



## Orphan Drug Act and Rare Disease Policy – Are they same?

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*Co-founder, Organization for Rare Diseases India*

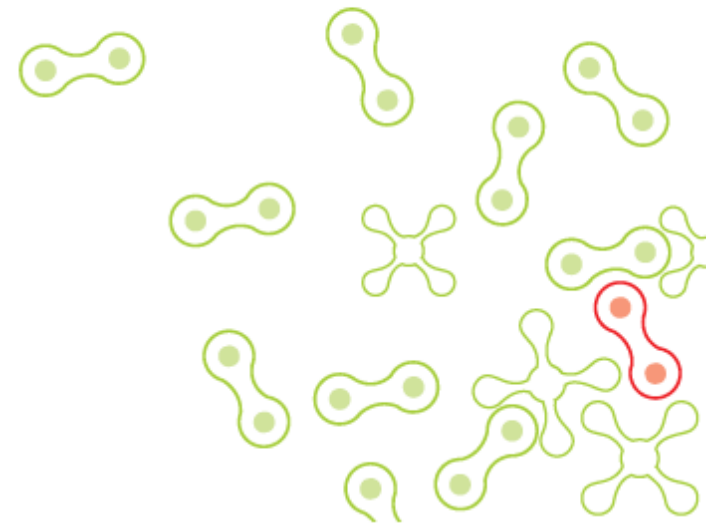
*Co-Director, Center for Rare Diseases, George Mason University*



# Affiliations and Disclosures

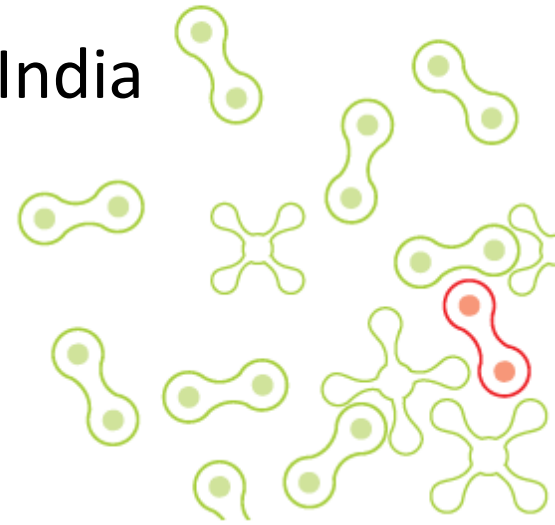


- ⌘ Organization for Rare Diseases India (ORDI)
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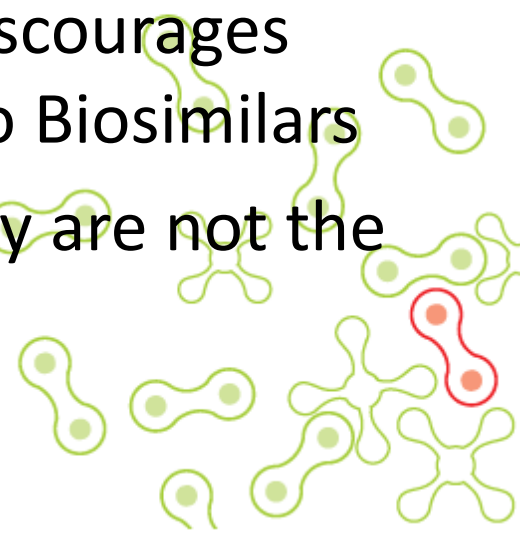
# Overview

- ⌘ The Policy Conundrum
- ⌘ The US Orphan Drug Act of 1983
- ⌘ Impact of ODA on Industry and Patients
- ⌘ Elements of Rare Disease Policy (RDP)
- ⌘ Status of Rare Diseases in India
- ⌘ A Call for Action
- ⌘ Role of Organization for Rare Diseases India



# Introduction

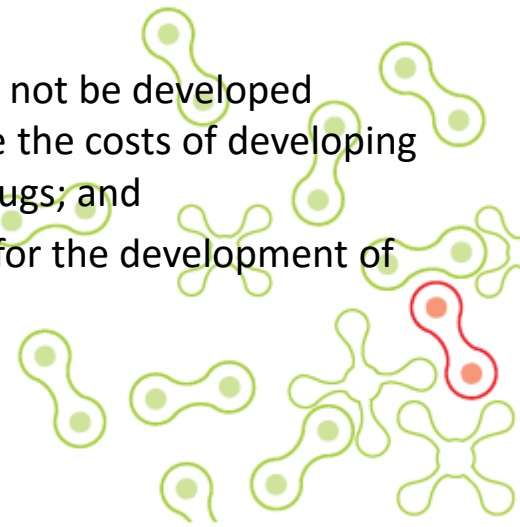
- ⌘ Lot of Activities and Attention for Rare Diseases in last 3 years
- ⌘ India does not have national or state policies that recognize the needs of patients with rare diseases
- ⌘ There are estimated >70 million patients with rare disorders living in India
- ⌘ Science and investment environment discourages Innovation in Biopharma R&D leading to Biosimilars
- ⌘ Orphan Drug Act and Rare Disease Policy are not the same



# Orphan Drug Act (ODA), Jan 4, 1983



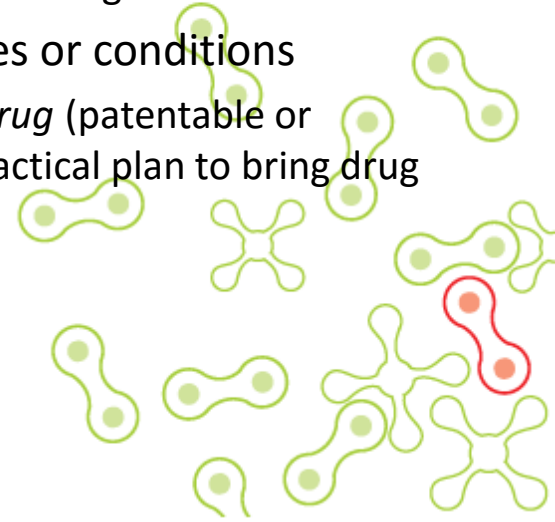
- ⌘ US passed an *Act to amend the food, drug and cosmetic Act to facilitate the development of drugs for rare diseases and conditions, and for other purposes.*
- ⌘ Why ODA?:
  - ⌘ Many diseases and conditions, such as Huntington's disease, myoclonus, ALS (Lou Gehrig's disease), Tourette syndrome, and muscular dystrophy which affect such small numbers of individuals residing in the United States that the diseases and conditions are considered rare in the United States; - **No formal definition existed**
  - ⌘ Adequate “orphan” drugs for many of such diseases have not been developed;
  - ⌘ Because so few individuals are affected by any one rare disease or condition, a pharmaceutical company which develops an orphan drug may reasonably expect the drug to generate relatively small sales in comparison to the cost of developing the drug and consequently to incur a financial loss;
  - ⌘ There is reason to believe that some promising orphan drugs will not be developed unless changes are made in the applicable Federal laws to reduce the costs of developing such drugs and to provide financial incentives to develop such drugs; and
  - ⌘ It's in the public interest to provide such changes and incentives for the development of orphan drugs.



# ODA Highlights; US Public Law 97-414



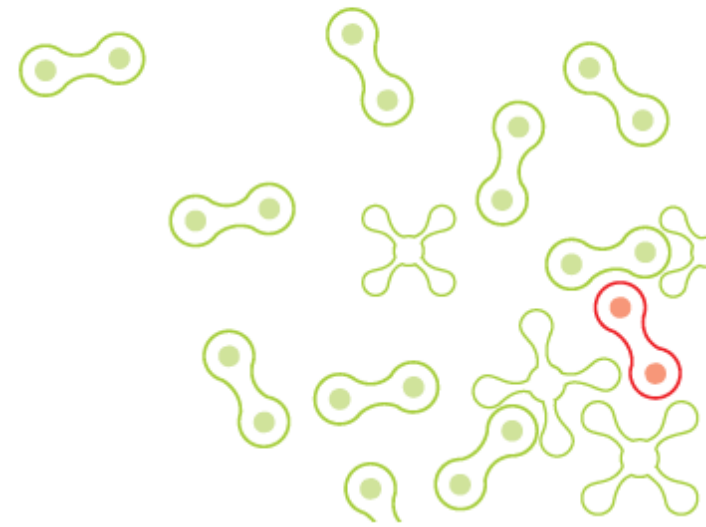
- ⌘ Recommendations for investigation of drugs for rare diseases or conditions
  - ⌘ Health secretary is required to provide written recommendations to an orphan drug sponsor on the non-clinical and clinical investigations necessary for the approval of such drug or biological product.
  - ⌘ The secretary by regulation will be required to promulgate the procedures for implementation of the non-clinical and clinical investigations.
- ⌘ Designation of drugs as Orphan for rare diseases or conditions
  - ⌘ In response to written request by sponsor of a drug, orphan designation is made by secretary based on facts/circumstances as of such date.
  - ⌘ Disease or condition with no reasonable expectation that cost of developing and making available such drugs in US will be recovered from the sales of the drug in US.
- ⌘ Protection for patented or unpatented drugs for rare diseases or conditions
  - ⌘ *Secretary shall not approve orphan designation for any other drug (patentable or unpatentable) for same condition for 7 yrs unless there's no practical plan to bring drug to market*
- ⌘ Open protocols for investigations of orphan drugs



# ODA Orphan Products Board



- ⌘ Appointed by Health Secretary to comprise:
  - ⌘ Assistant secretary of health of the dept of HHS (Chair of the board)
  - ⌘ Representatives from US FDA, NIH, CDC, and any other govt agency related to drugs or devices for rare diseases
- ⌘ Orphan products board will promote orphan drug sponsors, coordinate among all stakeholders, disseminate information to physicians/public, recognize efforts, issue annual reports of approved orphan drugs or designations



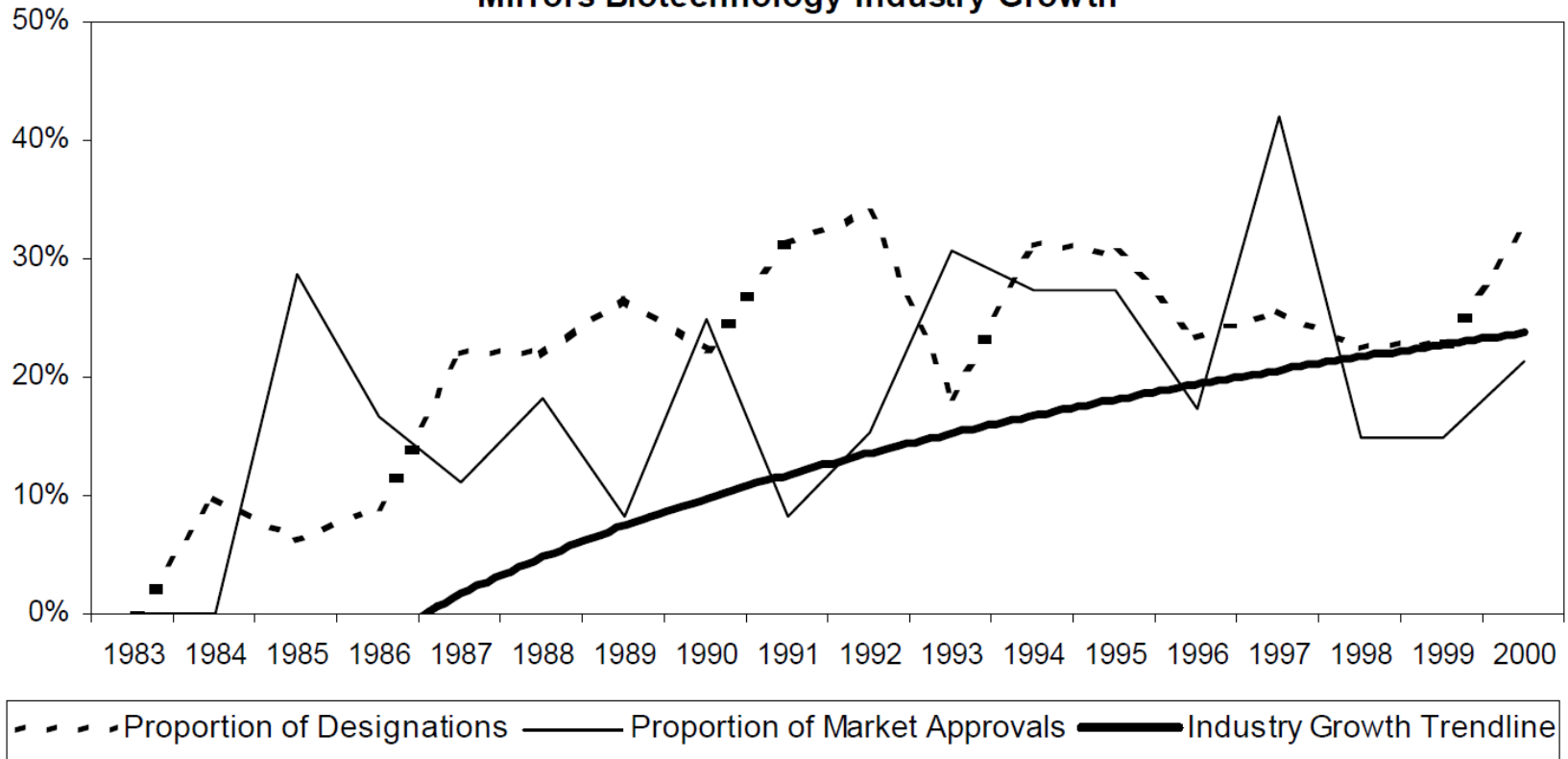
# ODA Highlights Contd.

- ⌘ Tax Credit for testing expenses for orphan drugs
  - ⌘ 50% of costs of Clinical testing of orphan designated drugs
  - ⌘ Research expenses
  - ⌘ Limitations based on tax amount, foreign testing, dates, and other clauses
- ⌘ Waiver of Drug Application or User Fees
- ⌘ Expedited review of orphan drug applications
- ⌘ Home Health Services – reimbursements and training
- ⌘ Grants and Contracts for Clinical Trials and Research



# Impact of ODA 1983-2000

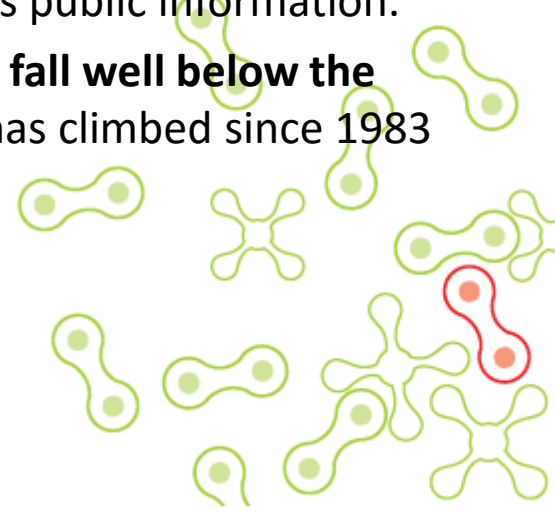
Chart 2  
Growth in Biologic Orphan Products  
Mirrors Biotechnology Industry Growth



Orphan Drug Act – Implementation and Impact; Report by Dept of Health and Human Services, Office of Inspector General; May 2001; OEI -09-00-00380

# Impact of ODA 1983-2000 Contd..

- ⌘ **The Orphan Drug Act's incentives and the Office of Orphan Products Development's clinical superiority criteria motivate drug companies to develop orphan products.** >2,900 designations and approved >450 products.
  - ⌘ 1/3<sup>rd</sup> of all new drugs approved by FDA in last 5 years are Orphan drugs
- ⌘ **Advocates report that orphan products are usually accessible to patients.** Orphan products are usually accessible, although they can be costly and in limited supply. Insurance typically pays for the treatments, and companies offer patient assistance programs to help patients obtain their products.
- ⌘ **The Office of Orphan Products Development provides a valuable service to both companies and patients.** Companies report excellent relationship with OOPD, which awards orphan product designations and disseminates public information.
- ⌘ **Orphan products meet the legal prevalence limit, and most fall well below the threshold of 200,000 patients.** Average patient population has climbed since 1983 but remains well below the legal limit.



# Rare Disease Policy

## ⌘ New Born Screening

- ⌘ Pompe added to RUSP of 58 diseases

Towards a Uniform Screening Panel and System." *Genetic Med.* 2006; 8(5) Suppl: S12-S252" as authored by the American College of Medical Genetics (ACMG) and commissioned by the Health Resources and Services Administration (HRSA).

## ⌘ Diagnosis and Treatment Options

## ⌘ Improving Accessibility and Affordability of Orphan Drugs

## ⌘ State level budget and implementation

## ⌘ Diagnosis and treatment delivery through private and government healthcare in India

## ⌘ What aspects of rare disease care should be made available to patients by Government hospitals?



# Organization For Rare Diseases India



- ⌘ 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
- ⌘ Started off with a literature review and stakeholder interviews

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14

Research Papers

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

HARSHA KARUR RAJASIMHA<sup>a1 c1</sup>, PRASANNAKUMAR BASAYYA SHIROL<sup>a1</sup>, PREVEEN RAMAMOORTHY<sup>a1</sup>, MADHURI HEGDE<sup>a1</sup>, SANGEETA BARDE<sup>a1</sup>, VIJAY CHANDRU<sup>a1</sup>, M. E. RAVINANDAN<sup>a1</sup>, RAMANI RAMCHANDRAN<sup>a2</sup>, KASTURI HALDAR<sup>a3</sup>, JIMMY C. LIN<sup>a4</sup>, IMRAN A. BABAR<sup>a4</sup>, KATTA M. GIRISHA<sup>a5</sup>, SUDHA SRINIVASAN<sup>a6</sup>, DURAISWAMY NAVANEETHAM<sup>a7</sup>, RAJANI BATTU<sup>a8</sup>, RAJASHREE DEVARAKONDA<sup>a9</sup>, USHA KINI<sup>a10</sup>, KINNIMULKI VIJAYACHANDRA<sup>a11</sup> and ISHWAR C. VERMA<sup>a12</sup>

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
<b>India (proposed)</b>	<b>&lt; 1 in 5,000</b>

<http://ordindia.org>



# 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

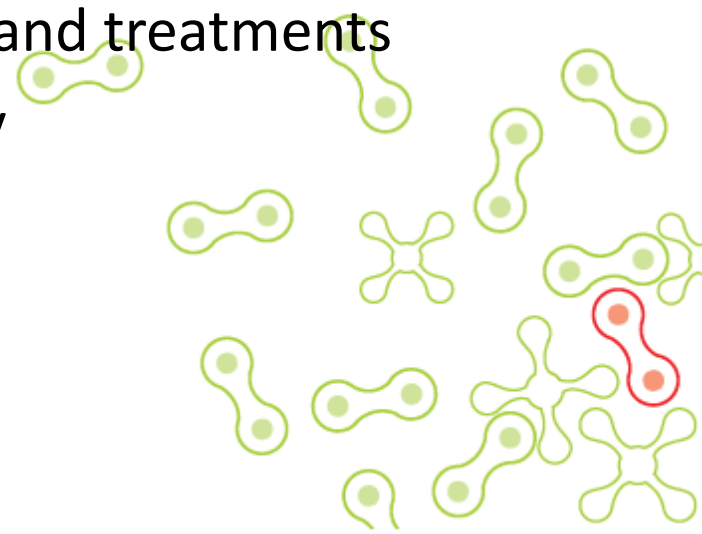
**US is at ~1:1500**  
**For 33 years**

**Largest number of affected infants per year in the world**



# Applicability to Indian Healthcare Ecosystem

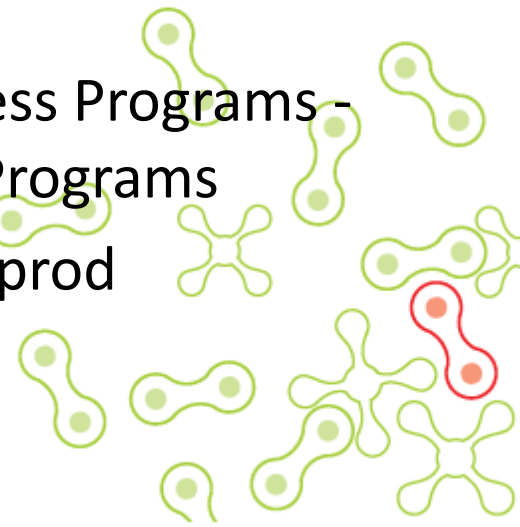
- ⌘ Majority population rural
  - ⌘ ~30 – 70 versus ~70 – 30 [Source: National Census data]
- ⌘ Lack of Awareness
- ⌘ Training doctors; [http://www.mciindia.org/tools/announcement/MCI\\_booklet.pdf](http://www.mciindia.org/tools/announcement/MCI_booklet.pdf)
  - ⌘ MCI added genetics and mol bio to MBBS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



# Goals of ORDI



- ⌘ Represent the collective voice of all stakeholders of rare diseases in India; Form an united coalition
- ⌘ Make rare diseases diagnosable and treatable like common diseases
- ⌘ 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

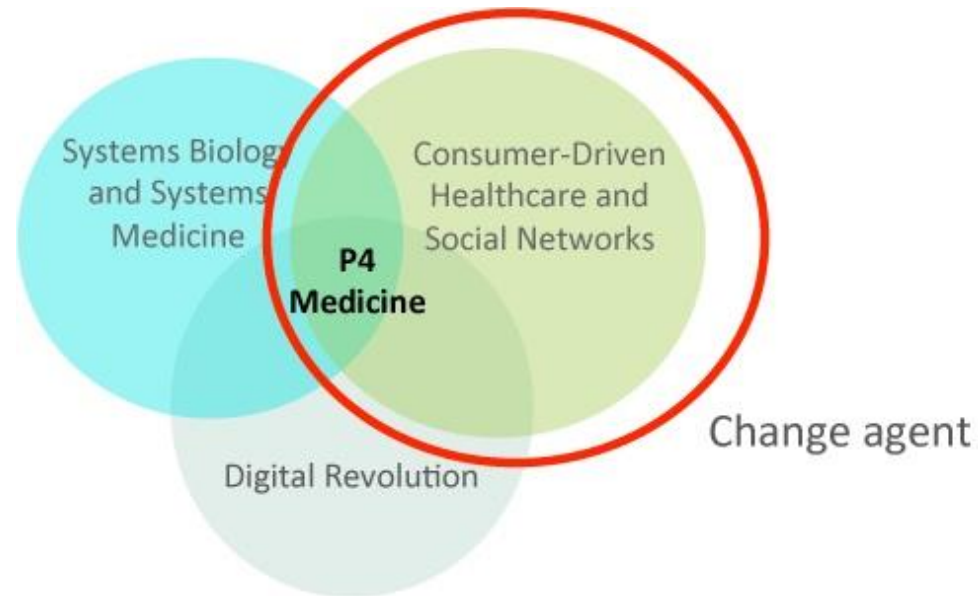
Integrated national strategy to accelerate rare diseases R&D:

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.

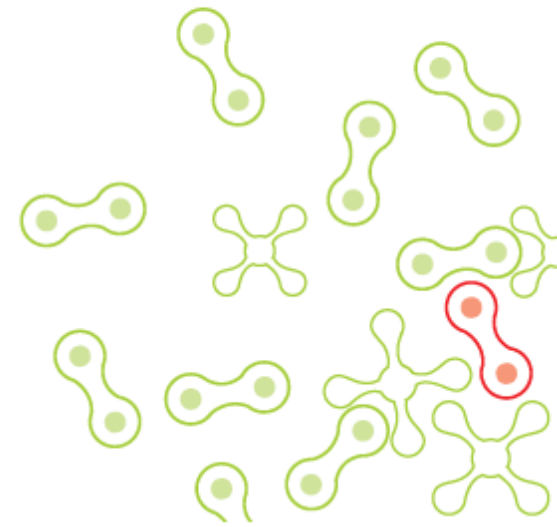


# P4 Medicine; Social Media; Change Agents

- ⌘ Preventive
- ⌘ Predictive
- ⌘ Participatory
- ⌘ Personalized



- ⌘ Technology is not a limiting factor
- ⌘ Practical plan and Funding are



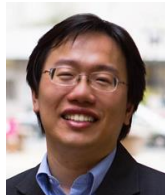
# Recommendations for India

- ⌘ Time for Action for us
- ⌘ Can GOI or our consortium form a RD Task Force?
  - ⌘ Representatives from all relevant agencies
    - INSA, ICMR, DBT, DST, CSIR, ISCR, ... ORDI? International collaborators?
- ⌘ Pledge annual minimum RD research fund
  - ⌘ A requirement to be part of iRDIRC
- ⌘ Implement SACHDNC Recommended Uniform NBS Panel
  - ⌘ Secretary's Advisory Committee on Heritable Disorders in Newborns and Children – 58
  - ⌘ <http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/recommendedpanel/index.html>
- ⌘ Support Clinical Research Grants for orphan drugs or products
- ⌘ Drive affordability and adoption of validated new technologies
  - ⌘ Next-generation DNA sequencing, Nanotechnology, stemcells

# Selected International Collaborations



- ⌘ Collaboration between ORDI and GMU
- ⌘ Academic Affiliation for Fostering Collaborations in US/EU
- ⌘ <http://cscmd.cos.gmu.edu>



- ⌘ Jimmy Lin, MD, PhD
- ⌘ 159 page book – Email me for a PDF



- ⌘ Foster Collaboration and Innovation; <http://genomicsandhealth.org>
- ⌘ Responsible data sharing globally; working groups

## Global Initiative



- ⌘ Diagnose most rare diseases and 200 new orphan therapies by 2020; Worldwide connecting of patient registries
- ⌘ 3 scientific committees on Dx, Therapies and interdisciplinary with 12 working groups; RD Classification and Codification
- ⌘ <http://www.irdirc.org>; Policies and Guidelines 2013

<http://ordindia.org>



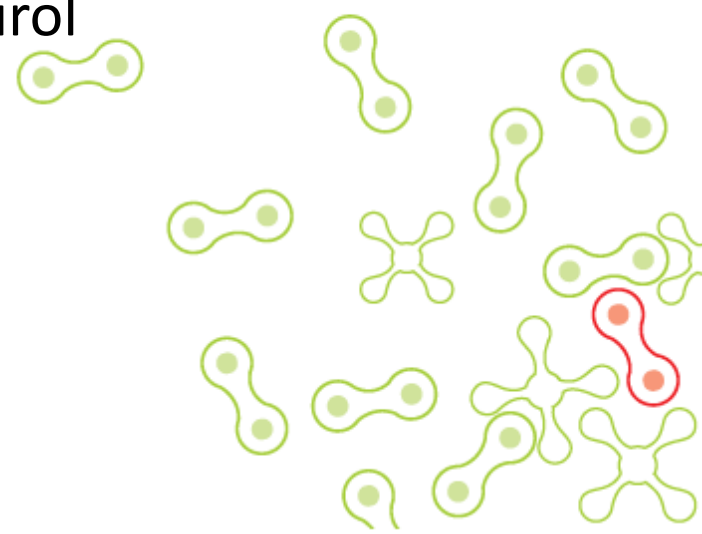
# Rare disease care coordination center



- ⌘ Hub and Spoke Model – Sponsored by Quintiles
- ⌘ ORDI is the central data collection/coordination site
- ⌘ Multi-specialty hospitals with commitment to develop rare disease board (Centers of excellence)
  - ⌘ MS Ramaiah Hospital is generously supporting the DCCS
  - ⌘ Kasturba Medical College
  - ⌘ Adding More Spokes and Sponsors
- ⌘ We are hiring! Contact Mr. Prasanna Shirol



<http://ordindia.org>



# Karnataka Rare Disease Policy Update



- ⌘ The Vision Group for BioTech (VGBT) is leading and liaising with the state Health Secretary in Karnataka
- ⌘ ORDI has submitted a draft policy framework to VGBT
- ⌘ Recognized institutions of leadership provided input into draft policy
- ⌘ This model of a Government appointment Vision group leading an united coalition of prominent stakeholders to present a well-rounded draft policy to the Government may serve well for other states and central governments



[www.Racefor7.com](http://www.Racefor7.com) Come, join the Race!



# MILLIONS OF PATIENTS HAVE BEEN RACING AGAINST RARE DISEASES.

LET'S MATCH STRIDES WITH THEM.

Date : 28<sup>th</sup> February 2016  
 Venue : St Joseph's Indian High School (beside Mallya Hospital)  
 Start Time : 6.45 AM



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 #ORDIRaceFor7

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

contactus@ordindia.org  
 www.ordindia.org

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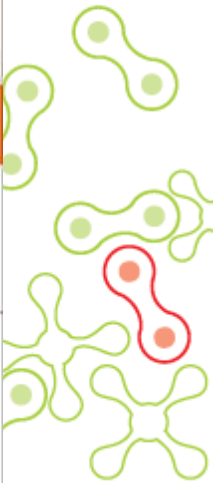


7000 Rare Diseases | 7000 meters

**Rare Disease Helpline +91 8892 555 000**

Organised by ORDI in association with NNF- Karnataka Organization for Rare Diseases India 	Platinum Sponsor QUINTILES	Diamond Sponsor CENTOGENE THE RARE DISEASE COMPANY PerkinElmer For the Better
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# Team ORDI



Mr. Prasanna Shirol



Dr. Harsha Rajasimha



Ms. Sangeeta Barde

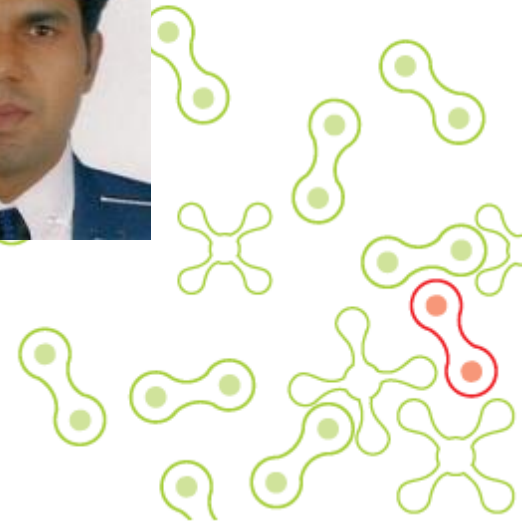


Dr. Madhuri Hegde, FACMG



***“If you have a sick child, it’s not a rare disease; it’s the only disease that matters.”***

Mr. Ravinandan M E



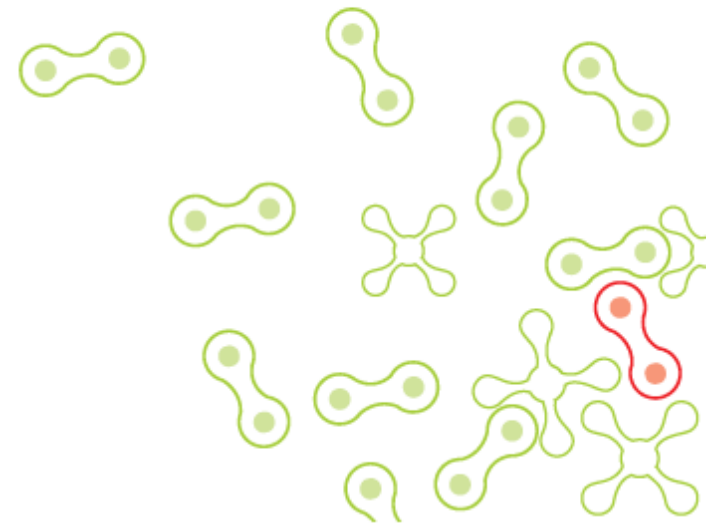
ORDI runs a national rare disease hotline (+91 8892 555 000)

<http://ordindia.org>

# Non-profit Neutral Framework for collaboration



- ⌘ It takes time to setup a NGO
- ⌘ ORDI can provide the administrative framework to enable grants for investigators with good research projects
- ⌘ Synergistic win-all collaborations are welcome and will be supported



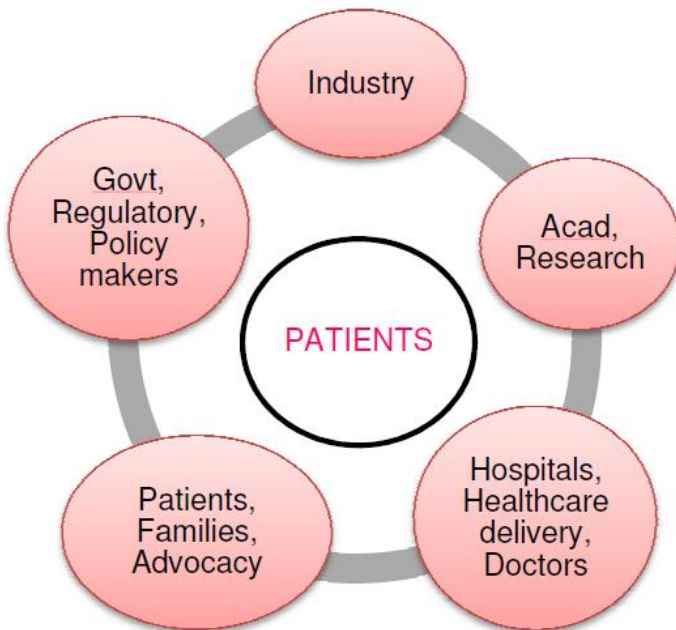


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  - ⌘ Peter Masschelin
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  - ⌘ Shreya Nagesh
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