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(71) Applicant: **GENZYME CORPORATION** [US/US]; 50
Binney Street, Cambridge, Massachusetts 02142 (US).

(72) Inventor: **KYOSTIO-MOORE, Sirkka R.M.**; c/o Sanofi,
55 Corporate Drive, Mail Code 55A-525, Bridgewater, New
Jersey 08807 (US).

(74) Agent: **DONAHUE, Brian** et al.; Morrison & Foerster
LLP, 755 Page Mill Road, Palo Alto, California 94304-1018
(US).

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(54) Title: HUMAN PAH EXPRESSION CASSETTE FOR TREATMENT OF PKU BY LIVER-DIRECTED GENE REPLACEMENT THERAPY

(57) Abstract: Provided herein are expression cassettes for expressing a transgene in a liver cell, wherein the transgene encodes a PAH polypeptide. Also provided are methods to treat phenylketonuria (PKU) and/or to reduce levels of phenylalanine in an individual in need thereof. Further provided herein are vectors (e.g., rAAV vectors), viral particles, pharmaceutical compositions and kits for expressing a PAH polypeptide in an individual in need thereof.



WO 2022/072657 A1