INTRATHECAL DELIVERY OF NUCLEIC ACID SEQUENCES ENCODING ABCD1 FOR TREATMENT OF ADRENOMYELONEUROPATHY

ABSTRACT

Methods of the invention encompass delivery of nucleic acid sequences encoding ABCD1 for the treatment of X-linked Adrenoleukodystrophy (X-ALD), e.g., for Adrenomyeloneuropathy (AMN).

Fig. 1