CLINICAL TRIALS **TRANSFORMATION** INITIATIVE

2019
CTTI ACCOMPLISHMENTS

Thanks to the invaluable input and support from members, experts, and many other stakeholders, CTTI ended the decade with many strong accomplishments. We continued to bring together the best and brightest across the clinical trials ecosystem to develop recommendations and resources that increase quality and efficiency in trials, while also paving the way for use of novel approaches, including mobile clinical trials, real-world data, and single IRBs (sIRBs).

And we didn't just initiate new work—we saw our work in action. CTTI's recommendations and resources were cited in the FDA strategic framework, highlighted in **Forbes**, used by many organizations for more high-quality, efficient clinical trials, and much more.

Completing a Hub of Solutions for Mobile Clinical Trials
New tools & real-life examples of CTTI's work showcase the actuality of digital trials

Creating a Pathway for Using RWD Sources
Resources & case studies highlight how to use RWD to plan trial eligibility criteria & recruit participants

Making "Engage Patients Early and Often" the New Status Quo
Strong collaborations & workshops drive the greater inclusion of patients in trial planning & execution

Driving sIRB Adoption
Enhancements to CTTI's extensive set of sIRB resources guide adoption

Improving Trial Quality & Efficiency
CTTI continues to lead efforts to advance clinical trials worldwide

Completing a Robust Hub of Solutions for Mobile Clinical Trials
New tools & real-life examples of CTTI's work showcase the actuality of digital trials

At the beginning of the year, we released our final set of work from the Mobile Clinical Trials (MCT) program— recommendations and resources for engaging patients and research sites when planning and conducting FDA-regulated clinical trials that use mobile technologies. We also launched an interactive database featuring 275 feasibility studies on mobile trials. These efforts round out many years of CTTI collaboration and work, resulting in a comprehensive hub of solutions for designing and running mobile clinical trials.

What's more, we increasingly saw our MCT work put into practice. For example, Genentech and the University of California, San Francisco (UCSF) are applying our suite of MCT recommendations to a 5-year breast health study. Meanwhile, Orikami used CTTI's novel endpoint recommendations to help develop a digital biomarker for multiple sclerosis research. With more organizations using our work to harness the power of technologies, we look forward to the great potential of improving the efficiency and quality of clinical trials.

> "The agency has worked closely with stakeholders, including the Clinical Trials Transformation Initiative, to identify innovative trial designs, evaluate the role of decentralized clinical trials and mobile technologies, and help validate novel endpoints that can enable trials to generate reliable evidence needed to assess product safety and efficacy more efficiently."
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> –(Then) FDA Commissioner Scott Gottlieb in a March 14 Statement

Creating a Pathway for Using RWD Sources
Resources & case studies highlight how to use RWD to plan trial eligibility criteria & recruit participants

Filling an important gap across the clinical trials ecosystem, CTTI released new Real-World Data (RWD) recommendations, resources, and case studies for using electronic health records (EHRs) and claims data to plan trial eligibility criteria and recruit participants. The work showcases the opportunities for using RWD sources to enhance the quality and efficiency of clinical trials. In particular, the three case studies offer a deep dive into the specific challenges faced by sponsors, and how they can be addressed. The release added to a growing portfolio of CTTI work focused on improving clinical trial quality and, when appropriate, embracing novel approaches for better trial design and execution.

> The FDA cited CTTI's Registry Trials and RWD work in its 2019 strategic framework for advancing the use of electronic tools to gather health-related data.

> "Regulators are working with industry and academia in advisory partnerships such as the Clinical Trials Transformation Initiative (CTTI), which has been actively issuing recommendations in areas such as mobile technologies and use of RWE."
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> –Jan. 7 Forbes article

Making "Engage Patients Early and Often" the New Status Quo
Strong collaborations & workshops drive the greater inclusion of patients in trial planning & execution

Collaborating with the FDA, CTTI held a successful workshop on "Enhancing the Incorporation of Patient Perspectives in Clinical Trials," attended by more than 1,000 in-person and virtual attendees. We also hosted a "Patient Engagement in Action: Insights from Patients & the FDA" webinar attended by 450+ people and co-led the Patient Engagement Collaborative (PEC) to achieve more meaningful patient engagement in medical product development.

Additionally, building on CTTI's existing comprehensive set of Patient Groups & Clinical Trials (PGCT) recommendations and resources, we launched a new tool that helps sponsors and patient groups identify high-value opportunities to collaborate.

> Several organizations – including Bayer, Fight Colorectal Cancer, and the Urinary Stone Disease Research Network – are using CTTI's PGCT work and sharing their stories in CTTI's forthcoming use case library, available in 2020.

> CTTI's PGCT work was cited in a FasterCures "Patient Organizations as Research and Data Partners" paper.

Driving sIRB Adoption
Enhancements to CTTI's extensive set of sIRB resources guide adoption

In the fall, CTTI unveiled [new resources](#)—including tools that walk research institutions, sponsors, and IRBs through considerations for determining engagement, as well as a library of available sIRB resources—to help facilitate the implementation of sIRBs in multicenter trials. This new work augments our existing set of sIRB recommendations and resources that address barriers to and drive adoption of sIRB implementation.

Also, in light of mandated changes set to take place in 2020, CTTI collaborated with an NIH workgroup to develop a [comprehensive plan for assessing the NIH's sIRB policy](#). Through this effort, we are advancing a shared goal of enhancing and streamlining the review process for multicenter studies so that research can proceed as quickly as possible.

> Collaborators on IMPACT-AFib – the first trial conducted under FDA-Catalyst – [used CTTI's sIRB recommendations](#), resulting in reduced study start-up time and eased administrative burden and costs for sites.

Improving Trial Quality & Efficiency
CTTI continues to lead efforts to advance clinical trials worldwide

CTTI led a multi-faceted effort to inform the appropriate renovation of ICH E6, with the broader goal of helping to improve clinical trials internationally. We ran a global, multi-stakeholder survey, conducted 20 in-depth interviews, and held an open comment opportunity to identify important areas of ICH E6 renovation. Analysis of this work is currently underway and will be reported to ICH in 2020.

We also saw increased use of [CTTI's Quality by Design (QbD) work](#). For example, the University of California, Irvine, is one of the latest organizations to implement QbD principles to improve quality and outcomes for their clinical studies.

> More than 4,300 researchers, analysts, and other visitors used [CTTI's Aggregate Analysis of ClinicalTrials.gov (AACT)](#) to answer questions about trends in clinical trials this year. In one instance, Washington University in St. Louis uses data from AACT for its Clinical Drug Experience Knowledgebase (CDEK), which is designed to explore every active pharmaceutical ingredient (API) with evidence of clinical testing.

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In the year ahead, CTTI looks forward to creating and sharing new resources that will help increase quality, diversity, and efficiency in clinical trials, and drive the use of mobile clinical trials, master protocols, and other novel design approaches.

Thank you again for your ongoing support for all of these accomplishments, as we work to transform clinical trials.