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Institut d'Investigació Biomèdica de Bellvitge

Innovation Portfolio
17OMT001

Multifunctional RNA molecules to treat Ewing Sarcoma

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HIGHLIGHTS

- ✓ **New effective treatment for ES and other sarcomas**
- ✓ **Multifunctional RNA conjugate targeting Ewing Sarcoma metastasis**

TECH STATUS

- ✓ **TRL**: Animal Model Validation
- ✓ **IP**: Patent On Process

Problem to be solved

Ewing's sarcoma (ES) is a very rare variety of bone cancer, mainly affecting children and young adults. In general, these tumors are very aggressive, and although they are chemosensitive, they tend to reappear and develop metastasis. The most pressing unmet clinical need for patients with Ewing sarcoma is the prevention and treatment of metastasis.

Background

Ewing sarcoma is a malignant small, round cell tumor arising from bone and soft tissue in children and young adults. It grows in the bones or soft tissues near the bones and it can develop in any part of the body, but it affects to a greater extent the arms, legs, ribs, spine and pelvis. Most Ewing's sarcomas are diagnosed before age 20.

The median time from first symptom to diagnosis of Ewing sarcoma is often long, with a median interval reported from 2 to 5 months. Longer times are associated with older age and pelvic primary sites. Approximately 25% of patients with Ewing sarcoma have metastatic disease at the time of diagnosis.

Due to the lack of effective treatments, the survival of patients with pediatric sarcoma that present metastasis at the time of diagnosis is barely 20%.

Technology

Developmental sarcomas associated with chromosomal translocations (Ewing's sarcoma, alveolar rhabdomyosarcoma, synovial sarcoma) usually present a simple genome, where the main driver (oncogene) of the disease is an aberrant fusion protein generated as a result of the translocation. These aberrant proteins are specific to the cancer cell and, therefore, constitute an exceptional therapeutic target.The group has designed and developed a conjugate capable of reducing the expression of this protein and consequently the progressive capacity of these tumors, especially their metastatic capacity..

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Applications

This technology will be mainly directed to treat Ewing Sarcoma.

Nevertheless, it could also be used in alveolar rhabdomyosarcomas (ARMS) and other sarcomas, since they area characterized by the presence of another chromosomal translocationand also have elevated levels of EphA2 receptor.

Technology status

Its therapeutic efficacy has been demonstrated in vitro in cell lines and is being demonstrated in vivo in animal models. The group also has proposed the possibility of expanding the use of the conjugate using different siRNAs against the fusion proteins characteristic of these sarcomas.

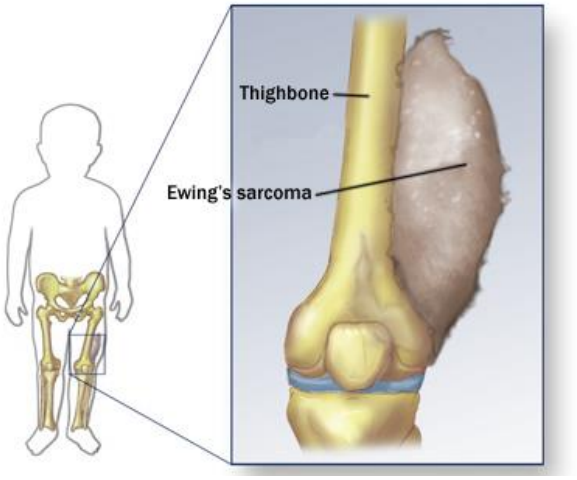
The therapeutic efficacy of the conjugate is being tested in an orthotopic ES model. The reproducibility of the conjugate in the ARMS is also being tested.

Market Opportunity

Between 1975 and 2010, childhood cancer mortality decreased by more than 50%. For Ewing sarcoma, the 5-year survival rate has increased over the same time from 59% to 78% for children younger than 15 years and from 20% to 60% for adolescents aged 15 to 19 years.

Childhood and adolescent cancer survivors require close follow-up because cancer therapy side effects may persist or develop months or years after treatment. The incidence for all ages is one case per 1 million people in the United States. In patients aged 10 to 19 years, the incidence is between nine and ten cases per 1 million people.

Ewing's sarcoma key players are Amgen Inc, Astellas Pharma Inc, Bristol-Myers Squibb Company, Cebiotex SL, Celgene Corp, Celldex Therapeutics Inc, among others.



Business Opportunity

Co-development or license agreement.

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