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Growth in Rare Disease R&D Is Challenging Development Strategy and Execution, According to Tufts Center for the Study of Drug Development

BOSTON – July 9, 2019 – Rare disease drug development, which now accounts for nearly one-third of all drugs in active R&D worldwide, presents scientific and operational challenges that will accelerate the adoption of new development strategies and operating models, according to a recently completed analysis from the Tufts Center for the Study of Drug Development.

"Our research on rare disease development programs suggests that sponsor companies are encountering unprecedented operating challenges in this area," said Ken Getz, associate professor and director of sponsored research at Tufts CSDD, who led the analysis. "Smaller market opportunities and longer development cycle times—driven in large part by difficulties identifying investigators and recruiting rare disease patients—will necessitate increased use of data and analytics and more flexible and mobile clinical trial models.

A rare disease that qualifies as an orphan disease is defined as a medical condition that affects 200,000 or fewer people in the United States, or fewer than five people per 10,000 population in the European Union.

The share of new drug approvals worldwide for rare diseases doubled from 29% of all approvals in 2010 to 58% in 2018, according to Tufts CSDD.

The analysis, summarized in the July/August *Tufts CSDD Impact Report*, released today, found that:

- Rare disease drug approval rates in the U.S. are now approaching non-rare drug approval rates.
- Clinical through approval phase durations for rare disease drug development on average take four years longer than those for non-rare diseases.
- Phase I clinical trials for rare diseases, on average, engaged six times the number of investigative sites to recruit a quarter of the number of patients, compared with those for non-rare diseases.

"Clinical trials for rare diseases have lower drop-out rates, compared to those for non-rare diseases. However, finding and enrolling study volunteers is extremely difficult," Getz said. "Benchmark data from recent clinical trials show that, in addition to long study start-up and enrollment periods, screen and randomization failure rates are much higher in studies among rare disease patients."

ABOUT THE TUFTS CENTER FOR THE STUDY OF DRUG DEVELOPMENT

The Tufts Center for the Study of Drug Development (http://csdd.tufts.edu) 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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