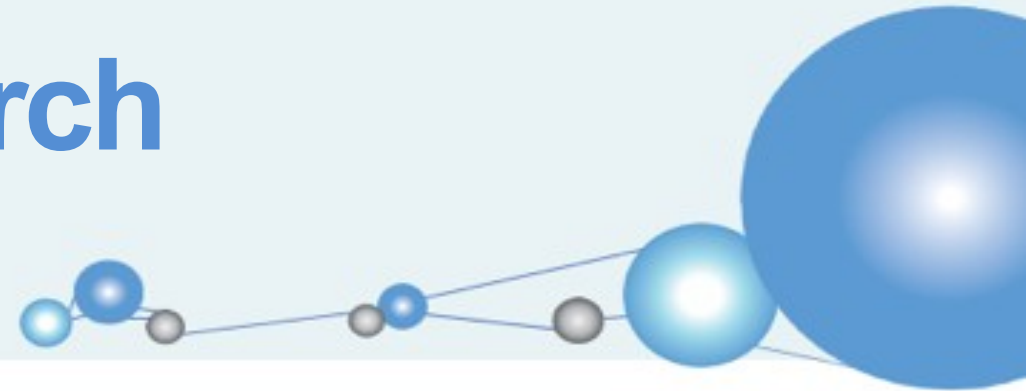




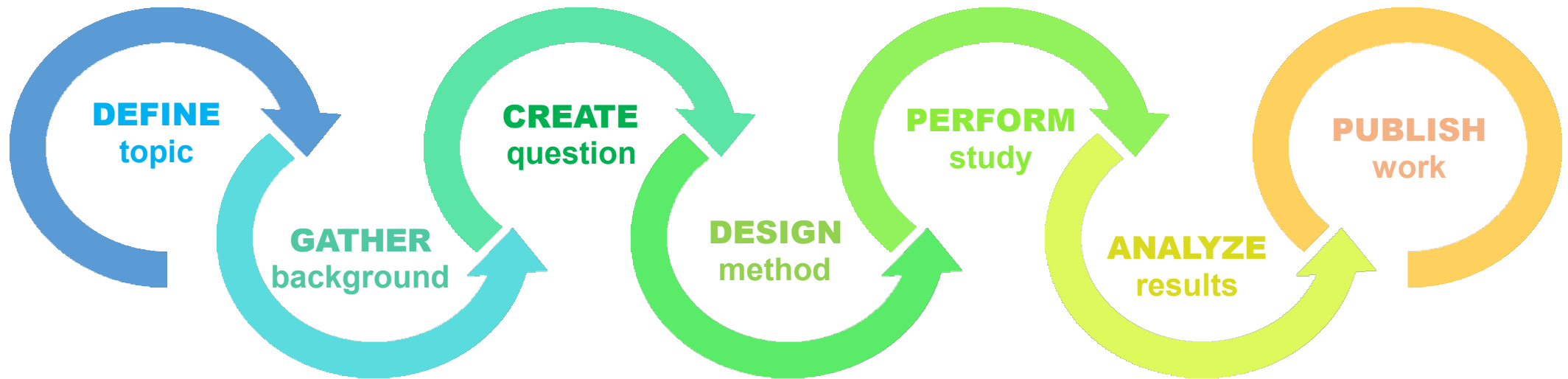
Learn About Clinical Research



What is Research?

Research is a careful and detailed study into a specific problem, concern, or issue using the scientific method.

Clinical Research is a specific type of research to help identify causes of illnesses and develop better treatments.



Research Process

What is Clinical Research?



Surveys



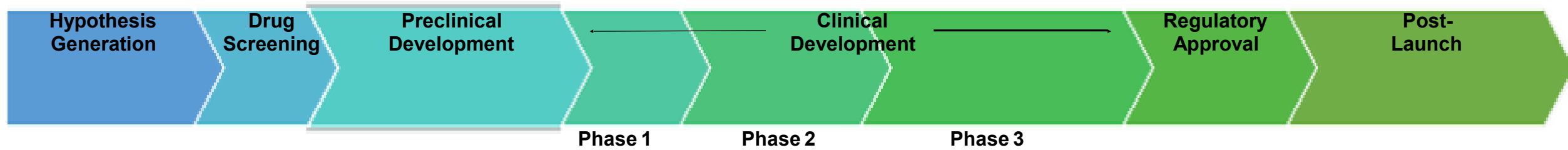
Sample
Studies



Drug
Testing
Studies



Drug Development Timeline



Phases of Clinical Development

Phase 1: Aims to find the best dose of a new drug with the fewest side effects.

The drug will be tested in a small group.

Often done in healthy individuals.

Phase 2: Further assesses safety and whether the drug works for a specific condition.

May still include various dosing to determine most effective dose.

Often done in larger groups of patients compared to Phase I trials.

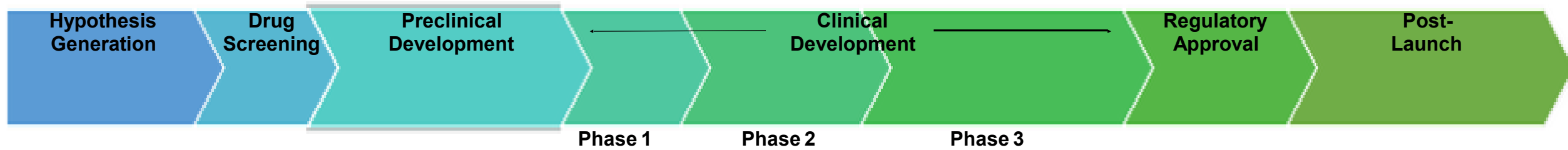
Phase 3: Compares a new drug to the standard-of-care treatment.

These trials assess the side effects of each drug/treatment and which works better.

Phase III trials enroll even more patients, if possible.



Drug Development Timeline



Some more important terms used in drug development

Open-label extension trial follows some early-phase studies to provide continued access to the investigational treatment for a longer time or until drug is approved by the regulatory agency.

Expanded access, sometimes called “compassionate use”, is a potential pathway for a patient with an immediately life-threatening condition or serious disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.



Why Participate in Research?

*NIH sponsored FB support group called
Children and Clinical Studies Campaign*

give back quality of life hope
access to new treatment
make a difference
find a cure help others

- New treatments are only available when research shows they work and are safe
- More treatments available for HI allows personalized approach to treatment
- § Research increases our knowledge about HI and potentially leads to:
 - More timely diagnosis
 - Even better treatment options
 - Fewer undesirable effects
- Allows access to specialist care for those who don't receive care at a CoE



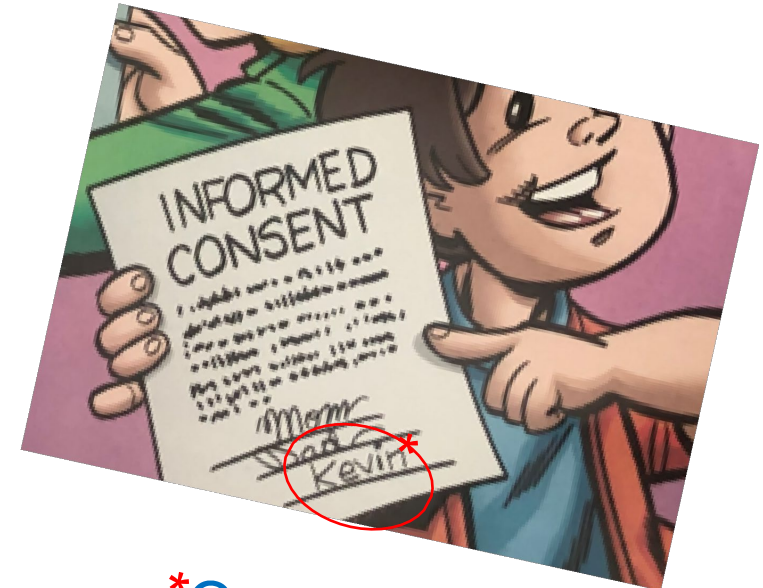
How to Participate?

Participating in research is a PARTNERSHIP

Ask questions!

- Before, During and After the study
- Understand what the researchers are hoping to learn from the study
- Clarify what is expected of you and your child

You may stop participating at any point, even after you have begun the study



*CHILD'S
ASSENT

How to Stay Informed?

 U.S. National Library of Medicine

ClinicalTrials.gov



<https://www.anzctr.org.au/Default.aspx>

EU Clinical Trials Register

<https://www.clinicaltrialsregister.eu/ctr-search/search>

WHO International Clinical Trials Registry

<https://www.who.int/clinical-trials-registry-platform/the-ictrp-search-portal>

