

## CRISPR / Cas Animal Model Platform for Precision Medicine applications

### DESCRIPTION OF THE TECHNOLOGY

The advancement of biomedical sciences and the development of new diagnostic methods and increasingly more effective and safe therapies requires a deep understanding of the physiological and molecular mechanisms that trigger disease onset and progression.

An insight of those mechanisms can be gained from *in vitro* experimental systems (as cell cultures), but in many cases it is only possible to obtain from the context and complexity that only a living organism can provide (*in vivo* animal model).

Although non-vertebrate animal models can reproduce simpler pathologies or aspects of more complex ones, the study of the mechanisms of human pathologies can generally only be achieved using models recreated in mammals.

The predisposition to suffer certain diseases has in many cases a genetic component. In that case, faithful reproduction of a pathology or clinical

condition requires the replication of the genetic alterations involved. For this purpose, introducing precise genetic modifications in an animal model (usually a rodent) is essential.

Genetically modified animals can be generated by combining genetic engineering, embryology and assisted reproduction techniques. More recently, new methodologies of gene editing has been developed, but it has undoubtedly been the last to appear, the genetic edition through the CRISPR/Cas ('Clustered Regularly Interspaced Short Palindromic Repeats / CRISPR-associated proteins') system, which is opening the doors to the conception and generation of new models with precise genetic alterations.

This technology allows to more effectively introduce genetic modifications and also add them to established transgenic and mutants mouse strains, reducing the time necessary for their generation by other conventional methods.

### MARKET APPLICATION SECTORS

- Public or private research entities.
- Pharmaceutical and diagnostic companies.
- Biotechnology companies.
- Hospitals

### TECHNICAL ADVANTAGES AND BUSINESS BENEFIT

The CRISPR/Cas Animal Model Platform has an experimental, applied and multidisciplinary nature, and integrates the support of OMICs (Genomic, Proteomics, Cytomics, Metabolomics), bioinformatics and access to a complete facility for phenotypic characterization of the mutant models generated.

The CIPF has a modern animal facility to develop the PDX, especially indicated for the maintenance of immunodeficient mice in optimal conditions. In addition, there is a Screening service that can be used to test different therapeutic combinations or the selection of active compounds against the therapeutic targets identified in animal models.

This proposal provides a novel, state-of-the-art experimental platform with applications both in the clinical field and in research and development of new therapies, as well as a clear commitment to the development of precision medicine.

### CURRENT STATE OF DEVELOPMENT

We currently have the technology, infrastructure and human resources to carry out the design, generation

and validation of genetically modified mouse models.

#### INTELLECTUAL PROPERTY RIGHTS

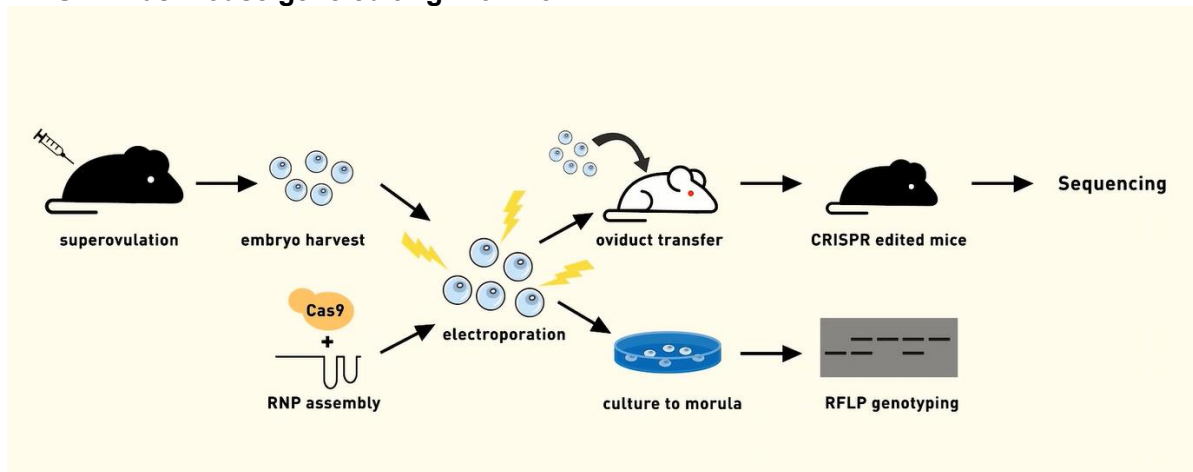
Does not apply.

#### COLLABORATION SOUGHT

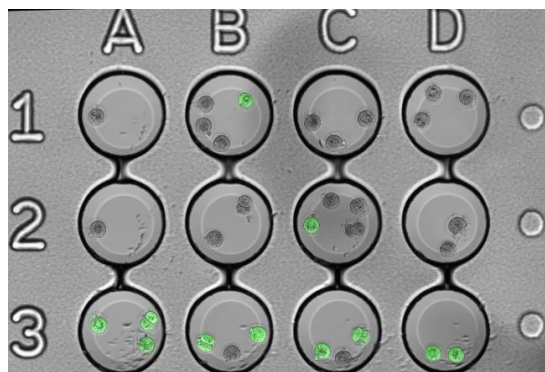
Collaboration in projects of basic and translational research at national and international level.

#### RELATED IMAGES

#### CRISPR/Cas mouse gene editing Workflow



Electroporated 1-cell embryos dividing to 2 cell embryo before being transfer to recipient mother



#### CONTACT INFORMATION

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