Pancancer Paradigm

FDA approves Rozlytrek for cancers with certain gene mutations

September 16, 2019 By Liz Highleyman

In August, the Food and Drug Administration (FDA) approved Rozlytrek (entrectinib), a new site-agnostic, or "pancancer," therapy designed to treat cancer anywhere in the body.

Rozlytrek represents a new approach to fighting cancer. It was developed to attack tumors that share specific genetic mutations regardless of where they occur in the body. It is the second medication designed to fight cancer anywhere, after Vitrakvi (larotrectinib), which was approved in November 2018.

"The whole idea is that tumor biology is far more important than where the cancer arises," says Rozlytrek clinical trial investigator Robert Doebele, MD, PhD, of the University of Colorado.

Like Vitrakvi, Rozlytrek is a tropomyosin receptor kinase (TRK) inhibitor. TRK proteins are encoded by three neurotrophic receptor tyrosine kinase, or NTRK, genes. When one of these genes fuses with another gene, it acts as an ignition switch to spur tumor growth. In addition, Rozlytrek also targets cancers with ROS1 and ALK gene alterations, found in some non-small-cell lung cancers.

In a combined analysis of three trials, which together included 54 adults with 10 different types of advanced cancer, Rozlytrek shrank tumors in 57% of participants, including four with complete remission. The response rate rose to 78% for those with ROS1-positive lung cancer. What's more, over half of patients whose cancer had spread to the brain experienced tumor shrinkage.

Another study, presented at the American Society of Clinical Oncology annual meeting, showed that Rozlytrek led to "striking, rapid and durable" responses in all 12 children and adolescents who had tumors—mostly brain cancers and sarcomas—with the targeted gene alterations.

The advent of site-agnostic therapies raises the question of whether everyone with advanced cancer should receive tumor genomic testing to see whether they could benefit from new medications. TRK fusions are rare, found in only around 1% of all malignancies, but they occur more often in some uncommon types of cancer.

"Even if it's just a 1 in 100 chance, it's so meaningful for that patient in terms of effectiveness, tolerability and survival that I think it's worth it," Doebele says.

Click here to learn more about site-agnostic cancer treatment.

© 2026 Smart + Strong All Rights Reserved.