

# Liver directed gene therapy for inherited metabolic diseases

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Inherited metabolic disorders (IMDs) are a growing group of genetic diseases caused by defects in enzymes that mediate cellular metabolism, often resulting in the accumulation of toxic substrates. The liver is a highly metabolically active organ that hosts several thousand chemical reactions. As such, it is an organ frequently associated with IMDs. In my talk, I will discuss current approaches and unresolved challenges for liver-directed gene-based therapy aimed at metabolite detoxification in a variety of IMDs.