Learning from the COVID-19 Pandemic:
Expanding Opportunities for Robust Evidence Generation

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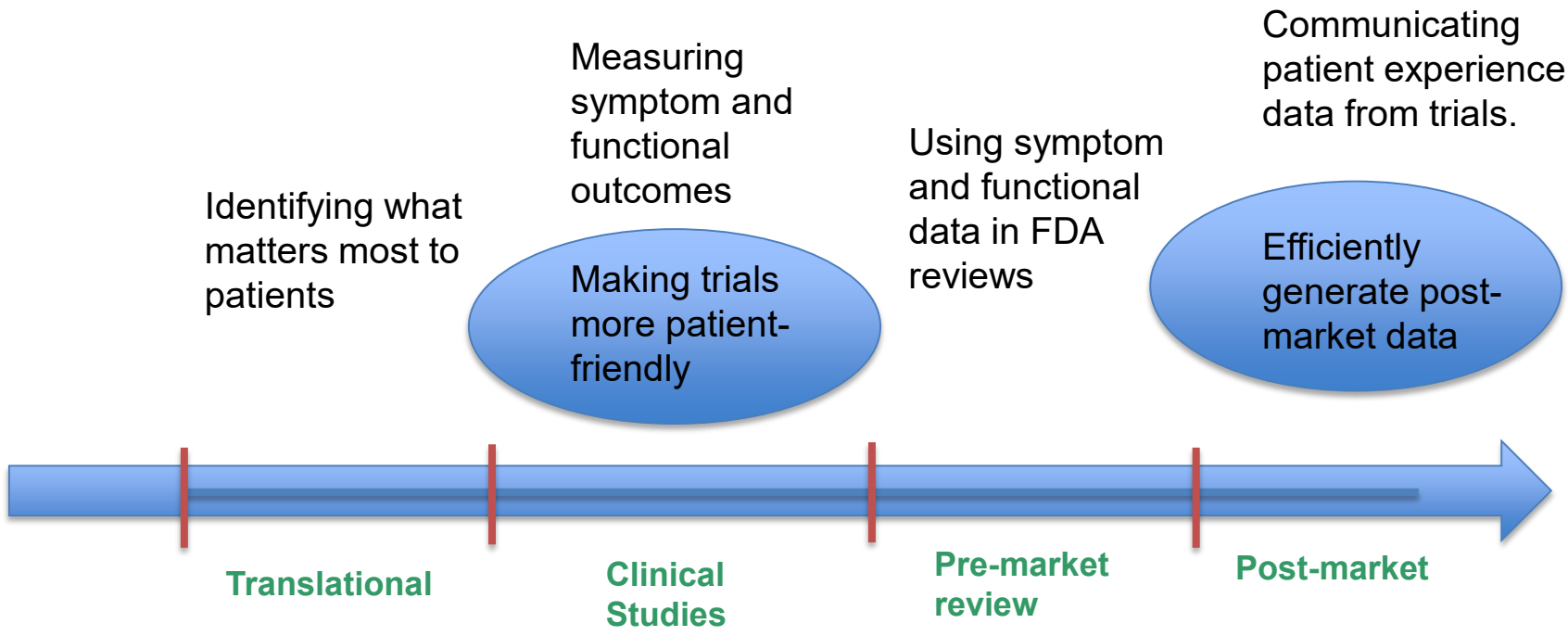
Disclosures

- I have no financial relationships to disclose

Patient-focused drug development (PFDD)

In many ways COVID-19 has accelerated our efforts to put the focus on patients when generating evidence for drug development.



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Making trials/ evidence generation more patient friendly

- Advance digital health technology and patient generated data (ePRO, wearable devices, etc.)
- Decentralize Clinical Trials (DCT)
- Foster learning healthcare systems to facilitate prospective pragmatic trials

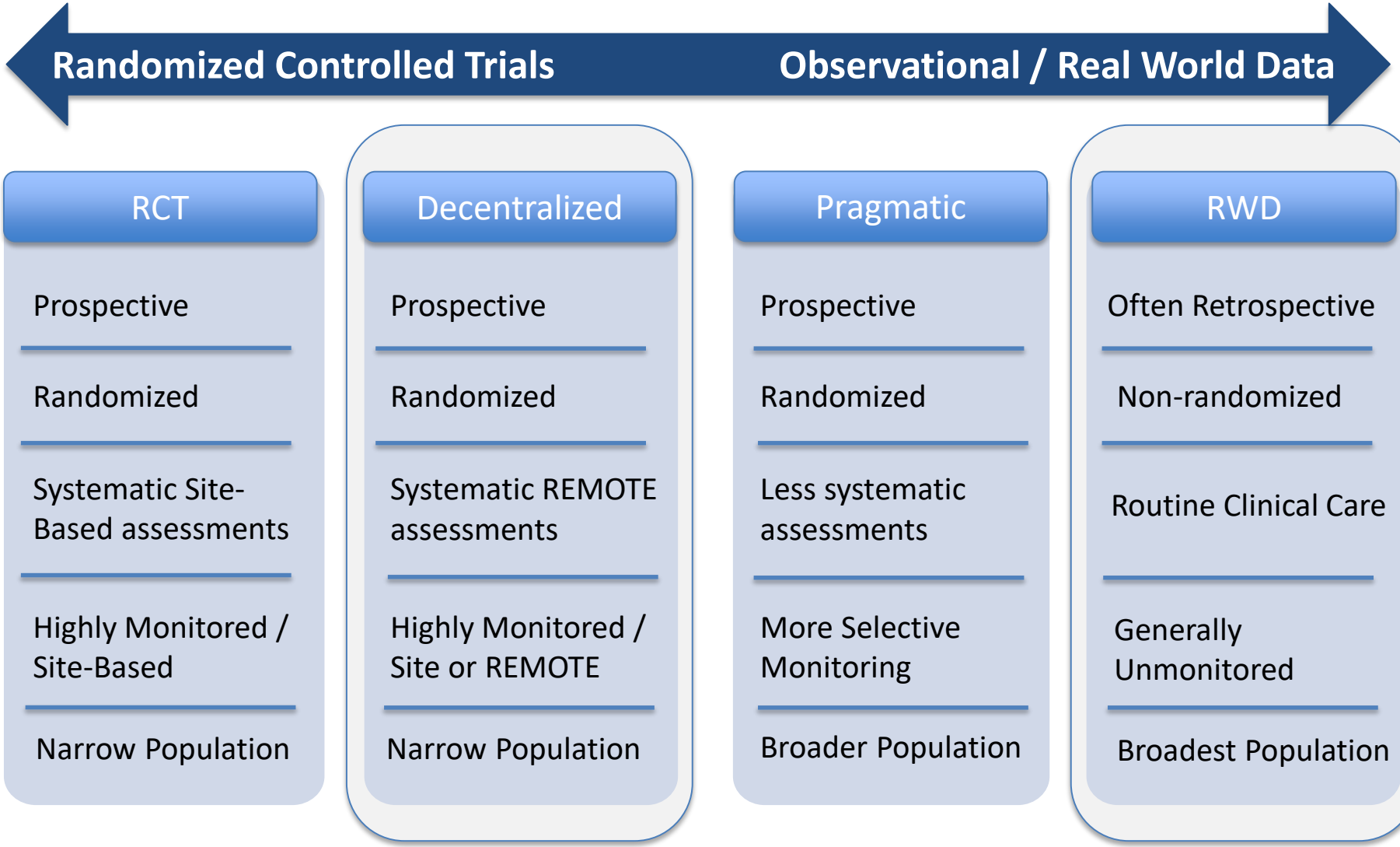
- ↑ Trial access
- ↑ Breadth of outcomes
- ↓ Disparities
- ↓ Development times
- ↓ Cost

Generating Evidence From Clinical Trials and Clinical Care

RCT	Decentralized	Pragmatic	RWD
Prospective	Prospective	Prospective	Often Retrospective
Randomized	Randomized	Randomized	Non-randomized
Systematic Site-Based assessments	Systematic REMOTE assessments	Less systematic assessments	Routine Clinical Care
Highly Monitored / Site-Based	Highly Monitored / Site or REMOTE	More Selective Monitoring	Generally Unmonitored
Narrow Population	Narrow Population	Broader Population	Broadest Population

Acceptability of various evidence sources is context dependent

Blurring the lines between Clinical Trials and Clinical Care



Acceptability of various evidence sources is context dependent

Advancing Decentralized Trials:

Industry is deploying aspects of decentralized trials to respond to the COVID-19 pandemic-

What can we learn about effects on data quality of:

1. Remote clinic visits (telemedicine)
2. Remote labs
3. Remote imaging
4. Remote administration of IP
5. Remote site monitoring

https://www.fda.gov/about-fda/oncology-center-excellence/advancing-oncology-decentralized-trials

Characterizing Data Quality from Remote Assessments

- **Issue:** No standard approach to how COVID-19 modifications are/will be identified in clinical trial datasets submitted to FDA

- **Standardize trial data submitted to FDA**
  - Dataset to describe permitted trial modifications
  - Flag individual assessments that were conducted remotely
  - Flag imaging assessments as interpretable (Y/N)

https://www.fda.gov/about-fda/oncology-center-excellence/advancing-oncology-decentralized-trials

Advancing Real World Evidence in Oncology

FDA Oncology Real World Evidence Program
- Initial Programmatic Goals

1. Foster development of consistent **terminology** (Glossary)
2. Standardize RWD characterization and establish **quality** metrics
3. Advance real-world **endpoints** like response rate (rwRR)
4. Support efforts to improve **source data** (Common Data Elements)

Collaborate with FDA's broader RWE Program, professional societies (ASH, ASCO), data vendors, regulated industry, technology companies, non-profit organizations and others interested in moving real-world data into real-world evidence.

https://www.fda.gov/about-fda/oncology-center-excellence/oncology-real-world-evidence-program

Conclusion

- Learn from efficiencies and novel approaches to evidence generation during COVID-19

- Prioritize modifications that benefit patients and provide efficiencies while maintaining patient safety and minimizing effects on data quality

- FDA's Oncology Center will continue efforts to advance trial efficiencies, real-world data, patient-generated data, digital health technology and decentralized trials.