Children's Cardiomyopathy Foundation™

A Cause for Today. A Cure for Tomorrow.

CCF at NORD Breakthrough Summit

2026 NORD Rare Diseases & Orphan Products Breakthrough Summit | Oct. 24-27, 2026 | Washington, D.C.

900+
Attendees

100+
100 NORD member organizations

7,000+
Rare diseases

30million
Affected Americans

KEY ADVOCACY PRIORITIES

NORD® IAMRARE® Registry Leaders Community Meeting

- *Registry collaboration: Shared successes, challenges and priorities for patient registries.*
- *Registry data: Using surveys and registry findings to communicate community needs and advance research and policy.*

NORD® Annual Member Meeting

- *FDA modernization: Improving clinical trial endpoints and study design.*
- *Donor engagement: Using storytelling to strengthen donor relationships.*
- *AI and efficiency: Applying AI to streamline operations while maintaining safeguards.*
- *Policy priorities: Discussing shared challenges and priorities across the rare disease community.*

NORD® Rare Diseases and Orphan Products Breakthrough Summit

- *Drug approval reform: Exploring regulatory approaches tailored to rare disease drug development.*
- *AI and research: Connecting genetic, clinical and natural history data to inform treatment development and clinical trial design.*
- *Cross-sector collaboration: Bringing together patients, families, advocates, clinicians, industry and government to advance research and access to therapies.*
- *Individualized gene therapies: Exploring collaboration to advance personalized treatment approaches.*
- *Treatment access: Addressing access to approved therapies and potential effects of Medicaid eligibility changes.*
- *FDA innovation: Examining how AI and machine learning may improve diagnosis, research and outcomes.*
- *Care and leadership: Highlighting NORD's Rare Disease Centers of Excellence and industry strategies to accelerate progress.*

CONNECTIONS MADE

Connected with patient advocacy groups (PAGs) across the rare disease community to discuss successes, challenges, and strategies for developing and improving access to patient resources through CCF.

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WHY THIS MATTERS TO CCF

NIH Research Funding Session

The meetings offered opportunities to learn from peer organizations, share approaches to patient resources and registry data, and follow developments in rare disease research, regulation, technology and policy. These connections and insights can help inform CCF's work supporting children with cardiomyopathy and their families.

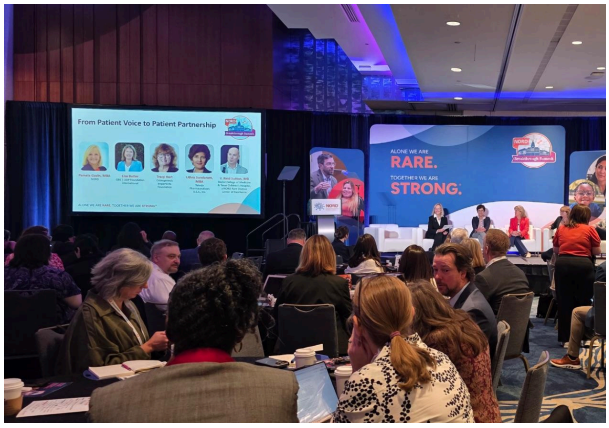


NIH Director Jayanta "Jay" Bhattacharya, MD, PhD, discussed improving access to rare disease treatments and using AI to connect genomic, clinical and natural history data to inform molecular development and clinical trial design.

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From left to right:
Amy Comstock Rick, JD, Associate Director for Rare Disease Strategy, CDER; Director of Strategic Coalitions, FDA Rare Disease Innovation Hub (moderator); Michael Davis, MD, PhD, Director, CDER, FDA; Michelle E. Tarver, MD, PhD, Director, CDRH; Karim Mikhail, B.Pharm, MSc, Director, CBER, FDA



Industry leaders, rare disease advocates and clinicians discussed how partnerships among patients, caregivers, healthcare providers and industry can advance research, clinical trials and patient outcomes.

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