The mammalian target of rapamycin (mTOR) is a serine/threonine kinase regulating cell growth, proliferation, metabolism and survival. The mTOR pathway integrates stimuli from growth factors, nutrients, and cellular energy status and leads to downstream activation of Akt, 4E-BP1 and S6K. Phosphorylation of 4E-BP1 and S6K results in increased protein synthesis in addition to ribosome biogenesis and plays an important role in cell cycle progression. mTOR pathway is overactivated in numerous cancer types including the acute myeloid leukemia (AML) and acute lymphoblastic leukemia (ALL), which are both characterized by abnormal proliferation of white blood cells and low patient survival rate.

Three distinct approaches that differ in efficiencies and research stages have been used to inhibit the mTOR pathway. Rapamycin and its derivatives are the most common inhibitors, but since they are not entirely specific, they provide only limited desirable outcomes. Dual inhibitors targeting mTOR as well as PI3K pathway have had several successes in treating both AML and ALL. However, the new generation of inhibitors such as PP-242 and Torin-1 are providing the most hopeful prospects for mTOR inhibition and long-term remission of acute leukemia.