

FOR IMMEDIATE RELEASE:

FDA APPROVES ZELBORAF (VEMURAFENIB) FOR ERDHEIM-CHESTER DISEASE WITH BRAF V600 MUTATION

First FDA-Approved drug for this rare disease

DeRidder, LA, - November 8, 2017 – On Monday, November 6, 2017, the FDA approved vemurafenib (Zelboraf) for the treatment of Erdheim-Chester Disease in patients with the BRAF V600 mutation. This is the first ever approved treatment for ECD.

This means that BRAF-positive ECD patients in the US will have much easier access to this treatment that has shown amazingly good results.

“The Erdheim-Chester disease community is very encouraged by this first FDA-approved treatment for ECD, bringing new hope to patients and their families,” said Kathleen Brewer, president of ECD Global Alliance. “This new treatment option shows that meaningful breakthroughs can occur rapidly when patients, families, research physicians, industry and the FDA work together to help patients.”

This breakthrough is the result of hard work on the part of many. Genentech is the pharmaceutical company who took the data to the FDA to ask for approval. The MSKCC team (Eli Diamond, MD; David Hyman, MD; Omar Wahab-Abdel, MD) was the leader of the study that provided the data for the FDA decision. And, of course, the patients and family members who had the courage to be part of the trial, were the ones who made it possible for data to be collected.

The ECD community is the structure that makes it possible for these entities to work together. The ECD Global Alliance brings patients and families together, providing a powerful voice to show that our community is strong. Together we can do what others say is impossible.

Thank you for your donations and participation that make breakthroughs like this possible.

For more press releases on this topic, see:

<https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm583931.htm> and
<https://www.mskcc.org/blog/vemurafenib-approved-erdheim-chester-disease-rare-blood-disorder>
<https://www.roche.com/media/store/releases/med-cor-2017-11-07b.htm>

About Erdheim-Chester Disease, it is a rare blood cancer in which a white blood cell, called a histiocyte, invade otherwise healthy organs and lead to life-threatening complications. Historically, this has been a very difficult disease to diagnose and treat. Unless successful treatment is found, organ failure can occur.



David Hyman, MD; Omar Wahab-Abdel, MD, Eli Diamond, MD; MSKCC

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