Expanded Access and Right to Try: The Impact of Recent Legislative Changes

Kate Gallin Heffernan, Partner, Verrill Dana
Marylana Saadeh Helou, Associate, Verrill Dana
Robert Romanchuk, IRB Chair, Advarra

May 30, 2018
About Advarra

- North America’s premier provider of IRB, IBC and global research compliance services
- Leverage strengths in technology, regulatory expertise and customer service to serve increasingly complex research needs
About Advarra

Combined 50+ years of experience

Access to over 2,000 unique institutional research sites

Global consulting services

The industry’s most comprehensive and efficient technology
Kate Gallin Heffernan, Esq.
Partner, Verrill Dana, LLP

- Chair of the Academic and Clinical Research Group
- Advises research stakeholders on a comprehensive range of issues affecting clinical research, including the protection of human subjects, research affiliations and contracting, research uses of stored materials, FDA requirements for clinical investigations, multi-site global trials, the privacy of research subjects’ information, and research related to organ donation and transplantation
- A frequent speaker and author on many issues related to the legal and ethical issues raised by biomedical research
- Graduate of New York University School of Law
About Today’s Presenters

Marylana Saadeh Helou, Esq.
Associate, Verrill Dana, LLP

- Member of Verrill Dana’s Health Care Group
- Advises a wide range of clients in the health care industry, including health systems, hospitals, academic medical centers, and pharmaceutical and medical device companies on regulatory considerations related to clinical research and health data privacy
- Graduate of Boston University School of Law
About Today’s Presenters

Robert Romanchuk
IRB Chair, Advarra

- Extensive experience in IRB and research operations, HSP and GCP auditing and training
- Frequent presenter at ACRP, MAGI and other venues
- BSHS, Clinical Research Administration, The George Washington University
Objectives

- Identify the recent streamlining changes undertaken by the FDA to increase the efficiency of the agency’s response to requests for expanded access.
- Describe key ways in which “right to try” legislation (both state and federal) diverges from the existing FDA expanded access pathway.
- Explain practical considerations for stakeholders involved in requests for expanded access given the shifting legislative frameworks and potentially competing requirements at the state and federal levels.
Disclaimers

- The views and opinions expressed in this presentation are the presenters’ alone and do not necessarily reflect the views and opinions of Advarra, Verrill Dana, or any of their respective clients.
- This presentation does not constitute legal advice.
Agenda

- History of FDA’s role regulating access to investigational drugs
- Current state of expanded access through FDA
  - Three categories of expanded access to investigational drugs (“Expanded Access Drug Program”)
  - Limitations to and criticism of the Expanded Access Drug Program
  - Recent streamlining efforts
  - Expanded access to investigational devices
- “Right To Try” movement – commencement and evolution
- Analysis of Congress’ recently approved federal “Right To Try” bill, S.204
- How will the various “Right To Try” legislative changes be implemented by manufacturers, treating providers, institutions, and IRBs?
History of the FDA’s Role Regulating Access to Investigational Drugs

1938 – Food, Drug, and Cosmetics Act ("FDCA")
- Required FDA approval before introducing new drugs in interstate commerce.
- Section 505(i) allowed FDA to exempt by regulation investigational drugs from the approval requirements.

1987 – FDA revised its Investigational New Drugs regulations
- Largely in response to the AIDS epidemic.
- Provided a pathway for access to investigational drugs outside of the clinical trial context for (i) certain broad patient populations, and (ii) individual patients in emergency situations.

1997 – FDCA amended by the Food and Drug Modernization Act ("FDAMA")
- To permit individual patients, acting through a physician, to obtain access to investigational drugs.

2009 – Expanded Access Drug Program regulations amended
- To ensure “broad and equitable access to investigational drugs for treatment.”

2017 – Expanded Access Drug Program regulations clarified
- In response to calls for streamlining and expediting the application process.
What Is the Expanded Access Drug Program?

A process regulated by FDA that allows the provision of an investigational drug to certain eligible patients where the primary purpose of the provision is to diagnose, monitor, or treat patients’ disease or condition.

- Primary goal is not to obtain information about safety and effectiveness of the drug.

May also refer to treatment use of other drugs.

- E.g., (i) drugs that have been withdrawn for safety, (ii) a similar, but unapproved drug during a drug shortage, or (iii) an approved drug where availability is limited by a risk evaluation and mitigation strategy (“REMS”).

Terminology: expanded access, treatment use, compassionate use
FDA’s Expanded Access Drug Program includes three categories:

- Individual patients, including emergency use [21 CFR 312.310](#)
- Intermediate-size patient populations [21 CFR 312.315](#)
- Treatment IND or treatment protocol: widespread treatment use/large patient populations [21 CFR 312.320](#)

Regulations describe **general** criteria, submission requirements, and safeguards that apply to all categories and **additional** criteria, submission requirements, and safeguards that are unique to each category.
Requirements for All Expanded Access Uses
21 CFR § 312.305

Criteria

• FDA must determine that:
  1. Patient(s) has/have a serious or immediately life-threatening disease or condition; no comparable or satisfactory alternative therapy;
  2. Potential patient benefit justifies potential risk; potential risk not unreasonable in context of disease or condition; and
  3. Provision of drug will not interfere with initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use.*

*Especially pertinent to expanded access uses treating larger patient populations.
Requirements for All Expanded Access Uses
21 CFR § 312.305

> Submission
  • A submission may be made by:
    – Pharmaceutical company/manufacturer of the drug (sponsor)
    OR
    – Licensed physician under whose immediate direction the drug is being administered/dispensed (sponsor-investigator)
  • A submission may be:
    – An expanded access protocol – a protocol amendment to an existing IND
      – If drug is being developed under existing IND.
      – Consolidating may facilitate identification of safety concerns, ease administrative burdens, and help in product review.
    – An expanded access IND – a new IND submission separate and distinct from any existing IND
      – If no existing IND is already in effect, or there is an existing IND but that IND’s sponsor declined to also be the sponsor of the expanded access use.
Submission (cont’d)

- A licensed physician may **not** submit an *expanded access protocol* to an existing IND for which he/she is not the sponsor.

- If an existing IND for a drug is in effect and the pharmaceutical company/manufacturer declines to be the sponsor, the licensed physician submitting the expanded access IND (*i.e.*, the sponsor-investigator) may satisfy some submission requirements by requesting the pharmaceutical company’s/manufacturer’s permission via a *letter of authorization* (“LOA”) to reference the existing IND.
  – If permission is obtained, physician should provide FDA with a copy of the LOA.
Requirements for All Expanded Access Uses
21 CFR § 312.305

Information required to be submitted to FDA

- An FDA-acceptable submission form
  - Form 1571 “IND Application” or Form 3926 “Individual Patient Expanded Access IND Application”
- Rationale for intended use
- Criteria for patient selection (or for an individual patient, a description of his / her disease or condition)
- Method of administration of the drug, dose, and duration
- Description of facility where drug will be manufactured
- Chemistry, manufacturing, and controls information
- Pharmacology and toxicology information
- Description of the monitoring procedures and tests necessary to evaluate the effects of the drug and minimize risks

Covered in LOA
Requirements for All Expanded Access Uses
21 CFR § 312.305

Responsibilities

- **Investigators** (*i.e.*, the licensed physician administering/dispensing the expanded access drug) must:
  - Report adverse drug events to sponsor;
  - Ensure informed consent requirements of [21 CFR Part 50](#) are met;
  - Ensure IRB review is obtained consistent with [21 CFR Part 56](#) are met;
  - Maintain and retain accurate case histories and drug disposition records.

- **Sponsors** (*i.e.*, the individual or entity submitting the expanded access IND or protocol) must:
  - Submit safety reports and annual reports to FDA;
  - Ensure only licensed and qualified physicians are administering the drug;
  - Provide information to minimize risk and maximize potential benefits (*e.g.*, investigator’s brochure);
  - Maintain an effective IND for expanded access use;
  - Maintain and retain accurate case histories and drug disposition records.

- **Sponsor-Investigators** must comply with responsibilities of sponsors and investigators.
The Three Categories of FDA’s Expanded Access Drug Program

- **Individual Access** [21 CFR 312.310]
  - Covered by FDA Form 3926.
  - Repeated requests may prompt use of Intermediate Access.

- **Intermediate Size Population Access** [21 CFR 312.315]
  - More than one, but less than typical treatment IND or protocol.
  - Submitted as protocol under a new IND or as under an existing IND. The former requires a 30 day waiting period.

- **Widespread Use** [21 CFR 312.320]
  - Treatment IND; submitted as a new IND thought the product is under active development. 30 day waiting period required.
  - Treatment Protocol: Submitted under existing IND under active development. 30 day waiting period may be waived by FDA.
Access to Investigational Devices

If enrollment in an existing clinical trial is not possible, patients/physicians potentially can receive expanded access to investigational devices under one of three alternative mechanisms:

• Emergency Use 21 CFR 812.35(a)
• Compassionate Use (Individual Patient/Small Group Access) 21 CFR 812.35(a)
• Treatment Use 21 CFR 812.36

  – Notable divergence from IND regulations:
    » Requirement of independent assessment by uninvolved physician in Compassionate Use.
    » FDA Form 3926 not applicable – full IRB review required.
Limitations to FDA’s Expanded Access Drug Program

➤ Industry participation
  • FDA cannot require a manufacturer to provide a drug.
  • Manufacturers may decline requests (e.g., limited supply, effects on clinical trial recruitment and product development, administrative burden, or liability risk).

➤ Physician participation
  • Physician must determine that probable risk from drug is not greater than probable risk of disease.
  • Institution's interests may not always be aligned with the physician's interests.
  • Administrative burden.

➤ Cost
  • Commercial insurers generally will not pay for access.
  • Medicare covers only treatments that are “reasonable and necessary.”
Criticism of FDA’s Expanded Access Drug Program

- Application is burdensome, long, and challenging.
- Process is slow and ineffective.
- Patients could get access to investigational drugs faster without FDA’s involvement.
- Poses a barrier to individuals’ access.
CBER and CDER Expanded Access IND and Protocol Submissions, FY 2012 - 2017

- **Not Allowed to Proceed**
- **Allowed to Proceed**

<table>
<thead>
<tr>
<th>Fiscal Year</th>
<th>Total Number of IND and Protocol Submissions</th>
</tr>
</thead>
<tbody>
<tr>
<td>FY 12</td>
<td>1081</td>
</tr>
<tr>
<td>FY 13</td>
<td>1200</td>
</tr>
<tr>
<td>FY 14</td>
<td>1999</td>
</tr>
<tr>
<td>FY 15</td>
<td>1416</td>
</tr>
<tr>
<td>FY 16</td>
<td>1732</td>
</tr>
<tr>
<td>FY 17</td>
<td>1831</td>
</tr>
</tbody>
</table>

Source: https://www.fda.gov/downloads/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/UCM597570.pdf
Is Expanded Access Broken? **Approval Rate: Device**

### COMPASSIONATE USE IDE SUPPLEMENTS

<table>
<thead>
<tr>
<th>Year</th>
<th>Total Submissions</th>
<th>Evaluable Submissions*</th>
<th>Percent Approved**</th>
</tr>
</thead>
<tbody>
<tr>
<td>2012</td>
<td>135</td>
<td>123</td>
<td>99.19%</td>
</tr>
<tr>
<td>2013</td>
<td>181</td>
<td>175</td>
<td>98.86%</td>
</tr>
<tr>
<td>2014</td>
<td>228</td>
<td>216</td>
<td>99.54%</td>
</tr>
<tr>
<td>2015</td>
<td>215</td>
<td>208</td>
<td>99.04%</td>
</tr>
</tbody>
</table>

*Excludes those withdrawn or converted to Emergency Use while under review

**Based on Evaluable Submissions

### COMPASSIONATE USE REQUESTS WITHOUT AN IDE

<table>
<thead>
<tr>
<th>Year</th>
<th>Total Submissions</th>
<th>Evaluable Submissions*</th>
<th>Percent Approved**</th>
</tr>
</thead>
<tbody>
<tr>
<td>2012</td>
<td>53</td>
<td>53</td>
<td>98.11%</td>
</tr>
<tr>
<td>2013</td>
<td>138</td>
<td>134</td>
<td>91.79%</td>
</tr>
<tr>
<td>2014</td>
<td>112</td>
<td>101</td>
<td>99.01%</td>
</tr>
<tr>
<td>2015</td>
<td>170</td>
<td>167</td>
<td>98.80%</td>
</tr>
</tbody>
</table>

*Excludes those withdrawn or converted to Emergency Use while under review

**Based on Evaluable Submissions

Source:
https://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/HowtoMarketYourDevice/InvestigationalDeviceExemptionIDE/ucm051345.htm
### Is Expanded Access Broken? Response Time

#### Table 4: Median Food and Drug Administration (FDA) Review Time Frames, in Days, for Expanded Access IND Requests by Center, Type, and Whether the Request Was Allowed to Proceed, Fiscal Years 2012 through 2015

<table>
<thead>
<tr>
<th>FDA center</th>
<th>Single-Patient Allowed to proceed</th>
<th>Multiple Patients Treatment (widespread)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Emergency</td>
<td>Non-emergency</td>
</tr>
<tr>
<td>Center for Drug Evaluation and Research</td>
<td>2,094</td>
<td>2,483</td>
</tr>
<tr>
<td></td>
<td>&lt;1</td>
<td>3.0</td>
</tr>
<tr>
<td></td>
<td>15</td>
<td>30</td>
</tr>
<tr>
<td>Center for Biologics Evaluation and Research</td>
<td>257</td>
<td>118</td>
</tr>
<tr>
<td></td>
<td>&lt;1</td>
<td>19.0</td>
</tr>
<tr>
<td></td>
<td>25</td>
<td>30</td>
</tr>
<tr>
<td>Center for Drug Evaluation and Research</td>
<td>10</td>
<td>8</td>
</tr>
<tr>
<td></td>
<td>&lt;1</td>
<td>25.5</td>
</tr>
<tr>
<td></td>
<td>4</td>
<td>30</td>
</tr>
<tr>
<td>Center for Biologics Evaluation and Research</td>
<td>4</td>
<td>20</td>
</tr>
<tr>
<td></td>
<td>&lt;1</td>
<td>28.0</td>
</tr>
<tr>
<td></td>
<td>&lt;1</td>
<td>30</td>
</tr>
</tbody>
</table>

**Legend:** N/A = not applicable

**Source:** U.S. GAO, Investigational New Drugs: FDA Has Taken Steps to Improve the Expanded Access Program but Should Further Clarify How Adverse Events Data Are Used (July 2017)
### Table 4: Median Food and Drug Administration (FDA) Review Time Frames, in Days, for Expanded Access IND Requests by Center, Type, and Whether the Request Was Allowed to Proceed, Fiscal Years 2012 through 2015

<table>
<thead>
<tr>
<th>FDA Center</th>
<th>Single-Patient</th>
<th>Multiple Patients</th>
<th>Treatment (widespread)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Emergency</td>
<td>Non-emergency</td>
<td>Intermediate-size</td>
</tr>
<tr>
<td>Center for Drug Evaluation and Research</td>
<td>2,094</td>
<td>15</td>
<td>2,483</td>
</tr>
<tr>
<td>Center for Biologics Evaluation and Research</td>
<td>257</td>
<td>25</td>
<td>118</td>
</tr>
<tr>
<td>Center for Drug Evaluation and Research</td>
<td>10</td>
<td>4</td>
<td>8</td>
</tr>
<tr>
<td>Center for Biologics Evaluation and Research</td>
<td>4</td>
<td>&lt;1</td>
<td>&lt;1</td>
</tr>
</tbody>
</table>

**Legend:** N/A = not applicable

**Source:** GAO analysis of FDA data. OAG-17-504.

**Note:** This table only includes data on expanded access IND requests. It does not include expanded access protocol requests because data were unavailable.

Source: U.S. GAO, Investigational New Drugs: FDA Has Taken Steps to Improve the Expanded Access Program but Should Further Clarify How Adverse Events Data Are Used (July 2017)
Recent Streamlining Efforts

New Form FDA 3926 “Individual Patient Expanded Access IND”
- Streamlined alternative submission form to Form FDA 1571 for physicians to use for individual patient IND submissions (including emergency use)
- Accompanied by step-by-step instructions

<table>
<thead>
<tr>
<th></th>
<th>Form 1571</th>
<th>Form 3926</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of pages</td>
<td>3</td>
<td>2</td>
</tr>
<tr>
<td>Number of elements</td>
<td>26</td>
<td>11</td>
</tr>
<tr>
<td>Attachments</td>
<td>7</td>
<td>1</td>
</tr>
<tr>
<td>Time to complete</td>
<td>100 hours</td>
<td>45 minutes</td>
</tr>
</tbody>
</table>
Recent Streamlining Efforts

- **Updated and new guidance documents**
  - Expanded Access to Investigational Drugs for Treatment Use – Questions and Answers (Updated October 2017)
  - Individual Patient Expanded Access Applications: Form FDA 3926 (Updated October 2017)
  - Charging for Investigational Drugs Under an IND – Questions and Answers (June 2016)

- **Overhauled website**

- **New “Fact Sheets” for** physicians, patients, and industry

- **New online tool:** Expanded Access Navigator
  - Comprehensive online resource that collects in one location links to manufacturers’ expanded access policies, procedures, and points of contact.
  - Includes information to help guide patients and physicians.
Recent Streamlining Efforts

 Expedited IRB review for individual patient expanded access
  • [2017 FDA Reauthorization Act, P.L. 115-52] ("FDARA").
  • A physician submitting an individual patient access IND can request that only one IRB member – chair or another designated IRB member – concur with the treatment use.
  • FDA effectuated this change by modifying Form FDA 3926.

 Clarity regarding how FDA will use adverse events data
  • Sponsor must report an adverse event as a suspected adverse reaction only if evidence suggests a causal relationship between drug and adverse event.
  • Only in a “very small number of cases,” have adverse event data from expanded access treatments been reflected in the product labeling.
  • No instances in which adverse event information from expanded access has prevented the FDA from approving a drug.
Expanded Access Transparency

Availability of drug/device for expanded access on clinicaltrials.gov

- Clinical Trials Registration and Results Information Submission [42 CFR Part 11](#)
- If an individual/entity is required to register a trial at clinicaltrials.gov and is both the sponsor and the manufacturer of the investigational drug/device being studied, that individual/entity must submit certain information about the ability to obtain the drug/device through expanded access.
Publicly posting expanded access policy

- Manufacturers and distributors of investigational drugs for diagnosis, monitoring, or treatment of “serious diseases or conditions” must make their expanded access policy public and readily available (e.g., posted on publically available website).
- Expanded access policy must include:
  - Contact information for manufacturer/distributor.
  - Procedures for making expanded access requests.
  - Criteria used to evaluate requests.
  - Time necessary for manufacturer/distributor to acknowledge receipt of request.
  - Hyperlink or other reference to clinical trial record containing information required to be submitted to clinicaltrials.gov regarding expanded access availability.

Posting of policy is **not** a guarantee of access.
“Right To Try” Movement

➢ In 2014, the Goldwater Institute released a model for “right to try” legislation:
  • Authorized access to and use of experimental treatments for patients with an “advanced illness” without FDA approval.
  • Established conditions for eligibility and use.
  • Prohibited sanctions on providers solely for recommending or providing experimental treatments.

➢ The Goldwater Institute maintains that “right to try” laws protect an eligible patient’s fundamental right to access experimental medical products.
  • Although, the U.S. Court of Appeals for the District of Columbia ruled in 2007 that patients have no right “to a potentially toxic drug with no proven therapeutic benefit.”

  Abigail All. for Better Access to Developmental Drugs v. von Eschenbach, 495 F.3d 695 (D.C. Cir. 2007)
A few months after the Goldwater Institute's model was released, Colorado passed the first “right to try” legislation in the U.S.

Since then, 40 states have passed “right to try” laws, with Nebraska becoming the 40th state in April 2018.

Despite the passing of so many state “right to try” laws, they have remained largely unused.

Goldwater Institute has pointed to only two physicians in Texas who have treated patients under a “right to try” protocol.
“Right To Try” Legislation – States

Although states have used the Goldwater Institute’s model as a template, each state has drafted its legislation slightly differently:

- Definition of “terminally ill.”
- Criteria for patients to be eligible for access.
- Coverage/care patients may have to forfeit (e.g., hospice, home health care, or health insurance).
- Whether patients must participate in data collection.

None mandates providing access.
“Right To Try” Legislation – States

Criticism

• Offers false hope – nothing more than a “right to ask.”
• More likely to harm patients than to help.
  - Eliminating FDA oversight (FDA oftentimes requires changes to treatment protocols based on information not available to physicians).
  - Eliminating signoff from the IRB on treatment protocol and consent form.
  - Phase I clinical trials test the safety of a drug on only about 20-80 healthy volunteers.
• May negatively impact clinical trial recruitment and drug approval process.
“Right To Try” Movement

Sources: www.statnews.com and www.americansforprosperity.org
Several federal bills mirroring the state “right to try” laws have been introduced in the Senate and the House.

In August 2017, the Senate passed the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, S. 204, introduced by Sen. Ron Johnson (R-WI).


On May 22, 2018, the House passed S. 204.
S. 204: Key Provisions

Revises the FDCA to create an alternative additional pathway (other than the FDA’s Expanded Access Drug Program) for eligible patients to access eligible investigational drugs.

Patient eligibility

• Patient (i) diagnosed with “life-threatening disease or condition,” (ii) exhausted approved treatment options and unable to participate in a clinical trial involving the eligible investigational drug, as certified by a physician in good standing and not directly compensated by manufacturer for so certifying, and (iii) provides written informed consent.

Eligible investigational drug

• An investigational drug (i) for which a Phase I clinical trial has been completed, (ii) that has not been approved or licensed for use under Section 505 of the FDCA or Section 351 of the PHSA, (iii) for which a “New Drug Application” has been filed or that is under investigation in a clinical trial, and (iv) that is in active development.
S. 204: Key Provisions

> Reporting obligations

- Sponsor or manufacturer must submit to Secretary an annual summary of use via this pathway, including any known serious adverse events.

> Use of clinical outcomes

- Secretary may not use associated clinical outcomes to adversely affect review/approval of the investigational drug, unless (i) critical to determining safety of investigational drug, or (ii) per sponsor’s request. Secretary required to provide sponsor with written notice of determination, including public health justification.
S. 204: Key Provisions

➤ Liability

• **Sponsor and manufacturer**: No liability for acts/omissions related to the provision of a drug via this pathway.

• **Prescriber, dispenser, or other individual entity**: No liability for acts/omissions related to the provision of a drug via this pathway, **unless conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under applicable state law**.

➤ No liability for not providing access.
S. 204 does **not**:

- Oblige manufacturers to make eligible investigational drugs available to eligible patients.
- Require manufacturers to cover the costs associated with the treatment use of the eligible investigational drug.
- Require insurers to pay for treatment coverage costs.
- Address what coverage or care might eligible patients may have to forfeit.
- Require an IRB’s approval of the treatment protocol and the informed consent form prior to the provision of the eligible investigational drug.
### FDA Expanded Access Drug Program vs. S. 204

<table>
<thead>
<tr>
<th>Requirement</th>
<th>FDA Expanded Access Drug Program</th>
<th>S. 204</th>
</tr>
</thead>
<tbody>
<tr>
<td>Requires FDA approval</td>
<td>✓</td>
<td>✗</td>
</tr>
<tr>
<td>Requires IRB oversight</td>
<td>✓</td>
<td>✗</td>
</tr>
<tr>
<td>Requires physician approval</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>Requires manufacturer approval</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>Informed consent required</td>
<td>✓*</td>
<td>✓</td>
</tr>
<tr>
<td>Reporting obligations</td>
<td>✓*</td>
<td>✓</td>
</tr>
<tr>
<td>Limitations on liability</td>
<td>✗</td>
<td>✓</td>
</tr>
<tr>
<td>Restrictions on use of clinical outcomes</td>
<td>✗</td>
<td>✓</td>
</tr>
<tr>
<td>Drug must be in development</td>
<td>✗</td>
<td>✓</td>
</tr>
</tbody>
</table>

* More robust requirements
Looking Forward: The FDA

Scott Gottlieb, M.D.  
@SGottliebFDA

When the House passes #RightToTry legislation I stand ready to implement it in a way that achieves Congress’ intent to promote access and protect patients; and build on #FDA’s longstanding commitment to these important goals #RTT

3:22 AM - 22 May 2018

Source: https://twitter.com/SGottliebFDA
“While well-intentioned," Merck, a pharmaceutical company, wrote in an emailed statement, "current ‘Right-to-Try’ legislation is not in the best interest of patients and is unlikely to help us bring forward innovative, safe and effective medicines to all patients as quickly as possible. In addition, we remain supportive of the FDA having an oversight role in the process around expanded access to investigational medicines.”


“In our view, the F.D.A. plays a really important role,” Dr. Joanne Waldstreicher, the chief medical officer of Johnson & Johnson, said in an interview. The F.D.A., Dr. Waldstreicher said, has “information that we don’t have necessarily; they see safety and efficacy information on products that may be similar.”

Looking Forward: Implementation Questions

What does this mean for the state “right to try” laws?

• Preemption analysis (state vs. federal)?
  - Express preemption: N/A
  - Implied preemption:
    » Conflict: State and federal law cannot coexist.
    » Field: Pervasive federal regulation; Congressional intention to occupy the field.

• Would parties proceeding under the FDA’s Expanded Access Drug Program need to comply with specific requirements in the state “right to try” laws that go beyond FDA’s requirements?

How will the federal “right to try” law coexist with the FDA’s Expanded Access Drug Program?
Looking Forward: Implementation Questions

➢ Will the enactment of S. 204 influence stakeholders’ behavior?
  • Liability?
  • Administrative burden?

➢ If a patient seeks an investigational drug under the state or federal “right to try” law, how will manufacturers, treating providers, institutions, and IRBs respond?

➢ Potential for non-regulatory protections/conditions to access products through “right to try.”
  • Institutional policies requiring IRB or other review?
  • Manufacturers insisting on FDA pathway to make product available?

➢ Regardless of “right to try” – expanded access remains available.
Questions

➢ To submit your questions, please use the in-webinar question tool or email webinar@advvarra.com.
Thank You!

▷ We hope you found today’s webinar informative and useful.
▷ Please complete our survey to provide feedback on this session.
▷ In the survey, you can also request a certificate of attendance for this event.
▷ Stay tuned for more information on our next webinar.
Expanded Access and Right to Try: The Impact of Recent Legislative Changes

Kate Gallin Heffernan, Partner, Verrill Dana
Marylana Saadeh Helou, Associate, Verrill Dana
Robert Romanchuk, IRB Chair, Advarra

May 30, 2018