Growth in rare disease R&D is challenging development strategy and execution

Rare diseases now account for 31% of the R&D pipeline, up from 18% in 2010

- More than half of all drug and biologic approvals worldwide in 2018 were for rare diseases.
- High rare disease drug approval rates in the U.S. are now approaching traditional drug approval rates.
- Sustained R&D investment in rare disease drug development is now driving rapid growth in later stage clinical trial activity.
- Clinical through approval phase durations for rare disease drug development take on average four years longer than those for non-rare diseases.
- Clinical trials for rare disease drugs overall engage more investigative sites to recruit fewer patients, reflecting the difficulties of patient identification and enrollment.
- Once recruited and enrolled in trials for rare disease drug trials, patients have lower drop-out rates, compared to volunteers for trials for non-rare diseases.

Rare disease drug development is now one of the most active and fastest growing areas in drug R&D, with nearly one-third of all products in the global R&D pipeline targeting treatments for rare disease indications. Biopharmaceutical companies in recent years have increased R&D investment in rare diseases not only to meet unmet medical needs, but also to support development programs at lower relative cost, with fewer competitors, strong demand from patient advocates, and greater opportunities for favorable pricing.

Recent Tufts CSDD research suggests that rare disease drug development presents scientific and operational challenges that will necessitate novel clinical development strategies, operating practices, and solutions. This report summarizes top research findings and provides salient benchmarks.