

Power of Rare Disease Patient Groups: Africa Stepping Up!



RAREX 2016

Patient Organizations Feedback

October 22, 2016

Key Points

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- (Almost) every rare disease/orphan drug policy was the result of patient group advocacy; the history of rare disease policy is the history of patient organizations
- Most rare disease patient groups are started by patients and parents; some are started when a new orphan drug is developed
- History of rare disease groups is also history of orphan drug discovery and pharmaceutical industry support
- Rare disease groups are (even more) impactful when they work in partnership with others groups and with other stakeholders
- Regional and international rare disease alliances will be even more important in the future

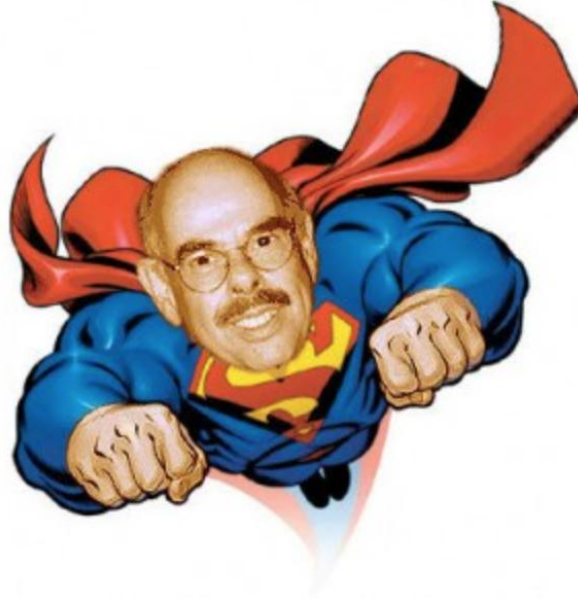
What Rare Disease Alliances Can Do

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- Orphan drug/rare disease legislation inspired by parents in patient organizations in USA, Europe, Taiwan, Japan, Canada
- EURORDIS: European patient organization with resources and ear of policy makers but powerful because of members: 600 active patient groups representing 4,000 rare diseases in 50 countries
- International alliances: international disease organizations and research consortiums regional alliances, Rare Disease International

USA Orphan Drug Act: A Mother, a Congressman, an Actor

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Taiwan Foundation for Rare Disorders: From Parent Hope to Patient Community



1999

1 Mother + 1 Father:
Serena Wu & Dr. Min-Chieh Tseng)

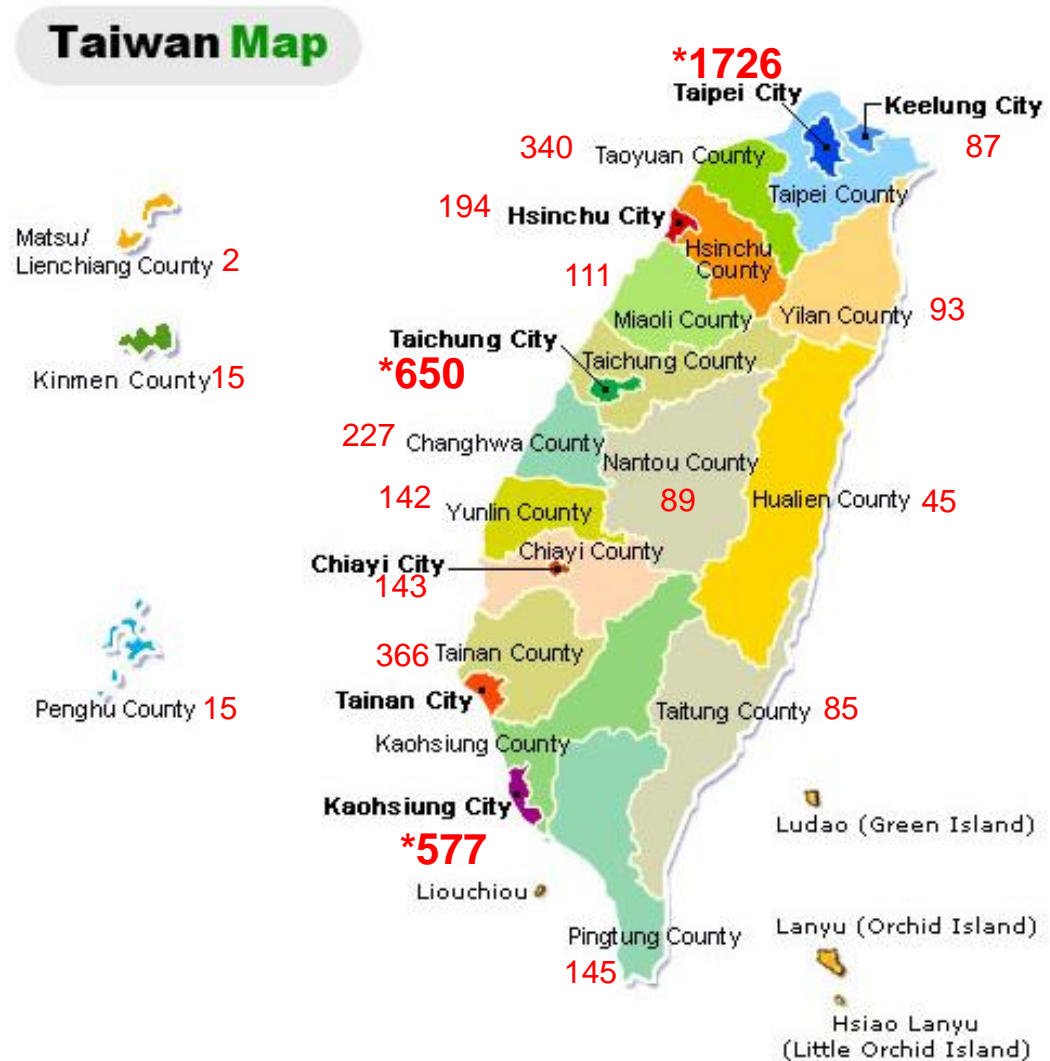
*"We can't take care of our children forever,
but a well-established system can."*



2011: TFRD awarded the 20th Medical Dedication Award

Where is TFRD Today? (2014)

- Currently TFRD serves **5,052** patients with **235** kinds of diseases and provides service to **35** patient groups in Taiwan.
- Three offices with 50 full-time staff
 - Taipei Main Office
 - Taichung branch office
 - Kaohsiung branch office



2014 Threat: Taiwan Pharmaceutical Benefit & Reimbursement Scheme Assesses Orphan Drugs for National Health Insurance

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- Challenge of Cost-Effectiveness: Save **ONE** rare disease patient (aHUS) @NT\$210,000 or Save **FIVE** other patients?
- Challenge HTA process: Delay of 2-3 years after FDA, so NO drug reimbursed
- Step 1: TFRD brings 32 Patient Groups to fight together (led by MPS Society)
- Step 2: TFRD takes problem to Medical Community (Taiwan Human Genetics Society)
- Step 3: Raise public awareness (13 episodes of TV talk show)
- Step 4: Lobby government—Resulting in revised law Rare Disease Prevention and Orphan Drug Act (V4) (2015)
 - Medical Subsidy fills gap between FDA approval and new drug assessment and reimbursement from NHI .
 - AND New RD Act provides more services from government, such as Psychological Consultation, Medical devices subsidy and funds for clinical research



Japan Patient Association: Success Through Coordinated Action

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- 1958: Outbreak of SMON; unknown origins = 1st NANBYO
- 1960: National Rheumatism Association = 1st NANBYO PG
- 1972: 1st Measures against Intractable Disease—research, medical care
- 1975: Establish All NANBYO PGs Association
- 1978 Symposium by Regional Liaison Committee
- 1979: Subsidy system for orphan drug development
- 1986: JPC (NANBYO PGs Association + Regional Liaison Committee)
- 1998: Pilot project for RD; inpatient facility for intractable RD patients
- 2003: Establish RD counseling and support center
- 2004: Revise Child Welfare Act to Enshrine pediatric/chronic disease
- 2005: Japan Patient Association (Merge JPC and NANBYO Centers)
- 2014: NANBYO law and revised Child Welfare Act enacted

NAN	BYO
難	病
Difficult	Disease



Sharing Expertise and Experiences

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October 2015

Who and How Patient Grops

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- Parent; place to share
- Share information
- Reviving organization



WHO

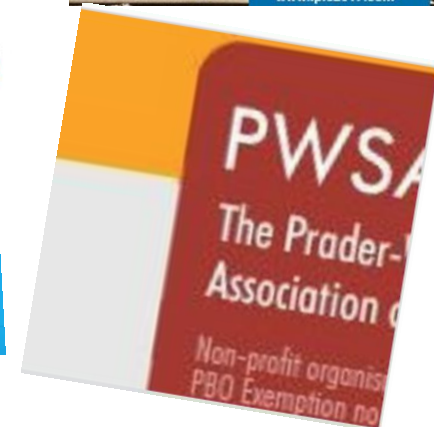


reach for a dream
inspiring hope

Rare Diseases South Africa



WHO



WHY

A word cloud of terms related to patient advocacy and digital health. The words are arranged in a roughly circular shape, with 'Information' written vertically on the right side. The words include: Patient-centric, Alone, Awareness, Child Advocacy, Support, Media, Twitter, Trusted, Facebook, Fundraising, Share, Chat, Parent, and Social. The words are in various colors (red, yellow, orange, green, blue, purple) and sizes, with 'Information' being the largest and most prominent.

Information

Parent Chat Social

Support Child Awareness Alone

Trusted Facebook Fundraising

Share Twitter Media Advocacy Patient-centric

EXPERIENCES of RAREX Patient Advocacy Groups/1å

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• PROUDS

- Stabilization
- Social Media
- Rebranded
- Bring “bit of hope”
- Committed volunteers
- “1” of only “2” in disease
- Support to other countries
- Facebook w/600 members
- Parents have place to share
- ICORD Conference
- Small Organization

• SORRY’S

- Need patient registry
- National footprint in all provinces
- Outreach to all candidates
- Reach Rural patients
- Network w/more POs
- Funding
- Referrals from doctor
- Delegate more
- Expand to public sector

Experiences of RAREX Patient Advocacy Group/2

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• PROUDS

- ICORD Conference (success)
- Small organization
- Traditional group almost closed; set up Facebook page
- Members have come back
- Give info on rare diseases
- Member have joined
- Meet bi-monthly; WhatsApp
- Hospital visits – to “cheer up”

• SORRY'S

- Parents (users) share more
- Outreach low income
- Learn to delegate
- Grow in public sector
- Increase medical community awareness
- Funding for therapy
- Funding
- Diagnosis
- Awareness RDs in hospital

Experiences of RAREX Patient Advocacy Group/3

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• PROUDS

- 350 patient registry
- Social media support group
- Chapters around the world
- Tweet chats, sustainable
- Awareness expanded
- Prodded government
- Patient centric
- Needs of patients first
- Trusted brand globally
- Getting delivery on time

• SORRY'S

- Look after all patients
- Waiting for CPGs
- People don't know how to use “#” in social media
- Reach patients in rural areas
- Include not just rare patient organizations
- Not just access
- Better social media
- Manage red tape

How Can RDI Help?

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- Fundraising
 - Social media tools for fundraising
 - Greater awareness of rare diseases
- Support for government relations
 - Give credibility to cause and to patient groups
 - Facts and figures re: rare diseases (scope, impact)
 - Data re: undiagnosed; implications of no diagnosis
- Country status
 - Contact for Rare Disease Day
 - Toolkits and templates to engage local patient group



Barriers to Collaboration

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Competition

- Perception of “zero-sum” resourcing
- Lack of perceived benefit



● Medical professionals

- Ego
- Intimidated to fight cause
- Lack perceived value of patient advocacy



● Professionalization of patient associations (needed)

- Training, tools, templates
- Train national groups to train local groups