Partners for a Healthier Future

CPAG: Perspective from an RDCRN Consortium
PAG as Research Partner
WHAT IS CPAG?

- The Coalition of Patient Advocacy Groups or ‘CPAG’ is the collective representation of patient groups affiliated with the consortia of the Rare Diseases Clinical Research Network (RDCRN), a program of ORDR/NCATS.

- CPAG is composed of representatives from each patient group in the network who are tasked with being FULL PARTNERS in the research undertaken by their RDCRN consortium.
WHAT IS CPAG?

- CPAG members influence the direction of the Rare Diseases Clinical Research Network as a whole. They participate in network-level discussions and meetings. The CPAG coalition, through its leadership, is a voting member of the RDCRN Steering Committee.
WHAT IS CPAG?

- 100+ voluntary health/patient advocacy organizations representing a variety of rare disorders

- Extremely diverse in resources, experience, name recognition and infrastructure (see “snapshot” slide)

- All committed to rare disorders research through the RDCRN
WHAT CPAG IS NOT

- CPAG is not an independent patient representative group
- CPAG as an entity exists only in the context of the RDCRN
- CPAG’s focus and activities are limited to (and only to) issues related to the RDCRN
The member groups that comprise the entity collectively known as “CPAG” are incredibly diverse and range from “Kitchen Table” organizations with no staff and little revenue to multi-million dollar non-profit corporations with professional staff and access to consistent funding sources.

Many of the PAGs associated with the RDCRN are operating with extremely limited financial and human resources.

How can we meet the needs of such a diverse coalition of member groups and fulfill our commitment to be partners with RDCRN investigators?
CHALLENGES FOR CPAG
HAT CHALLENGE

- The member groups that comprise the entity collectively known as “CPAG” are dedicated professionals with a laser focus on their individual diseases.

As CPAG representatives, we must wear several different hats and be sure we are considering the needs of the RDCRN as a whole, in addition to our individual consortia and groups.
ADDRESSING THE CHALLENGES

- CPAG leadership
  Chair: Chuck Mohan (UMDF/NAMDC)
  Co-Chairs: Janet Hieshetter (DRF/DC)
             Joyce Kullman (VF/VCRC)
             Cynthia LeMons (NUCDF/UCDC)

- Work closely and receive guidance from ORDR staff
OPPORTUNITIES FOR CPAG

1. Face to Face Meetings
2. Monthly Calls
3. Working Groups of CPAG
4. Committees of the Steering Committee
CPAG MISSION AND VISION

- **CPAG Vision**
  Through collaboration, patient advocacy groups and researchers can make faster progress toward new treatment options and cures, which can improve the lives of all persons and families affected by a rare disease.

- **CPAG Mission**
  The Coalition for Patient Advocacy Groups will promote collaboration between rare disease advocacy organizations and the Rare Diseases Clinical Research Network in order to facilitate better access to, and earlier benefit from, research conducted on rare diseases. As the patient advocacy arm of the Rare Diseases Clinical Research Network, CPAG members will use their position to advance the cause of rare disease research and improved patient outcomes through the network.
PCD Timeline

Indicates start of RDCRN/GDMCC

1904
1st Case Report
1/1/1904
Kartagener's Triad Described
1/1/1933
1918
1932
1946
1960
1974
1988
2002
2015

EM Defect Described/ICS
1/1/1976
Microscopy/PCD
1/1/1983

Low nNO Described
1/1/1994

First Gene Identified
1/1/1999

First PCD Family Day

First Cilia & Mucus GRC
1st
PCD/Ciliopathy Mtg

Second PCD Gene ID'd
PCD & Cri du Chat

1st Genetic for PCD--4 genes

1st PCD/Ciliopathy Mtg

PCD Clinical Centers Established

PCD CC Mtg

PCD & CHD Publication

1st Genetic for PCD--4 genes

PCD Foundation Incorporated

1/1/1999

1/1/2002

RDCRN Formed

32 Genes

Indicates start of RDCRN/GDMCC

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