Press alert - February 12, 2024

International Childhood Cancer Day

Pediatric brain tumors: the innovations of Institut Curie towards new therapies and fewer after-effects

On the occasion of the 22nd International Childhood Cancer Day on February 15, 2024, Institut Curie is focusing on children's brain tumors. These rare tumors, which affect nearly 500 new children each year in France, are biologically very different from those of adults and have a very variable prognosis. At Institut Curie, researchers and doctors are engaged in promising research to provide better and more extensive treatment to children.

In children under the age of 15, brain tumors are the most common cancers after leukemia[1]. These rare cancers can occur in the brain, brain stem, cerebellum, or spinal cord. There are many types of them: **embryonic tumors (medulloblastoma and atypical rhabdoid teratoid tumors (ATRT)), glial tumors (or gliomas), ependymomas**, and even more rarely, germ tumors, choroid plexus tumors, meningiomas, craniopharyngiomas, etc. **The standard treatment involves surgery, chemotherapy, radiotherapy, and proton therapy.** Thanks to very specific focusing and irradiation properties, proton therapy is particularly suitable for these pediatric brain tumors located in particularly sensitive and developing regions. **Institut Curie Proton Therapy Center in Orsay is the 1st center in France and 3rd in the world among the ones that focus on this specific type of treatment.**

At Institut Curie, in the heart of the SIREDO center[2], the 1stintegrated center in France dedicated to cancers affecting people under the age of 25. The continuous work between research and the clinic has allowed considerable advances in the fight against pediatric brain tumors.

Medulloblastoma: exploring the biology of tumors for more efficient treatment

Medulloblastoma develops in the cerebellum and is the most common malignant brain tumor in children (one case in 20,000 children per year in France). At Institut Curie, several teams are working to characterize and better understand the mechanisms involved in the development of medulloblastoma, a group of very heterogeneous tumors. **Signaling in Development and Brain Tumours team led by Dr. Olivier Ayrault** implements original and multidisciplinary approaches that integrate genomic, transcriptomic, proteomic, and even metabolomic data thanks to state-of-the-art equipment to identify new biological mechanisms. In 2018, works published in *Cancer Cell* described **for the first time the involvement of the SRC protein in the most widespread subgroup in the clinic.** Today, work continues: studies are underway to evaluate how to target this SRC protein and develop new therapeutic strategies. This work is carried out in collaboration on an international scale (Germany, England, Canada, Japan and the United States) and in direct link with the clinical teams in France, in particular as part of the Paris Kids Cancer led by the AP-HP, Gustave Roussy and Institut Curie.
Another team from Institut Curie led by **Dr. Celio Pouponnot is interested in an aggressive subtype of medulloblastoma named Group 3, which is quite particular by deciphering the signaling pathways involved within cells in tumor processes.** The researchers have in particular identified a pharmacological agent targeting the TGFß pathway (signaling pathway transforming growth factor beta) as

[1] Brain tumors (e-cancer.fr)
[2] SIREDO for Care Innovation Research in Oncology of Children, Adolescents and Young Adults

being of therapeutic interest in this subgroup of high risk of medulloblastoma. Studies are continuing internationally on the importance of targeting this pathway.

By the way, **the Somatic Genetics Unit of Institut Curie is the national reference laboratory for all European clinical trials on medulloblastoma**, and Professor François Doz, Deputy Director of Clinical Research, Innovation and Teaching at SIREDO, is the national leader of European clinical trials on medulloblastomas of standard risk in children over three years of age.

Elucidating the hereditary origins of pediatric brain tumors

Another field of investigation of the teams of Institut Curie: the search for **potential genetic predispositions of medulloblastoma**. Thanks to collaborative national work carried out jointly by the teams of Institut Curie and Gustave Roussy, genetic counseling for families of children with medulloblastomas as part of a predisposition could be more adapted to avoid unnecessary stressful situations and anticipate the real family risk as closely as possible.

The hope of immunotherapies in rhabdoid brain tumors

Rhabdoid tumors are particularly rare, aggressive and develop during infancy. They are mostly observed in the central nervous system, **under the name of atypical teratoid rhabdoid tumors (ATRT)**, whose overall survival does not exceed 40%. **The team of Professor Franck Bourdeaut, pediatrician and researcher at Institut Curie, revealed in October 2023, thanks to the confrontation between imaging and bioinformatics tools applied at the single cell scale, different neural progenitors likely to be at the origin of the establishment of ATRT tumors.** In addition, other major works led by Prof. Franck Bourdeaut, in collaboration with the teams of Dr. Eliane Piaggio and Joshua Waterfall at Institut Curie, have allowed **to identify an immune response of epigenetic origin**, hinting at the possibility of conducting clinical trials in the field. A collaboration with the team of Dr. Celio Pouponnot is studying how the irradiation of these ATRTS could make them more visible by the immune system, and a European clinical trial on rhabdoid tumors ATRT will be launched in 2024 to evaluate the effect of this irradiation.

Tumors with BCOR rearrangement

These tumors with BCOR gene rearrangement are rare embryonic tumors that appear in young children, without standard treatment and with a poor prognosis. **At Institut Curie, the Computational biology and integrative genomics of the cancer team led by Dr. Florence Cavalli is working to precisely characterize these tumors with alteration of the BCOR gene**. In collaboration with the teams of Dr. Raphaël Margueron, Prof. Franck Bourdeaut and Saint-Anne Hospital, whose project aims to better understand the tumor heterogeneity of these aggressive cancers in order to identify the mechanisms responsible for the progression of these rare pediatric tumors, for which there is no standard treatment.

Key figures

Pediatric cancers in France
2,283 new cases per year
1% of all cancers in France
83% survival at 5 years

Incidence of pediatric cancers
28% from leukemias
27% of tumors of the central nervous system, including brain tumors
11% lymphomas

At Institut Curie
390 new patients per year
SIREDO, the 1st integrated center in France dedicated to cancers affecting people under 25 years of age
43 clinical studies in 2022,
including **100 children and teenagers**

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About Institut Curie

Institut Curie, France's leading cancer center, combines an internationally-renowned research center with a cutting-edge hospital group, treating all types of cancer, including the rarest. Founded in 1909 by Marie Curie, Institut Curie has 3 sites (Paris, Saint-Cloud and Orsay) with over 3,700 researchers, physicians and health professionals working on its 3 missions: treatment, research and teaching. A foundation with public utility status, Institut Curie is authorized to accept donations and bequests, and thanks to the support of its donors, is able to accelerate discoveries and improve patient treatment and quality of life. *To learn more :* ***curie.fr,*** *Twitter, Facebook, LinkedIn, Instagram*