

# DEMYSTIFYING CLINICAL TRIALS

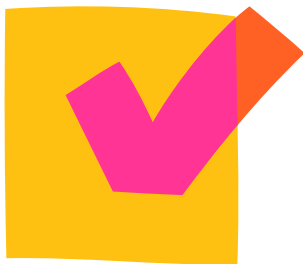
**A PATIENT EDUCATION GUIDE:  
WHAT THEY ARE, HOW THEY WORK AND  
WHY THEY MATTER IN RARE DISEASE**



# WHAT IS A CLINICAL TRIAL?

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A CLINICAL TRIAL IS A RESEARCH  
STUDY THAT EVALUATES WHETHER A  
TREATMENT IS:



**SAFE**



**EFFECTIVE**



**APPROPRIATE FOR  
WIDER USE**

These studies move therapies from  
**research to real-world care.**



# WHY CLINICAL TRIALS MATTER

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They **drive progress in treatments** for people living with rare diseases.



They **provide access to promising therapies** that are not yet available outside of research settings.



They **help researchers understand safety, dosing, relapse patterns, biomarker changes, and long-term outcomes.**



# WHO RUNS CLINICAL TRIALS?

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**ACADEMIC MEDICAL CENTERS**



**PHARMACEUTICAL OR BIOTECH COMPANIES**



**GOVERNMENT OR FEDERAL RESEARCH  
AGENCIES**



# WHO OVERSEES CLINICAL TRIALS?

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Clinical trials are overseen by several layers of authority to ensure **safety, ethics** and **scientific validity**.



**Principal Investigator (PI)** - The lead researcher at the trial site



**Sponsor** - Often a pharmaceutical company, biotech firm or academic institution funding the trial



**Regulatory Authorities** - Government agencies that approve and monitor clinical trials



Health  
Canada



**Ethics Committees / Institutional Review Boards (IRB)** - Independent committees that protect the rights and welfare of participants



**Data Safety Monitoring Board (DSMB)** - An independent group of experts monitoring data for safety and efficacy



# 4 PHASES OF A CLINICAL TRIAL

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## **PHASE 1: IS IT SAFE?**

Small group testing to understand safety and dosing



## **PHASE 2: DOES IT WORK?**

Researchers look for early signs the treatment helps



## **PHASE 3: DOES IT WORK BETTER?**

Large studies compare the new treatment to existing options or placebo




## **PHASE 4: HOW DOES IT PERFORM LONG-TERM?**


Monitoring safety and effectiveness after approval




# KEY TERMS & WHAT THEY MEAN


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 **Adverse Events** - Side effects or health issues during the trial

 **Blinded/Double Blind** - Treatment identity hidden to prevent bias

 **Control Group** - Comparison group without the new treatment

 **Open Label** - Everyone receives the active treatment

 **Placebo** - Inactive treatment used for comparison

 **Randomization** - Groups assigned by chance

 **Study Arm** - A specific group in the trial

 **Washout Period** - Break to clear previous medications

# PATIENTS RIGHTS & SAFEGUARDS

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**Informed Consent** - Understand before joining



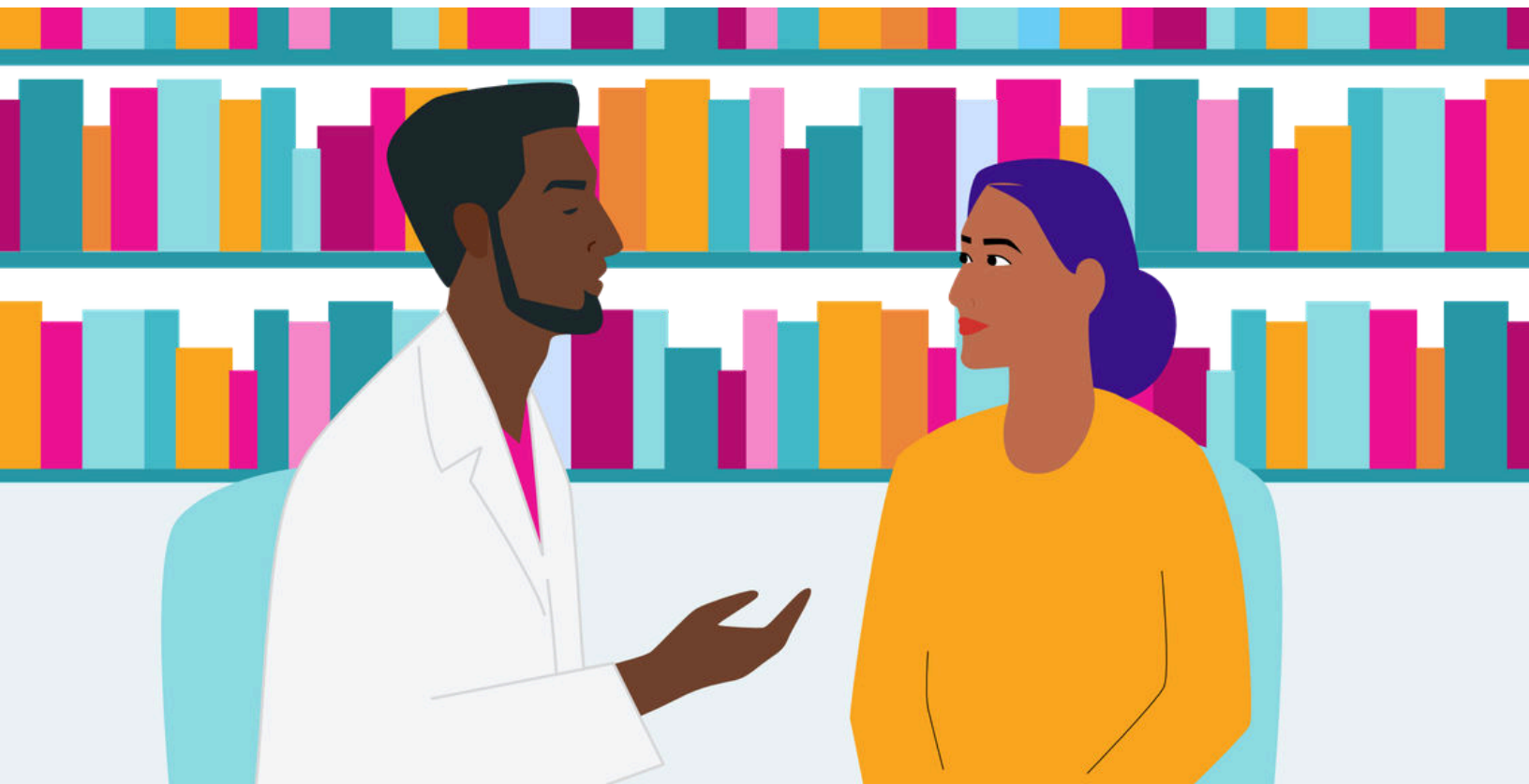
**Right to Withdraw** - Participation is voluntary



**Privacy Protection** - Your data stays confidential



**Safety Oversight** - Independent experts monitor trials



# IS A TRIAL RIGHT FOR YOU?

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## QUESTIONS TO ASK YOUR NEUROLOGIST:



**What is the trial studying?**



**How much time is required?**



**What are the risks and benefits?**



**How is safety monitored?**



**Are travel costs covered?**



**Will treatment continue after the study?**

