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2018年3月19日 - The CRISPR/Cas9 system makes genome engineering technology feasible for application in many fields including human diseases. ... Targeting multiple genes in the same cell by CRISPR/Cas9 technology enables the study of synergistic effects due to the loss of crucial genes.

The CRISPR/Cas system: An emerging technology in stem cell research

Maria Teresa Valenti, Michela Serena, Luca Dalle Carbonare, Donato Zipeto

Abstract

The identification of new and even more precise technologies for modifying and manipulating the genome has been a challenge since the discovery of the DNA double helix. The ability to selectively modify specific genes provides a powerful tool for characterizing gene functions, performing gene therapy, correcting specific genetic mutations, eradicating diseases, engineering cells and organisms to achieve new and different functions and obtaining transgenic animals as models for studying specific diseases.

CRISPR/Cas9 technology has recently revolutionized genome engineering. The application of this new technology to stem cell research allows disease models to be developed to explore new therapeutic tools. The possibility of translating new systems of molecular knowledge to clinical research is particularly appealing for addressing degenerative diseases. In this review, we describe several applications of CRISPR/Cas9 to stem cells related to degenerative diseases. In addition, we address the challenges and future perspectives regarding the use of CRISPR/Cas9 as an important technology in the medical sciences.

INTRODUCTION

Gene editing

The development of gene targeting by homologous recombination (HR) was one of the fundamental steps forward in the field of genome editing, allowing site-directed specific mutation of a desired locus by exploiting homology arms to facilitate recombination at the donor site[1]. HR-mediated gene targeting led to the generation of both knock-in and knock-out cell lines as well as many transgenic animal models. However, one of the weaknesses of this technology is that the frequency of recombination events is low (1 in 10^6-10^9 cells)[1],

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https://**www.ncbi.nlm.nih.gov**/pmc/articles/PMC4913977

Feb 11, 2016 · **CRISPR/Cas Machinery and Mechanisms** of Action. The **CRISPR/Cas system** of immunity is comprised of three steps; adaptation, expression, and interference. The adaption stage involves the recognition and cleavage of a protospacer from invading DNA by the **cas** genes. The subsequent insertion (acquisition) of foreign DNA...

Cited by: 18 **Author:** Simon N. Waddington, Riccardo Privolizzi...
Publish Year: 2016

The CRISPR/Cas System: Emerging Technology and ...

https://www.caister.com/**crispr**

The use of **CRISPR/Cas technology** for genome editing suggests many potential applications, including the alteration of **the germline of humans**, animals and **food crops**. The speed and efficiency of **the CRISPR/Cas system** make it a potentially useful system for **gene therapy**.

CRISPR Gene Editing in Stem Cells: Cell Press

https://www.**cell**.com/nucleus-**crispr**-gene-editing-**in-stem-cells**

Since their discovery, CRISPR-based systems have fundamentally transformed our ability to manipulate genomes. When combined with **stem cells**, these gene-editing tools also have the power to reshape our understanding of **human genetics**, **developmental biology**, and **regenerative medicine**.

CRISPR-Cas systems for genome editing, regulation and ...

https://**www.ncbi.nlm.nih.gov**/pmc/articles/PMC4022601

From bacterial CRISPR-Cas immune system to engineered RNA-guided nucleases CRISPR systems are adaptable immune mechanisms used by many bacteria to protect themselves from **foreign nucleic acids**, such as viruses or **plasmids** 2 - 5 .

Cited by: 2471 **Author:** Jeffry D Sander, J Keith Joung
Publish Year: 2014

CRISPR - Changing the gene editing landscape | Eurostemcell