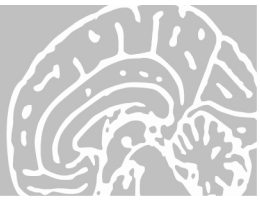
Future perspectives of CRISPR technology in neurological disorders



이 정 민
(주)툴젠

Jungmin Lee, PhD
ToolGen R&D Center

Programmable nucleases such as Zinc Finger Nuclease (ZFN), TALEN (Transcription activator-like effector nuclease) proved that the nucleases can be programmed to cut a specific sequence of genome with high specificity. However, those nucleases remain far from clinical applications due to difficulties in designing for targeting a specific sequence of genome and in delivering the nucleases into body. CRISPR/Cas9 was recently found and has drawn incomparably enormous interests from scientific and clinical fields due to its ease of use. CRISPR/Cas9 consists of two components, an enzyme called Cas9 which cuts DNA, and a piece of RNA called guide RNA which directs the Cas9 to the specific sequence of genome. Theoretically, 20 nucleotide sequences in guide RNA can target any region of genome and then Cas9 make double stand breaks at the point of genome directed by the guide RNA. Since CRISPR/Cas9 system is extremely easy to design and use, its application is being expanded rapidly to various fields including neuroscience. Here, we show therapeutic efficacy in an animal model of CMT1A (Charcot-Marie-Tooth type1A) using CRISPR/Cas9, indicating that CRISPR/Cas9 based gene editing can be utilized not only for basic scientific tools but also has potential to be translated into therapeutics.

Key Words: CRISPR, Genome engineering, CMT1A, Therapeutics

Jungmin Lee, PhD
ToolGen R&D Center, 1204 Byucksan Digital Valley 6-Cha, 219 Gasan Digital 1-ro, Geumcheon-gu, Seoul 08501, Korea
Tel: +82-2-873-8168 Fax: +82-2-873-8169
E-mail: jm.lee@toolgen.com