TYROSINE-KINASE INHIBITORS (TKIS) IN PEDIATRIC FIBROMATOSIS

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Background

Aggressive fibromatosis (desmoid tumors, DTF): locally invasive, non-metastatic fibroblastic tumors.

In children, tumors may arise in extremities, head/neck, chest wall, or abdomen.

Course is unpredictable: many regress spontaneously; others progress and threaten function.

Because of this, the first-line approach is often “active surveillance” unless the tumor is clearly progressive or threatening vital function.

When systemic therapy is required, low-dose chemotherapy (methotrexate/vinblastine) or TKIs are preferred over mutilating surgery or high-dose chemo.

Progressive, symptomatic, or function-/organ-threatening disease after (or instead of) observation and non-cytotoxic options. Pediatric data are limited, but TKIs are increasingly used given adult efficacy and tolerability. EJ CancerPubMed

Sorafenib (VEGFR/RAF inhibitor)

Adult RCT showed major PFS benefit (HR 0.13 vs placebo). Responses often slow but durable; dose reductions common for toxicity. Pediatric use supported by case series and small cohorts. New England Journal of MedicineRutgers Cancer InstituteMedical Journals Sweden

Pediatric reports include combination with celecoxib in refractory cases showing clinical benefit. PMC

Pazopanib (multi-target VEGFR TKI)

Retrospective/phase II data in adolescents & young adults suggest meaningful activity and pain improvement vs low-dose MTX/VBL; growing real-world support (including large 2024 series). Pediatric-only data remain sparse but encouraging. PubMed+1The LancetClinicalTrials.gov

Imatinib (PDGFR/KIT inhibitor)

Phase II studies (mostly adults) show disease stabilization in a majority and modest ORR; has the clearest pediatric dosing guidance among TKIs due to broader pediatric oncology experience. Annals of OncologyScienceDirect

Many patients achieve stable disease or gradual partial responses over months; symptomatic relief (especially pain) is common.

Responses can persist after stopping in some series, but optimal treatment duration—especially for children—remains undefined.

Summary

TKIs (imatinib, sorafenib, pazopanib) are safe and effective options for pediatric aggressive fibromatosis, mostly producing disease stabilization and symptom relief. They are considered in progressive, symptomatic, or high-risk tumors when observation or chemo isn't suitable. Pediatric dosing is adapted from adult experience, requiring careful monitoring of toxicity, growth, and blood pressure.