

Press release

Neuroblastoma: Liquid biopsies to detect relapse of childhood cancer early

(Vienna, 29.1.2024) **Neuroblastoma mainly affects toddlers and young children - in the EU region there are 1500 new cases per year. Neuroblastoma is a malignant tumor of the peripheral nervous system and around 50% of patients are high-risk cases. Recurrences occur frequently, and conventional therapies are no longer effective for these children. With liquid biopsies it is possible to monitor therapy success and to predict the recurrence of the tumor in time to take medical countermeasures. Scientists from leading European research institutions in pediatric oncology are testing this promising diagnostic tool under the coordination of the European Society for Pediatric Oncology (SIOPE) and scientific lead of St. Anna Children's Cancer Research Institute and Princess Máxima Center for pediatric oncology. The framework is a five-year Horizon Europe project.**

Liquid biopsies are a promise for the future of personalized cancer medicine: Many children with high-risk neuroblastoma, i.e. 50 percent of all neuroblastoma cases, are at risk of not responding to therapy and developing a recurrence. Until now, the success of the therapy has been monitored with medical imaging procedures and bone marrow examinations. These examinations are expensive, invasive, and stressful for children who need anesthesia. Liquid biopsies are small blood samples, so the procedure is minimally invasive, and comparatively simple. In the laboratory, the sample can be used to determine whether children have responded to therapies. Finally, an impending recurrence can be detected early. Genetic tumor markers in the blood plasma, small pieces of DNA and messenger RNA (mRNA), which are released by tumor cells and provide information about the genetic changes in the tumor, are examined. In the event of a relapse, these can help to find targeted therapies for children with neuroblastoma. For example, the enzyme anaplastic lymphoma kinase (ALK) plays a decisive role in cancer development. If a child's tumor has a fault in the ALK-gene, it could be targeted with ALK inhibiting drugs.

Liquid biopsies in the clinical setting for the first time

A total of 24 leading European research institutions in pediatric oncology have now joined forces for an EU project funded by Horizon Europe. The aim is to study the benefit of these new diagnostic methods – small blood samples will be collected several times from 150 patients with high-risk neuroblastoma and will be examined in laboratories such as Labdia Labordiagnostik GmbH and the Princess Máxima Center. The European Society for Pediatric Oncology (SIOPE) is coordinating the research project, which is funded with a total of eight million euros. The goal of the project with the title A SIOPE study to MONitor NeuroblastomA relapse with Liquid biopsy Sensitive Analysis - in short: MONALISA – is to develop a clinical study that can be quickly implemented in clinical practice. “And this is already the first special feature,” says Sabine Taschner-Mandl, PhD, Principal Investigator at St. Anna Children's Cancer Research Institute, who is co-leading liquid biopsy diagnostics in MONALISA. “We are using liquid biopsies for children with neuroblastoma for the first time in a clinical setting; so far, we have only tested them in research,” says the tumor biologist.

Lieve Tytgat, MD, pediatric oncologist specialized in neuroblastoma at the Princess Máxima Center for pediatric oncology, and scientific co-lead of the MONALISA project, says: “This research is a real breakthrough in the world of non-invasive diagnostics. Liquid biopsies are increasingly commonly used in adults with cancer. With this study we aim to catch up with research in adults; an important development for children with cancer.”

Monitoring the response to therapy and disease course is of great importance for improving the chance of survival of patients with high-risk neuroblastoma. MONALISA aims to close existing gaps in diagnostics and establish liquid biopsies as a standard procedure for monitoring relapsed neuroblastoma.

The method could also serve as a blueprint for other pediatric cancers. Marie Bernkopf, scientist at St. Anna Children's Cancer Research Institute and head of the Labdia Laboratory Diagnostics Department where the analyses are carried out, emphasizes that technological progress here will bring a significant improvement for



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children and parents. For example, in some countries, mobile nurses will be able to take blood samples at home for the check-ups, thus eliminating the burdensome trip to the clinic.

MONALISA also aims to analyze how patients and parents experience the use of liquid biopsies to be able to adapt the procedure in the future. With the project, the scientists are getting one step closer to effective personalized medicine in children with neuroblastoma and will offer hope for a better chance to be cured.

About MONALISA:

The project, funded by the Horizon Europe funding program of the European Commission with eight million Euro, will run for five years. Among the institutions participating in the project are St. Anna Children's Cancer Research Institute and Labdia Labordiagnostik GmbH, the Berlin Charité, the French Institute Curie, the Dutch Princess Máxima Center for pediatric oncology, the University Hospital in Motol, the Interuniversity Microelectronics Center, the Flemish Institute of Biotechnology, the Institute Gustave Roussy, the Academic Medical Center in Amsterdam, the University of Leeds, the National and Kapodistrian University of Athens, the Agia Sofia Childrens Hospital, the Eberhard Karls University Tübingen, the Solving Children Cancer, the Institute Giannina Gaslini, the University of Gent, the Childrens Health Ireland, the SIOPE Association, the Vall d'Hebron Research Institute, the Evaluation Software Development, Vlaams Instituut Biotechnologie - Flanders Institute for Biotechnology and further Austrian partners from the SIOPE Europe, Medical University of Innsbruck and the Austrian Institute of Technology (AIT).

The Kick-off Meeting of MONALISA takes place in the St. Anna Children's Cancer Research Institute from 29th January to 30th January. The European Society for Pediatric Oncology Neuroblastoma is organising the upcoming SIOPE Translational Research Meeting from 30.1. to 1.2. in Vienna at the Medical University, Van Swieten Saal, Van-Swieten-Gasse 1a, 1090 Vienna. The first day is dedicated to the topic of liquid biopsy.

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