User fee era in U.S. currently poses mixed regulatory burden for sponsors

Tufts CSDD analysis completed in advance of PDUFA reauthorization this fall

- Regulatory burden from the pre- to post-FDAAA period decreased for NME/newBLA approvals, but increased for sNDA/sBLA approvals.
- During the same period, spending on outsourced regulatory services rose dramatically.
- The difference between optimal and sub-optimal sponsor experiences with the FDA review process was less for NME/newBLAs than for sNDA/sBLAs.
- Ten of 21 regulatory activity variables were considered significant by sponsors, when optimal and sub-optimal approvals were compared on a disparity index.
- Overall, two variables were most consistently significant when both disparity index and statistical significance testing were considered for all cohorts.

As the fifth round of the Prescription Drug User Fee Act (PDUFA V) approaches in October 2012, questions persist about how to achieve the proper balance between review performance by the U.S. Food and Drug Administration (FDA) and the regulatory burden on product sponsors. Under PDUFA IV, also called the FDA Amendments Act (FDAAA), enacted in 2007, a number of regulatory enhancements were introduced, such as Risk Evaluation & Mitigation Strategies (REMS) and post-marketing requirements, to correct perceived deficiencies in the FDA's authority. Both the FDA and drug sponsors share a goal of streamlining the regulatory review process without sacrificing quality.

To explore this issue further, Tufts CSDD convened an industry working group comprised of eight top companies to consider two questions: 1) Has there been a detectable increase in the regulatory burden pre-FDAAA vs. post-FDAAA? and 2) What specific requirements/actions in the approval process are particularly problematic? This Tufts CSDD Impact Report highlights findings from pilot studies developed and conducted following discussions with the working group, which also provided data for the analyses.