

Day one-presentation-session IV
Sukirti Bagel

Patient advocacy groups & Patient registries: An overview

Sukirti Bagal, MD, MPH – National Organization for Rare Disorders, Inc.

NORD provides information about diseases, referrals to patient organizations, research grants and fellowships, advocacy for the rare-disease community, and Medication Assistance Programs (MAP) that help needy patients obtain certain drugs they could not otherwise afford. NORD works primarily with patients and families to facilitate the access and development of new treatments to become available to the rare disease community. Now, after more than 25 years, there still is a huge unmet need for treatments to be made available to the rare diseases community.

Via this presentation, NORD's undertaking is to provide an overview of the historical successes in advancing drug development from efforts of the patient advocacy groups and lessons learnt from them. Along with being the clearinghouse for information on rare disorders, NORD also has an enriched database of over 300, 000 individuals affected by rare diseases/disorders and currently undertaking the process of distilling it into disease specific registries along with the recent launch of an international online community in partnership with EURORDIS.

This presentation is geared towards capturing the essence of the change in role for the patient groups as a player in the drug development process by taking on an enhanced role of identifying, embracing and dressing the need for setting up the infrastructure along with developing tools such as on-line communities, patient registries, trainings that can facilitate to fill the void of current unmet needs in the rare disease community. NORD is especially interested in contributing to the promotion of unity and collaboration amongst the rare disease community by providing outreach and support to this initiative via its relationships with over 140 disease specific organizations that are part of the NORD consortia and to the patients that are reached by NORD.



Uniting Rare Diseases

Advancing Rare Disease Research: The Intersection of Patient Registries, Biospecimen Repositories and Clinical Data

Session IV

Patient Participation & Outreach Activities/Patient Advocacy

Sukirti Bagal MD, MPH.

Director of Medical Affairs-NORD

Patient Advocacy Group & Patient Registries: An Overview

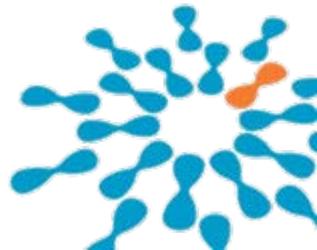


NORD
National Organization for Rare Disorders



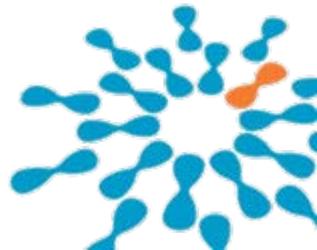
Some common problems that people with rare diseases describe to us...

- Frustration over lack of a treatment or cure for a particular disease.
- Treatments tend to be expensive, and there are many issues related to access and reimbursement.
- Getting an accurate diagnosis takes a long time.
- Finding a physician or treatment center familiar with their disease is challenging.



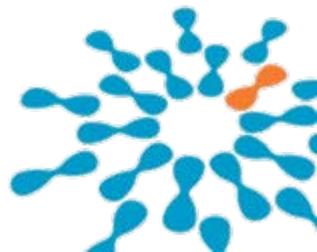
Today.....

- There are close to 7,000 rare diseases.
- There are over 2,000 orphan designated products by the FDA.
- But only 344 approved treatments by the FDA.
- For only about 200 rare conditions!!



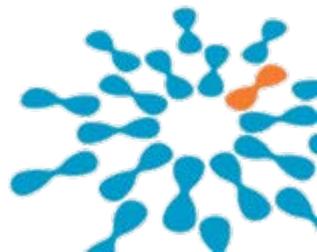
Earlier scenario for rare diseases

- Few companies were interested in orphan drug development.
- Fewer researchers were interested in careers investigating rare diseases.
- Limited funding opportunities.
- Fewer patients groups were established.



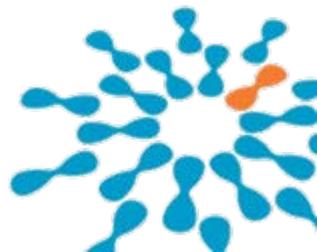
Understanding NORD's role in patient advocacy via our history

- When the U.S. [Orphan Drug Act](#) was approved in 1983, there were just a few rare disease patient organizations.
- These organizations provided crucial advocacy for the [Orphan Drug Act](#).
- Working together, they had greater power than working alone and formed NORD- National Organization for Rare Disorders.
- The patient community had success because they united together to reach a common goal...of making more treatments available for rare diseases!!



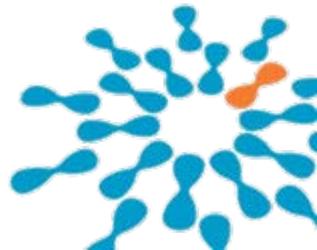
Successes from patient advocacy include.....

- NIH ORDR
- FDA OOPD
- NIH RDCRN
- And now NIH TRND



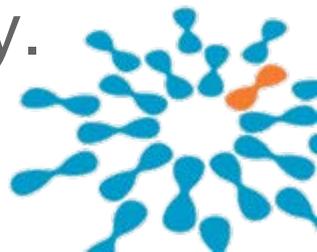
Scenario now.....

- Significant increase in interest from larger pharma and biotech sector.
- More researchers interested as funding has improved dramatically and rare diseases are “untapped” opportunities.
- Scientific advances such as gene test, therapies and personalized medicine on the horizon.
- Rare and Neglected diseases now part of the global healthcare scene.



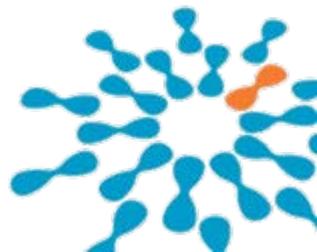
Yet ..only 344 treatments so far !

- Fewer successes in the R&D efforts for rare diseases.
- Pharma struggles to complete trials due to lack of patient recruitment.
- Researchers do not have budgets and time to study the natural history of rare diseases.
- Patients are wary of clinical trial participation & commercialization of drug development.
- The rare disease patients are a dispersed population and are often placed globally.



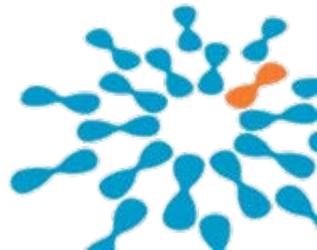
Role for patient groups today.....

- Once again the unmet need presents an opportunity to influence the drug development landscape!!
- Develop tools to facilitate research and innovation of new treatments for rare diseases such as Patient registries & On-line communities.



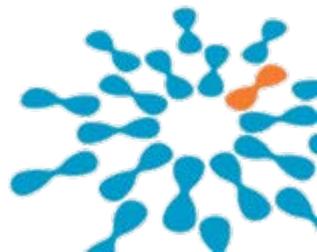
Patient Registries.....the need of the hour!!

- Patient groups to facilitate
 - Clinical trial participation: patients need treatments and treatments need patient participation
 - Translational research: patients need research and researchers need patients.
- Both these critical elements of developing treatments can be addressed/facilitated by Patient registries.
- Patient groups can be the “bridge” to meet the need.



Patient Registries: An ideal role for patient groups

- They have the trust from their patient community.
- They are connected with their patients.
- Have like minded goals and are motivated by similar mission.



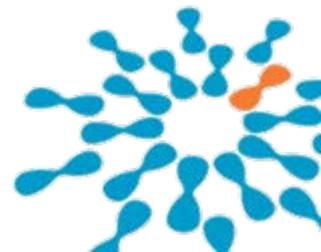
Challenges

- Not all diseases have patient groups.
- There may be more than one patient group for some of the diseases.
- There may be more than one registry for certain diseases.
- Not all patient groups are equipped to manage registries.
- There are too many rare diseases, each with their unique set of challenges.



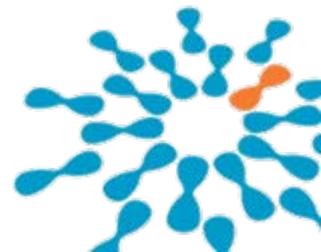
Cont.....

- Those that are equipped to manage registries have challenges too...
 - Funding for managing registries.
 - Type and nature of data collected.
 - Ethical utilization and distribution of data.
 - Access and location of registries.



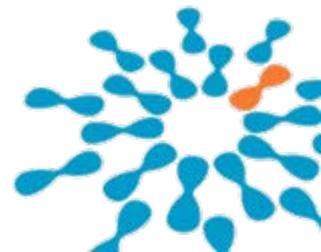
Solutions

- Have simple registries.
- Consider data pooling options with larger sophisticated groups.
- Collectively seek to secure funds instead of dividing the funding pool.
- If more than one group involved, avoid overlaps and duplications.
- Patient groups can play an important role in de-risking data collection from patients.



NORD's efforts

- Overhaul of current database of over 300,000 contacts for future registries & data mining applications.
- Efforts at updating our database of over 1,200 patient organizations.
- On-line community in partnership with EURORDIS.



Take away message for patient groups.....

- There is HUGE strength in numbers.
- Unity among groups is critical.
- Centralized information gathering facilitates and enhances research and drug development.
- Public-private partnerships have a key role.
- Important to share and celebrate successes and milestones.

