



# Clinical Trials in Rare Disease Populations

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# Disclaimer

The opinions expressed here belong solely to me and do not reflect the views of my employer or past employers.



# Agenda

Introduction to the Case Study

The Approach

What did not work?

Key takeaway

Q&A



# Case Study

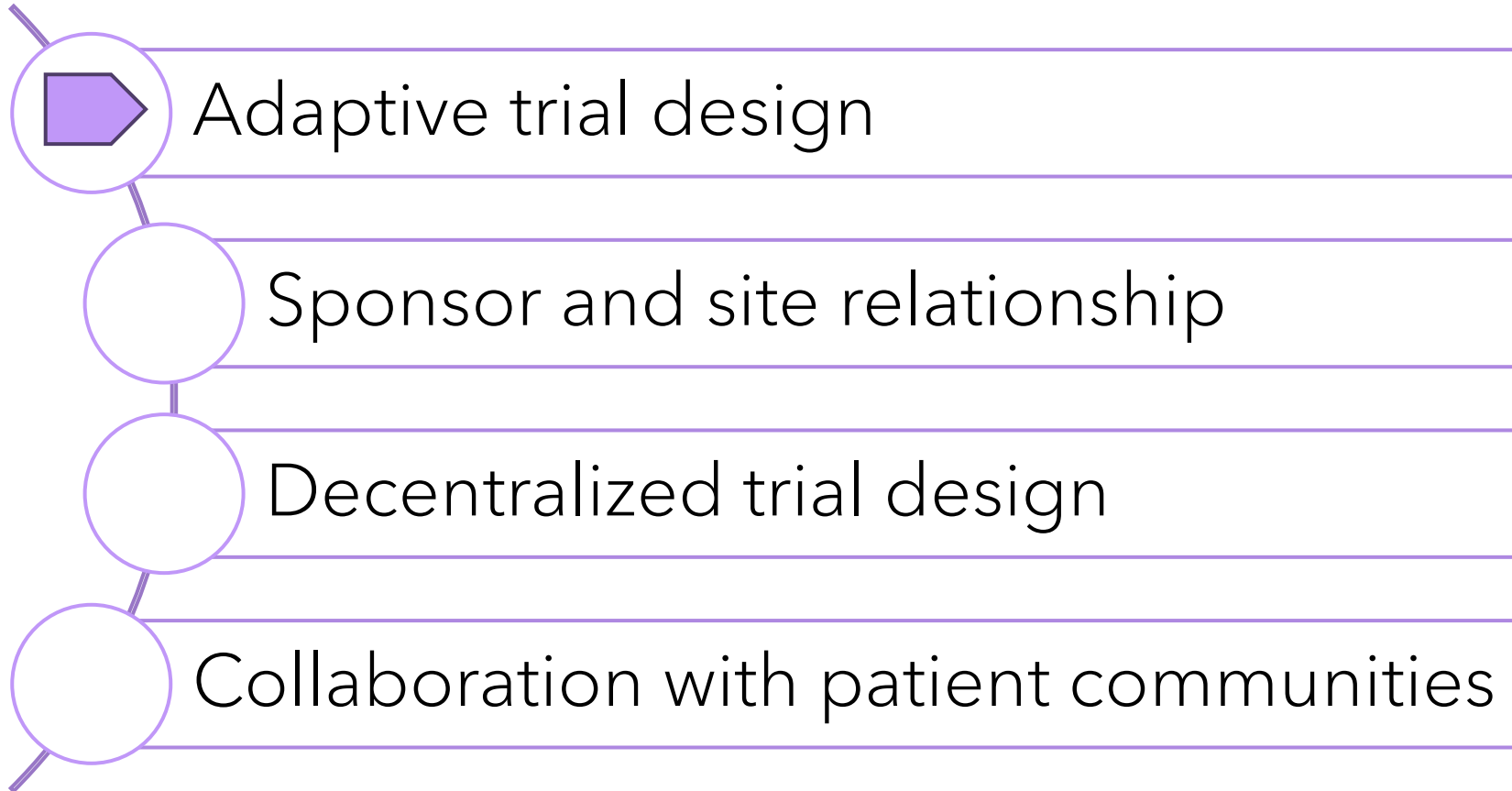
A rare and underdiagnosed disease with limited understanding by general medical practitioners.

Across the globe, varying diagnosis framework by specialists.

No treatment options besides symptoms management.


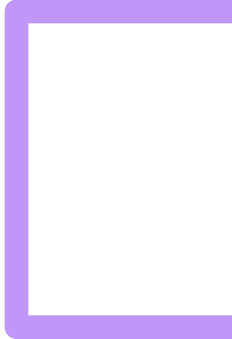
Potentially unreliable estimates of patient numbers.

# The Approach

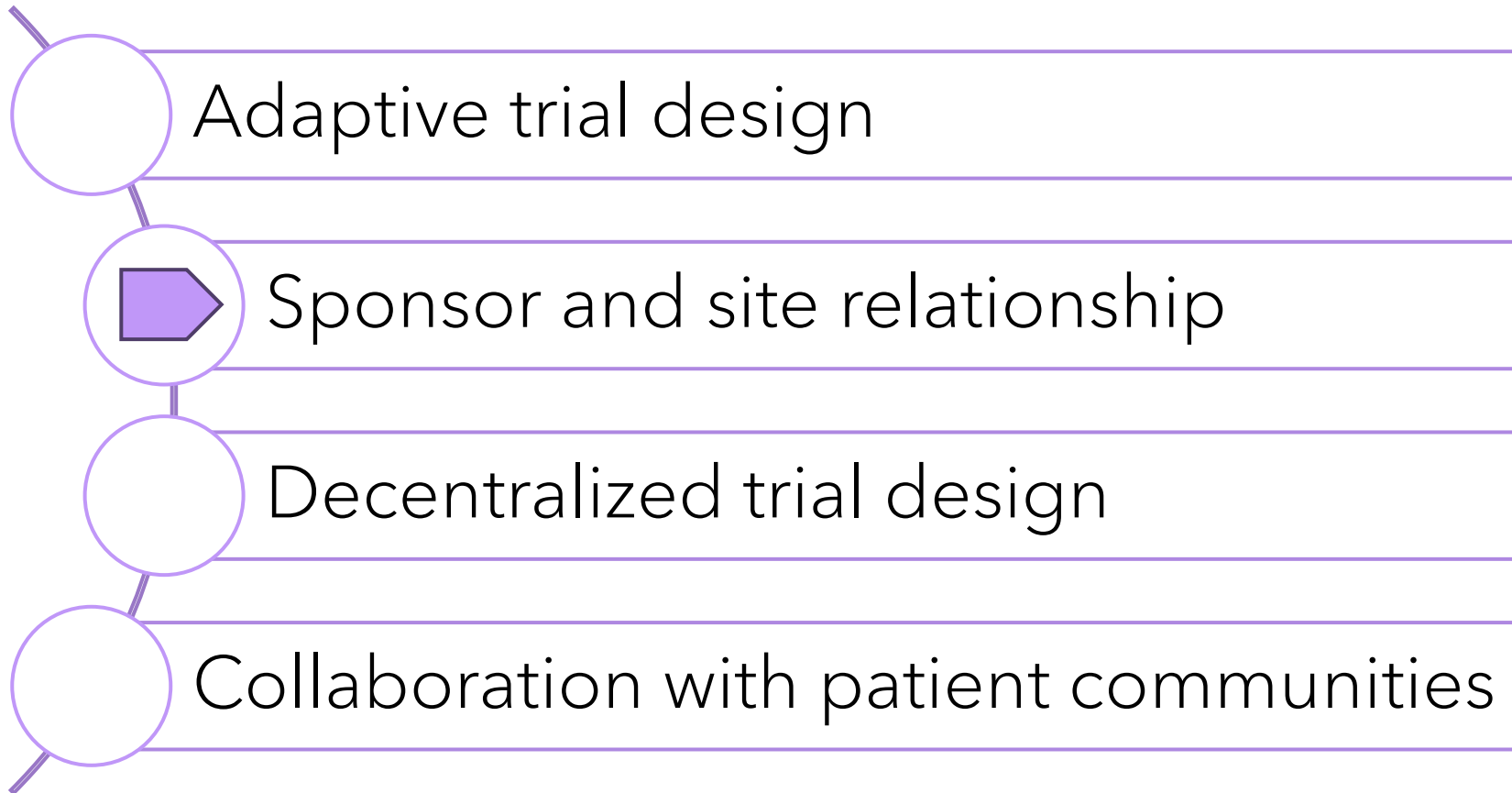




# Adaptive trial design


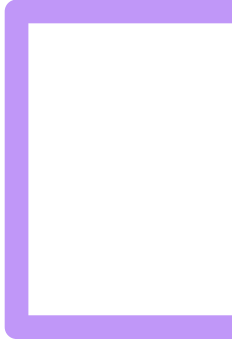
- Early engagement
    - Meet with agencies to align on regulatory expectations and trial design
    - Involve industry experts and sites in the trial design
  - Steering Committee of experts
    - Act as champion of the study and enhance site engagement
    - Advise on standardizing inclusion criteria, given variable diagnosis framework
  - Use real-world data to enhance study feasibility
  - Design with emerging data in mind
    - Planned interim analysis and potential earlier New Drug Application (NDA) filing
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# The Approach



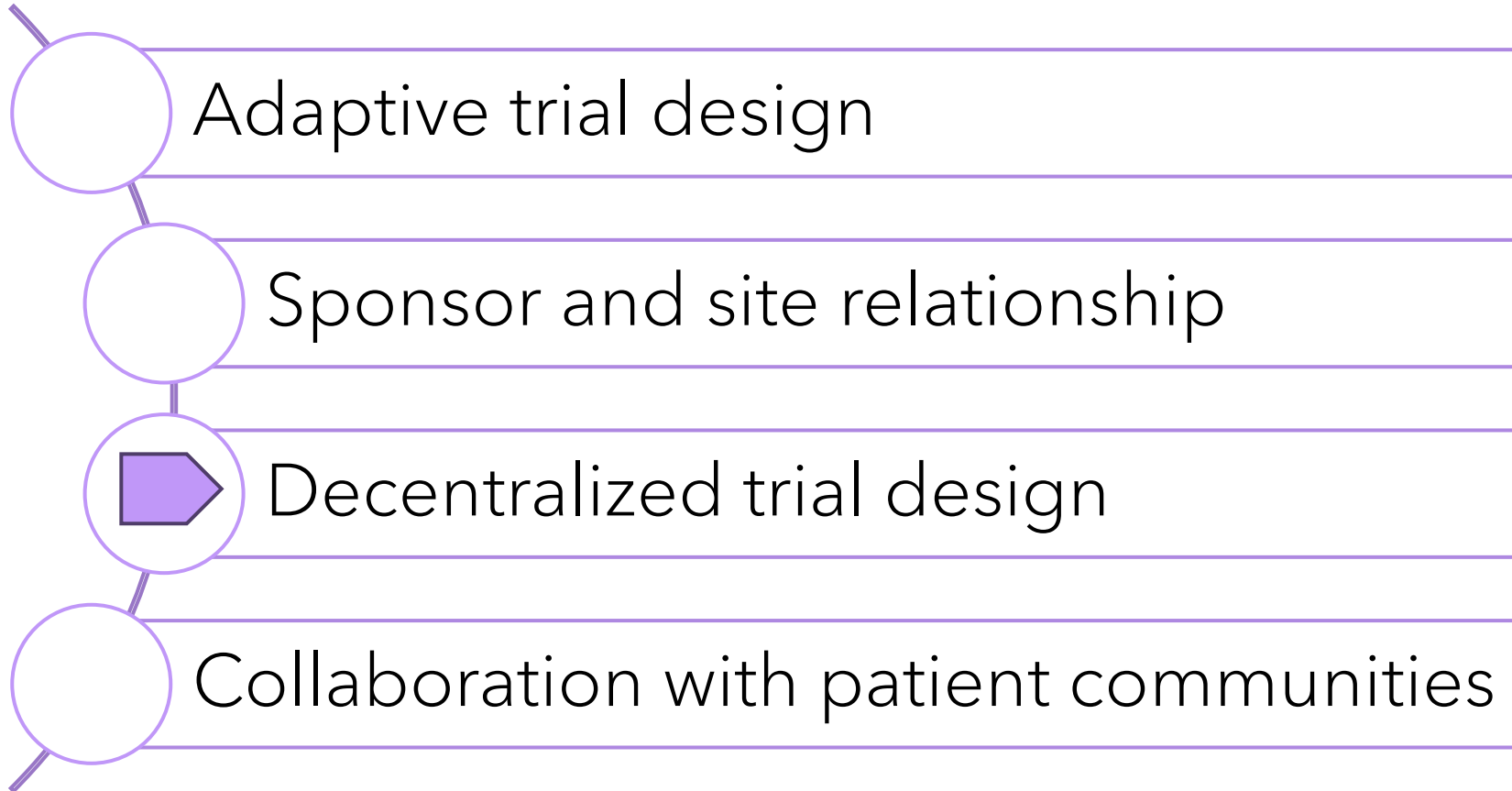


# Sponsor and site relationship

- Collaborate with Clinical Research Organization (CRO) to manage site relationship, and build into communication plan
  - Engagement through Face-to-Face visits, teleconferences, and academic/industry conferences
  - Sites are your partners! Prioritize problem-solving and on-time payment
  - Keep sites in the loop e.g. newsletters, study enrollment dashboards, Thank you cards
  - Make (budget) room for creativity
  - Seek ongoing feedback
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# The Approach



# Decentralized trial design

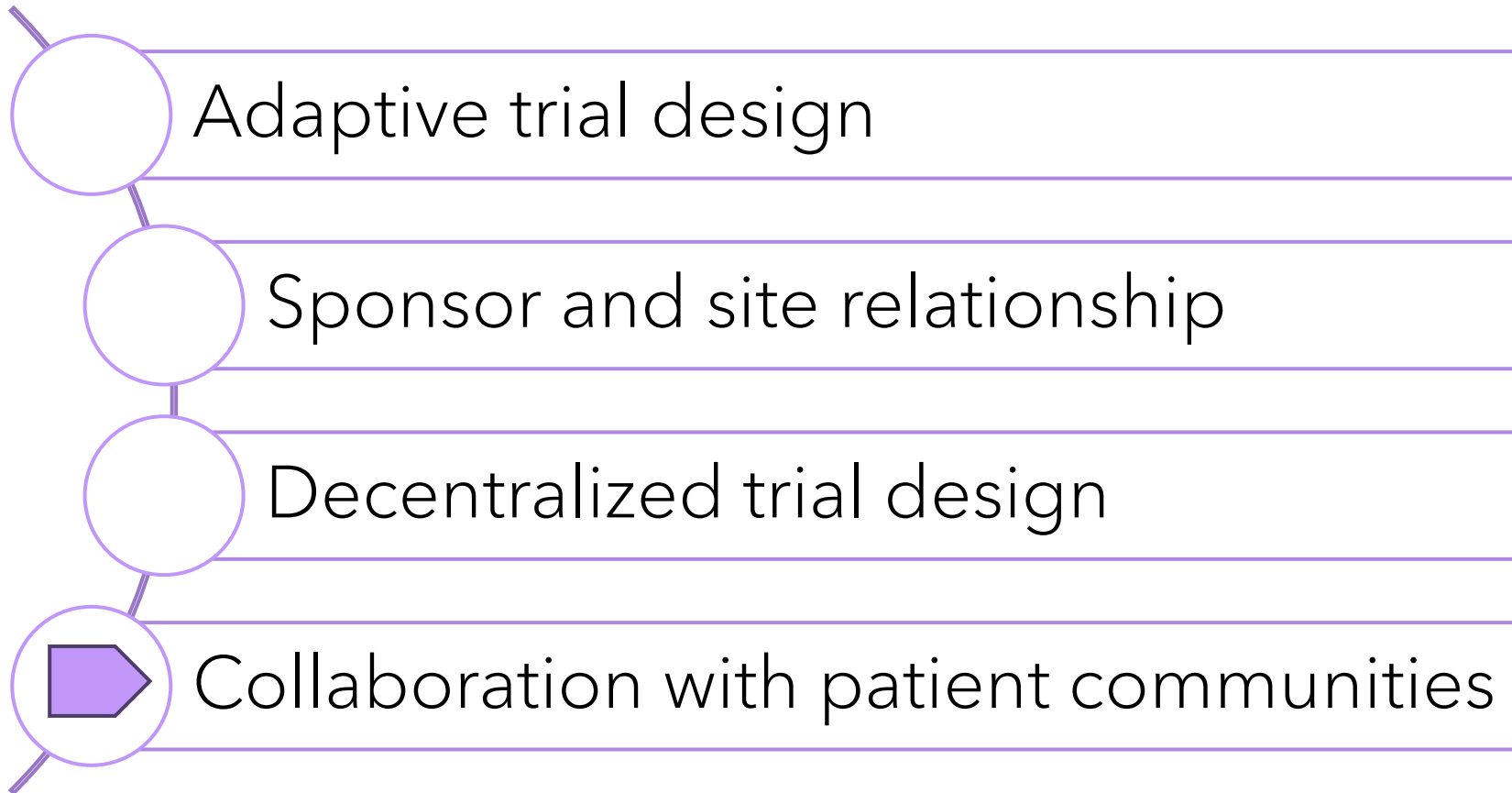
Assume 24 months treatment period

Leverage technology to support assessments

Consider the potential time-savings for a participant... they can add up.

Assessment	Frequency	Time Spent
Consent	Once	30 mins
Quality of life questionnaire	Q4W	20 mins
Dosing reminders	Daily	10 mins
Follow up visit	Once	2 hours

# The Approach



# Collaboration with patient communities

## Patient advocacy groups

- Support their mission
- Contribute to newsletters
- Co-organize patient focus group

## Patient communities

- Sponsor “Patient Day”
- Sponsor forum discussions
- Support community events

# What did not work?

Site identification with real-world data

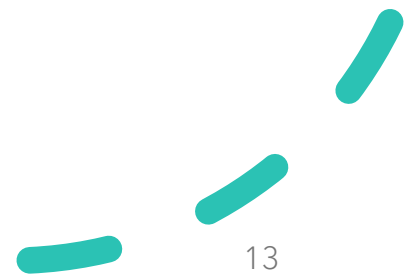
- Confidentiality made it difficult to ascertain potential patient numbers

Centralized communication platforms with sites

- Need to consider site's preferred way of working

Medical science liaisons

- The investigators and site staff were already the experts in their fields! We were duplicating effort



# Key takeaway

Be flexible and nimble





# Q&A Session

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Thank you

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