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(54) Title **COMPOSITIONS AND METHODS FOR TREATMENT OF HOMOCYSTINURIA**

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Patentkrav

1. Sammensetning omfattende et isolert humant trunkert cystationin beta-syntase (htCBS) mutant polypeptid konjugert til en PEG-del, hvori det isolerte htCBS-mutante polypeptidet omfatter aminosyresekvensen til SEKV ID NR:3 med en endring av et cystein ved aminosyreposisjonen som tilsvarer 15 av SEKV ID NR:3 til et serin og mangler den N-terminale første metioninresten som tilsvarer SEKV ID NR:3, og er PEGylert med ME-200GS;
for bruk i en fremgangsmåte for behandling av homocystinuri hos et menneske, hvori det PEGylerte htCBS-mutante polypeptidet administreres minst en gang i uken i minst seks uker i en dose mellom 0,33 og 10 mg/kg.

2. Sammensetning for bruk ifølge krav 1, hvori dosen er en mengde valgt fra gruppen bestående av: 0,33, 0,5, ca. 1, ca. 2 og ca. 5 mg/kg.

3. Sammensetning for bruk ifølge krav 1, hvori dosen er ca. 0,4 mg/kg.

4. Sammensetning for bruk ifølge krav 1, hvori det PEGylerte htCBS-mutante polypeptidet administreres minst to ganger i uken.

5. Sammensetning for bruk ifølge krav 1, hvori det PEGylerte htCBS-mutante polypeptidet er co-administrert med betain.

6. Sammensetning for bruk ifølge krav 1, hvori betain administreres før det PEGylerte htCBS-mutante polypeptidet.