The E*X*PeRT Initiative

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Gianni Bisogno

Working to improve the knowledge and find appropriate treatments for children with rare diseases means to confront with many disadvantages: lack of interest from the scientific, economic, and political community; lack of funds; and lack of colleagues to work with to exchange ideas and projects. Although cancer is a rare disease in children, pediatric oncologists have been able to improve treatment, perform research, raise interest, and find support. Maybe more importantly, the impossibility to perform meaningful studies on the few patients treated in each pediatric oncology center has fostered the search for links and connections with other centers, initially on a national level and subsequently to a more international dimension. Until recently, children suffering from cancers with extremely low incidence have participated in this progress to a far less extent. Therefore, several national groups specifically focussing on rare cancers in childhood have been founded in the new millennium. These initiatives have contributed to increase awareness of the problem of children with very rare tumors, and ultimately lead to the formation in June 2008 of a new cooperative group denominated E*X*PeRT – European Cooperative Study Group for Paediatric Rare Tumors.

The main aim of this group is to empower the research on rare pediatric tumors by promoting collaboration between the founder national groups: Italy, France, United Kingdom, Poland, and Germany. Data exchange, retrospective and prospective studies, harmonized and internationally recognized guidelines, and E*X*PeRT consultation to assist clinical decision and international case registry are the undergoing initiatives. The formation of similar groups in other countries is also expected and supported.

As an initial initiative, the E*X*PeRT group decided to combine the data collected by each national group on some tumor entities included in the list of very rare pediatric tumors. For this purpose, a harmonized core data sheet for uniform documentation of clinical data of children with rare cancers was developed. This data sheet has then be adapted for three retrospective studies focussing on ovarian Sertoli–Leydig cell tumors, pancreatoblastoma, and pleuropulmonary blastoma.

Pancreatoblastoma (PBL) was selected as the first tumor type to be analyzed and was the subject of the first publication: in a 10-year period, 20 cases only were collected from Italy, France, United Kingdom, Poland, and Germany. This suggests that even at a European level, it is too rare to allow the recruitment of sufficient number of cases to conduct clinical trials leading to evidence-based treatment guidelines. Nevertheless, the E*X*PeRT group would propose a sort of standard approach for PBL, including a surgical staging system, an initial conservative surgical approach, chemotherapy according to PLADO regimen, and a post-chemotherapy aggressive surgery, on both primary tumor and metastases, when present.

Additional studies included 42 Sertoli–Leydig cell tumors. Thus, they represent the largest series of these tumors reported to date. Both provide significant new information, in particular, with regard to the further development of therapeutic strategies in these rare

G. Bisogno
Division of Hematology/Oncology,
Department of Pediatrics,
University-Hospital of Padova, Padova, Italy
e-mail: gianni.bisogno@unipd.it

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tumors. In 2010, the first results have been presented at the meeting of the International Society of Pediatric Oncology (SIOP), and publication of these studies is expected in 2011.

To perform research on rare tumors requires an enormous and prolonged effort as results may be visible only in the long term. We do not expect that E*X*PeRT will radically change the scenario, but at least it will be able to bring this problem to the attention of the medical community. Remarkably, despite potentially heterogeneous diagnostic and therapeutic strategies used in the different national groups, such studies may provide new options to promote our knowledge of rare cancers and to advance clinical management. The abovementioned small studies, however, demonstrate that international cooperation in very rare tumors is feasible and support the benefit of the foundation of the E*X*PeRT group (Table 13.1). International cooperative studies on rare entities may thus significantly help in advancing our clinical understanding and in improving our clinical care in these tumors. Further steps are needed to facilitate the collection of larger numbers of cases by creating a prospective international registry and to set up a biorepository to stimulate biological studies to improve our understanding of the molecular genetic basis and the natural history of specific rare tumors.

Table 13.1 Founding members and "core group" of E*X*PeRT

National group	Coordinator	Clinical speciality
TREP, Italy	Gianni Bisogno	Pediatric oncology
	Giovanni Cecchetto	Pediatric surgery
	Andrea Ferrari	Pediatric oncology
UKCCSG, UK	Bernadette Brennan	Pediatric oncology
PPGGL, Poland	Ewa Bien	Pediatric oncology
	Jan Godzinski	Pediatric surgery
	Teresa Stachowicz-Stenzel	Pediatric oncology
France	Daniel Orbach	Pediatric oncology
	Yves Reguerre	Pediatric oncology
STEP, Germany	Ines Brecht	Pediatric oncology
	Dominik T. Schneider	Pediatric oncology

We hope that with E*X*PeRT, the enormous disadvantage of rare diseases may be transformed in the advantage that doctors and scientists recognize they are forced to collaborate on a large international level. This will hopefully improve the quality of research and the treatment results for children that have been, until recent years, partially neglected.