



(51) International Patent Classification:

A61K 31/145 (2006.01) A61P 9/10 (2006.01)
A01N 1/02 (2006.01) A61P 39/00 (2006.01)
A61K 45/06 (2006.01)

(21) International Application Number:

PCT/US2024/015136

(22) International Filing Date:

09 February 2024 (09.02.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/484,320 10 February 2023 (10.02.2023) US

(71) Applicant: **OBVIA PHARMACEUTICALS, LTD.**

[US/US]; 504 South Rosa Road, Madison, WI 53719-1217 (US).

(72) Inventors: **FAHL, William**; 2938 Bluff Street, Madison,

WI 53705 (US). **GOESCH, Torsten**; The Ladyhill #03-09, 1 Lady Hill Road, Singapore 258670 (SG). **GOESCH, Hannah, Rebecca**; Bomhardstrasse 16, 82031 Gruenwald (DE). **GOESCH, Sarah, Ricarda**; Bomhardstrasse 16, 82031 Gruenwald (DE).

(74) Agent: **ARMSTRONG, Richard, Todd** et al.; Clark & Elbing LLP, 101 Federal Street, 15th Floor, Boston, MA 02110 (US).

(81) Designated States (unless otherwise indicated, for every kind of national protection available):

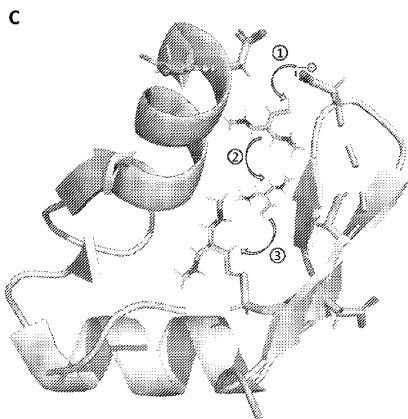
AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CV, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, MG, MK, MN, MU, 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, CV, GH, GM, KE, LR, LS, MW, MZ, NA, RW, SC, 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, ME, MK, MT, NL, NO, PL, PT, RO, RS, SE,

(54) Title: AMINOTHIOLS FOR THE TREATMENT OF CONDITIONS CHARACTERIZED BY PROTEIN MISFOLDING OR AGGREGATION, MITOCHONDRIAL DYSFUNCTION, OR CHRONIC INFLAMMATION

Figure 6: The PrC-210 Thiol to Disulfide Reaction Cycle



A) Reaction between anionic ammonium acetate and PrC-210 inducing PrC-210-PrC-210 disulfide bond formation. 1.) The free electron pair of the negatively charged acetate attacks the proton of the thiol on PrC-210. 2.) The deprotonated, activated, thiolate anion on the PrC-210 molecule can then 3.) react with PrC-210 thiol molecules to form a PrC-210 disulfide. B) Reduced PrC-210 half-life, from days in water to 3.1 hr in acetate C) Translation of reaction in A to a protein, such as TDP-43

(57) Abstract: The invention features aminothiols for treating (1) diseases and conditions characterized by misfolding and/or aggregation of endogenous proteins, (2) diseases and conditions associated with mitochondrial dysfunction, and (3) diseases and conditions associated with chronic inflammation in a subject in need thereof.

SI, SK, SM, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN,
GQ, GW, KM, ML, MR, NE, SN, TD, TG).

Declarations under Rule 4.17:

- *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*

Published:

- *with international search report (Art. 21(3))*