



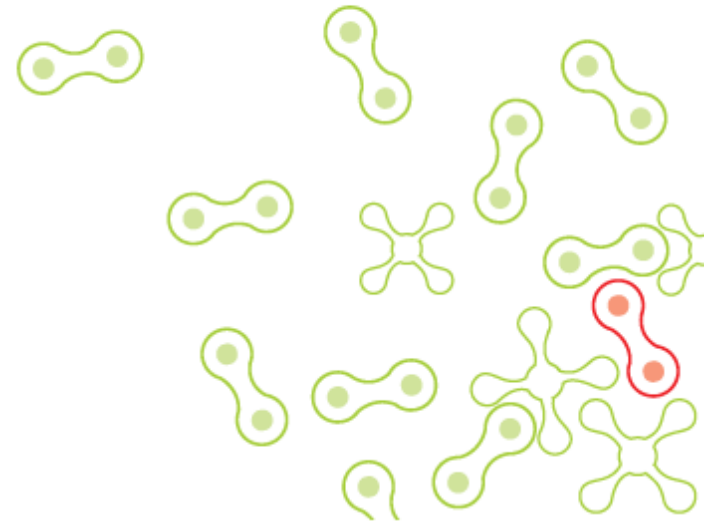
Uniting and Connecting >70M patients with rare diseases in India with Global initiatives

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Virginia, USA and India*



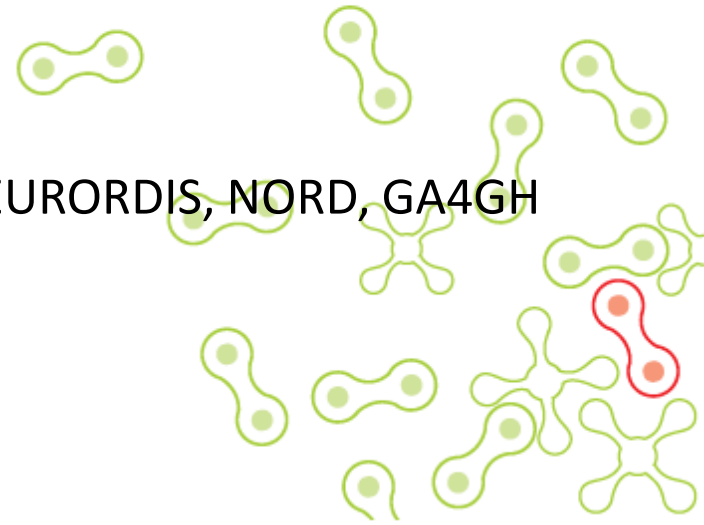
Affiliations and Disclosures

- ⌘ President and Founder, Jeeva Informatics Solutions, MD, USA
- ⌘ Center for metabolic and rare diseases, George Mason University, VA, USA
- ⌘ Life Sciences Solutions Consultant, NTT DATA Services (formerly Dell Services), VA, USA
- ⌘ Scientific Advisory Board, Synergy BIS, VA, USA



Overview

- ⌘ The Beginnings... History, Vision, Mission
 - ⌘ Best practices and inspiration drawn: NORD, EURORDIS,
- ⌘ Awareness
 - ⌘ Racefor7 – numerous corporate sponsors, 7000 runners/walkers/crawlers
- ⌘ Rare Disease Care Coordination Centers – Hub and Spokes Model
- ⌘ Research, Review, Facilitation, and Translation to Indian Languages
- ⌘ Government Policy Advocacy
 - ⌘ ODA versus Rare Disease Policy
- ⌘ Recent Progress in India
 - ⌘ Multiple rare disease organizations, volunteers
- ⌘ Upcoming Events
- ⌘ Next steps – Patient registries, IRDiRC, ICORD, EURORDIS, NORD, GA4GH



Vision, Mission, Goals

- ⌘ Founded in 2013, launched from the constitutional club of India in Delhi
- ⌘ Represent the collective voice of patients with rare diseases to GOI and Stakeholders
- ⌘ ORDI's vision is to make rare diseases diagnosable and treatable just like common diseases in India
- ⌘ ORDI's mission is to be the collective voice of every rare disease patient, family member, and healthcare professionals in India by pooling together expertise, efforts, and resources to help early diagnosis, treatment and management of rare diseases in India



⌘ First literature review published in 2014 with proposed definition for RD

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Research Papers

Organization for rare diseases India (ORDI) – addressing the challenges and opportunities for the Indian rare diseases' community

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DEVARAKONDA^{a9}, USHA KINI^{a10}, KINNIMULKI VIJAYACHANDRA^{a11} and ISHWAR C.
VERMA^{a12}

Country	Definition
United States	< 200,000 total
European Union	< 1 in 2,000
Australia	< 1 in 2,000
Taiwan	0.1% of population
South Korea	< 20,000
India (proposed)	< 1 in 5,000



Burden of Genetic Diseases in India

Source: <http://GeneticsIndia.org>; Sir Ganga Ram Hospital

India - Population 1.1 billion Births 27 Million Per Year

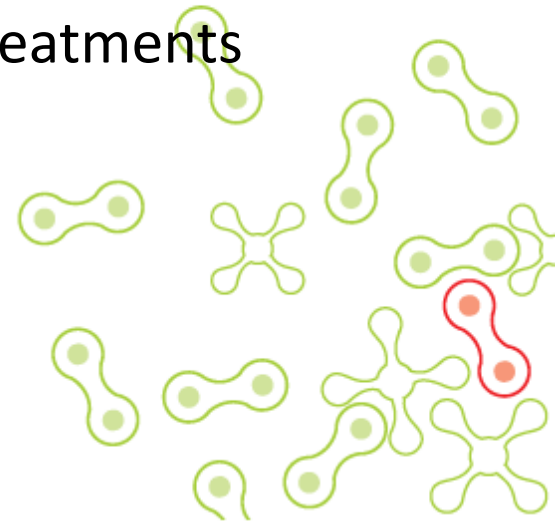
Disorder	Incidence	Births / year
Cong. Malformations	1: 50	678,000
Down syndrome	1: 800	34,000
Metabolic disorders	1:1201	22,477
B- thalassemia+SCD	1: 2700	16,700
Cong. Hypothyroidism	1: 2477	10,900
Duchenne MD	1:5000 (M)	2,700
Spinal muscular atr.	1:10,000	2,700

Largest number of affected infants per year in the world



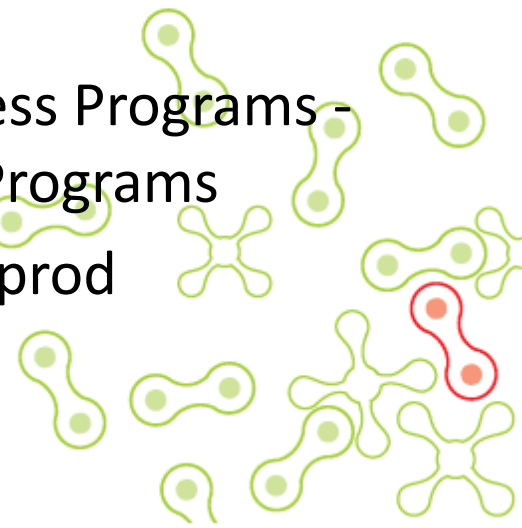
Indian Healthcare Ecosystem

- ⌘ Majority population rural
 - ⌘ ~30 – 70 versus ~70 – 30 [Source: National Census data]
- ⌘ Lack of Awareness
- ⌘ Training doctors; http://www.mciindia.org/tools/announcement/MCI_booklet.pdf
 - ⌘ MCI added genetics and mol bio to MBBS curriculum in 2012
 - ⌘ Number of medical genetics departments are inadequate
- ⌘ Lack of Infrastructure, accessibility
- ⌘ Prohibitive costs of Diagnostic odyssey and treatments
- ⌘ Cultural practices such as consanguinity
- ⌘ Government policy and
- ⌘ Funding



Goals of ORD

- ⌘ Represent the collective voice of all stakeholders of rare diseases in India; Form an united coalition
- ⌘ Make rare diseases diagnosable and treatable (much like common diseases are)
- ⌘ Enactment of ODA and Rare Disease Policies at central and state levels
- ⌘ Connecting 70+ M patients with any of 7000+ rare diseases in India with Global initiatives
- ⌘ Compassionate Use Programs - Expanded Access Programs - Early Access Programs - ATU - Named Patient Programs
- ⌘ Facilitate and encourage research and orphan prod development activities



Best Practices Recommendations

Integrated national strategy to accelerate rare diseases R&D:

1. Active involvement and collaboration by a wide range of public and private interests
 1. government agencies, commercial companies, academia, investigators, PAGs.
2. Timely application of advances in science and technology that can make rare diseases research and product development faster, easier, and less expensive.
3. Appropriate use and development of trial design and analytic methods tailored to the special challenges of conducting research on small populations.
4. Strategies for sharing research resources to make good use of scarce funding, expertise, data, biological specimens, and participation in research by people with rare conditions.
5. Reasonable incentives for private-sector innovation and prudent use of public resources for product development.
6. Adequate resources, including staff with expertise on rare diseases R&D, for public agencies that fund biomedical research on rare diseases and regulate drugs and medical devices.
7. Mechanisms for weighing priorities for R&D, establishing collaborative as well as organization-specific goals, and assessing progress toward these goals.

RACE
FOR

7000 Rare Diseases | 7000 meters

RACE
FOR

MILLIONS OF PATIENTS HAVE BEEN RACING AGAINST RARE DISEASES.

LET'S MATCH STRIDES WITH THEM, **YET AGAIN.**

Date : 26th February 2017
Venue : St Joseph's Indian High School (beside Mallya Hospital)
Start Time : 6.45 am



 facebook.com/ordindia/

 #ORDIRaceFor7

For registration, please visit
<http://racefor7.com>

contactus@ordindia.org
www.ordindia.org

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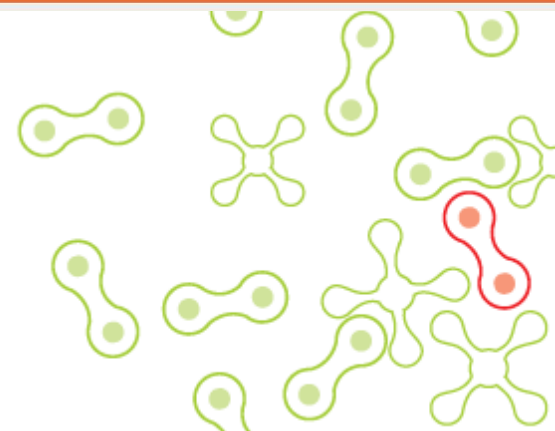


SANOFI GENZYME



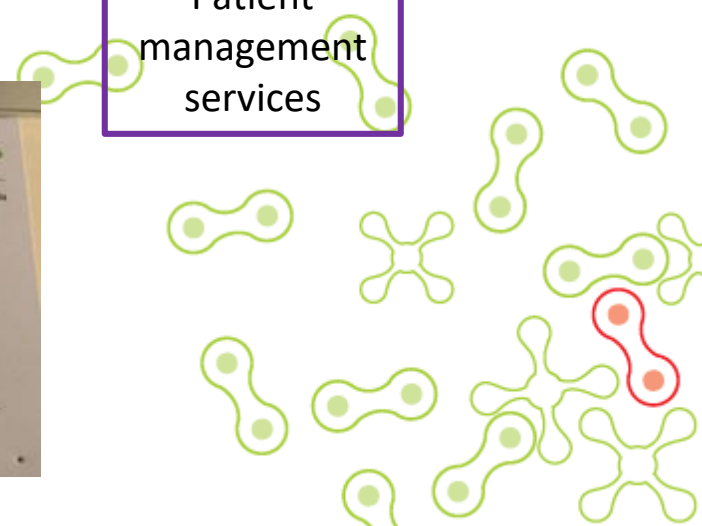
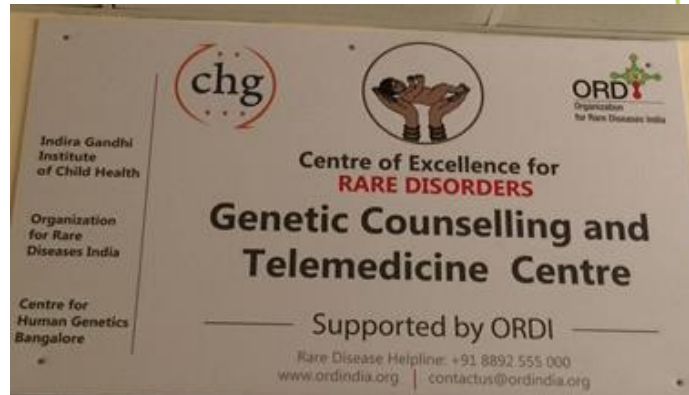
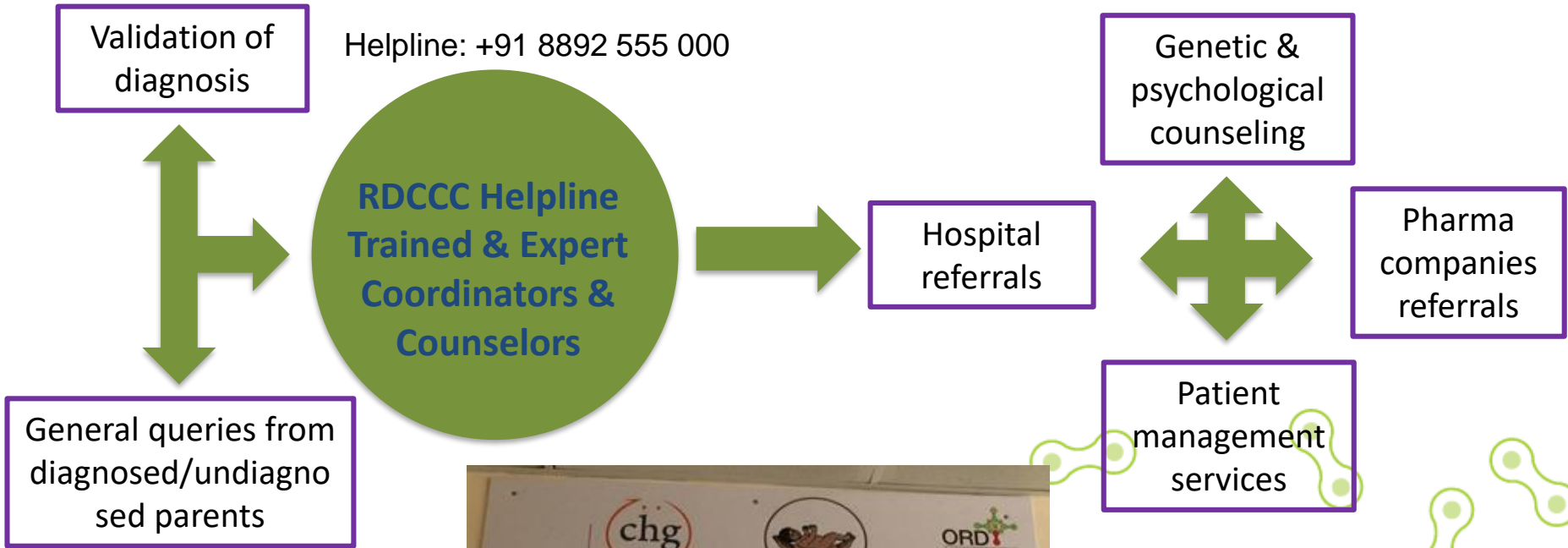
RACE
FOR

7000 Rare Diseases | 7000 meters



Rare Disease Care Coordination Center – Hub and Spokes

Finding patients of Rare Diseases & giving families hope through diagnosis, expert advise, access to potential treatments and potential study enrollments



Sanofi Genzyme Patient Advocacy Leadership Award



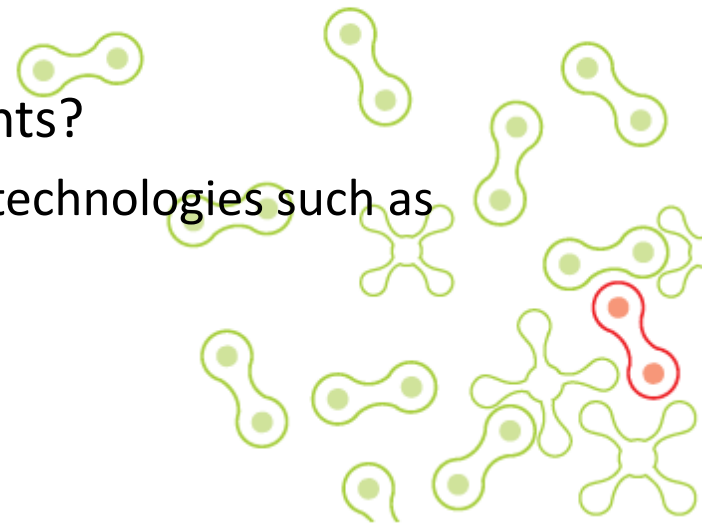
**A 501c3 organization in USA in process to enable international collaborative projects
Welcome collaboration with international**

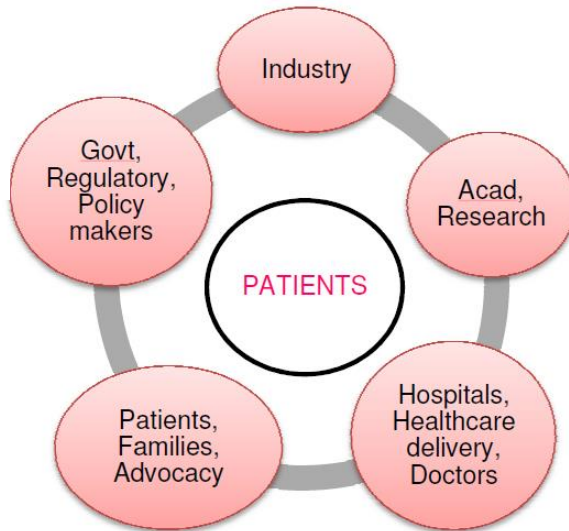


Upcoming...



- ⌘ ORDI Cohosting a conference Oct 30 – Nov 1, 2017 at San Antonio, TX
 - ⌘ <http://rarediseases.conferenceseries.com> Registrations and Abstract submissions encouraged
- ⌘ Promoting Clinical Trials and Research in India and Internationally
- ⌘ Submission of Indian clinical studies to <http://Clinicaltrials.gov>
- ⌘ GMU Student projects involving mobile/web applications addressing patient and pharma use cases
- ⌘ How technology can improve lives of patients?
 - ⌘ mclinical, wearables, telehealth, emerging technologies such as BlockChain





Acknowledgments

Prasanna Kumar B Shirol
(Exec Dir, India)

Co-Founders and Board Members:

- Sangeeta Barde
- Dr. Madhuri Hegde
- Ravinandan ME
- Former Members

- People running Racefor7
- Advisors and Mentors
- Ever growing list of Volunteers
- GMU Students and faculty

Patients

- Team Effort
- Collaborative Approach
- Engage with all PAGs
- Facilitate creation of new PAGs
- It takes a Village to play catch up

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Toughest job lies ahead!

