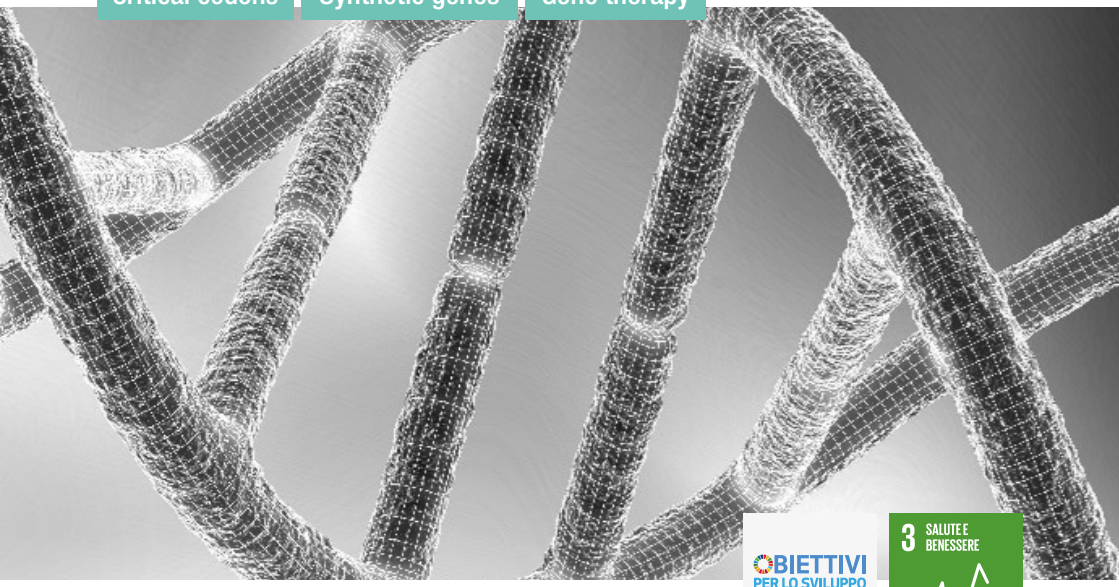
University of Ferrara

UNIFE PATENTS

biotechnologies

METHOD FOR IMPLEMENTING THE DESIGN OF SYNTHETIC NUCLEIC ACID MOLECULES FOR GENE THERAPIES IN RARE DISEASES

Critical codons | Synthetic genes | Gene therapy



OBIETTIVI PER LO SVILUPPO SOSTENIBILE

3 SALUTE E BENESSERE

AREA TECNOLOGICA

Biotechnologies

MERCATI DI RIFERIMENTO

Pharmacological, Biotech

PATENT OWNER: University of Ferrara
PRIORITY NUMBER: 102022000006119
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BRIEF DESCRIPTION OF THE INVENTION

The present invention is based on a method that comparatively analyzes genes different ones that cause rare diseases, identify codons (the triplets of nucleotides that are the genetic code base) «critical» that may both reflect susceptibility to mutations genics, help interpret the meaning of synonymous variations, and design a new algorithm for codon optimization that is used for synthetic genes.

CHARACTERISTICS OF THE INVENTION

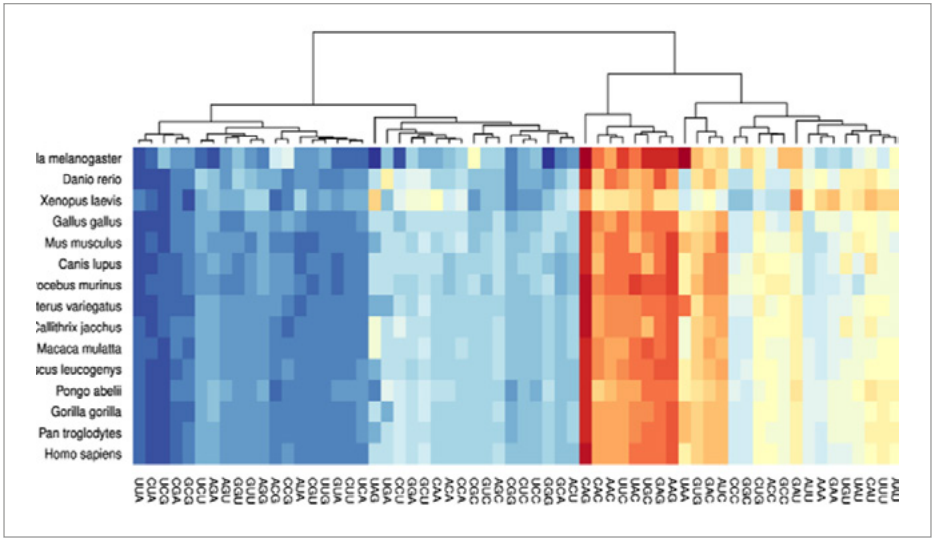
The present invention relates to calculation methods used to identify critical codons which, although “rare”, may be necessary in drawing a DNA sequence synthetic for the design of actual nucleic acid molecules for use in therapy gene, and also in the interpretation of genome information. The method is based on the calculation of CUB (Codon Usage Bias) and RSCU (Relative Synonymous Codon Usage) in genes that are both “wild type” and cause rare diseases, and compared with each other based on species (and therefore during evolution), to the tissues where they are expressed, and to the quantitative modalities of expression. This strategy, identifying some rare “critical” codons, that is, essential for some functions of the gene, it allows to optimize the nucleic acid design synthetic for gene therapy or other therapeutic applications, to make the protein translation, and to understand the meaning of synonymous variations. The present invention proposes new parameters that can be integrated into existing algorithms and create new software for the design of synthetic molecules and genome analysis.

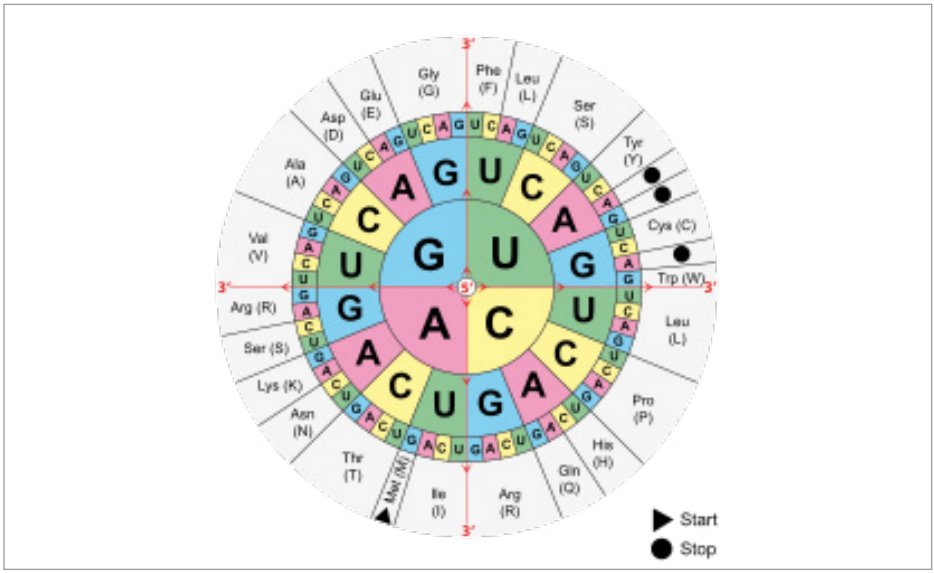
POSSIBLE APPLICATIONS

- **Pharmaceutical industries (synthesis of minigenes for gene therapy);**
- **In vitro diagnostic industries, for the prediction of pathogenicity of variations synonymous.**

ADVANTAGES

- Development of synthetic genes, with improved codon optimization, to be used both in the therapeutic and research fields;
- Greater efficiency in the synthesis of the target protein;
- Development of algorithms for the interpretation of synonymous mutations, which are often the source of confusion and misdiagnosis by clinicians.





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