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(54) Title: ARIMOCLOMOL FOR THE TREATMENT OF NIEMANN PICK DISEASE, TYPE C, IN PATIENTS WITH ER TYPE MISSENSE MUTATIONS

(57) Abstract: The present invention relates to an active pharmaceutical ingredient selected from N-[2-hydroxy-3-(1-piperidinyl)-propoxy]-pyridine-1-oxide-3-carboximidoyl chloride, its stereoisomers and the acid addition salts thereof; specifically arimoclomol, for use in improved methods of treating Niemann Pick disease, type C (NPC), wherein the patient 5 has an endoplasmic reticulum (ER) type missense mutation in an NPC gene.

WO 2022/136640 A1