Transcript of FDA Media Availability on FDA Approval of First Drug Comprised of an Active Ingredient Derived from Marijuana to Treat Rare, Severe Forms of Epilepsy

Moderator: Michael Felberbaum
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Coordinator: Welcome and thank you for standing by. At this time all lines have been placed in a listen-only mode until the question-and-answer session. At that time, if you'd like to ask a question, please press star 1.

Today's call is being recorded. If anyone has any objections, you may disconnect at this time. I would now like to turn the call over to Michael Felberbaum. Sir, you may begin.

Michael Felberbaum: Thank you. Good afternoon, and thank you for participating in today's media availability. My name is Michael Felberbaum and I'm with the FDA's Office of Media Affairs.

This is a media briefing regarding the FDA's approval of Epidiolex (cannabidiol oral solution), the first drug that contains a purified drug substance derived from marijuana to treat rare and severe forms of epilepsy. By now the agency's news release and the statement for this announcement have been issued and posted on the FDA's website.

Today I'm joined by FDA Commissioner Dr. Scott Gottlieb and Dr. Douglas Throckmorton, deputy director of regulatory programs for the FDA's Center for Drug Evaluation and Research, who will provide brief remarks and then take questions regarding today's announcement.

Reporters on the phone will be in a listen-only mode until we open up the call for questions. As a reminder, this call is being recorded. When asking a question, please remember to state your name and affiliation. Also, please limit yourself to one question and one follow-up so we can get to as many questions as possible. With that I will now turn the call over to Dr. Gottlieb.

Scott Gottlieb: Thank you, Michael. Good afternoon and thank you all for taking the time to join us today to discuss this important announcement. Today, the FDA approved Epidiolex cannabidiol oral

solution for the treatment of seizures associated with two rare forms of severe epilepsy, Lennox-Gastaut syndrome and Dravet syndrome, in patients two years of age and older.

This drug fills an important unmet need among this community as it's the first FDA approval of a drug for the treatment of patients with (Dravet syndrome). It offers an important additional option for those patients (with Lennox-Gastaut syndrome).

This is an important medical advance as the first FDA-approved drug to contain a purified drug substance derived from marijuana, but it's important to note that this is an approval of one specific CBD medication for a specific use and it was based on a well-controlled clinical trial to evaluate the use of this compound in the treatment of a specific condition.

It's not an approval of marijuana or all of its components. Marijuana remains a Schedule I compound with known risks. Epidiolex is a purified form of cannabidiol or CBD, which is one of more than 80 active chemicals in the cannabis plant, more commonly known as marijuana.

However, CBD does not cause intoxication or euphoria that comes from THC. Because it's a purified and consistently-manufactured drug, this drug is delivered to patients in a reliable dose form and through a reproducible route of delivery.

This is important because it helps to ensure that patients derive the anticipated benefit by receiving the medication in amounts that maximize benefit and minimize risks.

So in addition to taking note of this scientific achievement and the medical advance that this action represents to these patients and their families, we should also reflect on the path that made this possible.

Over the past decade we've seen a growing interest in the development of therapies derived from marijuana and its components. The FDA has been supportive of research in this area for many years and this approval serves as a reminder that advancing sound development programs that properly evaluate active ingredients contained in marijuana can lead to important medical therapies.

It’s a path that’s available to other product developers who want to bring forth marijuana-derived products through appropriate drug development programs, and we encourage sponsors to use it.

Research to demonstrate that marijuana or its components could be safe and effective in the treatment of medical disorders should be held to the same standards as other drug compounds, and certainly it should not be held to a lower standard as some proponents would suggest.

The FDA has an active program to assist drug developers who want to investigate marijuana or its components through properly-controlled clinical trials to demonstrate for the potential for safe and effective uses, and the FDA remains committed to collaborating with federal and state agencies and researchers and with product developers on advancing this type of important and conscientious work.

Dr. Throckmorton will discuss more about our support for research in this area but I wanted to also touch on our continued concerns about the proliferation and illegal marketing of unapproved CBD-containing products with unproven medical claims.

The promotion and use of these unapproved products may keep some patients from accessing an appropriate recognized therapy to treat serious and even fatal diseases. The FDA has taken recent actions against companies distributing unapproved CBD products claiming to treat or cure serious diseases such as cancer with no scientific evidence to support these claims.

We’ll continue to take action when we see the illegal marketing of CBD-containing products with unproven medical claims. We remain committed to our gold standard for product development and review and such a process ensures that any new therapies for marijuana and its constituents are safe and effective for patients and are manufactured to a high and consistent quality.

I’ll turn the call over to Dr. Throckmorton to discuss more about FDA’s work to support research on the therapeutic effects of marijuana and its components and I appreciate you all joining us today. Doug?

Douglas Throckmorton: Thank you, Dr. Gottlieb. Before a high-quality drug can be developed, evaluated and eventually approved by the FDA, it's critical that the necessary work be done to identify drugs with potential medical benefit and conduct the rigorous scientific research through adequate and well-controlled clinical trials.

This is true for all drugs, including ones derived from plant materials like marijuana. This research process – from early development through preclinical and clinical research – gives us a comprehensive understanding of the new drug.

This includes an understanding of whether the new product is safe and effective for treating a particular medical condition, what the proper dose is and what the population is that it's safe and effective for, as well as how the new compound could interact with other drugs and whether the new drug has side effects or safety concerns we've identified.

This work also helps product developers identify the appropriate dosage needed to achieve the desired therapeutic effect while minimizing toxicity and risk. Taken into totality, the scientific evidence generated by these studies forms the basis for the FDA's evaluation of benefits versus risk.

And it's because of this careful scientific and evidence-based evaluation by the FDA that health care providers can rely on having a quality product that delivers a consistent, uniform dose of an effective medication that is able to deliver predictable treatments to patients.

This is especially important when considering treatments for serious medical conditions that will be utilized in the clinical care of patients who may have any number of other health vulnerabilities.

The purified form of the drug CBD approved today by the FDA has been shown to meet these rigorous standards and this action demonstrates our commitment to the scientific process in working with product developers to bring marijuana-based products to market.

Research on the therapeutic effects of marijuana and its components involve a number of federal agencies in addition to the FDA, including the National Institute on Drug Abuse, part of the National Institutes of Health, and the Drug Enforcement Administration.

The FDA has taken several specific steps to support research of this kind. First, we meet regularly with researchers as they plan and carry out their trials. We have also formed a botanicals team to provide scientific expertise on botanical issues for researchers developing drugs derived from plants such as marijuana.

That team has published guidance to industry on clinical studies involving botanical plants as well as quality controls for lot-to-lot consistency. In recent years, the agency has also recommended to the DEA the approval of several hundred Schedule I research protocol licenses for research on marijuana or its constituent compounds.

Additionally, the FDA also works with companies to provide patients access to experimental therapies while clinical trials are ongoing through expanded access provisions.

These approaches help protect patients while also allowing for the collection of data necessary to support the FDA approval of safe and effective therapies for use in the broader population. And through this process hundreds of children were able to get access to investigational CBD product while this product was under investigation.

Drugs derived from marijuana are also eligible for several programs that are intended to facilitate and expedite development and review of new drugs that address unmet medical needs in the treatment of serious or life-threatening conditions.

Much of the work we've done to encourage research in this area has led to the approval action we took today. With that, we're happy to answer any questions we can.

Michael Felberbaum: Thank you. At this time we'll begin the question-and-answer portion of the briefing. As a reminder, this call is being recorded. When asking a question, please state your name and affiliation. Also please limit yourself to one question and one follow-up so we can get to as many questions as possible. Operator, we'll take the first question.

Coordinator: Thank you. At this time if you'd like to ask a question, please press star 1 and please record your name when prompted. If you'd like to withdraw the request, you may press star 2. Again to ask a question, please press star 1. One moment, please, for the first question. Thank you and our first question comes from Andrew Joseph and please state your affiliation.

Andrew Joseph: Hi, yes, it's Andrew at STAT, thanks. There's some concern from some patients and advocates that the FDA or senior state policies as well around CBD will in effect start taking away these products that they've been relying on at this point, which are unregulated, but they still use them and, I guess, what would you say to them and what should they do if they've been relying on these products for like self-treatment?

Douglas Throckmorton: With the approval of this product today, I hope patients' conversations with their physicians about whether this product provides them the treatment that they're looking for from those other unapproved products. As we said, those products aren't able to have evidence of safety and effectiveness and we're concerned about the lot-to-lot variability that may be seen with them and we think that an approved product using the FDA system is much preferable if it's available and appropriate for them.

Andrew Joseph: And then just following up on that, I think in that path FDA's enforcement actions about CBD have been limited to warning letters. Do you anticipate a broader or more aggressive crackdown at all?

Scott Gottlieb: Well, warning letters, this is Scott, a warning letter is the first step typically in FDA taking enforcement actions. You know, depending on how a sponsor reacts in response to a warning letter that we issue, we could follow-up with additional actions, including, as you've seen, we've in certain situations in other contexts we've moved to seek injunctions against companies that didn't, you know, didn't take these public health actions that we were requesting in the context of warning letters.

So there's a toolbox of enforcement actions and steps that we can take.

A warning letter is typically the first step that we would take when we have this specific public health concern.

Andrew Joseph: Thank you.

Michael Felberbaum: Thank you, operator, we'll take the next question, please.

Coordinator: Thank you. Your next question comes from Allison Aubrey and please state your affiliation.

Allison Aubrey: Hi, National Public Radio. Quick question, I've spoken to some CBD researchers who are looking into, who have clinical trials underway to look at whether CBD might be an effective treatment for alcohol use disorder combined with PTSD.

They are saying that there are many regulatory hurdles involved in studying CBD because of the classification, the DEA's classification. So I know that obviously marijuana is still a Schedule I drug but is there going to be—is CBD going to be taken-off Schedule I classification?

Douglas Throckmorton: With this action the DEA will need to make a different scheduling decision for CBD and yes, it will be a schedule other than Schedule I because it now has an accepted medical use. It will be in, you know, one of the other schedules. That's for the DEA to ultimately decide and we'll obviously work to provide them any technical assistance that we can.

Allison Aubrey: So that reclassification is underway now?

Douglas Throckmorton: Yes.

Allison Aubrey: Okay, thank you.

Michael Felberbaum: Thank you, operator, we'll take the next question.

Coordinator: Thank you. Our next question comes from Matt Perrone and please state your affiliation.

Matt Perrone: Hi, Associated Press. Thanks. Just real quick, you talk in the statement and you talked on the call about illegal marketing of CBD products. Just to be clear, I mean, the line is here, it becomes illegal when they're making specific disease-specific claims. If someone simply, you know, selling CBD without making a kind of specific health claim that's not an issue, correct?

Douglas Throckmorton: So Matt, this is Doug. There are two ways that products here could raise concerns with regard to marketing and we can really only speak to one of them, which is the Food, Drug and Cosmetics Act, and for us if a product is in violation of that act, mostly by making

the claims that you're talking about, claims regarding you know, treatment of diseases and the like, then we can take enforcement action as Dr. Gottlieb said. We have done so in the past.

There is another statute that regulates controlled substances, you know, the Controlled Substances Act that the DEA regulates and, you know, that would be another mechanism for enforcement but I obviously wouldn't want to comment on it myself.

Matt Perrone: Okay, thanks.

Michael Felberbaum: Thank you, operator, we'll take the next question, please.

Coordinator: Thank you. The next question comes from Pete Loftus and please state your affiliation.

Pete Loftus: Hi, Wall Street Journal. I just wanted to make sure I understood that the, that making claims about – is it making claims about any disease for these unapproved CBD products, that would be illegal or now that there's a version of this approved, could the dispensaries or whatever make claims about their own CBD products treating those specific diseases, these forms of epilepsy?

Douglas Throckmorton: So this approval was specific to this product and it, you know, the claims are regarding the product, Epidiolex. I'm not sure we could talk about the hypothetical about the other products.

Scott Gottlieb: Is your question whether or not the unapproved CBD products being sold that people might find online whether or not if they're making claims, for these specific conditions, whether or not that would instigate FDA enforcement action now that there's an approval?

Pete Loftus: Right.

Scott Gottlieb: I mean, I would say sort of broadly and this is Scott, you know, we have taken some enforcement action as you know against firms that are marketing CBDs for various disorders.

And I think if you look over the scope of the enforcement actions we've taken, you know, we've focused on areas where there's claims being made around you know, CBD for the

treatment of significant health conditions, things like cancer, claims where people are alleging that CBD could be used – I believe one was arguing that CBD could actually promote shrinkage of certain forms of cancer – things that for which there is no reliable evidence and situations where it would obviate you know, potentially otherwise available therapy that could potentially have a positive therapeutic effect.

I think with respect to how we're going to prioritize enforcement going forward, I think we're still going to prioritize it based on a public health assessment where we think that you know, claims are being made around the use of CBD in situations where patients could be put at particularly significant harm because there's otherwise effective available therapy.

For those patients and those patients suffer from a condition for which they wouldn't want to forego active treatment and so that's sort of the broad lens that we're going to continue to look through and we're going to continue to take enforcement actions there as we have been doing.

Pete Loftus: Thanks.

Michael Felberbaum: Thank you, operator, we'll take the next question, please.

Coordinator: Thank you. Our next question comes from Malcolm Spicer and please state your affiliation.

Malcolm Spicer: Thank you very much, Informa BI. This is a question that kind of well certainly carries-on from the previous several questions. You've been very clear about drug claims or disease claims for CBD products that are unapproved.

However, officially a stance on using CBD at all in any food or dietary supplement is prohibited regardless of any claim made by the product – it's not approved, it's not accepted, rather, as a dietary ingredient.

Will this approval and the related change in scheduling the DEA will make that Dr. Throckmorton referred to, will this lead to CBD being okay for use in dietary supplements regarding, you know, of course without any claim?

Douglas Throckmorton: This is Doug. No, I don't believe that it would. I'd refer you to our website where we talk about the basis for the decision to exclude CBD from the dietary supplements and I don't think that this action was going to change any of those conclusions.

Malcolm Spicer: Thanks very much.

Michael Felberbaum: Operator, we have time for one more question, please.

Coordinator: Thank you. Our next question comes from Laurie McGinley and please state your affiliation.

Laurie McGinley: This is Laurie from the Washington Post. Thanks so much, so I just want to make sure I understand this. Currently CBD is illegal under the federal law, correct, but it's available under state law in all those states where marijuana is legal so that's my first question.

My second question is in terms of reclassifying it and I know this is a DEA question, but we're not getting a lot of clarity from them. Is it likely that they would reclassify all CBD or would they say something like CBD as it appears in, you know, this specific product that was just approved or in all FDA-approved products or all CBD?

Douglas Throckmorton: So the second question, I'm not going to be able to comment. You know, exactly what form the rescheduling action DEA takes is you know, we're just going to have to wait and see. Our role is to provide them scientific help to the extent that they ask for it. We certainly have done that, continue to work on that and, you know, hope that this product is available very quickly.

Obviously our goal is to get this product available for the patients that need it as quickly as we possibly can. I'm sorry, you're going to have to ask the first question again.

Laurie McGinley: I just wanted to make sure I understood the current status of CBD, which is it's Schedule I, which means it's banned under federal law but that is available in many states that have legalized marijuana.

Douglas Throckmorton: Yes, so you know, we're certainly aware of the state actions but wouldn't be able to comment on them at all. This action today is going to affect the federal status because it now provides cannabidiol with an accepted medical use and so that requires a rescheduling, a

change from Schedule I to one of the other schedules, and that's what we've been working with the DEA on.

Laurie McGinley: But it's not clear whether it's just this product or all CBDs?

Douglas Throckmorton: Yes, that wouldn't be something I'd be able to answer.

Michael Felberbaum: Thank you. Ladies and gentlemen, this concludes today's media call. A replay will be available in about an hour and will be available for 30 days. Please remember to check the FDA website for the press release and statement. If you have any follow-up questions, please don't hesitate to contact FDA's press office. Thank you.

Coordinator: Thank you. This concludes today's conference. You may disconnect at this time.

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