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2       and they respect it to remain safe in the  
3       future.     They don't want to hear and be  
4       confused by labels or by side effects.   They  
5       don't want their doctors to be confused by  
6       those things. They want to make sure that the  
7       medicine they get is of the proper strength  
8       and that it has been manufactured and stored  
9       correctly.   They want to know that medicine  
10      remain safe in America.

11               The second thing that they know is  
12      that America has been the light of innovation  
13      of the world for cures and they want it to  
14      remain that way.   They don't want to hear the  
15      regulations got in the way of getting a quick  
16      cure brought to market.   They don't want to  
17      know that Congress can't get their act  
18      together to approve funding for the FDA so  
19      that they can do their job.   They don't want  
20      to hear that budget cuts will underfund the  
21      approval process.   They don't want to hear  
22      that an ounce of prevention has prevented a

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1 pound of cure or that that pound of cure now  
2 is going to get in the way and is going to not  
3 be able to stop a ton of expensive treatment.

4 That we really have our priorities in the  
5 right order.

6 The understand that the cost of  
7 heart disease and Alzheimer's and diabetes is  
8 huge and has a huge impact on healthcare  
9 costs, the very costs that people are trying  
10 to come up with a cure for right now. And  
11 they also understand that an effective cure or  
12 at least an enhanced treatment for any of  
13 these diseases could almost solve any of our  
14 healthcare costs in a heartbeat. If we could  
15 have a silver bullet for diabetes, what an  
16 impact that would have.

17 Older Americans look to the FDA to  
18 find the correct balance between safety and  
19 timely approval. In these times of fiscal  
20 accountability, we cannot be penny wise and  
21 pound foolish. Money should not be the impact  
22 or the controller over safety and innovation.

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1 Thank you.

2 MR. FREY: Thank you. Next we have  
3 Lisa Swirsky from Consumers Union.

4 MS. SWIRSKY: Can you hear me? CU  
5 is the nonprofit publisher of Consumer  
6 Reports. We have a longstanding interest in  
7 drug safety and efficacy through our Best Buy  
8 Drugs reports, we are proud to say we bring  
9 vigorous comparative effectiveness safety  
10 information to about 100,000 readers and we  
11 are particularly proud to say we do that for  
12 free.

13 We are gratified that the FDA's  
14 approach to speeding approval timeframes  
15 focuses on boosting submission quality.  
16 Nonetheless, we remain concerned that the  
17 overall focus of the draft comment letter  
18 still emphasizes the completion of approval  
19 processes within set timeframes.

20 While we understand the need for  
21 reasonable goals, the over focus on timeframes  
22 risks overshadowing FDA's primary role

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1 ensuring timely access to safe and effective  
2 drugs not just timely approval.

3 To facilitate improved quality of  
4 submissions, the FDA proposes new pre-  
5 submission meetings. It is important that FDA  
6 meet these new obligations without diverting  
7 attention to resources away from its current  
8 responsibilities. We are concerned that these  
9 safety initiatives such as REMS and Sentinel  
10 get relatively scant attention in the proposal  
11 and argue safety and efficacy should be at the  
12 heart of FDA's proposals and not a secondary  
13 concern.

14 With respect to the REMS process,  
15 we are concerned with a draft's emphasis on  
16 diminishing the burden of REMS process for  
17 industry and for patients. While we support  
18 efforts to make REMS more efficient, it is  
19 important to remember that the overall goal of  
20 REMS is provide access to higher risk drugs in  
21 a way that minimizes the impact of those  
22 risks. Standardization of REMS should not

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1 constrain necessary flexibility to address  
2 risks in a case-specific manner.

3 We also have some concerns about  
4 the agreement's use of the word targeted  
5 surveillance with respect to the scope of the  
6 Sentinel program. When CU first advocated for  
7 Sentinel, it was envisioned as a first-alert  
8 system rather than a follow-up safety system.

9 We hope that the use of the word targeted  
10 does not represent a narrowing of the scope of  
11 the program.

12 We also strongly disagree with some  
13 of the earlier comments about the need to put  
14 conflicted experts on advisory panels. We  
15 fail to see why advisory panels cannot consult  
16 with whatever experts necessary, including  
17 those with financial ties, without having the  
18 conflicted experts actually sit on the panels  
19 themselves.

20 Finally, I would like to reaffirm  
21 some of the disappointment expressed by some  
22 earlier speakers about the things that are not

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1 addressed by the draft agreement, including  
2 stronger oversight of some troubling marketing  
3 practices such as direct-to-consumer  
4 advertising and offering inappropriate  
5 promotion of off-label use.

6 And I also want to reaffirm the  
7 thanks that many other panels expressed to the  
8 FDA for seeking input from a diverse range of  
9 stakeholders. Thank you.

10 MR. FREY: Thank you very much.  
11 Are there any other comments from the room at  
12 this time? Okay, I have got two. Go ahead,  
13 Nancy.

14 MS. MYERS: Thanks. Hi. My name  
15 is Nancy Myers. I'm President of Catalyst  
16 Health Care Consulting but I would like to put  
17 a different hat on as I talk to this group.

18 One of my beloved volunteer  
19 activities is working with a group called the  
20 Alliance for a Stronger FDA. And you all have  
21 heard it a couple times mentioned today.  
22 There were a couple of panelists. We almost

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1 got everybody to mention it on the panel. And  
2 the next time you hear Alliance for a Stronger  
3 FDA, I expect everybody to start a wave.

4 But the Alliance is a nonprofit  
5 organization of 180 members both individuals  
6 and corporate, and consumer groups, patient  
7 groups, that focuses on strengthening the FDA  
8 through appropriations. And I know this  
9 activity is all about user fees but I think it  
10 is very important for maybe not those on the  
11 dais but everybody else who is interested in  
12 this topic. User fees deserve a great deal of  
13 attention in the policies that are being done.

14 But there also is an important responsibility  
15 to make sure that if you have got initiatives  
16 that you want funded or you want FDA to focus  
17 on new initiatives, we really have to make  
18 sure that FDA is adequately funded through  
19 appropriations.

20 So there is a group out there, the  
21 Alliance. It is a nonprofit.  
22 Strengthenfda.org is our website. But if you

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1 are interested in making sure that FDA is  
2 funded well, please join our effort because  
3 the next couple of years are going to be very  
4 difficult to make sure federal agencies are  
5 adequately funded.

6 Thank you.

7 MR. FREY: Okay, over here on my  
8 left.

9 MS. SHERIDAN: Thank you. We are  
10 also a member of the Alliance for a Stronger  
11 FDA so thank you for that.

12 My name is Jennifer Sheridan. I am  
13 the Associate Director for Federal Affairs at  
14 the Alzheimer's Associations.

15 As many of you know, Alzheimer's is  
16 a complicated progressive and fatal disease  
17 that is currently impacting 5.4 million  
18 Americans and by 2050, it will impact nearly  
19 16 million Americans.

20 Insufficient understanding of the  
21 basic biologies of Alzheimer's, lack of  
22 biomarkers, and slow disease progression make

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1 clinical development of innovative treatments  
2 a long and, in many cases, a prohibitively  
3 costly endeavor.

4 I actually will echo a lot of what  
5 has already been said today but the  
6 Association is pleased to see the  
7 recommendations that the FDA will augment the  
8 Agency's capacity to address the growing  
9 number and complexity of biomarker submissions  
10 by increasing the number of staff available  
11 for biomarker qualifications, as well as  
12 training for reviewers. We are also pleased  
13 to see a patient-centered process put forth to  
14 discuss the risk-benefit assessment and look  
15 forward to seeing additional details on how  
16 that process is actually going to work.

17 Moving forward we hope to see a  
18 renewed and continued focus on correcting any  
19 barriers that discourage the aggressive  
20 pursuit of preventive and other pre-  
21 symptomatic treatments for complex diseases  
22 like Alzheimer's and a renewed discussion

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1 about accelerating drug review process for  
2 complex and costly diseases that have serious  
3 unmet medical needs.

4 Thank you for the opportunity.  
5 Thanks.

6 MR. FREY: Thank you. I think we  
7 had a couple others who were interested.  
8 Darby, you want to go ahead?

9 MS. HULL: Hi, I'm Darby with the  
10 Consumer Federation of America. And I don't  
11 want to make a full set of comments but I did  
12 want to reiterate a concern that was raised on  
13 an earlier panel about oversight of foreign  
14 clinical trials. That was one of CFA's  
15 concerns and that was one of the concerns I  
16 think that the Patient Consumer Coalition had.

17 And I did have a question for the  
18 FDA panel and I don't know if you are taking  
19 questions or not. But if you are, I was  
20 wondering if you had any thoughts regarding  
21 the increase of legislation regarding  
22 overregulation. I think that is a theme that

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1 I have seen a lot in Congress lately.

2 DR. MULLIN: Well, there are a lot  
3 of proposals and a lot of discussion going on  
4 on the Hill. And I think that we will look  
5 forward to providing technical assistance when  
6 we have the opportunity to do so for  
7 particular legislation.

8 We think that the current standards  
9 are good ones and are good protective  
10 standards for safety and effectiveness. And  
11 we really think it is very important to have a  
12 timely process, a very rigorous and rapid  
13 process to get safe and effective medicines to  
14 patients as soon as possible.

15 MR. FREY: Any other comments from  
16 the room?

17 MR. VALENTINE: We have one comment  
18 from the webcast.

19 MR. FREY: Great, set him up.

20 MR. VALENTINE: This is from  
21 William Vaughan who is a consumer advocate.  
22 And he has posed a couple of questions to be

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1 included in today's discussion as a public  
2 comment.

3 He states that he thinks that it is  
4 important to consult patients on benefit-risk  
5 but asks will patients and patient groups be  
6 asked to disclose funding and any COI when  
7 they appear before FDA. He asks because many  
8 groups receive large amounts of money from  
9 particular drug sponsors and this could just  
10 institutionalize a new form of lobbying  
11 pressure on the FDA.

12 So he wonders if disclosure and COI  
13 rules need to apply.

14 DR. MULLIN: I think Bill's  
15 question is a good one in being indicative of  
16 I think some complexities that we will be  
17 looking at. There were some comments, a few  
18 people commented, I think Dan Perry and Becca  
19 O'Connor maybe there wasn't a whole lot of  
20 detail in the commitment letter about exactly  
21 how we would be collecting patient input and  
22 incorporating that into our process and that

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1 is because we have a lot of issues and things  
2 to look at and figure out. We want to get  
3 input that is rigorously collected, that is,  
4 as I think Dan said, representative of the  
5 patient population, and that is actually very  
6 rather challenging to do, and information that  
7 is really useable.

8 I know this is not just of interest  
9 to FDA and to you all. The Hastings Center  
10 has indicated that they think this is an  
11 important issue to look at. They look at  
12 ethical issues. So there are a lot of  
13 important questions to look at. And raising  
14 those questions and asking them is helpful to  
15 us as we think through the process because we  
16 want to do it right.

17 If we don't get reliable  
18 information, we won't be able to use it and we  
19 do want to be able to use it.

20 MR. FREY: You said he had a number  
21 of questions. Was that it? Okay. All right.

22 DR. MULLIN: I've asked Jane, who

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1 led the discussions in our PDUFA IV  
2 negotiations related to direct-to-consumer  
3 advertising user fees. And that has come up in  
4 the past. That is not something we have not  
5 considered. So she can give you a recap of  
6 the status of that.

7 MS. AXELRAD: Yes. In the  
8 negotiations over PDUFA IV we had recommended  
9 to Congress and in fact there is actually  
10 language in the statute that would have  
11 provided user fees for the FDA review of DTC  
12 broadcast advertisements. And it was a fairly  
13 elaborate program. Unfortunately, the  
14 Chairman of our Appropriations Committee did  
15 not believe that that program should be funded  
16 by user fees and instead appropriated some  
17 money for the review of broadcast ads.

18 And there is some other language in  
19 the statute that deals with reviews of direct-  
20 to-consumer advertisements and some  
21 authorities in Title IX, I think, to determine  
22 when we want to require review of direct-to-

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1 consumer advertisements.

2 So basically that program was  
3 negotiated as part of the last user fee  
4 reauthorization and because it didn't go  
5 forward, it was not the subject of  
6 negotiations this time around.

7 DR. MULLIN: I just want to make  
8 one more point of clarification. I have been  
9 saving my comments and began taking notes on  
10 what people were saying.

11 There were a couple of folks who  
12 were concerned that we were only going to be  
13 using Sentinel to look at expected risks and I  
14 think that the more likely scenario is that we  
15 get reports of what are actually serious and  
16 unexpected risks. Expected risks are going to  
17 be on the label. But it is the serious and  
18 unexpected risks that we hear about after the  
19 drug is on the market when we are likely to  
20 try to see whether that signal is confirmed by  
21 going and utilizing the Sentinel capability.

22 Although some talked about it as an

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1 active surveillance system going on all the  
2 time collecting and pouring information into  
3 FDA, that was never actually the way we  
4 thought it would get used. That would just  
5 inundate FDA with a lot of false signals. But  
6 structuring the query to see whether the large  
7 body of healthcare data that can be collated  
8 and used to explore a question about a  
9 particular safety risk, which is how we are  
10 envisioning trying to see how well it works  
11 here, is a really effective way to use that  
12 kind of information to see whether that much  
13 larger population can be loaded into a common  
14 data model, and what does it do. Does it  
15 confirm the signal that we are concerned about  
16 where we have preliminary information or does  
17 it not confirm it? So that would be very  
18 valuable to us and that is how we are planning  
19 to use it.

20 It is an adjunct to our passive  
21 surveillance system and other sources of  
22 information that we have to do post-market

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1 safety surveillance.

2 There is one other thing. That was  
3 the MedWatch, the concern about MedWatch data.

4 It is true that the current AERS system,  
5 which we call the legacy AERS system because  
6 we are hoping to make it legacy very soon,  
7 does allow the reporting of information that  
8 is not very standardized and it is not very  
9 easy to analyze.

10 We expect to retire that system  
11 within the coming year and replace it with the  
12 FDA Adverse Event Reporting System, known as  
13 FAERS and the data that will be collected and  
14 entered in FAERS will be using an Individual  
15 Case Safety Report format, ICSR data format,  
16 which will be much more amenable to analysis.  
17 And we expect that data to be much more useful  
18 to us and address the concerns that we were  
19 hearing from some of the panelists today.

20 MR. FREY: All right. The FDA  
21 panel has nothing else. So I think we will  
22 move to wrap up.

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1           A couple of thanks. Thanks to you  
2 all for coming out today for the meeting and  
3 being patient with us. Thank you to the  
4 panelists for their thoughtful comments.

5           And I want to also thank a number  
6 of folks who without their help this meeting  
7 wouldn't have been possible. Andrea Tan, go  
8 ahead and wave. James Valentine is also over  
9 there. Rokhsana Safaai-Jazi, Pat Kuntze and  
10 the staff of the White Oak Conference Center  
11 have been hugely helpful in putting this  
12 meeting on.

13           One last reminder. The FR notice  
14 that announced this meeting, there is a slight  
15 discrepancy in it. October 31st, as I said  
16 earlier, that is the day, the deadline for  
17 comments to the docket. So Halloween is the  
18 day, next Monday.

19           And the *Federal Register* notice  
20 includes instructions for how to submit to the  
21 docket.

22           If there is nothing else, we will

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1 wrap things up. Safe travels home.

2 (Whereupon, at 2:13 p.m., the foregoing  
3 proceeding was adjourned.)

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