

Stakeholder Interview guide for FDA Expanded Access and Compassionate Use Programs

PRA COMPLIANCE

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INTRODUCTION

In this interview guide, we are probing stakeholders' understanding of and experiences with the FDA's Expanded Access (EA) program for drugs and biologics and Compassionate Use (CU) program for devices, including perceived strengths and weaknesses, overall satisfaction, pain points, suggestions for improvement, and information availability. Though specific stakeholders are mentioned, this guide is intended to be flexible enough to facilitate discussions with parties not explicitly mentioned in this document, and questions may be adapted based on interviewees' experiences and the flow of the conversation.

For interviewees that have little or no experience with EA/CU programs, questions may be posed as hypotheticals, drawing on the participants' other relevant experiences. No participant will be asked every question below; Lower priority questions are identified in *grey, italic text*. The interviewer will use these questions as a guide and target areas relevant to the interview participant.

QUESTIONS**General knowledge (all)**

- How familiar are you with EA/CU programs? How did you first hear about EA/CU programs? *[If unfamiliar, give short explanation]*
- How often do you:
 - Consider submitting an application for treatment through EA/CU programs? Submit applications? (healthcare providers)
 - Receive an application for treatment through EA/CU programs? (IRBs)
 - Have a provider in your health system seek to treat a patient through EA/CU programs? (health systems)
 - Observe a member of your patient advocacy group or community seek treatment through EA/CU programs? (patient advocacy groups)
 - Receive a request for coverage of treatment through EA/CU programs? Are there certain attributes that would affect how often payors receive EA/CU requests (e.g., lives covered, geography, PPO/HMO split, commercial vs. CMS)? (healthcare payors)
 - Receive a request for access to an investigational product for EA/CU programs? (manufacturers)

High-level process evaluation (all)

- Given your understanding of EA/CU programs, what are one or two things that are going well?
- Given your understanding of EA/CU programs, what are one or two things that could be improved? How?
- What is your understanding of the purpose of EA/CU requests, and how effective are these programs at fulfilling this purpose? If medically appropriate, would you recommend that a friend or family member seek treatment through these programs?

Pain points (separated by stakeholder)

Healthcare providers who have used EA/CU programs

- Identification
 - What factors do you consider in evaluating whether EA/CU programs are appropriate for a given patient?
 - Can you think of a specific instance in which you were on the fence about whether a patient should seek treatment through an EA/CU program? What made this case especially challenging to assess?

- Has a patient ever come to you explicitly asking for treatment through an EA/CU program? How did they learn about this option?
- What challenges have you faced in identifying appropriate investigational products for patients?
- How does offering treatment through EA/CU programs impact a healthcare provider's image in the medical community (e.g., as an expert/leader)?
- *How do you approach the ethical concerns of the EA/CU program?*
- *How does the potential for (social) media attention impact your willingness to provide treatment through EA/CU programs?*

■ Application

- How did you learn how to navigate the application process for EA/CU programs? How well defined are your roles and responsibilities?
- How clear are the requirements for completing the application process for EA/CU programs? How much of an administrative burden do these requirements pose?
- How easy or challenging are your interactions with manufacturers while requesting access to investigational products for EA/CU programs? Are the standards for gaining access to an investigational drug, biologic, or device well understood?
- Have you ever had a request for access to an investigational drug, biologic, or device rejecting by a manufacturer? Why did that happen?
- Have you ever submitted a request to a manufacturer, gained access to the investigational drug, biologic, or device, and not completed the application process? Why?
- How easy or challenging are your interactions with IRBs and the FDA during the EA/CU application process?
- Have you ever had an application that was not approved by an IRB or the FDA? What happened?

■ Treatment

- What direct costs, if any, do you encounter from your role in EA/CU programs (e.g., medical supplies, cost of drug/biologic/device)?
- What indirect costs, if any, do you encounter from your role in EA/CU programs (e.g., time spent completing paperwork by you and/or your staff)?

- *How does your engagement with EA/CU programs impact your ability to handle the rest of your patient load?*

- Follow-up

- How clear are the requirements for completing documentation during and after treatment through EA/CU programs? How much of an administrative burden do these requirements pose?

Healthcare providers who have not used EA/CU programs

- Identification

- Have you considered using EA/CU programs in the past? Why haven't you used them?
- What factors would you consider in evaluating whether EA/CU programs are appropriate for a given patient?
- What challenges might you face in identifying appropriate investigational products for a specific patient?
- How does offering treatment through EA/CU programs impact a healthcare provider's image in the medical community (e.g., as an expert/leader)?
- If you were interested in finding more information on EA/CU programs, where would you look?

IRBs

- Identification

- How easy or challenging are your interactions (if any) with healthcare providers and/or manufacturers prior to receiving an application for treatment through EA/CU programs?
- Do you ever interact directly with patients and the FDA at this stage?
- Do you feel prepared and empowered to provide guidance on the IRB requirements at this early stage? Why or why not?

- Application

- What is your standard turnaround time on an EA/CU application? Does it vary by request type? Do you have any special procedures in place to handle these requests?

- 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?
- Are you in favor of this regulatory change? Why or why not?
- How did you learn the rules and requirements for reviewing EA/CU applications?
- What factors do you consider when evaluating an application for treatment through EA/CU programs?
- Can you think of a specific instance when you were on the fence about whether to approve an application? What made this case especially challenging to assess?
- What would cause you to not approve an application? Can you give an example? How do you work with the investigator in that case?
- How easy or challenging are your interactions (if any) with the following parties while reviewing an EA/CU application: Healthcare providers? Manufacturers? FDA?
- Can you think of an example in which timing and coordinating interactions with healthcare providers, manufacturers, or the FDA slowed down your review process?
- How much of your work is related to EA/CU programs?
- *Does your IRB ever coordinate with other IRBs on the same study or interact with IRBs that have processed SPINDs for the same drug, biologic, or device?*
- *How does your engagement with EA/CU programs compare to other IRB activities?*
- *How do ethical considerations of providing medical products to patients in need affect your review process for EA/CU requests?*
- Follow-up
 - How easy or challenging are your interactions (if any) with the following parties after treatment through an EA/CU program has begun: Healthcare providers? FDA? Manufacturers?

Health Systems

- Identification

- In general, what resources does your health system grant to healthcare providers dealing with especially challenging cases, and what is the process for handling these cases? Does your health system convene groups of providers to facilitate discussion (e.g., tumor boards)?
- What resources do you provide to educate healthcare providers on EA/CU programs?
- How closely do you work with healthcare providers considering EA/CU programs, and what are your roles / responsibilities?
- Do you ever aid healthcare providers in finding investigational products to treat patients through EA/CU programs? How?
- Do you interact directly with IRBs in the context of EA/CU programs? What causes you to engage with IRBs on behalf of your providers, and how easy or challenging are these interactions?
- How does offering treatment through EA/CU programs impact how a health system is perceived in the medical community (e.g., as an expert/leader)?
- *What factors do you consider in evaluating whether your health system should encourage healthcare providers to treat patients through EA/CU programs? Do you consider your health system's ability to manage adverse events that result from treatment?*
 - Have you ever discouraged a provider in your health system from seeking to treat a patient through EA/CU programs? Why?*
- *How do ethical considerations of providing medical products to patients in need affect your willingness to support a healthcare provider seeking to treat a patient through EA/CU programs?*
- *How does the potential for (social) media attention impact your willingness to support a healthcare provider seeking to treat a patient through EA/CU programs?*
- **Treatment**
 - What direct costs, if any, does your health system incur related to EA/CU programs (e.g., medical supplies, cost of drug/biologic/device)?
 - What indirect costs, if any, does your health system incur related to EA/CU programs (e.g., administrative time)

Patient Advocacy Groups

■ Identification

- What do you tell members of your patient advocacy group or community about EA/CU programs? Do you connect these individuals to resources to better educate them about these programs?
- How closely do you work with patients seeking treatment through EA/CU programs?
- 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?
- What challenges do members of your patient advocacy group or community confront when making this decision? Can you think of a time when a member of your patient advocacy group or community was on the fence about seeking treatment through an EA/CU program?
- How does supporting members of your patient advocacy group or community that seek treatment through EA/CU programs affect the perception of your group in the advocacy group community?
- Do you feel obligated to help members of your patient advocacy group or community seek treatment through EA/CU programs, regardless of risk/benefit analysis?

■ Application

- How well do members of your patient advocacy group or community understand the EA/CU application process, including who needs to approve their requests in order to begin treatment, which forms are required, and where these forms need to be submitted? Are there any parts that are especially unclear?
- Do you communicate directly with manufacturers, healthcare providers, 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?
- On average, how satisfied are members of your patient advocacy group or community with the EA/CU application process?

■ Treatment

- To your knowledge, have members of your patient advocacy group or community needed to pay out of pocket for treatment under EA/CU programs?

- 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?

Healthcare payors

■ Overview

- What is your understanding of how payor coverage impacts people's desire and ability to utilize EA/CU programs?
- How have payors typically approached requests for coverage of treatment through EA/CU programs? Does this differ based on the type of payor?
- How does this differ (if at all) from a patient participating in a clinical trial? That is, will a payor cover EA/CU requests if they agree to cover other investigational products in clinical trials?

■ Identification

- How does a payor interact with healthcare providers and patients during the Identification step, as they are trying to understand EA/CU requests but before they decide to go forward?
- What are the most common questions a payor may receive from patients or providers while they are looking for suitable EA/CU options?
- Are there any general policies or practices in place that payors use to guide patients and providers (e.g., well-documented coverage policies online or customer service center scripts)? Does this depend on the type of payor?
- What sorts of interactions would a payor have with other stakeholders (manufacturers, IRBs, hospitals, FDA) at this step?

■ Application

- How does a payor typically interact (if at all) with providers who are filling out applications for EA/CU programs?
- What sorts of interactions would a payor have with other stakeholders (manufacturers, IRBs, hospitals, FDA) at this stage?

■ Treatment

- How does a payor decide whether or not to pay for a treatment as part of an EA/CU program? Is there typically a cap on the policy? Are there characteristics of a payor that would cause this to vary?

- How does a payor decide whether or not to pay for adverse events that may be a result of said treatment? Is there typically a cap on the policy? Are there characteristics of a payor that would cause this to vary?
 - Are there situations in which a payor would decide to cover the initial treatment but not adverse events? Or vice versa?
 - Have you heard of situations in which a patient / provider successfully petitioned to get coverage for an EA/CU therapy, even if not typically covered by a payor? Can you elaborate on this?
 - Even if a payor does not have a blanket policy against covering treatment through EA/CU programs, are there common reasons for denial of coverage for an EA/CU product? How does this compare to requests for other unapproved investigational products?
 - Can you provide an estimate of the direct / indirect costs of treatment through EA/CU programs (either numbers, or relative to “typical” payment for treatment of a disease)? How does this compare to other unapproved investigational products?
- Follow-up
 - Are there specific documentation or follow-up requirements that a payor would (want to) request for a patient / provider who is currently undergoing treatment with an EA/CU product?
 - Would a patient / provider need to submit additional claims (e.g., if they needed to get more of the investigational product)?
- Other
 - What do you think are the biggest misconceptions around the role of healthcare payors in EA/CU programs?
 - What would need to happen to get more payors to consider coverage for EA/CU requests?

Manufacturers

- Identification
 - What is your company’s policy regarding access to investigational products through EA/CU programs, and what factors were considered in setting that policy?
 - Which departments/roles are involved in setting policy?

- How do the FDA's EA/CU programs impact your global approach to granting access to investigational products?
- What sorts of interactions would a manufacturer have with other stakeholders (healthcare payors, healthcare providers, IRBs, hospitals, FDA) at this step, and how easy or challenging are these interactions?
- Application
 - In this section, we'd like to understand how a variety of factors impact your company's ability and willingness to provide investigational products through EA/CU programs. When I read a statement, please describe how this factor affects your company's decision-making process:
 - Ethics of providing medical products to patients in need
 - Risk/benefit analysis for patients
 - Avoiding potential negative press surrounding denial of request
 - Potential opportunity to expand label
 - Risk of hindering ongoing development programs
 - Potentially limiting pool of participants available for current or future clinical trials
 - Limited clinical supply of product
 - Complexity / expense of providing medical product
 - What direct costs, if any, do you encounter from your role in EA/CU programs (e.g., cost of drug/biologic/device)?
 - What indirect costs, if any, do you encounter from your role in EA/CU programs (e.g., administrative time)?
 - Limited internal company resources to process and administer requests
 - Negative data impacting the product's application or changing perception of the product
 - Ambiguity about how resulting data will be used by FDA in regulatory decision-making
 - Which departments/roles are involved in assessing individual EA/CU requests?
 - What sorts of interactions would a manufacturer have with other stakeholders (healthcare payors, healthcare providers, IRBs, hospitals, FDA) at this step, and how easy or challenging are these conversations?

- Treatment and follow-up
 - How clear are the requirements for completing documentation during and after treatment through EA/CU programs? How much of an administrative burden do these requirements pose?

General feedback (all)

- What are the top 1-2 challenges that you've faced with EA/CU programs that we have not already discussed?
- Can you identify any bottlenecks that limit your ability to perform your role and slow down patients' access to investigational products?
- What suggestions do you have to address these problems and/or those discussed earlier in our conversation?
- Which group(s) could benefit most from more education about EA/CU programs?
- Who should provide these educational resources, and how should these resources be disseminated?
- What other feedback would you like to share?

Right to try (all)

- "Right to try" laws enable patients to try experimental therapies that have completed Phase I testing without soliciting FDA authorization. Are you in favor of right to try legislation? Why or why not?