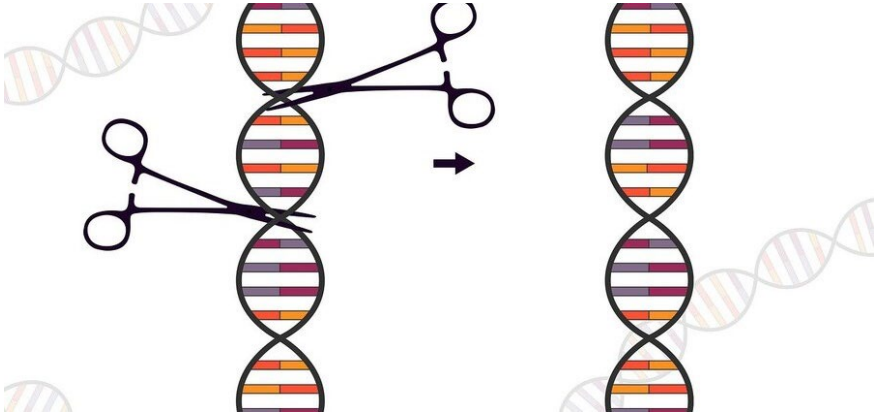
Technology Offer CSIC/MS/001

New Cas9 nucleases with unique characteristics for CRISPR-Cas genetic editing



New marine-origin Cas9 nucleases for genetic editing using the CRISPR-Cas system, with applications ranging from gene therapy to the improvement of plant and animal organisms.

Intellectual Property

Priority patent application filed

Stage of development

Proof of concept in *C. elegans* and few plant organisms

Intended Collaboration

Licensing and/or co-development

Contact

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Market need

CRISPR-Cas systems are widely used in genetic engineering to edit DNA and modify it precisely. They are currently used for gene therapy, as well as in biotechnology and agriculture to improve and modify specific organisms with desired characteristics.

However, current Cas9 variants show some drawbacks, such as lack of precision (off-targets), risk of immunogenicity, or large size, which makes it difficult to introduce them into certain cells.

For all these reasons, there is a need to characterize and develop new Cas9 molecules that can improve the versatility and efficiency of CRISPR-Cas systems.

Proposed solution

Two new Cas9 nucleases (DO1 and DO2) have been identified and characterized, which show low similarity to any other described functional Cas9. They also present unique tracrRNA and crRNA sequences. These new Cas9 proteins exhibit unique biophysical properties due to their marine microbial origin and deep-sea habitat.

Proof-of-concept tests have been conducted in the organism *C. elegans*, and new tests are being carried out in other cell types. Additionally, they have been tested in plat organisms, showing higher efficiency than current Cas9 variants.

Competitive advantages

- DO1 is smaller in size tan current Cas9 variants.
- They have high specificity, which reduces the possibility of off-targets.
- They are thermosensitive (they denature at temperatures >30°C).
- DO2 edits the same sequence as SpCas9 (to address immunogenicity issues).
- DO1 has catalytic activity at low temperatures (flexible protein structure)