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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
28 December 2000 (28.12.2000)

PCT

(10) International Publication Number
WO 00/78972 A2

(51) International Patent Classification[7]: C12N 15/52, C07K 14/705, C12Q 1/68, C07K 16/18, A61K 38/00

(21) International Application Number: PCT/US00/16765

(22) International Filing Date: 16 June 2000 (16.06.2000)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:

60/140,264	18 June 1999 (18.06.1999)	US
60/153,872	14 September 1999 (14.09.1999)	US
60/166,573	19 November 1999 (19.11.1999)	US

(71) Applicant *(for all designated States except US)*: CV THERAPEUTICS, INC. [US/US]; 3172 Porter Drive, Palo Alto, CA 94304 (US).

(72) Inventors; and

(75) Inventors/Applicants *(for US only)*: LAWN, Richard, M. [—/US]; 1927 8th Avenue, San Francisco, CA 94116 (US). WADE, David [—/US]; Apartment L, 383 Curtner Avenue, Palo Alto, CA 94306 (US). GARVIN, Michael [—/US]; 17 White Street, San Francisco, CA 94019 (US).

(74) Agent: HUGHES, A., Blair; McDonnell Boehnen Hulbert & Berghoff, 32nd Floor, 300 South Wacker Drive, Chicago, IL 60606 (US).

(81) Designated States *(national)*: AE, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CR, CU, CZ, DE, DK, DM, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, TZ, UA, UG, US, UZ, VN, YU, ZA, ZW.

(84) Designated States *(regional)*: ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG).

Published:

— *Without international search report and to be republished upon receipt of that report.*

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

WO 00/78972 A2

(54) Title: COMPOSITIONS AND METHODS FOR INCREASING CHOLESTEROL EFFLUX AND RAISING HDL USING ATP BINDING CASSETTE TRANSPORTER PROTEIN ABC1

(57) Abstract: The present invention relates to novel ABC1 polypeptides and nucleic acid molecules encoding the same. The invention also relates to recombinant vectors, host cells, and compositions comprising ABC1 polynucleotides, as well as to methods for producing ABC1 polypeptides. The invention also relates to antibodies that bind specifically to ABC1 polypeptides. In addition, the invention relates to methods for increasing cholesterol efflux as well as to methods for increasing ABC1 expression and activity. The present invention further relates to methods for identifying compounds that modulate the expression of ABC1 and methods for detecting the comparative level of ABC1 polypeptides and polynucleotides in a mammalian subject. The present invention also provides kits and compositions suitable for screening compounds to determine the ABC1 expression modulating activity of the compound, as well as kits and compositions suitable to determine whether a compound modulates ABC1-dependent cholesterol efflux.