An Introduction to

CLINICAL TRIALS

ALLIANCE TO CURE CAVERNOUS MALFORMATION

CLINICAL TRIALS ALLOW US TO LEARN:

WHETHER A NEW TREATMENT IS SAFE

WHETHER A NEW TREATMENT IS EFFECTIVE OR MORE EFFECTIVE FOR SOME PEOPLE THAN OTHERS.

HOW MUCH OF A NEW TREATMENT IS NEEDED TO HAVE THE DESIRED EFFECT – THE DOSE & FREQUENCY

WHETHER A NEW TREATMENT HAS UNINTENDED SIDE EFFECTS.

Clinical Trials

The Drug Development Process

Phase 1	Phase 2	Phase 3	FDA Approval Process	Market
20-100 Healthy volunteers for new drugs	100-200 Patients	200+ Patients	Reports are submitted for FDA Approval Drug production begins after approval	Post-marketing surveillance
> 1 Year	*1-2 Years*	*2-3 Years*	*6-12 Months*	
Purpose →	→	→	→	→
✔Safety	✔Safety, efficacy & dosing	✔Comparative effectiveness & risk benefit analysis	✔Approval & drug production	✔Long-term safety & comparative effectiveness

The duration of a trial and required number of participants is dependent upon the outcome that is measured. Trials that measure hemorrhage, for example, an infrequent event, are likely to be longer in duration than a trait that measures a more frequent outcome, like headache or seizure.

PLACEBOS

“Placebo effect”

A placebo is a substance or procedure that appears identical to the treatment being tested. Sometimes, a condition will improve just because a person thinks they are receiving treatment.

Some participants in larger clinical trials will take a placebo to ensure the real treatment is better. In trials where it would be unethical to withhold treatment, placebos are not used.

WHAT IS THE BENEFIT OF PARTICIPATING IN A CLINICAL TRIAL?

The investigational treatment studied in a clinical trial may or may not benefit the patient personally. The benefits of participating in a clinical trial may include:

CONTRIBUTION

Helping other patients by contributing to medical research and treatment advances.

ACCESS

Gaining access to cutting-edge research.

CARE

Receiving expert medical consultation for the condition being studied, since doctors conducting clinical trials are often specialists in the disease areas.

MYTHS VERSUS FACTS

Here are some common myths about clinical trials and the facts.

I CAN'T DROP OUT

TRUTH: You may leave a trial at any time, for any reason.

IT COSTS TO PARTICIPATE

TRUTH: Most trials are free. Participant's travel is reimbursed, and they receive a stipend.

I MUST STOP ALL OTHER MEDICINES

TRUTH: Every trial has different requirements. Many allow other medicines.

I MUST CHANGE DOCTORS

TRUTH: You can keep your current doctor for care and use the trial doctor for trial monitoring.

HOW ARE CLINICAL TRIAL PARTICIPANTS KEPT SAFE?

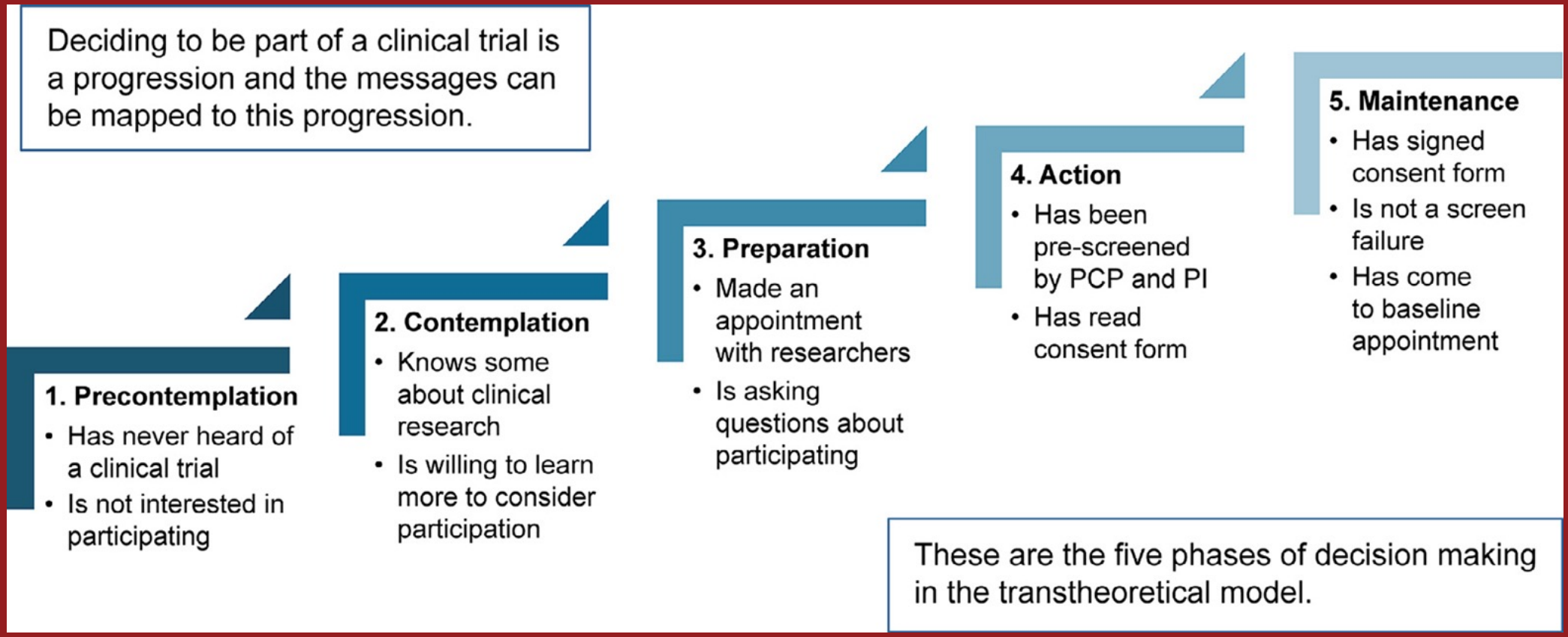
EACH TRIAL IS MONITORED BY MEDICAL STAFF, AND PARTICIPANTS ARE REGULARLY SEEN.

HEALTHY VOLUNTEERS HAVE ALREADY TAKEN THE TREATMENT AND NOT HAD SERIOUS SIDE EFFECTS.

EACH TRIAL IS APPROVED & OVERSEEN BY MULTIPLE SAFETY AUTHORITIES: FDA, INSTITUTIONAL REVIEW BOARD, DATA SAFETY MONITORING BOARD.

THE TRIAL MUST PROVIDE YOU WITH ALL THE INFORMATION YOU NEED TO MAKE A DECISION ABOUT PARTICIPATING.

5 Phases of Decision-Making



* PI = Principal Investigator. This is the doctor in charge of the clinical trial at the clinical trial site.