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(71) Applicant: **KANGLIN BIOTECH (HANGZHOU) CO., LTD.** [CN/CN]; Room 301, Building No. 291, Fucheng Road, Hangzhou, Zhejiang 310018 (CN).

(72) Inventors: **WU, Haoquan**; c/o KANGLIN BIOTECH (HANGZHOU) CO., LTD., Room 301, Building No. 4, No. 291, Fucheng Road, Hangzhou, Zhejiang 310018 (CN). **DANG, Ying**; c/o KANGLIN BIOTECH (HANGZHOU) CO., LTD., Room 301, Building No. 4, No. 291, Fucheng Road, Hangzhou, Zhejiang 310018 (CN). **SU, Lingling**; c/o KANGLIN BIOTECH (HANGZHOU) CO., LTD., Room 301, Building No. 4, No. 291, Fucheng Road, Hangzhou, Zhejiang 310018 (CN). **YE, Qing**; c/o KANGLIN BIOTECH (HANGZHOU) CO., LTD., Room 301, Building No. 4, No. 291, Fucheng Road, Hangzhou, Zhejiang 310018 (CN). **TAO, Jianjun**; c/o KANGLIN BIOTECH (HANGZHOU) CO., LTD., Room 301, Building No. 4, No. 291, Fucheng Road, Hangzhou, Zhejiang 310018 (CN). **HU, Xiaodong**; c/o KANGLIN BIOTECH (HANGZHOU) CO., LTD., Room 301, Building No. 4, No. 291, Fucheng Road, Hangzhou, Zhejiang 310018 (CN). **JIANG, Chao**; c/o KANGLIN BIOTECH (HANGZHOU) CO., LTD., Room 301, Building No. 4, No. 291, Fucheng Road, Hangzhou, Zhejiang 310018 (CN).

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(54) Title: NUCLEIC ACID CONSTRUCTS AND USES THEREOF

(57) Abstract: Disclosed herein are gene therapy vectors used for efficiently transducing cells to express a human β -globin gene. Specifically disclosed is an expression vector which comprises: an expression cassette for a β -globin gene, which comprises exons and introns of human β -globin gene, as well as cis-acting elements including one or more of WPRE, SV40 polyadenylation signal and/or SV40 ori. The disclosed expression vectors have significantly enhanced viral vector packaging efficiency in viral vector packaging cell lines, which leads to effective integration of lentiviral vectors and high expression level of β -globin gene in target cells. Also disclosed are pharmaceutical compositions and therapeutic methods utilizing such expression vectors.



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