



Tufts Center for the Study of Drug Development

TUFTS UNIVERSITY

# Impact REPORT

ANALYSIS AND INSIGHT INTO CRITICAL DRUG DEVELOPMENT ISSUES

## Patients face new challenges accessing a growing number of orphan drugs

### *Orphan drug reimbursement varies widely across markets*

- 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.
- Several European countries formally evaluate up to 75% of new orphan drug approvals. Cost-effectiveness evaluations of orphan drugs are expected to increase in the near future.
- There are fewer denials of orphan drug coverage in the U.S. than in Europe.
- Patient cost-sharing is higher in the U.S. than in Europe.
- 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.