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Rznomics Reports Interim Data for RNA Gene Therapy in Recurrent Glioblastoma

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The Phase 1/2a data support continued development of RZ-001, an RNA editing-based anticancer gene therapy for recurrent glioblastoma.



Rznomics has presented interim Phase 1/2a clinical data for RZ-001, its RNA editing-based anticancer gene therapy for recurrent glioblastoma, at the Asian Society for Neuro-Oncology Annual Meeting 2026 in Kanazawa, Japan.

The presentation was delivered by Dr Chae-Yong Kim from Seoul National University Bundang Hospital and covered safety and preliminary efficacy findings from the ongoing clinical trial.

This is relevant because recurrent glioblastoma remains one of the most difficult cancers to treat. The disease has a high recurrence rate and poor prognosis even after standard treatment, while many conventional immuno-oncology approaches have struggled to show meaningful clinical benefit.

RZ-001 uses Rznomics' proprietary RNA trans-splicing ribozyme platform. The therapy is designed to express a therapeutic gene specifically inside tumour cells, aiming to induce cancer cell death through a targeted RNA-based mechanism.

According to the interim analysis, 20 patients have completed screening, with 10 patients enrolled and treated. No new or unexpected treatment-related safety concerns were identified. No dose-limiting toxicity was reported, and no treatment-related Grade 4 or higher adverse events were observed.

Several patients also showed prolonged tumour recurrence inhibition and disease control exceeding six months. Dr Kim noted that this is encouraging in a disease setting where recurrence typically occurs within two to four months, particularly where some patients extended past nine months.

The therapy is still in early clinical development, and further patient enrolment and long-term follow-up will be needed to clarify durability, survival impact and risk-benefit profile. As with many gene therapy approaches, scalability will also depend on manufacturing, delivery, regulatory strategy and treatment-centre readiness.