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(57) Abstract :
A nasal spray composition for treating Huntington's disease (HD) includes a therapeutically effective amount of OSMI-1, a selective O-GlcNAc transferase (OGT) inhibitor. The composition comprises purified water as a solvent and carrier, sodium phosphate monobasic dihydrate and sodium phosphate dibasic heptahydrate as buffer components, EDTA as a stabilizer, polysorbate 80 as a penetration enhancer, benzalkonium chloride as a preservative, hydroxypropyl methylcellulose (HPMC) as a viscosity modifier, and sodium chloride as an osmolality adjuster. OSMI-1 binds to and inhibits OGT activity, reducing mutant huntingtin protein (mHtt) aggregation, oxidative stress, and neuroinflammation, providing neuroprotection against HD. The composition is administered intranasally and is sterile, with a pH adjusted to 6.5–7.5. It improves locomotor and cognitive performance, preserves hippocampal structure, and reduces mHtt levels, offering anti-inflammatory, antioxidant, and anti-apoptotic effects, and is a potential new treatment for HD. Reference Fig 1

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