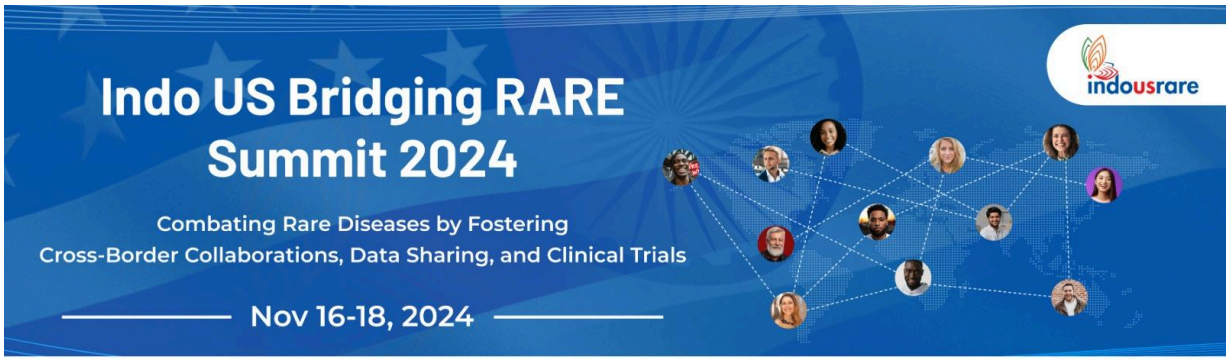

Indo US Bridging RARE Summit 2024

Nov 16 – 18, 2024
Indian National Science Academy (INSA)
New Delhi, IN

PROGRAM OUTLINE

The Indo US Bridging RARE Summit 2024 brings together key leaders representing all stakeholders of rare diseases from across the world with a specific focus on the US, and the Indian subcontinent.

Main Themes:

- Cross Border Patient Engagement
- Indo-US Contrast in Care Pathways: Screening, Diagnosis, and Treatment Options
- Data Privacy and Governance Across Borders
- Digitization of Rare Diseases - Registries, Emerging Markets
- Diversity, Equity, Inclusion, and Access (DEIA) & Globalization for Orphan Drugs
- Orphan Drug Clinical Trials
- Regulatory Pathways for Orphan Products
- Drug Repurposing and Generics for Rare Diseases

Highlights of the Hybrid Event:

- Keynote talks
- Abbey Meyers Khushi Bridging RARE Awards and Gala Celebration
- Networking with all stakeholders from the US and India in person and on the virtual platform.
- Pre-conference workshops
    - Clinical Trial Readiness for Clinical Researchers
    - Patient Advocacy
    - Caregivers
    - Clinical Genomics for Screening and Dx
- Startup Pitch Contest for startups focused on addressing unmet needs in rare diseases with diagnostics, therapeutics, or digital health
- Join working groups to shape the future of innovation in rare diseases
- Poster sessions and Exhibits

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Day 1 - Sat, Nov 16, 2024

Pre-Conference Workshops and Inaugural Gala

Session	Speakers Co-Chairs	Time (IST)
Registration opens		11:30 am
Pre-conference Workshops Concurrent Sessions		
1: **Clinical Trial Readiness for Clinicians** - What does it take to qualify a physician's clinic or hospital as a clinical trial site? - What qualifications or certifications are needed for a doctor to assume the role of Principal or Sub- or Co-investigator? - Survival guide for sites to survive a GCP audit by regulators such as the FDA or CDSCO - Driving patient-centricity, efficiency, and productivity, in clinical trials	- **Harsha Rajasimha, PhD, *IndoUSrare and Jeeva Clinical Trials*** - **Dr. Rakesh Lodha, *AIIMS, Delhi*** - Michelle Romero, *Consultant to CROs and IRB member* - Dr. Mathew T. Thomas - *Former USFDA India Office Country Director* - Prof. Bangarurajan, *JSS College of Pharmacy*	2:00 pm - 5:00 pm
2: **Professional Patient Advocacy & Legislative Advocacy** ● Who is a patient advocate? ● Goal setting for patient advocates ● What has patient advocacy achieved? ● Role of patient advocacy in drug development ● How to be an effective communicator as a patient or caregiver? ● Involving Industry and Academics in Drug Development	- **Jean Campbell, *Professional Patient Advocates in Life Sciences (PPALS)*** - **Dr. Ratna Devi, *DakshamA Health*** - Mohua Chakraborty Choudhury, PhD, MPH, *IISc* - David Rintell, Ed.D, *BridgeBio* - Nita Patel, BSN, *Ex-Amicus Therapeutics* - Nahid Zaman, *Astrazeneca India* - Dr. B.S. Charan, *DGHS*	12:00 pm to 2:30 pm
3: **Emotional Wellbeing for Caregivers, Patients, & Professionals** Occupational and physical therapies relevant to rare diseases Self-care, managing emotions, and guilt	- Cristol O'Loughlin, *Raregivers™* - Vaishali Pai, *Tamahar Trust*	2:30 pm - 5:00 pm

4: **Clinical Genomics for Screening and Dx**	Team Premas Life Sciences - Debjana Saha, PhD - Dr. Rahul Ramekar - Lakshman Teja - Dr. Vikas Sharma - Priyanka Singh - Ratna Dua Puri, MD, DM, *SGRH* - Neerja Gupta, MD, DM, AIIMS, Delhi	2:00 pm - 5:00 pm
Bridging RARE Gala and Reception **Venue: Swimming Pool, AIIMS Gymkhana, New Delhi** • **Art Exhibit - in partnership with Beyond the Diagnosis** • **Prayer and Invocations** • **Inauguration and Lamp Lighting** • **Abbey Meyers Khushi Bridging RARE Awards Ceremony** • **Keynotes** • **Patient Community Voices** • **Entertainment** • **Dinner and Networking**	**Chief Guest:** **Dr. Chandra Sekhar Pemmasani -** Minister of State for Rural Development and Communication **Keynote:** **Abbey Meyers Khushi Bridging RARE Award Honorees:** - **Dr. Rahul Purwar, Dr. Hasmukh Jain** - **Frank Sasinowski, JD, MPH** **Felicitation** Dr. Abbey Meyers, *Founder, NORD* **Patient community voices:** - Gautam Dongre, *NASCO* - Shikha Metharamani *PWSA - India* - Sunil Ladwa, *FOP India* **Performances:** • Tabla recital - Saksham Mishra • BharataNatyam performance - Mansa Gautam and Raunika Negi • Vocal performances - Chetan Chawla and Ms Ridisha	6 00 pm - 9:00 pm
Close of Day 1		

Agenda is Subject to Change

Day 2 - Sunday, Nov 17, 2024

Session	Speakers and Co-Chairs	Time (IST)
Registration opens		8:00 am
Welcome to the Summit and Opening Remarks	**Madhulika Kabra, MD** *All India Institute of Medical Sciences, New Delhi (AIIMS-Delhi)* **Harsha Rajasimha, MS, PhD** *Indo US Organization for Rare Diseases & Jeeva Clinical Trials*	9:00 am - 9:30 am
Opening Keynote Success stories from the IRDiRC working groups and what we can learn from other countries	**David A Pearce, PhD** *International Rare Disease Research Consortium (IRDiRC)*	9:30 am - 10:00 am
Session 1: Epidemiology of Rare Diseases and Digitization • Why are patient registries a critical prerequisite for advancing orphan therapy development? • Critical success factors while designing and implementing patient registries • It's a patient-led revolution • How policymakers can utilize epidemiology and registries • Status and plans for national registries in India and the USA	**Co-Chairs:** • **Madhulika Kabra, MD,** *AIIMS Delhi* • **Paul Mehta, MD,** *CDC/ATSDR* **Speakers/Panelists:** - Dr. Manoj Srinivasa, *CHG* and Dr. Ravi Hiremagalore, *CHG* - Dr. Amlin Shukla, *ICMR* - Nisha Venugopal, PhD, *IndoUSrare* - Kim Bloemendal, *Sanofi*	10:00 am - 11:10 am
Networking Coffee (Posters and Exhibits) 11:10 am - 11:40 pm		
Session 2: Screening and Diagnosis • What have we learned from newborn screening programs in India and the US? • What does it take to make newborn screening mandatory in India? • Quality – why it matters in clinical genomic diagnosis • Cross-border data sharing and broader implications	**Chair:** • **Vijay Chandru, PhD, FNAe, FASc** *Strand Life Sciences* **Speakers/Panelists:** - K Thangaraj, PhD, *CSIR-CCMB* - Seema Kapoor, MD, *Maulana Azad Medical College* - Inderneel Sahai, MBBS, *Massachusetts General Hospital* - Reety Arora, PhD, *CrisprBits* - Shaiket Deb, *Strand Life Sciences* - Suruchi Aggarwal, PhD, *Medgenome*	11:40 pm - 12:50 pm
Industry R&D Presentations	• Ganesh Sangle, PhD, *Shivanka Research*	12:50 pm - 1:20 pm

• Reducing the dosage of orphan drugs to minimize long-term adverse effects • Affordable diagnostics • How biopharma industry can influence public policy	• Vamshi Krishna Thamtham, MD, *LalPath Labs* • *AstraZeneca India Representative*	
Lunch: 1:20 pm - 2:10 pm		
Session 3: Care Pathways and Novel Therapies • Emerging novel therapies for rare diseases • Development of Indigenous counterparts • Drug repurposing for accessible therapies • Prioritizing Innovation, Accessibility, and Affordability • Success stories and most promising opportunities	**Co-Chairs:** • **Neerja Gupta, MD, DM,** *AIIMS-Delhi* • **Reena Kartha MS PhD,** *IndoUSrare and University of Minnesota* **Speakers/Panelists:** - Dr. Ashish Gupta, *University of Minnesota* - Dr. Jayandharan Rao**,** *IIT Kanpur* - Madhushudhan V, *LifeArc*	2:15 pm to 3:25 pm
Networking Coffee 3:25 pm - 3:40 pm		
Patient Voices • Living with a rare disease • Caregiving for a child with a rare disease • Common challenges across multiple rare diseases • Disease-specific challenges • What's working and what's not working for patients?	**Moderator: COE representative** **Speakers:** • Christina Raj, *CIRMF* • Saurabh Singh, *RDIF* • Nikhil Jayswal*, IBD India* • Srijan Mittal, *Myositis India* • Nadir Aman, *CDKL5 South Asia*	3:40 pm to 4:15 pm
Session 4: Industry Priorities for US-India Partnerships • Commercial launch of orphan drugs in India and LMICs • Manufacturing • Inclusion of Trial Sites in India • Role of patient advocacy in orphan drug development	**Moderator:** **Anish Bhatnagar, MD,** *IndoUSrare and Soleno Therapeutics* **Speakers/Panelists** - Parva Purohit, PhD, *Shivanka Research* - Pankaj Bhargava, MD, *ashibio* - David Rintell, Ed.D, *BridgeBio* - Anil Raina, MPIB, *Biopharma Executive*	4:15 pm to 5:30 pm
Networking Posters and Exhibits **Buffet Dinner 6:00 pm - 8:00 pm**		
Pitch4RARE **Start-Up Pitch Contest**	**Judges Panel:** - James A. Levine, MD, PhD*, Fondation Ipsen*	5:45 pm to 8:45 pm

Selected startups pitching their solutions to pressing unmet needs of patients with rare diseases	- Priyankana Mukherjee, PhD, *IKP Eden* - Amrit Ray, MD, MBA, *Biopharma Executive, Rare Parent* - Pankaj Bhargava, MD, *ashibio* - Dr. Dayaprasad Kulkarani, *AarogyaSeva* - Anish Bhatnagar, *MD, IndoUSrare & Soleno Therapeutics* - Dr. Krishna Subramanian, *Seelos Therapeutics* - Anand Kannan, *IFIA Bharat*	
Close of Day 2		

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Day 3 - Monday, Nov 18th, 2024

Session	Speakers Co-Chairs	Time
Recap of Day 1 & 2	**Ratna Puri, MD, DM,** *SGRH*	9:00 am - 9:10 am
Keynote Address: **Recent FDA regulatory progress and perspectives on cell and gene therapies**	**Peter Marks, MD, PhD** *CBER, USFDA* Introduction by Dr. Mathew T. Thomas, *Former USFDA India Office Country Director*	9:10 am - 9:30 am
Keynote Address **Massive Global Inequities in Access to Orphan Therapies: Perspectives from a Biopharma Executive and Rare Parent**	**Amrit Ray, M.D., M.B.A.,** *Biopharma Executive, Rare Parent*	9:30 to 9:50 am
Session 5: Evolving Policy and Regulatory Dynamics: The Time is Ripe for US-India Strategic Collaboration • NPRD and NDCTR in India • Biosecure ACT and IRA in the US. • FDA Project Asha for Cancer Clinical Trials • FDA START program • COGENT • Rare Disease Innovation Hub	**Co-Chairs:** - **Dr. Mathew T. Thomas,** *Former USFDA India* - **Dr. Geeta Jotwani,** *ICMR* **Speakers/ Panelists:** - Prof. Bangarurajan**,** *JSS College of Pharmacy* - Dr. B S Charan, *DGHS* - Bharti Sahai, IAS, *MoHFW* - Dr. A Visala, *CDSCO* - Dr. Krishna Subramanian, *Seelos Therapeutics*	9:50 am - 11:00 am
Networking Coffee (Posters and Exhibits) 11:00 am - 11:20 am		
Session 6: Global Inclusion and Access to Orphan Drugs • Diversity, Equity, Inclusion, Access in the Global Context • Current massive inequity in access • Focus on Sickle Cell Disease epidemiology for DEIA • Reimbursements and Pricing Orphan Drugs in India • Legislations and Regulations Impacting DEIA	**Co-Chairs:** • **David A Pearce, PhD,** *IRDiRC* • **Anil Raina, MPIB,** *Biopharma Executive* **Speakers/Panelists:** - Ginger Davis, PhD, *SCTPN* - Harpreet Ram, MBA, *GARDAccess* - Jenifer Waldrop, MSHRD, *Rare Disease Diversity Coalition* - Dr. Manisha Badak, *National Insurance Corporation*	11: 20 am to 12:30 pm
Rare Disease Hackathon Selected presentations	**Dr. Daya Prasad Kulkarani,** *AarogyaSeva*	12:30 pm to 1:00 pm

Lunch 1:00 pm - 2:00 pm		
Session 7: Advancing rare diseases: Lessons and Case Studies from Neuromuscular disorders • Rare Care Pathways to Success for ALS • Contrast in Care Pathways and Access in US/India • Global Access to Duvyzat for Muscular Dystrophy • Commonalities and differences among various muscular dystrophies	**Chair:** • **Dr. Sheffali Gulati,** *AIIMS, New Delhi* **Speakers/Panelist:s** • Jaime Raymond, MPH, *CDC/ATSDR* • Rama Murali, *BharathMD Foundation* • Alok Bhattacharya, PhD, *WWGM* • Archana Panda, *CureSMA Foundation*	2:00 pm - 3:10 pm
Perspectives from India's Centres of Excellence in Rare Diseases • NPRD and the role of CoEs in India • Current status of implementation of NPRD • Recent high court order and its implications for patients • Barriers for patients to benefit from NPRD • How CoEs are driving "patient centricity" • What advice would CoEs have for patients and advocates?	**Co-Chairs:** **Shubha Phadke, MD, DM,** *SGPGI, Lucknow* **Ashish Gupta, MD, MPH,** *UMN* **Speakers/Panelists** - Bhavna Dhingra, MD, DNB, *AIIMS Bhopal* - Sankar V. Hariharan, MD, DM *SAT Hospital, Trivadrum* - Kuldeep Singh, MD, DM, FAMS, *AIIMS Jodhpur* - Sanjeeva GN, DCH, DNB, *Indira Gandhi Institute of Child Health, Bengaluru* - Prajnya Ranganath, MD, DM, *NIMS, Hyderabad*	3:10 pm to 4:00 pm
Networking Coffee) 4:00 pm - 4:20 pm		
Session 8: *BREAK OUT WORKING GROUPS with Whiteboarding* Developing a Blueprint for US-India Strategic Cooperation for Rare Diseases Research and Orphan Drugs Development	**Leaders:** • Harsha K Rajasimha, MS, PhD • Amrit Ray, MD • Pankaj Bhargava, MD	4:20 pm to 5:15 pm
WORKING GROUP 1: NBS, Screening, Diagnosis, Patient Registries, Referral Networks *WORKING GROUP 2: Strategy to Achieve Equitable Representation of Indian Diaspora in CRAACO, Clinical Trials* *WORKING GROUP 3: Legislative, Regulatory, Financial Advocacy, the ASK: Indian and US*	*All participants pick a working group to lead or contribute or participate in* *WG Leaders:* • Dr. Seema Kapoor, *MAMC* • Dr. Ratna Dua Puri, *SGRH* • Dr. Ratna Devi, *DakshamA Health* • Rama Murali, *BharatMD Foundation*	

Legislators, CDSCO and FDA. E.g., ASHA program for Cancer Clinical Trials. *WORKING GROUP 4: BioSecure ACT and Inflation Reduction Act of USA and Food/Drugs/Cosmetics Act and NPRD of India - How India can play a greater role in affordable Innovation, R&D, and Manufacturing?*	• Anil Raina, MPIB • Dr. Mathew T. Thomas, *Former USFDA India Office Country Director*. • Parva Purohit, PhD, *Shivanka Research* • Ganesh Sangle, PhD, *Shivanka Research*	
Action Items from Working Groups Summaries to be published in Proceedings Article	Working Group Leaders	5:15 pm to 5:30 pm
Closing Remarks **Vote of Thanks**	**Madhulika Kabra, MD** *All India Institute of Medical Sciences, New Delhi (AIIMS-Delhi)* **Harsha Rajasimha, MS, PhD** *Indo US Organization for Rare Diseases & Jeeva Clinical Trials*	5:30 pm to 6:00 pm
Networking and Dinner on Your Own		

Agenda is Subject to Change

Close of Day 3

Summit Ends

Local Tours of Delhi and Taj Mahal on Your Own