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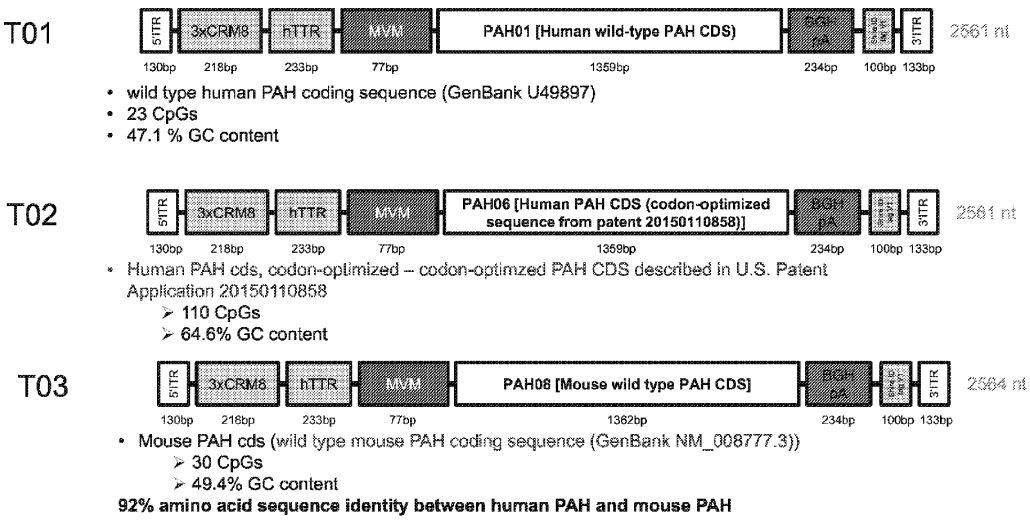


FIG. 1A

(57) Abstract: The present disclosure provides, among other things, a recombinant adeno-associated virus (rAAV) vector comprising an AAV8 capsid and a codon-optimized sequence encoding a human phenylalanine hydroxylase (PAH) enzyme. The disclosure also provides a method of treating a subject having phenylketonuria (PKU), comprising administering to the subject in need thereof a recombinant adeno-associated virus (rAAV) vector comprising an AAV8 capsid, and a promoter operably linked to a nucleic acid sequence that encodes PAH, and wherein administering results in a decrease in phenylalanine level in the subject.

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