KOSELUGO ™ FAQ

Learn more about **KOSELUGO ™**, also known as selumetinib, a drug approved in April of 2020 for the treatment of plexiform tumors, a complication of neurofibromatosis type 1.

1. **What is KOSELUGO ™ (selumetinib) prescribed for?**
   KOSELUGO ™ (selumetinib) is approved for the treatment of children two years of age and older with neurofibromatosis type 1 (NF1) who have plexiform neurofibromas (PN) that cause symptoms and cannot be completely removed by surgery or for which surgery is not advisable due to potential morbidity (unwanted side effects) with surgery.

2. **Will KOSELUGO ™ reduce the size of my plexiform tumor?**
   In clinical trials led by the National Cancer Institute (NCI), a division of the National Institutes of Health (NIH) and conducted at several pediatric hospitals, roughly 70% of the children enrolled on the studies had reduction in size of their tumors by 20-60% on volumetric MRI. Many patients also reported a reduction in pain, improved physical function, improved mobility, and an improvement in their emotional and psychological well-being. Even without a reduction in size, many patients reported a reduction in pain. It's important to recognize that KOSELUGO ™ does not work for everyone and it is a chemotherapy type medication that has many associated adverse effects that require regular appointments to monitor (including heart, eye and blood surveillance). A clinical trial in adults with plexiform neurofibromas is ongoing and preliminary results have shown disease response in this patient population as well, but the study is ongoing.

3. **How is KOSELUGO ™ (selumetinib) administered?**
   It is taken as capsules taken by mouth twice a day on an empty stomach. No food should be taken two hours before or one hour after taking KOSELUGO ™. Other formulations are being developed.

4. **What are the side effects of KOSELUGO ™?**
   Some of the more common side effects include rash (many types and appears to be worse in adolescents and adults), abdominal pain, diarrhea, nausea, vomiting, dry skin, fatigue, musculoskeletal pain, pyrexia (fever), stomatitis (mouth sores), headache, paronychia, and pruritus. Your doctor will also monitor you for rare but potentially serious side effects effecting sudden loss of vision and changes in heart function.

5. **How do I get this drug prescribed for me or my child?**
   This drug is only currently approved for people between the ages of 2-18 with NF1 and symptomatic plexiform neurofibromas for which surgery is not feasible or advisable. It's best to talk with your NF specialist about if your child meets these criteria and if this treatment is advisable for your child. For people who do not meet the FDA indication, there still may be an opportunity to try this drug, however, this requires accessing the drug "off-label" and you are likely to need a specialist to guide that process. NF specialists can be identified through nfcollective.org website.

6. **Can an adult get this drug prescribed?**
   KOSELUGO TM (selumetinib) is approved by the FDA for use on children ages 2 and up, however your doctor may prescribe it "off label". Off-label prescribing is when a provider prescribes a drug for you that the U.S. Food and Drug Administration (FDA) has approved to treat a condition different than your condition. This practice is legal and common; however, it is a multi-step process. First, it requires that the prescriber has enough expertise to judge that it is medically appropriate for the patient's unique circumstances and that they can oversee the safety monitoring and treatment of side effects appropriately. Secondly, it can have implications for third party payer coverage. In some cases, insurance companies will not pay for access to the drug when it is off label. A helpful resource about off-label prescribing in general is:
   https://www.fda.gov/patients/learn-about-expanded-access-and-other-treatment-options/understanding-unapproved-use-approved-drugs-label

7. **Will my insurance cover the cost of this drug?**
   Many insurance companies will have a coverage policy that is specific to the FDA labeling for a particular drug. However, the FDA does not control what drugs insurance companies may cover or to what extent the drug may be covered. Hence, the cost of the drug may be covered to a greater or lesser extent by various policies. The drug manufacturer, AstraZeneca, provides some assistance policies that can be studied at www.myaccess360.com/patient.html.

8. **Will KOSELUGO ™ help in the treatment of other complications of NF1?**
   There are multiple active studies or studies planned to evaluate the effect of KOSELUGO ™ or other MEK inhibitors on various complications of NF. However, MEK inhibitors are not being considered as a cure. Researchers are looking at the effect of KOSELUGO ™ and/or other MEK inhibitors on adults with plexiform tumors as well as on gliomas, cutaneous neurofibromas, and learning disabilities in NF1. Look for possible trials at clinicaltrials.gov.

9. **What about NF Type 2 and Schwannomatosis?**
   There's not currently any clinical evidence that KOSELUGO ™ will be effective for these conditions, but this will be best determined in potential future clinical trials.

10. **How can I learn more about KOSELUGO ™?**
    The most important thing is to discuss this drug with your NF specialist. You may also find more information about KOSELUGO ™ here. An addition, NF Midwest, NF Northeast, Texas NF and other NF organizations can put you in contact with patients who have taken KOSELUGO ™ or other MEK inhibitors.

11. **What about other MEK Inhibitors?**
    Research is currently ongoing on other MEK inhibitors including binimetinib, mirdametinib and trametinib and more studies may be done in the future. Please check clinicaltrials.gov for additional information. By participating in clinical trials, you are improving the overall knowledge about the best drugs for various NF manifestations, getting the attention and care of the study team as well as your regular medical team and often getting the drug without expense.

Find MUCH More Information Here