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(54) **CODON-OPTIMIZED TRANSGENE FOR THE TREATMENT OF PROGRESSIVE FAMILIAR INTRAHEPATIC CHOLESTASIS TYPE 3 (PFIC3)**

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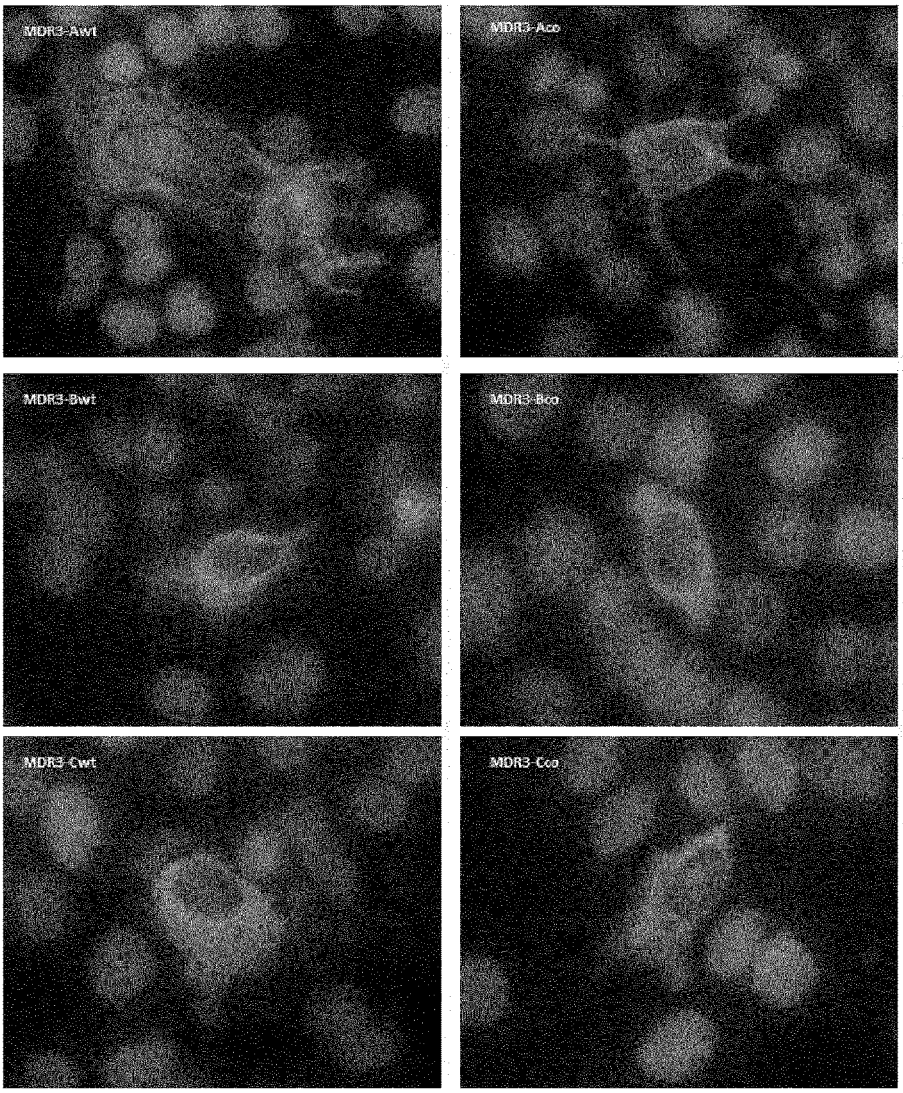
(57) **ABSTRACT**

Gene therapy vector is for use in the treatment of progressive familiar intrahepatic cholestasis type 3. More specifically, an adeno-associated virus vector includes codon-optimized sequence encoding for the MDR3 isoform A for the treatment of PFIC3.

Specification includes a Sequence Listing.