

Note for journalists: Further information, including a copy of the published article, is available online in the Science Translational Medicine press package <http://www.eurekalert.org/jrnls/scitransmed>. To access this information you need a subscription with a user ID and password.

### **SJD Barcelona Children's Hospital is, for the first time in the world, treating a childhood cancer of the retina with an oncolytic virus**

- The virus attacks and destroys the cancer cells of retinoblastoma or cancer of the retina
- The new treatment is aimed at more aggressive retinoblastomas, which do not respond to chemotherapy.
- The work appears on today's cover of the Science Translational Medicine journal.
- In addition, it has earned one of the most prestigious awards in the paediatric oncology world: the Odile Schweisguth Prize awarded by the International Society of Paediatric Oncology (SIOP).

**Barcelona, 24 January 2019.** A team of researchers from SJD Barcelona Children's Hospital has developed a new experimental treatment for retinoblastoma or tumour of the retina, a disease that accounts for 11% of malignancies in children under one year old. The treatment consists of injecting a genetically modified virus into the eye affected by the tumour. The virus seeks out, attacks and destroys cancer cells, and is for use in children whose tumours do not respond to conventional treatments. The work appears on today's cover of the prestigious journal Science Translational Medicine.

The research has experimentally reproduced tumours obtained from patients who had not been cured with the treatments currently available. Researchers at SJD Barcelona Children's Hospital and the biotechnology company VCN Biosciences have shown that the VCN-01 oncolytic virus, developed by genetic modification of adenovirus type 5—a common virus that normally causes cold-like symptoms—is only capable of infecting and multiplying in tumours and not in healthy cells of the retina. The virus's selectivity for tumours is based on the abnormal functioning of the retinoblastoma gene (*RB1*) in the cells affected by the tumour, in which there is an increase in the free amount of a molecule called E2F-1. The VCN-01 virus was genetically modified by VCN Biosciences so that its replication is initiated by the presence of free E2F-1 in the cells it infects. This property causes the virus to replicate selectively in retinoblastoma cells, whereas it does not replicate in healthy retinal cells, where E2F-1 is not free but bound to the product of the *RB1* gene.

SJD Barcelona Children's Hospital has begun a clinical trial, led by Dr Guillermo Chantada, Dr Jaume Catalá and Dr Jaume Mora, to treat patients with chemo-resistant eye tumours with the VCN-01 oncolytic virus. The objective of this experimental study, in which VCN Biosciences is also participating, is to describe the safety of the treatment and obtain the first indications of its clinical efficacy.

"Four years of work in the laboratory have been transformed into a clinical trial thanks to the committed support of the institutions and the involvement of professionals from multiple disciplines and hospital donors," explains Dr Ángel Montero Carcaboso, senior author of the publication. Dr Manel Cascalló, Executive Director of VCN Biosciences and an author of the work, stated that "The published data represent an important confirmation of the mechanism of action of our product, VCN-01, which is currently also being tested in adult patients suffering from other tumours, such as pancreatic cancer." The work has been paid for in part through the Spanish Ministry of the Economy's competitive "Retos" [Challenges] and "Miguel Servet" programs. Dr Guillem Pascual, first author of the work, received the prestigious Schweigsuth Prize from the International Society of Paediatric Oncology (SIOP) for this research. Other authors of the work belong to the Catalan Institute of Oncology (Barcelona), VCN Biosciences (Barcelona) and the Curie Institute (Paris), among other institutions.

Retinal cancer is diagnosed in 8,000 children around the world each year. It is the most common ocular cancer in children. Currently, when ocular preservation is indicated, children receive intra-arterial chemotherapy in a first phase, which is administered through a long, thin catheter introduced through the femoral artery (in the groin) and running to the ophthalmic artery, where it then administers the chemotherapy locally. Sometimes, chemotherapy drugs are injected directly into the eye, into the vitreous humour. In 30% of cases, however, the tumour does not respond to either of these treatments and ophthalmologists have no other option than to remove the affected eye to prevent the cancer from spreading to other organs in the body, since if this occurs, the likelihood of a cure is very low. The new viral treatment aims to prevent the removal of the eye and reduce cases of blindness in patients with retinoblastoma.

### **New therapies for childhood cancer**

This new treatment belongs to a group of new advanced therapies that are being implemented by SJD Barcelona Children's Hospital and that represent a new paradigm, allowing personalised treatment for some types of cancer. The advanced therapy trial with CART-19, for leukaemia, is based on the use of the patient's own cells to destroy their leukaemic cells.

The hospital has launched several clinical trials based on innovative therapies aimed at the treatment of diffuse brainstem glioma (a cancer that is currently incurable), retinoblastoma (cancer of the retina) and type B acute lymphoblastic leukaemias, treated with CART-19.

## **A new centre to promote these therapies**

SJD Barcelona Children's Hospital is currently the centre that has treated the most childhood cancer cases in Spain, and among the most in Europe, seeing 288 new cases last year and 38 second opinions. To promote and foster these new therapies, in 2020 SJD Barcelona Children's Hospital will open the SJD Paediatric Cancer Centre Barcelona, a centre for translational research and care, aimed at implementing innovative therapies such as CART-19 and new treatments based on immunotherapy and precision medicine, which detect each patient's genetic alteration to create a fully personalised treatment.

SJD Barcelona Children's Hospital raised the 30 million euros needed for the new centre through the #ParaLosValientes ["For The Brave"] campaign, which was joined by thousands of individuals, as well as a large number of companies and organisations from various fields. The construction of SJD Paediatric Cancer Centre Barcelona was then able to begin at the end of 2018.

The SJD Paediatric Cancer Centre Barcelona will be one of the largest centres in Europe, with a capacity to treat 400 new cancer patients per year. In total, its facilities will occupy an area of 8,345 square meters in a four-storey building connected to SJD Barcelona Children's Hospital.

The new centre will have 40 rooms, 8 isolation rooms for haematopoietic stem cell transplantation, 30 day-hospital bays, 20 offices for outpatient consultations, nuclear pharmacy facilities and spaces to expand existing research laboratories.

### **More information:**

Albert Moltó/Lourdes Campuzano  
Communication  
SJD Barcelona Children's Hospital  
Tel.: +34 932532150/+34 605695277/+34 665500214