

First precision protocol against children's cancer - Biotech

Highlights: First precision protocol against children's cancer - Biotech. Born in Italy and based on the genome, neuroblastoma in the sights. The first study, conducted on 18 children, was recently published in the Journal of Translational Medicine. Two-thirds of these cases were classified as 'very high priority', opening up the possibility of benefiting from drugs already approved or new therapeutic agents in development. The genetic identikit of the tumor is obtained and on this basis the therapies that have the greatest probability of success are then identified.

One of the most aggressive tumors in children, neuroblastoma, has entered the sights of precision medicine thanks to Preme, the first Italian protocol in this sector based on genetic data and promoted by multiple centers.

It is coordinated by the Giannina Gaslini Institute of Genoa with the group of Mirco Ponzoni, head of the laboratory of Experimental Therapies in Oncology, and is co-financed by Gaslini himself, by the Italian Foundation for the Fight against Neuroblastoma and the Open association;

Among others, Ceinge Biotecnologie Avanzate Franco Salvatore of Naples participates with groups directed by geneticists Mario Capasso and Achille Iolascon of the Federico II University of Naples.

The samples taken in several Italian centers from children affected by this form of tumor are characterized at Gaslini and then analyzed for mutations at Ceinge and Cibio; the data are then used to develop preclinical models of neuroblastoma again at Gaslini.

In this way, the genetic identikit of the tumor is obtained and on this basis the therapies that have the greatest probability of success are then identified.

This is possible thanks to the collaboration of biologists, geneticists, clinicians and bioinformaticians, observe those responsible for carrying out the project Chiara Brignole and Fabio Pastorino, both from Gaslini.

Born as a research project in 2019, Preme has become a true clinical protocol since 2022 led by the Gaslini Institute, with the oncologist Loredana Amoroso.

The first study, conducted on 18 children, was recently published in the Journal of Translational Medicine.

"Thanks to new generation sequencing techniques and sophisticated bioinformatics analyzes conducted at Ceinge and Cibio of the University of Trento - observes Capasso, who leads the team of bioinformatic scientists at Ceinge - it emerged that 84% of patients affected by neuroblastoma had genomic alterations susceptible to therapeutic intervention".

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"Molecular targeted therapy was applied to four patients, while genetic counseling - says Pastorino - was offered to two patients with germline mutations that indicated a familial predisposition to the development of tumors".

According to Ponzoni, "targeted therapies, adapted to the specific mutations present in patients, the personalization of treatment and the use of advanced preclinical models transform translational research into tangible benefits for patients, improving their prognosis".

For the scientific director of the pediatric hospital, Angelo Ravelli, the Preme program is "a fundamental resource for researchers, which favors the identification of new molecules susceptible to more effective and specific therapeutic interventions, especially for the benefit of refractory or relapsed forms".

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