Patients Driving Progress

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Nothing to disclose
The Evolution of Patient Advocacy

The Agnew Clinic, Thomas Eakins (1899)
Partial mastectomy
The Evolution of Patient Advocacy

Patient's Voice Included…
The Evolution of Patient Advocacy

HIV/AIDS Crisis (‘80s-’90s)  “We walked, we talked, we made a difference”

“Nothing about us, without us”
Opportunities for Patient Involvement in the Drug Development Process

Numerous opportunities available for patient involvement THROUGHOUT the drug development process

- Input on relevance of research to patient community
- Identifying unmet needs
- Providing data on therapeutic burden
- Characterize eligibility criteria for clinical trials
- Input on relevant clinical endpoints
- Input on PRO’s
- Input on informed consent form/process
- Working with regulatory agencies on benefit-risk & draft guidance
- Supporting sponsors at pre-IND FDA meeting
- Support for patient selection & recruitment
- Educating/motivating patient community
- Serving on Data & Safety Monitoring board
- Input for trial adaptations & modifications
- Participating in benefit-risk discussions
- Participating in patient preference studies
- Providing public testimony at FDA Advisory Committee
- Participating/attending FDA hearings
- Participate in post-marketing surveillance initiatives
- Support in returning study results to participants
- Presentation of results to patient community
- Feedback on patient community perception of results
- Working with payers in reimbursement
Patient Perspective is Powerful

- Is this clinical trial asking questions that are relevant to my disease?
- Why a clinical trial when I can get off label use?
- If a better drug is created while I’m on trial, will I be able to get it?
- Is this treatment likely to work for me?
- Will my genetic privacy be protected in this trial?
- What will my quality of life be if I enroll in this trial?
- Is the standard of care acceptable in the clinical trial?
- How personalized will my choices be?
- Do I have other choices if I don’t want to be randomized?
“Science may provide the most useful way to organize empirical, reproducible data, but its power to do so is predicated on its inability to grasp the most central aspects of human life: hope, fear, love, hate, beauty, envy, honor, weakness, striving, suffering, virtue.”

—Paul Kalanithi, When Breath Becomes Air
Washington, DC-based Think Tank & Advocacy Organization

Driving collaboration among partners from every healthcare sector to power advances in science, policy, and regulation that speed life-saving treatments to patients.

Develops groundbreaking partnerships:
• Federal Agencies (FDA, NIH, NCI)
• Academic Research Centers
• Professional Societies
• Industry
• Advocacy Organizations
Finding solutions
Helping patients

• Concerns with Timing of Drug Approvals
  Breakthrough therapy designation

• Concerns with Clinical Trial Designs
  Lung-MAP Master Protocol

• Concerns with Efficient Regulation of Cancer Drugs
  Oncology Center of Excellence (OCE)
“It's personal, it's personal for so many… Think of how many people you know who are saying, … doc I just want to make it one more month to see my daughter get married.


— Vice President Joe Biden
Breakthrough Therapies

Why should patients have to wait YEARS for drugs that show significant clinical activity early in development?

Opportunities for condensing the drug development process
The FDA worked with the advocacy community bringing Breakthrough Therapy Designation to life

Multi-stakeholder workshop (2011)
Bipartisan legislative action
Signed into law in 2012
Breakthrough Therapies

Faster drug approvals = Faster access to life-saving drugs

Approved 3 months earlier

Developed 2.2 years faster

Breakthrough Therapies

Faster drug approvals = Faster access to life-saving drugs

- Cancer: 113
- Cardiovascular: 4
- Infectious Disease: 25
- Other: 52
- Rare Inherited Disorders: 48

Total of 242 drugs

Cancer & several other conditions
“Clinical trials should be serving patients, not patients serving clinical trials”

– Jeff Allen, CEO of Friends of Cancer Research
LUNG-MAP Master Protocol

ISSUE BR
Conference on Clinical Cancer Research
November 2012

Introduction
Despite several impressive trials of death in the United States, no potential therapy from the initially complicated, expensive, and often many challenges of drug development. Extensive bureaucratic processes required to navigate through the lengthy regulatory review. Modernizing this process with innovative approaches and new clinical trial designs is of high importance.

Jeffrey

740 Activated Trial Sites
1634 Patients Enrolled
47 States Across the Country + Canada
Major Progress for Patients

**DYNAMIC STUDY DESIGN**
- Multi-arm phase II/III master registration protocol
- Each arm able to open & close independent of other arms

**NON-SMALL CELL LUNG CANCER**
- Advanced squamous cell carcinoma (SCCA)
- All histologies

**MULTIPLE DRUGS BASED ON GENOMIC PROFILE**
- Targeted agents
- Immunotherapies

**LEADING TO ACCELERATED APPROVAL AND/OR RANDOMIZED PHASE III TRIAL**

Abbvie – AMGEN – AstraZeneca/MedImmune – Bristol-Meyers Squibb – Foundation Medicine – Genentech - Pfizer
LUNG-MAP Master Protocol

Screening Protocols

LUNGMAP*

Biomarker-Driven Sub-Studies

Non-match Sub-Studies

Actively Accruing
In Development
Completed
Closed

Completed 12/12/16
S1400
PI3K inhibitor

Completed 09/01/16
S1400 C
CCGA

Completed 10/31/16
S1400 D
FGFR

Closed 11/25/14
S1400 E
MET

Anticipated 2018
S1400G
HRRD

S1400K
LOH

S1900A
Non-match

S1400I
Checkpoint Naive

S1400F
Checkpoint Refractory

S1800A
Checkpoint Refractory

Completed 12/18/15
PD-L1 inhibitor + CTLA-4 inhibitor

Completed 04/23/18
PD-L1 inhibitor

Anticipated 2018
PD-L1 inhibitor + VEGF inhibitor vs. SOC

*New screening protocol will include all NSCLC histologies. The new umbrella screening protocol will simply be referred to as LUNGMAP.

Only new sub-studies will be open to all NSCLC histologies. The rest of the current sub-studies are for patients only with squamous cell carcinoma.
Friends focused on finding opportunities to consolidate regulatory approaches to the clinical evaluation of cancer treatments.

The OCE leverages the combined skills of regulatory scientists and reviewers with expertise in drugs, biologics and devices to expedite the development of novel cancer products at the FDA.
Business as usual is not acceptable. If we are not challenging the status quo, are we really serving patients?

We need to pursue audacious ideas that are meaningful to patients even if they are not as clean or tidy as we expect.