U.S. orphan product designations more than doubled from 2000-02 to 2006-08

Designations increased from a total of 208 in 2000-02 to 425 in 2006-08

- During the 2000s, orphan products comprised 22% of all new molecular entities (NMEs) and 31% of all significant biologics (SBs) receiving U.S. marketing approval.

- Orphan products receiving priority review status rose from 35% of all orphan NMEs in 2000-02 to 50% in 2006-08; during the same time the share of orphan SBs receiving priority review status rose from 17% to 67%.

- From 2000-02 to 2006-08, average total development time for orphan products dropped by 2.3 months for NMEs and 37.5 months for SBs.

- Big biopharma’s share of orphan product approvals in the U.S. grew from 35% in 2000-02 to 56% in 2006-08.

- While biotech firms during the 2000s have garnered, on average, about one-third of all orphan drug approvals, they received just over 50% of orphan drug designations.

- Sponsors engaged in clinical development funded through orphan grants reported that 22% of their programs led to approvals, which compares with a clinical approval success rate of 16% among mainstream drug developers.

Since the Orphan Drug Act of 1983 was signed into law, it has resulted in more than 2,000 designations and 350 market approvals of drugs and biologicals for rare diseases considered “orphans,” that is, diseases affecting 200,000 or fewer patients in the U.S. Currently, approximately 6,000 orphan diseases affect more than 25 million people in the U.S.

Pharmaceutical and biotech firms have responded to the need to develop new medicinal products to treat orphan diseases. The Food and Drug Administration (FDA), in turn, has more than doubled the number of investigational compounds awarded orphan designation in the last five years alone. Reflecting the urgency in developing new drugs to treat orphan diseases, the FDA has increased the number of products getting priority review status during the past decade. These trends are summarized in this Tufts CSDD Impact Report, updating an earlier review reported in Tufts CSDD Impact Report 2002 May/June;4(3).