Clinical Trials 101: Why They Are Important and What You Should Know

Celebrating a Second Chance at Life Survivorship Symposium

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Clinical Trials 101:
Why They are Important and What You Should Know

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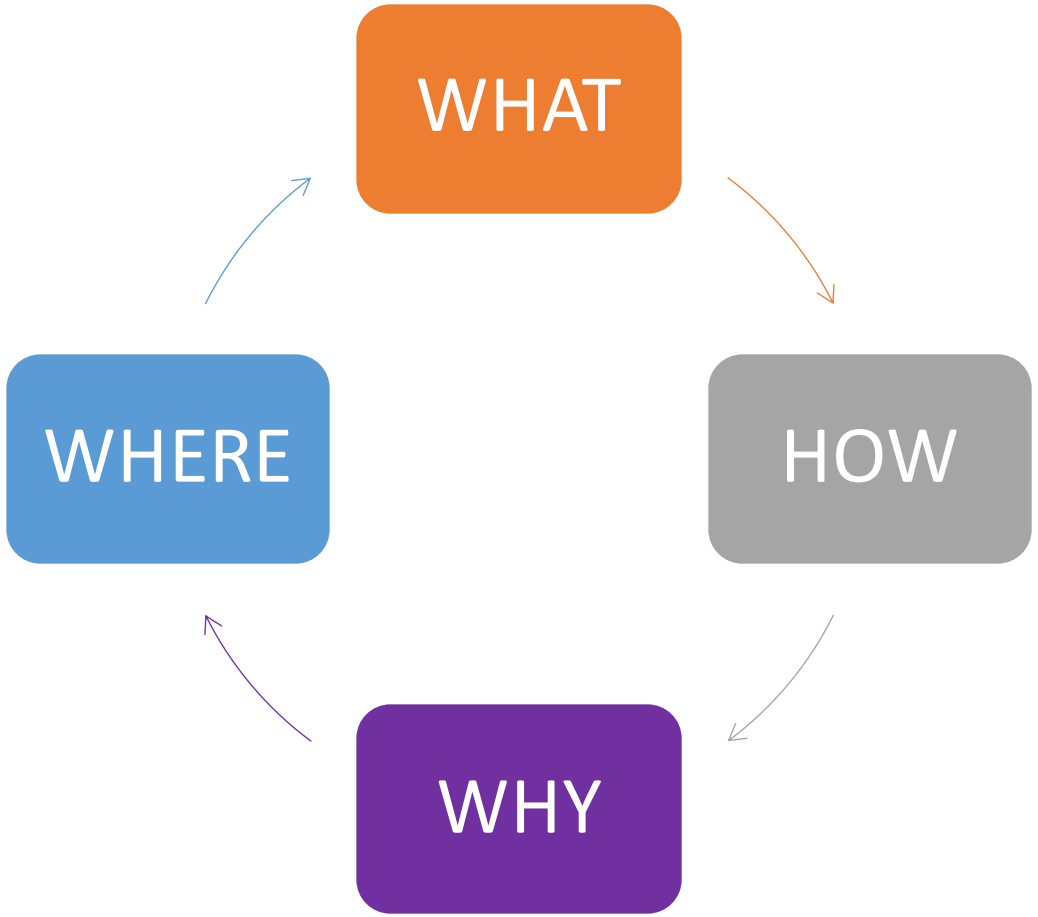
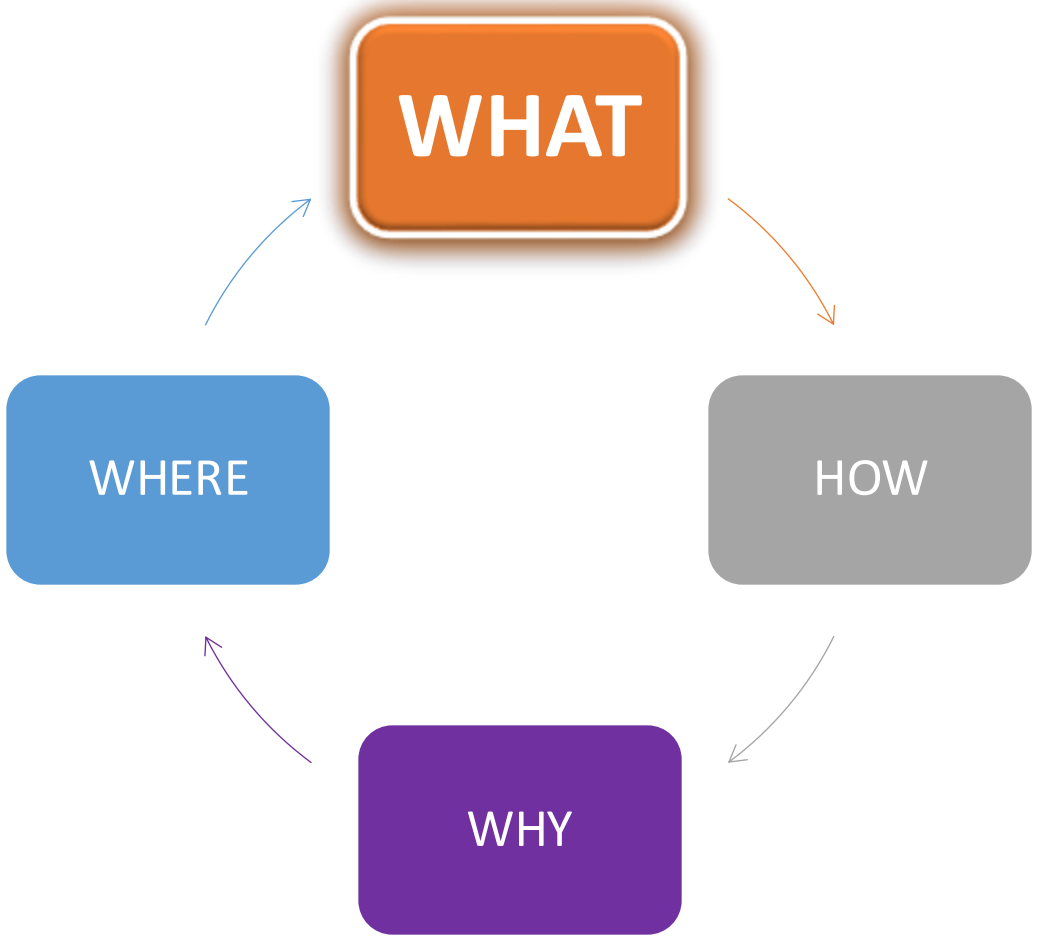
Agenda



WHAT?

- What is a Clinical Trial?
- What are the phases of a Clinical Trial?
- What is the purpose of a Clinical Trial?
- What are the general features of a Clinical Trial?



WHAT is a Clinical Trial?

- Who:
  - Patient **volunteers**
  - Healthy vs. Unhealthy
- What:
  - **Answer** questions
  - **Gather** information
- Why:
  - Find **new ways** to prevent, detect, diagnose, and treat disease
  - Better patient **outcomes**

- Observation
  - **Collection** of data while receiving standard medical care
  - Purpose is to find out **more information**
- Interventional
  - Treatment is given and **investigated**
  - **Develop new** treatments to prevent, detect, diagnose, and treat diseases

WHAT are the phases of Clinical Trials?

Interventional

- Phase 1
  - Focus on **tolerability** and the **proper dose**
  - Total accrual: 15-50 patients
- Phase 2
  - Focus on **effectiveness** and **side effects**
  - Total accrual: <100 patients
- Phase 3
  - Compare **new** treatment to **existing** treatment
  - Total accrual: 100+ patients

Observational

- Phase 4
  - Observe **long-term effects** of approved treatment
  - Total accrual: 1,000+ patients

WHAT is a Phase 1 Clinical Trial?

- Early investigation
  - Brand new treatment
  - Older treatment in a new indication
- Primary goal/objective
  - Tolerability
  - Safest dose
- Rationale
  - Test a new concept
  - Early non-clinical results are favorable

Generally First-in-Human (FIH)
=
Low Accrual Numbers

Patients dosing staggered to assess safety and tolerability

Dose escalation to the maximum tolerated dose (MTD)

WHAT is a Phase 2 Clinical Trial?

- Continual Investigation
  - Phase I goal achieved
  - Most common phase in oncology
- Primary goal/objective
  - Efficacy
  - Safety
- Rationale
  - Discover how well it works
  - Phase I data is favorable

More patients enrolled with less strict requirements

Open enrollment, no dose staggering

Dose expansion
=
More data collected at safest dose level

WHAT is a Phase 3 Clinical Trial?

- Comparison Investigation
  - Compare to standard of care
  - Randomization
- Primary goal/objective
  - Outcomes
  - True benefit
- Rationale
  - Unbiased results
  - Better assessment of side effects

Large trials with hundreds of patients enrolled

Randomization
=
Random assign to intervention, like a flip of a coin

Cannot choose treatment
Gives balance to investigations

WHAT is the purpose of a Clinical Trial?

TOLERABILITY

- New drug → new side effects
- Old drug → new indication → new side effects

EFFICACY

- Does it work well
- New, better ways to treat cancer

OUTCOMES

- Overall survival
- Relapse-, GVHD-free

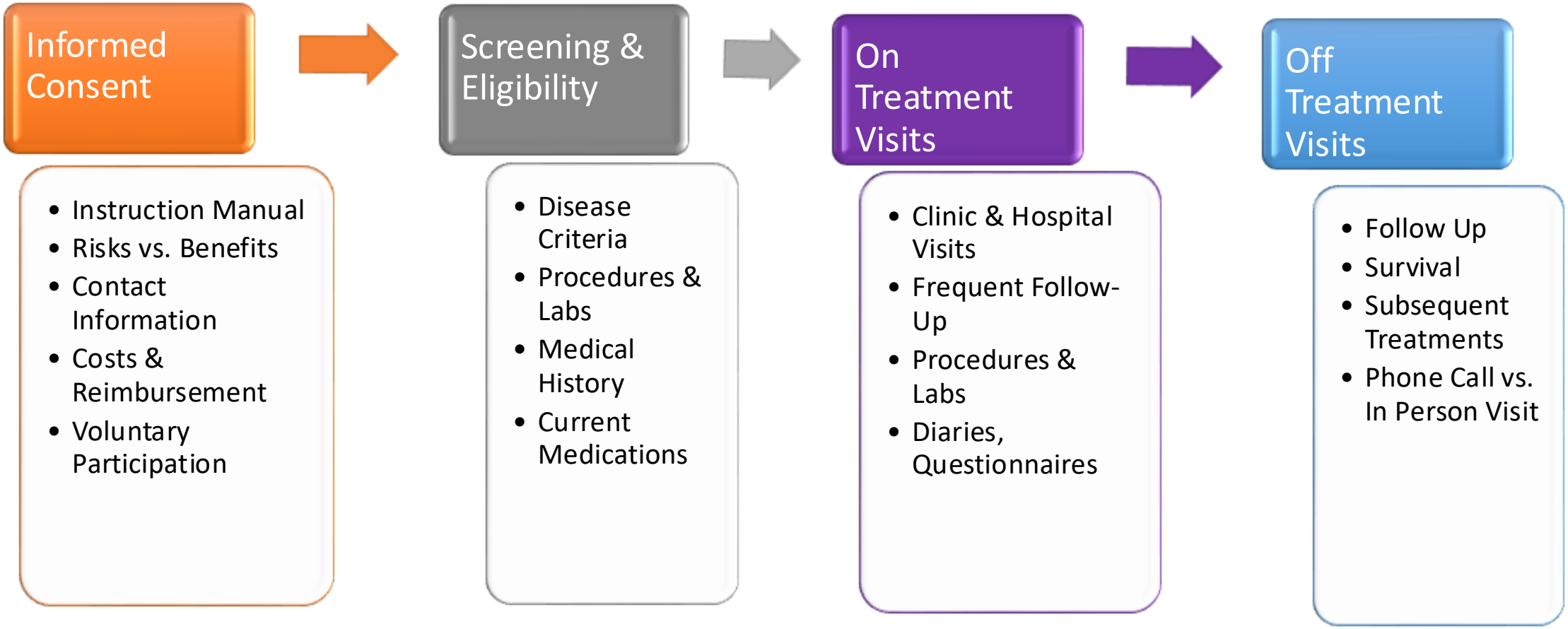
COMPARISONS

- Group A vs. Group B
- Who does better?

CHANGE

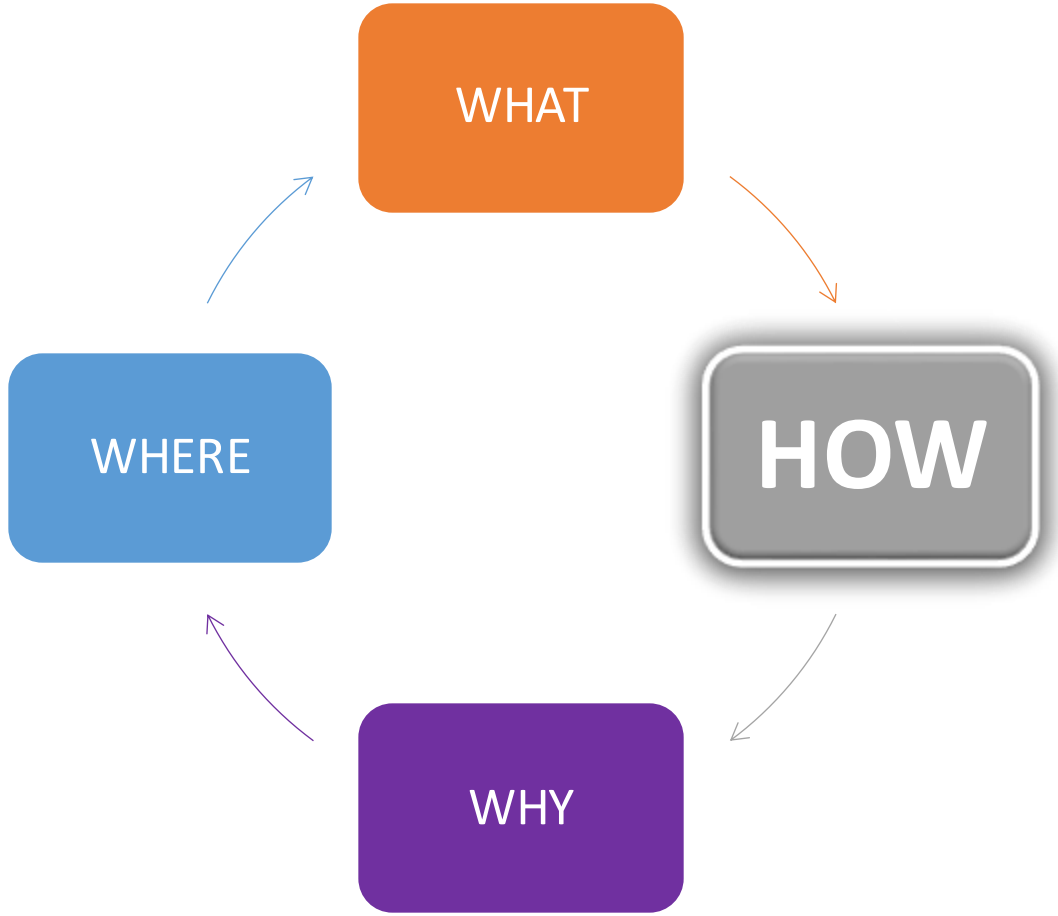
- The new standard
- Better outcomes

WHAT – are the general features of a Clinical Trial?



HOW?

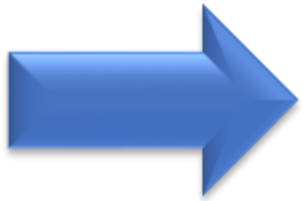
- How are Clinical Trials different?
- How are Clinical Trials reviewed and deemed appropriate for patients?
- How are patients selected and evaluated for a Clinical Trial?
- How are patients monitored during a Clinical Trial?
- How are Clinical Trials funded?



HOW are Clinical Trials different?

Clinical Trials

- Experimental
- Voluntary participation
- Informed consent
- Protocol guidelines and rules



Standard of Care

- Established care
- Proven results
- Physician choice
- Supportive care algorithms

Clinical Trials help establish Standard of Care algorithms and procedures

HOW are Clinical Trials different?

Chemotherapy

- New drug to kill cancer cells
- Newly diagnosed or relapsed (cancer comes back)/ refractory (cancer does not respond to initial treatment)

Transplant

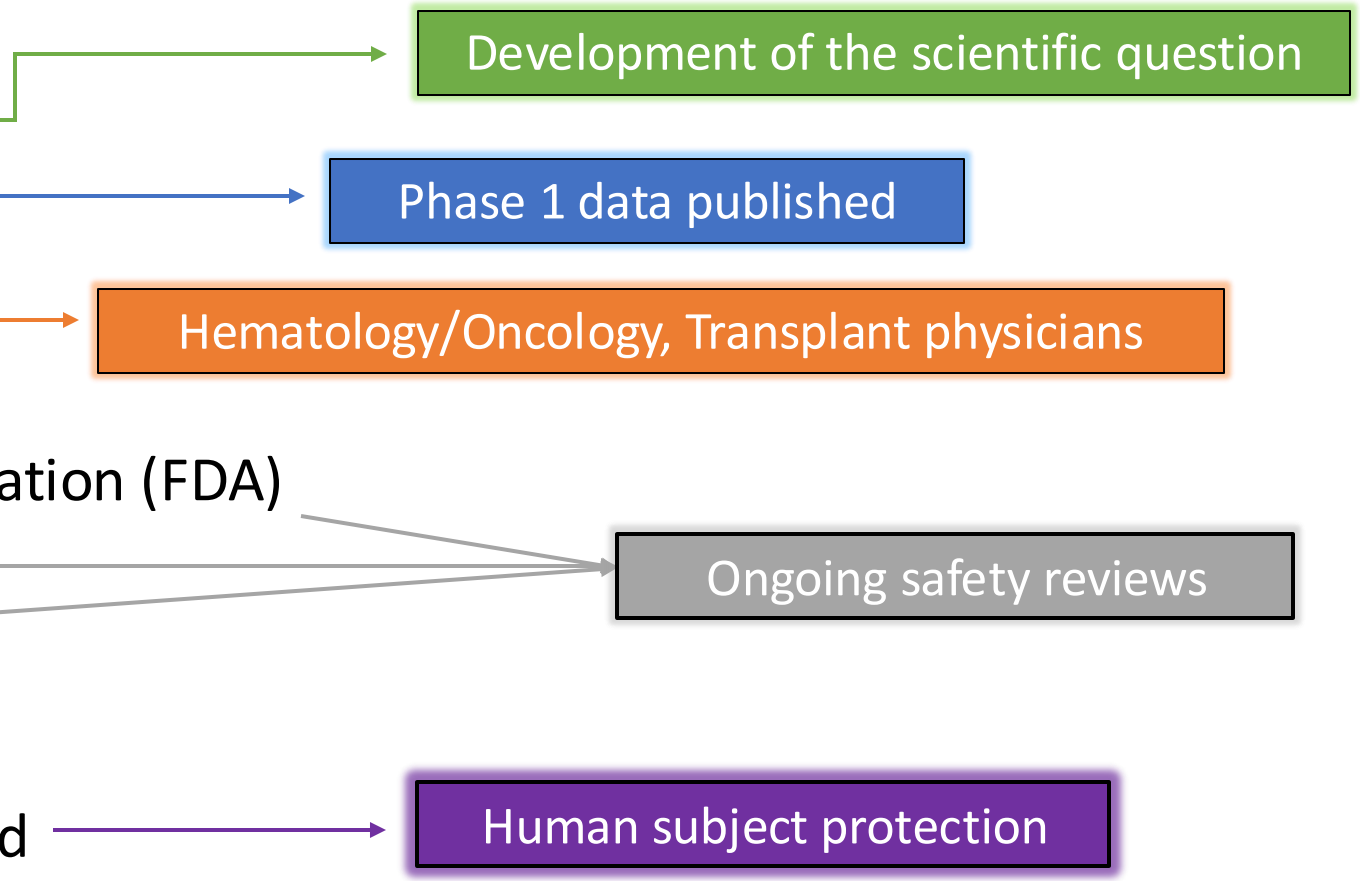
- New GVHD prophylaxis or treatment
- New conditioning regimen
- Standard medication given earlier to prevent infection

Immune Effector Cell (IEC) Therapy

- New kid on the block
- Using own or healthy donor immune cells to kill cancer cells

HOW are Clinical Trials reviewed and deemed appropriate for patients?

- Background review
  - Lab-based
  - Scientific results
  - Designed by experts
- Scientific review
  - Food and Drug Administration (FDA)
  - Oversight committees
  - Steering committees
- Ethics review
  - Institutional Review Board



HOW are patients selected and evaluated for a Clinical Trial?

- Physician consult
  - Pre-Screening
  - Basic eligibility criteria
  - Informed consent discussion
- Screening
  - Labs, physical exam, procedures, other specific tests
  - In-depth eligibility criteria
  - Protocol-specified, carefully designed

HOW are patients monitored during a Clinical Trial?

Clinical Trials detail specific criteria for investigators to review for patient safety

- Clinic visits
- Lab results
- Study team

- Data Safety Monitoring Boards
- Serious events
- Events of special interest

External boards review safety data without bias

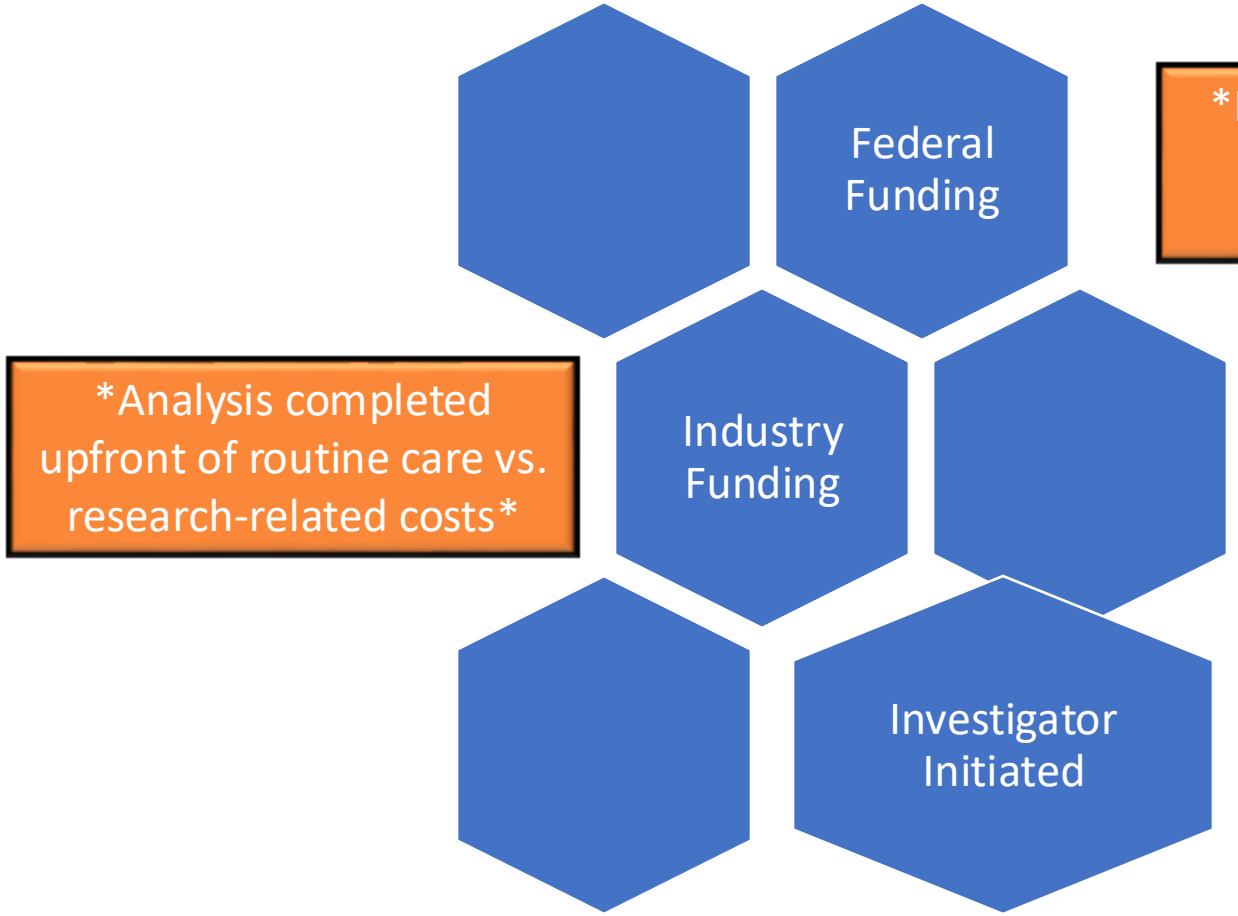
IRBs recommend updating language in the ICFs for any unexpected events that need to be reported to patients

- Unexpected events
- ICF review

- Medical Monitor
- Data gathering & review

Study sponsor reviews all data entered into the database in real-time

HOW are Clinical Trials funded?

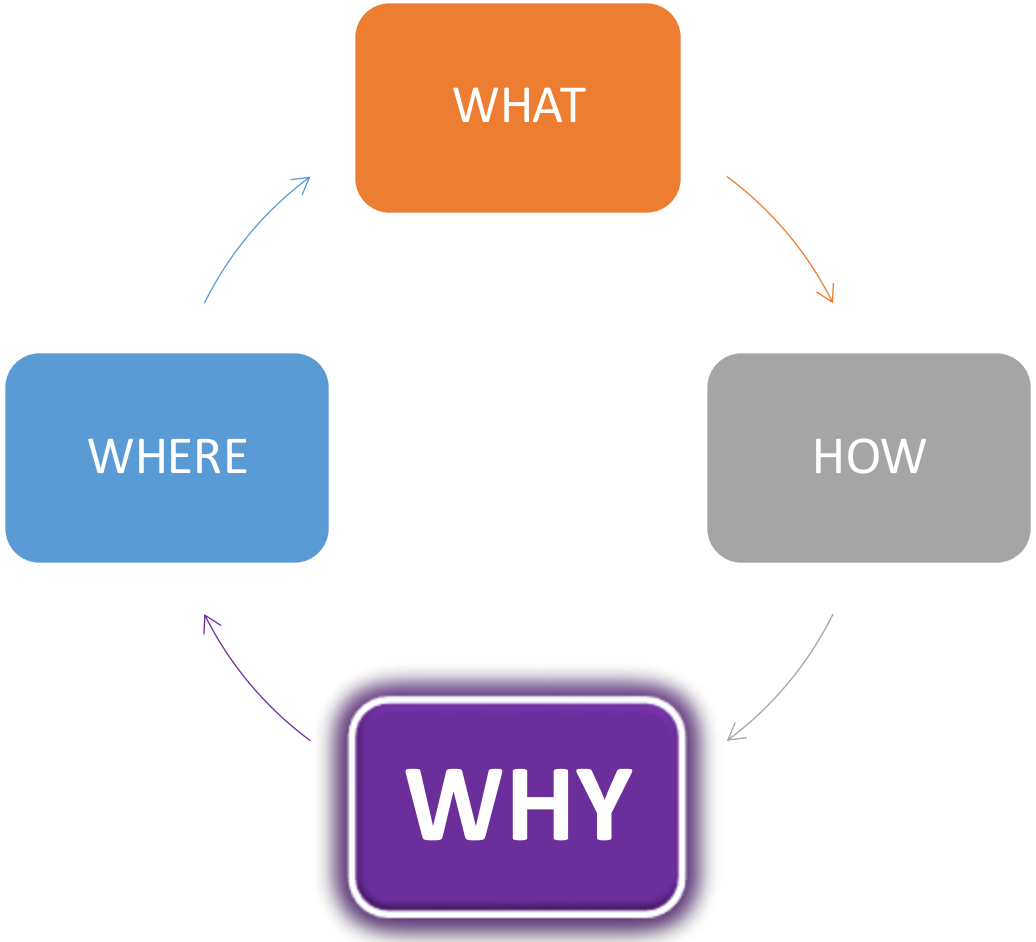


ICF includes details of extra costs, reimbursement for participation

Analysis completed upfront of routine care vs. research-related costs

WHY?

- Why are Clinical Trials stopped early?
- Do Clinical Trials use placebo?
- Should I participate in a Clinical Trial?



WHY are some Clinical Trials stopped early?

- Side effects
  - Risk vs. benefit analysis
  - Too much toxicity
- Clinical benefit
  - Good results
  - Better than routine care
- Sponsor decision
  - Re-prioritization
  - Slow enrollment

WHY do Clinical Trials use placebo?

- Randomization
  - Flip of a coin
  - Reduce bias
- Blinded vs. Unblinded
  - Blinded = unaware of treatment assignment
  - Unblinded = aware of treatment assignment
- Cause-and-effect
  - Relationship between treatment and its outcome
  - Reliable evidence

Randomized trials are considered the golf standard

No choice = less bias

Leads to more reliable evidence for clinical decision-making

WHY should I participate in a Clinical Trial?

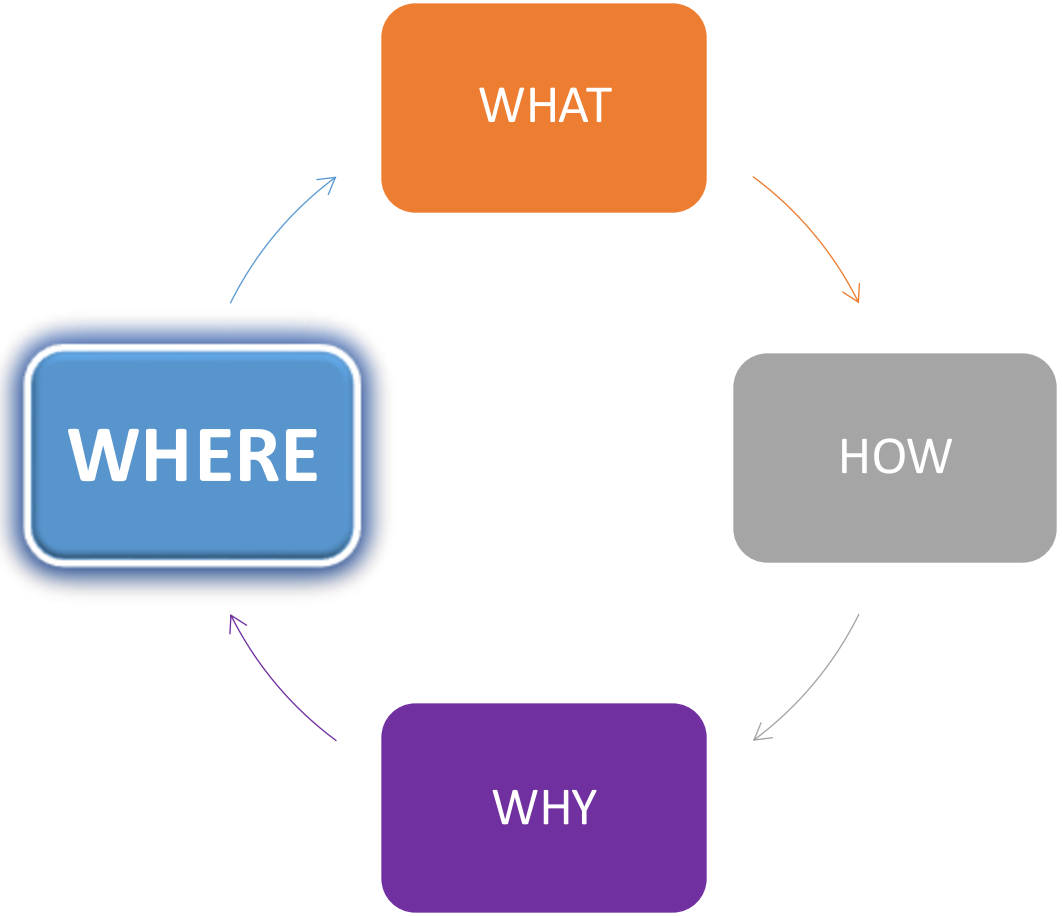
- Receive expert medical care
- Gain access to latest treatments and techniques
- Contribute to medical advancements
- Help future patients
- Things to consider:
  - Time commitments
  - Visit requirements
  - Second opinions

Clinical Trials are always OPTIONAL

Sponsors and sites try to reimburse patients for parking, meals, mileage, etc. to offset costs

WHERE?

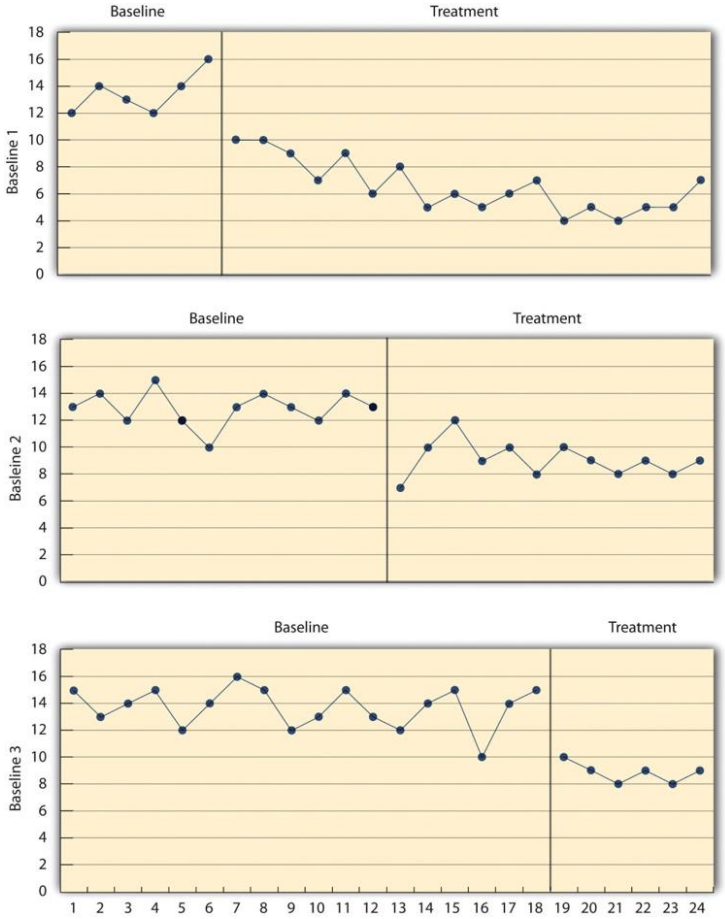
- Where can I find out the results of a Clinical Trial?
- Where can I find more information about a Clinical Trial?



Where can I find the results of a clinical trial?

- Sponsor provided information
- Study Physician
- Clinicaltrials.gov

Results are usually not available until the Clinical Trial has enrolled all patients and completed all data analysis



WHERE can I find more information about a Clinical Trial?

- Clinicaltrials.gov
- LLS.org
- Bmtinfonet.org
- Local hospital/university/clinic websites
- Social media

Useful questions to ask about a Clinical Trial?

- What is being researched?
- How is the treatment given?
- Will I need to make extra trips to see my doctor?
- Do I have to pay for any part of the trial?
- Will my insurance cover treatment if I participate in a trial?
- Will I be able to see my own doctor?
- Can I change my mind and withdraw from the trial at any time?
- What if the treatment doesn’t work for me or I have bad side effects?
- What happens to my medical care when the trial is over/finished?

Questions?

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Let Us Know How We Can Help You

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