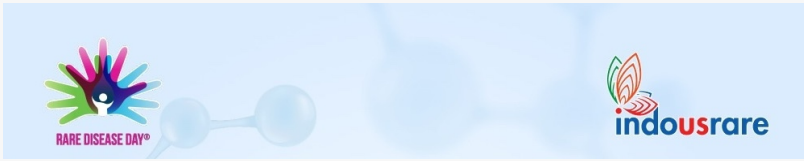
Rare Diseases at Crossroads: Building on 42 Years of Progress Powered by the Orphan Drug Act

Uniting the World for an UnCommon Cause



Rare Disease Day at IndoUSrare - Bridging Nations, Empowering RARE Lives

HERNDON, VA, UNITED STATES, March 11, 2025 /EINPresswire.com/ -- On February 28, 2025, global landmarks lit up in green, blue, purple, and pink for Rare Disease Day, an annual event raising awareness and advocating for improved care and research. However, this year, the NIH and FDA postponed their events, removing a vital platform for American families to engage with experts. IndoUSrare stands in solidarity with the rare disease community, working to navigate challenges and opportunities in this evolving landscape.

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>
> *Dr Mathew T. Thomas, former US FDA Director of the India Office*

Over 400 million people worldwide suffer from one or more of around 11,000 identified rare diseases. Today, awareness about the devastating effects of these diseases is at an all-time high. Rare Disease Day is a crucial platform advocating for early diagnosis, better treatments, and stronger policies, with hundreds of events in over 100 countries shining a spotlight on the cause.

In the US, numerous events marked Rare Disease Day. The University of Minnesota (UMN) hosted its 13th annual program. Dr. Reena Kartha, Director of Research Programs at IndoUSrare and Associate Director at UMN's Center of Orphan Drug Research, says, "This year we focused on getting the perspectives of individuals with lived experiences on navigating rare disorders. Speakers explored the complex realities surrounding rare disorders from a variety of angles—addressing the social, economic, and psychological hurdles encountered by patients and their families." The EveryLife Foundation's 14th Annual Rare Disease Week on Capitol Hill brought together nearly 1,000 advocates from 49 US states for over 300 Congressional meetings. On February 28th, every member of Congress received a copy of a petition signed by 10,000 advocates, including IndoUSrare, urging Congress

to support robust federal leadership, biomedical research funding, and public health resources.

In India, awareness efforts included walks, runs, and educational events. Dr. Mathew T. Thomas, IndoUSrare Board Member, and former US FDA Director of the India Office, states, “In India, the awareness of rare diseases, awareness of the plight of all associated with patients with rare diseases and awareness of the needs of the patients with rare diseases is expanding. However, the pace of advancement must be speeded up, and public-private partnerships and/or collaborations may expedite the process of finding much-needed and affordable solutions for the entire rare disease community.”

Dr. Harsha Rajasimha with Jenifer Waldrop at the RDDC RISE Awards ceremony. Dr Rajasimha was conferred the Caregiver RISE Award 2025

Combating Rare Diseases by Fostering Cross Border Collaborations, Data Sharing, and Clinical trials

Harsha Rajasimha, IndoUSrare’s Founder and Executive Chair, received the 2025 Caregiver RISE Award from the Rare Disease Diversity Coalition (RDDC). Senator Bill Cassidy (R-LA) won the Congressional RISE Award. Senator Cassidy is well known for championing rare disease legislation, including the RARE Act. Jenifer Waldrop, RDDC Executive Director, said, “At the Rare Disease Diversity Coalition, we are deeply honored to recognize Harsha Rajashima as the 2025 Caregiver RISE (Resilient, Impassioned, Strong, and Empowered) Award recipient. Harsha’s unwavering dedication to health equity and his profound commitment to rare disease advocacy exemplify the very spirit of this award. Through IndoUSrare, the nonprofit he founded in honor of his daughter, Harsha has transformed personal loss into a powerful force for change,

ensuring that families navigating the rare disease journey—both in the U.S. and India—are met with the support, resources, and community they deserve. His tireless efforts inspire us all, and we celebrate his impact in driving equity and access for rare disease patients and caregivers everywhere."

IndoUSrare's annual Rare Disease Day event featured a fireside chat with Dr. Madhulika Kabra, former head of pediatrics at AIIMS New Delhi. The event also featured Aisling Finn, a UK-based RARE parent and poet. The event emphasized cross-border collaboration and shared experiences.

The US leads the world in rare disease research. Spurred by the Orphan Drug Act of 1983, there are 1303 US FDA-approved orphan drugs (ODs), with an estimated 7561 in development and 6.9 billion US dollars in federal funding for rare disease research projects in 2023. The Rare Disease Innovation Hub and Project ASHA exemplify efforts to drive innovation and foster global collaborations for rare diseases and clinical trials, including with India.

However, policy shifts under the current administration have raised concerns. President Trump's push to streamline FDA approvals aims to accelerate drug development, yet uncertainties in leadership and funding cuts have caused panic in the rare disease community. The cancellation of the NIH-FDA Rare Disease Day event follows research funding pauses and scientist layoffs, stalling critical progress. In December, Congress failed to renew legislation incentivizing orphan drug research, further destabilizing momentum.

John Crowley, President and CEO of the Biotechnology Innovation Organization (BIO), said, "What's needed is a more precise approach to streamline and modernize agencies like the FDA so that they work both better and faster for all those in need. That's what it will take to unlock the full potential of America's life sciences sector and to build the nation of miracles that our children need — and deserve."

India has emerged as a key player in rare disease policy and innovation. The 2021 National Policy for Rare Diseases (NPRD) and 2019 New Drugs and Clinical Trial Rules established a regulatory framework for orphan drugs. In 2024, India's CDSCO approved the import of orphan drugs from the U.S., UK, Japan, Australia, Canada, and the EU, improving treatment access. India's pharmaceutical industry is also making an impact, producing affordable off-patent orphan drugs for domestic and global markets.

Despite these advances, gaps in financing and high treatment costs remain challenges. A recent Scrip Citeline article highlighted ongoing legal battles and policy hurdles limiting timely access to rare disease therapies. Extending policies

At the heart of IndoUSrare's mission is its commitment to uniting nations in the fight against rare diseases. Key partnerships between the US, France, and India are shaping the future of rare disease research, pooling resources, expertise, and knowledge. Despite international trade

concerns, US-India relations remain strong. Prime Minister Modi's visit to Washington D.C. reaffirmed commitments to expanding cross-border collaborations, aligning with IndoUSrare's goal to strengthen the global rare disease ecosystem.

To further discussions on these critical issues and foster collaborations to drive progress in rare disease research and advocacy, IndoUSrare invites stakeholders to the Indo US Bridging RARE Summit, November 3-4, 2025, at George Mason University, Manassas, VA

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