

Letter Of Intent (LOI)

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Project Title:

SAPERE – SARcoma in PEdiatric age: a molecular REgistry and network**ABSTRACT**

Malignant tumors are rare in children with an annual incidence rate of 12,7 new cases per 100.000 children per year in Europe. Brain tumors are the most frequent malignancies among the solid tumors, followed by Neuroblastoma, Wilms tumor and Soft Tissue Sarcoma (STS).

STS are a heterogenous group with variable epidemiological patterns and histology. STS may arise in different anatomic sites but, in some cases, they may be associated with underlying genetic predisposition syndromes. Some STS are typical of pediatric age (i.e. rhabdomyosarcoma), other more commonly develop during adulthood and adolescents. There is growing evidence that at least some of them are biologically and clinically distinct from their adult counterpart. A substantial group of mesenchymal tumors with uncertain clinical behavior are usually also included in the group of STS.

In spite of the progress in treatment with a multidisciplinary approach, the prognosis in patients with advanced disease has not improved in the last decade.

The advances in molecular characterization are redefining the histologic and prognostic categories in pediatric sarcomas and new entities with defined molecular characteristic are emerging. Molecular alterations such as the FOXO1-PAX3/7 fusion in rhabdomyosarcomas may influence the stratification of patients in clinical trials, while other molecular alterations, such as ALK or NTRK fusions in IMT or IF, may represent potential therapeutic targets.

In France and Germany, the creation of a national network for pediatric tumors with the establishment of a tumor registry, including also extensive molecular characterization, is paving the way to possible individualized treatment of these malignancies.

In Italy, the access to molecular characterization in the pediatric population is still limited and the limited knowledge still exists regarding their pathogenetic mechanisms.

The General objective of the project is to establish an infrastructure with technical, structural and information basis, to provide a consultation system for a personalized treatment and generate a registry of STS. This will ultimately result in improved patients' care and will allow to create an European and ultimately International, effective collaboration.

The UO3 will be involved in the creation of a radiology and surgery database to be integrated with the clinical database.

Il Progetto non richiede alcuna spesa da parte dell'Azienda Ospedaliera