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Rare gliomas: standard treatment approaches and new target therapies

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Abstract

Purpose of review: Rare gliomas, including circumscribed astrocytic, glioneuronal, and neuronal central nervous system (CNS) tumours, though collectively uncommon, present significant clinical challenges due to their heterogeneity and limited therapeutic evidence. Conventional management has relied predominantly on surgery and radiotherapy. Advances in molecular profiling have revealed actionable targets, prompting a timely reassessment of treatment paradigms. This review aims to describe current standard treatments and recent advances in molecularly targeted approaches for rare gliomas.

Recent findings: Gross total surgical resection remains the primary therapeutic modality for rare gliomas, providing optimal tumour control and symptom relief. Radiotherapy offers additional benefit in case of subtotal resection or recurrent disease, particularly in WHO grade 3 tumours. In contrast, conventional chemotherapy has shown limited efficacy and is typically reserved for refractory or progressive cases.The discovery of actionable molecular alterations in a substantial subset of rare gliomas has led to increasing integration of targeted therapies into clinical management. Notable recent advances include the use of BRAF/MAPK pathway inhibitors (e.g., dabrafenib/trametinib, tovorafenib), NTRK inhibitors (e.g., larotrectinib, entrectinib), FGFR inhibitors (e.g., erdafitinib, pemigatinib), and mTOR inhibitors (e.g., everolimus), which have demonstrated meaningful clinical activity in select patient populations.

Summary: Precision oncology is rapidly transforming the treatment landscape for rare CNS tumours. Integration of targeted therapies into clinical protocols - ideally guided by multidisciplinary molecular tumour boards - is increasingly warranted. Future research must optimise timing, combination strategies, and overcome resistance, while new biomarkers and liquid biopsy tools are needed to guide the choice of therapy and monitor response in this underserved population.

Keywords: precision oncology; rare gliomas; target therapy.