Roles for Public Foundations in Clinical Trial Enrollment and Retention

Maria Freire, Ph.D.
President and Executive Director

NIH Workshop on the Enrollment and Retention of Participants in NIH-funded Clinical Trials
July 25, 2014
Why we are here...

I just heard there’s a drug in trials that might stop my cancer!!

Great! Are you going to volunteer to participate for the trial?

Of course not...why would I do that?

I wouldn’t either. Sure hope they get some results soon...
Purpose
→ To support the NIH in its mission;
→ To advance collaboration with biomedical researchers from universities, industry and not-for-profit organizations.

Structure
• 501(c)(3) not-for-profit organization;
• Independent Board of Directors;
• NIH Director and FDA Commissioner ex-officio Board Members

Highlights
• Raised >$750 million since 1996;
• Supported >400 projects, ~100 currently active;
  • research partnerships
  • scientific education/training
  • conferences/events
  • capital programs
• 94 cents of every dollar spent directly supports programs;
• For 10 years Charity Navigator has rated FNIH as an organization that exceeds industry standards.
Our role...

*What we do, how we do it...*

**Identify:**
- Important scientific problem
- Key players
- Resources required and sources of support
- Neutral convener; trusted party to provide safe harbor for discussions

**Establish:**
- Highest level of ethical standards
- Clear goals and milestones
- Effective mechanism to generate scientific consensus
- Nimble infrastructure and project expert project management

**Facilitate:**
- Discussions with key opinion leaders and regulatory decision makers
- Integrated approach to cross-sector partnerships
- Communications; ensure all partners’ voices are heard;

**Enable:**
- Sharing of data and expertise to collaboratively address medical needs
- Resource mobilization
- Manage grants, contracts, and projects; oversee and conduct research
2010 Symposium on “Overcoming Barriers to Early Phase Clinical Trials”

FNIH role: Enabler

- Formed a public-private partnership to support research on enrollment barriers and the 2010 meeting.
- Grants to 6 cancer centers.
- Participants: NCI, ASCO, 5 industry partners.
- Goal: Investigate barriers that prevent patients, especially minority and elderly populations, from participating in early-phase clinical trials of innovative cancer therapies.
- Results:
  - Publication in 2013 with suggested research in:
    - Patient- and Community-Centered;
    - Physician/Provider-Centered;
    - Site-Centered.

Journal of Oncology Practice, 2013
The challenge:

- Targeted drugs are predicted to work in only 5-20% of patients;
- To meet accrual goals, large patient populations must be screened.

Lung-MAP (SWOG S1400):

- 5-year multi-drug, multi-arm, biomarker-driven squamous cell lung cancer clinical trial;
- Phase 2 – 3 registration trial, under a master protocol;
- Uses next-gen sequencing platform for genomic profiling to match patients to investigational treatments
- 5 drug companies currently participating
Patients with squamous cell lung cancer

2nd Line Therapy

Lung-MAP Trial Arms for Treatment

Tumor sample analyzed

- Tumor has none of the changes listed here
- Tumor DNA has PIK3CA gene mutation
- Tumor DNA has CCND1, D2, CDK4 gene amplification
- Tumor DNA has FGFR gene amplification, mutation or fusion
- Tumor contains high levels of c-Met protein

Chemo, MEDI, GDC, Palbociclib, Chemo, AZD, Eilotumamab + Erlotinib
Thank You
Rationale for Lung-MAP

- Multi-arm Master Protocol
  - Homogeneous patient populations & consistent eligibility from arm to arm
  - Each arm independent of the others
  - Infrastructure facilitates opening new arms faster
  - Phase II-III design allows rapid drug/biomarker testing for detection of “large effects”

- Screening large numbers of patients for multiple targets by a broad-based NGS platform reduces the screen failure rate
- Provides a sufficient “hit rate” to engage patients & physicians
- Bring safe & effective drugs to patients faster
- Designed to facilitate FDA approval of new drugs