

Regulatory Designations in Oncology: Signal or Substance?

Regulatory designations—**Priority Review**, **Breakthrough Therapy**, **Fast-Track**, and **Orphan Drug**—are designed to accelerate development timelines and regulatory review for therapies addressing serious or life-threatening conditions. These designations can shape internal strategy, drive investor interest, and influence resource allocation—but they are not all equally predictive of clinical success.

While designations consistently improve the likelihood of approval, our data shows wide variation in how much they help—and when they are most effective.

Data Insights: How Designations Perform in Practice

Category	Phase 1 Historical Approval Rate	Phase 2 Historical Approval Rate	Phase 3 Historical Approval Rate	Overall Historical Approval Rate	Phase 1 Transition Rate	Phase 2 Transition Rate	Number of programs
Priority review	88%	89%	91%	91%	100%	100%	431
Breakthrough	71%	74%	84%	72%	91%	87%	255
Fast-track	26%	28%	40%	24%	67%	55%	497
Orphan drug	29%	35%	55%	30%	63%	50%	1259
Overall Oncology	5%	9%	35%	6%	31%	17%	11676

Based on analysis of over **11,000 historical oncology clinical programs**, the performance differences across designations are substantial:

- **Priority Review** is associated with a **91 percent approval rate at Phase 3 and overall**. This designation, however, is usually granted once data is already mature and positive.
- **Breakthrough Therapy** shows a **72 percent overall approval rate and 87 percent Phase 2 transition success**, making it the most impactful designation for early- and mid-stage development.
- **Orphan Drug** and **Fast-Track** designations yield more modest results, with overall approval rates of **30 percent** and **24 percent**, respectively—well above the **6 percent** baseline for programs without any designation, but far below the top-performing categories.

Why Do These Differences Exist?

There is no single reason behind the performance gaps—but several contributing factors are clear:

- **Breakthrough Therapy** requires preliminary clinical evidence of substantial improvement over existing therapies. As a result, it tends to be awarded to programs with meaningful efficacy signals early in development. In practice, this may **self-select for stronger candidates**, and gives sponsors early access to FDA guidance that can de-risk subsequent trial phases.
- **Priority Review**, by contrast, is typically granted once a program has already demonstrated compelling safety and efficacy in pivotal trials. Its high success rate reflects **maturity of data**, not necessarily the designation's predictive value for earlier stages.
- **Fast-Track** and **Orphan Drug** designations can be granted with less stringent data requirements—based more on **mechanistic rationale or rarity of the condition** than on clear early clinical benefit. While these designations offer important regulatory and financial advantages, they may be applied to **riskier or less proven programs**, which helps explain their lower average success rates.
- In particular, **Orphan Drug status**, while often essential in rare cancers, is granted so broadly that it may **dilute its signal strength** when used as a proxy for program quality.

Key Takeaways for Decision-Makers

- **Not all designations signal the same level of clinical or regulatory readiness.** Breakthrough and Priority Review are more predictive of success than Fast-Track or Orphan Drug.
- **Breakthrough Therapy is especially valuable early**, where it combines meaningful FDA engagement with strong early efficacy filtering.
- **Fast-Track and Orphan Drug are easier to obtain**, but less clearly correlated with strong outcomes.
- **Designation status should be treated as a contextual indicator**, not a guarantee of future success.

How We Support Oncology Portfolio Strategy and Clinical Development

- **Targeted designation benchmarking**
We break down success rates by designation, tumor type, and phase of development—helping teams identify which designations are most likely to materially improve program outcomes in their specific context.
- **Scenario modeling for regulatory strategy**
Our platform enables users to simulate how the presence or absence of a designation (e.g., Breakthrough vs. Fast-Track) would affect the program's probability of success, based on historical benchmarks. This supports better prioritization when considering whether to pursue, delay, or deprioritize a designation strategy.
- **Early-stage decision support for high-value filings**
We help clinical and regulatory teams assess when a program has reached the maturity and data strength to justify pursuing a designation like Breakthrough—avoiding premature filings while capitalizing on opportunities that meaningfully shift the risk-reward profile.

Regulatory designations influence perception—but only data clarifies reality. We bring that clarity to every strategic decision.