

Brand Name: Kanuma

Generic: sebelipase alfa

Type: small molecule

Year Accepted/Phase: 2015

Mechanism:

Sebelipase alfa is a recombinant human lysosomal acid lipase (rhLAL) enzyme that replaces the deficient or malfunctioning LAL enzyme in patients with LAL deficiency, by helping to break down fats and prevent their accumulation in tissues.

Chemical Structure: N/A

Indication:

Iressa is indicated for the first-line treatment of patients with metastatic NSCLC whose tumors have EGFR exon 19 deletions or exon 21 (L858R) substitution mutations.

Clinical trials:

Clinical Trials for LAL Deficiency

Pubmed: <https://pubmed.ncbi.nlm.nih.gov/26352813/>

Purpose: Evaluate the efficacy and safety of sebelipase alfa in patients with LAL deficiency.

Dates: Clinical trials for Kanuma were conducted in the 2010s.

Results: These trials demonstrated that sebelipase alfa reduced the levels of fats in the liver and bloodstream, improved liver function, and reduced the size of the liver and spleen in patients with LAL deficiency.

Impact: The positive results from these trials supported the approval of Kanuma for the treatment of LAL deficiency.