CYSTINOSIS RESEARCH NETWORK

# SCIENTIFIC SYMPOSIUM

## Advancing the Future of Cystinosis Care

**Friday, November 13, 2026 | Weber's Hotel, 3050 Jackson Ave, Ann Arbor, MI 48103**

**Registration is required to attend. Breakfast, lunch, and reception are included within registration.**

**Presentations in the Atrium Ballroom | Breakfast & Lunch in the East + West Ballroom**

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| **7:30a - 8:30a** | Breakfast and Symposium Check-in |
| **8:30a - 8:45a** | Welcome \| Cystinosis Research Network \| Elizabeth Ames, MD, PhD |
| **8:45a - 9:15a** | Newborn Screening for Cystinosis – Implementation and Insights from Germany<br>Katharina Hohenfellner, MD* |
| **9:15a - 10:00a** | Newborn Screening for Cystinosis – Logistics for US Implementation<br>Elizabeth Ames, MD, PhD \| Andrea M. Atherton, MS, CGC |
| **10:00a - 10:30a** | Designing Drugs for Lysosomal Storage Disorders \| Jim Shayman, MD |
| **10:30a - 10:40a** | Break |
| **10:40a - 11:10a** | Continuous Cysteamine Exposure from a Once-Daily Prodrug: Translational Rationale for TTI-0102 in Nephropathic Cystinosis \| Patrice P. Rioux, MD, PhD, Thiogenesis CEO |
| **11:10a - 11:40p** | Update on the Development of the Cysteamine Prodrug CF10 \| Herbie Newell* |
| **11:40p - 12:10p** | mRNA Therapy for Cystinosis \| Paul Goodyer, MD |
| **12:10p - 12:40p** | Ready Steady Go Hello: What Good Looks Like in Transition Care \| Arvind Nagra, MD* |
| **12:40p - 1:25p** | Lunch |
| **1:25p - 1:55p** | Foundations of Engagement for Competitive PCORI Grant Applications \| Mathew J. Edick, PhD |
| **1:55p - 2:10p** | PCORI Discussion \| Elizabeth Ames, MD, PhD \| Cystinosis Research Network |
| **2:10p - 2:15p** | Remarks from Amgen Medical Affairs<br>Lance Kruger, PharmD, Amgen US Medical Affairs Lead, Ultra-Rare Disease |
| **2:15p - 2:45p** | Cystinosis Therapy by Sustained Delivery of Cysteamine from Contact Lenses<br>Anuj Chauhan, PhD |
| **2:45p - 2:55p** | Break |
| **2:55p - 3:25p** | Update on the Ph1/2 CYStem Study of Ex-vivo HSC Gene Therapy in Nephropathic Cystinosis<br>Melissa Wigderson, MD, Novartis Global Program Clinical Head |
| **3:25p - 3:55p** | Clinical Trials: Essential, but Challenging for Rare Diseases \| Jess Thoene, MD |
| **3:55p - 4:25p** | Swallowing Dysfunction and Myopathy in Cystinosis \| Melanie G. Krause M.A., CCC-SLP |
| **4:25p - 4:55p** | Cystinosis Guidelines: Next Steps \| Ewa Elenberg, MD |
| **4:55p - 5:00p** | Closing Remarks \| Elizabeth Ames, MD, PhD \| Cystinosis Research Network |
| **5:00p - 6:00p** | Reception hour |

*Presenting Virtually

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