

# What is PTCH1-LOF?

PTCH1 is a gene that regulates the Hedgehog pathway.

The Hedgehog pathway is involved in embryonic development where its signaling leads to the formation of critical systems such as the central nervous system, lungs and limbs. Once we reach adulthood this signaling pathway is shut down by PTCH1.

Mutations that occur during DNA replication and cell division can cause alterations in PTCH1.



Alterations that lead to a PTCH1 loss of function (LOF) can cause the Hedgehog pathway to re-activate which may lead to abnormal cell growth driving the initiation of cancer.

Advances in Next Generation Sequencing (NGS Testing) can help identify mutations like PTCH1-LOF. Knowing your personal mutations may help provide additional therapy options.

The investigational drug, ENV-101, acts on a protein that is thought to be involved with the growth of these abnormal cells associated with the PTCH1-LOF mutation.

