

**KOSELUGO® (selumetinib) is the FIRST and ONLY FDA-approved prescription medicine that is used to treat children 2 years of age and older with neurofibromatosis type 1 (NF1) who have plexiform neurofibromas that cannot be completely removed by surgery. It is not known if Koselugo is safe and effective in children under 2 years of age.**

**LESS < > MORE**  
TUMOR SIZE ME

— Cooper, age 8, actual patient

## YOUR TREATMENT GUIDE FOR KOSELUGO

We know that a diagnosis of NF1 plexiform neurofibromas can affect the entire family. As you and your family embark on treatment with Koselugo, we're here to provide you with the support you need, every step of the way.

This treatment guide is designed to help your family throughout treatment with Koselugo. You can expect important information on:

- What NF1 plexiform neurofibromas are
- What Koselugo is
- What tests are needed prior to taking Koselugo
- What to expect while taking Koselugo

Take time to go through this brochure with your child's doctor. More resources are available in the Starter Kit or at [Koselugo.com](https://www.koselugo.com).

### IMPORTANT SAFETY INFORMATION

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

- have heart problems
- have eye problems
- are pregnant or plan to become pregnant. Koselugo can harm your unborn baby
  - Females who could become pregnant and males with female partners who could become pregnant should use effective birth control (contraception) during treatment with Koselugo and for 1 week after your last dose
  - Tell your healthcare provider right away if you become pregnant or think you may be pregnant during treatment with Koselugo
- are breastfeeding or plan to breastfeed. It is not known if Koselugo passes into your breast milk
  - Do not breastfeed during treatment with Koselugo and for 1 week after your last dose
  - Talk to your healthcare provider about the best way to feed your baby during this time

**Please read additional Important Safety Information throughout. Please see full Prescribing Information, including Patient Information, by clicking [here](#) or at [Koselugo.com](https://www.koselugo.com).**



**Koselugo®**  
(selumetinib)  
10 mg & 25 mg capsules



### WHAT ARE PLEXIFORM NEUROFIBROMAS?

**30%-50%**

Plexiform neurofibromas may appear in 30%-50% of children with NF1



Plexiform neurofibromas grow along nerves and can be located anywhere in the body



They can be large or small in size and continue to grow if left untreated

### POSSIBLE SIGNS OF SYMPTOMATIC PLEXIFORM NEUROFIBROMAS



#### PHYSICAL CHANGES

Have a cluster of small lumps formed under your child's skin?

Have you noticed any skin discoloration?

Have any existing lumps become visible on the surface of the skin?



#### DIFFICULTY MOVING

Is your child having difficulty moving any part of their body?

Does your child get tired more often?

Can your child sleep comfortably?

Is your child experiencing any tingling or stinging sensations?



#### PAIN

Is your child no longer doing their usual activities?

Is your child fussier than usual?

Does your child seem bothered or uncomfortable?



#### OTHER SYMPTOMS

Is your child having trouble breathing?

Is your child experiencing vision changes or other eye problems?

Is your child having bowel or bladder problems?

**Remember—if your child experiences a new plexiform neurofibroma symptom, or if a symptom your healthcare team knows about has gotten worse, report it as soon as possible.**



### WHY CAN'T SOME PLEXIFORM NEUROFIBROMAS BE COMPLETELY REMOVED WITH SURGERY?

Surgery for NF1 plexiform neurofibromas may not be recommended by your child's healthcare provider. One reason is plexiform neurofibromas may be closely intertwined with critical nerves and located near major organs. Also, plexiform neurofibromas may not be completely removed by surgery.

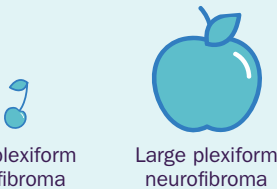
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**PLEXIFORM NEUROFIBROMAS CAN APPEAR IN DIFFERENT SIZES AND LOCATIONS IN THE BODY**

A child with NF1 may develop 1 or several plexiform neurofibromas. These tumors can grow on any nerve of the body and can be small or large in size.

**PLEXIFORM NEUROFIBROMAS CAN BE SMALL OR LARGE**

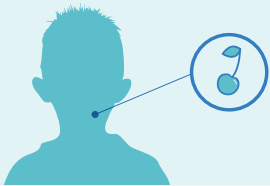


Small plexiform neurofibroma

Large plexiform neurofibroma

Plexiform neurofibromas start small but can grow and become large.

**THE LOCATION OF PLEXIFORM NEUROFIBROMAS MAY BE IMPORTANT**



Plexiform neurofibromas of any size may cause symptoms. If your child develops a tumor, it may or may not be completely removed by surgery.

◀ **Keeping an eye out for plexiform neurofibroma symptoms can be an important way to help your child during their NF1 journey** ▶

**IMPORTANT SAFETY INFORMATION (cont'd)**

**Tell your healthcare provider about all the medicines you take**, including prescription and over-the-counter medicines, vitamins, or herbal supplements. Especially tell your healthcare provider if you are taking aspirin, blood thinners, or other medicines to treat blood clots. Koselugo contains vitamin E, which may increase your risk of bleeding.

**Koselugo may cause serious side effects, including:**

**Heart problems.** Koselugo can lower the amount of blood pumped by your heart, which is common and can also be severe. Your healthcare provider will do tests before and during treatment with Koselugo to check how well your heart is working. Tell your healthcare provider right away if you get any of the following signs or symptoms:

- persistent coughing or wheezing
- shortness of breath
- swelling of your ankles and feet
- tiredness
- increased heart rate

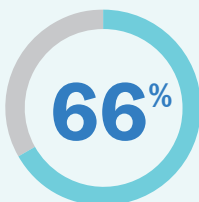
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## THE FIRST FDA-APPROVED ORAL TREATMENT FOR CHILDREN WITH NF1 PLEXIFORM NEUROFIBROMAS



### HOW MIGHT KOSELUGO HELP MY CHILD?



- Koselugo has been proven to shrink plexiform neurofibromas in children with NF1
- In a clinical study, 66% of 50 children responded to Koselugo, meaning that these children had a 20% or more decrease in their tumors' size, confirmed by 3D magnetic resonance imaging scan
- 82% of 33 children who responded to Koselugo continued to maintain the decrease in their tumors' size from treatment for at least 1 year

Actor portrayal.

Please read additional Important Safety Information throughout. Please see full Prescribing Information, including Patient Information, by clicking [here](#) or at [Koselugo.com](https://www.koselugo.com).

## THE FIRST FDA-APPROVED ORAL TREATMENT FOR CHILDREN WITH NF1 PLEXIFORM NEUROFIBROMAS (CONT'D)

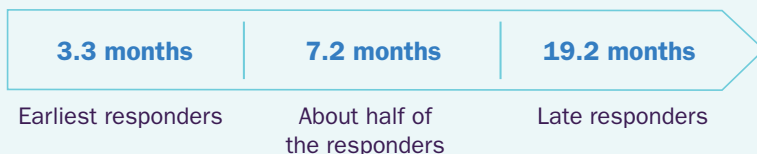


### WHEN DID CHILDREN RESPOND TO KOSELUGO IN THE CLINICAL STUDY?

**For children who responded in the clinical study, some had confirmed plexiform neurofibroma shrinkage in as early as 3 months**

Of the 33 children who responded to Koselugo treatment in the clinical trial, about one-half had their tumor volume shrink by at least 20% within 7.2 months. The time that it took for a response to be seen ranged from 3.3 months to 19.2 months.

#### The range of patient response time in the clinical study



**Response to Koselugo may occur at different times, so it's important that your child keeps taking their medicine as instructed by their doctor**

### IMPORTANT SAFETY INFORMATION (cont'd)

**Koselugo may cause serious side effects, including (cont'd):**

**Eye problems.** Koselugo can cause eye problems that can lead to blindness. Your healthcare provider will check your vision before and during treatment with Koselugo. Tell your healthcare provider right away if you get any of the following signs or symptoms:

- blurred vision
- loss of vision
- dark spots in your vision (floaters)
- other changes to your vision

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## WHAT ARE POSSIBLE **SIDE EFFECTS** OF KOSELUGO?



### THE MOST COMMON SIDE EFFECTS OF KOSELUGO ARE:



Vomiting



Feeling of tiredness, weakness, or lacking energy



Headache



Stomach pain



Muscle and bone pain



Redness around the fingernails



Nausea



Fever



Itching



Dry skin



Inflammation of the mouth

### These are not all the possible side effects of Koselugo.

As with any medicine, it's possible to experience side effects while taking Koselugo.

Be sure to tell your child's doctor if your child experiences any side effect that bothers them or does not go away.

Remember, the information on this page does not replace medical guidance or directions from your doctor. Always talk to them about side effects and ways to help manage them

### IMPORTANT SAFETY INFORMATION (cont'd)

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

**Severe diarrhea.** Diarrhea is common with Koselugo and can also be severe. Tell your healthcare provider right away the first time that you get diarrhea during treatment with Koselugo. Your healthcare provider may give you medicine to help control your diarrhea and may tell you to drink more fluids.

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### KOSELUGO MAY CAUSE SERIOUS SIDE EFFECTS, INCLUDING:

**Heart problems:** Koselugo can lower the amount of blood pumped by your heart, which is common and can also be severe. Your child's healthcare provider will do tests before and during treatment with Koselugo to check how well your child's heart is working. Tell your child's healthcare provider right away if your child gets any of the following signs or symptoms:

- persistent coughing or wheezing
- tiredness
- shortness of breath
- increased heart rate
- swelling of your child's ankles and feet

**Eye problems:** Koselugo can cause eye problems that can lead to blindness. Your child's healthcare provider will check your child's vision before and during treatment with Koselugo. Tell your child's healthcare provider right away if your child gets any of the following signs or symptoms:

- blurred vision
- loss of vision
- dark spots in your child's vision (floaters)
- other changes to your child's vision

**Severe diarrhea:** Diarrhea is common with Koselugo and can also be severe. Tell your child's healthcare provider right away the first time that your child gets diarrhea during treatment with Koselugo. Your child's healthcare provider may give your child medicine to help control diarrhea and may tell them to drink more fluids.

**Skin rash:** Skin rashes are common with Koselugo and can also be severe. Tell your child's healthcare provider if your child gets any of the following signs or symptoms:

- rash that covers a large area of your child's body
- blisters
- peeling skin

**Muscle problems (rhabdomyolysis):** Muscle problems are common with Koselugo and can also be severe. Treatment with Koselugo may increase the level of a muscle enzyme in your child's blood, which may be a sign of muscle damage. Your child's healthcare provider should do a blood test to check your child's muscle enzyme levels before they start taking Koselugo and during treatment. Tell your child's healthcare provider right away if your child gets any of the following signs or symptoms:

- muscle aches or pain
- dark, reddish urine
- muscle spasms and weakness

**Your child's healthcare provider may change your child's dose, temporarily stop, or permanently ask your child to stop taking Koselugo if they have any of these side effects.**

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## HOW ARE **TWICE-DAILY KOSELUGO CAPSULES** TAKEN?



**DOSES SHOULD BE TAKEN—ON AN EMPTY STOMACH—  
APPROXIMATELY THE SAME TIME EACH DAY, ABOUT 12 HOURS APART**



**FIRST DOSE**



**SECOND DOSE**



- **Your child must take Koselugo on an empty stomach**, which means your child should not eat 2 hours before each dose or 1 hour after each dose

**AT LEAST 2 HOURS  
BEFORE A DOSE**



**AT LEAST 1 HOUR  
AFTER A DOSE**

- **Swallowed whole with water:** Do not chew, dissolve, or open capsule

- **If your child misses a dose**, they should take it as soon as you remember. If it is less than 6 hours before the next scheduled dose, give the next dose at the regular time. Do not make up for the missed dose
- **If your child vomits at any time after taking Koselugo**, they should not take an additional dose. The next dose should be taken at the regular time

### **IMPORTANT SAFETY INFORMATION (cont'd)**

**Koselugo may cause serious side effects, including (cont'd):**

**Skin rash.** Skin rashes are common with Koselugo and can also be severe. Tell your healthcare provider if you get any of the following signs or symptoms:

- rash that covers a large area of your body
- peeling skin
- blisters

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**It's important to  
GIVE KOSELUGO EXACTLY AS YOUR CHILD'S DOCTOR PRESCRIBES  
AND KEEP TO A DOSING SCHEDULE THAT FITS BEST WITH YOUR  
CHILD'S DAILY ROUTINE**

**The goal is to make it easy to stay on track to get the most out of treatment**

- Do not change the dose or stop giving Koselugo unless directed by your child's doctor
- Your child's doctor may change the dose, temporarily stop, or permanently stop treatment if your child has side effects
- Your child's doctor will decide on the right dose of Koselugo based on your child's weight or size (body surface area) and how many capsules of Koselugo to take

Actor portrayal.

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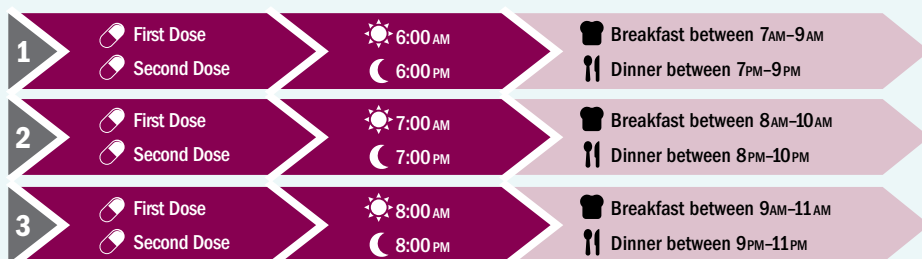
**Koselugo**  
(selumetinib)  
10 mg & 25 mg capsules



### TIMING MAY BE EVERYTHING

Work with your child's doctor to establish a dosing routine that can be easily followed. Taking each dose of Koselugo as directed, every day, is the best way to benefit from treatment.

#### Sample dosing schedules to share with your child's doctor



These sample schedules are designed as suggestions only.

You may want to use a dose-alert timer, or the alarm on your smartphone, as a reminder to take the medicine on time as part of your child's daily dosing routine.

Check the Starter Kit for your dose-alert timer.



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### SUGGESTIONS TO HELP GIVE MEDICINE TO YOUNG CHILDREN (3 TO 12 YEARS OLD)

- Role-playing can be helpful: Pretending to give a favorite doll or teddy bear some medicine can make taking medicine more fun
- Try to do a positive, fun activity after your child takes the medicine. This may help motivate your child to take the medicine again

For some helpful tips to teach your child how to swallow pills, you may want to download the brochure **Medicine & Your Child: A Guide for Parents on Adherence and Administration** at <https://ccr.cancer.gov/sites/default/files/medbooklet.pdf> or visit [www.kidshealth.org](http://www.kidshealth.org) to see **Teaching Your Child How to Swallow Pills** for additional suggestions.

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### IMPORTANT SAFETY INFORMATION (cont'd)

**Koselugo may cause serious side effects, including (cont'd):**

**Muscle problems (rhabdomyolysis).** Muscle problems are common with Koselugo and can also be severe. Treatment with Koselugo may increase the level of a muscle enzyme in your blood, which may be a sign of muscle damage. Your healthcare provider should do a blood test to check your muscle enzyme levels before you start taking Koselugo and during treatment. Tell your healthcare provider right away if you get any of the following signs or symptoms:

- muscle aches or pain
- muscle spasms and weakness
- dark, reddish urine

Your healthcare provider may change your dose, temporarily stop, or permanently ask you to stop taking Koselugo if you have any of these side effects.

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## BEFORE STARTING TREATMENT WITH KOSELUGO



### YOUR CHILD WILL TAKE 4 TESTS BEFORE STARTING KOSELUGO

- A test using sound waves to create pictures of the heart (called an echocardiogram)
- A blood test for certain enzymes and naturally occurring chemicals
- An assessment of your child's eyes
- A pregnancy test for females of reproductive potential



### BEFORE STARTING KOSELUGO, TELL YOUR CHILD'S DOCTOR ABOUT ALL OF YOUR CHILD'S MEDICAL CONDITIONS, INCLUDING IF THEY:



Have heart problems



Are pregnant or plan to become pregnant. Koselugo can harm an unborn baby



Have eye problems



Are breastfeeding or plan to breastfeed

Tell your child's healthcare provider about all the medicines your child takes, including prescription and over-the-counter medicines, vitamins, or herbal supplements. Especially tell your child's healthcare provider if your child is taking aspirin, blood thinners, or other medicines to treat blood clots. Koselugo contains vitamin E, which may increase the risk of bleeding.



### MAKE A LIST OF QUESTIONS YOU'D LIKE TO ASK YOUR CHILD'S DOCTOR AND INFORMATION YOU'D LIKE TO SHARE ABOUT YOUR CHILD

**1**

Ask what **tests are needed** while on treatment

**2**

Share details **about pre-existing conditions**

**3**

Provide names of **other medicines** your child may be taking

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### SOME ORGANIZATIONS THAT CAN HELP YOU CONNECT WITH THE NF1 COMMUNITY AND LEARN VALUABLE INFORMATION ABOUT THE DISEASE



The mission of the Children's Tumor Foundation is to drive research, expand knowledge, and advance care of the NF community. Learn more by visiting their website at [www.ctf.org](http://www.ctf.org).



The Littlest Tumor Foundation is focused on increasing the understanding of neurofibromatosis (NF), empowering affected families, and advancing research of preventative therapies. Find out more on their website at [www.littlesttumor.org](http://www.littlesttumor.org).



Visit the Neurofibromatosis Network website at [www.nfnetwork.org](http://www.nfnetwork.org) to discover news about treatment and their work toward a neurofibromatosis cure. They provide outreach through education and awareness, while offering hope and support to those affected by NF.

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The resources listed above are independent, nonprofit patient advocacy organizations. Their listing in this brochure does not imply endorsement.

### IMPORTANT SAFETY INFORMATION (cont'd)

#### The most common side effects of Koselugo are:

- vomiting
- muscle and bone pain
- stomach pain
- fever
- nausea
- inflammation of the mouth
- dry skin
- headache
- feeling of tiredness, weakness, or lacking energy
- redness around the fingernails
- itching

These are not all the possible side effects of Koselugo.

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## WHAT TO EXPECT WHILE TAKING KOSELUGO



### WHAT CHILDREN EXPERIENCED IN THE KOSELUGO CLINICAL TRIAL DURING THE FIRST YEAR

The chart below lists side effects children experienced while on Koselugo in the clinical trial. Please keep in mind that Koselugo may cause side effects other than those listed below and that timing may vary.

#### Tumor shrinkage\*

was observed as early as 3 months and as late as 19.2 months

##### +1 to 12 weeks

- Acne-like rash
- Nausea
- Dry mouth
- Increased muscle enzyme
- Diarrhea
- Vomiting
- Fatigue

##### +3 months

- Mouth sores
- Dry skin

##### +4 to 8 months

- Hair color changes
- Decrease in blood pumped by the heart†
- Fever

##### +8 months

- Redness around the fingernails

Let your child's doctor know right away about any side effects your child may experience.

#### Having the right support can make all the difference in your child's treatment journey.

Please see the back cover to learn more about the dedicated Patient Access Navigators from Alexion OneSource™ who are ready to connect caregivers and children to personalized support they need.



### USE THE TREATMENT JOURNEY TRACKER

Use the Treatment Journey Tracker to track your child's progress and any side effects that may occur. Be sure to share your child's experiences with their doctor.

**Ask your child's doctor for the Koselugo Starter Kit**

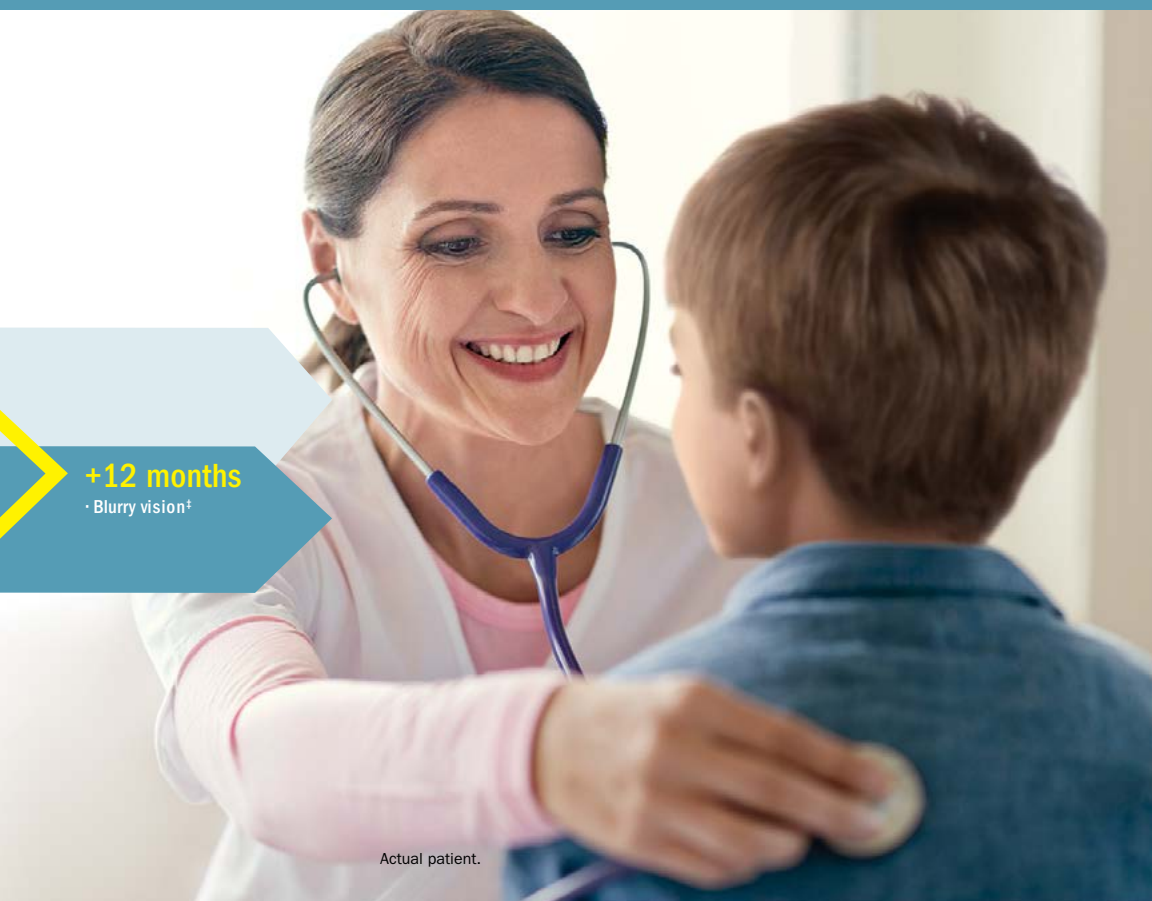
Use the Treatment Journey Tracker in the kit or download a copy at [www.Koselugo.com/resources](http://www.Koselugo.com/resources)

\*Of the 33 children that responded to Koselugo treatment in the clinical trial, one-half saw their tumor volume shrink by at least 20% within 7.2 months. The time of onset of response ranged from 3.3 months to 19.2 months.

†Koselugo can lower the amount of blood pumped by the heart. Your child's doctor should do tests before and during treatment to check how well your child's heart is working.

Koselugo can cause eye problems. Tell your child's doctor as soon as possible if your child's vision blurs or if any other changes to their sight occur during treatment.

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**+12 months**

• Blurry vision\*

Actual patient.

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- have heart problems
- have eye problems
- are pregnant or plan to become pregnant. Koselugo can harm your unborn baby
  - Females who could become pregnant and males with female partners who could become pregnant should use effective birth control (contraception) during treatment with Koselugo and for 1 week after your last dose
  - Tell your healthcare provider right away if you become pregnant or think you may be pregnant during treatment with Koselugo
- are breastfeeding or plan to breastfeed. It is not known if Koselugo passes into your breast milk
  - Do not breastfeed during treatment with Koselugo and for 1 week after your last dose
  - Talk to your healthcare provider about the best way to feed your baby during this time

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(selumetinib)  
10 mg & 25 mg capsules





## HELPING YOUR CHILD ACCESS THE CARE THEY NEED

OneSource™ is a free, personalized patient support program offered by Alexion. Whether your child is newly diagnosed or has had their condition for some time, our specialists will be by your side. We can help you make sense of your health insurance coverage, answer questions about your child's treatment with Koselugo, and connect you to community resources.

**We're committed to helping your child start and stay on track with their prescribed treatment.**



### OneSource™ Support Services

Your dedicated Patient Access Navigator is here to guide and give you the support you deserve—whatever your care plan may be. They can:

- Answer questions about neurofibromatosis type 1 (NF1) plexiform neurofibromas (PN), Koselugo, your insurance coverage, and more
- Provide support during treatment
- Help you avoid interruptions in your child's treatment during insurance changes, travel plans, or other life events
- Help you get involved with the rare disease community through events and meetings



### Health insurance can be complicated. We're here to help make sense of it all.

OneSource can help by:

- Providing information that explains your insurance coverage for Koselugo
- Addressing financial concerns or gaps in coverage



To learn more or contact your dedicated Patient Access Navigator, please call **1-888-765-4747**, Monday through Friday, 8:30 AM–8:00 PM ET, or visit [www.AlexionOneSource.com](http://www.AlexionOneSource.com).

**Ask your doctor if Koselugo is right for your child.**

For more information, visit [www.Koselugo.com](http://www.Koselugo.com).



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