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FDA-NIH Rare Disease Day

Each year, we support this event to raise awareness about rare diseases, the people they affect, and partnerships that promote research and advance knowledge of new treatments.



FDA-NIH RARE DISEASE DAY

Feb. 27–28, 2025 | #RDDatFDANIH

FDA-NIH Rare Disease Day 2025

[Rare Disease Day@FDA](#) takes place worldwide, typically on or near the last day of February each year, to raise awareness among policymakers and the public about rare diseases and their impact on patients' lives. This year, NCATS and the US Food and Drug Administration ("FDA"), both agencies within the Department of Health and Human Services, will sponsor a shared Rare Disease Day event as part of this global observance. FDA-NIH Rare Disease Day aims to provide the rare disease community — patients, caregivers, families, patient advocate groups, researchers, clinicians, health care providers, and trainees/students — with information relevant to their lived experiences, including activities by FDA and NIH that promote research and product development for this

- Initiate a mutually beneficial dialogue among the rare diseases community.
- Exchange the latest rare diseases information with stakeholders to advance research and therapeutic efforts.
- Shine a spotlight on stories told by patients living with a rare disease, their caregivers, and their communities.

FDA-NIH Rare Disease Day seeks to bring together a broad audience including patients, patient advocates, caregivers, health care providers, researchers, trainees, students, industry representatives, and government staff.

Contact

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[See past Rare Disease Day at NIH events.](#)

Registration - coming soon

Event Details

FDA-NIH Rare Disease Day 2025 will be held at NIH Main Campus (Natcher Conference Center) on Thursday, Feb. 27, 2025 and Friday Feb. 28, 2025, from 10 a.m. to 4 p.m. EST. The event will feature panel discussions, rare diseases stories, in-person exhibitors and scientific posters, and an art exhibition. The event is free and open to the public.

Partners in Planning

Planning committee members include representatives from the following organizations:

- NIH: NCATS, Clinical Center, National Cancer Institute (NCI), National Eye Institute (NEI), National Institute on Alcohol Abuse and Alcoholism (NIAAA), National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), National Institute of Neurological Disorders and Stroke (NINDS), Office of Research on Women's Health (ORWH)
- Rare Diseases Clinical Research Network's Coalition of Patient Advocacy Groups (RDCRN-CPAG)
- The Children's Inn at NIH
- FDA: Office of Orphan Products Development (OOPD), Office of the Commissioner (OC), Center for Biologics Evaluation and Research (CBER), Center for Devices and Radiological Health (CDRH), Center for Drug Evaluation and Research (CDER), Oncology Center for Excellence (OCE)
- EveryLife Foundation for Rare Diseases
- National Organization for Rare Disorders (NORD)
- Argenx

NCATS and Rare Diseases Research

Progress in data science and an increased understanding of disease genetics lead experts to agree that more than an estimated 10,000 rare diseases are affecting millions of people in the United States. Rare diseases often are difficult to diagnose — it can take years. Even after an accurate diagnosis, treatment often is not available because approximately 5% of rare diseases have FDA-approved treatments. Research led by NCATS [suggests that nationwide medical costs](#) for individuals with rare diseases are likely as high as those faced by people with common diseases, such as cancer and heart failure.

NCATS is committed to using research to address the [public health crisis](#) presented by rare diseases. NCATS Director Joni Rutter recently [wrote about using translational science](#) to make progress in rare diseases research, including in understanding rare disease biology and therapeutic targets and in building platform approaches that advance drug discovery and development. NCATS' [Division of Rare Diseases Research Innovation \(DRDRI\)](#) facilitates and coordinates NIH-wide research activities, which have the potential to speed development of treatments for multiple rare diseases and ultimately help more patients more quickly. Two recent examples of DRDRI work in this space include [new FDA designations for gene therapies in development](#) through the Bespoke Gene Therapy Consortium, a public-private partnership, and a [proposed clinical trial for a rare metabolic disease](#).

Learn more about DRDRI and NCATS' [rare diseases research programs](#) and the center's [impact on rare diseases](#).

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