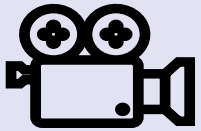
ACT EU Multi-stakeholder Meeting on Decentralised Clinical Trials

4 October 2022

Etiquette for
ACT EU multi-stakeholder meeting on decentralised clinical trials



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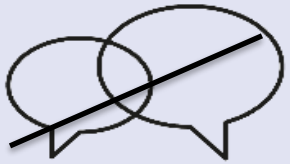

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Welcome & opening remarks

Chairs: **Emer Cooke**, EMA / ***Peter Arlett,*** EMA / ***Greet Musch****, FAMHP/CTCG*

Agenda – ACT EU Multi-stakeholder Meeting on Decentralised Clinical Trials

4 October 2022 09:00 - 17:00 (CEST)

Onsite at European Medicines Agency & live broadcast and recording (for plenary sessions)

4 October 2022 (09:30 – 13:00)

Room 1C/live broadcast and recording

- 09:30 Welcome and introduction
- 09:50 Authority Perspective
- 10:30 Sponsor and CRO Perspective
- ***11:20 20' coffee break***
- 11:40 Patient and Investigator Perspective
- 12:50 Closing session
- ***13:00 End plenary session***

Scope of the DCT collaboration across the European Medicines regulatory network

Presented by Ditte Zerlang Christensen (DKMA)

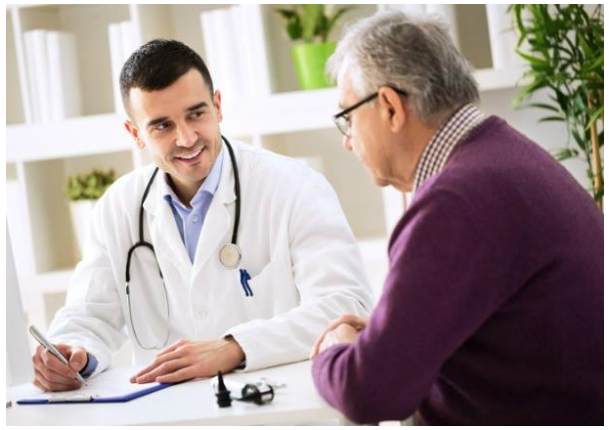
Decentralised Clinical Trials (DCT)

The DCT approach seeks to take advantage of the technological and scientific progress to introduce new methodologies to the conduct of clinical trials with the aim to make clinical trials more easily accessible and participation more convenient for trial participants.

The DCT methodology is based on elements such as:

- **Home health visits** including teleconsultation and visits where health care professionals come to the trial participant's home,
- **Direct shipment** of investigational medicinal products (IMPs) to trial participants,
- **Electronic informed consent** procedures.

Major impact on the environment for conduct of clinical trials



Recommendations on decentralised elements in CTs from European Medicines Regulatory Network

DCT Recommendation paper

Harmonised perspective focused on

- Trial participant-centred and risk-based approach
- Investigator and sponsor oversight
- Reliable and robust data fit for purpose

National provisions overview

Member state specific provisions

- Where national legislation does not currently allow for alignment

First publication: end Q4 2022

Best practice for EU CT authorisation and inspection

Update as knowledge and experience evolve

Cross disciplinary recommendations

45 experts across 12 MSs – 25 onsite today

Driven by

CTCG Clinical trial experts	CTEG Ethical experts	GCP IWG GCP inspectors

Incorporating perspectives by

SAWP Scientific advice experts	MWP Data methodology experts	PDCO and Enpr-EMA Paediatric experts
PCWP Patient representative	HCPWP Health care professionals	

Scope of todays meeting

Challenges
→ *manage and mitigate*

Opportunities

To create a harmonised approach and facilitate use of DCT elements in EU clinical trials, maintaining:

- Trial participant safety, right and dignity
- Reliable and robust data fit for purpose

Authority Perspective

DCT recommendations from the European Medicines regulatory network

Presented by Solange Levision (CCMO) & Monique AL (CCMO)

Some points of attention

Recommendation paper:

- focus is on decentralised elements in the conduct of clinical trials
- GDPR specific requirements not provided
- principles and rules “traditional clinical trials” similar for clinical trials with decentralised elements will not be repeated unless it fits a purpose
- definition on decentralised clinical trial, decentralised elements, etc – not included to avoid getting caught in words
- the recommendation paper will evolve over time

Recommendation paper on decentralised elements in clinical trials in the EU

Table of content:

General considerations (1)

Basic principle that **right, safety and well being** of trial participants to be **protected** and **prevail** over all other interests

Adherence to EU and national applicable laws, regulations and established standards for clinical trials (overview of national provisions in appendix of recommendation paper)

Involvement of **patients and investigators** in an early and sustained manner – implementation of DCT elements according to patient and investigator needs.

Trial specific **rationale** for selected decentralised elements, where any transfer of **burden to trial participants or investigators** should be weighed against the benefits of using decentralised elements in the clinical trial.

Trial specific **risk-benefit assessment**, focused **on selected decentralised elements** which may have an impact on scientific validity, patient safety, benefit/risk ratio or protection of participants rights.

General considerations (2)

Appropriate **training** and guidance of third parties, trial participants and investigators.

Qualifications of third parties performing trial related tasks.

Generating **reliable and robust data** fit for use for regulatory decision making and/or publication in peer-reviewed journals.

IT devices/technologies developed and utilized should be **fit for purpose**. Use of computerised systems or creation/capture of electronic data should be compliant with GCP-IWG Guideline on computerized systems and electronic data in clinical trials (EMA/226170/2021 – to be published).

A **contingency plan** should be in place to minimise the impact of any risk (e.g. malfunction of a digital tool, lack of web connection…)

Clinical trial oversight: roles and responsibilities

↓ On site visits

↑ Involvement of third parties

↑ Use of e-systems

↑ Amount of incoming data (wearables, home nursing staff, patient reported outcomes, etc.)

<u>More tasks are delegated - responsibilities stay the same (GCP)</u>

It should be ensured that the sponsor and investigator keep oversight on the safety and well-being of the trial participants:

→ **Document** which tasks are conducted at what place, when, and by whom (contractual agreements, protocol)

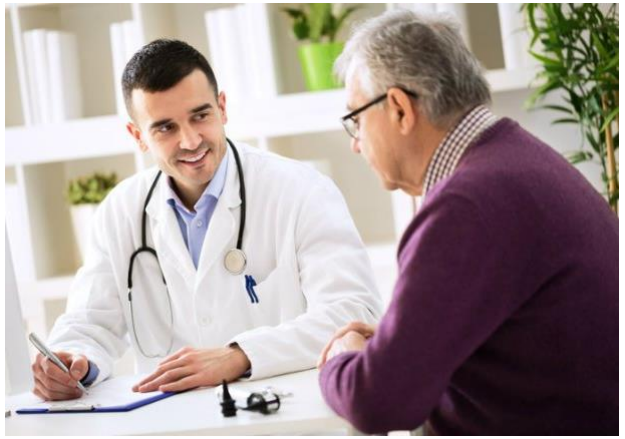
→ Clear **communication** plan between the different parties involved

→ Procedures should be in place to handle the constant flow of information (safety alerts) so that the **investigator has appropriate oversight** of patient treatment, safety and well being.

Informed consent process

Hybrid forms possible:

- Informed consent interview → remote or on site
- Patient information (leaflet) → digital, on paper, or video
- Signature → electronic or ‘wet ink’



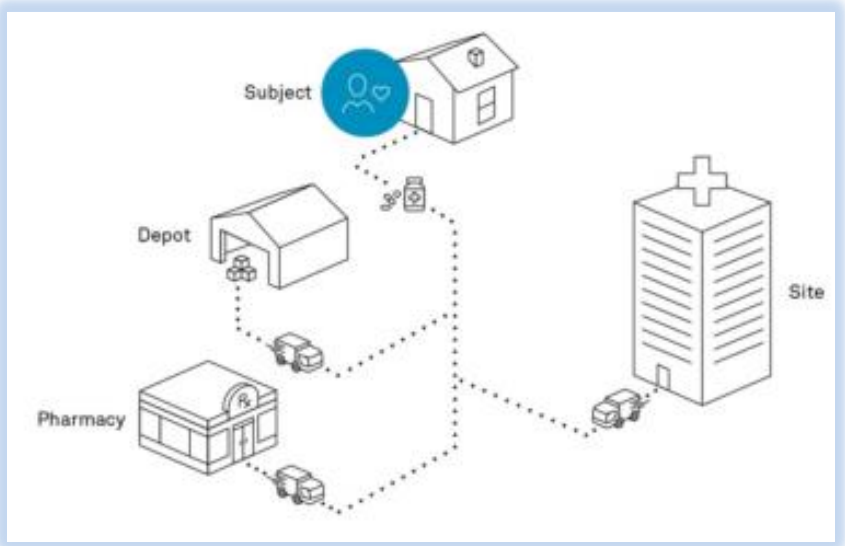
Risk-based approach:

→ **Interview**: Consider importance of face-to-face (use of videocall).

→ **Patient information leaflet**: Awareness of participants who cannot or prefer not to use electronic technology (non-digital methods available).

→ **Signature**: Depending on national legislation. Requirements described in *Guideline on computerised systems and electronic data in clinical trials.*

Delivery of medicinal products and administration at home



- From the sponsor depots/manufacturer/distributor to the participant's home
- From the pharmacy of the investigator's site
- From a local pharmacy (close to the participant's home)

Basic principles:

→ Highly restricted access to trial participants contact details.

→ The investigator remains responsible for requesting and initiating each shipment of IMP.

→ Investigator and sponsor should assess whether it is feasible with regard to storage conditions of IMP.

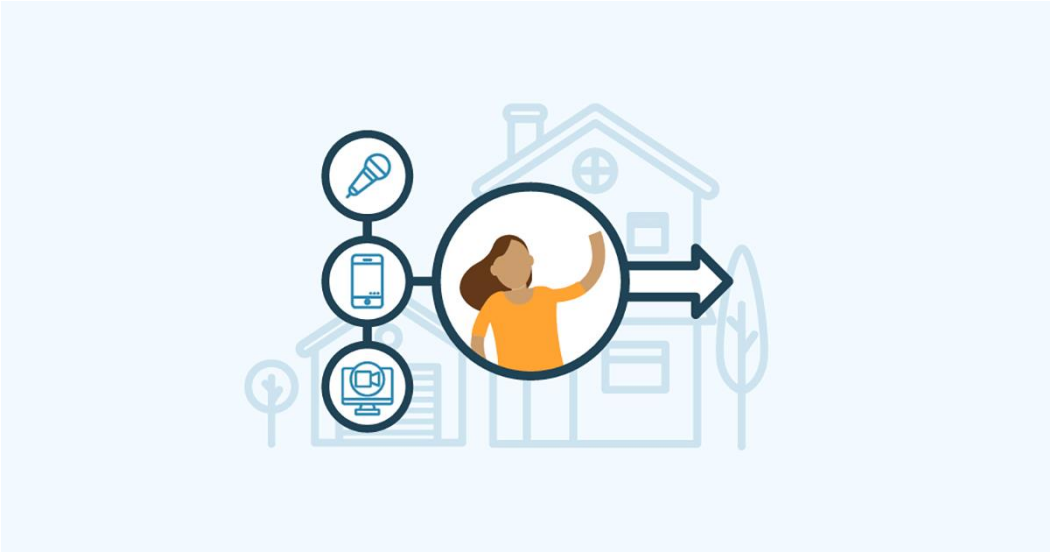
→ Consider who can do the administration of the IMP at home.

→ Participants should be clearly instructed on the use and storage of the IMP.

In the national provisions overview specific requirements for the IMP shipment are given

Clinical Trial Procedures at Home

Focus only on trial related procedures performed at the trial participant home by site personnel, third party or trial participant



→ Feasibility of performing trial related procedures at home

→ Impact on trial participant/investigator relationship with less on-site visits on compliance of trial participant (new role of GP?)

→ Need for on-site visits or visits at home (by investigator or other qualified persons)

→ Provision of devices by sponsor to capture data (as alternative for private devices)

Defining and handling of source data

Focus is on **raising awareness**:

↑ percentage of data collected outside clinical trial site

↑ complexity of data flow

→ Same principles regardless of the location of data capture

→ Sponsor responsibility to ensure that investigator has control and continuous access to source data, irrespective of location of data capture

→ Reference to the EMA GCP Q&A B.3 “How and where should source data be defined?” and GCP Q&A B.5 “What are the expectations of the investigator’s copy of the CRF when using a web-based application?”

→ Reference to SAWP opinion and notice to sponsor on validation and qualification of computerised systems

Remote monitoring

Recommendations on a high level as appropriate and in line with national provision.

Thank you for your attention!

Authority Perspective

Q & A

Sponsor and CRO Perspective

Industry sponsor perspective: Opportunities and challenges for the use of DCT elements in clinical trials

Presented by Alison Bond (EFPIA)

European Federation of Pharmaceutical Industries and Associations

Industry sponsor perspective: Opportunities and challenges for the use of DCT elements in clinical trials





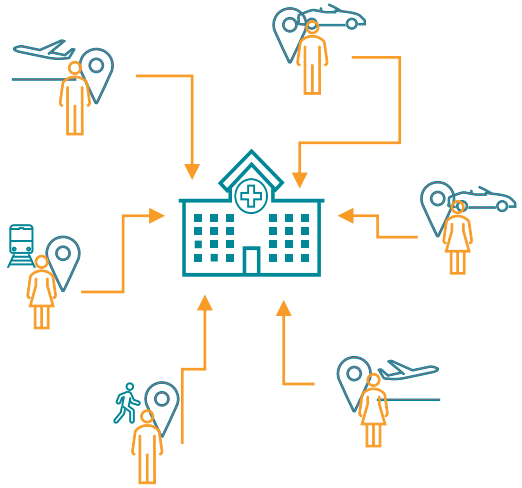

Alison Bond

Director, Global Regulatory and R&D Policy, Amgen

Co-chair, EFPIA DCT Team

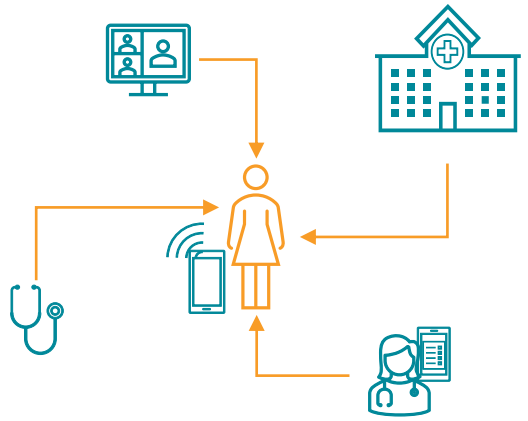
Bringing the trial to the participant – Providing options, reducing burden & facilitating participation

Traditional Clinical Trial Model



- Wherever the participant is, they generally have to travel to the site
- For many participants this can involve great distance to get to a specialist centre
- **The trial is organised around the site**

Decentralised Clinical Trial Model



- Some or all of the assessments can be undertaken local to the participant or remotely
- **The trial is organised around the participant & considers the needs of the site**

The selection of DCT elements depends on the trial design & participant population

Telemedicine

Direct to Participant IMP Shipment and Administration

Mobile Healthcare Providers *(e.g. Home Nurses)*

Electronic Informed Consent / Remote Consenting

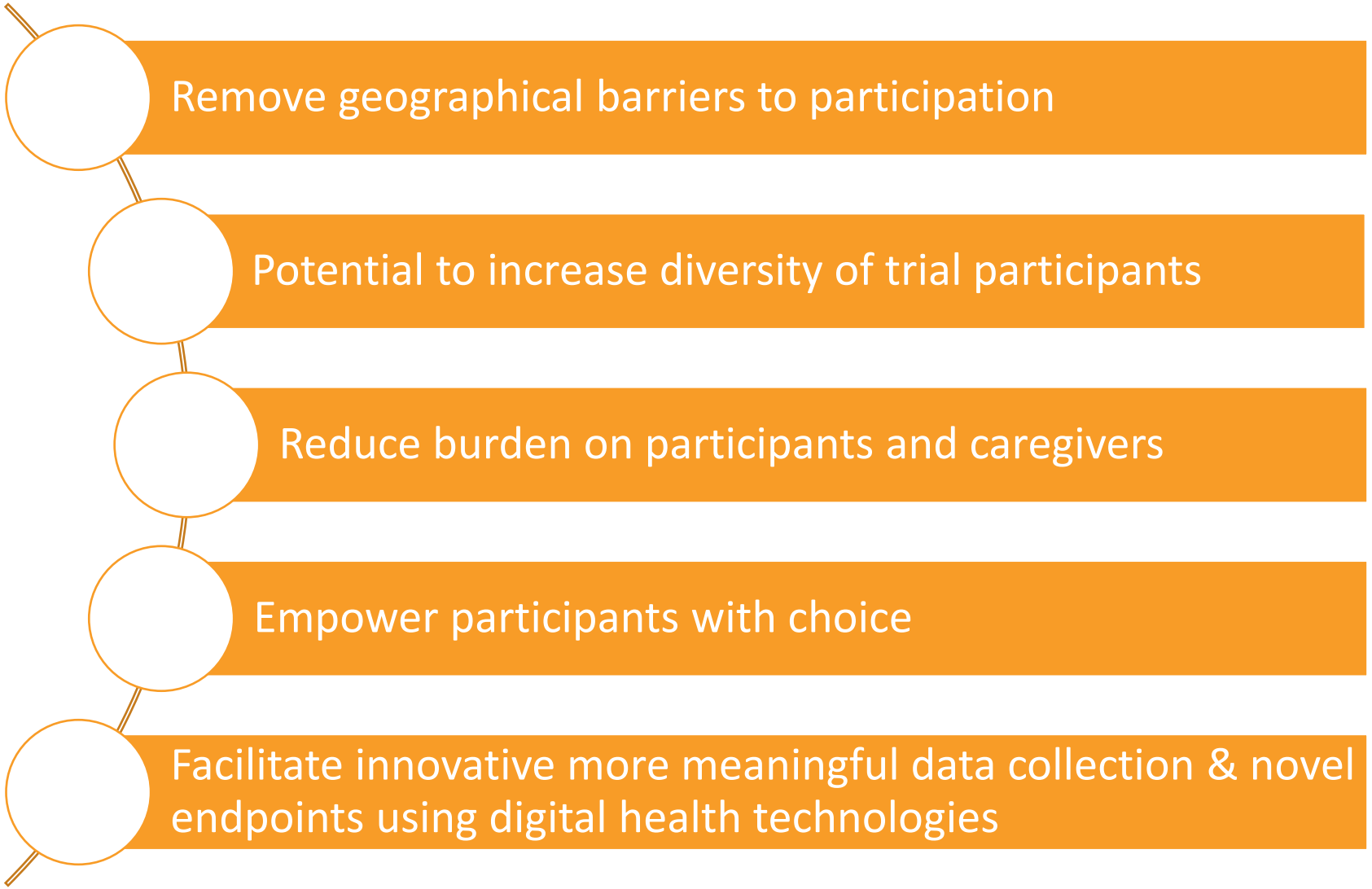
Local Healthcare Providers *(e.g. General Practitioner)*

Concierge Services

Local Laboratories and Imaging Centres

Digital Health Technologies *(e.g. wearables)*

Opportunities for further enhancing research through DCT approaches



Perceived and experienced challenges of DCTs

Operational & Technology

- Data consolidation
- Complexity of data flows
- Potential for selection bias
- Impact on existing operations

Relationships & oversight

- Participant/Investigator/sponsor relationship
- Investigator oversight of patients & distributed care team
- Reporting and handling of potential AEs/SAEs
- Site training & acceptance of the use of DCT elements

Data privacy/protection

- Participant names and addresses in platforms
- Electronic signatures

Data quality

- Comparability of data for site & remote assessments
- Data acceptability & management of large data sets

Regulatory framework

- Lack of regulatory guidance globally
- Fragmentation - adaption required according to local laws and regulations

Preserve participant safety, rights and well-being and data integrity, to provide reliable trial results

What are we doing to support use of DCT elements?

- DCT elements, if used, are selected to fit the participant population, Investigational Product, trial design & country
- Working together across trade associations and industry initiatives to have a shared understanding
- Collaboration, sharing expertise & experience, to employ best practice across sponsors
- Engagement of patients, advocates and HCPs in trial design, including DCT elements

All parties have the same aim – Participant rights, safety and well-being and reliable trial results

What is needed to further advance use of DCT elements?

- EU: Clearer understanding of differences between Member States - harmonised & transparent approach for use of DCT elements
- Globally: ICH E6 (R3) support use of DCT elements & provide a degree of harmonisation *(EU guidance should be aligned)*
- Agreement on where in a CTA DCT elements to be used in a trial should be described and justified
- Recognition of acceptability of data from trials with DCT elements – recommendations around (statistical) approaches for data

All parties have the same aim – Participant rights, safety and well-being and reliable trial results

The future of Decentralised Clinical Trials is.....

The future of Decentralised Clinical Trials is.....

...Clinical Trials

DCT elements become part of the clinical trial toolbox!

European Federation of Pharmaceutical Industries and Associations





Thank you

Academic Sponsor perspective: Experiences on use of DCT elements during covid-19

Presented by Vassilis Golfinopoulos (EORTC)

EORTC
European Organisation for Research and Treatment of Cancer

Academic Sponsor perspective: Experiences on use of DCT elements during covid-19

Vassilis Golfinopoulos, MD, PhD
EORTC Headquarters Director

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Any DCT measures were triggered by the sites and needed centralised oversight

- **Centralization** of COVID-19 related communications
- **Early sponsor assessment** of COVID-19 impact on ongoing trials, opening new trial sites, ongoing recruitment and starting new trials
- Clear **communication** to investigators and site staff on COVID-19 measures considerable based on:
  - Risk-benefit assessment
  - Local/national recommendations and measures

Remote informed consent on request

- Initial consent remained on site
- **Reconsent** in case of amendment or urgent changes in trial conduct:

  via phone or video call followed by email confirmation

Imaging exams done at remote site

In relation to imaging procedures, order of preference*:

- Onsite
- Offsite, on time
- Onsite, delayed (e.g., due to travel restrictions)
- Offsite, delayed (e.g., due to travel restriction)
- Skip the imaging only if impossible to perform due to travel restrictions or safety consideration.

* Unless imaging **must**, per protocol, be done at the main site when imaging result drives treatment allocation.

Patients visits allowed to switch to remote

- Patient visits could be changed to phone visits
- Standard repeated assessments (e.g., biochemistry) could be done by **contracted** laboratories
- Optimal **traceability** of protocol deviations due to COVID-19
  - Sites requested to discuss deviations with sponsor before implementation
  - Documentation of deviation in case report forms and medical records

Study treatment delivered at patients' home on request

- All oral formulations
- Shipment by vendor and temperature controlled
- Use of a Pharmacy Guideline Addendum
- Acknowledgment by patient once received
  - Ensure that content of the delivery is correct and undamaged
- Where assessments (e.g. blood counts, biochemistry) are required prior to study treatment administration
  - At the centre or contracted laboratory
  - Centrally reviewed

Additional consent for drug shipment to patients' home

Investigators should tell their patients that drug suppliers will get their name and address for delivery

Direct to patient delivery needs work for each case

For **52 patients**, from **19 sites**, in **10 clinical trials**

Study team involvement

- The regulatory manager checks the national guidance to assess what is allowed and what are the responsibilities of each party.
- The project manager calculates the additional cost.
- The medical monitor approves each request. Please note that whether or not direct-to-patient shipments are possible in each study has already been discussed while drafting the COVID-19 letter.
- The clinical research associate is informed.

Note to the trial master file for all.

Example of one member state (1/3)

- COVID-19 measures that have a significant impact on the protection and safety of participants (e.g., delivery of treatments to the patient's home) were put in place as Urgent Safety Measures
  - Followed by submission of substantial amendment to regulatory bodies

- Transitional measures during the pandemic period were submitted as appendix to the protocol to regulatory bodies

Example of one member state (2/3)

- Impact of crisis on all hospitals independent of size (20 sites reporting)
- Pharmacies kept open allowing drug reception during crisis
- Requested shipment of IMP to patient's home in 7 studies
- Patient visits: remote visits and use of local laboratories

Example of one member state (3/3)

- On-site monitoring: put on hold, follow-up with the site by phone
- Remote visits: discussing actual protocol deviations, assess IMP in stock, support data entry, follow-up and closure of major observations from past monitoring visits
- **No source data verification**
- Treatment dispatched to patients abroad, if unable to travel to the country

In oncology, we are not ready for fully remote procedures

Capability for decentralised processes comes **on top** of existing ones and requires different setup per member state

One can imagine specific clinical scenarios with decentralised-only processes (for example, follow-up of cancer patients) – these trials would assess the remote processes as much as the main trial endpoints

EORTC
European Organisation for Research and Treatment of Cancer

Thank you

vassilis.golfinopoulos@eortc.org

CRO Insights and Experiences: How to solve identified challenges on the implementation of DCT elements in clinical research

Presented by Yoanni Th. Matsakis (EUCROF) & Fiona Maini (ACRO)

CRO Insights and Experiences: How to solve challenges on the implementation of DCT elements in clinical research

EU DCT Workshop 4 October, EMA, Amsterdam

Joint session between ACRO and EUCROF

Fiona MAINI
Chair of the ACRO DCT Working Party

Yoanni Th. MATSAKIS
EUCROF Executive Board

Points for consideration

CYBERSECURITY - GDPR

- Patient Safety
- Data Protection
- Quality of data

HYBRID STUDIES – DATA HETEROGENEÏTY

Topic 1

- Regulatory harmonization

DCT elements that are implementable in some countries and regulatory jurisdictions but not in others:

- Electronic Informed Consent & Electronic Signature
- Telehealth visits
- Home Health (Nursing) Services
- Direct to Patient Shipment of the Investigational Medical Product …

Can some hybrid DCT elements be deployed in some countries and not in others under the same protocol? And for those countries in which DCT elements are not deployable, can we deploy traditional/in-person approaches under the same protocol?

Will this point be addressed in the recommendations in preparation ? Is it possible to implement some harmonization process & how ?

Topic 2

- Site readiness

DCT is having multiple impacts on how clinical research is carried out in Clinical Sites:

- Multiple sponsors with multiple technologies to be implemented …
- Some technologies may be highly site dependent : EMR, telehealth visits systems …
- Sites may not accept some of the suggested options ?
- They may require upgrades both from technical and organizational standpoint : e.g. for rSDV giving access to the EMR system of the hospital and only to the "need to know" patient records …
- Interoperability between CR systems and HIS software ?
- How to handle DPIA in certain situations ?

We witness the development of "proof-of-concept" approaches with sponsor / sites / CROs partnerships…

What would be EMA's recommendations on such issues ? How shall sponsors (academic and private) handle such heterogeneous situations ? Is this point addressed ? Shall we envisage some specific action plan ?

Topic 3

- Data Protection

The EU GDPR regulation is often perceived as an "inhibitor" for the adoption of innovation as it introduces new concepts for which there are different perceptions and obligations to sponsors and sites (DPIA, processes registries …)

To our view, it is an **innovative regulation** that shall facilitate the adoption of technological and organizational innovations for DCTS :

- Articles 40 & 41 of the General Regulation foresee the implementation of **codes of conducts** in domains with specificity, as well as other mechanisms (certifications …)
- The regulator provides to the stakeholders a unique opportunity to **co-build the regulation**
- A **cooperation mechanism** to create the conditions of a **harmonized landscape** throughout the 27 EU Member States
- Through the Process / Controller / Processor new concepts, contribute to better define responsibilities & mitigate cybersecurity risks and foster transparency and trust

Should Data Protection issues be left outside of the domain, and the opportunities offered by GDPR codes of conduct (or other approaches) could at least be mentioned and related initiatives supported…

Other topics for further discussions

- ## Protocol flexibilization / Hybrid situations

  Is there a way from the regulatory viewpoint to allow patients to change in-person versus remote visits during a DCT randomly and at their convenience? Or must patients commit to one way of participating in a DCT either remote or in person as dictated by the protocol?

  Can differences in the adoption of DCT tools & methods among sites be allowed in a the same trial ? Etc …

- ## Responsibility / role of local HC providers versus DCT personnel

  When is a local healthcare provider considered part of the DCT personnel? When is a local healthcare provider just that and not part of DCT personnel? How are responsibilities split between investigator and home nurses" ?

  Shall we reassess the scope of responsibility of the hospital investigator: keep the supervision of the study to the investigator, but limit the responsibility for homecare to the homecare healthcare professionals

EUCROF relevant activities

https://www.eucrof.eu/news-eucrof/publications
https://cro.eucrof.eu/eucrof-code-public-registry

- Electronic Informed Consent Implementation Guide – March 21
- Propositions Paper on Remote SDV/SDR (endorsed by EFPIA - 2 June 22)
- EUCROF GDPR Code of Conduct for Service Providers in the 27 EU Member states

➔ *Create the conditions of trust & confidence to foster innovation for better care with appropriate patient involvement*

- Implementing Decentralised Clinical Trials in Italy (June 2022)
- Recommendations on DCTs by FR Working Group …

Thank you for your attention!

Contributing Members' Contact Information

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ACRO DCT Working Party

Overview of the ACRO DCT Tool kit for for the Accelerating Clinical Trials in the EU Workshop

4th October 2022

Fiona Maini ACRO DCT WP Chair

ACRO
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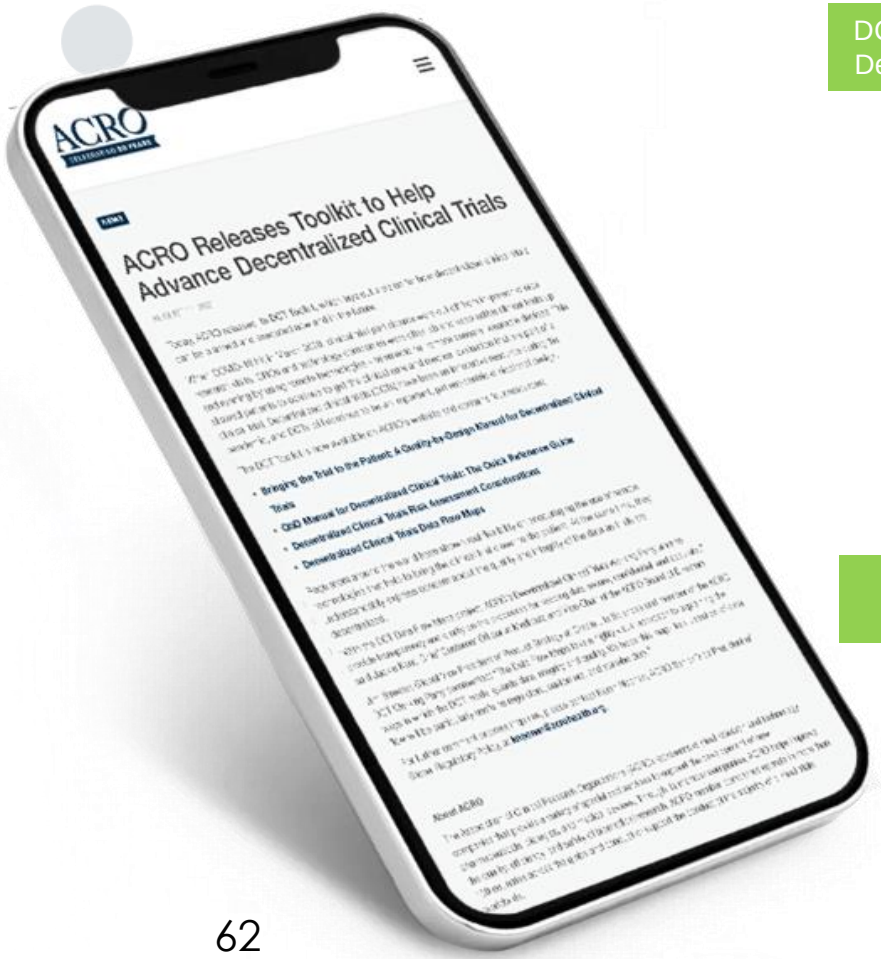
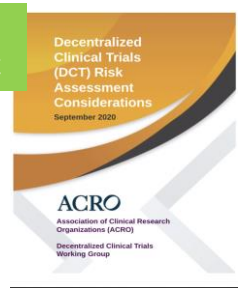
Introducing the ACRO DCT WP Tool Kit



DCT Quality by Design Manual

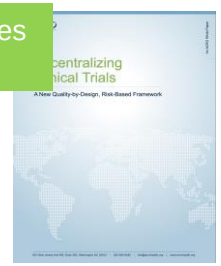
Published 2020 - Bringing the Trial to the Patient: A Quality-by-Design Manual for Decentralized Clinical Trials

DCT Risk Assessment



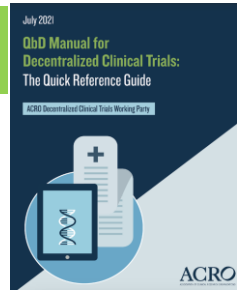
Published 2020 Decentralized Clinical Trials Risk Assessment Considerations

DCT Case Studies and Methods



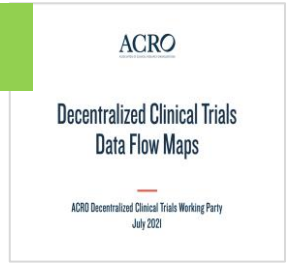
Published 2020 Decentralized Clinical Trials White Paper and use cases

DCT QbD QRG



Published 2021 QbD Manual for Decentralized Clinical Trials: The Quick Reference Guide

DCT Data Flow Maps



Published 2021 Decentralized Clinical Trials Data Flow Maps

Navigating DCT Change

To be published 2022 Decentralized Clinical Trials Change Management Tool

Accelerating DCT in the EU

Key Learnings and Recommendations

Connecting on Experiences - DCTs are not a new concept

DCT processes and technologies are established. Case studies, lessons learned, best practice recommendations, and training tools are available to help inform the EU DCT Recommendations.

Recognising variability across Member States

There are variations in national legislation provisons, regulation, and guidance on key DCT processes and tools (e.g., electronic informed consent, direct-to-patient shipment, and remote source data verification and review).

Enabling transparency of Member State requirements

The development of a central repository of national legislation, regulation, and guidance -- on each DCT process and tool (e.g., eConsent, DTP shipment, rsDV etc.) -- would mitigate challenges for sponsors and CROs in trying to execute pan-European DCTs.

Enabling greater coordination across regulatory agencies

DCTs and digital technologies highlight the importance of data protection. Greater communication and coordination between (1) health regulators and (2) data protection regulators, other regulations such as eIDAS– at both the EU level and the Member State level – would help advance DCTs.

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Appendix: Further Details for reference

ACRO DCT WP Toolkit

ACRO DCT TOOLKIT

ACRO Member Companies

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CRO Insights and Experiences:
Contributors to this joint session between ACRO and EUCROF

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EUCROF European CRO Federation

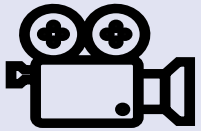
ACRO CELEBRATING 20 YEARS

Sponsor and CRO Perspective

Q & A

Coffee Break - 11.20 – 11.40h

Next up: Patient and Investigator Perspective



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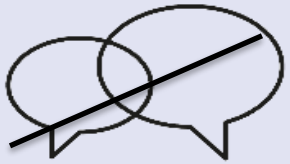

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Patient and Investigator Perspective

Patient Perspective

Presented by Julián Isla (COMP and Dravet Europe)

Decentralized clinical trials

A patient view

Julian Isla
Dravet Syndrome European Federation

dravet europe
Dravet Syndrome European Federation

Microsoft Consulting Services. AI

Founder

European Syndrome Federation founder

TAG. Therapeutic Advisory Group member

Orphan Drug Committee member

Scientific Advisory Board

I attend this conference as an individual expert, and do not represent the
EMA. The views expressed here are my personal views, and may not be understood or quoted as being made on behalf of the EMA or reflecting the position of the COMP

CV

http://www.ema.europa.eu/docs/en_GB/document_library/contacts/islaj_CV.pdf

DOI

http://www.ema.europa.eu/docs/en_GB/document_library/contacts/islaj_DI.pdf

Maybe the name is not correct

*Meaning of **decentralized** in English*

decentralized

adjective • MANAGEMENT, WORKPLACE • (UK also **decentralised**)

UK /ˌdiːˈsentrəlaɪzd/ US

used to describe organizations or their activities which are not controlled from one central place, but happen in many different places:

DECENTRALIZED | meaning, definition in Cambridge English Dictionary

And this is happening right now

Clinical recurrent seizures are the primary marker of the condition. They are of several types as classified in the International Classification of Epileptic Seizures, mainly: generalised onset, focal onset, which may become secondarily generalised and unclassified seizures.[1]

[Clinical investigation of medicinal products in the treatment of epileptic disorders | European Medicines Agency (europa.eu)]

How many seizures?

Hybrid could be an option

Fully decentralized ⟷ **Hybrid** ⟷ **Fully centralized**

All trial procedures are conducted virtually, enabled by digital technologies and supply delivery

Less complex trial procedures that don't require in-person visits (eg, vital signs, electrocardiograms) are conducted via telehealthcare, remote data collection, or direct-to-patient supply

Less complex trial procedures that require in-person visits (eg, injections) are conducted via mobile clinicians or alternative sites (eg, mobile clinics, retail sites)

Complex trial procedures (eg, complex screening protocols, cell therapy, magnetic resonance imaging) are conducted via research sites (eg, academic medical centers) or local hospitals

All trial procedures are conducted at a research site (eg, academic medical center)

Patients should make a choice

Decentralization can lower motivation if it is forced

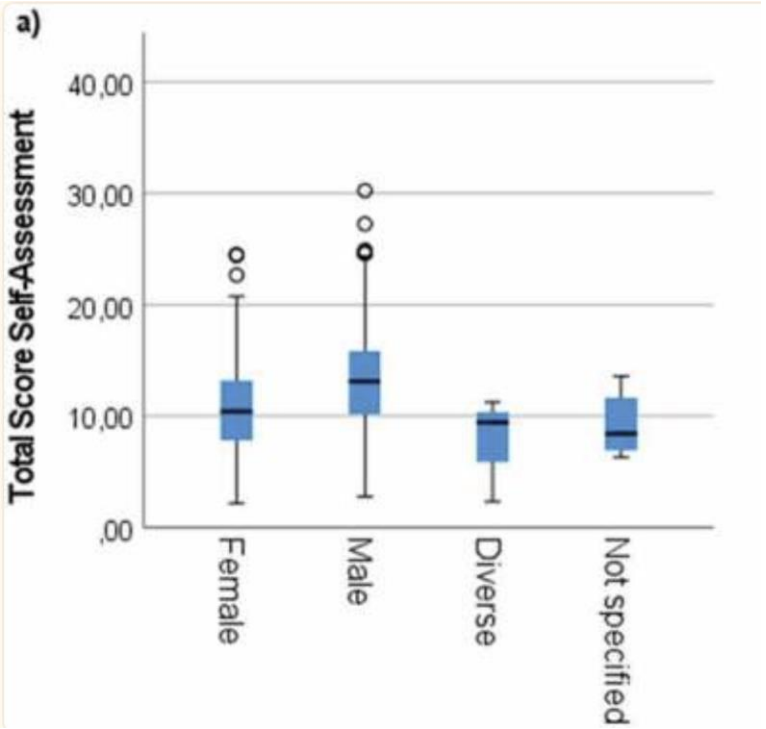
It could be an issue for people with low technical skills

Physicians' technical skills as a barrier

Med Educ Online. 2022; 27(1): 2114851.
Published online 2022 Aug 28. doi: 10.1080/10872981.2022.2114851
PMCID: PMC9423824
PMID: 36036219

Digital health understanding and preparedness of medical students: a cross-sectional study

Martin Baumgartner, Christoph Sauer, Kathrin Blagec, and Georg Dorffner



Digital health understanding and preparedness of medical students: a cross-sectional study - PMC (nih.gov)

“Distance” could be an problem

But we can mitigate it with:

- Hot line
- Nurses and physicians availability
- Visits to patient home if he wants
- Telemetry
- Digital assistants

Design the clinical trial with the patient

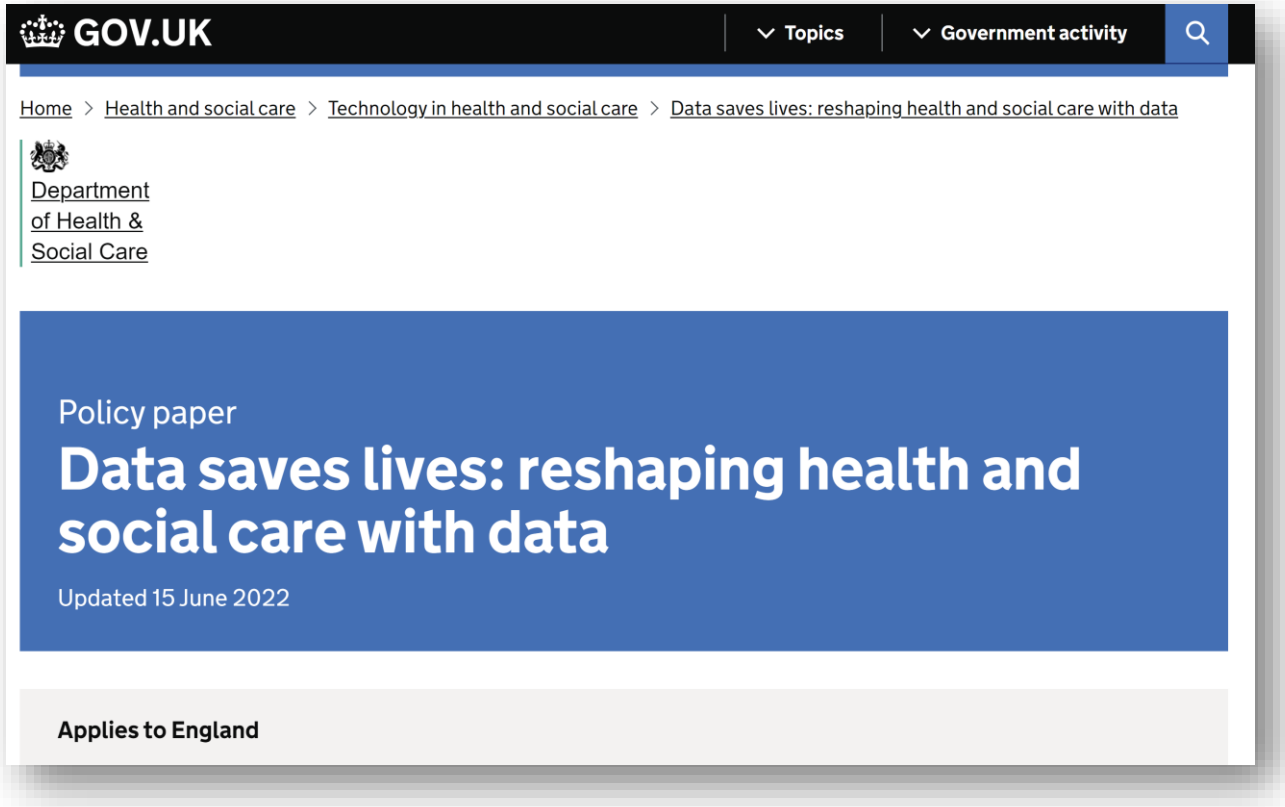
- Apps for capturing data need UX experts and co-design with patients
- Design depends of the severity of the condition
- Severity drives motivation
- Don’t forget the human touch

What about data privacy?

- Decentralization means patient data transfer and custody on digital assets
- This is underestimated for most of projects
- But it’s easy to solve with a PIA (Privacy Impact Assessment) done by a data security company

Patient is the owner of the data
Data should go back to patient after CT is done

- My son participated in two clinical trials
- Any data was shared with me
- Any data was reused
- My work has been lost



[Data saves lives: reshaping health and social care with data - GOV.UK (www.gov.uk)]

julian.isla@dravet.eu

Thanks

dravet europe
Dravet Syndrome European Federation

Investigator Perspective

Presented by Dr. Filippo Pieralli (University Hospital Careggi)

Investigator perspective

Filippo Pieralli

University Hospital Careggi – Firenze – Italy

Department of Clinical Research - FADOI

Patients' opinion about DCTs

66%

Of patients has to travel for more than 30 minutes to reach the Center where clinical research is made

73%

Of patients where very interested to be reached at home for visits during a clinical trial

50-61%

Of patients deemed very important the following possibilities:

- Biological sample collection at hone (50%);
- Avalaibility of apps (53%) on mobile for rial execution;
- Delivery of medications at home (61%)

SURVEY 1: CISCRP 2019 PERCEPTIONS AND INSIGHTS STUDY; SURVEY 2: CISCRP SURVEY : PATIENT INSIGHTS ON FACTORS IMPACTING CLINICAL TRIAL PARTICIPATION DURING THE COVID-19 PANDEMIC (AUG20))
Survey 3: BTKi survey May2020; Survey 4 : SCRS WHITE PAPER (site survey): Impact Assessment of Decentralized Clinical Trials (May/20)

Clinicians' opinion on DCTs

70%

Of clinicians wants the activation of more rapid procedures

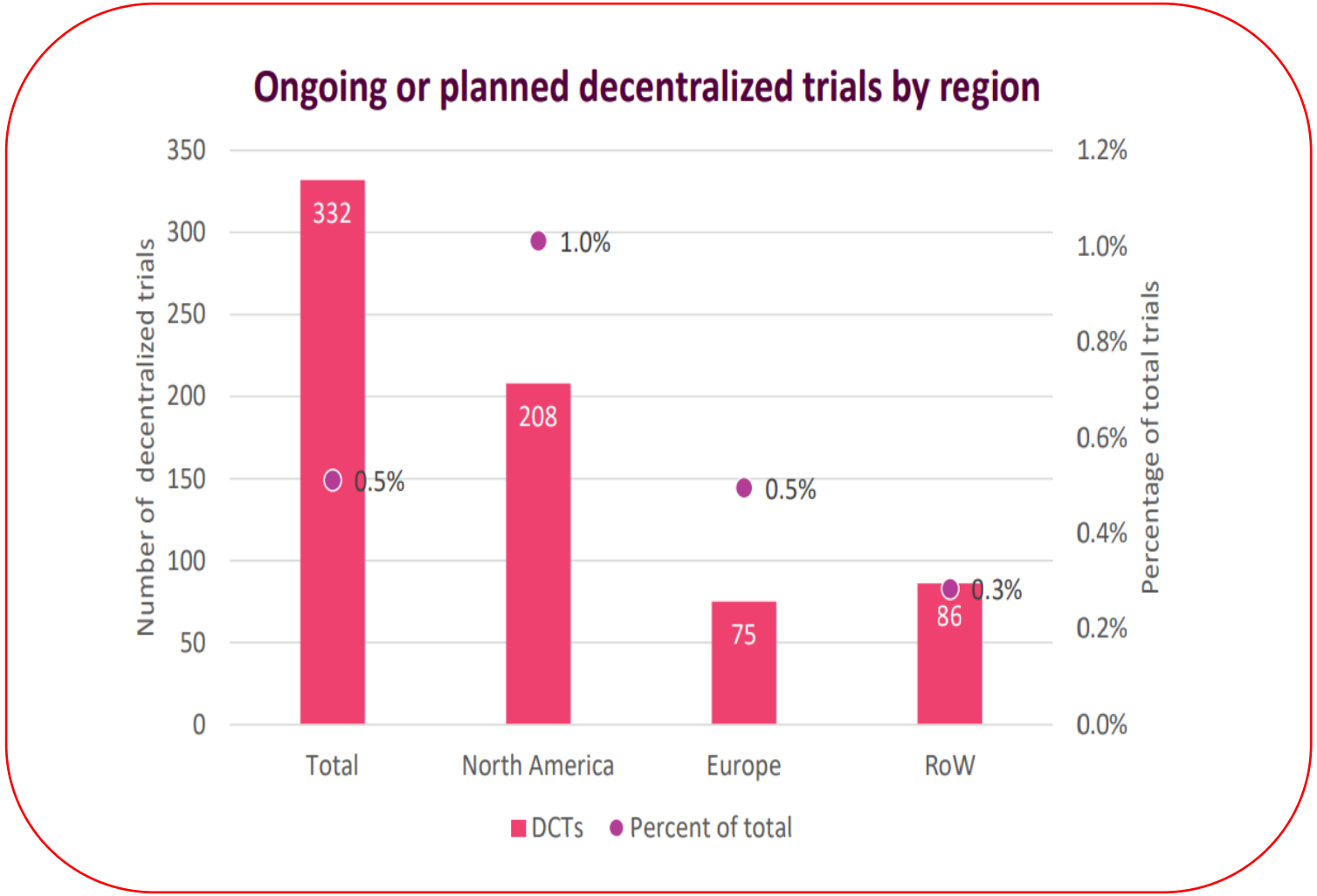
79%

Of clinicians refers that patients waste more than 25% of their in activities not directly related to the trial or study.

69%

Of clinicians with expertise on DCTs reports very good retention of patients in the study, even more than traditional clinical trials, and high compliance to procedures.

Decentralized Clinical Trials (DCT): a snapshot



Source: https://pharmaintelligence.informa.com/resources/product-content/2020/07/20/10/43/sitecore/shell/~/media/informa-shop-window/pharma/2020/covid-24-campaign/slides/decentralized-clinical-trials-in-2020.pdf

My POV

- DCTs are quite a new modality to conduct trials with respect to traditional research and they are still a little percentage of the volume of studies and trials.
- They are an evolving and important step in the field of clinical research.
- They are particularly useful for patients with rare diseases, for whom the referral center can be very distant and not easily reacheable, for patients with limited mobility and/or functional disability and that are not autonomous.

My POV

- When dialing with DCTs, technology is a key point.
- Technology must be a facilitator and not a barrier between patient and investigators/health care providers.
- We need strong involvement of patients since the first phases of the planning of the trial project to enhance trust and needings.
- Trasparency for CI disclosure (Investigators) is a key point to reinforce trust and good execution of the studies.

My POV

- Digital health technology gives us a continuous and extensive flow of data from patients with respect to traditional on site clinical visits.
- This offers (many) opportunities and threats:
  - More data = more info
  - Continuous flow data = possibilities to intercept earlier signs of alarm
  - Overload for the investigators ?

My POV

- Finding a balance between patient needs [e.g. for unscheduled access or visits or meeting (or simply questions)], and healthcare professionals workflow.
- Identifiyng adequate and secure (certificated?) electronic platforms is needed.
- Adequate training on platforms for investigators and patients is crucial before starting the trial.

Panel discussion to explore patient and investigator site perspective

Panellists:

Sally Hofmeister (World Duchenne Organization)

Julián Isla (COMP and Dravet Europe)

Aisling Walsh (EFCNI)

Mira Zuidgeest (trials@home)

Dr Filippo Pieralli (University Hospital Careggi)

Dr Francisco Bautista (Princess Máxima)

Moderator:

Kasper Bendix Johnsen (Danish National Center for Ethics)

Closing remarks

Peter Arlett, EMA

Greet Musch*, FAMHP/CTCG*

End Plenary Session

Appendix

ACRO DCT Working Party Objectives

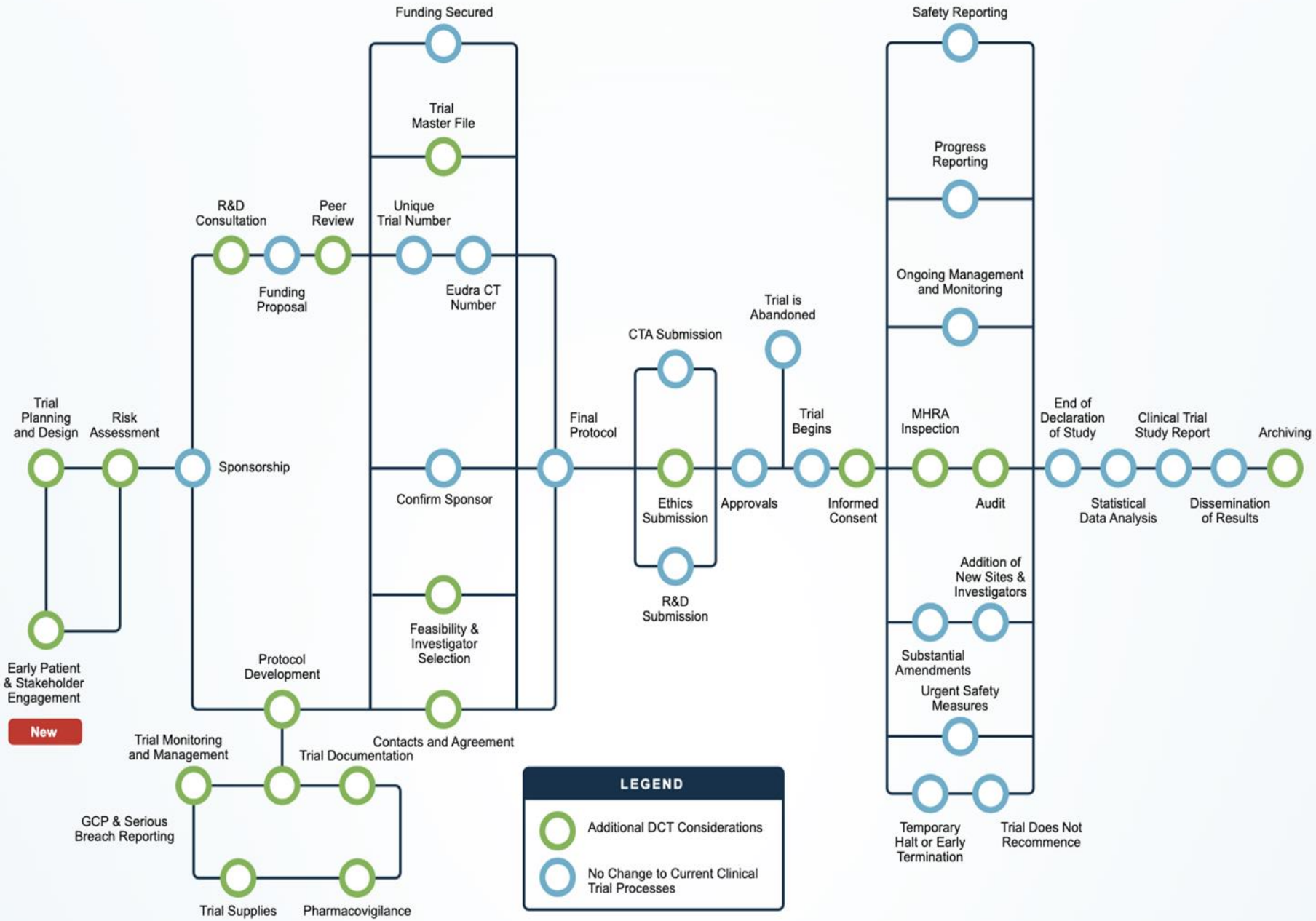
Scope:

- Landscape scanning to identify and address barriers to adoption of DCTs
- Short-term (6-month) projects to create deliverables to advance DCTs – for use by all stakeholders and available on ACRO website
- Engagement and informal listening sessions with stakeholders to share and discuss ACRO projects (industry, sites, regulators, ethics committees, patients)

How?

- ACRO DCT committee established in 2019
- Committee meets weekly so that all ACRO members can collaboratively develop and complete DCT projects
- Q1 & Q2 each year focuses on project development
- Q3 and Q4 each focuses on project dissemination via informal listening sessions and feedback with stakeholders

ACRO
CELEBRATING 20 YEARS



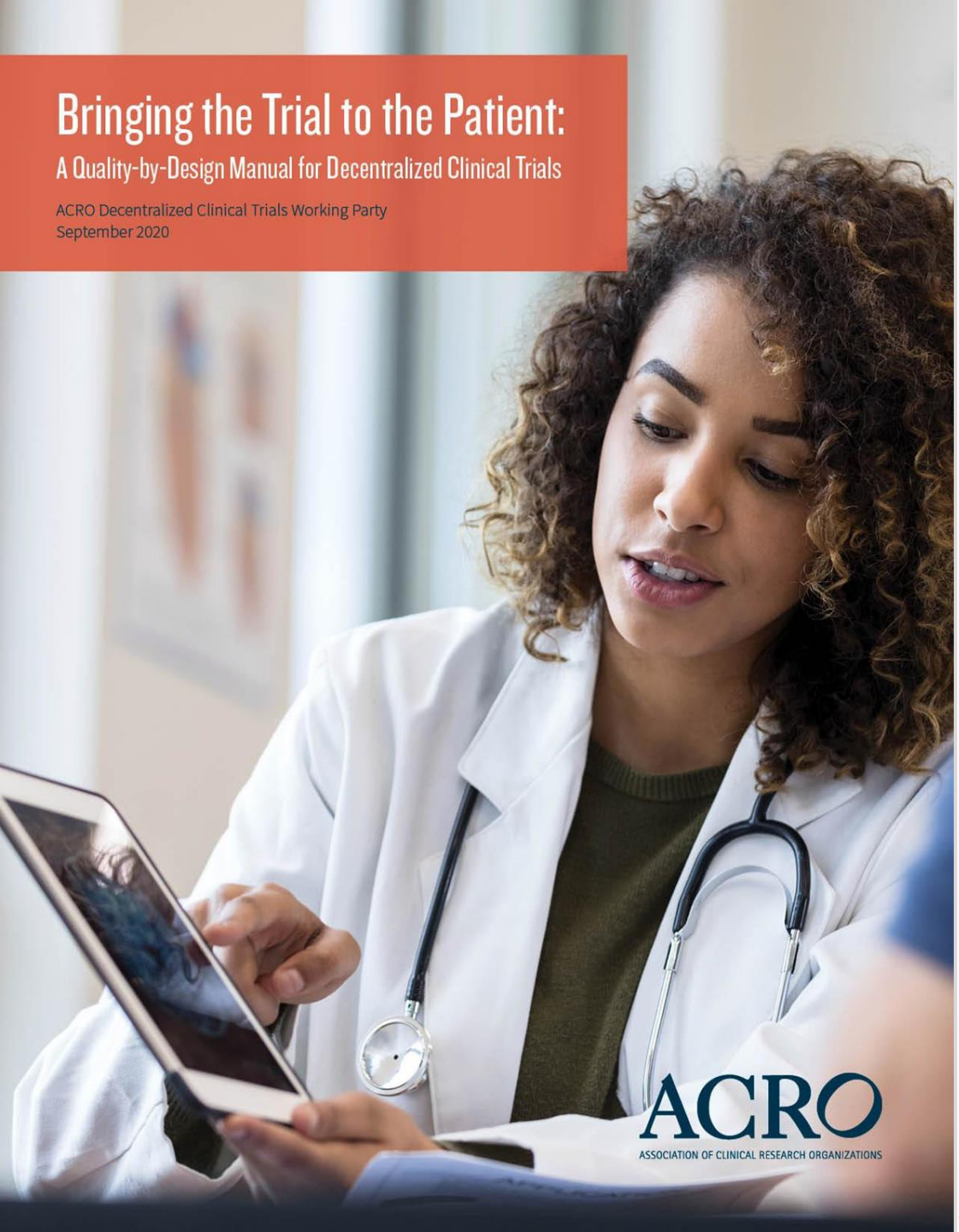
Adapted from the National Institute for Health Research (NIHR) Clinical Trials Toolkit Routemap

ACRO DCT TOOLKIT

Started with...

an in depth analysis of the End-to-end CT Process

The team analysed what is different with a DCT compared to a traditional paper based trial?



Quality by Design DCT Manuals

ACRO

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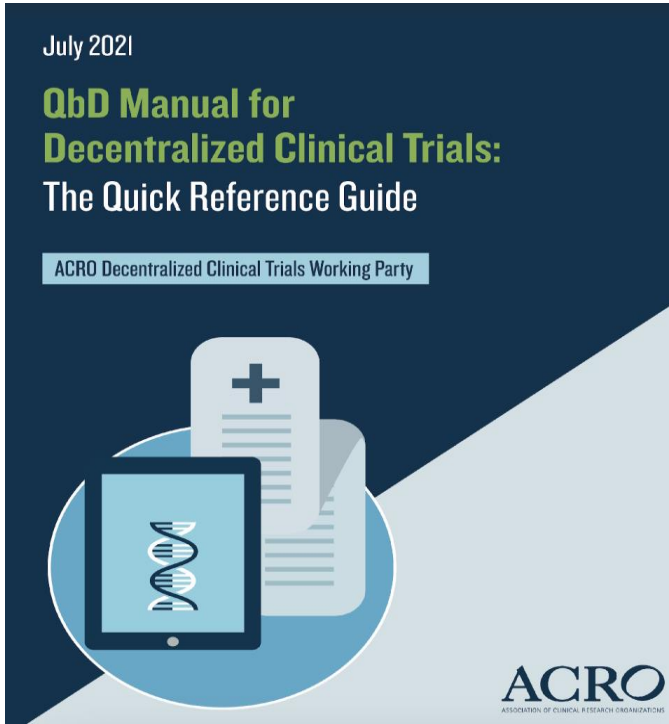
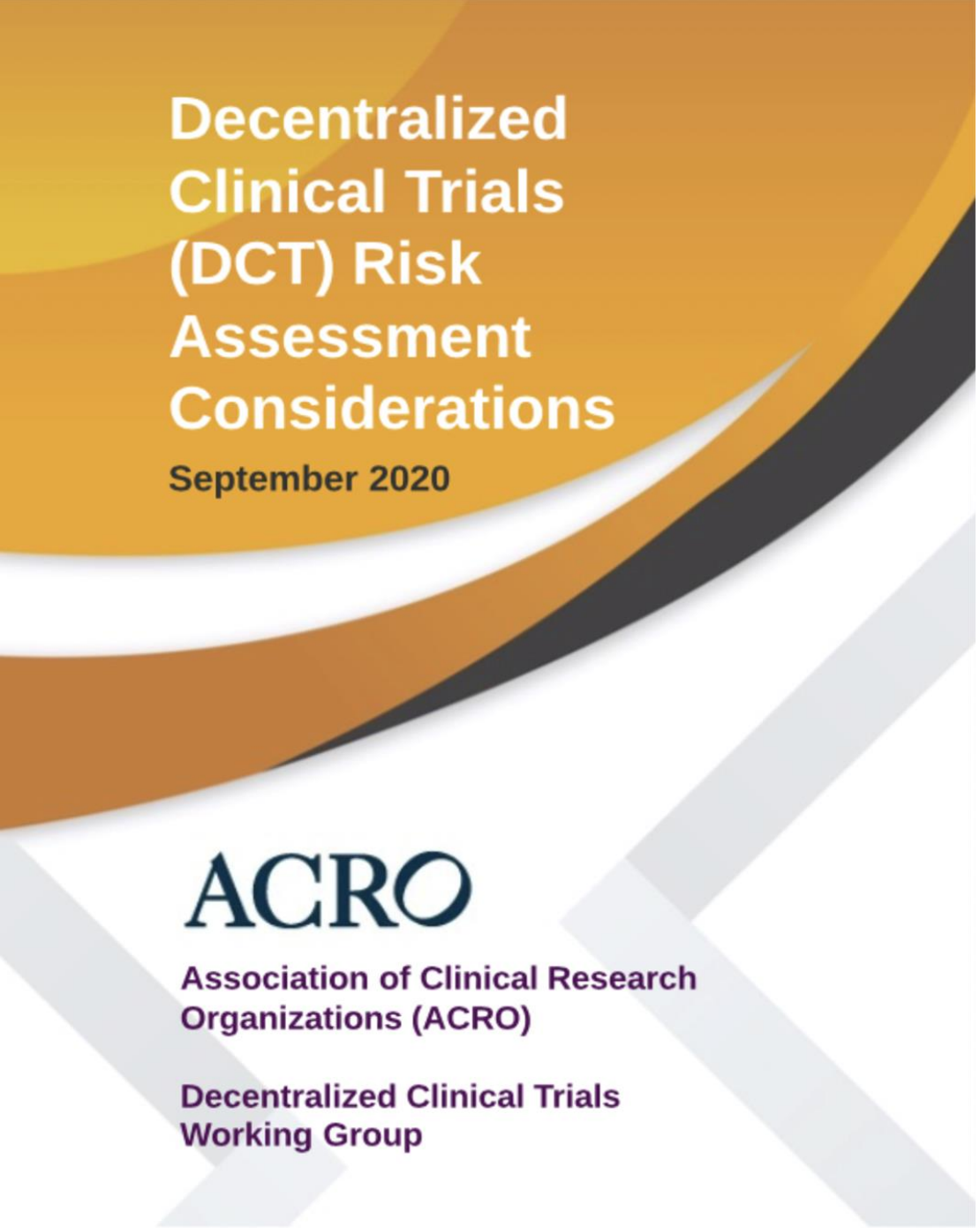


ACRO DCT TOOLKIT

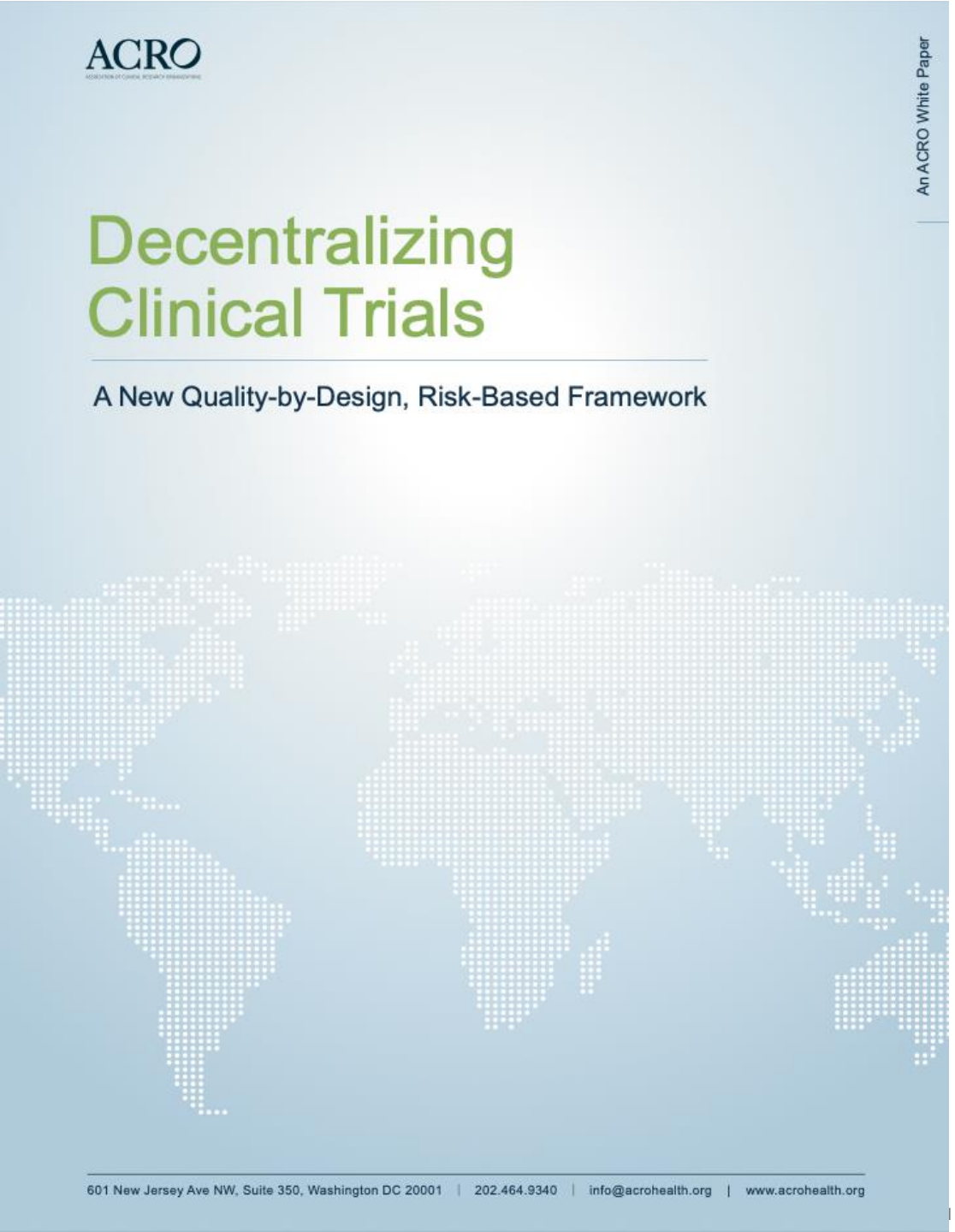
as public by the European Medicines Agency



DCT Risk Assessment

- Template to systematically raise questions that facilitate cross-functional discussion to identify and mitigate potential risk in decentralizing trial functions
- Complements a company's existing risk tools
- Leveraging Transcelerate Risk Assessment Categorisation Tool RACT

ACRO DCT TOOLKIT



DCT White Paper

- White paper to introduce the ACRO Toolkit
- Case studies and experiences running DCTs
- Feedback from authorities

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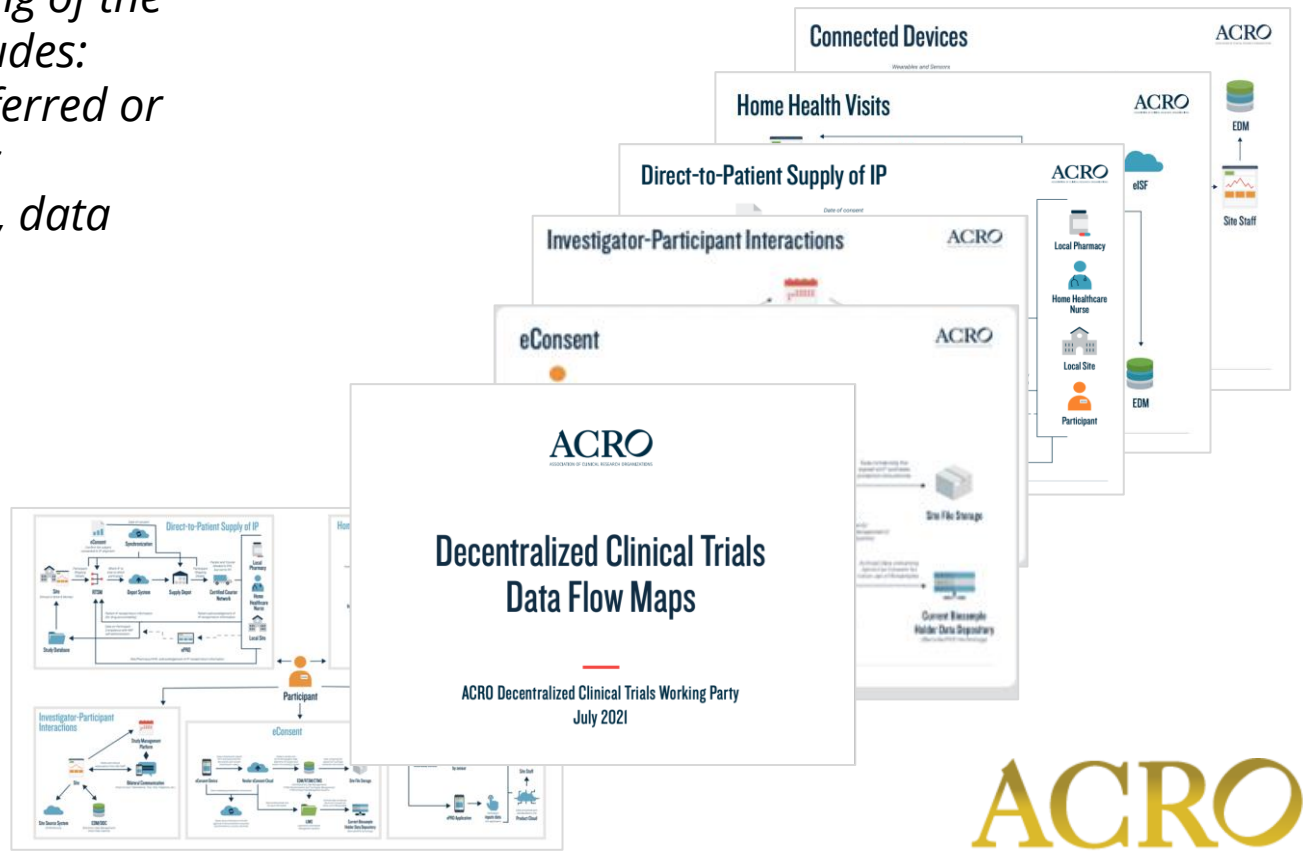
ACRO DCT TOOLKIT

DCT Data Strategy and Data Flow

'Sponsors/CROs need a detailed understanding of the data flow with a data flow diagram that includes: from whom and to whom the data are transferred or transmitted, including all third-party vendors contracted for data collection, data handling, data management and/or data processing'

MHRA/FDA GCP Symposium 2020



ACRO DCT TOOLKIT

Navigating DCT Transformational Change

Patients and Care Givers

Sites

Ethics Committees

Regulatory Authorities

Industry

Attitude and interest towards change?

Current levels of support?

Concerns and fears?

What are seen as key risks?

What the barriers that are foreseen?

What are the benefits to stakeholders?

What happens if we do not change?

Points of resistance to change?

To be published Q4 2022


Navigating Change during Rapid Transformation:
A Question-and-Answer Resource for Decentralized Clinical Trials

ACRO Decentralized Clinical Trials (DCT) Working Party

October 2022

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Common DCT Themes with stakeholders