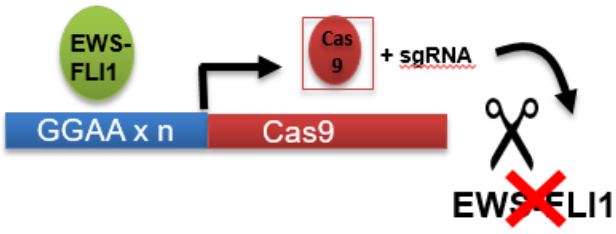
GENE EDITING FOR THE TREATMENT OF EWING'S SARCOMA

SUMMARY

The present invention relates to a new method of treatment for Ewing's sarcoma based on inhibition of proliferation in cells expressing EWS-FLI1.



DESCRIPTION

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.

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.

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 and the inhibition of proliferation for Ewing's sarcoma cells expressing EWS-FLI1.

COMPETITIVE ADVANTAGES

- Easy to prepare and obtain.
- Priority patent application (April 2022).

INNOVATIVE ASPECTS

- Inhibition of cell proliferation
- Greater specificity.

KEYWORDS

- Ewing´s sarcoma
- EWS-FLI1
- Cas9

MAIN ACTIVITY SECTOR

Ewing´s sarcoma

DEGREE OF DEVELOPMENT

Preclinical Stage.

COLLABORATION EXPECTED

Licensees of the patent application or interested in licensing and collaboration agreements for the development of the technology are sough.

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