

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
30 December 2021 (30.12.2021)



(10) International Publication Number
WO 2021/259507 A1

(51) International Patent Classification:

A61K 47/55 (2017.01) A61K 47/64 (2017.01)

A61K 47/59 (2017.01) A61K 47/54 (2017.01)

(21) International Application Number:

PCT/EP2020/069340

(22) International Filing Date:

09 July 2020 (09.07.2020)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

2025900 24 June 2020 (24.06.2020) NL

(71) Applicants: SAPREME TECHNOLOGIES B.V.

[NL/NL]; Professor Bronkhorstlaan 10 G 92,
3723 MB Bilthoven (NL). CHARITÉ -
UNIVERSITÄTSMEDIZIN BERLIN [DE/DE]; Charité-
platz 1, 10117 Berlin (DE).

(72) Inventors: POSTEL, Ruben; Yalelaan 62, LSI, Room

2.09, 3584 CM Utrecht (NL). HERMANS, Guy; Hun-
delgemsesteenweg 848, 9820 Merelbeke (BE). FUCHS,
Hendrik; Institut für Laboratoriumsmedizin, Klinische
Chemie und Pathobiochemie Charité - Universitätsmedizin,
Berlin Campus Virchow-Klinikum, Augustenburger Platz
1, 13353 Berlin (DE).

(74) Agent: NEDERLANDSCH OCTROOIBUREAU;

P.O.Box 29720, 2502 LS The Hague (NL).

(81) Designated States (unless otherwise indicated, for every

kind of national protection available): AE, AG, AL, AM,
AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ,
CA, CH, CL, CN, CO, CR, CU, CZ, DE, DJ, DK, DM, DO,
DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN,
HR, HU, ID, IL, IN, IR, IS, IT, JO, JP, KE, KG, KH, KN,
KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD,
ME, MG, MK, MN, MW, MX, MY, MZ, NA, NG, NI, NO,

NZ, OM, PA, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW,
SA, SC, SD, SE, SG, SK, SL, ST, SV, SY, TH, TJ, TM, TN,
TR, TT, TZ, UA, UG, US, UZ, VC, VN, WS, ZA, ZM, ZW.

(84) Designated States (unless otherwise indicated, for every

kind of regional protection available): ARIPO (BW, GH,
GM, KE, LR, LS, MW, MZ, NA, RW, SD, SL, ST, SZ, TZ,
UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, RU, TJ,
TM), European (AL, AT, BE, BG, CH, CY, CZ, DE, DK,
EE, ES, FI, FR, GB, GR, HR, HU, IE, IS, IT, LT, LU, LV,
MC, MK, MT, NL, NO, PL, PT, RO, RS, SE, SI, SK, SM,
TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW,
KM, ML, MR, NE, SN, TD, TG).

Published:

— with international search report (Art. 21(3))

(54) Title: CONJUGATE OF A SINGLE DOMAIN ANTIBODY, A SAPONIN AND AN EFFECTOR MOLECULE, PHARMA-
CEUTICAL 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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