New Breakthrough Therapy Designation program aims to cut clinical trial time

Only 30% of the first 113 BTD requests were approved

- The BTD was designed to supplement and complement the FDA’s other expedited development and review programs – priority review, fast track, and accelerated approval.
- BTD drugs can utilize biomarkers to measure impact on irreversible morbidity and mortality (IMM).
- Central nervous system drugs and diagnostics are likely to be significant beneficiaries of the BTD program.
- Orphan and oncology drugs dominated the first class of BTDs awarded by the FDA.

As part of its reauthorization of the Prescription Drug User Fee Act (PDUFA) in 2012, Congress created the Breakthrough Therapy Designation (BTD), a process that allows the U.S. Food and Drug Administration (FDA) to expedite the development and review of drugs intended to treat a serious condition where preliminary clinical evidence suggests substantial improvement over available therapy on a clinically significant endpoint or on symptoms that represent serious consequences of the disease. Although the FDA already had tools to speed drug development—priority review, fast track, and accelerated approval—those programs are limited in their ability to address scientific, regulatory, and economic factors that are dramatically shifting the R&D landscape. Whereas the BTD program generally has a higher evidentiary threshold for approval [i.e., substantial improvement on clinically significant endpoints], it provides a greater degree of facilitation by FDA [i.e., early intensive guidance and organizational commitment involving senior managers].

Launched in mid-2012, the BTD is still in its infancy. A key success factor for the program will be whether it serves the goal of helping drug sponsors and the FDA work together to cut development time, while encouraging the utilization of new development tools and methodologies, such as targeted diagnostics and adaptive clinical trial designs. This report provides an early look at how the BTD program differs from FDA’s other expedited development and review programs, and what the first graduating class of BTDs may contribute to addressing unmet medical needs in orphan diseases, oncology, and other critical therapeutic areas.