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**New Breakthrough Therapy Designation Has Potential to Shorten Development Time Considerably**

BOSTON – Jan. 23, 2014 – The recently launched Breakthrough Therapy Designation (BTD) program in the United States, aimed at expediting development and review of drugs intended to treat a serious condition, has the potential to shorten development time considerably, according to the Tufts Center for the Study of Drug Development, which recently completed an assessment of the BTD program.

While the U.S. Food and Drug Administration (FDA) approved only 30% of the first 113 BTD requests (with 60% denied/withdrawn and 10% pending) in the year and a half following the program’s 2012 launch, Christopher-Paul Milne, director of research at Tufts CSDD, said early intensive guidance from senior FDA managers, a key element of the program, suggests that BTD-designated development programs will likely learn, sooner rather than later, whether BTD products will be successful.

“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,” Milne said.

The FDA already has tools to speed drug development—priority review, fast track, and accelerated approval—but these programs are limited in their ability to address scientific, regulatory, and economic factors that are dramatically shifting the R&D landscape, he said.

The assessment, reported in the January/February *Tufts CSDD Impact Report*, released today, found that:

- Central nervous systems drugs (those for neurodegenerative and psychiatric disorders) and diagnostics currently comprise a small share of FDA approvals with special program designation (3% and 5%, respectively), but are likely to benefit from receiving the breakthrough designation.
- Although orphan designation technically is not an FDA expedited program for serious conditions, it often applies to the same investigational compounds as BTD.

The study, based on Tufts CSDD proprietary databases, FDA publications, Thompson Reuters Cortellis and the professional literature, examined BTD determinations between July 9, 2012 and Dec. 17, 2013.

ABOUT THE TUFTS CENTER FOR THE STUDY OF DRUG DEVELOPMENT
The Tufts Center for the Study of Drug Development at Tufts University provides strategic information to help drug developers, regulators, and policy makers improve the quality and efficiency of pharmaceutical development, review, and utilization. Tufts CSDD, based in Boston, conducts a wide range of in-depth analyses on pharmaceutical issues and hosts symposia, workshops, and public forums, and publishes Tufts CSDD Impact Reports, a bi-monthly newsletter providing analysis and insight into critical drug development issues.

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