

Compassionate Use: Perspectives from a Patient Advocacy Group

American Society of Clinical Oncology Annual Meeting 2016

Samantha Roberts, PhD

Director of Scientific Affairs, Friends of Cancer Research



Need for Patient Access to New Treatments

- Improved science has led to therapeutic advances for many conditions
 - FDA approval – broad availability, established safety and efficacy profile
 - Greater efficiency in drug development has accelerated timeliness of getting to market
- Publicity around therapeutic breakthroughs + continued unmet medical need = increased demand for patient access to investigational treatments
- Clinical Trials
 - Primary route to receive investigational treatments
 - Contributes to collection of data needed for regulatory approval
- Expanded Access & Compassionate Use
 - Provides an opportunity to access an investigational drug for seriously ill patients for whom clinical trials are not an option
 - Primary purpose is treatment, not research

Challenges/Barriers

- For sponsors:
 - Difficult to determine risk/benefit early in development
 - Limited clinical supplies, often just enough to conduct a study
 - Lack of resources to provide equitable access Potential for unintended consequences
- For patients and providers
 - Limited information about the drug
 - Lack of transparency in the process – perception that FDA denies requests
 - Administrative burden
- Institutional Review Board (IRB)
 - Lack of familiarity with EAP and differences in review without established timelines

Belief that FDA standing in the way

The Washington Post



Health & Science

'Right to Try' laws spur debate over dying patients' access to experimental drugs

By Brady Dennis and Ariana Eunjung Cha May 16, 2014

Colorado, Missouri and Louisiana are poised to become the first states in the nation to give terminally ill patients the right to try experimental drugs without the blessing of the Food and Drug Administration, setting the stage for what could be a lengthy battle over who should decide whether a drug is too risky to try.

Lawmakers in the three states have passed "Right to Try" laws with unanimous votes in recent weeks, after high-profile, social media campaigns in which families of dying patients have pushed for access to unapproved but potentially lifesaving drugs. Colorado's governor is expected to sign that state's law Saturday.

Proponents of the measures argue that patients desperate for treatments must navigate a lengthy, cumbersome process to get the FDA to approve early access to experimental drugs and to persuade companies to provide them. The Right to Try laws are intended to cut through some of that red tape by

STAT | Reporting from the frontiers of health and medicine

He's 20. Has brain cancer. And is caught in the crossfire between the FDA and a renegade doctor



EROS DERVISHI FOR STAT

By BOB TEDESCHI @bobtedeschi and SHEILA KAPLAN @bySheilaKaplan
JUNE 1, 2016

PRESENTED AT: ASCO ANNUAL MEETING '16

Slides are the property of the author. Permission required for reuse.

Presented by: Samantha Roberts, PhD

FRIENDS
of CANCER
RESEARCH

Right to Try – Not a solution

- Compromises drug development programs
 - Both regulators and sponsors have expressed concerns with the potential risks the laws could impose on enrolling eligible patients into clinical trials
 - Manufacture of additional product could compromise availability of trial material
- Potentially puts patients at risk
 - Circumvents FDA oversight – no assurance that potential benefits of drug justify the potential risks, or that patients haven't exhausted other available options
 - Patients responsible for treatment cost
- Doesn't solve the problem of obtaining access
 - No requirement that companies actually provide access
 - Although at least 24 states now have “Right to Try” laws, no patient has received an investigational drug through this process to date

How can we improve expanded access?

- Can FDA administrative process be simplified?
 - Recently released streamlined form (FDA form 3926) for physicians to request individual patient expanded access
- Can we improve accessibility of clinical trials to reduce need for patients to seek expanded access?
- Can we help patients navigate the process?

How can we improve expanded access?

- Can FDA administrative process be simplified?
 - Recently released streamlined form (FDA form 3926) for physicians to request individual patient expanded access
- Can we improve accessibility of clinical trials to reduce need for patients to seek expanded access?
 - Can eligibility criteria be broadened?
- Can we help patients navigate the process?

Modernizing Eligibility Criteria

- Joint Friends-ASCO-FDA Workshop May 12 at ASCO headquarters
- Identified scientifically and medically appropriate opportunities to expand eligibility criteria in cancer clinical trials in order to:
 - Increase patient access
 - Improve trial accrual
 - Improve applicability of trial results to real-world patient populations
- Four working groups focused on specific traditional exclusion criteria:
 - Organ dysfunction
 - HIV/AIDS
 - Brain metastases
 - Pediatric populations
- Final recommendations to be presented at Friends Annual Meeting Nov. 16th

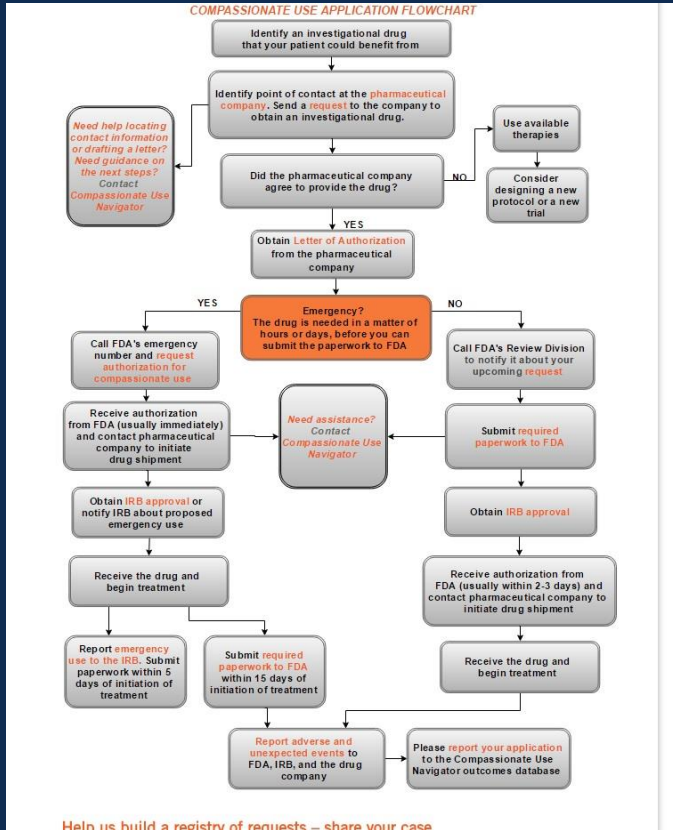
How can we improve expanded access?

- Can FDA administrative process be simplified?
 - Recently released streamlined form (FDA form 3926) for physicians to request individual patient expanded access
- Can we improve accessibility of clinical trials to reduce need for patients to seek expanded access?
- Can we help patients navigate the process?

Compassionate Use Navigators

- Kids V Cancer: <http://www.kidsvcancer.org/compassionate-use/>
 - Pediatric oncology advocacy organization
- Reagan-Udall Foundation: <http://www.reaganudall.org/news-and-events/events-2/public-workshop-expanded-access-navigator/>
 - Public-private organization created by Congress to advance regulatory science needed by the Food and Drug Administration (FDA) to accomplish its mission.

Kids V Cancer Navigator



- Instructions on how to apply for compassionate use
- Assistance in identifying expanded access policies and points of contact at drug companies
- Information on FDA procedures for obtaining compassionate use medications
- Information on the IRB approval process
- Registry of outcomes of compassionate use applications for children
- Company directory

Reagan-Udall EA Navigator

- Information on trials, types of expanded access, FDA and manufacturer roles
- Live assistance and directory:
 - Clinical trial availability for investigational drug
 - Expanded access policies of drug manufacturer
 - Status of drug availability
 - Point of contact at company
- Other proposed functions:
 - Track number of expanded access requests
 - Track turnaround times
 - Track outcomes

Final thoughts

- FDA process is reasonable and necessary to protect patients.
- Companies need to establish clear policies around expanded access and should be ready to field requests when announcing exciting preliminary data.
- Should identify ways to include more patients in trials – expanded access is vital but a lost opportunity for rigorous data collection.