FDA Approval of Koselugo (selumetinib) - Frequently Asked Questions

What is the big news exactly?
The U.S. Food and Drug Administration (FDA) approved Koselugo (selumetinib) for use in patients with inoperable plexiform neurofibromas, a common manifestation in the disease neurofibromatosis type one (NF1). The FDA's approval is a major milestone for patients living with neurofibromatosis (NF), a genetic disorder that causes tumors to grow on nerves throughout the body.

Why is this important?
This announcement is the first ever approved treatment for NF, and portends the potential for the development of treatment options for all NF patients.

How do I access Koselugo (selumetinib) for my use? How do I know if it'll work for me? Can my doctor prescribe it for me?
Please contact your doctor directly about taking Koselugo (selumetinib). Because each NF patient has unique medical needs and priorities, it is important that you speak to you trusted medical professional.

I'd like to learn more about Koselugo (selumetinib) and its potential for me. Can you help?
Yes, please read this document authored by our clinical team here.

Will I be able to access Koselugo (selumetinib) during this time of COVID-19?
Doctors are following the Centers for Disease Controls and Prevention (CDC) guidelines regarding social distancing and essential medical procedures. Please consult your doctor about availability.

Can I access this drug from outside the United States?
Koselugo (selumetinib) is approved only for use in the United States, but AstraZeneca is applying for use in other countries. We'll announce more on this when the time comes.

Where can I learn about other patients who have taken selumetinib?
We have posted MEK success stories (videos and write-ups) here.

I don't have NF1, or plexiform neurofibromas. Is this relevant for me?
Yes, absolutely! While Koselugo (selumetinib) is approved for one subset of NF1 patients, news of its progressive success has generated much interest in NF by researchers both within and external to the NF field, as well as pharmaceutical and biotech companies. Other MEK inhibitor drugs are currently in clinical trial for varying conditions of NF, and this increased interest is generating buzz – and investment – into NF2 and schwannomatosis as well. This is just the first victory on the way to ending all forms of NF.

What is on the horizon for patients with NF2 and Schwannomatosis?
The approval of Koselugo (selumetinib) is just the beginning of what's to come for patients with all manifestations with NF, including those living with NF2 and schwannomatosis. In fact, we are close to launching a new clinical trial this spring for patients with NF2, and there is a new drug in clinical trial for schwannomatosis. In fact, there are currently 14 drugs in clinical trials specifically for NF2 and schwannomatosis.

How can I stay informed about future medical developments?
If you are an NF patient, please join the NF Registry in order to stay informed of future clinical trials and to help researchers learn more about NF.

If you'd like to be added to the Children's Tumor Foundation mailing list, please contact us at info@ctf.org.

What was the role of the Children's Tumor Foundation in the FDA approval?
The first use of MEK inhibitors as a potential treatment for NF tumors came from early-stage discoveries by Children's Tumor Foundation (CTF)-funded researchers, who showed that MEK inhibitors could significantly affect NF tumor size. Positive early clinical results were first reported at CTF's annual scientific NF Conference in 2015, as well as in subsequent publications in the *New England Journal of Medicine* in 2016 and 2020.

Collaborative efforts among the NCI, the NIH, the NFRP-CDMRP (Neurofibromatosis Research Program of the Congressionally Directed Medical Research Programs), NTAP (Neurofibromatosis Therapeutic Acceleration Program), and CTF ensured that this 'MEK Story' proceeded expeditiously through proactive and strategic coordination, guaranteeing efficient use of donor/investor funding, including from the federal government.

Another hallmark of this path to approval has been the inclusion of patients throughout the process, including the first ever 'NF listening session' held at the FDA in 2019. Many other MEK inhibitors are also now in clinical trial, including mirdametinib from SpringWorks Therapeutics, a company which the Children's Tumor Foundation helped spin off from Pfizer.

I want to help. How can I make sure that more treatments are developed for NF patients?

Thank you, and yes, you can help by funding NF research at **ctf.org/donate**.

The pioneering research outlined above took place because of generous donors and investors who believe in the work we are doing and the researchers we support. There is still much work to be done. As we've increased our knowledge of NF, we've also increased the pool of possible drug treatments that will work for patients living with NF and ultimately providing hope. Fortunately, increased interest in NF from pharma and biotech, combined with the collaborative nature of NF researchers means that future research will be even more targeted and efficient.

You can learn more about this work by visiting ctf.org.

I have other questions that have not been answered here. Can you help?
Please contact the Children's Tumor Foundation at info@ctf.org or 1-800-323-7938. Please note that we cannot provide medical advice.