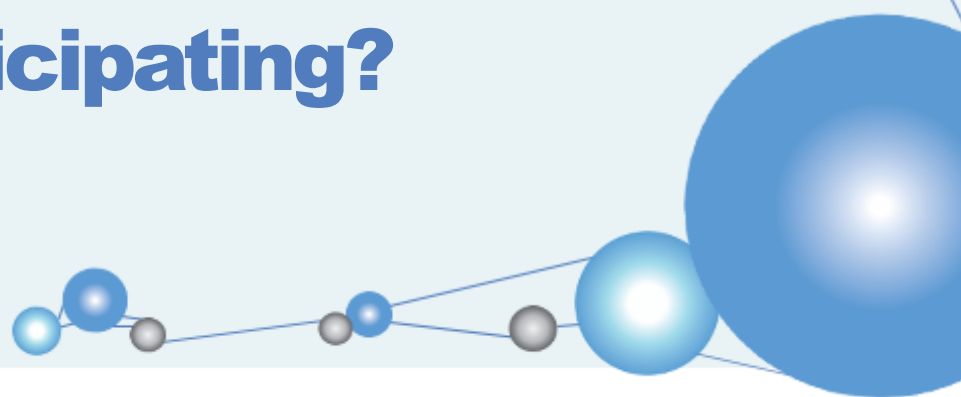




# What is Clinical Research and What do I Need to Know about Participating?

*Tai Pasquini, PhD*



# Our Community Goal: Future Without Lows

The HI community needs new therapies and approaches so people with HI can live life fully.



Willow loves to swim,  
but her mom worries about blood sugar drops  
when she does.

Learn about HI clinical trials.  
**#FutureWithoutLows**

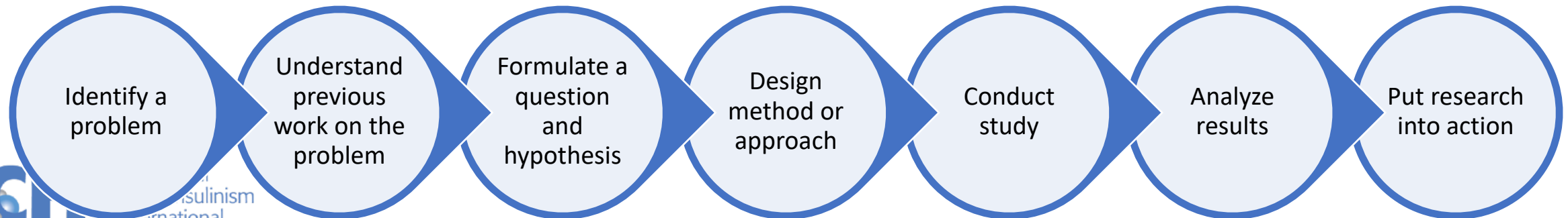
**CHI**

# Current Challenges: Why New Treatments are Needed?

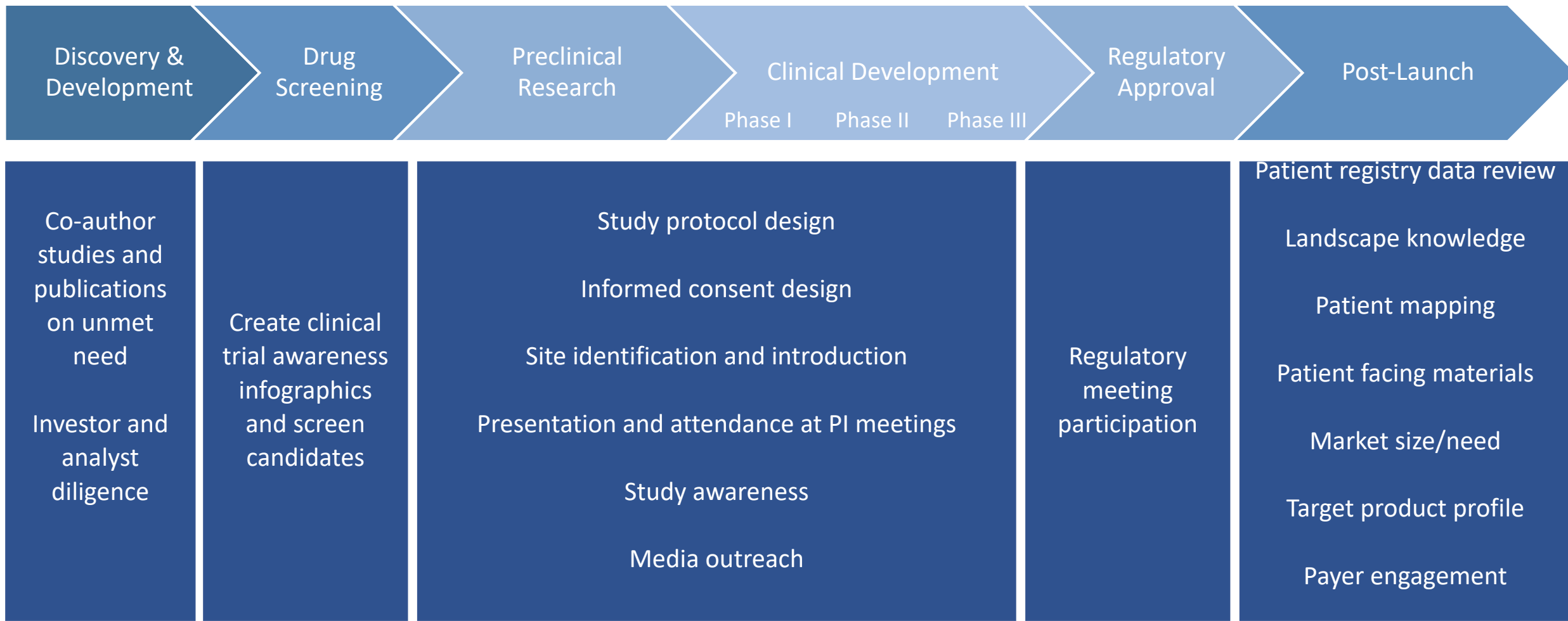
- Many with HI still have significant hypoglycemia and do not respond fully or at all to diazoxide.
- Some respond to diazoxide but can't take it because of adverse effects or don't like the physical changes it causes.
- Many are curtailing activities because of lows.
- Some are on a therapy that protects from lows but is so complicated that caregivers are exhausted and there are lifestyle restrictions.
- Many are on constant feeds or eat almost constantly so they don't experience hypoglycemia.

# What is Clinical Research

- Type of research to help identify causes of illnesses and develop better treatments
- A clinical research trial is a research project conducted with human participants to learn more about new investigational drugs
- Every new therapy or device must go through carefully monitored studies in human participants before it is commercially available
- Clinical trials that safeguard the health of study participants are necessary for the development of new HI treatments
- Conducted by industry or academic centers



# Drug Development Timeline



# Drug Development Timeline



Goal: Focus on safety, finding the proper dose range, and identify any side effects

Participants: A small group of healthy volunteers

Goal: Focus on effectiveness and side-effects

Participants: A relatively small group of individuals with the disease of interest

Goal: Compare the safety and effectiveness of the new treatment against the current treatment

Participants: A larger group of individuals with the disease of interest

# Why Participate in Clinical Research?

- Research increases our knowledge of HI and potentially leads to:
  - More timely diagnosis
  - New and better treatments
  - Fewer undesirable effects
- More treatments available for HI allows personalized approach to treatment
- Allows access to specialist care for those who may not have regular access to HI specialists
- Personal satisfaction of being part of scientific innovation as a member of the research team and process

# CHI's Role in Clinical Research

- One of CHI's important roles is to inform the patient community of clinical research opportunities in order to help raise awareness of and increase participation in clinical research and clinical trials
- CHI's role does not include endorsing specific studies
- CHI strongly recommends discussing clinical research participation for each specific study with your physician specialist or healthcare provider



# How to Participate?

- If you hear about a clinical trial that you may be eligible for, you can talk to your doctor, or your doctor may recommend a trial to you
- Most studies will have a screening survey to confirm your eligibility
- Participating in research is a partnership
  - You can ask questions before, during, and after the study
- Study staff will explain what they are hoping to learn from the study and ensure you know what to expect
  - This is called informed consent
  - If your child is participating, they will be given kid-friendly documents to provide assent as well

# Find Clinical Trial Opportunities

- CHI Clinical Trials Webpage
  - <https://congenitalhi.org/clinical-research-trials/>
- U.S. Clinical Trials
  - [www.clinicaltrials.gov](http://www.clinicaltrials.gov)
- Australian New Zealand Clinical Trials Registry
  - [www.anzctr.org.au/Default.aspx](http://www.anzctr.org.au/Default.aspx)
- EU Clinical Trials Register
  - [www.clinicaltrialsregister.eu/ctr-search/search](http://www.clinicaltrialsregister.eu/ctr-search/search)
- WHO International Clinical Trials Registry
  - <https://www.who.int/clinical-trials-registry-platform>