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(54) Title: ADENO ASSOCIATED VIRUS VECTORS FOR THE TREATMENT OF HUNTER DISEASE

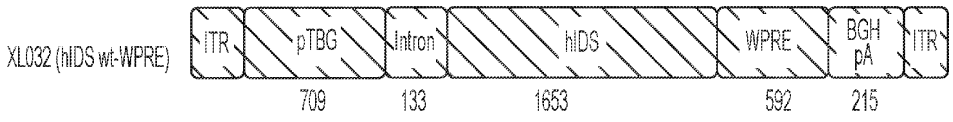


Figure 1A

(57) Abstract: The present disclosure provides, among other things, a recombinant adeno-associated virus (rAAV) vector comprising an AAV8 or AAV9 capsid and a codon-optimized sequence encoding a human iduronate-2-sulfatase (I2S) enzyme. The disclosure also provides a method of treating a subject having Hunter syndrome (MPS II), comprising administering to the subject in need thereof a recombinant adeno-associated virus (rAAV) vector comprising an AAV8 or AAV9 capsid, and a promoter operably linked to a nucleic acid sequence that encodes iduronate-2-sulfatase (I2S), and wherein administering results in an increase in I2S enzymatic activity in the subject.

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