How to Engage with Federal Agencies

Food and Drug Administration (FDA):

Ryan Hohman
Vice President of Public Affairs
Friends of Cancer Research
How to Engage with Federal Agencies – FDA

A New Paradigm for Patient Impact on Drug Development: Research, Regulation, & Policy

Ryan Hohman, JD
Vice President, Friends of Cancer Research
Friends of Cancer Research drives collaboration among partners from every healthcare sector to power advances in science, policy and regulation that speed life-saving treatments to patients.

A unique model to create a path to better drug development and approval through scientific, regulatory, and legislative solutions.
Overcoming Delays in Drug Approvals: Breakthrough Therapy

**Problem:** Progress in personalized medicine is producing drugs with unprecedented impact visible early in their development, but better regulatory tools were needed to keep pace.

Drugs showing obvious, outsized potential to help patients still were required to go through the standard review procedure.

**Solution:** 2011 *Friends*-Brookings Conference: Advocacy-initiated collaboration of experts from FDA, NIH, NCI, academia, and industry created Breakthrough Therapy pathway to expedite the drug development process for products that show remarkable clinical activity early.

✓ Patients get revolutionary drugs faster, industry gets their therapies to market sooner, FDA is more efficient— But didn’t happen until advocacy got it started.

1 Year: Panel ➔ Whitepaper ➔ Bipartisan Legislation ➔ New Pathway at FDA
1 Year: 100+ Applications ➔ 38 Designations (13 in cancer) ➔ 3 Full Approvals
Breakthrough in Action

As of November 30, 2016 the FDA has approved 51 breakthrough therapy designated products

• There have been 464 total requests for the designation with 158 designations granted.

www.focr.org/breakthrough-therapies

*FDA does not disclose information regarding specific drugs or sponsors.
Overcoming Hurdles in Clinical Trials:

**Problem:** Clinical trials can be inefficient, expensive, time-consuming, and infrastructure-intensive, difficult to enroll patients and often times require expensive genetic testing.

**Solution:** Multi-drug, multi-arm, biomarker-driven clinical trial protocol.

- **A more efficient and effective model:** Trial matches companies with the patients whose tumors are most genetically relevant to the therapies they are developing.

- **Groundbreaking Public-Private Partnership:** Five major pharmaceutical companies, Foundation Medicine, NCI, SWOG, FDA, FNIH, and multiple advocacy organizations

- **Better trials for patients, more efficient for industry, increased government collaboration.**
GENOMIC PROFILE SCREENING
Patients are screened using a comprehensive genomic profiling platform (FoundationOne) that looks at over 200 cancer-related genes for genomic alterations. Instead of having to undergo multiple diagnostic tests to determine eligibility for many different studies, enrollees are tested just once.

SUB-STUDY ASSIGNMENT
Based on the results of this screening, patients are assigned to whichever one of up to five sub-studies testing different investigational treatments best suits their genomic profile.

INNOVATIVE APPROACH
This innovative approach improves a patient’s likelihood of receiving a drug that will work for them while allowing for new therapies in development to be added as the trial progresses.
Tumors contain high levels of c-Met protein.

Tumor DNA has FGFR gene amplification, mutation or fusion.

Tumor DNA has PIK3CA gene mutation.

Tumor has CCND1, D2, CDK4 gene mutation.

Tumor has none of the changes listed here.

- Arm 1: 50% Chemo-therapy
- Arm 2: 50% AZD4547
- Arm 3: 50% GDC-0032
- Arm 4: 50% MEDI4736
- Arm 5: 50% Chemo-therapy
- Arm 6: 50% Palbociclib
- Arm 7: 50% AZD4547
- Arm 8: 50% Erlotinib
- Arm 9: 50% Bilotum amab+Erlotinib

Lung-MAP Trial Arms for Treatment: Patients with squamous cell lung cancer.
“Lung-MAP will, I think, set a standard for how we want to conduct this sort of precision medicine for cancer going forward.” – NIH Director Dr. Francis Collins

**Timeline of Success**
- **Nov. 2012** Industry, FDA, NCI, academic research, & advocacy develop concept
- **Nov. 2013** Final trial design and first experimental drugs announced
- **June 2014** Lung-MAP launched at cancer centers nationwide
- **January 2015** Over 700 sites across the United States now participating
- **January 2016** New arms and therapies are added to trial
- **January 2017** Over 1200 patients enrolled
Creating a 21st Century FDA: Vice President Biden and the Centers of Excellence at the FDA

*Friends concept to establish an Oncology Center of Excellence at the US Food and Drug Administration (FDA) working with Vice President Biden, Congress, and the FDA*

- Leverage the skills of regulatory scientists and reviews across product categories and introduce efficiencies and expedite the development of novel combination products and support an integrated approach in:
  - Streamline the development of companion diagnostic tests, and the use of combinations of drugs, biologics and devices to treat cancer that are currently regulated in different centers
Oncology Center of Excellence
Product-Oriented Regulation

The current FDA process for a combination oncology product

Devices
Biologics
Drugs

Evaluation
Evaluation
Evaluation

Approval
Approval

The new FDA Oncology Center of Excellence will facilitate optimal regulation of complex medical treatments

Center of Excellence
Evaluation

Approval

FDA
Bringing together those who seek innovative policies and solutions to advance science through engaging, educating, and empowering patients.
Patient advocates need knowledge and understanding of the drug development process

- *Friends* has created an online learning community to help patient advocates
- Through this training program, advocates will acquire the necessary tools to effectively communicate with:
  - Drug researchers
  - Drug developers
  - Regulators
- This will enable them to make the connections necessary to engage with all sectors who need to be better guided by patient input.