# PROTOCOL ADVOCACY PANOBINOSTAT + INTERFERON GO/NO GO???

CROI COMMUNITY CURE WORKSHOP

FEBRUARY 21, 2016

# PROTOCOL ADVOCACY

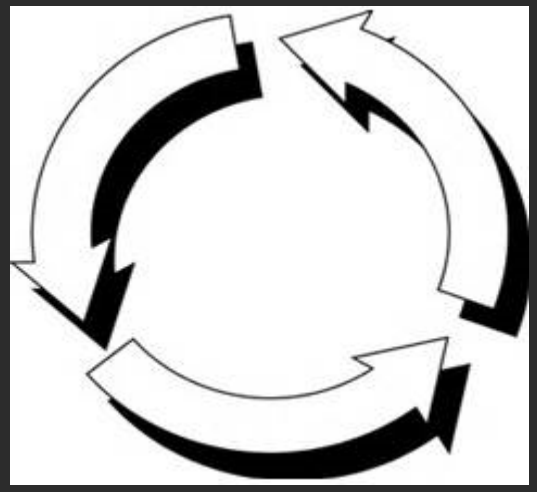
COMPETING STAKEHOLDER ROLES IN RISKY CURE RESEARCH

IN HEALTHY PEOPLE WITH MANY SAFE & EFFECTIVE OPTIONS:

FOOD AND DRUG ADMINISTRATION

CLINICAL RESEARCHERS & SPONSORS

COMMUNITY ADVOCATES



# PROTOCOL ADVOCACY

**FDA**

**IND APPROVAL?**

**SAFETY AND EFFICACY**


**RESEARCHERS**

**IND APPROVAL**

**NO DELAYS**

**NOVARTIS**

**PANOBINOSTAT**

**FULL FDA APPROVAL**

# PROTOCOL ADVOCACY

ROLE OF THE COMMUNITY

ETHICAL RESEARCH

CONDUCTED EXPEDITIOUSLY

# PROTOCOL ADVOCACY

- FDA INVESTIGATIONAL NEW DRUG PROCESS
  - PROTOCOL SUBMISSION WITH RATIONALE & SAFETY DATA
  - ELECTRONIC MAIL COMMUNICATIONS
  - MORE SAFETY DATA AND PROTOCOL AMENDMENTS
  - FDA HAS 30 DAYS TO RESPOND WITH A GO OR NO GO
  - CLINICAL HOLD IF FDA HAS ADDITONAL QUESTIONS

# PROTOCOL ADVOCACY

- FDA: RISK vs. BENEFIT IN HEALTHY PATIENTS-NO GO-CLINICAL HOLD
- PANOBINOSTAT: CANCER DRUG THAT WAS ALMOST NOT APPROVED

- RESEARCHERS: SHORTER DURATIONS; MORE SAFETY INFORMATION
- LESS TOXICITY IN HEALTHIER PATIENTS/WILLINGNESS TO COMPROMISE

- COMMUNITY: ETHICAL AND EXPEDITIOUS RESEARCH
- INDEPENDENT RESEARCH & CONSULTATION

# PROTOCOL ADVOCACY

- HDAC INHIBITORS: CANCER DRUGS FOR VERY ILL PATIENTS
  - VORINOSTAT
  - PANOBINOSTAT
  - ROMIDEPSIN

# PROTOCOL APPROVAL

- PANOBINOSTAT: HIGHLY TOXIC/RISK OF HEART ATTACKS
- ONLY APPROVED AS SECOND LINE THERAPY IN CANCER PATIENTS

- PANOBINOSTAT & INTERFERON
- RISK OF HEMORRAGING

- ETHICAL IN HEALTHY PATIENTS?
- PROCEED EXPEDITIOUSLY?

# PROTOCOL ADVOCACY

- COMMUNITY ADVOCACY ACTIONS
  - CALLS WITH FDA, PI & COMMUNITY
  - FDA CALL WITH NOVARTIS, PI & COMMUNITY
  - SUPPORTED ON THE NEED FOR ADDITIONAL SAFETY DATA
  - SUPPORTED SHORTER DURATIONS AND LOWER DOSES
  - ETHICAL AND EXPEDITIOUS UNDER THE CIRCUMSTANCES

# PROTOCOL ADVOCACY

- LESSONS LEARNED
  - INDEPENDENT RESEARCH
    - CONSULTATION WITH ALL STAKEHOLDERS
  - COMMUNITY FOLLOW UP CALL WITH THE FDA
    - SUBMIT AS MUCH SAFETY DATA AS POSSIBLE
    - PRE-IND CONSULATIONS TO PREVENT CLINICAL HOLDS

# PROTOCOL ADVOCACY

- THANKS
  - RICHARD JEFFERYS
  - FDA
  - NOVARTIS
  - DAN KURITZKES