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abstract

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abstract

Targeted therapy in pediatric patients with extracranial solid tumors

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Introduction: Introduction: Targeted therapy (TT) in pediatric oncology is rarely used. Next-generation sequencing (NGS) is instrumental for clinical decisions on TT.

The aim of the study was to the identification of predictive makers during NGS in pediatric tumors and evaluation of the possibility of using TT in pediatric practice

Methodology: The investigation enrolled 334 pediatric patients diagnosed with extracranial solid tumors including a majority with neuroblastoma (168/334, 50.3%), soft tissue sarcoma (84/334, 25%) and other rare tumors (Wilms tomor, malignant rhabdoid tumor etc.) treated at the Dmitry Rogachev National Medical Research Center in 2018-2023. Tumor DNA was sequenced using a customized QiaSeq panel (Qiagen, Germany). The identified somatic variants were interpreted in accordance with the AMP/ASCO/CAP Guideline recommendations.

Results: TT sensitivity predictors were identified in 120/334 cases (35.9%): Tier II in 83 pts, Tier IB in 32 pts (almost always ALK in neuroblastoma) and Tier IA in 5 pts: tumor-agnostic BRAF p.V600E in 3 pts (melanoma, melanocytic nevus, low-grade spindle-cell sarcoma) and tumor-specific NF1 aberrations in 2 pts with plexiform neurofibroma. TT was commenced in 21/334 cases (6.3%), often first-line or as a first relapse therapy (14/21 cases), combined with chemotherapy (TT-CT, 13/21 cases). The regimens continued 10.9 (0.8–43.5) months for single-mode and 12.3 (0.3–61.5) months for TT-CT.

Objective tumor response and stabilization of the disease for over 6 months was achieved in 14/21 pts (66.7%). In 9/21 pts (42.8%) the response persists at the time of analysis. The time to best response and disease stabilization on TT was 6 (0.8–12.3) months. The tolerance was generally good: the therapy was discontinued for toxicity in 1 case only.

Conclusion: Predictive markers of different levels during NGS of DNA in pediatric tumors are detected in 36% of cases. Targeted therapy in mono-regimen or in combination with chemotherapy agents is well tolerated and beneficial in some cases.