Orphan Drug Approvals Have Increased, But High Costs Pose Challenges for Patients

BOSTON – July 10, 2014 – Although the pace of approvals for new orphan drugs—medicines that treat relatively rare conditions—have increased in the United States and Europe in recent years, patients are facing growing challenges accessing those drugs, a newly completed study by the Tufts Center for the Study of Drug Development at Tufts University has concluded.

During the 14-year period 2000–2013, 86 orphan drugs were approved in the U.S., up from 65 during the prior 18-year period 1983–2000, while in Europe 96 orphan drugs were approved in 2000–2013, more than double the 44 approved in the earlier period, according to Tufts CSDD.

Orphan drugs are those developed for rare diseases and conditions that affect fewer than 200,000 people in the U.S., or five per 10,000 or fewer people in the European Union.

Among the challenges that limit patient access to orphan drugs in the U.S., relative to Europe, is higher cost-sharing by patients, which can lead to increased levels of non-compliance, according to Joshua Cohen, Assistant Professor at Tufts CSDD, who conducted the analysis.

“The encouraging news is that more orphan drugs are in development today than ever before, with more getting marketing approval—in 2013 alone, nine orphan drugs were approved in the U.S., the most in a single year. But the high cost of these medicines is leading insurers to reassess their reimbursement policies, which likely will mean more out-of-pocket costs for patients,” Cohen said.

The most expensive orphan drugs can cost more than half a million dollars per year per patient in the U.S.

Key findings from the study, reported in the July/August Tufts CSDD Impact Report, released today, include the following:

- Since 1983, 7% more orphan drugs have been approved in the United States than in Europe, and 17% more were approved first in the U.S. than were approved first in Europe.
- There are fewer denials of orphan drug coverage by U.S. payers than by European payers.
- While U.S. payers often require prior authorization as a condition of reimbursement, European health authorities employ more stringent conditions, such as on-label indication restrictions, step edits, and coverage with evidence development.
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.

--end--

Contacts:

Tufts Center for the Study of Drug Development
Sandra Peters
617-636-2170
CSDDpublications@tufts.edu

Business Communication Strategies
Peter Lowy
617-734-9980
lowy@bus-com.com