

MEK Makes a Difference

Koselugo: First FDA-approved treatment for NF

The U.S. Food and Drug Administration (FDA) announced the approval of Koselugo (selumetinib) for use in patients with inoperable plexiform neurofibromas, a common manifestation in neurofibromatosis type one (NF1). The FDA's approval of AstraZeneca's and MSD (Merck)'s submission was a major milestone for patients living with neurofibromatosis. Koselugo is the first-ever approved treatment for NF, and portends the potential for the development of more treatment options for patients living with all types of NF.

Announced in April of 2020, Koselugo's approval followed comprehensive clinical testing of the drug in patients at the National Cancer Institute (NCI), a division of the National Institutes of Health (NIH). In those clinical trials, over 70% of NF patients with inoperable plexiform neurofibromas saw tumor size reduction anywhere from 20–60% in size. In addition to both visible and actual tumor reduction, patients reported higher-quality physical function, reduced pain, improved mobility, and enhanced emotional and psychological status.

The first use of MEK inhibitors as a potential treatment for NF tumors came from early-stage discoveries by Children's Tumor Foundation-funded researchers, who showed that MEK inhibitors could significantly affect NF tumor size. 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, and support from the federal government.

“CTF has funded groundbreaking preclinical work and early clinical studies that have moved forward into treatment trials, and ultimately led to the first FDA-approved drug for NF1.”

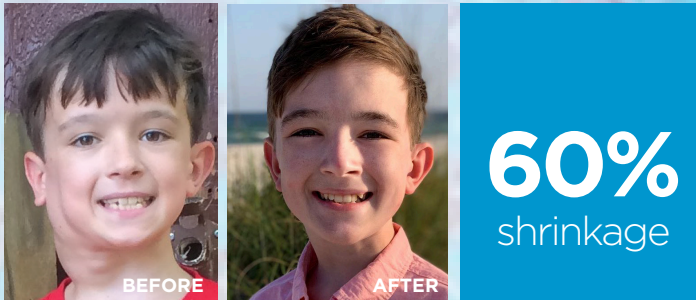
— Nicole Ullrich, MD, PhD, Boston Children's Hospital/Harvard Medical School

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 trials, including mirdametinib from SpringWorks Therapeutics, a company which the Children's Tumor Foundation helped spin off from Pfizer. Our partners at NFlection are also working on a clinical trial with a topical MEK inhibitor for patients with cutaneous neurofibromatosis type 1.

Since the 2020 announcement, Koselugo has been approved for the treatment of NF1 patients in more than 30 countries. The AstraZeneca group of companies acquired Alexion, a global biopharmaceutical company focused on rare disorders. Alexion now distributes Koselugo throughout the world, adding to the growing list of partners working with the Children's Tumor Foundation to end NF.

For more information, go to ctf.org/mek

Koselugo: Stories of the Road to Approval



“People don’t ask me what is wrong with my neck anymore. The drug does make me fatigued, which is tough since most kids my age are active in sports or physical activities that are challenging for me. But I’ve found activities that I enjoy, and friends that enjoy being a part of my life. I enjoy reading, gaming, boy scouts, coding, and more. I’m getting ready to start high school and I’m thankful for all the donors that funded the doctors and researchers who made selumetinib possible. Now that it is FDA approved, I am thankful that others may experience what I have experienced.”

— NF Hero Philip Moss

“I am thankful for the Children’s Tumor Foundation’s enormous efforts to support the research that led to such a trial. Now that selumetinib has been approved as the first-ever FDA-approved treatment, I am thrilled that now other people with plexiform neurofibromas will have access to and can benefit from selumetinib; proud that Jane was one of the first 24 children in the world to take selumetinib. She has seen it through from a Phase I trial to FDA approval, and her experiences, both good and bad, with the medication have helped guide researchers on how best to use it. I am relieved that all of Jane’s hard work—all the trips to the NIH, all the blood draws and MRIs and other tests, all the side effects and uncertainty—has paid off and will benefit others.” — NF Hero Jane Constable, as told by her mom, Kristy



“We are so thankful that the Children’s Tumor Foundation invested in the science that made the clinical trial for selumetinib possible! We are incredibly thankful that all NF Heroes will now have access to the drug that changed Cooper’s life. We shudder to think of what would have happened if Cooper’s tumor had continued to grow. Before starting the drug, his tumor was nearly doubling in volume every 18 months. Since starting selumetinib, his tumor has not only stopped growing, it has shrunk 21%! Our hope is that it will be as life-changing for others as it has been for our family.”

— NF Hero Cooper, as told by Cooper’s mom, Kirsta

For more
information, go to
ctf.org/mek

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