

Harmonizing Treatment and Improving Outcomes for Pediatric Adrenocortical Tumors: A Pan-European Prospective Clinical Trial by EXPeRT and ERN

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INTRODUCTION

Pediatric adrenocortical tumors (pACTs) are extremely rare, accounting for approximately 0.2% of childhood cancers, yet they present significant clinical challenges due to their aggressive nature and heterogeneous presentation. Current treatment protocols across Europe vary widely, leading to inconsistent outcomes and fragmented understanding of the disease.

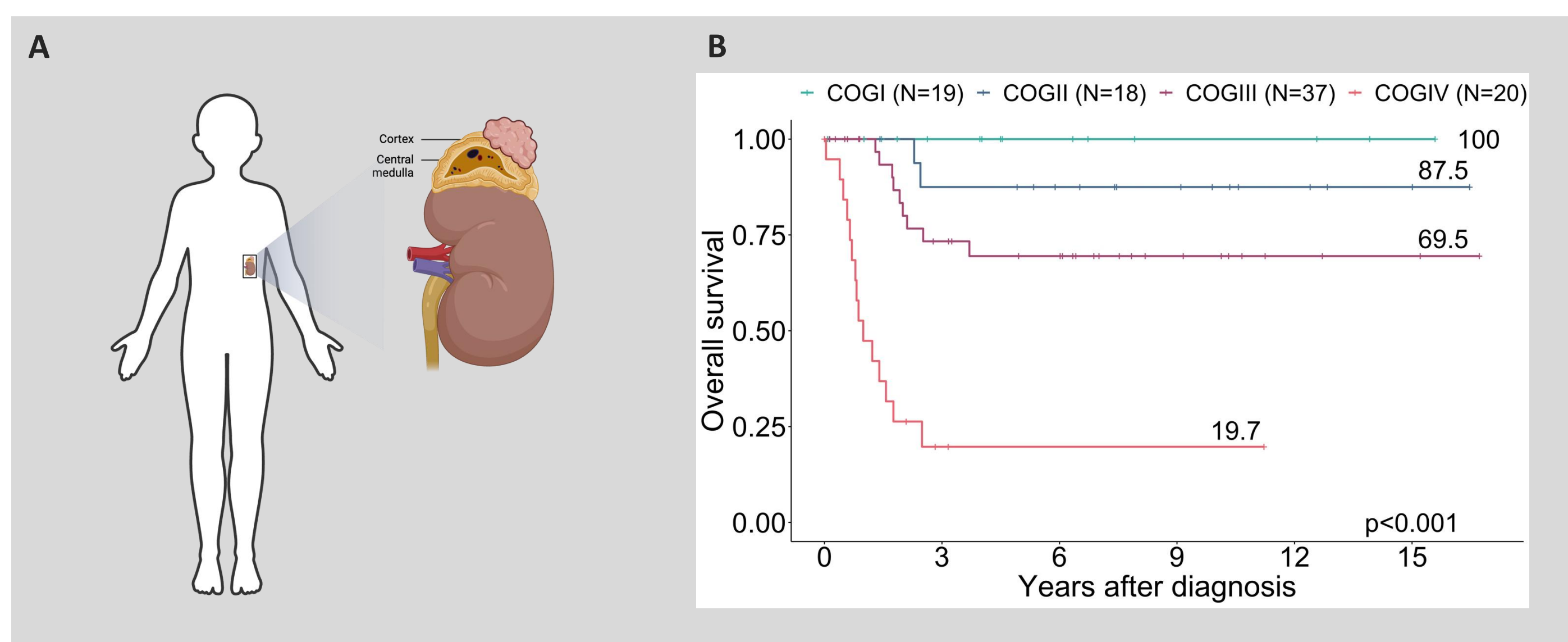


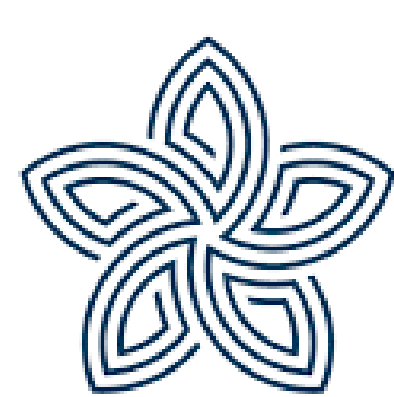
Fig. 1: Pediatric adrenocortical tumors arise from the adrenal cortex (A). Prognosis is closely associated with disease stage (COG) and is dire in advanced cases. (B)



The EXPeRT group [European Cooperative Study Group for Pediatric Rare Tumors in collaboration with the Endo-ERN] proposes a Pan-European prospective clinical trial aimed at harmonizing treatment protocols, improving outcomes, and enhancing our understanding of pACTs.

EXPeRT

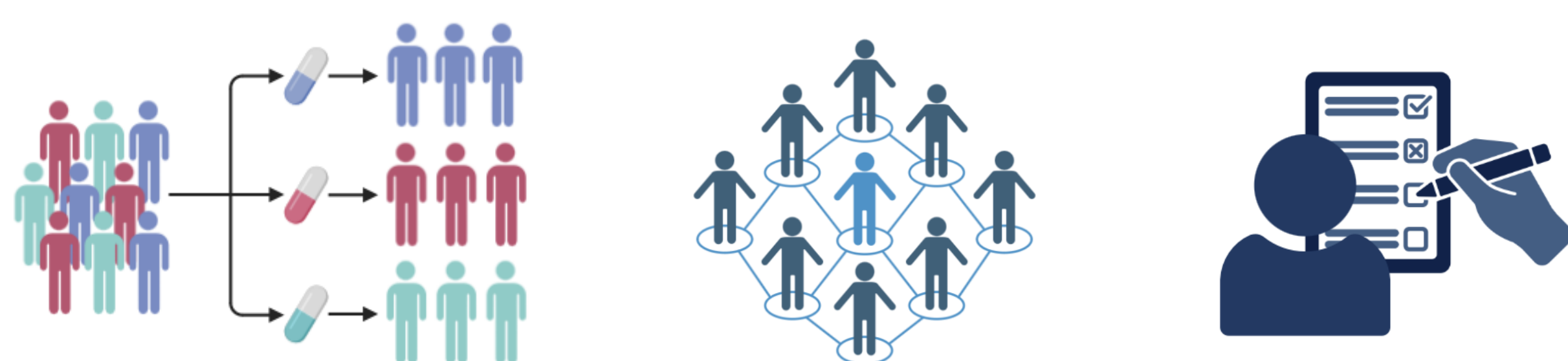
European Reference Network



Endo-ERN

OBJECTIVE

This trial aims to establish a standardized treatment approach for pACTs across Europe, particularly focusing on high-risk patients with poor prognosis under current treatments. By creating a cohesive and collaborative research environment, we intend to gather comprehensive data that will inform evidence-based practices, ultimately improving survival rates and quality of life for affected children.



CONCLUSION

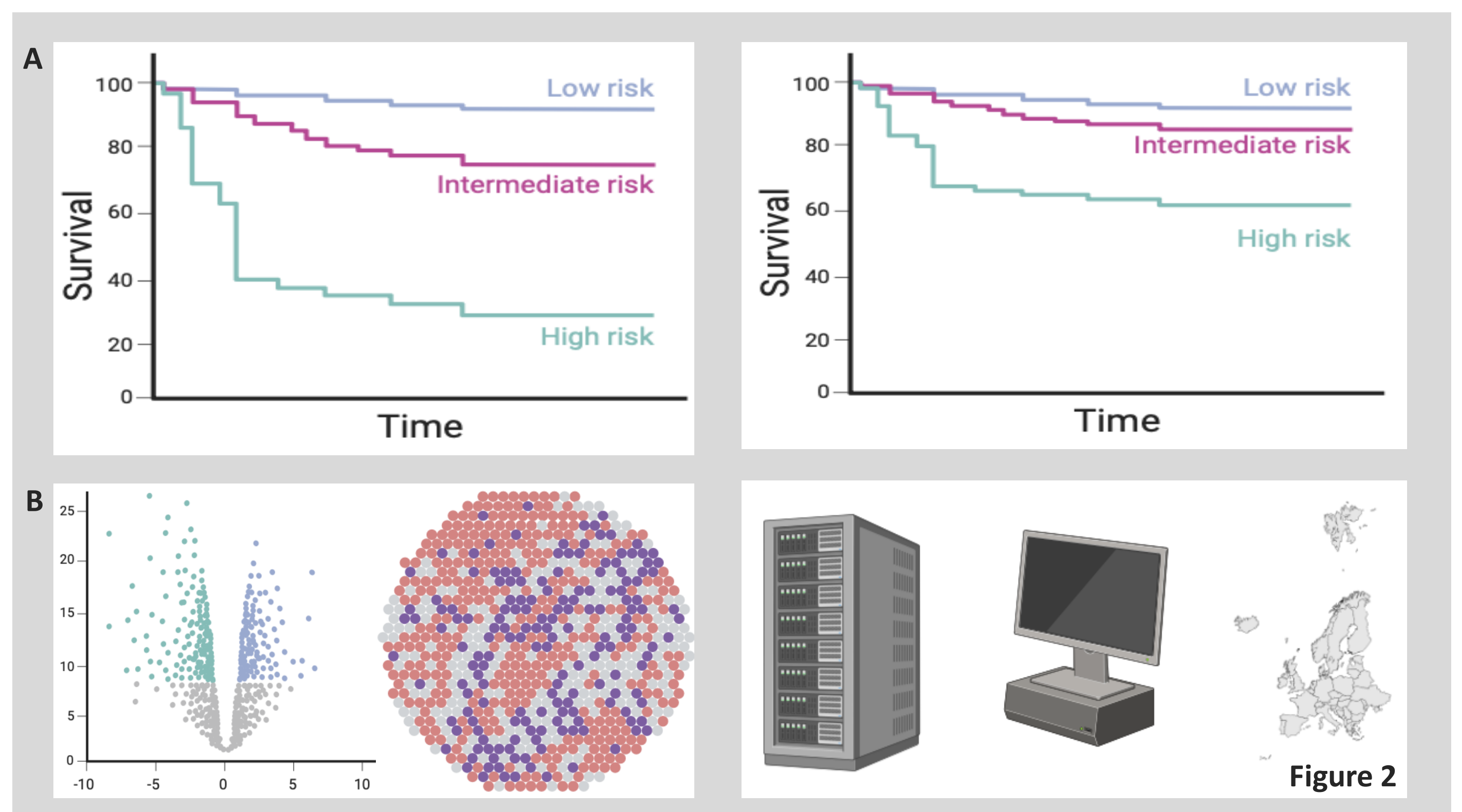
This Pan-European prospective clinical trial is a crucial step toward improving the management and outcomes of pACTs. By fostering collaboration and standardization, the EXPeRT group and ERN groups aim to pave the way for more effective treatments and improved quality of life for children with this rare and challenging disease.

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EXPECTED OUTCOMES

The trial aims to:

- Improve overall survival and disease-free survival rates for children with pACTs. (Fig. 2A)
- Reduce variability in treatment outcomes by standardizing care practices.
- Enhance understanding of the biological basis of pACTs through genetic and molecular analysis. (Fig. 2B)
- Establish a robust European database to inform future research and treatment innovations.



METHODS

The trial will be conducted across multiple pediatric oncology centers in Europe, enrolling patients diagnosed with pACTs. Key components include:

1. Standardization of Diagnostic and Treatment Protocols: Uniform guidelines will be developed and implemented for the diagnosis, staging, and treatment of pACTs, ensuring consistency across participating centers.
2. Stratification and Tailored Therapies: Patients will be stratified based on factors such as tumor stage, genetic background, DNA methylation group, circulating tumor DNA, and clinical presentation. Treatment regimens will be tailored accordingly, with an emphasis on precision medicine.
3. Data Collection and Analysis: Systematic collection and analysis of data on patient demographics, treatment modalities, responses, and outcomes will be conducted. This will include molecular and genetic profiling to identify potential biomarkers for prognosis and therapeutic targets.
4. Interdisciplinary Collaboration: The trial will foster collaboration among oncologists, endocrinologists, surgeons, radiologists, pathologists, and geneticists to ensure a comprehensive approach to patient care and research.
5. Patient and Family Support: Psychosocial support for patients and their families will be prioritized, recognizing the profound impact of pACTs on quality of life.