MEK



MAKES A DIFFERENCE

BEFORE



NF HERO: PHILIP MOSS

"[Since being on the trial] fast forward to the start of fifth grade in 2016 when Philip returned to the new school year, and virtually no one asked him about his neck because the tumor is barely visible anymore! Sometimes, it's the little things that make a huge difference when living with NE."

-Renie Moss, Philip's Mother

60% shrinkage

NF HERO: PAIGE DOANE

32% shrinkage

"Paige was diagnosed at six months old with NF1...the tumor [on her face] goes from her eyebrow to behind her ear, down her throat, and then comes up under her tongue. In August of 2015, Paige started the MEK trial. Her side effects have been very minimal...Paige is doing wonderful and we couldn't be happier!"

-Shelley Doane, Paige's Mother

BEFORE



AFTER



BEFORE



AFTER



NF HFRO: RYKER BENNETT

"When Ryker was born, his face was symmetrical. Doctors now consider him to have a major facial disfigurement. He has the daily challenge of being treated differently because of his tumor. Currently, Ryker is on the MEK inhibitor trial and we are so hopeful for him!"

-Sarah Bennett, Ryker's Mother

40% shrinkage

NF HERO: JANE CONSTABLE

30% shrinkage "After Jane was diagnosed, when I would hear people say that they were hoping for, or working toward, a cure for NF, I was skeptical. As a physician, I knew how complex NF was... In my heart I hoped for effective treatments for NF, but didn't believe that there would ever be a cure. The MEK trial has changed my outlook."

-Kristina Rath, Jane's Mother

BEFORE



AFTER



NF HERO: QUENTIN LARET

"I cannot explain how thankful we are for this opportunity. He has had such a better quality of life. I am not sure how much more his little body could have handled the surgeries... and no one wants to give their child chemotherapy. Every month I pray he can continue on this trial."

-Kim Laret, Quentin's Mother

27% shrinkage

ABOUT

THE MEK TRIAL

The Children's Tumor Foundation made a significant investment in the NF Preclinical Consortium, which demonstrated that MEK inhibitors have significant impact on tumor size in animal models. That work was instrumental in the development of a clinical trial led by Dr. Brigitte Widemann of the National Institutes of Health, who announced at the Foundation's 2015 NF Conference that over half of the patients in the trial were seeing significant reduction in tumor size of their inoperable plexiform neurofibromas. Since that time, the trial has entered its registration phase (the last step before submission to the FDA for approval), with continued positive results. These success stories mark another significant step on the road to effective, approved treatments for NF. Over 70% of trial participants have seen tumor reduction of at least 20% in size, a first in NF research.