Expanded Access Programs for Drugs and Biologics (in plain English)

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What is expanded access?

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[Code of Federal Regulations]
[Title 21, Volume 5]
[Revised as of April 1, 2017]
[CITE: 21CFR312]

TITLE 21--FOOD AND DRUGS
CHAPTER I--FOOD AND DRUG ADMINISTRATION
DEPARTMENT OF HEALTH AND HUMAN SERVICES
SUBCHAPTER D--DRUGS FOR HUMAN USE
PART 312 INVESTIGATIONAL NEW DRUG APPLICATION

Subpart I--Expanded Access to Investigational Drugs for Treatment Use

Sec. 312.300 General.

(a) *Scope.* This subpart contains the requirements for the use of investigational new drugs and approved drugs where availability is limited by a risk evaluation and mitigation strategy (REMS) when the primary purpose is to diagnose, monitor, or treat a patient's disease or condition. The aim of this subpart is to facilitate the availability of such drugs to patients with serious diseases or conditions when there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the patient's disease or condition.

(b) *Definitions.* The following definitions of terms apply to this subpart:

Immediately life-threatening disease or condition means a stage of disease in which there is reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment.

Serious disease or condition means a disease or condition associated with morbidity that has substantial impact on day-to-day functioning. Short-lived and self-limiting morbidity will usually not be sufficient, but the morbidity need not be irreversible, provided it is persistent or recurrent. Whether a disease or condition is serious is a matter of clinical judgment, based on its impact on such factors as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one.

Sec. 312.305 Requirements for all expanded access uses.

The criteria, submission requirements, safeguards, and beginning treatment information set out in this section apply to all expanded access uses described in this subpart. Additional criteria, submission requirements, and safeguards that apply to specific types of expanded access are described in 312.310 through 312.320.

(a) *Criteria.* FDA must determine that:

21 CFR 312.300, Subpart I:
Aim is to facilitate the availability of investigational new drugs to patients with serious diseases or conditions when there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the patients condition

Expanded access: plain English

- “Compassionate” use
- You have a serious illness and you’ve tried everything else
- You and your doctor think an investigational drug(not approved) might be a good option
- The drug may be studied in clinical trials, but you can’t participate in these trials

Access to treatments

Approved Drugs

Safety and efficacy established

Broadest availability

3rd party reimbursement

Clinical Trials

Provide data to determine safety & effectiveness

Path to approval and broad availability

Expanded Access

For unapproved drugs or approved drugs with restricted availability

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Trial enrollment not possible

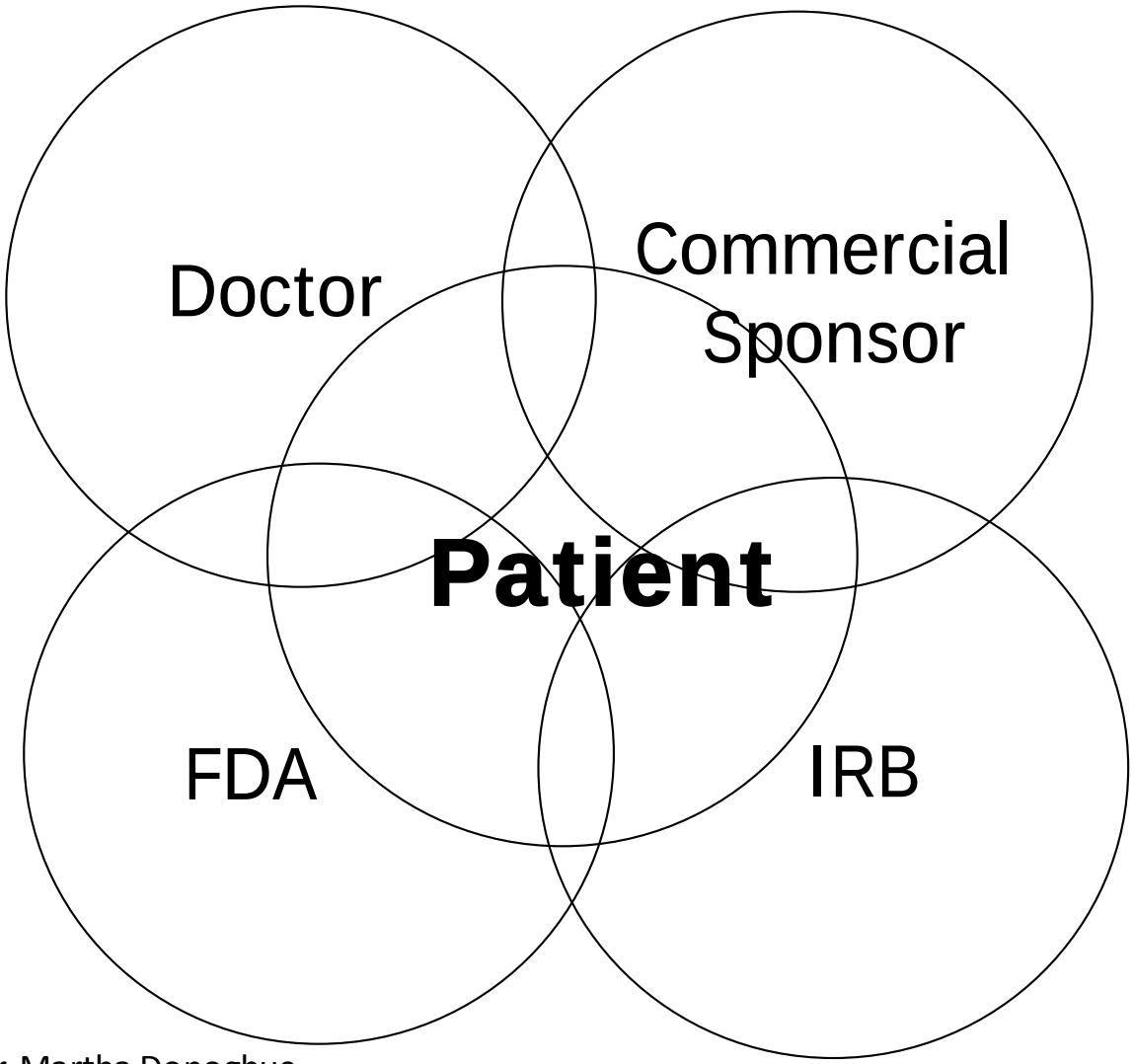
Slide adapted from Dr. Martha Donoghue

What do you need for expanded access?

- Serious and life threatening illness
- No satisfactory alternative therapy
- Potential benefit outweighs the potential harm
- Providing the drug will not interfere with getting the information needed for approval

How to Apply for Expanded Access?

The single patient IND



Slide adapted from Dr. Martha Donoghue

Misconceptions

- Expanded access is rarely granted and FDA denies many/most requests
- It takes weeks/months for FDA to review requests for expanded access
- If an adverse event occurs, it could jeopardize drug approval
- There is too much paperwork and the process is confusing

Form FDA 3926

DEPARTMENT OF HEALTH AND HUMAN SERVICES
Food and Drug Administration

Individual Patient Expanded Access Investigational New Drug Application (IND)

(Title 21, Code of Federal Regulations (CFR) Part 312)

Form Approved: OMB No. 0910-0814
Expiration Date: April 30, 2019
See PRA Statement on last page.

1. Patient's Initials

2. Date of Submission *(mm/dd/yyyy)*

3.a. Initial Submission

☐ Select this box if this form is an initial submission for an individual patient expanded access IND, and complete only fields 4 through 8, and fields 10 and 11.

3.b. Follow-Up Submission

☐ Select this box if this form accompanies a follow-up submission to an existing individual patient expanded access IND, and complete the items to the right in this section, and fields 8 through 11.

Investigational Drug Name

Physician's IND Number

4. Clinical Information

Indication

Brief Clinical History *(Patient's age, gender, weight, allergies, diagnosis, prior therapy, response to prior therapy, reason for request, including an explanation of why the patient lacks other therapeutic options)*

5. Treatment Information

Investigational Drug Name

Name of the entity that will supply the drug *(generally the manufacturer)*

FDA Review Division *(if known)*

Treatment Plan *(Including the dose, route and schedule of administration, planned duration, and monitoring procedures. Also include modifications to the treatment plan in the event of toxicity.)*

6. Letter of Authorization (LOA), if applicable *(generally obtained from the manufacturer of the drug)*

☐ I have attached the LOA. *(Attach the LOA; if electronic, use normal PDF functions for file attachments.)*

Note: If there is no LOA, consult the Form Instructions.

7. Physician's Qualification Statement *(Including medical school attended, year of graduation, medical specialty, state medical license number, current employment, and job title. Alternatively, attach the first few pages of physician's curriculum vitae (CV), provided they contain this information. If attaching the CV electronically, use normal PDF functions for file attachments.)*

8. Physician Name, Address, and Contact Information

Physician Name *(Sponsor)*	Email Address of Physician
Address 1 *(Street address, No P.O. boxes)*	
Address 2 *(Apartment, suite, unit, building, floor, etc.)*	Telephone Number of Physician
City / State	Facsimile (FAX) Number of Physician
ZIP Code	Physician's IND number, if known

PSC Publishing Services (301) 443-6740 EF

9. Contents of Submission

This submission contains the following materials, which are attached to this form (select all that apply). If none of the following apply to the follow-up communications, use Form FDA 1571 for your submission.

- ☐ Initial Written IND Safety Report
- ☐ Follow-up to a Written IND Safety Report
- ☐ Annual Report
- ☐ Summary of Expanded Access Use (treatment completed)
- ☐ Change in Treatment Plan
- ☐ General Correspondence
- ☐ Response to FDA Request for Information
- ☐ Response to Clinical Hold

10.a. Request for Authorization to Use Form FDA 3926

☐ I request authorization to submit this Form FDA 3926 to comply with FDA's requirements for an individual patient expanded access IND.

10.b. Request for Authorization to Use Alternative IRB Review Procedures

☐ I request authorization to obtain concurrence by the Institutional Review Board (IRB) chairperson or by a designated IRB member, before the treatment use begins, in order to comply with FDA's requirements for IRB review and approval. This concurrence would be in lieu of review and approval at a convened IRB meeting at which a majority of the members are present.

11. Certification Statement: I will not begin treatment until 30 days after FDA's receipt of a completed application and all required materials unless I receive earlier notification from FDA that treatment may begin. I also agree not to begin or continue clinical investigations covered by the IND if those studies are placed on clinical hold. I also certify that I will obtain informed consent, and that an Institutional Review Board (IRB) will be responsible for initial and continuing review and approval of this treatment use, consistent with applicable FDA requirements. I understand that in the case of an emergency request, treatment may begin without prior IRB approval, provided the IRB is notified of the emergency treatment within 5 working days of treatment. I agree to conduct the investigation in accordance with all other applicable regulatory requirements.

WARNING: A willfully false statement is a criminal offense (U.S.C. Title 18, Sec. 1001).

Signature of Physician	**Date**
To enable the signature field, please fill out all prior required fields. For a list of required fields which have not yet been filled out, please click here.	

For FDA Use Only

Date of FDA Receipt	Is this an emergency individual patient IND? ☐ Yes ☐ No	Is this indication for a rare disease (prevalence < 200,000 in the U.S.)? ☐ Yes ☐ No
IND Number		

This section applies only to requirements of the Paperwork Reduction Act of 1995.

DO NOT SEND YOUR COMPLETED FORM TO THE PRA STAFF EMAIL ADDRESS BELOW.

The burden time for this collection of information is estimated to average 45 minutes per response, including the time to review instructions, search existing data sources, gather and maintain the data needed and complete and review the collection of information. Send comments regarding this burden estimate or any other aspect of this information collection, including suggestions for reducing this burden, to:

Department of Health and Human Services
Food and Drug Administration
Office of Operations
Paperwork Reduction Act (PRA) Staff
PRAStaff@fda.hhs.gov

"An agency may not conduct or sponsor, and a person is not required to respond to, a collection of information unless it displays a currently valid OMB number."

Pros and Cons

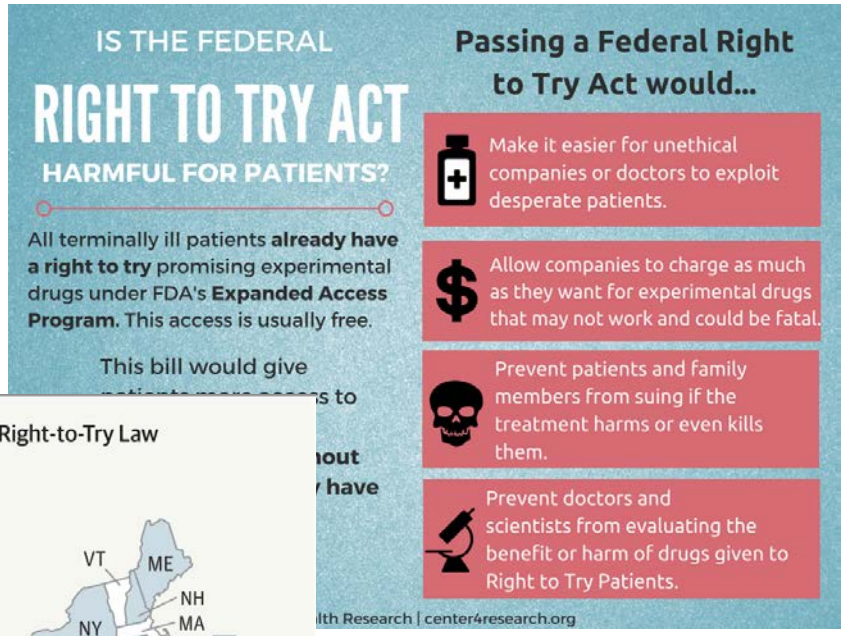
Pros

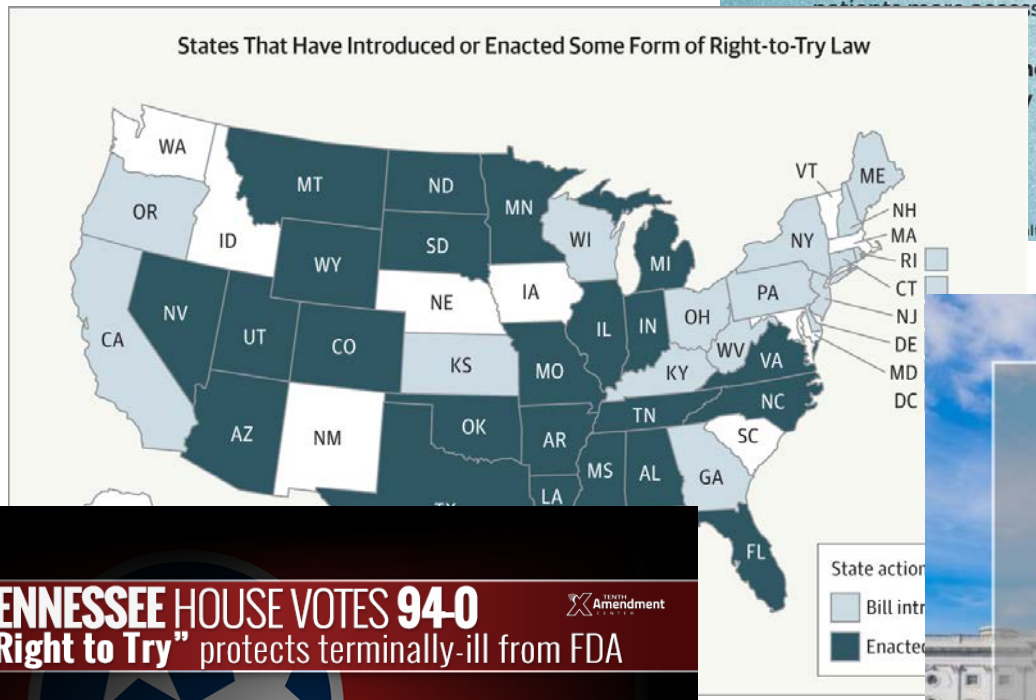
- Provides access to potentially lifesaving therapies
- Bridges gap between drug development and FDA approval
- May provide data to support development

Cons

- Limited safety and efficacy information
- May overestimate benefit and underestimate risk
- Loss of information
- Paperwork!

Slide adapted from Dr. Martha Donoghue


IS THE FEDERAL
RIGHT TO TRY ACT
HARMFUL FOR PATIENTS?
All terminally ill patients already have a right to try promising experimental drugs under FDA's Expanded Access Program. This access is usually free.
This bill would give
Passing a Federal Right to Try Act would...
Make it easier for unethical companies or doctors to exploit desperate patients.
Allow companies to charge as much as they want for experimental drugs that may not work and could be fatal.
Prevent patients and family members from suing if the treatment harms or even kills them.
Prevent doctors and scientists from evaluating the benefit or harm of drugs given to Right to Try Patients.
Research | center4research.org


States That Have Introduced or Enacted Some Form of Right-to-Try Law
WA
OR
CA
ID
NV
MT
WY
UT
AZ
CO
NM
ND
SD
NE
KS
OK
TX
MN
IA
MO
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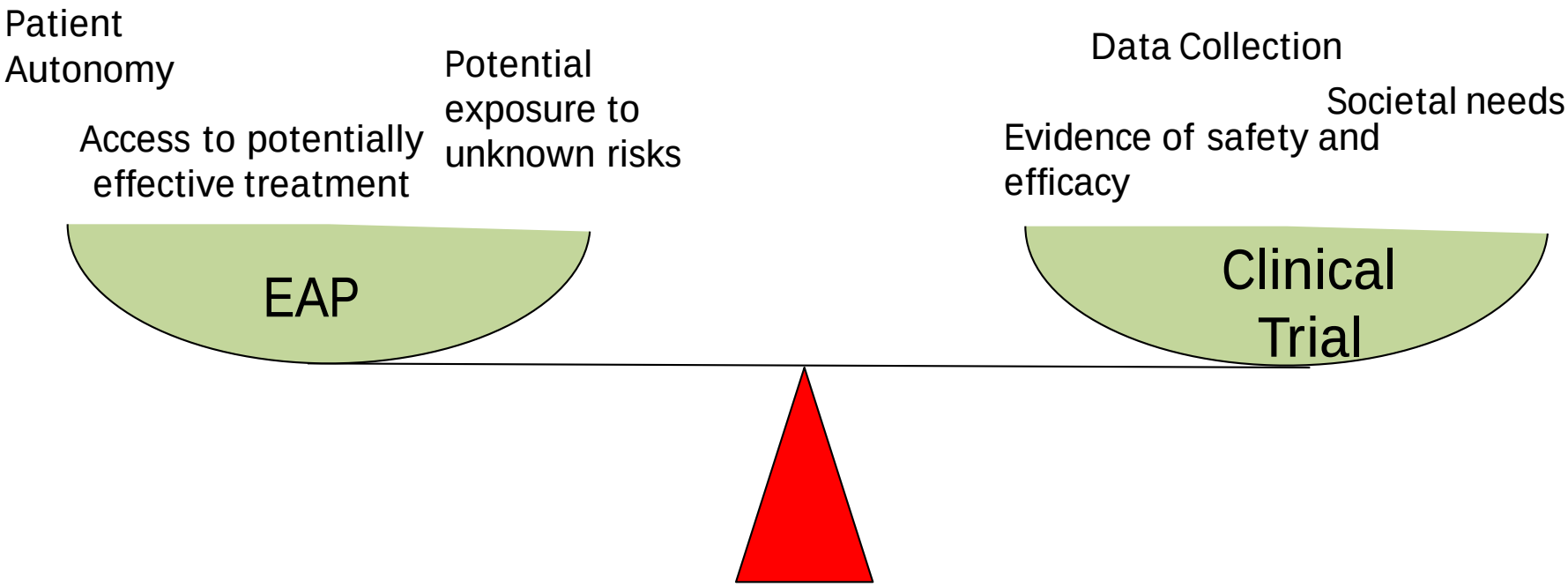
LA
WI
IL
MS
MI
IN
KY
TN
AL
OH
WV
GA
FL
SC
NC
VA
PA
NY
VT
ME
NH
MA
RI
CT
NJ
DE
MD
DC


TENNESSEE HOUSE VOTES 94-0
"Right to Try" protects terminally-ill from FDA
Tenth Amendment


U.S. Senate Passes
'Right to Try' Bill
to help Terminally ill
Patients get
Experimental
Treatments such as
Stem Cell Therapy

Balanced Approach



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Thank you!


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U.S. FOOD & DRUG
ADMINISTRATION