PROTOCOL ADVOCACY
PANOBINOSTAT + INTERFERON
GO/NO GO???
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 ADDITIONAL 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 EXPEDIENTIOUS 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 CONSULTATIONS TO PREVENT CLINICAL HOLDS
THANKS

RICHARD JEFFERYS

FDA

NOVARTIS

DAN KURITZKES