

## VECTORES LENTIVIRALES Y USOS DE LOS MISMOS

### LENTIVIRAL VECTORS AND THEIR USES

#### INNOVATION DESCRIPTION:

The present invention relates to a lentiviral vector, wherein the lentiviral vector comprises a polynucleotide, wherein said polynucleotide is characterized in that it comprises a transcription unit that in turn comprises a human phosphoglycerate kinase (PGK) promoter, a nucleotide sequence encoding for the RPS19 protein and an optimized woodchuck hepatitis virus post-transcriptional regulatory element (WPRE). The invention also relates to compositions and uses of said lentiviral vector, particularly the use thereof for the treatment of Diamond-Blackfan anemia.

#### APPLICANTS:

- CIEMAT - CENTRO DE INVESTIGACIONES ENERGÉTICAS, MEDIOAMBIENTALES Y TECNOLÓGICAS (66%)
- FIIS-FJD - FUNDACIÓN INSTITUTO DE INVESTIGACIÓN SANITARIA FUNDACIÓN JIMÉNEZ DÍAZ (13%)
  - CIBER - CENTRO DE INVESTIGACIÓN BIOMÉDICA EN RED (13%)
- FUNDACIÓN PARA LA INVESTIGACIÓN BIOMÉDICA DEL HOSPITAL INFANTIL UNIVERSITARIO NIÑO JESÚS (3 %)
  - ULB - UNIVERSITÉ LIBRE DE BRUXELLES (5%)

#### IPR STATUS:

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#### FURTHER DETAILS:

Fundación para la Investigación Biomédica del Hospital Infantil Universitario Niño Jesús

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