indousrare
Indo US Organization for Rare Diseases

Indo US Bridging RARE Summit 2023

Connecting Indian Diaspora with Rare Disease Research and Clinical Trials Globally

OCT 29-30, 2023

George Mason University, Arlington, VA, USA

@IndoUSrare

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MARK R. WARNER
VIRGINIA

UNITED STATES SENATOR
WASHINGTON, D.C.
October 29, 2023

Dear Friends,

I am pleased to extend warm greetings to all who are gathered for the Indo US Bridging RARE Summit 2023.

We are so fortunate to have a thriving Indian-American community in both Virginia and the United States. I take special pride in serving as Co-Chair of the bipartisan Senate India Caucus—one of the largest country caucuses in the United States Senate—which is a platform that allows me to stay engaged while continuing to learn and advocate for the community's diverse interests. It is also an indispensable tool that enables me to work to deepen the United States-India relationship, including by establishing and maintaining robust strategic partnerships across a range of domains from technology, telecommunications, and defense, to global health, research, and clean energy.

I am pleased to see so many experts, policymakers, health care professionals, researchers, industry representatives, patient advocacy groups, and rare disease patients from the India Diaspora join together for this special two day event. I hope that you will leave the summit with a better understanding of rare disease research and clinical trials that are occurring around the globe.

I send my best wishes for an informative and successful event.

Sincerely,

Mark R Warner

MARK R. WARNER
United States Senator

तरनजीत सिंह संधू
Taranjit Singh Sandhu

सत्यमेव जयते

भारत का राजदूत
AMBASSADOR OF INDIA
2107 Massachusetts Ave. N.W.
Washington, D.C. 20008

23 October 2023

MESSAGE

I extend my best wishes to the first annual Indo-US Bridging RARE Summit being held in Arlington, Virginia on October 29-30, 2023. I welcome the Summit's focus on accelerating India-US cooperation on rare disease research and development. I do hope that discussions at the Summit produce innovative solutions benefiting rare disease communities across the globe.

2. India-US healthcare and knowledge partnership is crucial for both our countries, and for the world. Our robust cooperation in areas such as health policy, vaccine development, biomedical research, supply chain management, and streamlining regulatory procedures has contributed to the strength and sustainability of our healthcare systems. Both bilaterally and via mechanisms such as the Quad, we are jointly working with third countries on pandemic response, therapeutics, sharing of best practices, and many other areas. With the rising number of skilled healthcare professionals in India, and our expertise in ensuring affordable access to high-quality medical products, opportunities for further collaboration are immense.

3. The Indo-US Bridging RARE Summit is also a facet of this bilateral cooperation, and will contribute towards strengthening it. By providing a platform for knowledge sharing and discussion, the Summit has the potential to generate valuable ideas and research. I congratulate the Bridging RARE organizing committee, and once again wish the Summit all the very best.

Taranjit S. Sandhu

(Taranjit Singh Sandhu)

GERALD E. CONNOLLY
WASHINGTON, D.C.

October 29, 2023

Dear Friends,

I'm pleased to welcome you to Northern Virginia for the inaugural Indo-US Bridging RARE Summit. I first want to thank the summit planning committee for organizing this gathering with leaders from across the world with a specific focus on the US and the Indian subcontinent.

Invoking trusted biopharmaceutical partnership and collaborating research efforts can expedite the development of treatments for rare diseases, expand accessibility to regions in pressing need, and pave the way for more inclusive clinical trials. It's important to forge a path for affordable clinical innovation via strengthened Indo-US collaborations, raise awareness in as many nations as possible, and develop more inclusive clinical trials for minimizing the devastating health consequences arising.

In Congress, I'm proud to serve as a member of the Rare Disease Caucus. I share the concerns of many impacted by rare diseases. As a member of the Rare Disease Caucus, I will continue working to advance legislation, education regarding specialized and uncommon health issues and diseases, and promote patient advocacy.

I also want to congratulate Dr. William A. Gahl and Dr. Ishwar Chander Verma for receiving the Abbey Meyers Khushi Bridging RARE Award for their dedicated lifetime of achievements fostering cross-border collaborations in the field of rare genetic and undiagnosed diseases.

Please have a wonderful summit and enjoy the evening.

With warmest regards,

Gerald E. Connolly
Member of Congress
11th District, Virginia

ALL INDIA INSTITUTE OF MEDICAL SCIENCES

DEPARTMENT OF PEDIATRICS
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October 25, 2023

Harsha K Rajasimha, PhD
Founder &Executive Chair,
Indo US Organization for Rare Diseases (IndoUSrare)
13687 Neil Armstrong Ave Herndon, VA 20171

Dear Dr Rajasimha:

As a member of the organizing committee for this Summit and on behalf of the All India Institute of Medical Sciences, New Delhi, I am pleased to support the first annual Indo US Bridging RARE Summit being held in Arlington, Virginia on October 29th and 30th, 2023.

As an initiative of Indo US Organization for Rare Diseases - a public charity organization addressing the unmet needs of diverse patients with rare diseases, especially from the Indian diaspora - it is a distinct honor to support your effort of building collaborative bridges for all stakeholders in India, the US, and globally, through this conference.

As an initiative of Indo US Organization for Rare Diseases - a public charity organization addressing the unmet needs of diverse patients with rare diseases, especially from the Indian diaspora–I sincerely applaud the efforts of the whole team for addressing the needs of patients with rare diseases and bringing out an outstanding program for the summit for building collaborative bridges for all stakeholders.

I am sure that the Indo US Bridging RARE Summit will provide a platform for knowledge sharing and discussion among experts, policymakers, healthcare professionals, researchers, industry representatives, patient advocacy groups, and rare disease patients from India and the United States. This would help in learning from the experiences of both countries by fostering a multidisciplinary approach, exploring care pathways, discussing innovative solutions and find ways to integrate with the existing health care systems. I also hope that discussing opportunities for collaborative research and networking would also be an important agenda. My very best wishes for a grand success for the summit.

My heartiest congratulations to this year's awardees of the Abbey Meyers Khushi Bridging RARE Award – Dr. Ishwar Chander Verma who is my mentor too and Dr. William A Gahl, for their lifetime of dedicated work to foster cross-border collaborations in the field of rare genetic and undiagnosed diseases between US, India, and globally.

I appreciate the time, energy, and resources expended by the Bridging RARE organizing committee, as well as the attendees and speakers at this Summit, to make the event successful. Your commitment to this noble cause will eventually reap significant benefits to the global rare disease community. Please accept my best wishes.

We look forward to collaborating with you to jointly organize the 2nd Indo US Bridging RARE Summit from November 16th-18th 2024 to be held in New Delhi, India.

With warmest regards,

Madhulika Kabra

डॉ. मधुलिका काबरा / Dr. Madhulika Kabra
प्रोफेसर / Professor
आनुवंशिकी एकक (संकाय चिकित्सा विभाग)
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BioHealth Innovation
1 CHURCH STREET, SUITE 801 | ROCKVILLE, MD 20850

October 25, 2023

Harsha K Rajasimha, PhD
Founder & Executive Chair,
Indo US Organization for Rare Diseases (IndoUSrare)
13687 Neil Armstrong Ave Herndon, VA 20171

Dear Dr. Rajasimha:

As a member of the organizing committee for this Summit and Founding CEO of BioHealth Innovation Inc., BHI is pleased to support the first annual Indo US Bridging RARE Summit being held in Arlington, Virginia on October 29th and 30th, 2023.

As an initiative of Indo US Organization for Rare Diseases - a public charity organization addressing the unmet needs of diverse patients with rare diseases, especially from the Indian diaspora - it is a distinct honor to support your effort of building collaborative bridges for all stakeholders in the BioHealth Capital Region, as well as globally, through this conference.

The Indo US Bridging RARE Summit will provide a platform for knowledge sharing and discussion among experts, policymakers, healthcare professionals, researchers, industry representatives, patient advocacy groups, and rare disease patients from India and the United States. By fostering a multidisciplinary approach and exploring innovative solutions, it will bridge the gap between rare disease communities in both nations.

My heartiest congratulations to this year's awardees of the Abbey Meyers Khushi Bridging RARE Award – Dr. Ishwar Chander Verma and Dr. William A. Gahl, for their lifetime of dedicated work to foster cross-border collaborations in the field of rare genetic and undiagnosed diseases between US, India, and globally.

I appreciate the time, energy, and resources expended by the Bridging RARE organizing committee, as well as the attendees and speakers at this Summit, to make the event successful. Your commitment and leadership to this noble cause will reap significant benefits to the global rare disease community and our BioHealth Capital Region. Please accept my best wishes.

Best Regards,

Richard A. Bendis

Richard A Bendis
President and CEO, BioHealth Innovation Inc.

A Personal Message from the Chairperson

Dear Friends and Colleagues,

Welcome to the Inaugural Indo US Bridging RARE Summit 2023!

Congratulations to everyone involved in the rare disease revolution on the 40th anniversary of the United States Food and Drug Administration (US FDA) Orphan Drug Act (ODA) of 1983. On this momentous occasion, we celebrate the progress in the development and the FDA approval of over 1100 orphan drugs by honoring Dr. Abbey S. Meyers, the force behind the ODA. We take this opportunity to honor all patients, families, tireless advocates, researchers, purpose driven orphan drug industry leaders, who have not accepted the status quo but have instead chosen to become warriors and entrepreneurs bending the needle and pursue treatment options for the rare and neglected diseases.

Reflecting on the scientific and medical breakthroughs leading to therapies developed during the last 40 years since ODA was signed into law, we now have some form of FDA approved treatments for about 5-7% of about 11,000 known rare diseases. Over 90% of the rare diseases still remain without any treatment options and are in the earlier stages of research and development (R&D) life cycles. Most of the approved and life saving orphan drugs are not accessible to patients outside of the US and EU. It is encouraging that about 2000 rare disease clinical trials are underway globally. However, less than 2% of them are accessible to patients in the Indian subcontinent or the “rest of the world”. Out of the estimated 400 million patients affected by one of the rare diseases, for a large number of patients, clinical trial might be the best care option or even the ONLY option. These massive global inequities in access to life saving therapies for rare diseases cannot be ignored anymore.

Each rare disease, by definition, affects a small number of persons (less than 200,000 in the US, less than 500,000 persons in India). Despite advances in diagnostic, genomics, and artificial intelligence (AI) technologies, the average time to diagnose a rare disease is still well over 5 years in most countries. If we have to truly achieve

global Diversity, Equity, Inclusion, and Access (DEIA) the entire continuum of patient journey and the R&D process has to be reimagined. Unlike other disease areas, advances in rare diseases are predominantly led by patients and their caregivers. They need to start with the end in mind, ultimately, the approval, availability, affordability of life saving therapies. The commercial viability of orphan drugs and the recovery of the cost of long R&D cycles hinges on early, strategic, and long-term planning of the entire process. We should remove barriers for smaller innovative biotechnology companies (not just the large global pharmaceutical giants) to run clinical trials efficiently on a global scale and create pathways for commercial orphan drugs beyond the US, EU, and Japan. If we expect the next 40 years of ODA progress to significantly leapfrog compared to the last 40 years, we haveto think, plan, act, and push each other differently. Engage the rest of the world in ways not done before. Reimagine the whole process.

The US FDA has announced Operation Warp Speed or START program which will treat each rare disease as a global public health emergency just like we did for the COVID19 vaccine during the pandemic. If we bring the same sense of urgency and speed but orders of magnitude greater cost efficiency to the process, achieving a treatment approval and accessibility for all rare diseases can be brought into sight. This requires innovation and cross-border collaboration among all stakeholders of rare disease, training, education, new regulatory policy frameworks, and data sharing technology platforms. This is why we are here at the Indo US Bridging RARE Summit.

Today, we have a lot of reasons to be excited about new treatment modalities such as mRNA based therapies, antisense oligonucleotide therapies, and cell/gene therapies that are achieving breakthroughs for patients. The Inflation Reduction Act of 2022 is minimizing financial burden on patients but hurting the orphan drugs industry by denying the incentives of ODA for repurposing small molecule drugs. We need to fix this. On the other hand, India has the largest population in the world with over 1.5 billion people. India has adopted a National Policy for Rare Diseases (NPRD) of 2021 with a growing number of centers of excellence to administer a grant of up to 50 lac INR (approx. $61,000) per patient towards the treatment of a rare disease. Why is this not sufficient to start thinking of India as a market opportunity for orphan drugs? These are critical questions to ask very early during orphan drug clinical R&D process so that access issues can be addressed for the long haul.

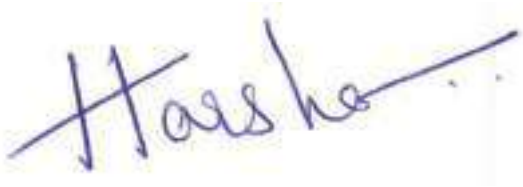
Why is the industry continuing to think of India as a compassionate humanitarian ground for orphan therapies? Furthermore, a Biotech startup from Indian Institute of Technology, Mumbai, succeeded in developing the first CAR-T (Chimeric Antigen Receptor T-cell) therapy for relapsed/refractory B-cell lymphomas and leukemia

and received approval by the Indian regulators (CDSCO) in Oct 2023 making a cure affordable.

The US and India together represent a quarter of the World's population. Opportunities abound to foster cross-border collaborations for breaking barriers and accelerating affordable innovation. I invite you to get involved, get informed, get engaged so we can, together ensure that the next 40 years of ODA will bring affordable rare disease treatments for **ALL PATIENTS.** Please save the dates November 16-18, 2024 for Indo US Bridging RARE Summit in New Delhi, IN. We will be back at this George Mason University venue in 2025. I sincerely thank the planning committee, sponsors, partners, collaborators, and our staff for their tireless efforts and support in putting this Summit together.

When we collaborate, everybody wins! Let's collaborate! We are all in this together!

On behalf of the Summit Planning Committee,



Harsha K Rajasimha, PhD

Founder and CEO, Jeeva Informatics Solutions Inc.

Founder and Executive Chair, Indo US Organization for Rare Diseases

Affiliate Faculty, George Mason University

Table of Contents

About the Bridging RARE Summit

In the 40 years since the Orphan Drug Act was legislated in the USA, there is a growing understanding of the importance of rare diseases that affect over 30 million Americans. While the situation in Europe is similar, no such awareness exists in many parts of the world including India which is home to over a quarter of the world's population. It is time to change that, for the 1.5 billion people in India, and the 8 billion people across the world.

The Bridging RARE Summit 2023 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. The program will explore the challenges and potential solutions for rare diseases with a specific focus on cross border collaborations to drive patient focused drug development.

Themes:

- Cross Border Patient Engagement
- Care Pathways: Screening, Diagnosis, and Treatment Options
- Digitization of Rare Diseases with a focus on Registries and Emerging Markets
- Diversity, Equity, Inclusion, and Access (DEIA), and Globalization for Orphan Drugs
- Orphan Drug Clinical Trials
- Regulatory Pathways for Orphan Drugs

IndoUSrare Announces Bridging RARE Summit to Combat Rare Diseases by Fostering Cross-Border Collaborations, Data Sharing, and Clinical Trials

With an increasing number of U.S. biopharmaceutical firms feeling the financial constraints imposed by the Inflation Reduction Act (IRA) and subsequently shutting down or suspending their research programs in the rare disease domain, hundreds of millions of patients in both the U.S. and India face the grim prospect of not receiving a breakthrough treatment for their condition within their lifetimes. IndoUSrare, a US-based nonprofit committed to stanching rare diseases, launches the inaugural Bridging RARE conference to forge a path for affordable clinical innovation via strengthened Indo-US collaborations and more inclusive clinical trials for minimizing the devastating health consequences arising from this policy shift.

HERNDON, Va., Oct. 11, 2023 /PRNewswire-PRWeb/ -- Rare diseases, as defined by the World Health Organization (WHO), refer to some 10,867 medically significant conditions, each with a prevalence of one or less per 1,000 individuals. With only about 8% of them being treatable,[1] rare diseases include congenital malformations, autoimmune disorders, lysosomal storage disorders, thalassemia, muscular dystrophies, and infrequent forms of cancer and infections. Despite substantial progress during the 40 years since the enactment of the Orphan Drug Act of 1983, which incentivized the U.S. private sector to develop 1,100 FDA-approved orphan treatments,[2] the global thrust against rare diseases is on the verge of losing steam.

Unintended victims of the Inflation Reduction Act

IndoUSrare seeks to collaborate with the GoI, patient groups, and industry stakeholders to foster cross-border collaborations to accelerate research and clinical trials

In August 2022, the U.S. Congress passed the Inflation Reduction Act (IRA) to reduce the cost of prescription drugs borne by patients. While many individuals certainly benefited from the legislation, the 30 million Americans suffering from rare diseases seem to have been left on the wayside.[3] Due to the act's provision that allows certain FDA-approved drugs, including those targeting multiple diseases and small molecule medicines, to be eligible for price negotiation after just seven years, one can anticipate a sustained slowdown in drug development and innovation. This stagnation is

expected to be especially acute in rare disease research, as the field relies extensively on experimentation with repurposed drugs and small-molecule medicine to achieve medical advancements. Alnylam Pharmaceuticals Inc., for example, halted its research on Stargadt treatment in October 2022, explicitly attributing the pause to the IRA.[4] As Alnylam was basing its treatment on a drug that was approved to treat a different condition, the resulting cure would have been subject to price renegotiation.Since its enactment, the IRA has sent shockwaves across the world. Given that the U.S. plays a prominent role in biopharmaceutical research, the abrupt restrictions imposed by the IRA present a major setback to the global battle against rare diseases.

New hurdles on the horizon for India and the Indian Diaspora

One country that is likely to bear the brunt of the deceleration in rare disease research is India, a long-standing partner of the U.S. in this field. With 70 to 96 million living with rare diseases,[5] many of which are unique to the Indian subcontinent, India has its hands full managing these conditions. The Indian government has a great start in 2021 with the Ministry of Health and Family Welfare formulating and adopting a National Policy for Rare Diseases (NPRD) that includes a one-time treatment subsidy of USD 61,000 per patient. The program initially promises to be accessible to 40% of the population and has since established over 11 diagnostic and treatment centers of excellence across the country.[6]

However, this program needs significant expansion to cover all 10,867 (and growing) known rare diseases and not just those small number of currently treatable rare diseases. The policy currently only covers persons afflicted by a subset of the 450 recognized rare diseases. The implementation of NPRD will also need to at least triple the number of centers of excellence to meet the needs of the entire rare disease population of India and provide more accessibility across the country including the rural population. As for the diagnostic and treatment centers, their impact is currently hampered by the absence of formal referral channels established between primary care, secondary care, and tertiary care centers, which hinders patient access.

“A national policy with funding is a great start but is insufficient to cover the treatment of many rare diseases as orphan drugs have to be imported into India,” says Dr. Harsha Rajasimha, founder and executive chairperson of IndoUSrare, a nonprofit centered on combating rare diseases through cross-border collaborations. “Furthermore, many known rare conditions in the U.S. such as ALS (also known as Lou Gehrig’s disease or motor neuron disease (MND)), ultra rare indications caused by single gene mutations such as ADSSL1, DLG4, MNGIE, VCP, and SYNGAP, are not officially recognized by NPRD, thus leaving over 90% of the rare diseases on the fence. Thousands of rare diseases fall under the realm of genomics, precision medicine, patient registries, and clinical research. This is where IndoUSrare seeks to collaborate with the GoI, patient groups, and industry stakeholders to foster cross-border collaborations to accelerate research and clinical trials.” Says Dr. Rajasimha who has also pioneered an intelligent technology platform for efficient implementation of patient registries and clinical trials.

The call for more inclusive research and clinical trials

Considering the rare disease research slowdown in the U.S. and the growing concerns about these disorders in India, increased Indo-US collaboration, particularly in the context of clinical trials, can help mitigate consequences. The U.S. conducts nearly half of the global clinical trials for rare diseases. However, these trials are not representative of the country's ethnic makeup, underrepresenting many diasporas, Indian included. For example, despite India having one-third of all rare disease cases worldwide, only nine of the 202 trials registered in the U.S. were conducted there. Participation of the Indian diaspora in these trials is hardly better; in the 193 trials that did not take place in India, only 1% of participants were of Indian origin.[7] This lack of representation in clinical trials is problematic because it raises questions about the applicability of the findings; different patient groups may respond differently to identical treatments. This is relevant for specific Indian demographics, as their long history of genetic isolation and unique cultural practices may shape the way they respond to treatments.

By failing to capture the ethnic diversity within the U.S., these non-representative clinical trials may not only lead to a misinterpretation of treatment effects on American rare disease patients but also impede India's efforts due to the absence of Indian participants. Indeed, considering the additional costs associated with conducting clinical trials in India, Indian regulators have in the past waived the requirement for representative trials if the orphan treatment has already been approved by the U.S. Food and Drug Administration. Consequently, Indian patients may at times receive treatments may be ineffective for them.

"Without a diverse base of study participants, researchers lack representative data on disease progression and research efficacy," says Frank Sasinowski, MS, MPH, JD, Director at Hyman Phelps McNamara P.C., and Founding Board Member of IndoUSrare.

The need for more inclusive clinical trials is timely because it provides both the U.S. and India with an opportunity to deepen their understanding of the rare diseases affecting both countries — even under the clamping effect of the IRA. Cross-border initiatives, such as those focused on research digitization, data sharing, and the creation of patient registries, can increase the participation of Indian patients as well as those from other groups in these trials.

Coming to a head in the Bridging RARE Summit 2023

Though the U.S. deserves to be congratulated on the 40th anniversary of the Orphan Drug Act of 1983, recent policy changes such as the IRA have stifled biopharmaceutical innovation in the field of rare diseases. This shift is poised to exacerbate India's struggles in addressing these disorders as it endeavors to improve access to orphan drugs and advance rare disease research and treatment.

One way for both countries to alleviate the fallout resulting from the IRA is to invoke their trusted biopharmaceutical partnership. Collaborative research efforts can expedite

the development of treatments, expand their accessibility to regions in pressing need, and pave the way for more inclusive clinical trials.

The intricacies of managing rare diseases, including challenges and potential solutions, will be explored in greater depth during the two-day Bridging RARE Summit 2023. Join the host, IndoUSrare, alongside representatives from the rare disease community, on October 29-30 in this culminating event.

The Summit planning committee has selected two legendary leaders to honor this year with the Abbey Meyers Khushi Bridging RARE Award in recognition of their dedicated lifetime of achievements to foster cross-border collaborations in the field of rare genetic and undiagnosed diseases. The two honorees this year are Dr. William A. Gahl, Senior Investigator at the National Human Genome Research Institute, National Institutes of Health, and Padmashri Dr. Ishwar Chander Verma, Senior Advisor at the Institute of Medical Genetics and Genomics, Sir Ganga Ram Hospital, Delhi. "The U.S. and India possess complementary skillsets and resources in the context of researching and treating rare diseases," says Dr. Verma upon accepting the award. "Collaboration is the way forward."

"Dr. Abbey Meyers is a living legend who was instrumental in the enactment of the U.S. FDA Orphan Drug Act of 1983 which has had immense impact in the U.S. and kicked off a global crusade. By honoring these legends who have advanced research in rare and undiagnosed diseases, the Indo US Organization for Rare Diseases together with the Bridging RARE planning committee seeks their vast wisdom and guidance to carry forward the mission of catalyzing cross-border collaborations for patient-focused research and the next generation of affordable orphan drug development." Says Rajasimha.

IndoUSrare thanks the Gold Sponsors of the Indo US Bridging RARE Summit 2023, Exela Pharma Sciences and Soleno Therapeutics.

About the Bridging RARE Summit:

The Bridging RARE Summit 2023 is organized by the Indo US Organization for Rare Diseases, a non-profit organization based in the Biohealth capital region and a platinum member of the National Organization for Rare Diseases (NORD). The main collaborating U.S. and Indian organizations for the 2023 Summit include George Mason University, Virginia BIO, BioHealth Capital Region Forum, All India Institute of Medical Sciences (AIIMS) New Delhi, Centre for DNA Fingerprinting and Diagnostics (CDFD) India, Global Genes, EveryLife Foundation for Rare Diseases, other collaborators and sponsors. For the full agenda, keynote speakers, Gala, Registration, and Sponsorship opportunities, visit https://bridgingrare.org.

About Indo US Organization for Rare Diseases:

IndoUSrare is a humanitarian nonprofit 501(c)(3) tax-exempt public charity organization based in the United States. Founder and Executive Chairman Dr. Harsha Rajasimha, who lost a child to a rare disease in 2012, has been a rare disease advocate for more than 10 years. To address the unmet needs of diverse patients with rare diseases globally, the leadership team comprised of experienced professionals from research, advocacy, regulatory, and drug development seeks to build cross-border collaborations connecting stakeholders of rare diseases in low- and middle-income regions such as India, with their counterparts and clinical researchers in the United States to improve the diversity of clinical trial participants, accelerate research and development, and improve equitable access to life-saving therapies to diverse populations of rare disease patients. Visit https://indousrare.org.

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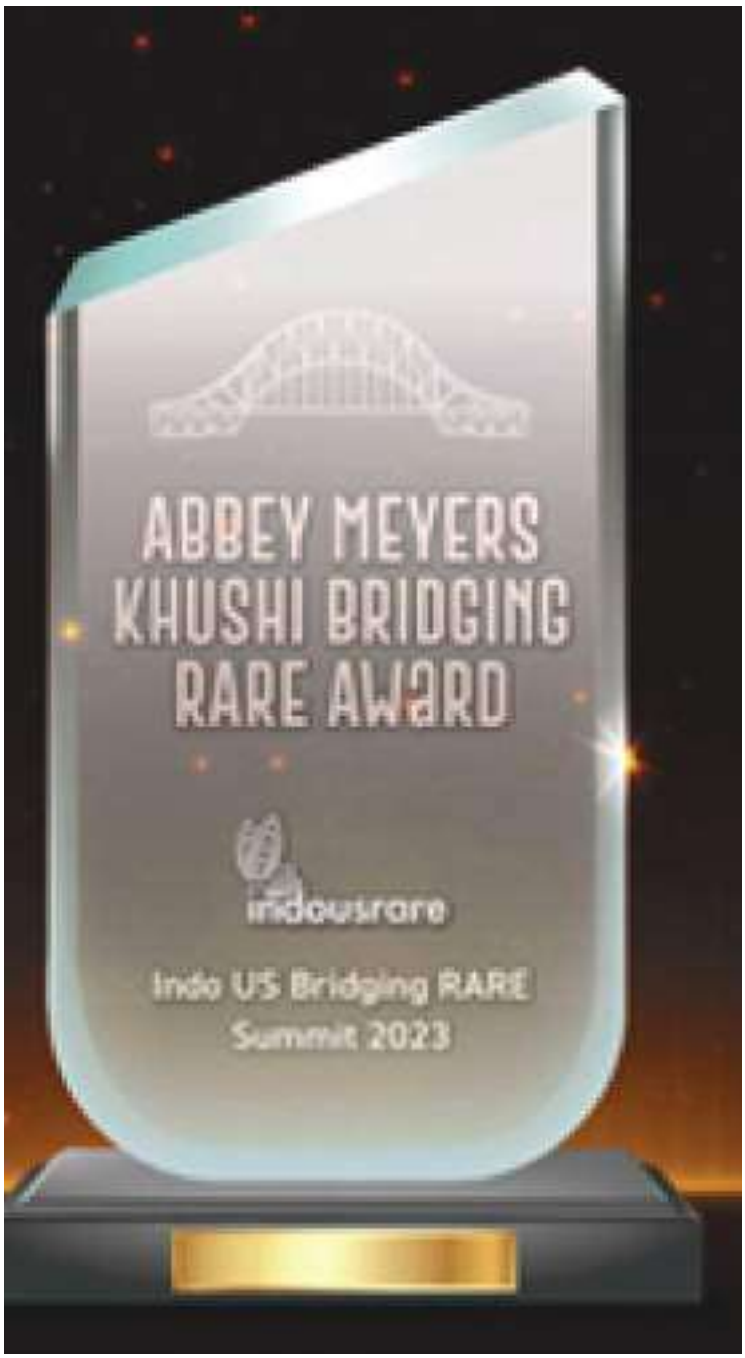
SOURCE JOTO PR™

"Thank you for your work bridging the science of India and the United States to benefit people with rare diseases wherever they may live." — Dr. Abbey S. Meyers

Abbey Meyers Khushi Bridging RARE Award Ceremony

About the Award



The Abbey Meyers Khushi Bridging RARE Award honors the lifetime of dedicated work and accomplishments of two stalwarts who have done groundbreaking work to foster cross-border collaborations in the field of rare genetic and undiagnosed diseases between US, India, and globally.

The two honorees this year are Dr. William A. Gahl, Senior Investigator at the National Human Genome Research Institute, National Institutes of Health, and Padmashri Dr. Ishwar Chander Verma, Senior Advisor at the Institute of Medical Genetics and Genomics, Sir Ganga Ram Hospital, Delhi.

By honoring these legends who have advanced research in rare and undiagnosed diseases, the Indo US Organization for Rare Diseases together with the Bridging RARE planning committee seeks their vast wisdom and guidance to carry forward the mission of catalyzing cross-border collaborations for patient-focused research and the next generation of affordable orphan drug development.

About Abbey Meyers

Dr. Abbey Meyers is a living legend who was the force behind the enactment of the U.S. FDA Orphan Drug Act of 1983 which has had immense impact in the U.S. and kicked off a global crusade. She is the founder and past President of the National Organization for Rare Disorders (NORD), a coalition of national voluntary health agencies and a clearinghouse for information about little known illnesses. Dr. Meyers currently holds the honorary title of President Emeritus of NORD. She has also served as Honorary President of the European Organization for Rare Disorders (EURORDIS) before she retired from her busy life as a leader of the rare disease movement in 2009.

About Baby Khushi

Baby Khushi was diagnosed at birth in 2012 with Trisomy 18 or Edwards syndrome. Her parents were told "she is not a viable baby and that she was not compatible with life" by the neonatologist. Baby Khushi lived for 4 days with the support of life support ventilators in the NICU. This shifted the career trajectory of Khushi's father, Harsha Karur Rajasimha, PhD. The NIH trained genomics data scientist turned into a serial entrepreneur co-founding the national umbrella organization for rare diseases in India in 2013, led the publication of the first peer-reviewed journal article reviewing the challenges and opportunities for rare diseases in India (Rajasimha et al., 2014). Harsha later founded the digital platform company, Jeeva Informatics Solutions, in Virginia to make clinical trials most efficient and universally accessible. In 2019, Harsha founded the Indo US Organization for Rare Diseases recognizing the unmet need to engage the Indian subcontinent with the patient-led orphan drug revolution in the US.
"Khushi" means Joy in multiple Indian languages. Baby Khushi's legacy lives on as she continues to inspire her parents and the Karur family to work towards making it better for all persons affected by rare and undiagnosed diseases.

I cannot begin to tell you how many patients....how many extended families..... have been helped by these two physicians. I...thank them both for their lifetimes of groundbreaking scientific work. — Dr. Abbey S. Meyers

Meet the Awardee: Ishwar Chander Verma

Dr. Ishwar Chander Verma, shortly known as IC Verma, is currently Advisor and Senior Consultant at the Institute of Medical Genetics and Genomics at Sir Ganga Ram Hospital in Delhi. He has spent a lifetime working on raredisorders; from establishing their burden to their diagnosis and prevention. For his stellar contributions in this field, he received the BC Roy – Medical Council of India National Award. He chaired the committee that prepared the first draft of the National Policy for Rare Disorders in India.

He set up the first Genetic center in India – at AIIMS, Delhi in 1968, the first registry for genetic disorders, and was the first to provide advanced genetic tests at low cost using indigenous methods.

Dr. Verma obtained medical education at Amritsar Medical College, post graduate training in medicine and child health from the Royal College London, and Glasgow University. He received Genetic training in USA at MGH in Boston and NIH. He has been involved in a number of Indo-US projects on rare disorders – Genetic studies in deafness with NIH, and Birth defects with March of Dimes and CDC, Atlanta.

He served as Professor of Pediatrics at AIIMS, New Delhi which is a premier Medical institute in India. The Genetic unit he established was recognized as WHO Collaborating Center in Genetics, the only one in Asia. After retirement developed a state-of-the-art-genetic center in Sir Ganga Ram Hospital in 1997. He has been involved for 20 years in investigating rare disorders among tribal communities in Andaman and Nicobar Islands and other regions in India. He has provided prenatal diagnosis and counseling for the prevention of genetic disease to thousands of patients with rare disorders.

He was instrumental in starting a number of patient societies - for thalassemia, mental retardation, muscular dystrophy etc. Dr. Verma has 457 research publications to his credit. He has been editor-in-chief of the Indian Journal of Pediatrics for 25 years, a journal that has the highest Impact Factor of 4.2 among all medical journals in India.

He has fellowship of National Academy of Medical Sciences, India, Fellowship of American Academy of Pediatrics and American Pediatric Research Society. He has served as president of Indian Society of Human Genetics, Indian Academy of Pediatrics Delhi, Society of Fetal Medicine and Indian Society of IEMs. He has made immense contributions to improve the health of patients with genetic diseases and rare disorders in India, for which he has been conferred Padmashri by the President of India in 2023.

"The U.S. and India possess complementary skillsets and resources in the context of researching and treating rare diseases. Collaboration is the way forward."
Dr. I.C Verma

Meet the Awardee: Dr. William A. Gahl

Dr. Gahl's extensive research portfolio has been dedicated to rare inborn errors of metabolism. His research approach encompasses both clinical observations and treatment of patients in the clinic and intricate biochemical, molecular biological, and cell biological investigations in the laboratory.

His groundbreaking work has led to the elucidation of the fundamental defects in rare genetic disorders, including cystinosis and Salla disease. Notably, these discoveries have contributed to the development of innovative therapies, such as cysteamine, which gained approval by FDA for the treatment of cystinosis, improving the lives of patients affected by this rare condition.

Dr. Gahl's commitment to advancing medical knowledge is evident in his extensive publication record, which includes 450+ peer-reviewed papers. His mentorship has been equally impactful, as he has trained 42 biochemical geneticists, nurturing the next generation of experts in the field. Moreover, he extends to leadership roles within prominent medical organizations. He has served on the board of directors of the American Board of Medical Genetics and Genomics and the American Society of Human Genetics. His presidency of the Society for Inherited Metabolic Disorders further underscores his leadership and advocacy efforts within the rare disease community.

He has served on the board of directors of the American Board of Medical Genetics and Genomics and the American Society of Human Genetics. His presidency of the Society for Inherited Metabolic Disorders further underscores his leadership and advocacy efforts within the rare disease community.

Dr. Gahl has been the recipient of Dr. Nathan Davis Award for Outstanding Government Service: Presented by the American Medical Association (AMA), Service to America Medal in Science and the Environment and Election to the National Academy of Medicine. In addition to these accolades, Dr. Gahl received the EURORDIS Lifetime Achievement Award, a prestigious acknowledgment of his lifelong dedication to addressing the needs of individuals living with rare and undiagnosed diseases. This award recognizes his pivotal role in the creation of the National Institutes of Health (NIH) Undiagnosed Diseases Network (UDN) and his leadership in the development of the International Network on Undiagnosed Diseases (UDNI). These initiatives have significantly impacted rare disease research on a global scale.

"IndoUSrare is connecting resources and expertise across international borders by building the much-needed collaborative bridges between silos." — Dr. Marshall Summar, Uncommon Cures

Indo-US Fusion: Indian Classical and Bollywood Dance Production

At the Indo-US Rare Bridging RARE Celebration, Mani and Anagha Sreenivas present to you a dance production celebrating the cultural diversity of India and America with a fusion of Indo-US dance styles.

The production begins with a prayer showcased by the Pushpanjali and Ganesha Stuthi, followed by Contemporary styles, Garba, Jade Kolata, and ends with a grand finale basking in the grand colors of India and America. Experience the beauty of combining both of these cultures through dance as students of Nupura Naadam Dance Academy and various other local dance schools get together to present to you this wonderful production directed by Mani Sreenivas and choreographed by Anagha Sreenivas.

Dr. Mani Sreenivas and Anagha Sreenivas

Dr. Mani Sreenivas serves as the Artistic Director of Nupura Naadam Dance Academy in Virginia. She orchestrates dance workshops and the annual charity event "KARUNYA," collaborating with international artists to create conceptual dance productions involving over 100 performers. Beyond her community involvement and Presidency at the Kaveri Kannada Sangha in 2017, she holds national positions as a Board of Director and Joint Secretary for the Association of Kannada Kootas of North America (AKKA). Mani has organized numerous AKKA conventions and judges local dance competitions. Professionally, she's a medical doctor from India, working on the Clinical Research Team at Johns Hopkins.

Anagha Sreenivas, a versatile artist from Virginia, is a dancer, choreographer, model, and dance instructor. Graduating from Virginia Tech, she holds expertise in Indian classical dance forms, Bharatanatyam and Kathak, with 15+ years of training and completing her Arangetram at age 11. Crowned Miss India DC 2018 and a Top 5 finalist for Miss India USA, Anagha showcased her talents on various TV shows like Dance India Dance, Boogie Woogie, and Jhalak Dikhhla Jaa. She's received acclaim from celebrities like Madhuri Dixit and Saroj Khan. Her YouTube channel has nearly 50,000 subscribers, and she collaborates with her mother, Mani Sreenivas, on cultural dance productions. Beyond dance, Anagha judges competitions and pageants, holding a black belt and the title of Grand Champion Winner in Tae Kwon Do in 2010.

Agenda

Day 1 - Sunday, Oct 29, 2023

All Times Listed are in Eastern Time.

Registration and Breakfast	**7:00 am to 8:00 am**	
Welcome to the Summit and Opening Remarks	**Nisha Venugopal, PhD** *Indo US Organization for Rare Diseases*	**8:00 am to 8:30 am**
Welcome Notes	**Amy Adams, PhD** *Institute for Biohealth Innovation, George Mason University* **John Newby, JD** *Virginia Bio*	
Why IndoUSrare? Why Now? Why are we here?	**Harsha Rajasimha, MS, PhD** *Indo US Organization for Rare Diseases*	
IndoUSrare Programs and Impact	**Nisha Venugopal, PhD** *Indo US Organization for Rare Diseases*	
Session 1: **How Patients and Caregivers are moving the needle in the US and India**	**Harsha Rajasimha, PhD** *Indo US Organization for Rare Diseases*	**8:30 am to 10:00 am**
Patient/Caregiver Keynote *(US)*	**Naveen Baweja, MBA and Priyanka Kakkar** *Cure ADSSL1*	
Patient/Caregiver Keynote *(India)*	**Gautam Dongre** *National Alliance of Sickle Cell Organizations (NASCO)*	
Panel Discussion	**Samir Sethi** *Indian Rett Syndrome Foundation (IRSF)* **Suyog Sathe** *International Gaucher Alliance & Lysosomal Storage Diseases Support Society India (LSDSS-India)* **Dorothea Lantz, MPA** *Prader Willi Syndrome Association* **Alison Bones, BBA** *T.E.A.M. for Travis*	
Networking - Coffee		

Session 2: **What does Diversity, Equity, Inclusion, and Access (DEIA) mean to us today - definitions and inadequacies, and the way ahead**	**Sarah Friedhoff** **Astria Therapeutics**	**10:30 am to 12:00 pm**
Plenary Keynote	**K Thangaraj, PhD** *CSIR - Centre for DNA Fingerprinting and Diagnostics*	
Panel discussion	**Maria G. Della Rocca, MS, PMP** *Global Genes* **Teresa Ginger Davis** *Sickle Cell/Thalassemia Patients Network* **Bonnie Schneider** *IGA Nephropathy Foundation*	

Lunch: 12:00 pm- 1:00 pm

Breakout sessions (12:10 - 12:50 pm)

- **Rare Epilepsies** - *Room 120: Gabi Conecker (Decoding Developmental Epilepsies (DDE)), Prof. Sheffali Gulati, MD, FRCPCH (UK), FAMS, FIAP, FIMSA (AIIMS - Delhi)*
- **Undiagnosed Diseases** - *Room 118: Marlene Soto Riera (Helping Swans Co.), Shifali Gupta, MD (Post Graduate Institute of Medical Education and Research)*
- **Disease "Avatars" & Preclinical Research** - *Room 113: Srujana Cherukuri, PhD (Noble Life Sciences), Srinath TL, PhD (GenoPhe Biotech Pvt Ltd)*
- **Living with a Rare Disease** - *Room 111: Dorothea Lantz, Cristol Barret O'Loughlin, Suyog Sathe*

Session 3: **Care Pathways: Screening, Diagnosis, Treatment, CRAACO** *Focus on differences in care pathways across geographies*	**Reena Kartha, MS, PhD** *Indo US Organization for Rare Diseases*	**1:00 pm to 2:30 pm**
Plenary Keynote	**Madhulika Kabra, MD** *All India Institute of Medical Sciences, New Delhi (AIIMS-Delhi) -* **Harsh Sheth, PhD** *FRIGE - Institute of Human Genetics*	
Panel Discussion	**Ashish Gupta, MD** *University of Minnesota* **Tiina Urv, MD** *National Center for Advancing Translational Sciences (NCATS, NIH)* **Tamanna Roshan Lal, MD** *Uncommon Cures*	
Networking - Coffee		

Session 4: **Digitization of Rare Diseases - Real World Data, Registries, Emerging Markets** *Current status and strategies to increase global representation*	**Harsha Rajasimha, PhD** *Indo US Organization for Rare Diseases*	**3:00 pm to 4:30 pm**
Plenary Keynote	**Subha Madhavan, PhD** *Pfizer, Inc.*	
Panel Discussion	**Sophia Zilber** *Cure Mito* **Paul Mehta, MD** *Centers for Disease Control and Prevention (CDC)* **Dr. Amlin Shukla** *Indian Council of Medical Research (ICMR)*	
Networking Happy Hour: 4:30 pm to 6:00 pm		

Bridging RARE Celebration	Emcee for the evening: **Amulya Karur &** **Rohan Honganoor**	**6:00 pm to 8:30 pm**
Live Music Performance	**Sasanka Sreedevi Naresh** *Briar Woods High School*	
Parent/Caregiver of a Rare Disease Diagnostic Odyssey in the DMV area	**Ms. Urvi Bham**	
A Short Video About Indo US RARE	**Ms. Geetha Bhat**	
Importance of legislative advocacy	**Krystle Veda Kaul, PMP** *Candidate for Congress, VA District 10*	
Inaugural Keynote	**Vikram Karnani, MBA** *Horizon Therapeutics*	
Abbey Meyers Khushi Bridging RARE Awards Ceremony	**Honoree 1:** *Dr. Ishwar Chander Verma* *Sir Ganga Ram Hospital* **Honoree 2:** *Dr. William A. Gahl* *NHGRI, NIH and UDNI*	
Congratulatory Message	**Abbey S. Meyers, PhD** **Founder, NORD**	
Letter of Recognition and Support from Congressman Gerald Connolly	**Sreelakshmi Vayal Veettil** *Intern*	
A tribute to Indo US RARE – *A dance production* **Entertainment and Gala Dinner**	**Dr. Mani and Anagha Sreenivas** *Nupura Naadam Dance Academy*	
Vote of Thanks *Donor and Volunteer Recognition*	**Narayanan Govindarajan, MS** *IndoUSrare* **Ramya T. Karur, MSc** *IndoUSrare* **Juhi Naithani, MBA** *IndoUSrare and bGlobal Consulting*	**8:30 pm to 9:00 pm**

Dance Floor is Open

Close of Day 1 (9:30 pm)

Day 2 - Monday, Oct 30th, 2023

Networking Breakfast 7:30 am to 8:20 am		
Recap of Day 1	**Reena Kartha, MS, PhD** **IndoUSrare**	**8:25 am**
Session 5: **Regulatory Pathways for Orphan Drugs** *Regulatory and policy differences across geographies.* *Developing global frameworks to bring access to treatments everywhere.*	**Frank Sasinowski, JD, MPH** *IndoUSrare & Hyman Phelps and McNamara PC*	**8:30 am to 10:00 am**
Keynote Address (US)	**Peter Marks, MD, PhD** *US Food and Drug Administration (US FDA)*	
Keynote Address (India)	**Dr. L Swasticharan** *Directorate General of Health Services, India*	
Rare Disease Revolution: Bridging Technology, Equity, and Therapies	**Isaac Rodriguez-Chavez, PhD** *4Biosolutions Consulting*	
What are the pathways for rare disease drugs in India?	**Bhaskar Sonowal, MBBS, MBA** *HED Healthcare*	
Gene Therapies	**Sukumar Nagendran, MD** *TAYSHA Therapeutics*	
Global product development: Gaps and considerations	**Sumati Nambiar, MD, MPH**	
Networking Break		

Session 6: **Orphan Drugs Clinical Trials** *Challenges with conducting rare disease clinical trials in areas other than the Western World with an emphasis on India*	**Anish Bhatnagar, MD** *Soleno Therapeutics*	**10:30 am to 11:30 am**
	Moke Sharma, MBA *Bristol Myers Squibb (BMS)* **Rusty Clayton, DO** *Larimar Therapeutics* **Pankaj Bhargava, MD** *āshibio* **Harsha Rajasimha, PhD** *Jeeva Clinical Trials*	
Session 7: **Global Launch Strategies:** *Going beyond US and EU to India & Emerging Markets*	**Anish Bhatnagar, MD** *Soleno Therapeutics*	**11:30 am to 12:30 pm**
	Meredith Manning, MBA *PharmaEssentia Corp* **Mike Blum, MBA** *Homology Medicines* **Matthew Pauls, MBA, JD** *Savara, Inc.* **Neil Kumar, PhD** *BridgeBio*	

Lunch 12:30 pm to 1:30 pm

Roundtable discussions - 12:45 pm to 1:25 pm

- *Mental Wellbeing in Rare Diseases* - **Cristol O Loughlin**
- *AI and Data Sharing* - **Subha Madhavan, PhD**
- *Environmental Factors Affecting Rare Diseases* - **Paul Mehta, MD**
- *Startup Ecosystem in Rare Diseases* - **Supriya Doshi, BDS, MPH, MBA, FAHM**
- *Newborn Screening, Diagnosis* - **Reena Kartha, MS PhD, Harsh Sheth, PhD**
- *Global Clinical Trial Operations for Orphan Drugs* - **Rusty Clayton DO, Harsha Rajasimha, PhD**

- *Global Commercialization of Orphan Products* - **Anish Bhatnagar,MD, Neil Kumar, PhD**
- *Global Regulatory Strategies* - **Isaac Rodriguez-Chavez, PhD, Bhaskar Sonowal, MBBS**
- *Policy Advocacy - Impact of Recent Legislations and Policy Guidelines* - **Frank Sasinowski,JD, MPH**

- *Standard of Care Pathways, Clinical Research as a Care Option (CRAACO)* - **Tamanna RoshanLal, MD**
- *Rare Diseases in Virginia and the BioHealth Capital Region* - **John Newby, JD, Amy Adams, PhD**

Session 8: **Looking Ahead: Building Bridges for Rare Diseases**	**Harsha Rajasimha, MS, PhD** **IndoUSrare and Jeeva Informatics**	**1:30 pm to 2:55 pm**
Plenary Keynote	**Prof. Sheffali Gulati, MD, FRCPCH (UK), FAMS, FIAP, FIMSA** *All India Institute of Medical Sciences (AIIMS), New Delhi*	
India's population history enables discovery and development of therapies for inherited disorders	**Sekar Seshagiri, PhD** *MedGenome Inc., and AntlerA Therapeutics*	
Panel Discussion	**Dean Suhr, BS** *MLD Foundation* **Mohua Chakraborty Choudhury, PhD** *Johns Hopkins University* **Theresa Strong, PhD** *Foundation for Prader-Willi Research (FPWR)* **Avni Santani, PhD** *OpusGenomics*	
Closing Remarks	**Harsha K Rajasimha, PhD**	**2:55 pm to 3:00 pm**
Networking (3:00 pm to 4:00 pm)		
Close of Day 2 (4:00 pm)		
Summit Ends		

NOTES

Speakers

Cristol Barret O'Loughlin

Founder and CEO, Angel AID Cares

Cristol is a seasoned executive and storyteller. As Founder & CEO of ANGEL AID CARES, she is fiercely passionate about providing social, emotional, physical and financial relief to mothers of children with rare diseases. A former UCLA instructor, she co-founded advertising firm, The Craftsman Agency, and is humbled to advise global brands: NBA, Disney, Fox, Cisco Systems and Google. During her tenure at IBM Life Sciences, she helped accelerate advancements in cheminformatics and biotechnology

Priyanka Kakkar & Naveen Baweja

Co-founders, Cure ADSSL1

Priyanka and Naveen live in Los Angeles, CA with their two children - a 20-year-old girl and a 16-year-old boy. Both their children were diagnosed with an extremely rare genetic condition called ADSSL1 Myopathy. It is a progressive disease that causes loss of mobility within a few years of onset. As the disease progresses, it impacts the heart, lungs, and swallowing muscles. Priyanka and Naveen together run Cure ADSSL1, the only non-profit organization that supports and advocates for the ADSSL1 patient community and is actively working with researchers and clinicians across the world to develop treatment for this disease. Both Priyanka and Naveen have worked in Digital and Technology roles across various industries. Priyanka holds a B.E. in Computer Science from CRSCE, India. Naveen holds a B. Tech. in Electrical Engineering from IIT Delhi and an M.B.A. from the Booth School of Business at University of Chicago.

Pankaj Bhargava, MD

Founder, āshibio

Dr. Pankaj Bhargava is CEO of āshibio, a biotech company developing novel therapies for rare bone and connective tissue disorders. He also serves as an Executive Partner at MPM Capital and Clinical Assistant Professor at University of California, San Francisco. Dr. Bhargava has led drug development programs in oncology and rare diseases, during which time he oversaw several functional areas, filed multiple INDs and led global approvals and product launches. Dr. Bhargava prior roles include Head of the Oncology Therapeutic Area at Gilead Sciences and Chief Medical Officer at Dicerna Pharmaceuticals where he led RNA therapeutics for rare diseases and oncology. Previously, he worked at Sanofi and co-led the portfolio integration with Genzyme. Dr. Bhargava is a physician-scientist with post-graduate training in medicine, oncology and clinical pharmacology. Prior to his industry career, Dr. Bhargava was an Assistant Professor at Harvard Medical School and Dana-Farber Cancer Institute in Boston.

Michael Blum, MBA

Vice President, Commercial Strategy
Homology Medicines, Inc.

Michael Blum brings over 20 years of experience to Homology as a commercial leader in rare disease and biologics. He developed sales, marketing, market access and patient support services across multiple treatment areas, including Duchenne muscular dystrophy (DMD), Gaucher disease, mucopolysaccharidosis type II (MPS II), Prader Willi syndrome, hereditary angioedema, multiple sclerosis and psoriasis. Prior to Homology, Mr. Blum was the Head of Commercial Operations at Zafgen, Inc., where he was responsible for commercial strategy for rare disease and other treatment categories, including Type 2 diabetes. Mr. Blum was previously Head of Global Market Access at Sarepta Therapeutics responsible for the pipeline of DMD products that were then in development . Mr. Blum was also the North American Business Lead at Shire Pharmaceuticals where he worked in the Rare Disease Business Unit. He was responsible for marketing, sales, reimbursement and patient services for three Orphan drug treatments , including the launches of Elaprase, VPRIV, and Firazyr. Mr. Blum holds a Bachelor of Arts in English from the College of the Holy Cross and an M.B.A. from Babson College.

Alison Bones, BBA

President and CEO,
T.E.A.M. 4 Travis (Together Ending Asplenia Mortality)

Allison D'Ambrosio Bones came to the rare disease community in early 2019, following the death of her four-year-old son Travis. Travis died because his rare disease (isolated congenital asplenia or ICA) went undetected, undiagnosed and untreated. Determined to prevent this tragedy from being repeated by other families, Allison founded T.E.A.M. 4 Travis (Together Ending Asplenia Mortality) and serves as its President. The nonprofit organization focuses on awareness, advocacy and fundraising to support research into identifying genetic mutations and developing a method of early diagnosis. Under Allison's leadership, the organization received Congressional recognition from Arizona Representative Debbie Lesko in 2020 and Governor's Proclamations in 2021, 2022 and 2023, declaring T.E.A.M. 4 Travis Asplenia Awareness Day in Arizona. Allison served from 2020-2022 as an inaugural Rare Disease Legislative Advocates (RDLA) Advisory Committee Member and currently serves as an Everylife Foundation for Rare Diseases Advocacy Mentor. She is a graduate of Baylor University.

Mohua Chakraborty Choudhury, PhD

MPH student at Johns Hopkins University
Visiting Scholar at Indian Institute of Science

Dr. Mohua Chakraborty Choudhury is a Health Policy Researcher at the DST Center for Policy Research Indian Institute of Science (IISc), Bengaluru, and an Honorary Associate at the Institute of Public Health Bengaluru. Presently she is pursuing her Masters of Public Health at Johns Hopkins University. She holds a Ph.D. in Molecular Biology. Dr. Choudhury research work focuses on understanding the Rare disease ecosystem in India

and identifying policy and public health interventions to strengthen it. She has had actively contributed as a working member of the WHO Collaborative Global Network for Rare Diseases, which is instrumental in establishing the WHO National hub for Rare diseases in India. She was invited by the European Parliament for the European Union Visitors Program 2023, where she had the privilege to engage with EU institutions and Directorate Generals to explore bilateral opportunities in health and rare diseases between the EU and India. Additionally, she has worked with many Rare Disease patient organizations. Her impressive background and multifaceted involvement in rare disease research, policy formulation, international collaborations, and advocacy make Dr. Mohua Chakraborty Choudhury a distinguished and influential voice in the field of public health.

Srujana Cherukuri, PhD

Noble Life Sciences

Before becoming CEO, Dr. Cherukuri served in multiple roles at Noble Life Sciences including Chief Operations Officer, Chief Scientific Officer, Vice President of Product Development and Operations, and Director of Scientific Affairs. In her previous roles at Noble, Dr. Cherukuri was instrumental in optimizing new preclinical animal models and streamlining operations to achieve operational efficiency. In addition, Dr. Cherukuri led the successful consolidation of Noble operations at the Spring Valley Laboratories site. Prior to joining Noble, Dr. Cherukuri held positions at the Cleveland Clinic Foundation, National Cancer Institute and University of Maryland where she led projects focused on understanding the basic biology and mechanistic actions in cancer cells. Dr. Cherukuri has expertise in diverse areas of Biology and has +12 years of research experience in the areas of oncology and stem cell biology. Dr. Cherukuri completed her PhD in biology at Cleveland State University.

Russel (Rusty) Clayton, DO

Chief Medical Officer

Larimar Therapeutics, Inc

Dr. Russell (Rusty) Clayton is the chief medical officer at Larimar Therapeutics. Formerly, Rusty was the founder and principal at Aeremedea LLC where he provided consulting services to companies developing pharmaceutical and biologic treatments and therapeutic and diagnostic devices for use in rare disease populations. Rusty has also served as the chief medical officer for Alcresta Therapeutics, Inc. and was the Senior Vice President of research and Development at Discovery Laboratories, Inc. Prior to his over 20 year career in the pharmaceutical industry, Rusty was an attending pediatric pulmonologist at the Children's Hospital of Philadelphia and St. Christopher's Hospital for Children.

Gabi Conecker, MPH

Executive Director and Co-founder
Decoding Development Epilepsies

Gabi Conecker, MPH is mother to ten year old Elliott, who has a severe form of SCN8A-DEE. She is Executive Director and Co-Founder of Decoding Development Epilepsies, which houses s number of collaborative efforts aimed at improving care, quality of life and treatments for those with rare Epilepsies. In 2014, when confronted with a diagnosis that came with no answers for her child, she co-founded Wishes for Elliott to advance research into her son's newly discovered genetic disorder. Working collaboratively, she joined efforts with other advocates to co-found the International SCN8A Alliance, which works globally to coordinate efforts in order to better understand and treat SCN8A disorders. Noting a gap in support and education for families like hers, who have children severely affected by DEEs, she also co-founded DEE-P Connections - a partnership of +40 patient advocacy groups working to improve outcomes for those most severely affected by DEEs via both online education/webinars and joint research into the DEEs. One of the most exciting research efforts of DEE-P Connections is The Inchstone Project, an effort to advance tools able to capture the small but cherished "inchstones," not milestones, of those most severely affected in both clinical care and relatively short clinical trials.

Maria Della Rocca, MS, PMP

Sr. Director, Support & Education Programs
Global Genes

Maria Della Rocca, MS, PMP is a bilingual genetic counselor with more than 20 years of experience in the rare disease space. She worked as a bilingual genetic counselor at Gallaudet University researching the genetic causes of non-syndromic hearing loss. Following this position, she worked at the Genetic and Rare Diseases Information Center (GARD), funded by the Division of Rare Diseases Research Innovation of the National Center for Advancing Translational Sciences at the National Institutes of Health, where she initiated and built the infrastructure to provide services to the Spanish-speaking rare disease community and expanded support to other underserved populations. While at GARD, she served as a collaborator on MONDO's plain-language medical vocabulary for precision diagnosis. She is now the Senior Director of Support and Education Programs at Global Genes and oversees the RARE Concierge, RARE Compassion Program, Global Advocacy Alliance, and Global Genes' Mental Health Initiative teams.

Gautam Dongre

Secretary
National Alliance of Sickle Cell Organizations (NASCO), India

Gautam Dongre is the father of two Sickle Cell Disease (SCD) warriors. He is the Secretary of National Alliance of Sickle Cell Organizations (NASCO), India's national patient advocacy group for SCD.He represents the Government of India (GOI) National SCD Council, India's highest policymaking multi-stakeholder forum on SCD. He is a Board Member of Global Alliance of Sickle cell Disease Organization GASCDO. Gautam had been working with Sickle Cell Society of India, Maharashtra for a decade and is passionate about preventing new births of Sickle Cell across India.

Sarah Friedhoff

Director

Patient Advocacy at Astria Therapeutics

Sarah is a patient advocacy and engagement professional who brings experience designing thoughtful, innovative, and compliant relationships with both patient and professional organizations. She serves as the Director, Patient Advocacy at Astria Therapeutics, a biotechnology company devoted to bringing life-changing therapies to patients and families impacted by allergic and immunological diseases. Prior to this, Sarah served as the Director of Patient Advocacy at Abeona Therapeutics. She has also held consulting roles, including Director of Client Services at VOZ Advisors, a leading healthcare advocacy relations consultancy, where she was responsible for providing both strategic counsel and project execution to ensure clients' advocacy strategy incorporated patients' and caregivers' voices consistently—from early R&D through Phase IV programs. Prior to joining VOZ, Sarah led patient recruitment, engagement, and advocacy activities at Axovant Sciences, Inc. In this role, she supported and implemented several groundbreaking patient-centric initiatives that gained local and national press, including a first-of-its kind engagement with a ride-sharing service to ease transportation-related trial burden (partnership featured in the Wall Street Journal), and an Alzheimer's disease awareness campaign (launched at Superbowl 50's Radio Row). These activities contributed to completing recruitment for Axovant's Phase III MINDSET trial in record time -- over 1,200 patients in one year.

Teresa Ginger Davis

President

Sickle Cell/Thalassemia Patients Network Inc.

Teresa Ginger Davis lives as an adult living with a hemoglobinopathy and has been a life-long health and education advocate and spokesperson for sickle cell disease. She has more than 30 years of experience in the health industry. Davis has participated in developing national health policy with organizations like NYMAC (NY-Mid Atlantic Genetics Council), and the NYS Sickle Cell Advisory Consortium. SCTPN is currently developing its own Project ECHO course offerings. Through SCTPN, Ms. Davis applied her biology and naturopathic health science education towards working with 20 NYC hospitals to deliver care coordination for pediatric, adult clients, and their families.

Ginger's three top priorities for the next -3years are to:

1. Collaborate with other sickle cell organizations and other health-focused CBOs to increase public education and testing for sickle cell trait (SCT) and other abnormal hemoglobin variants.
2. Disrupt and break down barrios preventing global access to disease-modifying and curative therapies for sickle cell and other rare diseases.

Prof. Sheffali Gulati, MD, FRCPCH (UK), FAMS, FIAP, FIMSA

Professor, Program Director, DM Pediatric Neurology
AIIMS, New Delhi

Dr. Sheffali Gulati joined AIIMS in 1988. She was part of the team that started the first DM-Pediatric Neurology Program in South Asia in 2004 and has been heading the specialty since 2008. She has helped in establishing such programs in SAARC countries.

Dr Gulati envisioned, conceptualized, and is currently the faculty in-charge of the Centre of Excellence and Advanced Research for Childhood Neurodevelopmental Disorders: high-end diagnostic/therapeutic research, National Registry, National Child Neurology Helpline(24X7; toll-free)/Tele-Consultation Services;(www.pedneuroaiims.org; E-learning modules: www.pedneuroaiims.chalopadho.com) Major Recognitions include FRCPCH(UK), FAMS, FIAP, FIMSA, global senior leader-INSAR (International Society for Autism Research). She has won over 25 international/national awards including the Sheila Wallace Award, the National Award for outstanding efforts in Science & Technology communication, innovative/traditional methods, 2021; National Women BioScientist Award:22-2021 (Senior category). She is also Adjunct Faculty at National Brain Research Centre, India. Dr Gulati is the Editor for Autism (Sage Journal-IF:6.684), the Vice-Chairperson of the Technology Governance Steering Committee in Healthcare - All India Council for Robotics and Automation (AICRA). Her accomplishments include over 409 publications; h-Index 39; i-10index 143; and over 580 abstracts; and 570 invited talks.She is working on multiple collaborative research projects (NIH, MRC-UK, DBT, ICMR, DST, UNICEF, WHO, National-trust, USAID, MOHFW, AIIMS-UCL collaboration) in various childhood neurological disorders, Neuromuscular Disorders, Cerebral Palsy, Autism, and other neurodevelopmental disorders.

Ashish Gupta, MD, MPH

Assistant Professor
Pediatric Blood and Marrow Transplantation & Cellular Therapy
University of Minnesota

Dr. Gupta's primary interest focuses on improving access and effective delivery of cell and gene therapy interventions for children with sickle cell disease. Current treatment strategies for these children include use of hematopoietic stem cell transplant to treat the underlying disorder in the hematopoietic system. He continues to lead transplant and transplant-based gene therapy trials at University of Minnesota and has developed the upper Midwest sickle cell treatment collaborative. He is also involved in an international collaboration with physicians and public health team in Uganda to improve knowledge and access to curative therapies for sickle cell disease. Dr.Gupta is also involved in treatment of rare disorders such as lysosomal storage diseases and adrenoleukodystrophy. He is also the principal investigator for a rare disease registry and bio-specimen bank for patients with adrenoleukodystrophy.

Shifali Gupta, MD

Medical Professional

Post Graduate Institute of Medical Education and Research

Shifali Gupta is a medical professional. She is an MD in Pediatrics with a Gold Medal from a reputed university in India. Dr Gupta is currently pursuing her DM Medical Genetics from the Post Graduate Institute of Medical Education and Research, Chandigarh, India. She is interested in Genetics and Genomics of Rare Diseases and want to work in this Field.

Vikram Karnani, MS, MBA

Executive Vice President and President, Global Commercial Operations and Medical Affairs, Horizon Therapeutics

Vikram Karnani is Executive Vice President and President, Global Commercial Operations and Medical Affairs, at Horizon Therapeutics. Before joining Horizon, Mr. Karnani was with Fresenius Kabi, where he served as vice president of the therapeutics and cell therapy business, with a focus on sales, marketing and clinical implementation. He also held various positions in business development, corporate strategy, and strategic marketing within Fenwal Inc. Mr. Karnani has a Master of Business Administration from the Kellogg School of Management at Northwestern University, a master's degree in electrical engineering from Case Western Reserve University, and a bachelor's degree in electrical engineering from the University of Bombay, India.

Dorothea Lantz, MPA

Director of Community Engagement

Prader-Willi Syndrome Association | USA

Dorothea Lantz is a licensed Real Estate Broker with ONE Sotheby's International Realty and a lifetime resident of South Miami, Florida. After the birth of her son Hunter in 2017, Dorothea and her husband became involved in PWSA | USA through volunteer and fundraising events. She began working for PWSA | USA as the Director of Community Engagement in May 2021 and has created a grassroots advocacy and awareness program to help engage, educate and activate members of the PWS community throughout the Unites States. Prior to her transition into real estate in both Florida and New York, Dorothea enjoyed a successful career in public service and government administration in the state of Florida. Through her time serving as a legislative assistant and later, chief of staff, in the Florida House of Representatives, Dorothea asserted herself as a leader of advocacy and constituent rights in the state of Florida. After her departure from the state legislature in the early 2000's, Dorothea spent several years lobbying on behalf over 32 South Florida municipalities, several healthcare organizations, and participated in pro- bono services for underserved South Florida communities. Dorothea has been a Parent Mentor with PWSA | USA since 2019 and is the Chair of PWSA | USA's Advocacy Committee, which currently maintains over +60 members of the PWS community throughout the US. In addition, Dorothea is an appointed member of the City of South Miami Environmental Review and Preservation Board and a Mentor with the ACE Foundation Women&39#;s Leadership Institute. She is a member/specialist with the Institute for Luxury Home Marketing (CLHMS), holds a Transnational Referral Certification under the supervision of the International Real Estate Associates (ICREA) and is a Certified Residential Specialist (CRS), which is the highest credential awarded to residential sales agents, managers, and brokers.

Subha Madhavan, PhD

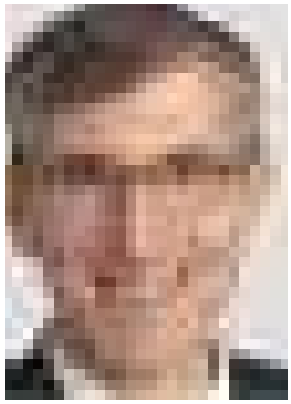
VP & Global Head of AI/ML & Digital Sciences, Pfizer R&D

Subha is a dynamic and results-driven leader with a strong track record of excellence in organizations that operate at the nexus of science, technology and business. She has initiated and successfully directed several productive clinical research and development programs at the Georgetown Lombardi Comprehensive Cancer Center, MedStar hospital network, FDA, NIH and BioPharma industry. She was co-leader of the FDA's Center for Excellence in Regulatory Science and worked with the Oncology and Vaccine teams. She was an advisory member to the Biden Foundation's Cancer Moonshot Program and advised on pre-competitive data sharing initiatives across Pharma, Health Tech companies and research organizations to drive innovation. She has been recognized for her work through several awards including the Service to America award in the Science and Environment category (2005), Research Acceleration Award by AACR and Pancreatic Cancer Action Network (2015), and Women in Tech Global award (2021). She is currently the Head of Clinical AI/ML & Digital Sciences at Pfizer worldwide R&D where she leads a team focused on advancing precision therapies across multiple treatment areas including Anti-Infectives, Oncology, Immunology & Inflammation among others.

Meredith Manning

President – The Americas
PharmaEssentia USA Corporatio

Meredith Manning joined PharmaEssentia USA Corporation in 2020 and currently serves as President of the Americas. She has more than 25 years of experience in global commercialization and brand building for biopharmaceuticals and medical devices. Meredith has played an instrumental role in defining and leading PharmaEssentia's strategic growth and ambition across the Americas, including building a robust leadership team, commercializing the organization's first asset and growing the company into a recognized global biopharmaceutical innovator. Prior to joining PharmaEssentia, Meredith was Chief Commercial Officer at resTORbio™, and previously served as Vice President of Global Product Strategy in Hemophilia for Shire (formerly Baxalta) and Baxter. She has also held a series of marketing leadership roles at Vertex® and Pfizer. Meredith received a BS at Colorado College and an IMBA from the University of Chicago Booth School of Business.



Peter Marks, MD, PhD

Director
Center for Biologics Evaluation and Research (CBER)
FDA

Peter Marks, M.D, PhD is the director of the Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration. The center is responsible for assuring the safety and effectiveness of biological products, including vaccines, allergenic products, blood and blood products, and cellular, tissue, and gene therapies.

Dr. Peter Marks received his graduate degree in cell and molecular

biology and his medical degree at New York University. Following this, he completed an Internal Medicine residency and Hematology/Medical Oncology fellowship at Brigham and Women's Hospital in Boston, where he subsequently joined the attending staff as a clinician-scientist and eventually served as Clinical Director of Hematology. He then moved on to work for several years in the pharmaceutical industry on the clinical development of hematology and oncology products prior to returning to academic medicine at Yale University where he led the Adult Leukemia Service and served as Chief Clinical Officer of Smilow Cancer Hospital. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in 2016. Dr. Marks is board certified in internal medicine, hematology and medical oncology, and is a Fellow of the American College of Physicians. In 2022, he became a Member of the National Academy of Medicine, one of the highest honors in the fields of health, science and medicine.

Paul Mehta, MD
Principal Investigator
National ALS Registry, CDC/ATSDR

Dr. Mehta is the Principal Investigator for the United States Congressionally-mandated National Amyotrophic Lateral Sclerosis (ALS) Registry which resides within the Centers for Disease Control and Prevention and the Agency for Toxic Substances Disease Registry (CDC/ATSDR) and is responsible for providing medical, scientific, and epidemiological expertise on matters related to ALS. Dr. Mehta has been in this role for over 10 years. Prior to joining the Registry, Dr. Mehta was responsible for overseeing external research initiatives for the National Center for Environmental Health (NCEH). Dr. Mehta started his career at CDC as a Team Leader for the Division of Select Agents and Toxins (DSAT) where he was responsible for CDC's Etiological Agent Import Permit Program (EAIPP) as well as conducting audits of high containment laboratories nationally. Dr. Mehta has a Doctor of Medicine (MD) from Fatima Medical Science Foundation and Bachelor of Arts in Biology from the University of Toledo.

Sukumar Nagendran, MD
TAYSHA Therapeutics

Sukumar Nagendran, MD, is the President and Head of R&D at Taysha Gene Therapies, Inc., a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic rare diseases of the central nervous system (CNS). He is a physician leader with more than 30 years of experience in key functional areas, including gene therapy development, clinical development strategy, medical affairs and diagnostics of therapeutic products. Before joining Taysha, Dr. Nagendran served as Chief Medical Officer and President of R&D at Jaguar Gene Therapy. Prior to that, Dr. Nagendran served as Chief Medical Officer of AveXis, Inc. where he oversaw the development of Zolgensma for the treatment of spinal muscular atrophy, the first one-time systemic gene therapy approved in the U.S. Dr. Nagendran has also held key leadership positions at Quest Diagnostics, Pfizer, Novartis, Daiichi Sankyo, and Reata Pharmaceuticals. Prior to transitioning to the biotech industry, Dr. Nagendran practiced internal medicine, with a focus on diabetes and cardiovascular disease. He currently serves on the Board of Directors of SalioGen Therapeutics, Solid Biosciences, Cove, Medocity, Project

Healthy Minds, and Taysha Gene Therapies.
He holds an undergraduate degree in Biochemistry from Rutgers University and earned his M.D. from Rutgers Medical School, and he trained in Internal Medicine at Mayo Clinic, Rochester. Dr. Nagendran is a Mayo Alumni Laureate and founding member of the Robert Wood Johnson Legacy Society. He is also the sponsor for the Jerry Mendell award for Translational Science at the American Society of Gene and Cell Therapy which recognizes the extensive work required to bring gene and cell therapies to clinical trial, and the Fonseca-Nagendran Scholar award at the American Diabetes Association to enhance research in minority populations.

Sumati Nambiar, MD MPH

Senior Director, Child Health Innovation and Leadership Department, Johnson and Johnson

Dr Nambiar is board-certified in pediatrics and pediatric infectious diseases, a Fellow of the American Academy of Pediatrics with over 20 years experience in drug development and regulatory science. In her current role in the Child Health Innovation and Leadership Department at Johnson and Johnson, she provides strategic, scientific, and regulatory input on pediatric development programs. Prior to joining J&J, Dr. Nambiar worked at the US FDA for 22 years in various capacities, including 8 years as Director, Division of Anti-Infectives providing scientific input and regulatory oversight on anti-infective development programs. Dr. Nambiar trained and worked as a pediatrician in India prior to moving to the US. She completed her pediatric residency, including a year as Chief Resident at Inova Fairfax Hospital for Children. She completed a fellowship in pediatric infectious diseases at Children's National Hospital, Washington DC and obtained a master's degree in Public Health from the George Washington University School of Public Health.

Mathew Pauls, MBA, JD

CEO
Savara,Inc.

Mr. Pauls has served as Chair of the Board of Directors and Chief Executive Officer of Savara since 2020. He is also currently on the Board of Directors of Amplo Biotechnology, a private gene therapy company focused on rare neuromuscular disorders and Soleno Therapeutics, a publicly traded biopharmaceutical company focused on rare diseases. From 2014 to 2019, Mr. Pauls was President and Chief Executive Officer and a member of the Board of Directors of Strongbridge Biopharma plc., a publicly traded rare-disease focused biopharmaceutical company that he took public via IPO on the NASDAQ. Prior to 2014, he served as Chief Commercial Officer of Insmed, Inc., where he built the global Commercial organization and led Global Technical Operations. Prior to Insmed, Mr. Pauls worked at Shire Pharmaceuticals, most recently as Senior Vice President, Head of Global Commercial Operations, and earlier in his career held senior U.S. and global commercial leadership positions at Bristol-Myers Squibb and Johnson & Johnson. In addition to his other Board positions, Mr. Pauls has also served as Chair of the Board of Directors of Mast Therapeutics and was a member of the Board of Directors of Zyla Life Sciences. Mr. Pauls holds B.S. and M.B.A. degrees from Central Michigan University and a J.D. from Michigan State University College of Law.

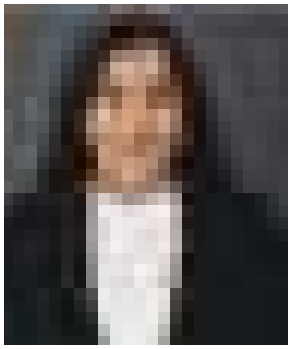
Isaac Rodrigues - Chavez

Principal Consultant & CEO
4Biosolutions Consulting

Dr. Isaac R. Rodriguez-Chavez is a distinguished scientific and regulatory expert with a -30year career spanning Infectious Diseases, Viral Immunology, Viral Oncology, Microbiology, Vaccinology, and rare diseases. Currently an independent clinical research, regulatory affairs and digital medicine consultant, he has held influential roles, including Senior Vice President at ICON plc, where he led Decentralized Clinical Trials (DCTs) and Digital Medicine strategies. He made significant contributions as an FDA, CDER Senior Officer, focusing on modernizing clinical research through DCTs and Digital Health Technologies.

Dr. Rodriguez-Chavez is a board member of BlueCloud by HealthCarePoint, Reveles Clinical Research, Digital Medical Society, and the Hypertrophic Cardiomyopathy Association (HCMA). He's also engaged with Global Genes as a Rare Disease Health Equity Council member and serves as a Regulatory Science Editor at the DIA Global Forum Magazine. He actively shapes industry standards as Co-Chair of the Institute of Electrical and Electronics Engineers (IEEE).

His academic background includes a PhD in Virology and Immunology, an MS in Microbiology, an MHS in Clinical Research, and a BS in Biology. With post-doctoral specialty training in Infectious Diseases, Viral Immunology, Viral Oncology, and Clinical Research, he has authored +60 scientific publications and presented at over 140 global conferences.



Tamanna Roshan Lal, MB ChB

Chief Medical Officer
Uncommon Cures

Dr. Roshan Lal is the Chief Medical Officer at Uncommon Cures. She previously was an Attending Metabolic Geneticist at the Rare Disease Institute (RDI) in Children's National. At RDI, she played pivotal roles as Director of Clinical Trials, Director of International Patient Consultation and Care Referrals, and Director of Genomic Therapeutic Clinics. Dr. Roshan Lal's medical education journey started at University of Manchester, UK. She then underwent training in various medical specialties, including Pediatrics, Obstetrics & Gynecology, General Medicine, General Surgery, and Critical Care. Her specialization continued with a Pediatric Residency at Sinai Hospital. She did her Clinical Genetics and Biochemical Genetics fellowships at Johns Hopkins and the NIH. Dr. Roshan Lal is currently pursuing an MBA at the Alliance Manchester Business School and is known for her expertise in rare disease diagnosis and management, clinical trial leadership, and contributions to peer-reviewed publications and book chapters on rare diseases.

Suyog S

Director, International Gaucher Alliance (IGA)
Secretary, Lysosomal Storage Disorders Support Society (LSDSS) India

Suyog has been involved in patient advocacy both national and internationally for over 10 years. He currently works as the Secretary of LSDSS, the first and leading rare-disease patient advocacy group in India that is dedicated to the welfare of those suffering from Lysosomal Storage Disorders (LSDs). He also serves on the Board of the International Gaucher Alliance, a UK-based international umbrella organization representing the interests of Gaucher disease patients and Gaucher patient organizations worldwide. Suyog has also been a member of the Rare Disease International (RDI)-WHO Collaborative Global Network for Rare Diseases (CGN4RD) Panel of Experts. Professionally, he's an Electronics Engineer and also holds an MBA in Marketing, Operations and Supply Chain Management.

Dr. Avni Santani

Co- Founder
OpusGenomics

Dr. Santani is a co-founder of Opus Genomics, a precision medicine company for diagnosis and interventional support for autism. She is also the Chief Genomics Officer of LetsGetChecked, a global digital healthcare solutions company. She is responsible for the strategy, direction, and execution of LetsGetChecked's genomics initiatives to enhance personalized healthcare. Dr. Santani has held prior positions including the Chief Medical Officer at Veritas Genetics where she was responsible for the company's clinical development plans and medical affairs. Her expertise lies in developing a compelling product portfolio by leveraging the power of sequencing, informatics, and AI for rapid and effective disease diagnosis. Previously, Dr. Santani served as the Director of Clinical Laboratories and Strategic Initiatives at the Children's Hospital of Philadelphia (CHOP) and was an Associate Professor of Pathology at the University of Pennsylvania. With over 17 years of experience at the forefront of clinical genomics, Dr. Santani is actively involved with the College of American Pathologists and the Association for Molecular Pathology, among other organizations. Dr. Santani holds a Ph.D. in Genetics from Texas A&M University and is board-certified in clinical laboratory genetics and genomics by the American Board of Medical Genetics and Genomics (ABMGG).

Bonnie Schneider

Co-Founder & Director
IgA Nephropathy Foundation

Bonnie Schneider is co-founder and director of the IgA Nephropathy Foundation. In 2004, there was no place for Bonnie and Ed Schneider to turn when their -13year-old son was diagnosed with IgA Nephropathy, a strange-sounding disease it seemed that no one had ever heard of, nor was any research being done to find a cure. Alone, confused and afraid, it would be years before they would meet another person who lived with the disease. Determined that no one else would have to face IgAN by themselves, Bonnie quit her New York City marketing job to start the IgA Nephropathy

Foundation with her husband. The organization was conceived to offer much-needed support to people affected by IgAN – both patients and their loved ones – while also seeking a cure for the disease. Today, it remains the only dedicated patient advocacy organization for people affected by IgA Nephropathy – providing support, answers, and hope for the future through the community it has created the information it shares, and the research it funds. Bonnie lives with her husband Ed in Wall, NJ, and enjoys spending time with her five children and 2 grandchildren. She is grateful for the expanded IgAN family that is now available to people diagnosed, so they and their loved ones don't have to face their diagnosis and journey alone.

Sekhar Seshagiri

Chief of Genomics & Informatics, MedGenome Inc.
CEO/CSO AntlerA Therapeutics

Dr. Sekar Seshagiri servers as Chief of Genomics and Informatics at MedGenome Inc., where he oversees the Scientific activities of the company and its US operations. He also serves as CEO and CSO of AntlerA Therapeutics, a company that is developing regenerative medicines therapies. Recently, he and his team along with their partners have started testing a molecule they developed in clinical trials for treating retinopathies. Prior to his current role, he spent over 20 years at Genentech where he was a Staff Scientist and Associate Director. During his tenure at Genentech, he established a state-of-the-art genomics laboratory where he conducted research in cancer and cell signaling areas to support drug discovery and development. He has authored and co-authored over 120 articles in peer-reviewed journals including Nature, Nature Genetics, Science, and Cancer Cell.

Samir Sethi

President
Indian Rett Syndrome Foundation

Mr. Samir Sethi is a father of a child diagnosed with Rett syndrome. He is the President of IRSF since 2014 and has conducted aggressive awareness campaigns to increase membership to over 400 from India and Nepal. Mr. Sethi has done several awareness programs all across the country besides periodic webinars. He has assisted parents and CRO's for clinical trials. Mr. Sethi has implemented SAMARTH program for distributing free medicines to 'Silent Angels', assisted for Disability Certificates and Health Insurance for Silent Angels. He has also provided social, psychological and financial support of families.

Moke Sharma

SVP, Head of Global Development Operations

Moke Sharma joined BMS in October 2022 and serves as Head of Global Development Operations. He is accountable for leading an end-to-end, development operations function charged with the global clinical trial execution of the BMS product portfolio (Phase I – IV). He also leads a major initiative to transform Drug Development at BMS to accelerate the delivery of new medicines to patients. Moke previously worked at Alexion

Pharmaceuticals where he was the Head of Development Operations and Quantitative Sciences, accountable for creating and leading an integrated, rare disease development engine. Prior to joining Alexion, Moke held senior positions with Novartis Oncology including Head of Global Development Operations, Head of R&D in Japan, and Head of Project Management. He also held clinical program leadership roles at Pfizer and GSK. Moke is a graduate of the University of Leeds in the UK with a degree in chemical engineering and holds an MBA.

Harsh Sheth, PhD

Assistant Professor & Head,
Advanced Genomic Technologies Division,
FRIGE Institute of Human Genetics, India

A clinical geneticist with a diverse experience in the areas of cancer prevention, personalised medicine, male infertility, autism spectrum disorders, next generation sequencing technologies, synthetic biology and public engagement. Proficient in molecular biology and biostatistics coupled with effective communication, planning and management supports Dr. Sheth's ambition to carry out large scale collaborative work. To date, he has co-authored 39 publications, 2 book chapters, 1 white-paper and >10 press articles along with a co-inventor on 2 patents. He is a recipient of prestigious John Corran Book Award, New York Academy of Science's Interstellar Initiative and numerous research grants from national and international funding agencies

Amlin Shukla, MD

Scientist D, Indian Council of Medical Research (ICMR)

Dr Amlin Shukla is a Scientist working at the Indian Council of Medical Research, India. She coordinates the National Registry of Rare and Other Inherited Disorders funded by ICMR. Her other research interests include neonatal mortality, early child development, and implementation research. She is a Pediatrician by training. She joined the Indian Council of Medical Research in June 2019 to further her research interests. Before joining ICMR, she worked as an Assistant Professor in Pediatrics & Neonatology at erst. PGIMER & RML Hospital, New Delhi, and HIMSR, New Delhi from Jan 2015 to June 2019. She completed her MD in Pediatrics in 2011.

Bhaskar Sonowal, MBBS, MBA

IndoUSrare & HED Healthcare

Bhaskar Sonowal is the Managing Director of HED Healthcare Pvt. Ltd. based in Gurgaon, India. He brings more than 20 years of rich experience in innovative business development, creating access projects, formulating policy changes, and serving the people of India – as a Clinician and a Program Manager for some of the country's largest healthcare programs. He is credited with creating and leading large-scale market development strategies, which have impacted businesses, patients, and society for high-end biotechnology products and medical devices. His areas osf interest include Rare Diseases, Cancer, Vaccine Preventable Diseases, Musculoskeletal conditions, and Nutritional imbalances.

Marlene Soto-Riera

RARE Parent, Caregiver & Advocat,
Helping Swans Co.

Empowering individuals with disabilities to soar! I'm a passionate advocate, author, and founder of Helping Swans, a nonprofit organization focused on disability inclusion. Coming from a disabled person myself and a special needs mom join me on my mission to educate and inspire through books and parent workshops.

Theresa Strong, PhD

Co-founder and Director of Research Programs
Foundation for Prader-Willi Research (FPWR)

Theresa Strong, PhD, is a Co-founder and Director of Research Programs at the Foundation for Prader-Willi Research (FPWR, www.fpwr.org), a nonprofit that supports research to advance the understanding and treatment Prader-Willi syndrome (PWS). Theresa has a PhD in genetics and led a gene therapy research lab as a Professor at the University of Alabama at Birmingham (UAB), prior to transitioning to a full-time position at FPWR. She remains a Volunteer Adjunct Professor at UAB. At FPWR, she directs the grant program, leads the PWS Clinical Trials Consortium and is principal investigator for the Global PWS Registry. She is active in the broader patient advocacy community as well, acting as a mentor in the Chan Zuckerberg Initiative "Rare as One" Project and having served as a member of the US Food and Drug Administration (FDA) Patient Engagement Collaborative. Theresa and her husband have four children, including a young adult son with PWS.

Dean Suhr, BS

President, MLD Foundation
President, DreamBuilders Resources

Dean co-founded MLD Foundation in 2001 to support families, improve clinical care, and advance therapies for metachromatic leukodystrophy (MLD), a rare genetic condition affecting two of his three children. Now in its 22nd year, MLD Foundation continues its global support of families and research, including facilitating clinical trials and basic science research in academic, medical & academic institutions, biopharma, government, and with other public and private partners. Dean got involved in Newborn screening around 2010 and has attended a majority of the ACHDNC advisory committee meetings since then. In 2015 Dean launched the RUSP Roundtable (not MLD-specific) as a forum to bring together key perspectives from the NBS, public health, research, clinical care, biopharma, advocacy, payor, regulatory, and other ecosystems to address current and future opportunities to improve the system. In 2016 he provided the RUSP-alignment language tying California's condition review timeline to the federal RUSP (SB 1095). Dean is currently leading MLD's efforts to implement MLD MLD newborn screening by addressing policy, biochemical, state public health, access & reimbursement, Standards of Care, and the federal RUSP nomination. He is also shepherding the https://mldregistry.com/, several clinical trials, and numerous MLD

development projects while working to enable access & reimbursement for MLD gene therapy which was approved in the EU in 2020 and is under current FDA Priority Review in the US. Dean is very engaged with many broader rare disease policy, access, reimbursement, and research initiatives. Dean has a BS in Electrical Engineering from the University of Southern California and spent 30 years working primarily in high tech executive leadership positions in marketing, operations, strategic planning, sales, and business development.

Dr. L. Swasticharan

Additional DDG and Director (EMR),
Directorate General of Health Services
Ministry of Health and Family Welfare, Govt of India

Dr. L. Swasticharan is currently the Additional DDG and Director(EMR) in the Directorate General of Health Services, Ministry of Health and Family Welfare, Govt of India. He is in-charge of National Tobacco Control Programme, National Oral Health Programme, Emergency Medical Relief, National Policy for Rare Diseases, Non Communicable Diseases, Mental Health, Geriatric and Pallative, Iodine & Fluorosis National Programme. He has an MD in Community Health Administration from Delhi University and an MPH from Massey University, Palmerston North, New Zealand. He is also a WHO Fellow in Health Impact Assessment at Liverpool University, United Kingdom. His areas of work interest include many public health issues such as integration of Health systems strengthening for synergistic, better implementation of programs & scientific evidence to policy to implementation, systems thinking and innovations in healthcare delivery.

Srinath TL, PhD

CEO & Founding Director
GenoPhe Biotech Pvt. Ltd

Dr. T L Srinath is the CEO & Founding Director of GenoPhe Biotech Pvt. Ltd., Bengaluru, India; a startup generating zebrafish platforms and avatars to enable drug discovery and personalised medicine for people with rare diseases, respectively.Srinath earned his M. Sc. in Microbiology (1996) and Ph. D. in Microbiology (2002) from Dr RML Avadh University, Faizabad. During his post-doc work at Indian Institute of Science, Bengaluru, India, he was part of a research team that worked extensively to identify mycobacterial DNA repair enzymes, characterize and decipher its physiological relevance. Later between 2012 to 2017, Srinath taught Undergraduates of Indian Institute of Science, Bengaluru. It's during this tenure he got fascinated by utility of zebrafish in pre- and non-clinical research and in 2018, GenoPhe Biotech Private Limited was formed to cater needs of stakeholders.

Tiina Urv

Program Director
Rare Diseases Clinical Research Network (RDCRN)

Tiina Urv is the program director for the Rare Diseases Clinical Research Network (RDCRN), a multidisciplinary international program in the Division of Rare Diseases Research Innovation. As the lead for the RDCRN program, Tiina collaborates with 10 NIH Institutes to manage 22 consortia and a central Data Management Coordinating Center. The RDCRN has more than 200 participating sites in 17 countries and more than 100 Patient Advocacy Groups as research partners and conducts research on about 200 rare diseases. Before joining the division, Urv was a program director in the Division of Clinical Innovation where she provided stewardship for multiple Clinical and Translational Science Awards Program hubs and worked with the Trial Innovation Network as well as NCATS' Division of Rare Diseases Research Innovation.

Dr.Urv came to the National Institutes of Health (NIH) in October 2006, working as a program director at the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) in the Intellectual and Developmental Disabilities Branch. Prior to joining NIH, she was an assistant professor at the University of Massachusetts Medical School's Eunice Kennedy Shriver Center and a research scientist at the New York State Institute for Basic Research in Developmental Disabilities. At NICHD, Dr. Urv coordinated the Hunter Kelly Newborn Screening Research Program, chaired the trans-NIH Fragile X research program, and managed a diverse portfolio of basic, behavioral and bio-behavioral research related to developmental disabilities and rare diseases.

Dr. Urv is a developmental disabilities specialist with a Ph.D. from Columbia University. She earned her undergraduate degree from the University of Washington.

Sophia Zilber

Associate Director, Statistical Programming at Alexion
Board member and Patient Registry Director at Cure Mito Foundation

Sophia Zilber has 20 years of experience in the pharmaceutical industry, focusing on data analysis and statistical programming of clinical trial data. Sophia's newborn daughter Miriam died from Leigh Syndrome, a form of the Mitochondrial Disease, in 2017. Following this tragedy, Sophia has volunteered her time and experience to help the rare disease community. Sophia has been involved in multiple efforts with the goal of raising awareness in the mitochondrial disease community and the rare disease community in general regarding patient registries and what is involved in collecting high-quality data. Sophia also shared her personal experiences through blog posts and articles published in the American Academy of Pediatrics Palliative Care Newsletter, KevinMD blog, Courageous Parents Network, and Global Genes. Sophia is proud to be a board member for the Cure Mito Foundation where she's also responsible for the Worldwide Leigh Syndrome patient registry.

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Summit Organizing Committee

Amy Adams, PhD

Institute for BioHealth Innovation, George Mason University

As Executive Director of the Institute for Biohealth Innovation at George Mason University, Amy Adams is responsible for nurturing and advancing biohealth research and innovation interests for a broad range of academicians. Her role includes fostering partnerships with industry, healthcare, government, and academic partners.

Rich Bendis

BioHealth Innovation and BioHealth Capital Region Forum

An entrepreneur, corporate executive, and innovation leader, Rich Bendis serves as Founder and CEO of BioHealth Innovation, Inc. He also spearheads Innovation America, a national partnership for fostering innovation. With extensive board and advisory roles, including service on Governor Hogan's Life Science Advisory Board, he is a key influencer in the innovation and technology sectors.

Anish Bhatnagar, MD

IndoUSrare and Soleno Therapeutics

Anish Bhatnagar is the CEO of Soleno Therapeutics, which is dedicated to the development of treatment for rare diseases. Their lead program is for DCCR, a once-a-day tablet, for the treatment of Prader Willi Syndrome. Dr. Bhatnagar is a physician with over 20 years of experience in the biopharmaceutical, medical device, and diagnostic industries. His experience spans the development of biologics, drugs, drug-device combinations, and diagnostic as well as therapeutic medical devices..

Madhulika Kabra, MD

All India Institute of Medical Science, Delhi

An academic researcher from the reputed All India Institute of Medical Sciences, Madhulika Kabra has contributed to the research on Prenatal diagnosis & Exome sequencing. With an h-index of 32, she has co-authored 350+ publications receiving over 5000 citations.

Reena Kartha, MS, PhD

IndoUSrare and University of Minnesota

Reena is an Asst. Professor in the Dept. of Experimental and Clinical Pharmacology and Associate Director of Translational Pharmacology in the Center for Orphan Drug Research, University of Minnesota. Her research focuses on understanding the pathophysiological role of oxidative stress and inflammation in rare inherited metabolic disorders as well as the pharmacology of agents that target these molecular pathways. Her career goal is to help patients with rare diseases by developing therapies, train the next generation of healthcare providers and increase awareness about rare diseases

Juhi Naithani, MBA

IndoUSrare and bGlobal Consulting

Juhi is principal at bGlobal Consulting and provides support to organizations ready to Go Global. She has public and private sector experience in business strategy across a range of disciplines. Juhi also served as Assist. Director of International Business Investment at the Fairfax County Economic Development Authority (FCEDA), Virginia, USA, where she managed the India office of FCEDA, implementing new outreach strategies and events to bring emerging technology companies to the United States. A strong proponent of entrepreneurship, Juhi was a member of the Board of Directors of the TiE (The Indus Entrepreneur) DC chapter.

John Newby, JD

Virginia Bio

Virginia Bio a non-profit association representing Virginia's diverse life science industry. John Newby brings extensive experience from leadership roles in government, law, and the military. Previously, he served as the Commissioner of the Virginia Department of Veterans Services, practiced law in Richmond and Washington DC, and had a military career, including commanding an Air Force special operations unit and serving in Iraq. John is dedicated to fostering innovation and collaboration in the life sciences sector in Virginia.

Frank J. Sasinowski, JD,MPH

IndoUSrare and Hyman, Phelps & McNamara, P.C.

As a NORD Lifetime Achievement Awardee, Frank Sasinowski has extensive experience with the FDA, from both the agency and client side. He has assisted in the approval for hundreds of new drugs. He was also a key player in implementing the 1983 Orphan Drug law and the 1984 Hatch-Waxman law. In 2012, President Obama recognized Frank's contributions to the President's Council of Advisors on Science and Technology (PCAST) report, "Propelling Innovation in Drug Discovery, Development and Evaluation."

Harsha Rajasimha, MS, PhD

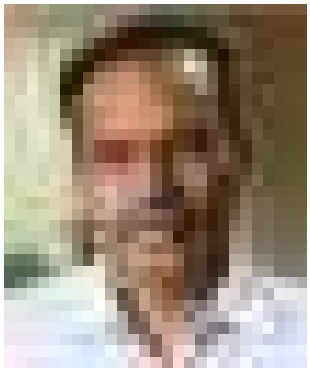
IndoUSrare and Jeeva Informatics Solutions

Driven by a personal cause, Harsha is a recipient of numerous awards for his work in genomics, precision medicine and rare diseases including awards to join the Committee on Rare Diseases at the United Nations headquarters, Lead India Foundation 2020 Award for leadership and outstanding achievement in rare diseases, NTT DATA Healthcare Life Sciences Silver award for outstanding achievement 2017, Sanofi Genzyme patient advocacy leadership award 2016, Bioscience company of the year 2014, NEI director's innovation award 2012 for building their genomics cyber infrastructure.

Bhaskar Sonowal, MBBS,MBA

IndoUSrare and HED Healthcare Pvt. Ltd

Dr Sonowal is the Managing Director of HED Healthcare Pvt. Ltd., India. He brings more than 20 years of rich experience in innovative business development, creating access projects, formulating policy changes, and serving the people of India – as a Clinician and a Program Manager for some of the country's largest healthcare programs. He is credited with creating and leading large-scale market development strategies, which have impacted businesses, patients, and society for high-end biotechnology products and medical devices.



K. Thangaraj

CSIR- Centre for Cellular and Molecular Biology

Dr. K Thangaraj joined the Centre for Cellular and Molecular Biology (CCMB), Hyderabad, India in the year 1993. He took a responsibility of Directing the Centre for DNA Fingerprinting and Diagnostics (CDFD) during August 2020 – June 2023. In July 2023, he returned to CCMB as a JC Bose Fellow. His main research interests are: population genetics, rare genetic diseases, cardiovascular diseases, mitochondrial disorders, male infertility and developmental sex disorders. He has published about 300 research articles, some of which are in Cell, Lancet, Science, Nature and Nature Genetics. He is an elected Fellow of - Indian National Science Academy, Indian Academy of Sciences, and National Academy of Sciences. He is a recipient of several awards, including J C Bose Fellowship, Sun Pharma Research Award in Medical Sciences, Raman Research Fellowship, Life-time achievement Award, Excellence in Science Award, Distinguished Scientist Award, Sir CV Raman Memorial Lecture Award, Sir Dr. UN Brahmachari Award, and delivered several Orations. He is a Board Editor of Mitochondrion; Associate Editor of BMC Medical Genetics; BMC Genetics; Tropical Medicine and International Health, and member of the Editorial Board of the journals – Scientific Reports, Human Genetics, and Clinical Genetics. He was the President of the Indian Society of Human Genetics (2011 – 2015) and the founder of the Society for Mitochondrial Research and Medicine..

KiwiTech™

KiwiTech is Proud to Support the Indo-US RARE Community

KiwiTech is a technology development and equity investment company headquartered in Washington, D.C.

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"We commend the mission and enormous undertaking of IndoUSrare in educating the Indian diaspora about rare diseases and connecting them with experts, resources, and access to global clinical trials. We need more collaboration and engagement with our international partners. No country or organization can do it all." — Craig Martin, RithmHealth

About IndoUSrare

Indo US Organization for Rare Diseases is a non-profit 501(C)(3) public charity on a mission to accelerate affordable therapies for rare diseases by catalyzing cross-border collaborations between stakeholders of rare diseases in the USA, India, & Globally

We build collaborative bridges between the Eastern and Western siloes to accelerate R&D of diagnostics and therapies for rare diseases

Our Vision

To educate, empower and advocate for all persons with rare diseases, especially those in India and those in diaspora from India in the US and everywhere

Patient Centric Programs

Patient Concierge

Tech-enabled platform to provide information support. We connect rare disease patients in India, the USA, and around the world with the latest information, patient registries, and expert advice on care navigation and clinical trials.

Policy Advocacy

Driving bilateral ties between the USA and India with a focus on policy frameworks that support the exchange of resources, cross-border collaborations and investments in the BioPharma industry.

Patients Alliance

Coalition of patient groups across different continents. We connect patient foundations to the latest cutting-edge clinical research, cost-effective preclinical research, and other resources.

Education, Awareness and Outreach

Accelerating awareness about rare diseases through Educational webinars, Art competitions, Walk/Run events, Newsletters, and Multimedia campaigns

Corporate Alliance

Alliance of biopharmaceutical, medical device, and other companies in the life science industry that are involved in rare disease research with a commitment to collaboration between industry and patient communities to ensure equitable and affordable access of therapies to all patients with rare diseases globally.

Research Programs

Our research programs align with the needs of our alliance members and our organizational mission. We bring together the expertise in our Research Corps and other collaborators and partners to drive diverse grant funded research initiatives with the broad goal of accelerating diagnosis and access to care for rare diseases

Indo US RARE Impact

Healthworld.com

News Exclusives Leaders Speak Events Awards Webinars More

Addressing Massive Health Inequities on Rare Disease Day

February 28 marks International Rare Disease Day. While many improvements have been made in clinical trials for rare diseases, the drug development process is still too slow, expensive, and not accessible for most patients – particularly in low-and-middle income countries (LMICs) such as India. Dr. Harsha Rajasimha, Founder and Chairman of IndoUSrare, explains the need for increased global collaborations for accelerating rare disease clinical trials, access to these, and patient access to treatments.

APPLIED CLINICAL TRIALS

MEDIA CONFERENCES COLUMNS RESOURCES MARCAETS SUBSCRIBE

IndoUSrare: Purposeful Clinical Research For Rare Diseases In India

Published on: January 24, 2023
Lisa Henderson, Andy Studna

Dr. Harsha Rajasimha, Founder and Chairman of IndoUSrare, discusses the emotional reason behind founding the non-profit, programs they offer that are helping rare disease parents/patients, rare disease research in India, and much more.

45 Patients Alliance Members

6 Corporate Alliance Members

75+ patients assisted through the Concierge

500+ rare diseases represented

20,000+ patients across **50+** countries

Hosted **19** global events, Invited Speaker at **14** conferences

16 Global Partnerships

Policy inputs to US FDA and Indian Govt on data sharing and diversity

Raised **$150,000+** through donations and grants

Publications

1. Venugopal, N., Naik, G., Jayanna, K., Mohapatra, A., Sasinowski, F.J., Kartha, R.V., & Rajasimha, H.K. (2023). Methods for estimating the prevalence and incidence of a rare disease. RDOD Journal (Manuscript in review)
2. Khera, H. K., Venugopal, N., Karur, R. T., Mishra, R., Kartha, R. V., & Rajasimha, H.K.(2022). Building cross-border collaborations to increase diversity and accelerate rare disease drug development – meeting report from the inaugural IndoUSrare Annual Conference 2021. Therapeutic Advances in Rare Disease. https://doi.org/10.1177/26330040221133124
3. Venugopal, N., Kartha, R.V., Baranova, A., & Rajasimha H.K.(2021). Impact of COVID-19 on rare disease research and clinical development: a global perspective. Translational Genomics 2:e1-8
4. Diversity, access, and inclusion: How IndoUSrare is addressing these grand challenges by building collaborative bridges for stakeholders of rare diseases between the western world and the Indian subcontinent. RARE Revolution Issue 020, Page 52-55

Press Releases

1. AI Is Revolutionizing Rare Disease Research, but a Lack of Diversity Undermines Its Full Potential (September 2023)
2. Asia and the Indian Subcontinent: A Vision for Clinical Studies of Rare Diseases with Varied Prevalence Globally (August 2023)
3. Global Registries Could Change the Lives of Hundreds of Millions of Rare Disease Patients Everywhere (March 2023)
4. Celebrating Rare Disease Day and 40 Years of the Orphan Drug Act Advocating for Rare Disease Patients and Their Families (February 2023)
5. Millions Suffer From Untreatable Rare Diseases While Medical Costs Skyrocket (December 2022)
6. Families in India Face Ongoing Challenges When Coping With Rare Diseases (October 2022)
7. Political Squabbles Slow Drug Development for Rare Diseases (September 2022)
8. Addressing Massive Health Inequities on Rare Disease Day (February 2022)

Highlights from Media

1. Why are Patient Registries Key to Accelerating Rare Disease Research? - Onco'zine, July 2023
2. Tackling Rare Disease in India - Inside Precision Medicine, June 2023
3. IndoUSrare Advocates for Increased Rare Disease Drug Development in India BioSpace(BioBuzz), March 23
4. Affordability of Orphan Drugs Hinges on Global Collaboration Pharmacy Times, March 2023
5. The Future of Rare Disease Care Depends on Thinking Globally - Genetic Engineering & Biotechnology News, March 2023
6. IndoUSrare Hopes to Promote Global Efforts in Rare Disease Research - AADC News, Dec 2022
7. Clinical Research Is Probably the Best Option for Most Patients with Rare Diseases - Medical Device News Magazine, April 2023
8. Breaking Barriers in Rare Disease Research and Drug Development – Insights from IndoUSrare's Rare Disease Day Event - BioBuzz, March 2022
9. Addressing Massive Health Inequities on Rare Disease Day - ETHealthworld.com, February 2022

Indo US RARE Patients Alliance Members

21st Century Dads Foundation
Area: Issue of father absence
Website: https://21stcenturydads.org/

ALD Connect, Inc
Disease: Adrenoleukodystrophy
Website: https://aldconnect.org/

Anushree Foundation/MNGIE India
Disease: MNGIE
Wesite: https://www.facebook.com/MngieIndia/

Aarskog Foundation
Disease: Aarskog Syndrome
Website: https://www.aarskogsyndromefoundation.co.uk/

APS Type 1 Foundation, Inc.
Disease of Interest: AutoImmune Polyglandular Syndrome Type 1
Website: https://apstype1.org/

Auto Immune registry
Disease of Interest: AutoImmune disease
Website: https://www.autoimmuneregistry.org/

Bharat MD Foundation
Disease of Interest: Muscular Dystrophy
Website: https://www.bharathmd.org/

CDKL5 South Asia
Disease of Interest: CDKL5 deficiency disorder
Website: https://m.facebook.com/CDKL5SouthAsia/;https://cdkl5southasia.com/

Child and Youth Care Zimbabwe
Disease of Interest: CDKL5 deficiency disorder
Website: http://www.cyc.org.zw/

CSNK2A1 Foundation
Disease of Interest: Okur-Chung Neurodevelopmental Syndrome
Website: https://www.csnk2a1foundation.org/

Cure ADSSL1
Disease of Interest: Distal Myopathy - 5 (MPD5)
Website: https: //www.cure-adssl1.org/

Cure MITO Foundation
Disease of Interest: Leigh syndrome and mitochondrial disease
Website: https://www.curemito.org/

Cure MLD
Disease of Interest: :Met achromatic leukodystrophy (MLD)
Website: https://www.curemito.org/

Cure VCP Disease, Inc.
Disease of interest : VCP disease aka IBMPFD
Website: https://www.curevcp.org

Defeat MSA Alliance
Disease of interest : Multiple System Atrophy, Parkinson's Plus, Atypical Parkinson's, Shy-Drager Disease, Parkinson"s Disease
Website: https://defeatmsa.org/

DLG4
Disease of interest:DLG4
Website: https://dlg4researchfund.org/

Dr. Shyama Narang Foundation
Disease of interest:Motor Neurone Disease
Website: https://mndtrust.co.in/

Ehlers-Danlos Society
Disease: Ehlers-Danlos syndromes (EDS), hypermobility spectrum disorders (HSD), and related conditions
Website: https://www.ehlers-danlos.com/

Families of spinal muscular atrophy india
Disease of interest:Spinal Muscular Atrophy
Website:http://fsmaindia.org/

IGA Nephropathy Foundation
Disease: IgA Nephropathy
Website: https://igan.org/

Indian Patients Society for Primary Immunodeficiency (IPSPI)
Disease: Primary Immunodeficiency Disorders
Website: https://www.ipspiindia.org/

Indian Rett Syndrome Foundation (IRSF)
Disease: Rett Syndrome
Website: https://www.rettsyndrome.in/

International Pemphigus Pemphigoid Foundation (IPPF)
Disease:Pemphigus and Pemphigoid
Website:https://www.pemphigus.org/

IPWSA
Disease: Prader Willi Syndrome
Website: http://www.ipwsa.in/

Koolen-de Vries Syndrome Foundation
Disease:Koolen-de Vries Syndrome
Website:https://kdvsfoundation.org/

Lightning and Love Foundation
Disease:THAP12, epilepsy
Website:https://www.lightningandlove.org/

LYSOSOMAL STORAGE DISORDERS SUPPORT SOCIETY
Disease: Lysosomal Storage Disorders
Website: http://www.lsdss.org/

MEPAN Foundation
Disease: MEPAN Syndrome
Website:https://www.mepan.org/

MLD Foundation
Disease: Metachromatic leukodystrophy (MLD)
Website:https://mldfoundation.org/

NASCO
DIsease:Sickle Cell Disease
Website:https://www.facebook.com/IndiaScdAlliance

National Ataxia Foundation
Disease: Ataxia
Website: https://www.ataxia.org/

Neuromuscular Disease Foundation
Disease: GNE Myopathy
Website:https://curehibm.org/

Parent Project Muscular Dystrophy
Disease: Duchenne Muscular Dystrophy
Website: https://ppmdindia.org/

Progressive Familial Intrahepatic Cholestasis (PFIC)
Disease: Progressive Familial Intrahepatic Cholestasis (PFIC)
Website: https://www.pfic.org/

RARE AUTOINFLAMMATORY CONDITIONS COMMUNITY - UK (RACC - UK)
Disease: Rare Autoinflammatory Conditions
Website: https://raccuk.com/

Rare Disease Society for Nepal
Disease: agnostic
Website: https://www.facebook.com/nirmalmdon

Rare Disease Uganda
Disease: Agonstic

Remember The Girls
Disease: All X-linked disorders
Website:https://www.rememberthegirls.org/

Sickle Cell Thalassemia Patients Network
Disease: Sickle Cell Disease, Thalassemia, and other inherited blood disorders
Website:https://sctpn.net/

STXBP1 Foundation
Disease: STXBP1 Encephalopathy
Website:https://www.stxbp1disorders.org/

Team Telomere
Disease: Telomere Disorders
Website: https://teamtelomere.org/

Team4Travis
Disease: Isolated Congenital Asplenia
Website:https://www.team4travis.org/

Aarskog Foundation
Disease: Aarskog Syndrome
Website: https://www.aarskogsyndromefoundation.co.uk/

APS Type 1 Foundation, Inc.
Disease of Interest: Autoimmune Polyglandular Syndrome Type 1
Website: https://apstype1.org/

Snyder-Robinson Foundation
Disease of Interest: Snyder-Robinson Syndrome
Website: https://snyder-robinson.org/

Wishes for Elliott: Advancing SCN8A Research
Disease of Interest: SCN8A and DEEs (developmental and epileptic encephalopathies)
Website: https://www.wishesforelliott.com/

Indo US Corporate Alliance Members

Exela Pharma Sciences
Website:https://www.exelapharma.com/

Aceragen
Website: https://www.aceragen.com/

Engage Health
Website:https://www.engagehealth.com/

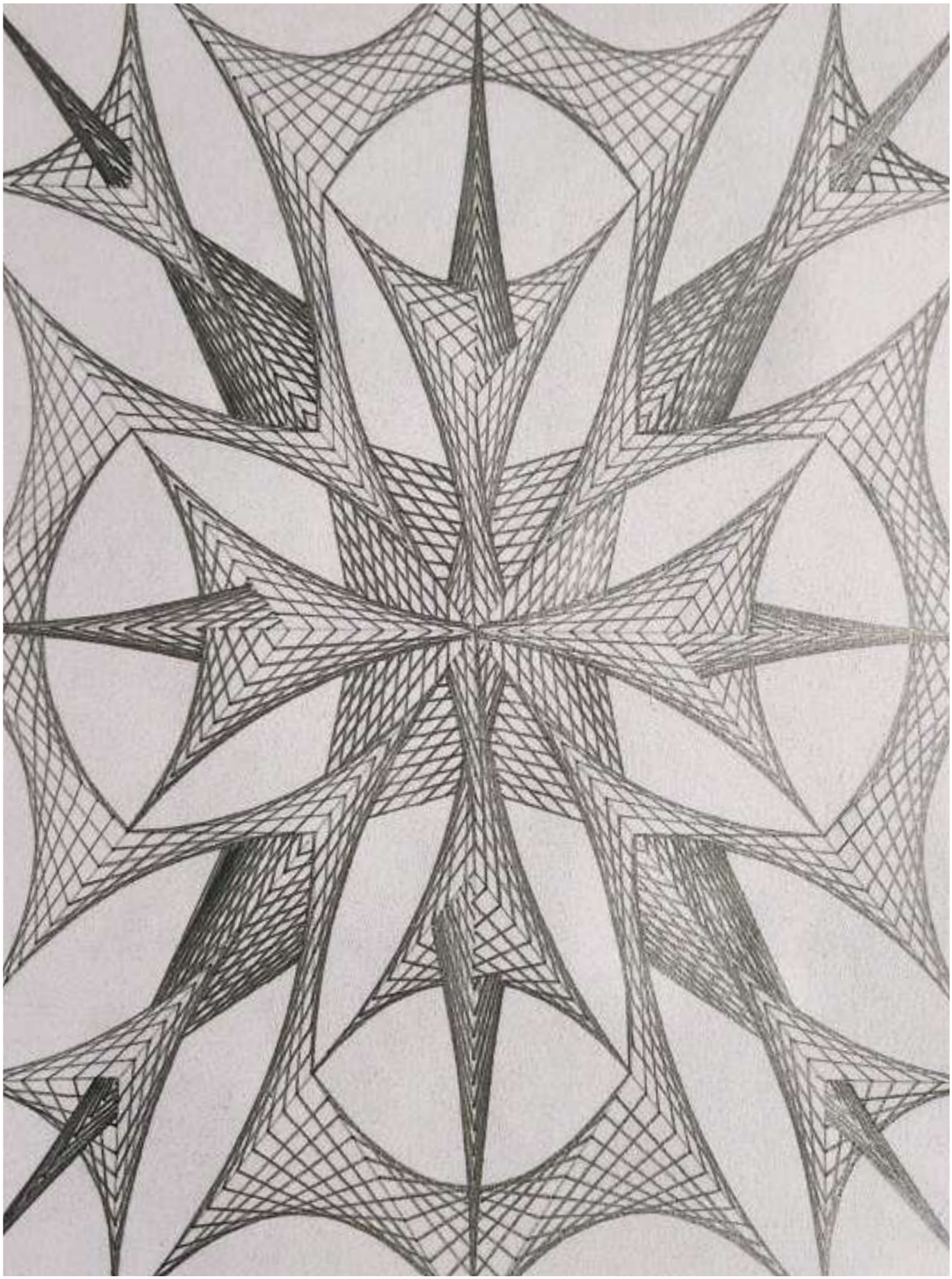
Soleno Therapeutics
Website : https://soleno.life/

Beaini Financial Solutions
Website : https://www.bfs-partners.com/

Larimar Therapeutics
Website: https://larimartx.com/

IndoUSrare acknowledges all our volunteers for their continued support and tireless work through the years.

NOTES



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Nov 16-18, 2024

for

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