

## GENE EDITING FOR THE TREATMENT OF EWING'S SARCOMA

A research group from CIBER-ISCIIII, lead by Javier Alonso García, has developed a new alternative tool based on gene editing for the treatment Ewing's Sarcoma.

### The Need

Ewing's sarcoma is a rare and very aggressive tumor that affects children and adolescents. Overall survival is approximately 70% at 5 years of age, although this value varies significantly depending on the stage of the tumor.

Thus, patients with localized tumors at diagnosis have an overall survival of close to 80%, while patients with metastatic disease (mainly in other bones) have survival rates of less than 20%.

### The Solution

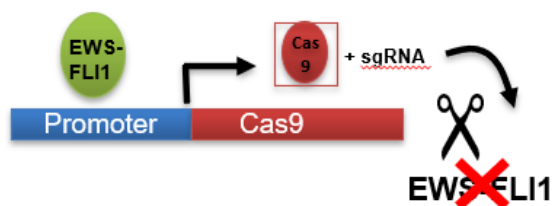
Several studies have shown that inhibition of EWS-FLI1 expression is able to inhibit cell proliferation, demonstrating the dependence of these tumors on this oncogenic chimeric protein.

Therefore, a drug or procedure aimed at inhibiting thactivity of the chimeric EWS-FLI1 protein or capable of e fully or partially reducing the expression of EWS-FLI1 could be useful in the treatment of these tumors.

### Innovative Aspects

In this technology we describe a procedure and kit for its application, which allows the nuclease to be expressed exclusively in EWS-FLI1 expressing Ewing sarcoma cells. The laboratory has shown through infection and western-blot assays that the Cas9 nuclease placed downstream of the specific promoter sequence is expressed exclusively in Ewing's sarcoma cells and not in other cell types tested, even though they express high levels of native FLI1. These experiments demonstrate the high specificity of the proposed strategy for Ewing's sarcoma cells expressing EWS-FLI1.

On the other hand, it has been shown that in addition to expressing the nuclease specifically, genetic inactivation of EWS-FLI1 has also been demonstrated by sequencing studies and that this genetic inactivation was associated with an inhibition of cell proliferation.



### Stage of Development:

Preclinical stage. The following objectives are aimed at evaluating different methods of delivery of the promoter-nuclease construct and the evaluation of other genes with therapeutic potential

### Intellectual Property:

- Priority Spanish patent application filed
- Suitable for international extension (PCT application)

### Aims

Looking for a partner interested in a license and/or a collaboration agreement to develop and exploit this asset.

### Contact details