Title: A Novel Compound To Inhibit Microglobulin Amyloidosis

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KEYWORDS: Peptidomimetic, Inhibitor, His-tagged β 2-microglobulin protein, Amyloid, Dialysis

DOMAIN: Life Science (Chemical)

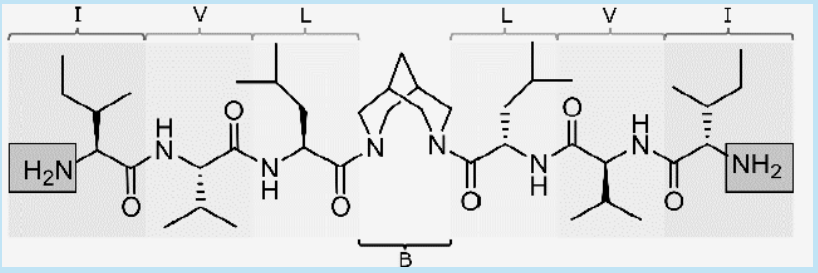
SUMMARY:

A novel peptide-like compound is composed to manage, prevent, and treat Dialysis-related amyloidosis (DRA). DRA is a misfolding protein disorder where excess microglobulin protein accumulates around the organs and eventually converts into toxic amyloid fibrils. It has a detrimental effect on patients with chronic kidney failure as the excess β 2-microglobulin proteins are not cleared by the kidney, hence deposits in the organ. To manage DRA, the available clinical therapeutic strategies are dialysis, medical or surgical therapy, and kidney transplantation. The described peptidomimetic compound uses a first-of-its-kind strategy that inhibits the amyloid formation of Hβ2m protein. The mechanism of the compound depends on altering the conformation of pre-amyloid species of Hβ2m protein which will result in hindering the amyloid production pathway. Hence, the method will prevent toxic Hβ2m fibrils formation around the organ.

The chemical formulation of the peptidomimetic compound consists of a central bicyclic diamine moiety with three different amino acids attached on either side of the central moiety

Wherein,

B depicts a bicyclic diamine;
I is isoleucine;
V is valine; and
L is leucine.



ADVANTAGES:

1. First-of-its-kind amyloid inhibition strategy to treat DRA.
2. Non-cytotoxic to mammalian cells.
3. Generation of potentially non-toxic soluble and insoluble amyloid species upon administration of the compound.
4. The compound can be administered orally, intravascularly, intravenously, intramuscularly, subcutaneously, and intraperitoneally.

APPLICATION: The compound is used in the treatment of DRA amyloidosis.

SCALE OF DEVELOPMENT: Compound development and testing performed at a lab scale.

TECHNOLOGY READINESS LEVEL: TRL 3

IP STATUS: Indian Patent Application (202111033516)