HYPEROXALURIA GLOBAL ALLIANCE
A Community Without Boundaries

Drug Development

A Guide for Patients & Families For Primary Hyperoxaluria (PH) & Enteric Hyperoxaluria (EH)

How New Treatments Begin

Researchers test new treatments in the lab before testing in people. This step is called the **preclinical stage**. It helps scientists understand how a treatment works and if it appears safe.

Clinical Trial Phases

Phase 1

Safety First — a small group of people receives the treatment to check safety and find the right dose.

Phase 2

Does It Help? — a larger group is studied to see if the treatment works and to continue checking safety.

Phase 3

How Does It Compare? — the treatment is compared to standard care or a placebo to confirm it is safe and effective.

Phase 4

After Approval — doctors continue to monitor safety and how the treatment works over time.

Why Rare Disease Research is Hard

- Small patient communities
- Limited awareness
- Few specialist doctors

Patient Advocacy Groups Support Every Step of the Journey



Everyone Plays a Role

Progress happens when patients, caregivers, researchers, clinicians, industry, and hyperoxaluria patient advocacy groups work together.

Your Choice, Your Care

Joining a trial is your choice. You can stop at any time.

Your Voice Matters

Sharing your story helps shape better treatments and move us closer to a cure.

Visit WeAreHGA.org to learn more and get involved.



Disclaimer: This guide is for educational purposes only and does not replace medical advice. Always consult your healthcare provider for diagnosis and treatment. The Hyperoxaluria Global Alliance (HGA) is a program sponsored by the Oxalosis & Hyperoxaluria Foundation (OHF).

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