Clinical success rates for new cancer drugs double while more enter testing

Success rates rose from 9.9% in the mid-1990s to 19.8% in the early-2000s

- The number of new cancer drugs entering clinical testing increased 50% between 1993-98 and 1999-04.
- The clinical success rate for new cancer drugs entering clinical testing across the 12-year period of 1993-04 was 13%.
- 72% of new, investigational cancer compounds focused solely on solid tumor indications, whereas 20% were studied in both solid tumor and hematological indications.
- Small molecule drugs had higher success rates than biologics in transitioning from Phase I to Phase II and from Phase II to Phase III.
- Drugs focused solely on hematologic tumors had a 36% success rate over the 1993-04 period, compared to 10% for drugs focused only on solid tumors.

To meet the growing demand for new cancer therapeutics, drug companies are employing a number of innovative approaches to cancer drug development, which include focusing on new targets within validated and new pathways, designing novel drug formats, and improving clinical study design. To provide an understanding of how new cancer compounds have progressed through the clinical study process, Tufts CSDD analyzed success rates for compounds transitioning from one phase of clinical study to the next, key findings of which are summarized in this report.

While the clinical success rate for new cancer drugs entering clinical testing during the 12-year period studied was 13%, that rate doubled over the study period—from 9.9% in 1993-98 to 19.8% in 1999-04—which suggests that drug companies are making headway in improving the development process. Drug development overall remains a highly complex undertaking. Another Tufts CSDD study found that, for the top 50 firms, clinical approval success rates for self-originated compounds across all therapeutic areas that entered clinical study during the same period ranged from 14% to 18%. For more, see Tufts CSDD Impact Report 2010 September/October: 12 [5].