Patent-to-launch time for orphan drugs is 2.3 years longer vs. other drugs

Patient enrollment poses unique challenges for orphan drug sponsors

- Average development time (first patent filing to launch) for first-in-class orphan NMEs that won FDA approval during 1999-12 was 15.1 years, which was 18% longer than the 12.8 years for all new drugs.

- Designations and approvals for orphan oncology drugs outpaced orphan drug development in all other therapeutic areas.

- Orphan drugs focused on central nervous system or cardiovascular indications experienced the greatest number of development challenges.

- Orphan drug developers encountered an average of 4.2 special challenges in the course of research and development (R&D) conducted during 1999-12.

- Ethnic, geographic, and gender differences make orphan conditions, on occasion, hard to diagnose, difficult to study and quantify, and complicated to follow up.

Orphan diseases encompass more than 7,000 diseases and conditions, affecting up to 30 million people in the U.S., 50% of whom are children. More than 85% of the cases are serious or life-threatening, thereby constituting a serious unmet medical need – and important opportunities for drug developers. Their efforts got a boost with passage in 1983 of the Orphan Drug Act (ODA). However, creating new medicines to treat orphan diseases continues to pose unique challenges, not the least of which is the logistical difficulty of working with small patient populations that are, in the vast majority of cases, widely geographically dispersed. While new approaches to study design, including use of patient advocacy groups and adaptive clinical trials, are helping to mitigate development problems, progress remains slow; orphan drug development time, on average, lasts nearly two-and-half years longer than for other drugs. The difficulties only increase for so-called ultra-orphan drugs.

To better understand the special challenges sponsors face in developing orphan drugs, Tufts CSDD studied all 46 first-in-class, orphan-designated, new molecular entities (NMEs) approved by the FDA from 1999 through 2012. Key findings are summarized in this report.