* PharmaScienceHub Talks

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Lecture Hall 001 @Center for Bioinformatics, Campus E2.1, 66123 Saarbrücken



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Turning big data into patient benefit in pediatric oncology

Transforming molecular data into clinical benefit remains a central challenge in pediatric oncology, mainly based on the rarity of diseases. At KiTZ/DKFZ Heidelberg, interdisciplinary teams integrate high-resolution genomics and epigenomics—especially methylation-based tumor classification and next-generation sequencing—into diagnostic and therapeutic decision-making. These classifiers, widely adopted in WHO tumor taxonomy and accessible via www.molecularneuropathology.org and www.mnp-outreach.com, are now being further developed into certified medical software products by Heidelberg Epignostix, a KiTZ/DKFZ spin-off.

Within INFORM, an international precision oncology registry for high-risk pediatric cancers, multi-omic profiling is linked to medium-throughput drug screening in patient-derived models. Functional assays uncover therapeutic options even in tumors without genetic drivers, informing individualized interventions. This strategy is shared with European partners through PedCanPortal.eu, which connects INFORM with other national pediatric precision medicine initiatives.

The ITCC-P4 consortium extends this concept to preclinical drug development, enabling industry-ready pharmacological profiling in disease-relevant models. Further, the new Pediatric Cancer Drug Discovery Unit at DKFZ/KiTZ and the PROTECT Cancer Grand Challenge advance novel therapeutic strategies by integrating biology, modeling, and compound development.

Together, these initiatives exemplify how translational infrastructure, datadriven diagnostics, and open platforms can convert big data into actionable insights for children with cancer.



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