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Improving the care of children in Europe with rare cancer

The European Society for Paediatric Oncology together with Coordinators for the PARTNER Project and ERN PaedCan highlight the importance of collaboration in order to improve the care of children in Europe with rare cancer

The challenge of **Very Rare Tumours (VRTs)** in paediatrics form a heterogeneous group of cancers that are infrequently encountered in daily practice, even in large paediatric oncology centres. For children affected by exceptionally rare tumours, the very low incidence of their disease has often limited the interest in research capable of collecting significant clinical and biological data. It is particularly difficult to produce evidence-based treatment guidelines for these patients with VRTs and consequently, physicians are forced to treat such patients only on an individual basis.

Until recently, the paediatric oncology community has shown little interest in this group of tumours, which partially explains our lack of understanding of the biology of these disorders and the difficulties encountered in conducting clinical trials.

Some VRTs arise at the paediatric age, such as pleuro-pulmonary blastoma or pancreatoblastoma. Other tumours develop more commonly during adulthood or may even be frequent in adult patients (i.e. colon cancer or malignant melanoma), but they only rarely develop in children and adolescents. There is growing evidence that at least some of them are biologically and clinically distinct from their adult counterpart. In addition, a substantial group of “borderline” tumours with uncertain clinical behaviour and no standard treatments are usually also included in the group of VRTs.

Diagnosis and treatment guidelines adopted for children often are extrapolated from those used to treat adults. However, the clinical and biological characteristics of the diseases may differ in children. Moreover, the more severe long-term consequences of radiotherapy and chemotherapy in children have to be considered.

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Within such a context, these factors emphasise the need for a tailored therapeutic approach.

The recognition and treatment of such patients pose a challenge for paediatric oncologists and it may take many years to establish diagnostic guidelines and treatment concepts.

Even at the European level, some diseases are too rare to enable the recruitment of a sufficient number of cases to conduct clinical trials leading to evidence-based treatment guidelines. Therefore, the need to develop international collaborations dedicated to paediatric VRTs is imminent.

An international network dedicated to VRTs should also be able to take in charge these 'rare tumours' as promptly as possible, reducing the time gap between the first description of a tumour and the formulation of guidelines or treatment protocols.

The European paediatric oncology community, including all SIOP Europe members, have increasingly recognised the necessity to develop projects dedicated specifically to rare paediatric tumours to overcome all these obstacles. Investing more funds in investigating the relatively uncharted territory of rare cancers may be one way to maximise impact.

Introducing the PARTNER project

PARTNER (Paediatric Rare Tumours Network – European Registry) is a three-year EU project that is part of the European Reference Network for Paediatric Cancer (ERN PaedCan). Over the three-year duration, it aims to create a Paediatric Rare Tumour European Registry dedicated to children and adolescents with very rare tumours (VRTs) linking existing national registries and to provide a registry for those countries that do not already have a registry for VRTs in place.

Concretely, the following points detail the reasons why the PARTNER project is needed to tackle the challenge of VRTs:

- Very rare tumours (VRTs) are clearly part of the orphan diseases in Europe;

- A European registry will provide oversight and is a step further on the way to improve the care of VRTs patients;
- Data collection may contribute to optimised consultation of patients with VRTs;
- Experts are ready to increase clinical research and knowledge and;
- A European registry is a fundamental tool to sustain a European network dedicated to VRTs.

The objective of this project is a platform for VRTs that could represent a model for a comprehensive approach (case registration, international case consultation and treatment recommendations and website to provide information to parents/patients) in the field of rare diseases. Specifically, the PARTNER project will create such a platform including a European registry through:

- The identification of the VRTs entities;
- The selection of a core of variables for specific entities;
- The use of common definitions and;
- Implementation of harmonised procedures for data collection, data quality control and central review.

The expected outcomes of the PARTNER project are quite concrete and specific and can be summarised as follows:

- Creating a harmonised platform that will constitute a necessary instrument to improve care for children and adolescents with VRTs;
- Harmonising data in the existing national registries in collaboration with the JRC registry platform;
- Linking the EU registry with a virtual consultation system;
- Providing a registry for those countries that do not already have a registry for VRTs in place;

- Enabling detailed diagnostic/treatment recommendations that can be easily accessed by EU healthcare providers;
- Increasing the capacity to provide international consultation and fostering access to expert diagnosis, and treatment improving the chances of cure for children with VRTs across Europe and;
- Improving care for patients through the instruments outlined and hence reducing inequalities in cancer outcome across the EU Member States through improved information channels and accessible expertise.

Links to the European Reference Networks in Paediatric Cancer (ERN PaedCan)

As of the year 2000, national initiatives were launched in different European countries having recognised the necessity to develop dedicated projects for rare paediatric tumours. Although they have shown various activity levels, these national groups share the idea that multinational cooperation is of great importance in order to improve knowledge and the outcome for children with VRTs.

The European Reference Network on Paediatric Cancer (ERN PaedCan) aims to help national health systems cooperate in the interest of patients and is, therefore, the ideal framework within this multinational collaboration on VRTs.

The PARTNER project is very much within the scope of the ERN PaedCan network facilitating the lives of both healthcare providers and patients, whose conditions require specialist expertise and tools not widely available, due to low case volumes or a lack of resources. By cooperating and exchanging life-saving knowledge at European level, patients across the EU should be able to gain access to the best expertise available. This network offers a platform for virtual consultations allowing information to travel, rather than the patients and supports training and knowledge sharing.

PARTNER project benefits for public health

The strategic value of PARTNER in the field of public health is based on the European wide gathering of information on treatment of VRTs and the provision of

this information to experts generating new guidance recommendations for daily practice for use by ERN and non-ERN Institutions. The collection of data will contribute to optimised consultation of patients with VRTs. Consequently, experts will increase clinical research and knowledge. The proposed tasks and milestones will increase the collaboration amongst the member states in paediatric oncology and will be able to also include LHEAR (Low Health Expenditure Average Rate) countries in the process.

Innovation will be embedded in the new platform: original IT tools will link the existing databases with the help of the EUPID system assuring interoperability and anonymisation of patients' data. This may serve as a model for data set integration at the EU level, thus, paving the way for the integration of registries in the near future across the globe. This project builds upon work and actions from the EU-funded ENCCA and ExPO-r-Net projects. The information collected and tools developed will be disseminated to key stakeholders (other ERN, European Clinical Trials Groups, parents and patients) to enhance further collaborations.

In conclusion, it is expected that the PARTNER project will strengthen the collaboration between the countries involved and stimulate the formation of similar groups in other European countries. In addition, collaboration with already existing disease-oriented registries and collaborative groups will be actively sought, as worldwide initiatives are necessary. This will improve research quality hopefully and the outcomes of treatment for children who have, until recently, been rather neglected. ■

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