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.

In 1983, the U.S. enacted the Orphan Drug Act to encourage development and commercialization of drugs to treat rare diseases. Seventeen years later an analogous law was passed in Europe. Both pieces of legislation are considered major successes in terms of spurring development and launches of orphan drugs. In the past five years, 39 orphan drugs were launched in the U.S. across numerous therapeutic categories, including multiple myeloma, chronic myelogenous leukemia, metastatic non-small cell lung cancer, hemophilia, tuberculosis, homozygous familial hypercholesterolemia, and cystic fibrosis. In 2013 alone, nine orphan drugs were approved, the most in a single year.

In the U.S., the growing number of commercially available orphan drugs and the high cost of various orphan treatments have led to reconsideration of payer reimbursement policies. Tufts CSDD examined patient access to orphan drugs to identify possible barriers to orphan drug treatments. Differences in U.S. and European orphan drug approvals, as well as payer reimbursement policies for orphan drugs in the U.S., France, Germany, Netherlands, and the United Kingdom, were analyzed, and are summarized in this report.