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(54) Title: CONJUGATE OF A SINGLE DOMAIN ANTIBODY, A SAPONIN AND AN EFFECTOR MOLECULE, PHARMACEUTICAL COMPOSITION COMPRISING THE SAME, THERAPEUTIC USE OF SAID PHARMACEUTICAL COMPOSITION

(57) Abstract: The invention relates to a conjugate for transferring an effector molecule from outside a cell into said cell, the conjugate comprising at least one effector molecule to be transferred into the cell, at least one saponin of the mono-desmosidic triterpene glycoside type or the bi-desmosidic triterpene glycoside type, and at least one single-domain antibody (sdAb), covalently bound to each other, wherein the sdAb is capable of binding to a cell-surface molecule of said cell. The invention also relates to a pharmaceutical composition comprising the conjugate of the invention. Furthermore, the invention relates to a pharmaceutical composition of the invention, for use as a medicament. In addition, the invention relates to a pharmaceutical composition of the invention, for use in the treatment or the prophylaxis of any one or more of: a cancer, an auto-immune disease such as rheumatoid arthritis, an enzyme deficiency, a disease related to an enzyme deficiency, a gene defect, a disease relating to a gene defect, an infection such as a viral infection, hypercholesterolemia, primary hyperoxaluria, haemophilia A, haemophilia B, alpha-1 antitrypsin related liver disease, acute hepatic porphyria, an amyloidosis and transthyretin-mediated amyloidosis. The invention also relates to an in vitro or ex vivo method for transferring the conjugate from outside a cell to inside said cell or for transferring the effector molecule comprised by the conjugate of the invention from outside a cell to inside said cell, preferably to the cytosol of said cell.

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