



Pediatric Neurology Part III: Chapter 191. Gene therapy for disorders of the central nervous system (Handbook of Clinical Neurology)

Patrick Aubourg

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Though heavily hyped in the early 1990s as the answer to many genetic diseases, gene therapy has largely failed to live up to its promises up to now. The scientific challenges of conveying replacement genes into the brain have prevented it from having an impact on more than a handful of conditions. In addition, gene therapy has been beset by safety problems. During the last 5 years, new vectors and techniques have started to resolve not only safety issues that once held gene therapy back but also markedly to improve efficient gene delivery within the brain and even the spinal cord. Brain gene therapy will not be suitable for every neurological disease, but considerable progress made in the field means we can now envisage that CNS gene therapy will be able to treat many neurological conditions that are not obviously treatable in any other way.

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