Financial modeling from Tufts Center for the Study of Drug Development demonstrates substantial net benefits to sponsors who use decentralized clinical trials (DCTs) technology

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The drug development process is fraught with risk, high failure rates, and high costs, and if we are to meet growing unmet patient need, "the status quo is no longer a viable option."[1] The Tufts Center for the Study of Drug Development (CSDD) estimates the total capitalized cost for an approved new compound at $2.6 billion dollars.[3] Driving costs down is imperative so that limited available funds can be used most efficiently to get drugs to market and address the needs of patients.[2]

Although many have discussed the potential for decentralized clinical trials (DCTs) to reduce the cost of drug development,[4] until now, a demonstration of the actual benefit with a quantifiable dollar amount has been elusive. New research from Tufts CSDD, supported by Medable, Inc., **a technology provider of DCT software**, found substantial net benefits for the use of DCTs in drug development. An evaluation of prior industry data combined with selected data from the portfolio of DCT studies on the Medable Platform demonstrated that:

In phase II studies, the typical DCT deployment for a clinical trial resulting in a 1 - 3 month time savings yields a net benefit that is up to five times greater than the upfront investment required.

In phase III studies, a similar time savings yields a net benefit that is up to 14 times greater than the upfront investment required.

The findings illustrate that DCTs are associated with reduced clinical trial cycle times (described in more detail below), and that this reduction has significant financial impacts. DCTs use technology to reduce visits to a central research site, thereby removing geographic barriers for potential participants.[1] These technologies can include econsent, telemedicine and mobile or local healthcare providers and may use one or more procedures that vary from traditional clinical trial models, such as electronic clinical outcome assessments (eCOAs), connected sensors, home health nursing and local labs or shipping of investigational medical products directly to the trial participant.

Despite the advantages these technologies confer, factors including higher initial expenses, concerns about aggregating data and remote communication, and patient perception[5] have made it challenging to drive change towards DCTs.

This Tufts CSDD and Medable, Inc. analysis, the first to demonstrate net benefits for DCT deployment, could drive a paradigm shift. In a forthcoming paper, researchers from Tufts CSDD and Medable Inc., will describe how they calculated net financial benefits to drug sponsors by determining expected net present values (eNPVs), and will publish details on the assumptions and calculations used in the analysis. Here, we provide a broad view.

Background

To develop these insights into the business case for DCTs, researchers conducted a data-driven analysis of the value proposition and return on investment for DCTs using an expected net present value (eNPV) model. **The benefits from DCT deployment that were measured and applied to the financial modeling were derived from published benchmarks on clinical trial cycle time, cost, and performance in the literature as well as conservative assumptions about the impact of, and the investment required to deploy a DCT.** Benefits used in the model include **shorter development cycle times, lower clinical trial screen failure rates, and fewer protocol amendments.**

To calculate eNPV, researchers at Tufts — in partnership with Medable — used data collected from previous trials to assume a dollar amount associated with each of the three factors described above to determine the net benefit of a DCT platform compared to a traditional trial framework. Because many Phase II and III clinical trials do not result in regulatory approval of an investigational medical product or drug, calculating the eNPV is a dependable way to estimate potential return on investment, as the eNPV analysis combines these risks of failure with actual costs and other drivers of value.

Key Findings

1. Shorter development cycle times
In this analysis, cycle time reductions associated with DCT deployments had a substantially greater impact on net financial benefits than any other factor.

The need for improving cycle time is great, and 85% of all clinical trials will experience some sort of delay, with the financial impact of delays costing between $600,000 - $8 million a day.[6] A benefit of DCTs may include more rapid trial completion.[7]

A separate Tufts CSDD study revealed that although 80% of respondents who have invested in technology report time savings for site initiation through activation, most indicate that their tools could be improved.[8]

2. Lower clinical trial screen failure rates
Screen failures are cost drivers in clinical trials,[8] as they consume team effort, time and resources.[9] Additionally, although 85% of people state they wish to participate in research, more than 70% live more than 2 hours away from their study site.[10] Other figures are even more sobering: less than 5% of the US population participates in clinical research,[11] up to 50% of trials are not completed because of insufficient enrollment,[12] and 30% of participants drop out of studies.[13] Perceived participant burden is associated with screen failures and retention rates, and it increases clinical trial timelines.[14] DCTs have the potential to reduce this burden, and thereby reduce screen failure incidence and increase retention rates.

DCTs shift the paradigm to allow people to participate in clinical trials outside of the typical clinical site, enabling faster screening, more convenient consent and enrollment, and in

some cases, the remote delivery of an intervention and the measurement of outcomes, all of which contribute to a more geographically diverse population and one that may better represent the diversity of patients with the disease under study.[1] DCTs also lower burdens on participants by reducing time and travel costs, which can be especially important for patients who are often seriously ill.[1] Uptake of DCTs has accelerated during the COVID 19 public health emergency, as use of telemedicine and eConsent increased, and clinical trial volunteers were "highly receptive to virtual and remote approaches."[5]

3. Fewer protocol amendments

Protocol amendments often cause delays and dramatically increase the costs of developing new therapies.[12] The potential for fewer research sites in a DCT leads to fewer institutional review boards and a corresponding reduction in regulatory costs and increased flexibility around protocol changes.[1]

Previous work by Tufts CSDD found that 57% of protocols had at least one substantial amendment, with a mean number of 2.2 amendments for phase II trials and 2.3 for phase III trials with commensurate costs of $141,000 and $535,000 (in US dollars) respectively for each substantial amendment.[16] Protocol amendments take time and incur direct costs, such as increased investigative site time and fees and contract change orders with clinical research organizations and other providers.

Conclusions

The cost of drug development is high and increasing, and the process is lengthy. These factors delay access to new therapeutics and limit the number of treatments available to patients.[17] One study found that for every billion U.S. dollars spent on research and development, the number of new drugs approved has decreased by 50% every nine years since 1950.[18]

We must reverse these trends and use available funds as efficiently and effectively as possible to help improve patient lives; DCTs are an important and evolving part of this change.

Tufts CSDD financial modeling found substantial net benefits to sponsors for deploying DCT technology solutions during clinical development of new drugs. According to Joseph DiMasi, director of economic analysis at Tufts Center for the Study of Drug Development (CSDD), "Our investigation found that, on average, the financial returns to drug sponsors from shorter development times, lower clinical trial screen failure rates, and fewer clinical trial protocol amendments associated with DCTs substantially exceeded the costs of investing in DCT technologies."

Medable SVP of Research and Strategy, Ingrid Oakley-Girvan, PhD, MPH, said: "Many aspects of DCTs require proof to facilitate a responsible shift so research is more accessible and truly represents the population of interest. We document that the initial investment results in substantial benefits ie: shortened enrollment periods, lower screen failure rates and fewer amendments. We anticipate that these findings will promote willingness to change the traditional brick and mortar research model to one with more decentralized elements. Ultimately this will increase the pace and decrease the cost of discovering drugs that improve the lives of patients."

Acknowledgements

This research represents a tremendous team effort, and the author gratefully acknowledges the hard work of Tufts research colleagues Kenneth Getz, Joseph DiMasi, and Zachary Smith, and Medable researchers Mary Costello, Andrew Mackinnon and Ingrid Oakley-Girvan.

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