducted to determine **efficacy** (how good the treatment is at achieving its intended result) and safety. These studies were designed to determine MFD-free survival in children with cerebral ALD who were treated with SKYSONA.

The MFDs are defined as:

• loss of communication • cortical blindness • requirement for tube feeding • total incontinence
• wheelchair dependence • complete loss of voluntary movement

STUDY INFORMATION

STUDY	TREATMENT	PATIENTS ENROLLED	DURATION
STUDY 1 (Completed)	SKYSONA	32 patients with early cerebral ALD • Elevated VLCFA levels • Loes score: 0.5 to 9.0 • Aged 4 to 14 years	24 months
STUDY 2 (Completed)	SKYSONA	35 patients with early cerebral ALD • Elevated VLCFA levels • Loes score: 0.5 to 9.0 • Aged 4 to 14 years	24 months
LTF-304 (Long-term follow-up study)	SKYSONA	Patients from Study 1 and Study 2	13 years
STUDY 3 (Completed)	• Untreated (natural history population) • Allo-HSCT	Existing medical records for patients with early, active CALD at diagnosis	N/A
STUDY 4	ALLO-HSCT	Retrospective allo-HSCT data collection study	N/A

efficacy AGAINST CALD

Treatment efficacy was determined based on an analysis in symptomatic patients that compared time from onset of symptoms to time to first MFD or death (ie, MFD-free survival) in SKYSONA treated and untreated (natural history) patients.

To be included in the analysis, patients had to have symptoms at baseline or be asymptomatic at baseline and have developed symptoms during the course of the study. Additionally, they had to have at least 24 months of follow-up after becoming symptomatic or have had an event (MFD or death).

ESTIMATED MFD-FREE SURVIVAL RATE WAS HIGHER WITH SKYSONA AT MONTH 24 FROM TIME OF SYMPTOM ONSET*†

MFD-FREE SURVIVAL RATE IN SYMPTOMATIC SKYSONA PATIENTS
(N=11)

MFD-FREE SURVIVAL RATE IN SYMPTOMATIC UNTREATED PATIENTS
(N=7)

*Based on post hoc enrichment analysis in symptomatic patients that compared time from onset of symptoms (NFS ≥1) to time to first MFD or death (ie, MFD-free survival) in SKYSONA treated and untreated natural history controls whose data derived from existing medical records for patients with CALD.
†To be included in the analysis, patients had to have symptoms at baseline (NFS=1) or be asymptomatic (NFS=0) at baseline and have developed symptoms (NFS ≥1) during the course of follow-up in the study. Additionally, they had to have at least 24 months of follow-up after initial NFS ≥1 or have had an event (MFD or death).

IMPORTANT SAFETY INFORMATION (cont'd)

What is the most important information I or my caregiver should know about SKYSONA? (cont'd)

SKYSONA may cause life-threatening allergic reactions as it contains DMSO. Please inform your healthcare provider if you have been told that your child has a DMSO allergy or has experienced a reaction after receiving a DMSO-containing product.

It is important that you or your caregiver tell your healthcare providers that you have received SKYSONA.

Please see Important Safety Information on pages 10-11, and full Prescribing Information, including Boxed WARNING and Medication Guide.

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IMPORTANT *safety information*

What is the most important information I or my caregiver should know about SKYSONA?

SKYSONA may cause cancer of the blood and bone marrow, which can be life-threatening and lead to death. Blood cancer has resulted from treatment with SKYSONA because cancer-causing genes have been turned on by the gene therapy. Patients have developed cancer as early as 1 year to more than 9 years after SKYSONA administration. The percent of patients who will develop cancer and the maximum timeframe when cancer caused by SKYSONA could develop is unknown. Blood cancer is usually treated with chemotherapy and may require stem cell transplant. Stem cell transplant using cells from a donor have been used to treat children diagnosed with blood cancer that was caused by SKYSONA. Cancer may recur and require further treatment. Treatment of blood cancer has led to death.

Before SKYSONA treatment, discuss the risks and benefits of SKYSONA and alternative treatment options with your doctor. Because of the risk of cancer caused by SKYSONA, your doctor may recommend that you are evaluated by a hematologist to determine if you are at an increased risk for cancer and change whether SKYSONA is appropriate for you.

Because of the risk of cancer, it is important for you to be monitored lifelong. At a minimum we recommend blood tests every 3 months for 15 years. Blood tests will look at your blood cell counts and the locations in your blood cells where the gene therapy is inserted. If your blood counts are too low or too high, or if you have lots of cells with the same gene therapy insertion sites, additional testing may be recommended. Additional testing might include more frequent blood tests and a bone marrow evaluation, which can tell your doctor more than blood tests about the health of your bone marrow and if there is cancer forming.

If blood cancer develops quickly or if you have not been having the recommended blood or bone marrow tests, you might experience symptoms of cancer before it is diagnosed. You or your caregiver should call your healthcare provider right away for any of these signs or symptoms:

- Abnormal bruising or bleeding (including nosebleed)
- Severe headache
- Unusual stomach or back pain
- Fever (100.4ºF/38ºC or higher)
- Blood in urine, stool, or vomit
- Coughing up blood
- Swollen glands
- Abnormal tiredness

If you are diagnosed with a cancer, have your treating physician contact Genetix Biotherapeutics at 1-833-999-6378.

SKYSONA may cause life-threatening allergic reactions as it contains DMSO. Please inform your healthcare provider if you have been told that your child has a DMSO allergy or has experienced a reaction after receiving a DMSO-containing product.

It is important that you or your caregiver tell your healthcare providers that you have received SKYSONA.

How will I get SKYSONA?

Before treatment: Your healthcare providers will give you other medicines, including chemotherapy medicine, as part of your treatment before you are given SKYSONA. It's important that you or your caregiver talk to your healthcare providers about the risks and benefits of all medicines involved in your treatment.

After receiving the chemotherapy, it may not be possible for you to father a child. You or your caregiver should consider discussing options for fertility preservation with your doctor before treatment.

STEP 1: SKYSONA is made specifically for you from your own blood stem cells. Your healthcare provider will collect your blood stem cells through a process called mobilization and apheresis. This process takes approximately one week. 'Back-up' stem cells (or 'rescue cells') are also collected and stored at the hospital. This is a precaution in case there is a problem during the treatment process. If a problem happens, your back-up stem cells will be given back to you. If you receive back-up cells, you will have no benefit from SKYSONA.

STEP 2: Your blood stem cells will be sent to a manufacturing site where they are used to make SKYSONA. It takes approximately 51 – 65 days from the time your cells are collected to make and test SKYSONA before it is shipped to your healthcare providers, but the time may vary.

STEP 3: Before you receive SKYSONA, your healthcare providers will give you chemotherapy for a few days to make room in the bone marrow. You will be admitted to the hospital for this step and remain in the hospital until after SKYSONA infusion.

STEP 4: SKYSONA is given by an intravenous infusion (into your vein). You may receive one or two bags of SKYSONA. Each bag is infused in less than 60 minutes.

After SKYSONA infusion, you may stay in the hospital for up to approximately 2 months so that your healthcare team can closely monitor your recovery. Your healthcare providers will determine when you can go home.

What should I avoid after receiving SKYSONA?

- Do not donate blood, organs, tissues or cells.

What are additional possible or reasonably likely side effects of SKYSONA?

- While receiving chemotherapy to prepare your body for SKYSONA: Nausea, vomiting, decreased appetite, constipation, abdominal pain, headache, and rash.
- On the day of treatment with SKYSONA: Life-threatening allergic reaction, nausea, and vomiting.
- Following treatment:
  - **Blood cancer.**
  - **Low blood counts leading to a risk of bleeding and/or infection.** Until your blood counts (platelets, white blood cells, red blood cells) return to safe levels, you may be treated with blood and platelet transfusions and other medicines that prevent bleeding and infection by increasing your blood counts. Most patients' blood counts return to safe levels in about one month after treatment with SKYSONA. Some patients' blood counts may not recover for > 1 year. Tell your healthcare provider right away if you get a fever, are feeling tired, or have easy bleeding or bruising.
  - **Life-threatening infections.** Patients treated with SKYSONA may experience serious or life-threatening infections, including infections of the bloodstream by bacteria or viruses. Infections often occur in the first 1 or 2 months after treatment with SKYSONA, but can occur > 1 year later. Tell your healthcare provider right away if you develop fever, chills, or any signs or symptoms of an infection.
  - Inflamed and painful mouth (typically occurs during the first 2 months after SKYSONA), nausea, vomiting, decreased appetite, constipation, abdominal pain, diarrhea, headache, and new onset seizures.

These are not all the possible side effects of SKYSONA. Call your doctor for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

General Information

SKYSONA treatment failures have been reported and progression of CALD may occur. After treatment SKYSONA you will need to continue follow-up with your doctor for monitoring of clinical and radiographic CALD disease progression.

Treatment with SKYSONA may cause a false-positive human immunodeficiency virus (HIV) test result by some commercial tests. If you need a HIV test, talk with your healthcare provider about the appropriate test to use.

Please see full Prescribing Information, including Boxed WARNING and Medication Guide for SKYSONA.

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ADDITIONAL *safety data*

Safety should always be a consideration for any type of treatment. You need to know how it will interact with someone's body, and what the side effects or risks are.

THE RISK OF GRAFT-VERSUS-HOST DISEASE (GVHD) WITH ALLOGENEIC TRANSPLANTS

With any sort of allogeneic transplant, there's a risk that the body will reject and possibly attack these foreign cells. As a gene therapy, SKYSONA utilizes a child's own blood stem cells to treat cerebral adrenoleukodystrophy. By using their own cells, SKYSONA does not carry the same risk of GVHD commonly associated with transplants of donor blood stem cells.*

*Immunological risks defined here as GVHD and graft rejection only.

In clinical trials, NO PATIENTS TREATED WITH SKYSONA EXPERIENCED GRAFT-VERSUS-HOST DISEASE (GVHD)†

†Acute (≥grade II) GVHD or chronic GVHD.

IMPORTANT SAFETY INFORMATION (cont'd)

What should I avoid after receiving SKYSONA?

- Do not donate blood, organs, tissues or cells.

What are additional possible or reasonably likely side effects of SKYSONA?

- While receiving chemotherapy to prepare your body for SKYSONA: Nausea, vomiting, decreased appetite, constipation, abdominal pain, headache, and rash.
- On the day of treatment with SKYSONA: Life-threatening allergic reaction, nausea, and vomiting.

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THE SKYSONA *treatment journey*

SKYSONA treatment is a complex process, but we can help you understand what lies ahead so that you and your child are prepared for what's next. To start, this treatment process involves coordination among your child's dedicated care team at a **Qualified Treatment Center (QTC)**—a specialized hospital qualified to administer a Genetix Biotherapeutics gene therapy.

All these teams work as one in helping your child as they move through 3 main phases of the treatment journey: pre-treatment, treatment, and post-treatment. Lengths of time shown for each part of the treatment journey are approximates only. As each treatment is made specially for each child, times may vary.

What's a Qualified Treatment Center?

SKYSONA requires highly specialized hospitals with care teams that have transplant experience and experience treating children with cerebral adrenoleukodystrophy (ALD). These hospitals are referred to as QTCs and the care teams at these centers have been specially trained to administer SKYSONA.

It's at these hospitals your child will receive their gene therapy and recover from their treatment.

Visit SKYSONA.com to see all of the SKYSONA QTCs.

BEFORE TREATMENT

Your healthcare providers will give you other medicines, including chemotherapy medicine, as part of your treatment before you are given SKYSONA. It's important that you or your caregiver talk to your healthcare providers about the risks and benefits of all medicines involved in your treatment.

Before your child is treated with SKYSONA, you should have a detailed discussion with their doctor about the risks and benefits of SKYSONA and alternative treatment options. Because of the risk of cancer caused by SKYSONA, your child's doctor may recommend that they're evaluated by a hematologist to determine if they have underlying risk factors that could further increase their risk for cancer and change whether SKYSONA is appropriate for your child.

IMPORTANT SAFETY INFORMATION (cont'd)

What are additional possible or reasonably likely side effects of SKYSONA? (cont'd)

- Following treatment:
  - **Blood cancer.**
  - **Low blood counts leading to a risk of bleeding and/or infection.** Until your blood counts (platelets, white blood cells, red blood cells) return to safe levels, you may be treated with blood and platelet transfusions and other medicines that prevent bleeding and infection by increasing your blood counts. Most patients' blood counts return to safe levels in about one month after treatment with SKYSONA. Some patients' blood counts may not recover for > 1 year. Tell your healthcare provider right away if you get a fever, are feeling tired, or have easy bleeding or bruising.

PRE-TREATMENT

CELL COLLECTION (APPROXIMATELY 1 WEEK)

- Mobilization/apheresis is the procedure where blood is collected from the body. Blood stem cells are then collected to be used for SKYSONA creation
- Your child is brought into a QTC, where your child receives medication that moves their blood stem cells from the bone marrow into their bloodstream (clinically this is referred to as **mobilization**)
- Once the blood stem cells are collected, a machine (known as an **apheresis** machine) separates the blood components to help isolate the specific stem cells needed. More than one day of apheresis and more than one cycle of mobilization may be necessary to collect enough cells to manufacture SKYSONA
- Additional "back-up" stem cells are also collected and stored at the hospital. This is a precaution in case there is a problem during the treatment process. If this happens, back-up stem cells, sometimes called "rescue cells," will be given back to your child. If your child must receive "rescue cells" they will have no benefit from SKYSONA

SKYSONA MANUFACTURING (51-65 DAYS)

- These collected stem cells are then transported to a **manufacturing center**, where they are modified to carry the functional *ABCD1* gene needed to produce ALD protein to break down very long-chain fatty acids and to slow the progression of damage to the brain and slow the decline in neurologic function
- It takes approximately 51–65 days from the time your cells are collected to make and test SKYSONA before it is shipped to your healthcare providers, but the time may vary.
- Once the stem cells have been modified, your child's personalized treatment of SKYSONA is frozen before being shipped back to the QTC

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THE SKYSONA *treatment journey* (CONTINUED)

TREATMENT (PERFORMED AT A QTC)

CONDITIONING (6-8 DAYS)*

*Conditioning days were based off SKYSONA clinical trials.

- This is the process by which your child's body is prepared to receive gene therapy. This involves your child receiving chemotherapy to remove stem cells in their bone marrow to make way for the new cells that will be added
- Before receiving SKYSONA, your child will be admitted to the hospital and will need to undergo conditioning
- Conditioning involves 4-6 days of chemotherapy to make room in the bone marrow for SKYSONA
- After conditioning, your child will need at least 2 days of rest before receiving SKYSONA
- After conditioning, your child will remain in the hospital while they receive SKYSONA
- After receiving the chemotherapy it may not be possible for your child to father a child. Consider discussing options for fertility preservation with your child's doctor before treatment

SKYSONA INFUSION (2 HOURS OR LESS)

- SKYSONA will be infused into your child's bloodstream. Your child may receive 1 or 2 bags. Each bag takes less than 1 hour

RECOVERY (APPROXIMATELY 2 MONTHS)

- Once infused, the SKYSONA blood stem cells engraft into your child's bone marrow and begin producing ALD protein
- Your child will remain in the hospital for roughly an additional 2 months, so that the healthcare provider can monitor their recovery and watch for any side effects that occur

IMPORTANT SAFETY INFORMATION (cont'd)

What are additional possible or reasonably likely side effects of SKYSONA? (cont'd)

- Following treatment:
  - **Life-threatening infections.** Patients treated with SKYSONA may experience serious or life-threatening infections, including infections of the bloodstream by bacteria or viruses. Infections often occur in the first 1 or 2 months after treatment with SKYSONA, but can occur > 1 year later. Tell your healthcare provider right away if you develop fever, chills, or any signs or symptoms of an infection.

POST-TREATMENT

REGULAR FOLLOW-UP CARE (AT LEAST 15 YEARS)

- Once your child's doctor has determined it's safe to do so, your child can return home
- It's possible your child may not feel completely better right away. This is normal. The speed at which they will feel well again varies from child to child
- There will still need to be regular visits with your child's doctors to continue monitoring and managing your child's cerebral adrenoleukodystrophy (ALD)
- Because of the risk of cancer, your child will need lifelong monitoring. Monitoring will include blood tests and additional testing may be recommended. Additional testing might include more frequent blood tests and a bone marrow evaluation, which can tell your child's doctor more than blood tests about the health of their bone marrow and if there is cancer forming. Seek help for any signs or symptoms of cancer, such as fatigue, easy bleeding, or bruising. If your child is diagnosed with a cancer, have your child's treating physician contact Genetix Biotherapeutics at 1-833-999-6378 or medinfo@genetixbiotx.com

REGISTRY (15 YEARS)

- We encourage that anyone treated with SKYSONA be enrolled in the 15-year registry study that collects information on the long-term effects of SKYSONA
- During participation in the registry your child will be monitored, and you are encouraged to continue lifelong follow-up monitoring after the registry ends

Each child has their own journey

This whole treatment process is personalized to meet the needs of each individual child. SKYSONA is made using a child's own cells and can only be used to treat that same child.

If you would like to know more, talk to your child's doctor to understand how SKYSONA is individualized for them.

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life AFTER SKYSONA

Once SKYSONA treatment has been administered, your child can focus on their recovery from treatment. Your child will continue to be monitored for cerebral adrenoleukodystrophy (ALD).

It's important to keep in mind that each child recovers at their own rate as they work to regain their strength. Here's some guidance for when your child returns home after SKYSONA.

DOCTOR CHECK-UPS

Establish regular check-ins with the child's doctor to keep monitoring their cerebral ALD. As a caregiver, you can talk to your child's doctor about how your child's recovering, focusing on both their physical recovery and mental recovery.

Your child will also have lifelong monitoring for blood cancer and will continue to be monitored for cerebral adrenoleukodystrophy. Work with your child's doctor to establish regular follow-up care.

PHYSICAL RECOVERY

The goal is that children will live an active life after treatment with SKYSONA. That said, it's important to always discuss with your child's doctor when they may be able to return to physical activity and what physical activity would be appropriate. Ask your provider if your child should stay away from certain sports where head injuries are more common.

MENTAL RECOVERY

Keep in mind that your child's mental health is also important when receiving gene therapy for cerebral ALD. Please talk with your child's doctor about concerns you may have regarding their mental health.

IMPORTANT SAFETY INFORMATION (cont'd)

What are additional possible or reasonably likely side effects of SKYSONA? (cont'd)

- Following treatment:
  - Inflamed and painful mouth (typically occurs during the first 2 months after SKYSONA), nausea, vomiting, decreased appetite, constipation, abdominal pain, diarrhea, headache, and new onset seizures.

These are not all the possible side effects of SKYSONA. Call your doctor for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

Please see Important Safety Information on pages 10-11, and full Prescribing Information, including Boxed WARNING and Medication Guide.

15-YEAR REGISTRY

By enrolling your child in the 15-year registry study, your child will be monitored.

CAREGIVER CARE

Just because you're taking care of someone doesn't mean you should stop caring for yourself. It's important to focus on yourself in a way that can help reduce your stress and better position yourself to care for another. Some small goals to help you care for yourself could include:

- Participating in activities that bring you joy or peace of mind
- Setting aside time to exercise, care for yourself, and eat healthy
- Knowing when to ask for support from family, friends, or an advocacy organization
  - Some resources for community support include Angel Flight, Ronald McDonald House, and Childhood Neurology Society

Note this list of resources is provided for information only and is not comprehensive.

Talk to your doctor about connecting with an advocacy support group that works for you and your child.

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support STARTS TODAY

RELY ON THE GENETIX PATIENT SUPPORT PROGRAM ALONG THE WAY

Your Patient Navigator will be available to help navigate, educate, and elevate when needed during the decision-making process with your doctor and throughout the treatment process.

NAVIGATE

Guiding you and your loved ones through the treatment journey while connecting you with helpful people and organizations

EDUCATE

Sharing important resources about gene therapy and the benefits information you need to access treatment with insurance

ELEVATE

Collaborating with you to help reach your personal health goals

GENETIX™ Biotherapeutics

For additional support and to connect with a Patient Navigator:

Call 1-833-888-6378

Email patientsupport@genetixbiotx.com

Visit GenetixPatientSupport.com

IMPORTANT SAFETY INFORMATION (cont'd)

General Information

SKYSONA treatment failures have been reported and progression of CALD may occur. After treatment SKYSONA you will need to continue follow-up with your doctor for monitoring of clinical and radiographic CALD disease progression.

Treatment with SKYSONA may cause a false-positive human immunodeficiency virus (HIV) test result by some commercial tests. If you need a HIV test, talk with your healthcare provider about the appropriate test to use.

Please see Important Safety Information on pages 10-11, and full Prescribing Information, including Boxed WARNING and Medication Guide.

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glossary

ABCD1 — The gene responsible for providing instructions for producing the adrenoleukodystrophy protein (ALDP).

ALD protein — This protein (produced by the *ABCD1* gene) is involved in the transport of very long–chain fatty acids in the brain.

Apheresis — The process of separating blood stem cells from blood cells using a machine.

Blood stem cells — Cells found in the bone marrow and circulating blood that develop into different types of blood cells, such as red blood cells, white blood cells, and platelets.

Bone marrow registry — A database of individuals willing and able to donate blood stem cells from their bone marrow to those in need.

Cerebral adrenoleukodystrophy — A type of adrenoleukodystrophy that's focused in the brain and can cause serious neurological problems as a result of a defect in the mobilization of very long–chain fatty acids.

Efficacy — A medication's ability to achieve its desired effect.

Engraftment — The process by which transplanted stem cells travel through the blood to the bone marrow, where they begin to make new white blood cells, red blood cells, and platelets.

Genetic mutation — A change in the sequencing of someone's DNA that could prevent a gene from working properly.

Gene therapy — A method of treating genetic diseases at the genetic level (the source) with the goal of changing the course of a disease.

Immunological — Relating to the immune system, which detects and protects organisms from diseases, pathogens, viruses, and even cancer cells.

Intravenous infusion — The administration of medication or fluids through a needle or catheter directly into a person's vein.

Loes score — A scoring system used to assess the severity and extent of cerebral ALD from white matter lesions observed in an MRI.

Magnetic resonance imaging — A form of medical imaging that uses high-frequency radio waves in a strong magnetic field to produce images of internal organs.

Major functional disabilities — A categorization of 6 severe disabilities commonly attributed to cerebral ALD (loss of communication, cortical blindness, requirement for tube feeding, total incontinence, wheelchair dependence, and complete loss of voluntary movement).

Matched sibling donor — A brother or sister to the patient (or donor recipient) who typically has a high chance of being an optimal donor because there is less risk of the donor's body rejecting the transplantation.

Mobilization — A process in which a medicine is used to get blood stem cells to move out of the bone marrow and into the circulating blood.

Neutrophil — A type of cell that's an essential part of our immune system.

Platelets — Components in the blood that assist in clotting.

Qualified Treatment Center — A hospital that has been carefully selected based on their expertise in areas such as transplant, cell, and gene therapy, and are trained to administer a Genetix Biotherapeutics gene therapy.

Very long–chain fatty acids — These are the fatty acids that can build up in the brain if there's a genetic mutation on the *ABCD1* gene preventing the production of the ALD protein.

talk TO YOUR CHILD'S DOCTOR ABOUT SKYSONA

This guide is full of information to help you learn what your child's journey may look like with SKYSONA. That said, it's always important to ask your child's doctor to answer any specific questions regarding treatment.

Here are some questions you may want to consider in order to have a productive conversation with your child's doctor about what the journey ahead may look like.

PRIOR TO TREATMENT

- Is my child a candidate for SKYSONA?
- How could SKYSONA affect my child's condition?
- Can you tell me more about the clinical results for SKYSONA?
- Can you tell me more about the safety profile for SKYSONA?

DURING TREATMENT

- What support will my child need when SKYSONA begins?
- What's my role as my child recovers from treatment?

AFTER TREATMENT

- How should I educate my child so they're able to continue managing and monitoring their condition as they grow up?

Please see Important Safety Information on pages 10-11, and full Prescribing Information, including Boxed WARNING and Medication Guide.

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GENETIX IS *committed* TO PATIENTS AND CAREGIVERS

LEARN MORE ABOUT SKYSONA AT SKYSONA.COM

Please see Important Safety Information on pages 10-11, and full Prescribing Information, including Boxed WARNING and Medication Guide.

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