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Tufts Center for the Study of Drug Development
TUFTS UNIVERSITY

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ANALYSIS AND INSIGHT INTO CRITICAL DRUG DEVELOPMENT ISSUES

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.