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FOR IMMEDIATE RELEASE

FDA Granted Breakthrough Designation

FDA granted breakthrough designation for Cobimetinib for BRAF-negative histiocytosis

DeRidder, LA – October 2, 2019 – On Wednesday, October 2, 2019, the US Food and Drug Administration (FDA) granted a Breakthrough Therapy Designation to cobimetinib in the treatment of patients with histiocytic neoplasms (HN) (Erdheim-Chester Disease, Rosai-Dorfman, Langerhans Histiocytosis), who do not bear the *BRAF*-V600 mutation. This is a great step forward for all histiocytosis patients who do not have the *BRAF* mutation.

"This critical designation will allow patients access to this targeted treatment while it continues through the review process with the FDA," said Dr. Hyman, Chief of the Early Drug Development Service at Memorial Sloan Kettering Cancer Center (MSK).

Many ECD patients could benefit from the hopeful results that have been seen in the phase II trial conducted at MSK that led to the FDA breakthrough designation. For the approximately 50% of ECD patients who do not have the BRAF mutation there is no FDA-approved treatment. A breakthrough designation paves the way for an expedited review of a new treatment that could ultimately lead to FDA-approval for the treatment. Cobimetinib, a MEK-inhibitor, is showing good promise for patients who either do not bear the *BRAF* mutation or have progressed during or after other treatments.

"*Through a phase II trial, we were previously able to show that treatment with cobimetinib results in consistent and durable responses across clinical and genetic subtypes of HNs, which represents an area of a previously unmet need for these patients*," explained Dr. Diamond, neuro-oncologist and neurologist at MSK. "*We've recently seen great advances in the treatment of histiocytosis, with the first approval just two years ago. This designation will ensure that we are continuing on a path that may ultimately offer a viable treatment option for all histiocytosis patients."*

This breakthrough is the result of collaborative efforts of the pharmaceutical company, Genentech, and the MSK team that includes, among others, Eli L. Diamond, MD; David Hyman, MD; Benjamin Durham, MD; and Omar Abdel-Wahab, MD. The courage of the patients and family members who joined the trial allowed this advancement to occur. 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 improve the future for those affected by ECD.

We are grateful for the donations and participation that make this all possible.

For more press releases on this topic, see:

https://www.mskcc.org/trending-topics/fda-grants-breakthrough-therapy-designation-cobimetinib-mek-inhibition-histiocytic-neoplasms-research-led-msk

To learn more about ECD visit http://erdheim-chester.org/.

Erdheim-Chester Disease is an ultra-rare condition that is believed to be under diagnosed. It is considered a histiocytic neoplasm (type of blood cancer). The illness is characterized by the accumulation of histiocytes, cells that normally fight infections, in tissue and organs. The tissue and organs become dense and fibrotic due to the infiltration of the histiocytes and can lead to organ failure unless a successful treatment is found.

The ECD Global Alliance is a 501(c)(3) non-profit organization dedicated to awareness, support, education, and research related to Erdheim-Chester Disease.

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