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(71) Applicant: **FUNDACIÓN PARA LA INVESTIGACIÓN MÈDICA APLICADA** [ES/ES]; Avda Pio XII 55, 31008 Pamplona (ES).

(72) Inventors: **MURILLO SAUCA, Oihana**; CIMA - Oficina Propiedad Industrial, Avda Pío XII 55, 31008 Pamplona (ES). **GONZÁLEZ ASEGUINOLAZA, Gloria**; Cima - Oficina de Propiedad, Industrial, Avda Pio XII 55, 31008 Pamplona (ES). **HERNÁNDEZ ALCOCEBA, Rubén**; CIMA - Oficina de Propiedad Industrial, Avda Pio XII 55, 31008 Pamplona (ES).

(74) Agents: **RENKEN, Joachim** et al.; Hoffmann Eitle Patent- und Rechtsanwälte PartmbB, Arabellastraße 30, 81925 Munich (DE).

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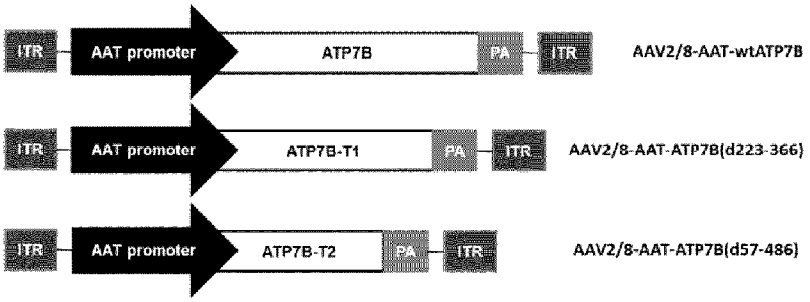


Figure 1

(57) Abstract: The invention relates to nucleic acid constructs and gene therapy vectors that comprise an ATP7B variant for use in the treatment of conditions associated with a deficiency or dysfunction of Copper-transporting ATPase 2, and particularly of Wilson's disease. An AAV vector devised according to the invention significantly reduced urine Cu excretion, and liver Cu content in Wilson's disease mice treated with the vector, while ceruloplasmin activity was significantly restored. On the other hand, the administration of the vector resulted in the normalization of serum transaminases' levels and of liver histology, together with a marked reduction of the inflammatory infiltrate.

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