Regulatory Science Working Group
Webinar:
Clinical Development for Rare Diseases: A Primer for Rare Disease Patients and Advocates

Wednesday, March 29th 2017
Today’s Agenda

- Introduction to Community Congress and Regulatory Science Working Group
  - Max Bronstein, Chief Science Policy and Advocacy Officer, EveryLife Foundation for Rare Diseases
  - Lynne McGrath, Vice-President of Regulatory Affairs, RegenXBio
  - Isabelle Lousada, CEO and President, Amyloidosis Research Consortium

- Importance of Natural History Studies and Patient Registries
  - Austin Letcher, Senior Research Associate, CoRDS at Stanford Research

- Overview of Rare Disease Clinical Development Process - Phases and Timelines of Drug Development, Unraveling Industry Jargon, importance of Endpoints
  - Juliet M. Moritz, MPH, Executive Director, Strategic Development Department - Rare Diseases, Premier Research

- Special Approval Pathways and Incentives - Accelerated Approval, Fast Track, Priority Review, Breakthrough, Orphan Drug Act Incentives
  - Jonathan Goldsmith, Associate Director for Rare Diseases, Food and Drug Administration

- Early Access and Right to Try
  - David Farber, Partner, King & Spalding

No Disease Is Too Rare to Deserve Treatment
Mission and Core Principles

What We Believe:
- ALL patients deserve treatment, no matter how rare the disease
- Rare disease therapies should be safe and effective
- We could do more with the science we already have

What We Do:
- ADVOCATE for evidence-based policy and regulatory reform

How We Get it Done:
- Grassroots action
- Scientific and policy expertise
Program designed to foster collaboration between patient organizations and industry

Strategic advisory committees

On call to respond to urgent policy issues

Provide a forum for discussion with a goal of taking action
Our Structure

**Newborn Screening**
Co-Chairs: Jen Helfer, bluebird bio
Elisa Seeger, Aidan Jack Seeger Foundation

**Public Policy**
Co-Chairs: Mark Lenker, Shire
Christina Might, NGLY1.org

**Regulatory Science**
Co-Chairs: Lynne McGrath, RegenxBio
Isabelle Lousada, Amyloidosis Research Consortium (ARC)

EveryLife Foundation – Staff Support
Self-Selected Issues in 2016

- Public Policy – Early Access
- Newborn Screening – Legislation in California
- Regulatory Science – Navigating the Accelerated Approval Pathway
Self-Selected Issues 2017

- Public Policy – Sign-on Letter to Administration on Hiring Freeze
- Newborn Screening – Legislation in Florida
- Regulatory Science – Clinical Development 101

February 27, 2017

President Donald J. Trump
1500 Pennsylvania Avenue NW
Washington, DC 20500

Dear President Trump,

The undersigned 147 patient organizations write today about the challenges facing rare disease patients in America and the vital role that federal agencies play in helping to accelerate the research, development, review, and approval of treatments. These agencies must have the ability to hire and retain personnel in order to accomplish their respective missions and to achieve the broader goal of treating and curing diseases.

It is estimated that 1 in 10 individuals has a rare disease, defined as a condition affecting 200,000 or fewer patients in America. When combined, these diseases are not “rare” as more than 30 million Americans have a rare disease. There may be as many as 8,000 rare diseases, but unfortunately the vast majority (95%) do not yet have a treatment approved by the Food and Drug Administration (FDA). Many rare disease patients go years without receiving an accurate diagnosis, during which time their disease may progress unchecked.

However, the Orphan Drug Act of 1983 spurred substantial progress in the development of new treatments. This legislation helped enable the creation of an orphan drug industry, which is undergirded by critical investments in basic and applied research through the National Institutes of Health (NIH).
Joining

- You can sign-up today
- **FREE** for patient organizations to join
- Fee structure for industry – funding enables us to keep the program free for patient organizations and helps support events and programming
- Questions? [Mbronstein@everylifefoundation.org](mailto:Mbronstein@everylifefoundation.org)

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**EveryLife Foundation Community Congress**

**Click here to register for the November 16, 2016 in-person meeting**

**What is the Community Congress?**

The Community Congress is a membership-based program of the EveryLife Foundation dedicated to bringing patient organizations, industry leaders, and other rare disease stakeholder organizations together. The Congress acts as a strategic advisory council, providing key advice and insight on existing Foundation programs. Members of the Congress have the opportunity to help shape and drive Foundation policy initiatives and will have access to exclusive, members-only content through webinars and an annual, in-person meeting in Washington, D.C.

**Current working groups include:**

- **Public Policy** – Focusing on the topic of expanded/early access and guiding the development of the Foundation’s annual Scientific Workshop.
- **Regulatory** – Focusing on improving the drug approval process at FDA through the qualification of biomarkers and enhanced specialization.
- **Newborn Screening** – Focusing on expanding newborn screening for rare diseases through state-level legislation.

Each working group is co-chaired by a patient organization participant and industry participant. Working groups engage on an ongoing basis and meet annually in November in Washington D.C. Ultimately, Community Congress serves as a platform for engagement with rare disease stakeholders and provides critical guidance to the Foundation and leadership to the rare disease community.
Thank You Sponsors!
Regulatory Co-Chair: Isabelle Lousada

- President and CEO of Amyloidosis Research Consortium (ARC)
Regulatory Co-Chair: Lynne Fahey McGrath

- Vice-President, Regulatory Affairs, RegenxBIO
Importance of Patient Registries and Natural History Studies

Austin Letcher
Sr. Research Associate
Coordination of Rare Diseases at Sanford
Sanford Research
Mission: To accelerate research into rare diseases

Goal: To establish an international rare disease patient registry for all rare diseases

- Established by David Pearce, PhD in 2010
- Create resource of contact information and clinical data on individuals diagnosed with any rare disease to enable a comparative analysis across diseases
- Connect researchers to participants interested in participating in research
- Partner with patient advocacy groups to create disease specific registries and natural history studies
- IRB approval

*The term “disease” is used to encompass all rare conditions.*
Objectives

• Patient Registry vs. Natural History Study
• CoRDS Background
• Benefits
• Challenges
• Summary
What is a patient registry?

- A registry is a collection of information about individuals, usually focused around a specific diagnosis or condition. – NIH

- Contact registries collect basic demographic and contact data
  - Not the same as registering for an email list
  - It’s important to collect data which may be used as inclusion/exclusion criteria (birth date, geographic location, etc.)
What is a natural history study?

• Natural history is the natural course of the disease from the time immediately prior to its inception, progressing through its pre-symptomatic phase and different clinical stages to the point where the disease has ended without external intervention – *FDA R01 Natural History Study RFA*
  – Focuses on progression
  – Foundation of drug development/clinical trials
Registry ≠ Natural History

- Developing a patient registry around one rare disease does not mean that the natural history of the disease is being captured.
- Natural history studies are the foundation of drug discovery/clinical trials; however, patient registries are the foundation of natural history studies.
- Both natural history studies and registries are important for rare diseases:
  - Collect information to engage community, educate public and researchers.
Foundation

• Define your population
  – That’s the easy part!

• Identify questions in which the answers would be important to researchers, physicians or patients and families

• Collection of certain types of information can drive the policies and procedures
  – Types of participants (adults vs. children)
  – Informed consent
  – Data sharing preferences
Community

• Creating a patient registry is a great way to foster community (support group)
  – A means to develop new relationships
  – Gives participants a voice
  – Remove sense of isolation
  – Identity

• A registry and natural history study creates hope for the community
Education

• Patient registries and natural history studies:
  – Create awareness
  – Drive research
• Information collected will hopefully lead to publication of materials more accessible to physicians
• If physicians have more resources available they can make better decisions and better diagnose patients
• Ex: number of research articles available on PubMed
  – Type 1 diabetes ~ 76,000+
  – Bohring Opitz Syndrome (1 in a million prevalence) ~ 30
Research

• A rare disease diagnosis often means the patients are the experts
• Patient registries and natural history studies can help your organization attract the attention of researchers
• Researchers are often introduced to rare disease research by chance
  – There may not be a thought leader in the community so it’s best to be prepared for when an expert is found
Operational Challenges

- Patients are often motivated, but it is difficult to identify them
- Establishing partnerships with organizations
  - Trust is a key issue
- May need to recruit globally in order to accrue adequate patient populations (especially in ultra-rare cases)
- Data entry can be time consuming
- Critical to ensure patients aren’t lost to follow-up
Summary

• Patient registries and natural history studies present unique opportunities to foster community, educate, promote awareness and research
  – Benefits outweigh the challenges
• Patient registries are the phone book
• Natural history studies are the encyclopedia
• CoRDS is both
The CoRDS registry collects & organizes contact and clinical information on individuals diagnosed with a rare disease/condition (and unaffected carriers) as well as those undiagnosed cost-free.
Thank you!

Questions?

Austin Letcher
Sr. Research Associate, CoRDS
605.312.6423
Austin.Letcher@sanfordhealth.org
www.sanfordresearch.org/cords
Overview
Clinical Trials 101

Juliet Moritz, MPH
Executive Director, Strategic Development, RareDiseases
Premier Research
The Clinical Research Process in General

- **Pre-Clinical Testing**: In cells/tissues and animals, Characterize the Mechanism of Action (MOA)
- **Clinical Trials**: Controlled exposures in humans, Evaluate safety and efficacy of therapeutic
- **Post-Marketing Surveillance**: May be required as part of approval or may be spontaneous, Additional data regarding real-world use of the drug
The Clinical Research Process: A Deeper Dive

Phase I

• AKA “first in human” trials
• Small numbers of participants mainly to establish safety (10s)
• Focuses on really characterizing the pharmacodynamics (what the drug does to the body) and pharmacokinetics (what the body does to the drug)
• Often completed in healthy, normal, volunteers (HNVs); exceptions may include:
  • Actual patients in some conditions
  • Patients who have renal or liver impairments that could affect how the drug is processed by the body
• Usually last several months as patient advance in cohorts as exposed to higher or multiple doses

➢ 70% of drugs tested in Phase I advance into Phase II

Phase II

• Tests both safety and efficacy of the therapeutic
• May include the introduction of a placebo to help establish efficacy data or comparison against the standard of care
• Includes only patients with the condition under study; no HNVs
• Will involve increased numbers of patients compared to Phase I, sometimes up to hundreds of patients (100s)
• May include more than one dose to better clarify dosing going into larger populations
• Protocols are well-defined in terms of excluding concurrent conditions or concomitant medications that could potentially make signal detection more difficult

➢ 33% of drugs that make it into Phase II advance into Phase III
The Clinical Research Process: A Deeper Dive

Phase III (Pivotal)

- Tests both safety and efficacy of the therapeutic
- In order to be approved, most therapeutics are required to have two adequate and well-controlled studies to support the application for approval
- Not the result of coincidence
- Reproducible
- Robust
- Protocols are well-defined in terms of excluding condition or concomitant medications that could potentially make signal detection more difficult
- Larger number of patients than Phase II trials, sometimes thousands of patients and multiple countries (100s-1000s)
- 70%-90% of drugs tested in Phase III advance into Phase IV (i.e., receive marketing authorization)

Phase IV (Post-Marketing)

- Represent more “real-world” use of the therapeutic which may include patients who have some of the concurrent conditions or who take the concomitant medications that were excluded in Phases II and III
- May be required as part of the approval of a therapeutic if there are some safety concerns or if the number of patients exposed to the therapeutic in Phases II and III was relatively small
- Data to be collected may be pre-specified if required by regulatory agencies and may contribute to re-evaluation of the approval of the drug after a certain period of time
Time and Cost to Get to Market – Prevalent Conditions

$2,558,000,000*
average cost to develop and gain marketing approval for a new drug

*The Tufts CSDD estimate also accounts for expenses incurred for product development efforts that did not reach fruition

http://csdd.tufts.edu/news/complete_story/tufts_csdd_rd_cost_study_now_published

10,000 compounds tested in discovery
~250 compounds identified as potential drug candidates
~3-6 years

10,000 compounds tested in discovery
~250 compounds identified as potential drug candidates
~3-6 years

Drug Discovery and Pre-Clinical

Pre-Clinical Research

~5 candidate therapies advance into clinical trials
~1 candidate therapy completes clinical trials
~6-8+ years

Depending on the regulatory pathway can take ~0.5-2 years after submission for approval
Payer decisions occur after approval

Review and Approval
Rare Disease Research Challenges

- **Small Population**
  - How to find patients?

- **Geographically Widespread**
  - How to access patients?

- **Research Naïve Sites**
  - How to guarantee data integrity?

- **Unknown/Sparse Natural History**
  - What controls to use?

- **Lack of Defined Biomarkers**
  - How to measure activity?

- **Lack of Surrogate Endpoints**
  - How to define success?

- **Obstacles to Patient Retention**
  - How to get all the data?
Drug Development – Rare Disease Differences

- Rare disease trials don’t always follow the “standard” regulatory process
- Due to small number of patients targeted for rare disease trials can impact the development pathway
- Depending on the therapeutic or mechanism of action, early phase trials may be conducted in actual patients vs. HNVs
- It may be unethical or very challenging to include a placebo in the development program of a rare disease therapeutic
- The small number of patients and impact of the therapeutic under study can result in combining research phases (the “slash” studies, e.g., Phase I/II or II/III)
- Combining research phases does not usually result in faster development timelines for rare disease therapeutics mainly due to the small numbers of patients from which to recruit or lengthy follow-up times
Rare Disease: Phase I/II

Proof of Concept Study

- Early stage of clinical drug development when a compound has shown potential in animal models and early safety testing
- Small-scale studies are designed to detect a signal that the drug is active on a pathophysiologically relevant mechanism, as well as preliminary evidence of efficacy in a clinically relevant endpoint
- In rare diseases elements of the proof-of-concept study are often combined with elements of phase I studies such as PK or PD studies
Some Differences Between Rare and Non-Rare Clinical Trials

http://www.ncbi.nlm.nih.gov/pmc/articles/PMC4255432/
What is the average time and cost to develop a therapeutic for a rare disease?
EveryLife Foundation: Community Congress and Regulatory Science Working Group

Jonathan C. Goldsmith, MD, FACP
Associate Director

Rare Diseases Program
Office of New Drugs/CDER/FDA

29 March 2017
Disclosures

• No Conflicts of Interest
• Nothing to Report
• Opinions expressed are personal and may not reflect those of the FDA
Outline

• Incentives for Orphan Drug Development
• Drug Approval Standards
• Expedited Programs for Serious Conditions with Unmet Medical Need
Rare Disease Regulation at FDA (1)

• Orphan Drug Act (ODA)
  – Signed into law in 1983
  – Purpose

  • To promote the development of products that demonstrate promise for the diagnosis, prevention and/or treatment of rare diseases
Rare Disease Regulation at FDA (2)

- Orphan Designation
  - The Office of Orphan Products Development reviews and decides whether designation is granted

- Requirements:
  - Rare disease/condition with an affected population of less than 200,000 Americans
  - Adequate demonstration of scientific rationale to establish medical plausibility for the drug’s expected benefit
  - If more than 200,000 are affected but there is no “reasonable expectation” that the development costs would be recovered from U.S. sales
Rare Disease Regulation at FDA (3)

– Orphan Designation mainly provides financial incentives:

• Exemption from application fees ($2.038 million per application – CY 2017)
• 50% tax credit for clinical study costs
• Seven (7) years marketing exclusivity for an approved Orphan product
• Eligible to apply for FDA Orphan grants program to support clinical research
Drug Approval Standards

• The standard for approval of rare disease drugs and biological products is the same as for prevalent disease drugs - substantial evidence of product effectiveness.

Kefauver Harris Amendment –FD&C Act § 505(d), 21 USC 355(d) (1962)
See Guidance for Industry Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products, May 1998
Drug Approval Standards

• “Substantial evidence” is defined as: “evidence consisting of adequate and well-controlled (A&WC) investigations… on the basis of which it could fairly and responsibly be concluded by such experts that the drug will have the effect it purports or is represented to have under the conditions of use…”

Kefauver Harris Amendment –FD&C Act § 505(d), 21 USC 355(d) (1962)
See Guidance for Industry Providing Clinical Evidence of Effectiveness for Human Drug and Biological Products, May 1998
Flexibility

• The regulations do, however, allow for flexibility and scientific judgment in applying the standard, and in determining the kind and quantity of data required to be provided for a particular drug.

Kefauver Harris Amendment –FD&C Act § 505(d), 21 USC 355(d) (1962)
As amended by Food and Drug Administration Modernization Act Sec 115 (1997)
21 CFR 314.105 Approval of an Application
Flexibility

• For rare disease drugs, relying on one A&W trial with supporting evidence is not unusual, but the adequacy of this approach for any given drug must be considered in the context of the disease, the population, the properties of the drug, and the magnitude of the clinical trial results.

Kefauver Harris Amendment -FD&C Act § 505(d), 21 USC 355(d) (1962)
As amended by Food and Drug Administration Modernization Act Sec 115 (1997)
21 CFR 314.105 Approval of an Application
Flexible Clinical Development Programs
CDER NME and Therapeutic Biologic Approvals - 2008-2016

<table>
<thead>
<tr>
<th>Flexible Development Programs</th>
<th>Rare Approvals</th>
<th>Non-Rare Approvals</th>
</tr>
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<tbody>
<tr>
<td>Use of one or more flexible development approaches*</td>
<td>88 (78%)</td>
<td>68 (35%)</td>
</tr>
<tr>
<td>Traditional development program**</td>
<td>25 (22%)</td>
<td>127 (65%)</td>
</tr>
</tbody>
</table>

*Flexible Development approaches are defined as approval supported by other than 2 AWC Studies and/or use of a novel end point

**Traditional Development defined as ≥2 AWC studies using endpoints with prior precedents
Expedited Programs for Drug Development

Programs for drugs that target serious or life threatening diseases with unmet medical need

• **Fast Track Designation** (FDAMA 1997/FDASIA 2012)
  – More frequent meetings with the agency and rolling review

• **Breakthrough Designation** (FD&C Act/FDASIA 2012)
  – Intensive guidance on efficient drug development, senior manager commitment and rolling review

Guidance for Industry Expedited Programs for Serious Conditions – Drugs and Biologics, May 2014
Expedited Programs for Drug Development

• **Priority Review Designation** *(PDUFA 1992)*
  - A shorter review clock for marketing applications, 6 months compared with the standard review time of 10 months

• **Accelerated Approval** *(21CFR314 subpart H, 601 subpart E/FDASIA 2012)*
  - Favorable effect on a surrogate or intermediate clinical endpoint reasonably likely to predict a drug’s clinical benefit. FDA expects the sponsor to confirm the clinical benefit through performance of a trial post-marketing.

Guidance for Industry Expedited Programs for Serious Conditions – Drugs and Biologics, May 2014
## CDER New Molecular Entity and New Therapeutic Biologic Approvals 2008-2016*

<table>
<thead>
<tr>
<th>Expedited program for serious unmet need</th>
<th>Rare disease drugs (n = 109)</th>
<th>Non-rare disease drugs (n = 193)</th>
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<tbody>
<tr>
<td>Fast Track</td>
<td>54%</td>
<td>22%</td>
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<tr>
<td>Breakthrough Therapy</td>
<td>18%</td>
<td>4%</td>
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<tr>
<td>Priority Review</td>
<td>75%</td>
<td>30%</td>
</tr>
<tr>
<td>Accelerated Approval</td>
<td>26%</td>
<td>2%</td>
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<tr>
<td>Used any expedited program</td>
<td>86%</td>
<td>35%</td>
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</tbody>
</table>

*as of September 7, 2016
Thank you very much for your attention!

Questions?

Jonathan.Goldsmith@fda.hhs.gov

Rare Diseases Program/OND/CDER/FDA

CDERONDRareDiseaseProgram@fda.hhs.gov
EXPANDED ACCESS:
A RADICALLY DIFFERENT NEW DAY

March 29, 2017

David J. Farber
Partner
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King & Spalding LLP
What is Expanded Access?

- Program established by Congress and regulated by FDA
- Goal is to improve access to investigational drugs by certain patients for treatment purposes
- Differs from a clinical investigation where the primary purpose is research (i.e., systematic collection of data to determine safety and/or effectiveness)
FDA Statistics – Single Patient Requests

CBER and CDER Single Patient Expanded Access
IND Submissions,
FY 2010 - 2015

<table>
<thead>
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<th>Year</th>
<th>Number of Submissions</th>
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<tr>
<td>FY10*</td>
<td>19</td>
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<tr>
<td>FY11*</td>
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<td>FY13</td>
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<tr>
<td>FY14</td>
<td>11</td>
</tr>
<tr>
<td>FY15</td>
<td>10</td>
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- Red: Not Allowed to Proceed
- Blue: Allowed to Proceed
So What Is Driving Policy Change?
Trump Highlights the Issue

January 31, 2017 Trump Meeting with Pharmaceutical Executives

“One thing that’s always disturbed me, they come up with a new drug for a patient who’s terminal, and the FDA says, ‘We can’t have this drug used on the patient,’ but they say that the patient within four weeks will be dead. They say, ‘Well, we still can’t approve the drug. And we don’t know if the drug works or it doesn’t work, but we can’t approve the drug because we don’t want to hurt the patient.’ But the patient is not gonna live more than four weeks. So we’re gonna be changing a lot of – a lot of the rules.”
February 7, 2017: VP Pence meets with Right to Try advocates in the White House

A Right to Try law is fundamentally about restoring hope & giving terminally ill patients a fighting chance. @POTUS & I support your cause.
Policy Changes and New Requirements

- Revised request form for physicians
- Finalized draft guidances
- User-friendly website

- 21st Century Cures Act
- Federal Right to Try Legislation pending

- ClinicalTrials.Gov disclosure requirements

- State Right to Try Legislation
FDA Reduces the Paperwork

- In February 2015, FDA issued Draft Guidance containing a new form (Form FDA 3926) for physicians to make expanded access requests for individual patients
- Form FDA 3926 streamlines the process for making such requests, which would otherwise need to be made on the general IND submission form (Form FDA 1571)
New Patient-Targeted “How To” Information

Q. What is single patient expanded access, and do I qualify?
A. Expanded access is the use of an investigational drug outside of clinical trials to diagnose, monitor, or treat serious or life-threatening illnesses. Your physician will help guide you through the process.

To obtain expanded access, you must:
- Have a serious or immediately life-threatening disease or condition;
- Have no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; and
- Generally be unable to participate in a clinical trial.

Q. What are the risks to me?
A. Because these are investigational drugs and they are not yet FDA-approved, there may be unknown risks.

Q. What helps protect me from risk?
A. Your doctor must request approval for expanded access use of a drug from an Institutional Review Board (IRB) and authorization from FDA. An IRB is a committee that reviews the plan for expanded access to ensure that your rights and welfare are protected. Before starting treatment, your doctor must give you an informed consent document to sign that makes you aware of the potential risks associated with the drug. FDA will only authorize the application if your doctor makes a determination that the risks of using an experimental medicine are justified in your case.

For more information about drugs in development and clinical trials around the world, visit www.clinicaltrials.gov. You can also contact the drug company or patient advocacy organizations to see if they have information on expanded access programs or ongoing clinical trials.

Q. How do I request expanded access?
A. Physicians request expanded access from the FDA for their patients. The physician should first contact the drug company to make sure it is willing to provide an investigational drug to you before requesting expanded access from the FDA. FDA cannot make a drug company provide their investigational drug for expanded access; they must do so voluntarily. The approval from the drug company is a letter of authorization (LOA).

Q. If I am authorized for expanded access, when can I begin treatment?
A. If the drug company agrees to provide the drug, you may begin treatment 30 days after FDA receives the request, unless your doctor receives earlier notification from FDA that the treatment may proceed. Your doctor must also receive IRB approval before treatment can begin. FDA reviews and responds to expanded access requests often within days of receiving the request. Your physician is responsible for administering the drug and monitoring you carefully.

Q. What are the procedures if I need the drug on an emergency basis?
A. In an emergency, FDA can grant access over the phone and treatment can start once your physician receives the medication from the drug company. However, your physician must complete an expanded access application within 15 working days and notify the IRB within 5 days of obtaining approval from FDA.

Q. How much will it cost? Will insurance cover it?
A. The treating physician may request authorization from FDA to charge for an investigational drug, and can only charge you the direct costs of making the drug available, such as manufacturing and shipping. Any additional costs for administering the drug and monitoring its use will depend on your insurance coverage and do not require FDA authorization. FDA has no authority to require that the Centers for Medicare and Medicaid Services (CMS) or any private health insurance company reimburse for investigational drugs for which FDA has authorized charging. It is important that you and your physician consider the cost of the investigational drug and the medical services associated with its use that are not covered by third-party payers such as insurance or Medicare.

Q. What are some reasons I might not receive expanded access?
A. The drug company may choose not to provide the drug or there may not be enough drug supply. The FDA may deny a request for a number of reasons, including if there are available clinical trials for that drug or if the clinical severity of the disease or condition does not outweigh the risks of the drug. However, FDA grants expanded access for almost all of the applications it receives.

Contact
PatientNetwork@fda.hhs.gov or 301-796-8460
with any questions.

More Information:
- FDA Information for Patients: Expanded Access
- FDA Guidance: Expanded Access to Investigational Drugs for Treatment Use
- Questions and Answers
- Application for Individual Patient Expanded Access

U.S. FOOD & DRUG ADMINISTRATION

King & Spalding
Overview of 21st Century Cures
Section 3032: Requires Manufacturers to Publicly Post Expanded Access Policies

- Contact information to facilitate communication about expanded access requests
- Procedures for making such requests
- General criteria the manufacturer will use to evaluate requests for individual patients, and responses to requests
- Length of time the manufacturer anticipates will be necessary to acknowledge receipt of requests
- Hyperlink or other reference to the Expanded Access Record in ClinicalTrials.Gov
The Public Health Service Act § 402(j) requires the submission of information regarding whether, for an applicable drug clinical trial of an unapproved drug/biological product, expanded access is available under FDCA § 561. HHS New Rule published to little fanfare in September 2016.
## What Information Will be Available?

<table>
<thead>
<tr>
<th>Brief Title</th>
<th>Official Title</th>
<th>Brief Summary</th>
<th>Study Type (which is “expanded access” for this type of record)</th>
<th>Primary Disease or Condition</th>
</tr>
</thead>
<tbody>
<tr>
<td>Intervention Name(s)</td>
<td>Other Intervention Names(s)</td>
<td>Intervention Description</td>
<td>Intervention Type (which is typically “drug”)</td>
<td>Expanded Access Type</td>
</tr>
<tr>
<td>Eligibility Criteria</td>
<td>Sex/Gender</td>
<td>Age Limits</td>
<td>Expanded Access Status</td>
<td>Name of the Sponsor</td>
</tr>
<tr>
<td>Responsible Party, by Official Title</td>
<td>Contact Information</td>
<td>Unique Protocol Identification Number</td>
<td>Secondary ID</td>
<td>U.S. Food and Drug Administration IND Number</td>
</tr>
</tbody>
</table>

Record Verification Date

Responsible Party Contact Information

Only bolded data elements are required for individual patient expanded access records.
More Transparency for Expanded Access

ClinicalTrials.gov
A service of the U.S. National Institutes of Health
Try our beta test site

Advanced Search

Search Terms: [Field]
Study Type: Expanded Access Studies
Study Results: All Studies
Recruitment: All Studies
Status of State “Right to Try” Laws

33 states have adopted Right to Try laws, and 14 have pending bills.

Green = Passed Law
Blue = Introduced Legislation
Red = Vetoed

Source: Goldwater Institute (righttotry.org/in-your-state/)
Some in Congress Trying to Build on State “Right to Try” Laws

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

1. Intended to treat a patient who has been diagnosed with a terminal illness;
2. Authorized by, and in accordance with, State law;
3. Drug is past Phase I clinical trials

S.204 - Trickett Wendler Right to Try Act of 2017 (Sen. Ron Johnson) - 44 co-sponsors

H.R.878 – Right to Try Act of 2017 (Rep. Andy Biggs) - 36 co-sponsors
Summary and What’s Next

Patients and physician will have significant increased information, both due to recent changes through CURES and ClinicalTrial.gov

This is not a simple issue – there is as much opportunity to do harm than to do good

With all the new available data, and even without right to try laws, expanded access will look very different two years from now than it does today.
THANK YOU!

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Thank you attendees!

- Questions about joining Community Congress: mbronstein@everylifefoundation.org
- Visit: http://everylifefoundation.org/community-congress/ For more information