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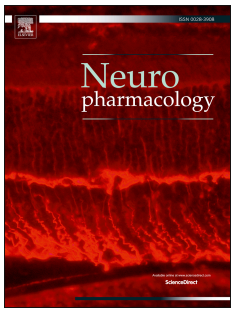

Gene therapy and editing: Novel potential treatments for neuronal channelopathies

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Gene therapy and editing: novel potential treatments for neuronal channelopathies

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Highlights

- Neuronal channelopathies need new therapeutic approaches
- Gene therapy approaches that manipulate network excitability may rescue these brain pathologies.
- Gene editing with CRISPR/Cas9 can potentially correct the underlying mutation and permanently cure the disease.
- Advances in viral-vector mediated transgene delivery may allow the transduction of large brain areas following systemic injection.
- The optimal gene therapy or gene editing strategy will depend when during development treatment can be administered.