



What is Clinical Research from the Patient Advocacy Perspective?

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Our Community Goal: Future Without Lows

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



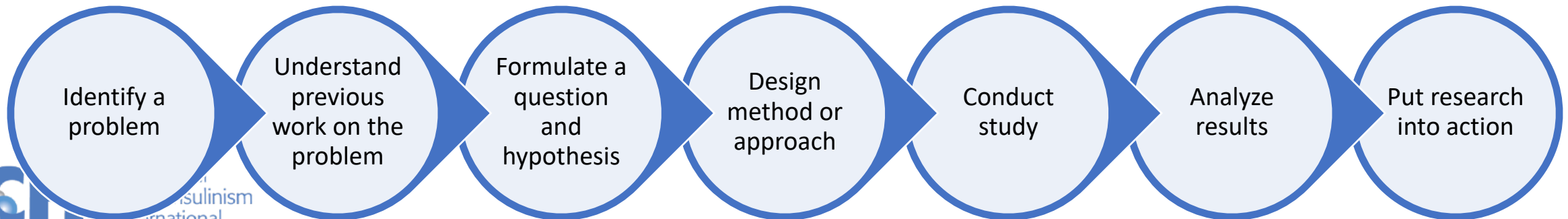
Willow loves to swim,
but her mom worries about blood sugar drops
when she does.

Learn about HI clinical trials.
#FutureWithoutLows

CHI

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



Current Challenges: Why New Treatments are Needed?

- Many with HI still have significant hypoglycemia and do not respond diazoxide, fully or at all.
- From HI to diabetes: the conundrum of subtotal pancreatectomies
- 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.

Drug Development Timeline



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

Participants: A small group of healthy volunteers

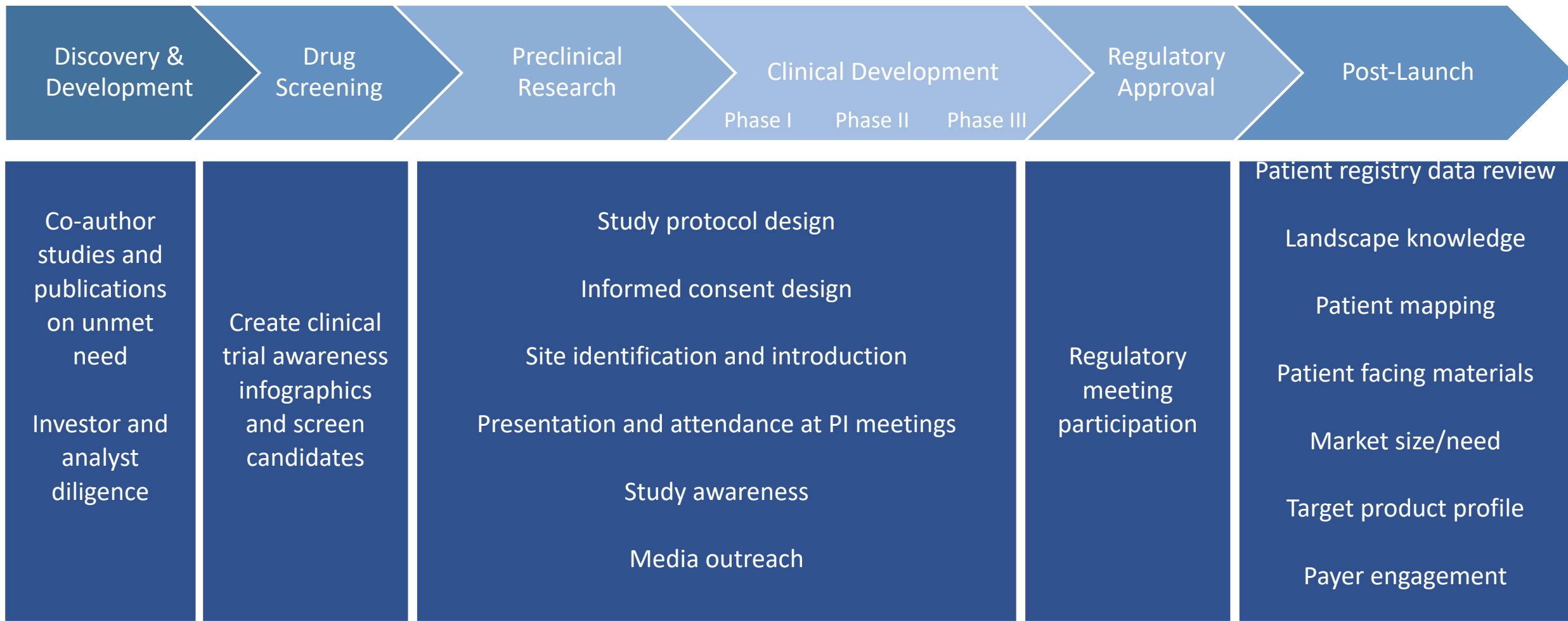
Goal: Focus on effectiveness and side-effects

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

Drug Development Timeline



CHI's Role in Clinical Research

- Patient Voice: Convey the experience of living with HI to drug developers and ensure they try and develop treatments that are what patients want.
- 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
- Australian New Zealand Clinical Trials Registry
 - www.anzctr.org.au/Default.aspx
- EU Clinical Trials Register
 - www.clinicaltrialsregister.eu/ctr-search/search
- WHO International Clinical Trials Registry
 - <https://www.who.int/clinical-trials-registry-platform>