The Public Policy Landscape – Emerging State & Federal Laws

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EveryLife Foundation Rare Disease Workshop

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<th>2007-08</th>
<th>2009-10</th>
<th>2011-12</th>
<th>2013-14</th>
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<td>110th Congress</td>
<td>111th Congress</td>
<td>112th Congress</td>
<td>113th Congress</td>
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<td>• Bi-partisan, Democratic-led effort (Rep. Watson, D-CA)</td>
<td>• Same as previous bill, but no Senate counterpart</td>
<td>• Rep. Ron Paul (R-TX)</td>
<td>• H.R. 5805 – Rep. Michael McCaul (R-TX) introduces Andrea Sloan CURE Act, requiring manufacturers to post contacts for requests and provide denials in writing, and requiring GAO and Task Force Reports to Congress</td>
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<td>• Required FDA to create new expanded access regime</td>
<td>• Permitted expanded access upon informed consent of patient</td>
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<td>• Permitted manufacturers, following Phase I, to apply for expanded access authority and to charge for drugs</td>
<td>• Prohibited FDA from regulating access, or even collecting information</td>
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<td>• Provided manufacturer liability protection</td>
<td>• No Phase I requirement</td>
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114th Congress: 2015-2016 Pending Legislation

- H.R. 909 – Andrea Sloan Compassionate Use Reform and Enhancement (CURE) Act
- H.R. 6 ("21st Century Cures") Section 2082 – modified version of H.R. 909
- S.2912 – Trickett Wendler Right To Try Act of 2016
Current CURES Proposal

Andrea Sloan Compassionate Use Reform and Enhancement (CURE) Act (H.R. 909)

- Introduced in 113th and 114th Congresses, by Rep. Michael McCaul (R-TX)
- Requires manufacturers of “breakthrough drugs” to make expanded access policies publicly available, and provide written explanations for denials
- Commissions a GAO analysis of expanded access requests
- Establishes an Expanded Access Task Force at HHS to explore ways to improve patient access to investigational drugs
- Requires FDA to define how adverse drug event data reported through expanded access is used

Sections 2082-83 of H.R. 6 “21st Century Cures” Act

- Passed by House on July 10, 2015 (still needs to be passed by Senate to become law)
- Would require manufacturers to make publicly available a policy to evaluate and respond to expanded access requests
- Policy requires point of contact, procedures for making requests, general criteria for approval, and anticipated timeline for acknowledgement of receipt of requests
- Would require FDA to finalize Expanded Access Q&A Draft Guidance (May 2013)
- Final guidance shall define how FDA interprets and uses adverse event data reported from expanded access
S.2912 – Right to Try Act of 2016

Proposes to federalize state RTT laws by prohibiting FDA from blocking access if:

- Intended to treat a patient who has been diagnosed with a terminal illness
- Is authorized by, and in accordance with, State law
- Drug is past Phase I clinical trials

39 Senate Republican co-sponsors to date (37 Republicans, including Majority Leader, and several Committee Chairs), and companion House bill (H.R. 3012) with 55 co-sponsors
Status of State “Right to Try” Laws


Green-adopted, blue-not adopted, grey-pending, red-vetoed

Source: http://righttotry.org/in-your-state/
What the “Right to Try” Laws Actually Do

What They DO:
- Generally, they aim to grant patient access to investigational treatments if:
  - Patient is terminally ill
  - Physician recommends treatment
  - Patient provides informed consent, and
  - Treatment has completed Phase I clinical safety/dose limitation trial
- Most grant physicians and manufacturers liability protection against claims arising from adverse events caused by investigational treatments

What They DON’T DO:
- They don’t create a right for terminally ill patients to access life-saving treatments exempted from FDA rules
- State laws do not permit manufacturers to provide patients with access to unapproved drugs, when the federal law mandates “no person shall introduce or deliver for introduction into interstate commerce any new drug”
- They don’t address questions of patient welfare and public health
- They don’t provide free access to experimental treatments
- They don’t compel anyone or any company to fulfill a patient request
Bringing Back the Policy “Middle”

- The CURES provisions are important
  - Transparency
  - Clarity

- The RTT Provisions are Misguided
  - Access at what cost
    - STAT (8/27/16): 37 Congressional letters re: Dr. Stanislaw Burzynski alone
    - Beginning of the end of the FDA

- But Other Issues Need to be Addressed
  - New FDA Pathway
  - Reimbursement
  - Liability Protection
DISCUSSION & QUESTIONS