



# ECRD

**Rare Diseases 360°**

Collaborative Strategies to leave no one behind

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Orphan Products**

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# Goal - Panel

**Create a shared vision to access the care to RD patient's that suits local Environment**

**Know the existing programs, challenge and opportunities**



**India**





# Current Status of RD in India

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- **RD are not priority**
- **Many burning issues**
- **Patients have no voice**



# We Have Dreams

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we dream alone, they will be dreams only  
we dream together, they become reality

# Diagnosis & Care

**“Why bother to diagnosis (or undiagnosed) when there are no treatment options” attitude**

**(partial) wish list)-**

- **Raise the awareness of rare diseases**
- **Emphasize Importance of early diagnosis with patients, health care promoters and medical professionals**
- **Educate the public - people with no illnesses (92%)**
- **Gain the support from the government, industry etc**

## Diagnosis & Care -2

- Initiate insurance plans
- Make available (inexpensive) Medical foods to treat inborn errors (folinic a, Betaine, cobalamine, manose etc.
- Exclude orphan drugs from import taxes and tariffs
- Uniform policy throughout the country
- Make orphan drugs available in the country (400!)

# Challenges

How to

**“Make equity for all persons with RD”**

- Political will
- RD issues are Central or State Government?
- Diagnoses and treatments - expensive & not reachable
- Technologies (developed countries) move global equity
- support from Developed Countries- opportunities, obligations
- Charity programs; surplus medical equipment, Drug Discounts



# What are we (IORD) doing? -1

- National survey to get the base-line understanding of RD
- Formulation of Orphan Drug Act - A road map to Govt. India - National Health policy - currently status
- Amendment of Drugs and Cosmetic Act 1945 to include OD & RD
- Workable, effective interventions - Prevention, reducing the incidence
- Educational programs at non-medical and medical colleges

(Medical council of India, Indo-US medical association etc.)





## What are we (IORD) doing? -2

- Diagnosis is a real problem
- Genomic Diagnostic labs
- Newborn Screening - primary healthcare centers
- Simple patient registries (help Rare connect)
- Raising the awareness through non-profit social organizations such as Rotary, Lions clubs
- Access to social services, education and occupation and rehabilitation services through CSR programs
- Patient voice: through patient support groups



# Collaborations – work in progress

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**MoU – Manipal Educational Trust**

✓ **MoU – Kapadia Diagnostic  
laboratories**

**Strand Life Sciences (no MoU)**

**Mapmygenome Inc, (no MoU)**

**MoU – AYUSH Ministry**

# Collaborations

- Genetics & Biomedical Research Centers - (half dozen)
- Collaborations (MoU) with Foundation For Research in Genetics and Endocrinology (FRIGE), Institute of Human Genetics  
ERT program: Shire Charitable program
- Orphan Drug Development: repurposing: encouraging and consulting pharma companies
- Encouraging NGO's and universities to initiate R&D for Orphan Drugs (NIPER, etc.)

**"If you think Research is expensive try Disease"**

THANK YOU