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Sommario/riassunto	Neurodegenerative diseases (NDs) are a heterogeneous group of disorders affecting the central nervous system. Despite significant differences in their causes, neuropathological abnormalities, and clinical outcomes, some similarities can be found among them, as for example: 1) frequent aggregation and deposition of misfolded proteins, 2) common molecular mechanisms leading to neurodegeneration, and 3) certain overlap in symptoms and clinical features. To date, there is no cure that could stop or delay the progression of these diseases. The advent of advanced gene therapy techniques such as gene silencing and gene editing opened a new avenue for the development of therapeutic strategies for NDs. The discovery of the RNA interference (RNAi) mechanism, in 1998, by Andrew Fire and Craig Mello allowed an important boost to the gene therapy field, providing a potential therapeutic strategy to treat inherited dominant genetic disorders. The use of small RNA sequences to control the expression of disease-causing genes rapidly implemented in the preclinical studies for different diseases. In the field of NDs, several successful studies using this technology proved its potential as a therapeutic option. However, issues like the type of delivery system (non-viral versus viral) or the potential toxicity of the small RNA molecules, made the translation of gene silencing therapeutics to human application very slow and difficult. Recently, a new hope in the gene therapy field emerged with the development of gene editing techniques like TALENs or

CRISPR/Cas9 systems. The opportunity of editing or deleting gene sequences drove the scientific community euphoric, with an enormous increase in the number of published studies using this type of techniques. Recently, the first clinical trial using one of these systems was approved in China. For NDs, gene-editing technology also represents an important therapeutic option, and the first preclinical studies are now being published, showing the potential accomplishment for this technology.