Review Childs Nerv Syst. 2026 Jun 27;42(1):271. doi: 10.1007/s00381-026-07376-9.

Pediatric medulloblastoma

Kazuhiro Sabet [1], Hope Lovell [2], Adriana Fonseca [2]

PMID: 42365163 DOI: 10.1007/s00381-026-07376-9

Abstract

Medulloblastoma is the most common malignant brain tumor of childhood and remains a leading cause of pediatric cancer-related mortality. Once treated as a single clinicopathologic entity, medulloblastoma is now recognized as a biologically heterogeneous family of diseases defined by four principal molecular subgroups (WNT, SHH, Group 3, and Group 4) and further refined by methylation-defined subtypes. These biologic categories differ in genetics, developmental origin, age distribution, dissemination patterns, and prognosis, and they increasingly guide risk stratification and treatment selection. Over recent decades, advances in neurosurgery, radiotherapy, and multiagent chemotherapy have improved survival, but at the cost of substantial neurocognitive, endocrine, and psychosocial late effects. Contemporary trials are aimed at reducing therapy intensity for biologically favorable disease while intensifying or innovating approaches for very high-risk subgroups. In parallel, emerging biomarkers such as cerebrospinal fluid (CSF) cell-free DNA (cfDNA) assays are under evaluation for measurable residual disease monitoring and response-adapted therapy. This review summarizes key concepts in medulloblastoma biology, outlines the evolution of pediatric therapy and trials, describes modern treatment strategies across age and risk groups, and highlights future directions in molecular risk stratification and biomarker-driven clinical trials.

Keywords: Medulloblastoma; Molecular subgroups; Pediatric brain tumors; Risk-adapted therapy.