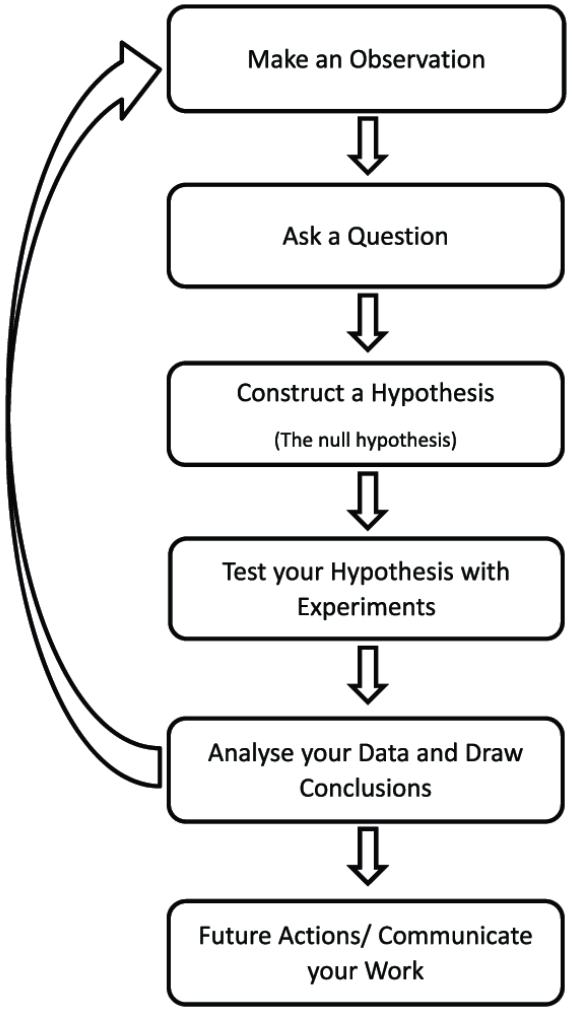
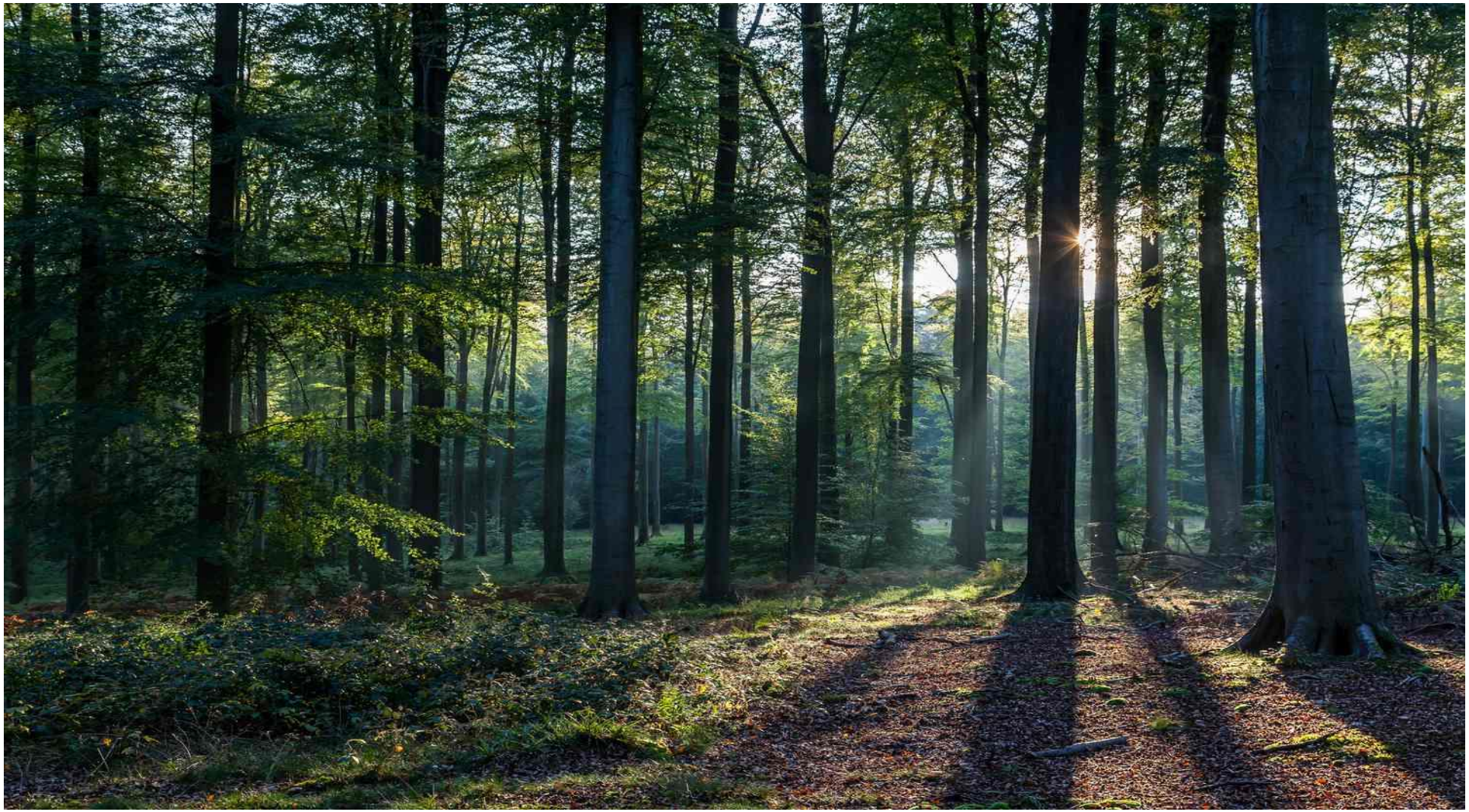
Key Concepts of Clinical Research

The Scientific Method



What is a Research Question and Hypothesis?

Concept Of Throwing A Ball And Hitting a Tree



Define Research Question and Hypothesis

- **Research Question** – defines the main objective of the study
  - Formulated as a question
  - Contains details
  - Good research questions reflect FINER criteria

- **Hypothesis** – educated guess of study outcome
  - Formulated as a statement
  - Needed for biostatistical analyses

- Forms basis of study protocol

Let's Practice

- **You want to determine whether ClearRX helps get rid of warts.**

  - **What steps do you want to take to formulate your research question?**
    - What do you need to know about warts?
    - What do you need to know about ClearRX?
    - What do you need to know about people with warts?

  - **What steps do you want to take to formulate your hypothesis?**
    - What are all the ways you can determine whether a wart is gone?
    - What is the best way to measure whether ClearRX got rid of the wart?
    - How long does it take ClearRX to get rid of warts?

Getting Ready To Do The Experiment

- Requires careful planning

- Need to define:
  - The study sample- who will be recruited and enrolled
  - Study variables -how phenomena of interest will be measured
  - Study design – methods by which experiment will be performed

Defining The Study Sample

- **All clinical research focuses on a target population**
  - Reflects the entirety of the group of interest
  - Example: adolescents 12-16 years old with digital warts
  - Impossible to enroll the entire target population

- **The study team must narrow down the target population into the intended sample**
  - Feasible to enroll and study
  - Decreases heterogeneity of the group
  - Reflects the target population to allow generalizability

Measuring Phenomena of Interest

- **Variables are measurements taken during the course of a study**
  - **Two types of variables**
    - **Outcome variables** – measures the effect of an intervention or phenomenon of interest
      - Examples include: blood pressure, blood glucose, hospitalizations, disease free survival, death
    - **Predictor variables** – measures of the characteristics of the study participants important to the research question
      - Examples include: sex/gender, age, duration of disorder under study, or tumor characteristics
  - **All study variables need to be carefully recorded**

How Do You Know How Many People to Enroll?

- **Concept of sample size and power**
- **Calculate how many people are needed to enroll to make sure that:**
  - There are enough people to detect a difference between groups
  - Any observed difference is not due to chance alone
- **Sample size is a function of:**
  - Effect size
  - Variability of the participants
  - Variability and sensitivity of the measurement
  - Type of biostatistical test used

Methods of Research