Break On Through to the Other Side: How FDA Can Rescind Breakthrough Designation

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Surely Jim Morrison of The Doors wasn’t thinking about the Food and Drug Administration’s (FDA) Breakthrough Therapy program when he famously sang, “Break On Through to the Other Side,” although drugs might have been on (or affecting) his mind. However, the song’s lyric, “Dug our treasures there, but can you still recall the time we cried,” seems prescient in light of the FDA’s recently-announced policy concerning rescission of Breakthrough Therapy (BT) designation. While there is the treasure of such designation, an FDA rescission decision surely will leave a person crying.

In a previous Bulletin, we discussed the benefits of BT designation, but we also cautioned that one should not necessarily consider it a magic lottery ticket and that FDA would step carefully. As a brief overview, the Food and Drug Administration Safety and Innovation Act, enacted in 2012, allows sponsors to request that their drug be designated as a “Breakthrough Therapy” if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. If a drug is so designated, FDA will expedite the development and review of the application for approval of the drug. As of August 22, 2014, FDA’s Center for Drug Evaluation and Research (CDER) had granted breakthrough status to 55 products, and none have been rescinded. The recent issuance of Manual of Policies and Procedures (MAPP) 6025.6, “Good Review Practice: Management of Breakthrough Therapy-Designated Drugs and Biologics,” demonstrates FDA’s willingness to rescind the BT designation in specific cases.

### Highlights of Rescission MAPP

- The MAPP describes the procedures the CDER Review Staff will follow when evaluating rescission decisions.
- CDER’s Medical Policy Council, comprised of senior CDER staff, will ensure there is consistent policy implementation across review divisions.
- The release of the MAPP follows a brief discussion about rescission in FDA’s May 2014 final “Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics.” In the guidance, the agency said that it may notify the drug sponsor of its intent to rescind a designation if emerging data no longer support the designation or the drug development program is no longer being pursued.

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Legal Insight

- The MAPP outlines reasons for potential rescission:
  1. FDA believes emerging data no longer demonstrate substantial improvement over available therapy;
  2. the designated drug development program is no longer being pursued; or
  3. the designated drug no longer demonstrates substantial improvement over a new available therapy, e.g., where another drug obtains traditional approval or the clinical benefit is confirmed for a drug granted accelerated approval for the same indication as the designated drug.

- The written notice of a possible rescission should include:
  A description of the reasons for making such a determination and provide the sponsor with an opportunity to submit additional data and justification to support the continuing breakthrough therapy designation and/or to request a meeting with the division to discuss the breakthrough therapy designation for the drug.

- The sponsor will have an opportunity to submit a response and additional information. The aforementioned Medical Policy Council will meet with the review division. If FDA continues to believe rescission is appropriate, it will send a formal, official letter to the drug sponsor and describe its rationale.

- If, on the other hand, CDER concludes that the product maintains criteria, it will work with the drug sponsor and, if additional information leads the review division to conclude that the breakthrough criteria continue to be met, “plans for a path forward for the development of the drug should be discussed, documented, and communicated to the sponsor via a letter or teleconference.”

AGG Observations

- While breakthrough designation is certainly a benefit for a drug company for a number of reasons, FDA makes clear it is not sacrosanct. If certain conditions occur, the agency can rescind the benefit.

- We would not expect FDA immediately to start rescinding designations left and right, as BT designation is an incentive the agency wants to continue to promote. However, it would not surprise us if we see FDA rescind one or two in the near future to show it means business.

- While one might expect a sponsor that loses designation to consider suing FDA, we do not know how successful, or even prudent, such a lawsuit would be. First, a rescission does not mean a refusal to file or the rejection of an application; it merely means one loses a benefit afforded to a small handful. In essence, the company takes its place with everyone else. So, a sponsor might not want to jeopardize its relationship with FDA in this case. Second, even if a lawsuit is brought, based on past experience, we believe a court will defer to the agency’s discretion unless the agency is found to have acted arbitrarily and capriciously.

- An initial decision by FDA to rescind is not a final decision. A sponsor will have an opportunity to respond to the agency and attempt to demonstrate designation remains appropriate. In addition, the sponsor might obtain useful information from FDA concerning its drug development program.

- We end as we started. Designation is a treasure. A sponsor must be cognizant of FDA’s authority and rationale for rescission and remain vigilant to maintain its designation. The sponsor doesn’t want to recall the time it cried and wants to continue to break on through to the other side. RIP Jim.
not if, but how.

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