Journal of Plant Genetics and Breeding

Kargas et al., J Plant Genet Breed 2022, 6:5

Extended Abstract Open Access

Computational Methods for Improving Genetic Therapies for Duchene Muscular Dystrophy

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Abstract

Genetic engineering involves different techniques to intentionally modify genetic material (primarily DNA) in order to alter, restore, or boost shape or function. Established in the late 20th century, recombinant DNA technologies include recombination of different strands of DNA, usually using bacteria (such as Escherichia coli), bacteriophages (such as λ phage) or by way of simple microinjection. In recent years, modern techniques to design and create -literally to engineer- new life forms, typically referred to as synthetic biology, have supplemented these conventional methods [1].

A flexible and efficient gene therapy technique is precision genome editing. The area has been subject to continuous developments since the introduction of CRISPR/Cas systems for genome editing [2]. This new biotechnology technique includes the development of a site-specific double strand break (DSB) accompanied by two key forms of repair mechanisms: non-homologous end-joining and homology directed-repair [3].

In the current work, we broke down and understood the main components of the prime editing technique. An automated approach is required for advancing our understanding of the evolution and diversity of prime editing and for finding new candidates for genome engineering. We used Duchenne Muscular Dystrophy (DMD) as a case study for this proposed approach. More specifically, we applied machine learning algorithms over prime editing data for the genetic disease of DMD. We designed multiple prime editing guide RNAs (pegRNAs) for the potential correction of the mutated exon 44 of DMD gene [4,5].

Biography

Georgios Kargas if affiliated to the Department of Digital Systems, University of Piraeus, Greece. His research interests reflect in his wide range of publications in various national and international journals.

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Received: 01-Sep-2022, Manuscript No: jpgb-22-130, **Editor assigned:** 05-Sep-2022, Pre QC No: jpgb-22-130(PQ), **Reviewed:** 19-Sep-2022, QC No: jpgb-22-130, **Revised:** 26-Sep-2022, Manuscript No: jpgb-22-130 (R), **Published:** 30-Sep-2022, DOI: 10.4172/jpgb.1000130

Citation: Kargas G, Krithara A, Nentidis A, Palliouras G, Filippakis M (2022) Computational Methods for Improving Genetic Therapies for Duchene Muscular Dystrophy. J Plant Genet Breed 6: 130.