CLINICAL TRIALS **TRANSFORMATION** INITIATIVE

2021 CTTI ACCOMPLISHMENTS

Following a year that shook the world (and spun the research field), CTTI entered 2021 standing tall and with a new vision forward: Transforming Trials 2030. Knowing that cross-system collaboration is critical to creating true and lasting transformation, we enlisted the entire research and healthcare community to join us in achieving this bold, five-pillared plan for how clinical trials should be run by 2030.

And, in the meantime, we got to work.

CTTI teams, members, and staff made significant headway creating solutions as part of three key projects that support the "Patient-Centered and Easily Accessible" vision pillar: Decentralized Clinical Trials (DCT) Update, Novel Endpoint Acceptance, and Diversity in Clinical Trials. At the same time, we led key efforts – including convening global web conferences and developing new case studies – to further the "Designed with a Quality Approach" pillar.

We also laid foundations for the other three pillars. Our new Trials in Health Care Settings work supports "Fully Integrated into Health Processes," ClinicalTrials.gov Reporting project drives "Maximally Leverage Data," and efforts to make HABP/VABP study data publicly available will "Improve Public Health."

All the while, we emphasized the critical importance of sharing best practices and learning from each other – from launching our new Building Better Trials: Case Study Exchange portal, to participating on the Federal COVID Lessons Learned Initiative, to sharing important insights on COVID-19 treatment trials via public webinars.

We hope you'll read more about these many accomplishments in the report below. Thank you to the wonderful and committed CTTI community – members, partners, staff, and others – for making all of these successes possible.

Transforming Trials 2030: A New Vision for the Clinical Trials Ecosystem and Beyond

Providing a Blueprint for Smarter, Faster, and More Inclusive Clinical Research

Ensuring that Research is Patient-Centered and Easily Accessible

New Solutions for Furthering Decentralized Approaches, Novel Endpoints, and Diversity in Clinical Trials

Infusing a Quality Approach into the Start of Every Clinical Trial

Case Studies Showcase the "How, What, and Why?" of Quality by Design

Laying the Groundwork for Other Vision Priorities

New Work to Advance Clinical Trials that are Integrated with Health Care, Leverage Data, and Improve Public Health

Continued Leadership in COVID-19 Treatment Clinical Trials and Beyond

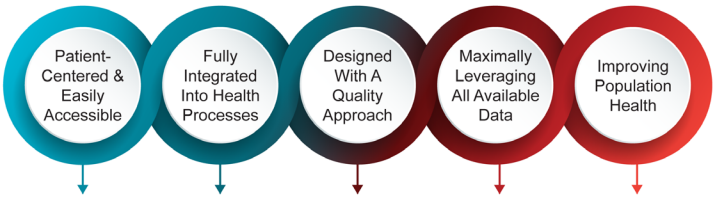
Applying Lessons Learned from the Past to Trials of the Future

Transforming Trials 2030: A New Vision for the Clinical Trials Ecosystem and Beyond

Providing a Blueprint for Smarter, Faster, and More Inclusive Clinical Research

CTTI formally announced our bold vision, Transforming Trials 2030, for how clinical trials should be done in 2030 – five pillars that will guide CTTI's work for the next nine years. Recognizing that cross-system collaboration is critical to unlocking the changes needed to make this vision a reality, we encouraged others across the clinical trials ecosystem to prioritize and contribute to Transforming Trials 2030. To date, 25 organizations have publicly proclaimed their involvement and commitment, the National Academies of Sciences, Engineering, and Medicine published an aligned Envisioning a Transformed Clinical Trials Enterprise for 2030 publication, and many others are engaging with CTTI to help advance this shared vision.

By 2030, clinical trials need to be:



A critical part of the Evidence Generating System

"We are really focused on ensuring that remote data are accurate and reproducible."
– Lynne Quittell, Cystic Fibrosis Foundation

"We're only collecting the things we need, and that's a good thing. Keep it very simple and don't get too complicated."
– Jules Mitchel, Target Health LLC

Ensuring that Research is Patient-Centered and Easily Accessible

New Solutions for Furthering Decentralized Approaches, Novel Endpoints, and Diversity in Clinical Trials

CTTI will only accomplish its vision if patients are partners with us on the journey. Because of that, we focused on three key projects that will help drive patient-centricity and accessibility in clinical trials:

- DCT Update
- Novel Endpoints Acceptance
- Diversity in Clinical Trials

The multi-stakeholder project teams that conducted this work collaborated closely all year to analyze research, host Expert Meetings, and draft – or in some cases, refresh – recommendations and resources that will be available in early 2022 to support more efficient and inclusive trial design and conduct.

In addition, we proudly announced new patient and caregiver members to both our Steering Committee and the Patient Engagement Collaborative, a co-effort with the FDA. Adding these unique and passionate perspectives will be invaluable in CTTI's effort to build more patient-centered and accessible trials.

The Healthy Mind Lab at Washington University in St. Louis (WUSTL) used CTTI's existing DCT recommendations to navigate licensing requirements, recruitment, and other challenges in their quest to design a fully remote trial to test if fluvoxamine, a commonly used medication for depression and anxiety, could help treat clinical deterioration from COVID-19.

DiMe highlighted CTTI's existing Novel Endpoints recommendations and resources in its *The Playbook*, a go-to resource for building digital health programs.

Infusing a Quality Approach into the Start of Every Clinical Trial

Case Studies Showcase the "How, What, and Why?" of Quality by Design

We've long talked about the benefits of Quality by Design (QbD), offering a full suite of recommendations and resources since 2015. This year, however, others showed how QbD can be put into action and how those benefits really pay off.

Case studies from Alexion, Duke Clinical Research Institute, The Medicines Company (now part of Novartis), and University of Oxford's Clinical Trial Service Unit & Epidemiological Studies Unit (CTSU) showcased the implementation of CTTI's QbD work and provided tangible strategies and examples for how other organizations can implement such an approach.

In another critical effort to drive quality in clinical trials worldwide, CTTI hosted two web conferences for the International Council for Harmonisation (ICH) E6 Good Clinical Practice (GCP) Expert Working Group (EWG) to provide an update to the global research community on the progress to revise its important guidelines. More than 6,000 people around the world attended or re-watched the conferences, underscoring the growing importance of and focus on building quality into clinical trials.

"The trial met its timeline, and when the FDA approved the investigational product, the news was met with celebration across the organization. In an all-hands meeting with the Alexion's CEO, every single product team member mentioned quality as a critical driver to the trial's success. An organization that once saw quality as a tick box to be checked was transforming into one with a holistic quality perspective and individual ownership across the enterprise. This change, Alexion's leaders say, is the true value of implementing QbD."

– Alexion QbD case study

Laying the Groundwork for Other Vision Priorities

New Work to Advance Clinical Trials that are Integrated with Health Care, Leverage Data, and Improve Public Health

In addition to making immediate headway with the Transforming Trials 2030 "Patient-Centered and Easily Accessible" and "Designed with a Quality Approach" pillars, we also expanded our project portfolio to support the three other vision pillars:

- "Fully Integrated into Health Processes" – We conducted in-depth interviews and are developing case examples highlighting the barriers and solutions to integrating trials as part of our Trials in Health Care Settings work. This will help shape the development of these much-needed recommendations and resources to help integrate clinical trials into clinical care.
- "Maximally Leverage Data" – We launched a new project to identify and explore the key challenges to clinical trial registration and results reporting in ClinicalTrials.gov. Through this work, we aim to develop best practices and recommendations that will ensure the site includes timely and complete information.
- "Improve Public Health" – We are working to make the data from our 7,000+ patient observational hospital-acquired and ventilator-associated bacterial pneumonia (HABP/VABP) study publicly available.

Looking ahead, CTTI will continue take on new projects, partnerships, and efforts to advance each Transforming Trials pillar.

The Aggregate Analysis of ClinicalTrials.gov (AACT) website remains one of the most visited and used resources that CTTI offers to date, with 87,167 web views and 7,618 downloads in 2021.

Continued Leadership in COVID-19 Treatment Clinical Trials and Beyond

Applying Lessons Learned from the Past to Trials of the Future

Along with dedicated efforts and momentum around Transforming Trials 2030, CTTI also continued to lead COVID-19-related efforts, serving as a leader in lessons learned in transitioning to virtual trials amid the pandemic, as well as designing and conducting COVID-19 treatment trials.

In collaboration with the Duke-Margolis Center for Health Policy and FasterCures, we held "The Fastest Path to Effective COVID-19 Treatments: Using Master Protocol Studies" public summit to offer best practices and insights from those involved in COVID-19 treatment master protocols. We also led a State of U.S. COVID-19 Treatment Clinical Trials public webinar to share findings from an analysis of data downloaded from the database for AACT.

These efforts, in addition to our participation on the Federal COVID-19 Lessons Learned Initiative, speak to CTTI's ongoing commitment to applying lessons of the past to inform better, faster clinical trials of the future.

Why COVID master protocols haven't been productive in the U.S., and how to fix it

–BioCentury Extra, Jan. 22, 2021

BIOCENTURY

Thank You!

And now, we look toward 2022 (our 15th anniversary!) with great momentum, hope, and opportunity, bolstered by the fresh perspective and ideas from new CTTI Executive Director Sally Okun.

It took a village to weather the storm of a pandemic, and it's going to take that same village to build a better, stronger clinical trials system than ever before. Thank you for joining us on this exciting journey!

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