Expanded Access / Compassionate Use stakeholder focus groups

MODERATOR GUIDE

CONFIDENTIAL

PRA compliance

Control number and expiration date	OMB control number: 0910-0497 Expiration date: 10/31/2020
Public reporting burden	Public reporting burden for this collection of information is estimated to be 90 minutes, including time for reviewing instructions, searching existing data sources, gathering and maintaining the data needed, and completing and reviewing the collection of information. Send comments regarding this burden estimate or any other aspect of this collection of information, including suggestion for reducing burden to 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 control number. The control number for this project is 0910-0497.
Voluntary participation	Your participation/nonparticipation is completely voluntary, and your responses will not have an effect on your eligibility for receipt of any FDA services. In instances where respondent identity is needed (e.g., for follow-up of non-responders), this information collection fully complies with all aspects of the Privacy Act and data will be kept private to the fullest extent allowed by law.

Contents

- **Patient advocacy groups**
- IRBs
- Health system administrators

Agenda for patient/patient advocacy focus groups

	Topics covered	Time allocation [min]
A Welcome and moderator introduction	▪ Purpose of focus group and agenda for the session ▪ How/why participants were chosen ▪ Role of moderator (e.g., time management)	5
B Participant introduction	▪ Names of participants and their patient advocacy groups ▪ Number of members with EA/CU experience	10
C Structured discussion around potential pain points	▪ Hypothesized pain points across identification, application, and treatment ▪ Role of advocacy group in EA/CU programs	35
D Open discussion on successes/challenges of existing programs	▪ Additional challenges and successes ▪ Suggestions for improvement ▪ Approach to educating potential patients about EA/CU	25
E General feedback questions and wrap-up	▪ Likelihood of recommending the program to someone else ▪ Other feedback not previously covered ▪ Favorability of right to try legislation	15
Total		**90**

A Welcome and moderator introduction

Moderator instructions

This guide will be used to manage time effectively and ensure coverage of certain topics of conversation. Participants should freely discuss topics presented by the moderator, rather than following the question/answer cadence of an interview. Due to time restrictions, it is possible that not every question will be asked, and it is the moderator's responsibility to decide when to transition to a new topic. Additionally, the moderator should adapt questions based on the composition of each group and the group's prior responses. "Voting" sections should be completed using Mentimeter, and time estimates for these questions are included in this guide.

A Welcome and moderator introduction

Moderator introduction script

Hi, my name is [fill in name], and I will be leading our discussion today on the FDA's Expanded Access and Compassionate Use programs for drugs/biologics and devices, respectively.

I'd like to start by thanking each of you for your participation in this session, which will last 90 minutes. The input from this session will be reported back to FDA in aggregate to help improve EA/CU programs, but all responses will be anonymized. This session will not be recorded in any form, but I will take notes, as will [fill in name] in the back of the room.

Each of you represents a patient advocacy group with some degree of experience with EA/CU. During this session, I will pose questions to the group and allow time for open discussion. There are no right or wrong answers, and I would encourage everyone to share their honest opinions.

To start the conversation, I'll give everyone a chance to introduce themselves. Then, we'll transition to discussing specific challenges and successes with the existing EA/CU programs. Lastly, I'll leave time for general feedback not covered earlier in the session.

As moderator, it is my job to actively manage the time and make sure that we cover all of our desired topics. Please do not be alarmed if I stop an on-going discussion in order to move to a new topic.

B Participant introduction

Prioritized questions in **bold**

Participant introduction script	To begin with, in order to facilitate good dialogue amongst ourselves, can everyone please say their name, the group they represent, and how they first learned about EA/CU programs? *Wait for everyone to introduce themselves. Allow no more than one minute per participant.*
Participant introduction script	Thank you for those introductions. Now, I'll ask you to use the iPad in front of you to answer a question about your patient advocacy group's experience with EA/CU programs. I'll give everyone a minute to respond to the question, and please let me know if you're having any difficulty with the device. *Wait one minute.*
Demographics (voting)	1**) How many members of your patient advocacy group or community have sought or are seeking access to treatment through the FDA's Expanded Access (EA) or Compassionate Use (CU) programs?** ☐ None ☐ 1-2 ☐ 3-5 ☐ 6-8 ☐ >8 ☐ N/A ☐ Don't know

C Structured discussion around potential pain points

Prioritized questions in **bold**

Pain point script	In this part of the conversation, we'll break the EA/CU application process into 3 parts. In the first, identification, an EA/CU program is deemed appropriate for a patient by their doctor, and the patient must decide whether to proceed. I'll start by asking:
Identification	**1) What do you think about EA/CU programs overall, and what do you tell members of your patient advocacy group or community about them?** 2) Do you connect members of your patient advocacy group or community to resources to better educate them about these programs? 3) What would members of your patient advocacy group or community consider in deciding whether to seek treatment through an EA/CU program, and who would they consult for guidance? **4) What main challenges would be encountered in making this decision?** ***Can give examples if responses are going in a different direction than desired: high stress, short timeline, lack of information, finding investigational products***

C Structured discussion around potential pain points

Prioritized questions in **bold**

Pain point script	Next, we'll talk about the application process, in which access to an investigational drug, biologic, or device is sought from a manufacturer and documentation is submitted to regulatory bodies. To begin with, I'd like to know:
Application	**5) How well have members of your patient advocacy group or community understood the EA/CU application process, who needs to approve their requests in order to begin treatment, which forms are required, and where these forms should be submitted?** 6) Do you communicate directly with manufacturers, healthcare providers, IRBs, health insurance companies, or the FDA on behalf of members of your patient advocacy group or community? What causes you to engage with these parties, and how easy or challenging are these interactions? **7) What other roles, if any, does your group play in the application process?** **8) On average, how satisfied are members of your patient advocacy group or community with the EA/CU application process?**
Pain point script	Lastly, we'll talk about the treatment of patients under EA/CU programs. We are especially interested in understanding how much financial hardship patients endure as a result of treatment through EA/CU programs. To begin with:
Treatment	**9) To your knowledge, have members of your patient advocacy group or community needed to pay out of pocket for treatment under EA/CU programs?** 10) In general, how challenging is it for members of your patient advocacy group or community to determine whether health insurance covers some or all of their EA/CU treatment? Does your patient advocacy group ever communicate directly with health insurance companies on behalf of your members?

D Open discussion on successes/challenges of existing programs

Prioritized questions in **bold**

Open discussion script	Now that we've covered specific difficulties associated with existing EA/CU programs, I'd like to ask the group some more open-ended questions to fill in any gaps that we may have missed. *As time allows, ask participants to brainstorm answers to each question for one minute separately before discussing as a group.*
Challenges	1) Beyond the challenges that we have already discussed, what are the top 1-2 challenges that members of your patient advocacy group or community face with EA/CU programs?
Suggestions	**2) Based on these challenges and those described earlier, what suggestions do you have for improvement?**
Successes	**3) Thinking about members of your patient advocacy group or community, are there parts of existing EA/CU programs that function especially smoothly that you would not want to see changed?**
Education	**4) What can be done to better educate members of your patient advocacy group or community about the existence of these programs and how to best navigate the application process?**

E General feedback and wrap-up

Prioritized questions in **bold**

General feedback script	Finally, I'd like to gauge the group's overall satisfaction with existing EA/CU programs and cover any general feedback that we've missed to this point:
Likelihood of use	**1) If medically appropriate, would you recommend treatment through the FDA's Expanded Access and Compassionate Use programs to a member of your patient advocacy group or community? To a friend or family member? Why or why not?**
Right to try	2) "Right to try" laws enable patients to try experimental therapies that have completed Phase I testing without soliciting FDA authorization. Would members of your patient advocacy group or community be in favor of right to try legislation?
General feedback	**3) What other feedback or suggestions for improvement would you like to share?** ***Save no more than five minutes for this question.***
Wrap-up script	Again, I want to thank everyone for taking the time to participate in this session. Your input is incredibly valuable in informing our assessment of EA/CU programs.

Contents

- Patient advocacy groups
- **IRBs**
- Health system administrators

Agenda for IRB focus groups

	Topics covered	Time allocation [min]
A Welcome and moderator introduction	▪ Purpose of focus group and agenda for the session ▪ How/why participants were chosen ▪ Role of moderator (e.g., time management)	5
B Participant introduction	▪ Names of participants and their IRBs ▪ How participants first learned about EA/CU programs ▪ Frequency with which IRB deals with EA/CU requests	10
C Structured discussion around potential pain points	▪ Hypothesized pain points across identification, application, and follow-up ▪ Role of IRB in EA/CU programs	35
D Open discussion on successes/challenges of existing programs	▪ Additional challenges and successes ▪ Suggestions for improvement ▪ Approach to educating stakeholders about EA/CU	25
E General feedback questions and wrap-up	▪ Likelihood of recommending the program to someone else ▪ Other feedback not previously covered ▪ Favorability of right to try legislation	15
Total		**90**

A Welcome and moderator introduction

Moderator instructions

This guide will be used to manage time effectively and ensure coverage of certain topics of conversation. Participants should freely discuss topics presented by the moderator, rather than following the question/answer cadence of an interview. Due to time restrictions, it is possible that not every question will be asked, and it is the moderator's responsibility to decide when to transition to a new topic. Additionally, the moderator should adapt questions based on the composition of each group and the group's prior responses. "Voting" sections should be completed using Mentimeter, and time estimates for these questions are included in this guide.

A Welcome and moderator introduction

Moderator introduction script

Hi, my name is [fill in name], and I will be leading our discussion today on the FDA's Expanded Access and Compassionate Use programs for drugs/biologics and devices, respectively.

I'd like to start by thanking each of you for your participation in this session, which will last 90 minutes. The input from this session will be reported back to FDA in aggregate to help improve EA/CU programs, but all responses will be anonymized. This session will not be recorded in any form, but I will take notes, as will [fill in name] in the back of the room.

Each of you represents an institutional review board with some degree of experience with EA/CU. During this session, I will pose questions to the group and allow time for open discussion. There are no right or wrong answers, and I would encourage everyone to share their honest opinions.

To start the conversation, I'll give everyone a chance to introduce themselves. Then, we'll transition to discussing specific challenges and successes with the existing EA/CU programs. Lastly, I'll leave time for general feedback not covered earlier in the session.

As moderator, it is my job to actively manage the time and make sure that we cover all of our desired topics. Please do not be alarmed if I stop an on-going discussion in order to move to a new topic.

B Participant introduction

Prioritized questions in **bold**

Participant introduction script

To begin with, in order to facilitate good dialogue amongst ourselves, can everyone please say their name, the IRB they represent, and how they first learned about EA/CU programs?

Wait for everyone to introduce themselves. Allow no more than one minute per participant.

Participant introduction script

Thank you for those introductions. Now, I'll ask you to use the iPad in front of you to answer a question about how often your IRB handles EA/CU requests. I'll give everyone a minute to respond to the question, and please let me know if you're having any difficulty with the device.

Wait one minute.

Demographics (voting)

1) How often does your IRB receive Expanded Access (EA) / Compassionate Use (CU) requests?

- ☐ Never
- ☐ Rarely (once or twice in the past few years)
- ☐ Occasionally (one or twice per year)
- ☐ Frequently (multiple times per quarter)
- ☐ N/A
- ☐ Don't know

C Structured discussion around potential pain points

Prioritized questions in **bold**

Pain point script	In this part of the conversation, we'll look at 3 parts of the EA/CU application process. In the first, identification, an EA/CU program is deemed appropriate for a patient by their doctor, and the patient must decide whether to proceed. I'll start by asking:
Identification	**1) How easy or challenging are your interactions (if any) with healthcare providers and patients prior to receiving an EA/CU application?** 2) Would you feel prepared and empowered to provide guidance on IRB requirements at this early stage? Why or why not?

C Structured discussion around potential pain points

Prioritized questions in **bold**

Pain point script

Next, we'll talk about the application process, in which access to an investigational drug, biologic, or device is sought from a manufacturer and documentation is submitted to regulatory bodies. Please start by using the iPad in front of you to answer a second voting question, which asks you to rate the importance of a list of factors (from 1-10) in determining whether or not to approve an application for treatment through an EA/CU program. I'll give everyone a couple of minutes to respond to the question, and please let me know if you're having any difficulty with the device. *Wait three minutes.*

Application (voting)

3) How relevant would each of these factors be in deciding whether to approve an application for treatment through an EA/CU program? Please rate from 1-10 with 1 being completely insignificant and 10 being extremely significant.

1	2	3	4	5	6	7	8	9	10	N/A	Don't know	
○	○	○	○	○	○	○	○	○	○	○	○	Product / device safety
○	○	○	○	○	○	○	○	○	○	○	○	Product / device efficacy
○	○	○	○	○	○	○	○	○	○	○	○	Risk / benefit analysis of using investigational product
○	○	○	○	○	○	○	○	○	○	○	○	Sufficiency of informed consent, including awareness of out of pocket costs
○	○	○	○	○	○	○	○	○	○	○	○	Prior experience with the sponsor
○	○	○	○	○	○	○	○	○	○	○	○	Emergent / non-emergent nature of the request
○	○	○	○	○	○	○	○	○	○	○	○	Patient population (e.g., adult vs. pediatric vs. elderly)
○	○	○	○	○	○	○	○	○	○	○	○	Proposed monitoring protocol
○	○	○	○	○	○	○	○	○	○	○	○	Data to be collected

C Structured discussion around potential pain points

Prioritized questions in **bold**

Pain point script	Thank you for answering that question. Continuing with the application process:
Application	**4) What is your standard turnaround time on EA/CU applications, and what challenges does this timeframe impose? Do you have special procedures in place to handle EA/CU requests?** **5) As part of FDA's effort to simplify the EA/CU application process, only one member of an IRB, either the chair or another appropriate person, now needs to approve a healthcare provider's use of an investigational product to treat a patient. How has this change in regulation impacted the manner in which your IRB reviews EA/CU applications?** 6) Are members of your IRB in favor of this change in regulation? Why or why not? **7) What would cause your IRB to take longer than the standard amount of time to approve a request? To not approve a request?** **8) How easy or challenging are your interactions with healthcare providers while processing EA/CU applications?** 9) Does your IRB ever speak with other IRBs about how they handle EA/CU requests? Why or why not?
Pain point script	Lastly, we'll talk about follow-up after patients have begun treatment under EA/CU programs:
Follow-up	10) How easy or challenging are your interactions (if any) with patients, healthcare providers, and the FDA after treatment has begun?

D Open discussion on successes/challenges of existing programs

Prioritized questions in **bold**

Open discussion script	Now that we've covered specific difficulties associated with existing EA/CU programs, I'd like to ask the group some more open-ended questions to fill in any gaps that we may have missed. *As time allows, ask participants to brainstorm answers to each question for one minute separately before discussing as a group.*
Challenges	1) Beyond the challenges that we have already discussed, what are the top 1-2 challenges that your IRB faces with EA/CU programs? ? Can you identify any bottlenecks in the approval process?
Suggestions	**2) Based on these challenges and those described earlier, what suggestions do you have for improvement?**
Successes	**3) Reflecting on your experience, are there parts of existing EA/CU programs that function especially smoothly that you would not want to see changed?**
Education	**4) What can be done to better educate IRBs about EA/CU programs and how to best handle requests? Who else should education efforts target?**

E General feedback and wrap-up

Prioritized questions in **bold**

General feedback script	Finally, I'd like to gauge the group's overall satisfaction with existing EA/CU programs and cover any general feedback that we've missed to this point:
Likelihood of use	**1) If medically appropriate, would you recommend treatment through the FDA's Expanded Access and Compassionate Use programs to a healthcare provider with whom you've worked? To a friend or family member? Why or why not?**
Right to try	2) "Right to try" laws enable patients to try experimental therapies that have completed Phase I testing without soliciting FDA authorization. Would you be in favor of right to try legislation?
General feedback	**3) What other feedback or suggestions for improvement would you like to share?** ***Save no more than five minutes for this question.***
Wrap-up script	Again, I want to thank everyone for taking the time to participate in this session. Your input is incredibly valuable in informing our assessment of EA/CU programs.

Contents

- Patient advocacy groups
- IRBs
- **Health system administrators**

Agenda for health systems focus groups

	Topics covered	Time allocation [min]
A Welcome and moderator introduction	▪ Purpose of focus group and agenda for the session ▪ How/why participants were chosen ▪ Role of moderator (e.g., time management)	5
B Participant introduction	▪ Names of participants and their health systems ▪ How participants first learned about EA/CU programs ▪ Frequency with which health system deals with EA/CU requests	10
C Structured discussion around potential pain points	▪ Hypothesized pain points across identification and treatment ▪ Role of health system in EA/CU programs	35
D Open discussion on successes/challenges of existing programs	▪ Additional challenges and successes ▪ Suggestions for improvement ▪ Approach to educating health system admins about EA/CU	25
E General feedback questions and wrap-up	▪ Likelihood of recommending the program to someone else ▪ Other feedback not previously covered ▪ Favorability of right to try legislation	15
Total		**90**

A Welcome and moderator introduction

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A Welcome and moderator introduction

Moderator introduction script

Hi, my name is [fill in name], and I will be leading our discussion today on the FDA's Expanded Access and Compassionate Use programs for drugs/biologics and devices, respectively.

I'd like to start by thanking each of you for your participation in this session, which will last 90 minutes. The input from this session will be reported back to FDA in aggregate to help improve EA/CU programs, but all responses will be anonymized. This session will not be recorded in any form, but I will take notes, as will [fill in name] in the back of the room.

Each of you represents a health system with some degree of experience with EA/CU. During this session, I will pose questions to the group and allow time for open discussion. There are no right or wrong answers, and I would encourage everyone to share their honest opinions.

To start the conversation, I'll give everyone a chance to introduce themselves. Then, we'll transition to discussing specific challenges and successes with the existing EA/CU programs. Lastly, I'll leave time for general feedback not covered earlier in the session.

As moderator, it is my job to actively manage the time and make sure that we cover all of our desired topics. Please do not be alarmed if I stop an on-going discussion in order to move to a new topic.

B Participant introduction

Prioritized questions in **bold**

Participant introduction script	To begin with, in order to facilitate good dialogue amongst ourselves, can everyone please say their name, the health system they represent, and how they first learned about EA/CU programs? *Wait for everyone to introduce themselves. Allow no more than one minute per participant.*
Participant introduction script	Thank you for those introductions. Now, I'll ask you to use the iPad in front of you to answer a question about how often your health system handles EA/CU requests. I'll give everyone a minute to respond to the question, and please let me know if you're having any difficulty with the device. *Wait one minute.*
Demographics (voting)	**1) How often does your health system handle Expanded Access (EA) / Compassionate Use (CU) requests?** ☐ Never ☐ Rarely (once or twice in the past few years) ☐ Occasionally (one or twice per year) ☐ Frequently (multiple times per quarter) ☐ N/A ☐ Don't know

C Structured discussion around potential pain points

Pain point script

In this part of the conversation, we'll focus on two parts of the EA/CU application process. In the first, identification, an EA/CU program is deemed appropriate for a patient by their doctor, and the patient must decide whether to proceed. Please start by using the iPad in front of you to answer a second voting question, which asks you to rate the importance of a list of factors (from 1-10) in evaluating whether or not a provider in your health system should apply to treat a patient through an EA/CU program. I'll give everyone a couple of minutes to respond to the question, and please let me know if you're having any difficulty with the device. *Wait three minutes.*

C Structured discussion around potential pain points

Prioritized questions in **bold**

Identification (voting)

1) How relevant would each of these factors be in evaluating whether a provider in your health system should apply to treat a patient through an EA/CU program? Please rate from 1-10 with 1 being completely insignificant and 10 being extremely significant.

1	2	3	4	5	6	7	8	9	10	N/A	Don't know	
○	○	○	○	○	○	○	○	○	○	○	○	Product / device safety
○	○	○	○	○	○	○	○	○	○	○	○	Product / device efficacy
○	○	○	○	○	○	○	○	○	○	○	○	Sufficiency of informed consent
○	○	○	○	○	○	○	○	○	○	○	○	Effect on hospital reputation, including potential media backlash
○	○	○	○	○	○	○	○	○	○	○	○	Cost to health system, including but not limited to medical supplies and staff time
○	○	○	○	○	○	○	○	○	○	○	○	Provider's experience with EA/CU
○	○	○	○	○	○	○	○	○	○	○	○	Provider's experience with other clinical trials
○	○	○	○	○	○	○	○	○	○	○	○	Provider's reputation within health system
○	○	○	○	○	○	○	○	○	○	○	○	Health system's experience with EA/CU
○	○	○	○	○	○	○	○	○	○	○	○	Whether health system is seen as a leader in the therapeutic area of the EA/CU request
○	○	○	○	○	○	○	○	○	○	○	○	Legal risk

C Structured discussion around potential pain points

Prioritized questions in **bold**

Pain point script	Thank you for answering that question. Continuing with our discussion of the identification step:
Identification	2) In general, what resources does your health system grant to providers dealing with especially challenging cases, and what is the process for handling these cases? Does your health system convene groups of healthcare providers to facilitate discussion (e.g., tumor boards)? **3) What resources (e.g., educational materials) does your health system grant to providers considering EA/CU programs?** **4) What procedures and resources (if any) does your health system have in place to enable patients to be treated through an EA/CU program?** **5) How closely do health system administrators work with providers who are interested in applying for one of these programs? What role(s) do they play?** 6) Do you feel prepared and empowered to engage with other relevant stakeholders (including IRBs) at this stage? Why or why not?
Pain point script	Secondly, we'll talk about treatment of patients under EA/CU programs:
Treatment	7) Once treatment through an EA/CU program has begun, how prepared is your health system to deal with adverse events related to treatment? **8) What costs does your health system incur from administering treatments through an EA/CU programs, and how significant are these costs?**

D Open discussion on successes/challenges of existing programs

Prioritized questions in **bold**

Open discussion script	Now that we've covered specific difficulties associated with existing EA/CU programs, I'd like to ask the group some more open-ended questions to fill in any gaps that we may have missed. *As time allows, ask participants to brainstorm answers to each question for one minute separately before discussing as a group.*
Challenges	1) Beyond the challenges that we have already discussed, what are the top 1-2 challenges that your health system faces with EA/CU programs?
Suggestions	**2) Based on these challenges and those described earlier, what suggestions do you have for improvement?**
Successes	**3) Reflecting on your experience, are there parts of existing EA/CU programs that function especially smoothly that you would not want to see changed?**
Education	**4) What can be done to better educate health system administrators about the existence of these programs and how to best approach requests within their health system? Who else should education efforts target?**

E General feedback and wrap-up

Prioritized questions in **bold**

General feedback script	Finally, I'd like to gauge the group's overall satisfaction with existing EA/CU programs and cover any general feedback that we've missed to this point:
Likelihood of use	**1) If medically appropriate, would you recommend treatment through the FDA's Expanded Access and Compassionate Use programs to a provider in your health system? To a friend or family member? Why or why not?**
Right to try	2) "Right to try" laws enable patients to try experimental therapies that have completed Phase I testing without soliciting FDA authorization. Would you be in favor of right to try legislation?
General feedback	**3) What other feedback or suggestions for improvement would you like to share?** ***Save no more than five minutes for this question.***
Wrap-up script	Again, I want to thank everyone for taking the time to participate in this session. Your input is incredibly valuable in informing our assessment of EA/CU programs.