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Progress and prospects of engineered sequence-specific DNA modulating technologies for the management of liver diseases

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Abstract

Liver diseases are one of the leading causes of mortality in the world. The hepatic illnesses, which include inherited metabolic disorders, hemophilias and viral hepatitides, are complex and currently difficult to treat. The maturation of gene therapy has heralded new avenues for developing effective intervention for these diseases. DNA modification using gene therapy is now possible and available technology may be exploited to achieve long term therapeutic benefit. The ability to edit DNA sequences specifically is of paramount importance to advance gene therapy for application to liver diseases. Recent development of technologies that allow for this has resulted in rapid advancement of gene therapy to treat several chronic illnesses. Improvements in application of derivatives of zinc finger proteins (ZFPs), transcription activator-like effectors (TALEs), homing endonucleases (HEs) and clustered regularly interspaced palindromic repeats (CRISPR) and CRISPR associated (Cas) systems have been particularly important. These sequence-specific technologies may be used to modify genes permanently and also to alter gene