UNDERSTANDING CLINICAL RESEARCH FRAMEWORK & CHALLENGES IN THE LIFE SCIENCES INDUSTRY

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Overview of Clinical Research

The Clinical Research Process

What is a Clinical Study?

- A clinical study involves research using human volunteers (also called participants).
- There are two main types of clinical studies: clinical trials (also called interventional studies) and observational studies.

Why Conduct a Clinical Study?

- In general, clinical studies are designed to add to medical knowledge related to the treatment, diagnosis, and prevention of diseases or conditions and to lead to approval of new drugs or devices (or new indications).

The Clinical Research Process

Who Conducts Clinical Studies?

- Led by a **principal investigator**, often a medical doctor or PhD.
- Can be **sponsored** by a variety of organizations—pharmaceutical companies, academic medical centers, voluntary groups, individual health care providers, or federal agencies.
- These studies can occur at hospitals, universities, doctor's offices, community clinics, etc.
- **Role of the Clinical Research Organization (CRO)** - A third party organization that manages trials and complex medical testing responsibilities.

How are Clinical Studies Regulated?

- **Food & Drug Administration (FDA) Regulations Relating to Good Clinical Practice and Clinical Trials**
- **Department of Health and Human Services (HHS)** - 45 CFR 46.116(h) or the revised "Common Rule"
- **National Institute of Health (NIH)** - ClinicalTrials.gov
- **Role of Institutional Review Boards (IRBs) -** Per FDA regulations, an IRB is a group that has been formally designated to review and monitor research involving human subjects.

Phases of a Clinical Trial

- Phase I trials-Testing of drug or treatment on small group for first time. Evaluating safety, safe dosage range and identify side effects.
- Phase II trials-Experimental drug or treatment given to a larger group of people to determine effectiveness and further evaluate its safety.
- Phase III trials- Experimental drug or treatment is given to large groups of people. Researchers confirm its effectiveness, monitor side effects, compare to commonly used treatments, collect information on how the experimental drug or treatment can be used safely.
- Phase IV trials-These are post-marketing studies which are conducted after drug or treatment is approved by FDA. Provide additional information including the treatment of drug or treatment's risks, benefits and best use.

Informed Consent

To make an informed decision about whether to participate or not in a clinical trial, people need to be informed about:

- What the research involves and what is being studied
- How the protocol (plan of research) works
- What risks or discomforts they may experience
- Participation being a voluntary decision

Before enrolling in a clinical trial, the FDA requires certain basic elements of consent to be included in an informed consent document:

- Statement explaining that the study involves research
- Explanation of the purposes of the research
- Expected length of time for participation
- Description of all the procedures that will be completed, including information about all experimental procedures
- Description of any known predictable risks
- Possible discomforts that could occur
- Any possible benefits that may be expected
- Information about any alternative procedures or treatment
- Confidentiality statement, how records will be kept, who might access the data, e.g., the Sponsor, vendors working on the study and the possibility that the FDA may inspect the records

For research involving more than minimal risk, the FDA requires information including:

- Explanation as to whether any compensation or medical treatments are available if injury occurs
- What the risks consist of, or where more information may be found
- Questions about the research
- Research subjects' rights
- Injury related to the clinical trial
- Research subject participation is voluntary
- Research subjects have the right to refuse treatment
- Research subjects may choose to stop participation at any time
- Contact information

A potential research subject must have an opportunity to:

- Read and understand the consent document
- Ask any questions about anything they do not understand

Clinical Trial Protocol

What is it?

- A clinical study is conducted according to a research plan known as the "protocol."

Why?

- The protocol is designed to answer questions relating to the research and safeguard the health of participants and sets out how the research will be measured and length of the study.

What information does it contain?

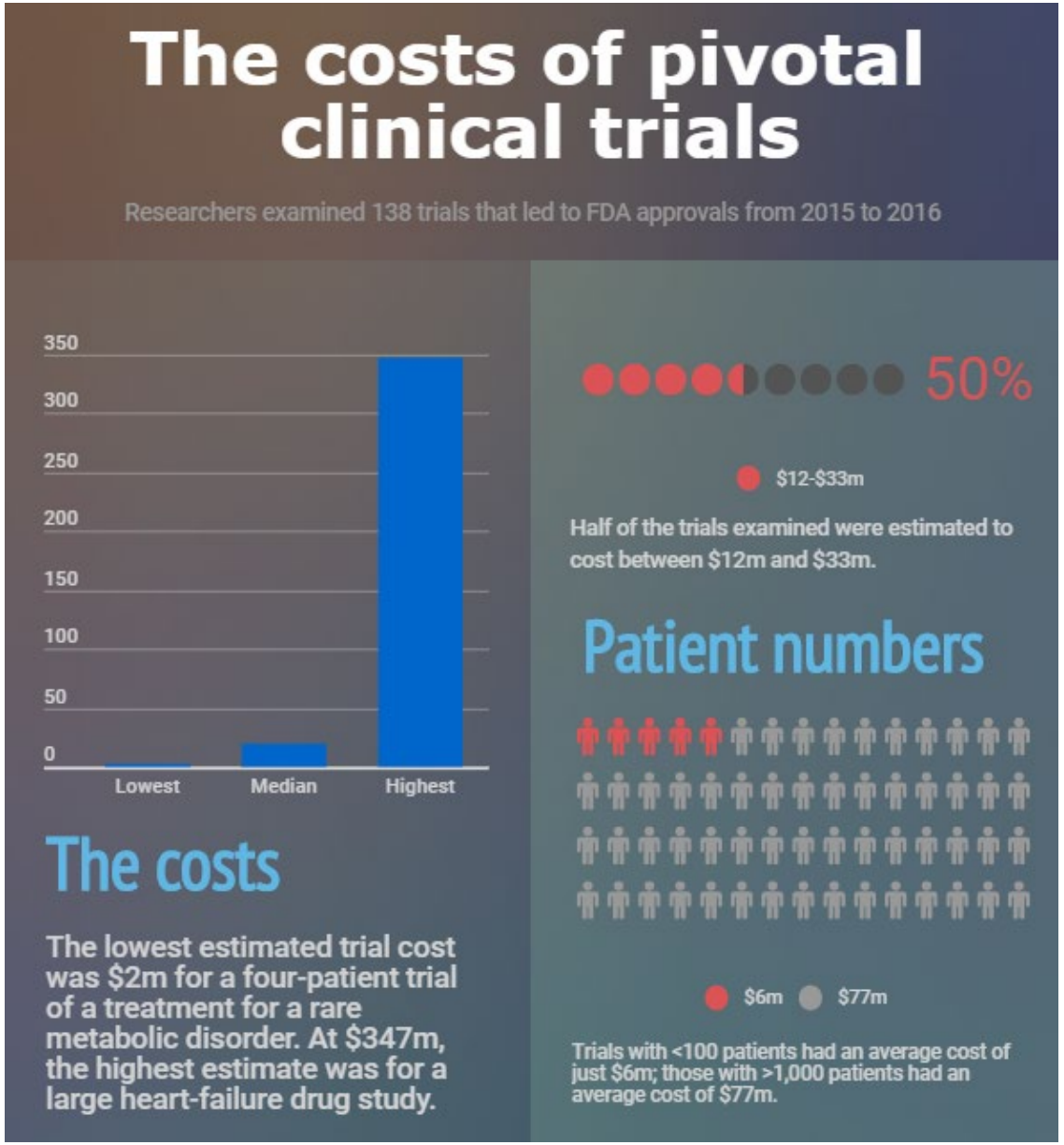
- The reason for conducting the study
- Who may participate in the study (eligibility criteria)
- The number of participants needed
- The schedule of tests, procedures, or drugs and their dosages
- The length of the study
- What information will be gathered about he participants

Challenges in Clinical Research

Challenges: Time & Money

What factors affect the cost of a clinical trial?

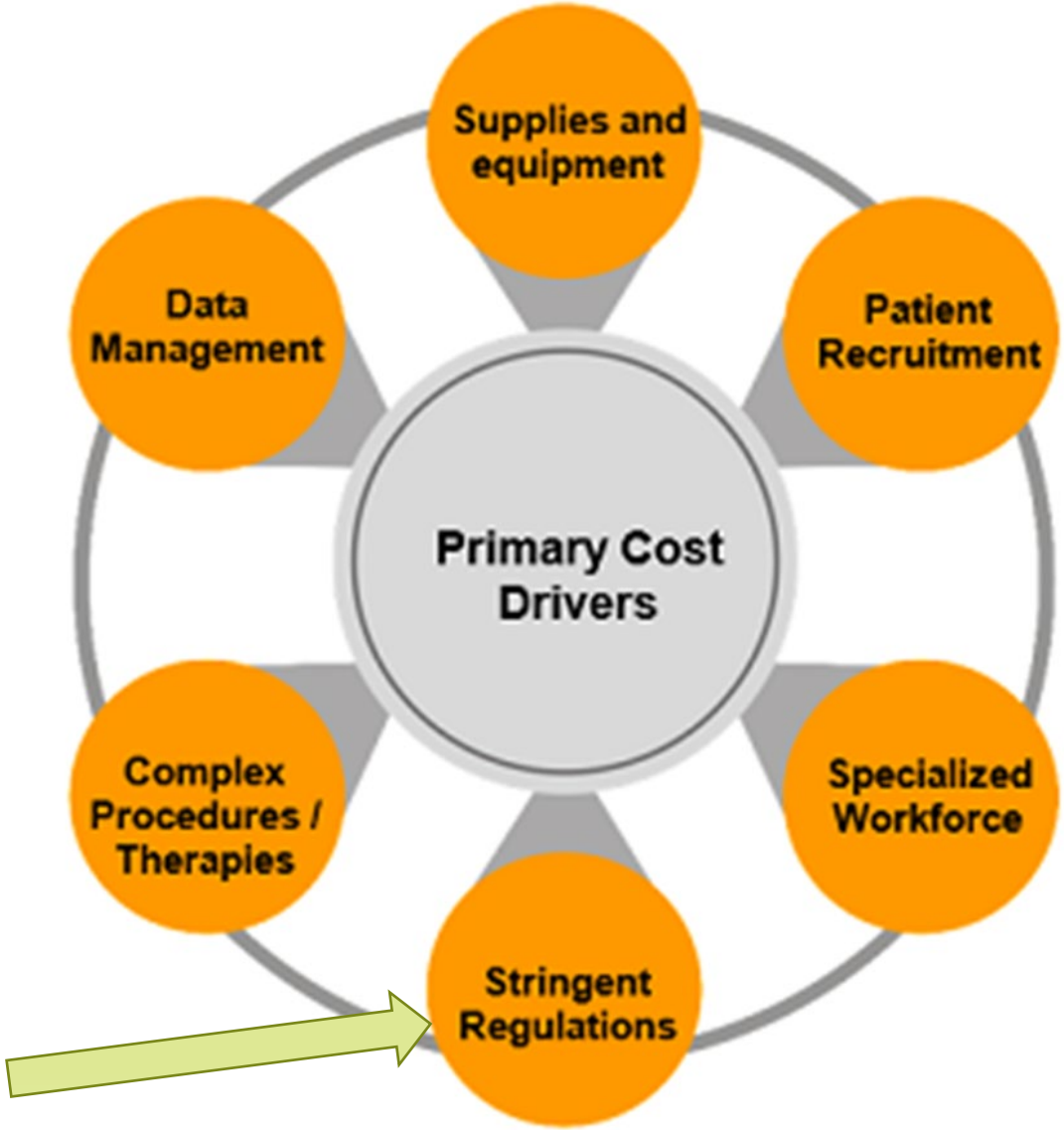
- Supplies and equipment costs
- Extended timelines
- Increased regulations
- Monitoring complexities
- Patient recruitment intricacies
- Workforce competence
- Data collection and synergy complexities



Clinical trial cost is a fraction of the drug development bill (outsourcing-pharma.com)

Challenges: Regulatory Process

- The regulations for good clinical practice and clinical trials are stringent, complex and costly to comply with.
- *See* Regulations: Good Clinical Practice and Clinical Trials | FDA for a detailed list with links to FDA regulations governing human subject protection and the conduct of clinical trials.



Getting A Handle On Clinical Trial Costs (clinicalleader.com)

Impact of COVID-19 on Clinical Research

Impact of COVID-19 on Clinical Research

- Covid-19 lockdown affected clinical trial sites.
- Remote clinical trial sites were implemented.
- Use of Emergency Use Authorization by FDA.
- Operation WARP Speed-Priority was put on increased development time.

COVID-19 Impact

National Cancer Institute @theNCI · Oct 5

From #COVID19 vaccines to cancer drugs, #RNA-based therapies have benefited millions. A new study showing the ability of #AI to predict 3D shapes of RNA molecules marks an advance that will expand their use in healthcare, writes @NIHDirector. directorsblog.nih.gov/2021/09/23/art...

Deloitte Health Care @DeloitteHealth · Oct 16

COVID-19 has forced a global rethink on how new drugs should be brought to market. As the landscape evolves, how could the way stakeholders engage with each other change? Register now @FTLiveHealth

Deloitte Health Care @DeloitteHealth · Oct 20

How can life sciences companies glean #ProductInsights from their vast quantities of patient and health care provider data? #AI and #MachineLearning could be the key. More in our new report.

Alliance for Clinical Trials in Oncology Retweeted

National Cancer Institute @theNCI

Researchers now hope to use the #telemedicine experience gained during the pandemic to improve cancer clinical trials moving forward. go.usa.gov/xMqW4 #NCIFuture #NothingWillStopUs

COVID-19 Impact

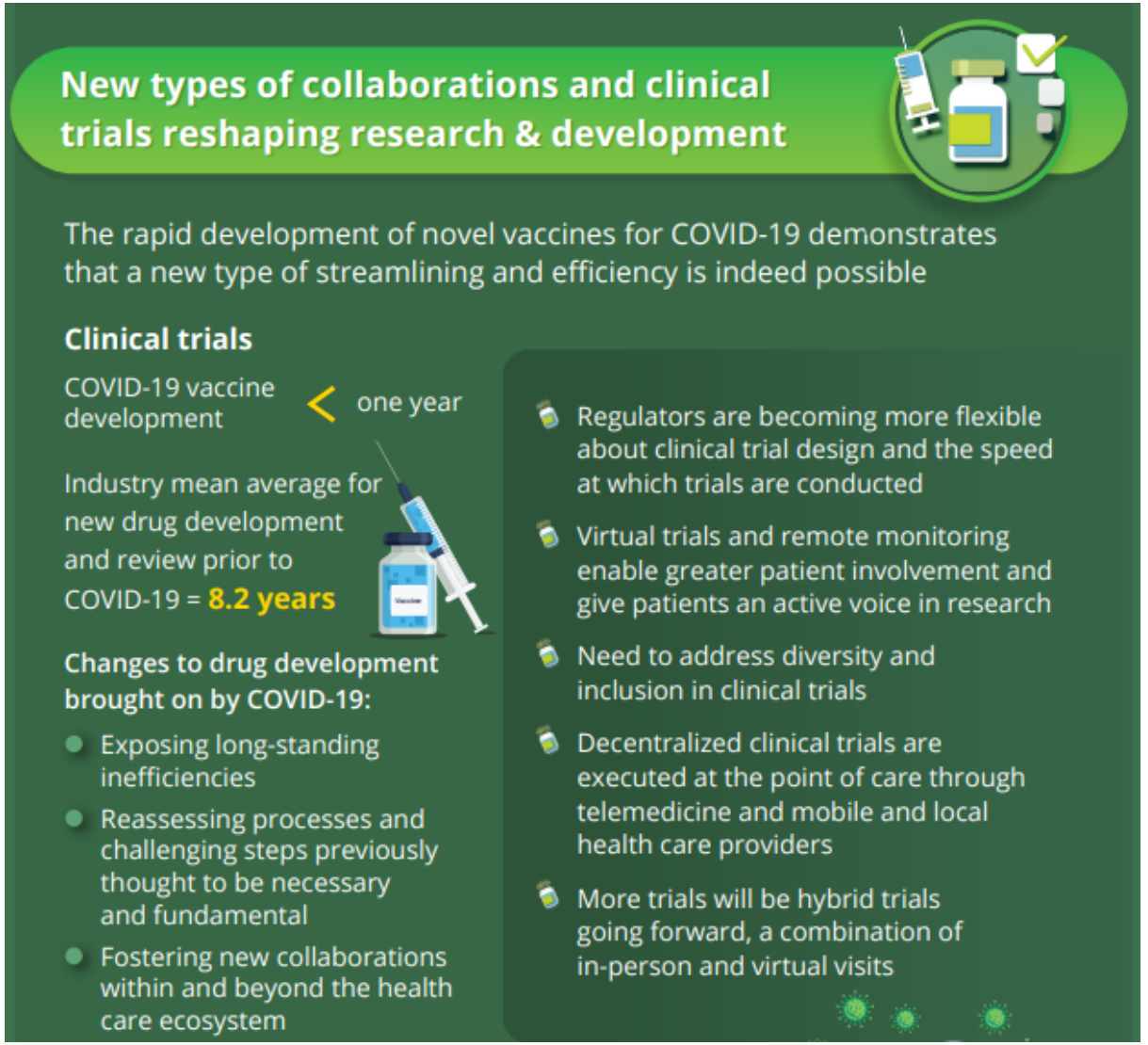
For pharma organizations, we see three potential horizons.

During the immediate crisis	As the healthcare system shifts to recovery		Settling into a next normal	
				
Resolve	Resilience	Return	Reimagination	Reform

McKinsey & Company

COVID-19 Vaccine Development

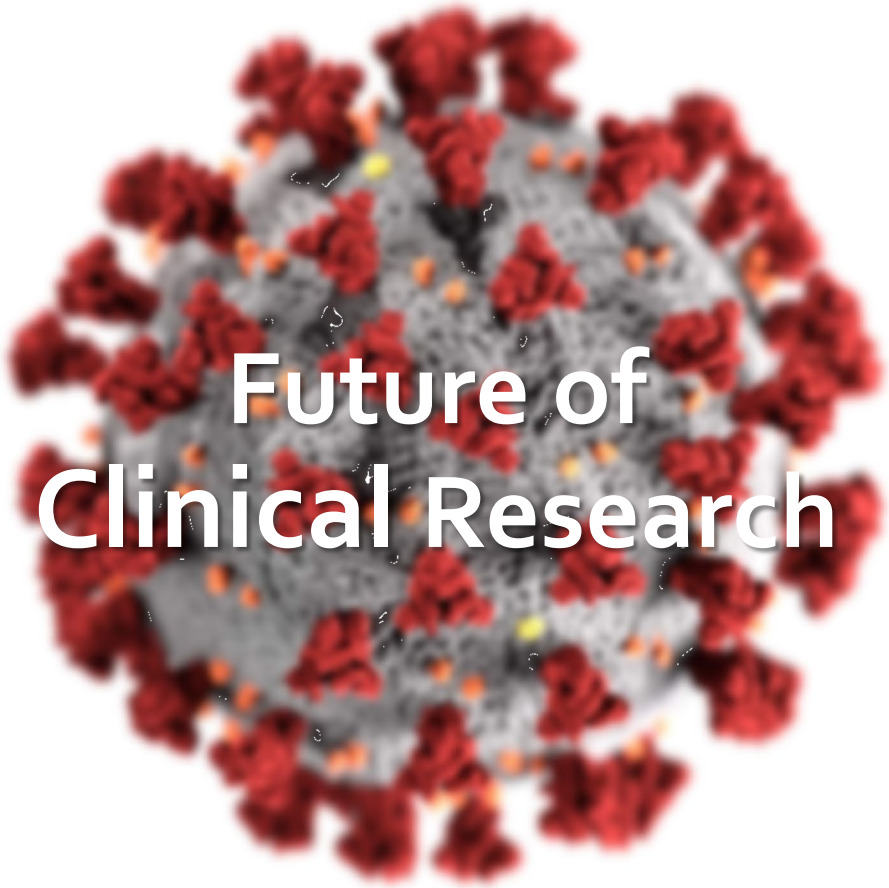
- Breaking Barriers: Biopharma companies are now reassessing their previous process and considering various new strategies–innovative partnerships, AI, virtual trials, remote monitoring, telemedicine, mobile health care, etc.



2021 Global life sciences sector outlook | Deloitte

COVID-19 Therapeutic Treatments

- Researchers are working at record speed to find the best ways to treat and prevent COVID-19–thousands of clinical trials for therapies are taking place across the world.
- NIH has set up a partnership among government, industry, and university researchers to identify promising drugs and other treatments.
- Current approaches to COVID-19 therapies generally fall into 2 categories: antivirals and immune modulators.



Future of Clinical Research

Future of Clinical Research

- Increased pressure to accelerate the clinical research process
- Balance between finding an endpoint in a trial that is clinically meaningful to the FDA but which also beneficial to patients
- Increased use of more decentralized clinical research
- Use Artificial Intelligence in clinical trials
- Increased use of digital therapeutics / wearable technology and continuous patient data
- Increase use of precision medicine driven clinical trials
- Increased use of patient advocates / representatives of people who are living with disease being studied
- Addressing how to manage large amounts of data

Questions?

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Sources

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- https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/institutional-review-boards-irbs-and-protection-human-subjects-clinical-trials
- Informed Consent for Clinical Trials | FDA
- 2021 Global life sciences sector outlook | Deloitte
- Getting A Handle On Clinical Trial Costs (clinicalleader.com)
- Regulations: Good Clinical Practice and Clinical Trials | FDA
- Everything you need to know about the COVID-19 therapy trials - The Pharmaceutical Journal (pharmaceutical-journal.com)