ACCELERATED APPROVAL FOCUS GROUPS MODERATOR'S GUIDE
AS OF 1/15/2021

[SECTION A: INTRODUCTION AND GROUND RULES (5min.)]

Thank you for taking the time to join us today. I am _____ from Fors Marsh Group, a research organization. The purpose of this group is to get your thoughts about how you make treatment choices at the doctor's office. Your feedback is important to us and may ultimately be used to provide guidance to drug manufacturers about the information they give to people like you. Our discussion will last about 60 minutes.

I am an independent consultant hired to moderate these discussions. Therefore, I don't have a vested interest in receiving any particular point of view. I simply want to have an active and lively discussion. Also, I am not an expert about the topics we are going to discuss today. Therefore, you may have questions that I can't answer.

Has everyone checked in with the front desk? Did you receive a copy of the informed consent sheet?

There are a few ground rules I'd like to go over before we begin our discussion.

1. Your participation today is voluntary. If you do not wish to participate, you may stop at any time. The risks associated with participating in this focus group are the same as those you would experience talking in a group of people that you do not know.
2. We do not expect that any of the topics discussed during the focus group will make you uncomfortable or upset you. However, we will talk about difficult situations that some of you may have experienced firsthand, especially if you or a loved one has faced a life-threatening illness. Emotions are a natural part of every discussion, particularly one of this sensitivity. If you feel an emotion rising in you, feel free to express it. Feel free to be upset, feel free to cry, feel free to use swear words if that helps you express yourself, just as long as we are being respectful of each other. Also, keep in mind that you do not have to participate in every discussion if you do not want to.

3. There are no wrong answers in this room, and we are not here to judge each other. Our whole purpose is to hear your perspectives, opinions, and experiences.
4. Anyone can speak out; you don't need to wait for me to call on you. However, we have a lot to talk about and materials to review, so it's important that I hear from everyone and that we discuss all of the topics.
5. What we talk about here is confidential. This means that your name will not be connected with anything you say in our reports.
6. Likewise, we want to respect the privacy of everyone in this room and, therefore, ask that you please not share any of our discussions with others.
7. We are audio and video recording this discussion so that I can give you my full attention and not have to take notes. In addition, I have colleagues listening to our discussion and taking notes so that your opinions are accurately captured. When writing up our findings, we will not include any information that could identify you. Your name, address, and phone number will not be given to anyone, and no one will contact you about this research after this group is over.
8. Speak one at a time and be sure to speak up so that the microphones can catch your voice.
9. Please avoid side conversations—if it's important enough for you to say, I want to hear it.
10. If you disagree with anything that is said, I want to hear about it. Even if you are the only person in the room who feels that way—your opinion represents hundreds of people who are not in this room today.
11. Please set your cell phones to vibrate or turn them completely off. If you need to use the restroom, please feel free to step out and join us once you are done. I just ask that only one of you leave at a time, so we can maintain a lively discussion.
12. In this session, we will be explaining some new concepts. Please feel free to speak up if you are confused about anything that we talk about.

Do you have any questions before we continue?

[SECTION B: ICE BREAKER (5 min.)]

Thanks again for being here. Let's warm up by going around the room and saying your first name and something you like to do in your free time. I'll go first.

[Introductions and Icebreaker]

It’s wonderful to meet you all—let’s get started.

[SECTION C: ACCELERATED APPROVAL EDUCATIONAL PIECE PLUS REACTIONS (15-20 min.)]

Imagine you are talking to your doctor about a serious illness that you have. Your doctor tells you about some treatment options; one of those options is a new prescription drug. Your doctor mentions that this drug received accelerated approval from the Food and Drug Administration.

- What would your initial reaction be?
- What do you know about drug approval generally?
- Regardless of whether you have heard of accelerated approval or not, when you consider the phrase “accelerated approval” what do you imagine it means?
-

Let’s talk more about the process for drug approval.

[GIVE OUT INFORMATION SHEET] Here is an information sheet to help you understand this process. I will also give you a few minutes to review this on your own.

- On one side, you have the standard approval pathway. Once a drug is developed, it is tested in patients to determine the clinical benefits of the drug, such as whether it helps people live longer or feel better. The tests, called clinical trials, also examine the risks associated with the drug. After this process, which can take up to seven years, the FDA may approve the drug for public use.
- On the other side, you have the accelerated approval pathway. This pathway is only available for drugs that treat serious or life threatening conditions – like many cancers or HIV.
    - Here, we also have a new drug being tested in clinical trials. During this testing period, scientists look for an effect on what’s called a surrogate endpoint. A surrogate endpoint is a measure of a drug's effect that may or may not be associated with clinical benefit. An example of a surrogate endpoint for cancer may be measuring a shrinking tumor over a shorter period of time, or determining if blood tests get better. A surrogate endpoint is an early sign that a drug *might* be effective, but it doesn’t mean the drug *will* be effective. At this point in the testing period, the clinical trial has not shown that the drug helps people live longer or feel better, or that it has any other benefits, but it is enough evidence for the FDA to grant the drug a kind of approval for it to

be available to people with the illness. This type of approval is called accelerated approval.

Companies must continue to test the drug and either show that it helps people live longer or makes them feel better. They must also show that the benefits of the drug continue to outweigh the risks. If the drug company can't do that, then the FDA may withdraw approval of the drug. I will let you review this for a couple of minutes.

[GIVE OPPORTUNITY TO ASK QUESTIONS OR CLARIFY ANTHING]

- What are your initial thoughts or reactions? Let me know which parts are not clear.

[GIVE OUT ACCELERATED APPROVAL WORKSHEETS]

- If you were in a position to explain this process to a friend or family member, how would you go about it? I'm going to give you a few minutes to write it down using this worksheet. You can use any one of the prompts on the sheet to help you get started and organize your thoughts.
- Who would like to share what they wrote?

[AFTER PARTICIPANTS HAVE HAD A CHANCE TO SHARE]

- What questions pop into your mind when you hear about this process?
- What are you concerned about when you hear about this process?

Now that we have had a chance to learn more about accelerated approval, let's go back to the situation where you are talking to your doctor about a serious illness that you have. Your doctor gives you a treatment option that is a new prescription drug that received accelerated approval.

- What would you ask your doctor about this treatment?

- What would be important for you to know?

- How would you try to learn about the new drug that your doctor suggests?

- What information would make you decide to try a drug that has received accelerated approval?

- What information would make you decide ***not*** to use the drug?

[SECTION D: STIMULI (10—20 min.)]

I'd like for you to imagine, for a moment, you or someone you know has been diagnosed with cancer and you are interested in finding more information regarding treatment options. You are online and you come across a website of a prescription drug. I'm going to show you some information from this website and ask you to write down your thoughts and reactions to it using these worksheets. After that, we will discuss together your reactions to it. Keep in mind that if this topic is uncomfortable or upsets you, you do not need to participate [OPTION: Just to reiterate, if anything that you see here is confusing I want to know about it.]

[PASS OUT MOCK ACCELERATED APPROVAL STATEMENTSTATEMENTS (**STATEMENT A** AND **STATEMENT B**) ONE AT A TIME WITH A WORKSHEET SECTION FOR EACH. HAVE PARTICIPANTS PASS BACK THE **STATEMENT A** WORKSHEET BEFORE PASSING OUT THE **STATEMENT B** WORKSHEET.THEN, PASS BOTH STATEMENTS OUT AGAIN AND DISCUSS TOGETHER.]

STATEMENT A

This use of FLOVET is based on an improvement in the percentage of patients whose cancer shrinks or disappears after treatment. Currently, no data have shown whether treatment with FLOVET prior to surgery improves survival.

- What do you understand from this statement? [If it is difficult to elicit a response, probe sentence by sentence.]
    - What does an "improvement in the percentage of patients whose cancer shrinks or disappears after treatment" mean to you?
    - What does it mean to say that there is no data about how this drug improves survival?
- What do you find confusing about this statement?
- What is missing from this statement? What questions do you have that don't seem to be answered?
- What do you think are the likely benefits of this drug?
    - What about the risks of taking it?
- Would you say that taking this drug would likely help you or harm you? [Probe—ask about the specific parts of the statement that make them believe that].

[COLLECT "VERSION A" STATEMENT AND WORKSHEETS]

STATEMENT B

Look at this statement.

The effectiveness of SUNOX is based on a study that measured two types of responses to treatment (response rate and duration of response). There is no clinical information available to show if patients who are treated with SUNOX live longer or if their symptoms improve. There are ongoing studies to find out how SUNOX works over a longer period of time.

- What does the information written here mean to you?
    - What are some reasonable interpretations?
    - How would you put it in your own words?
- What about this information is confusing?
    - Does it make you wonder about anything?
    - Is there anything that is explained badly?
    - What, if anything, do you feel is explained well?

- Would reading this information make you more or less likely to decide to use this drug? How so?
- How would this information help you make a decision about whether to take this drug?
    - What are the benefits of taking this drug? What risks might you face?
    - What information is not helpful?
- Is there anything more you want to know?

[AFTER 'STATEMENT B' CONVERSATION; PARTICIPANTS HAVE SEEN ALL STATEMENTS. PASS OUT BOTH STATEMENT A AND STATEMENT B]
Now, considering both of these statements:

- Looking back at these statements, is there a difference between these two types of statements?
    - If yes, what is different? What makes them different?
    - If no, how so?

[SECTION F: CLOSING (2–5 min.)]
[FALSE CLOSE—TIME PERMITTING] If you don't mind, I am going to step out for just a moment to see if my team has any additional follow-up questions for you. [Ask any additional questions.]

[GIVE MORE INFORMATION ABOUT THE PURPOSE OF THE FOCUS GROUPS] These conversations will help us understand what information people need to make important health and treatment decisions for themselves and their family. We are looking to see how we can present this information so that it's clear and people can feel confident and informed in their decisions.

This has been a very helpful session. Thank you so much for taking the time out of your day to be with me and to share your perspectives and experiences. Before we wrap up, is there anything else that you would like to share or that we might have missed?

Again, thank you for your time. You are free to go. Please leave behind your worksheets and writing utensils. Have a wonderful evening!

ACCELERATED APPROVAL WORKSHEET

If a drug received accelerated approval it means it might…	If a drug received accelerated approval it means it might NOT…
_____ _____ _____	_____ _____ _____
Accelerated approval means a drug received approval because…	Accelerated approval is different from standard FDA approval because…
_____ _____ _____	_____ _____ _____
You might want to take a drug that has accelerated approval if…	[intentionally blank]
_____ _____ _____	

STATEMENT A WORKSHEET

This use of FLOVET is based on an improvement in the percentage of patients whose cancer shrinks or disappears after treatment. Currently, no data have shown whether treatment with FLOVET prior to surgery improves survival.

Write down what you understand from this statement:

_____

_____

_____

_____

STATEMENT B WORKSHEET

The effectiveness of SUNOX in these patients is based on a study that measured two types of responses to treatment (response rate and duration of response). There is no clinical information available to show if patients who are treated with SUNOX live longer or if their symptoms improve. There are ongoing studies to find out how SUNOX works over a longer period of time.

Write down what you understand from this statement:

_____

_____

_____

_____