ABSTRACT OF THE DISCLOSURE

COMPOSITIONS AND METHODS FOR TREATING MPSI

A vector have an expression cassette containing have a hIDUA gene has a sequence of SEQ ID NO: 1 (FIG 1) or a sequence at least about 95% identical thereto which encodes a functional human alpha-L-iduronidase is provided. The vector may be a production vector or a rAAV8. Also provided are compositions containing these vectors and methods of treating MPSI and the symptoms associated with Hurler, Hurler-Scheie and Scheie syndromes.

FIG. 2