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Washington, DC 20044

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Dockets Management Staff (HFA-305) Food and Drug Administration
5630 Fishers Lane, Rm. 1061 Rockville, MD 20852

RE: Docket No. FDA-2024-N-1809:

Listening Session: Optimizing FDA's Use of and Processes for Advisory Committees

About I AM ALS

Founded by Brian Wallach and Sandra Abrevaya in 2019, I AM ALS is a patient-led organization focused on empowering and mobilizing the ALS community. Over the past five years, we have helped create meaningful change, including the elimination of disability waiting periods for those with ALS and the passage of the monumental ACT for ALS. We hope to continue this momentum by amplifying the voices of our community in ways that impact the lives of people living with ALS (pALS), their families, and caretakers.

About ALS

Amyotrophic Lateral Sclerosis (ALS) is a 100% fatal, neurodegenerative disease that has no cure and no significant treatment options. Currently, the average lifespan following an ALS diagnosis is 2-5 years, with the disease impacting motor neurons, causing an individual to lose their ability to be independent. The incidence of ALS is continually increasing, with the NIH predicting a 69% increase by 2040. It is our goal to push for continued strides in research and funding so that ALS may become a livable disease rather than a death sentence.

Comments for Docket No. FDA-2024-N-1809

We are incredibly grateful to the Food and Drug Administration ("Agency" or "FDA") for the opportunity to comment and share our recommendations for optimizing Advisory Committees. We thank the FDA for its continued commitment to hearing from community stakeholders directly impacted by its decisions.

Advisory Committees (AdComms) have long provided a platform for multiple stakeholders to share their expertise to better inform the Agency's decisions. We strongly believe in the importance of AdComms; however, we have also come to recognize various factors that can be modified to improve the efficiency of these sessions. Our comments address three main themes of change we would like to see in the Advisory Committee process: 1) a stronger focus on patient voices, 2) improved transparency, and 3) increased inclusivity.

Patient Voice



We are a patient-led community revolutionizing how to cure disease.

Although the Advisory Committee ("Ad Comm") composition currently includes patient representatives, we firmly believe that the voices of those living with the diseases being discussed should be weighed more heavily when appropriate. We applaud the Agency's commitment to grounding its decisions based on clinical evidence and scientific expertise, and there is also an urgent need to incorporate qualitative lived experiences and real-world evidence into decision-making, especially for diseases with no standard of care, no treatments, and definitively fatal, such as ALS. These experiences can provide crucial insights that may not be captured by clinical evidence alone.

By incorporating a rubric for including real-world lived experiences into decision-making, the Agency can make sure those insights are fully heard and utilized. People with ALS (pALS), their caretakers, and their loved ones are the ones that have the most stake in these conversations. Therefore, their expertise are the only advisors not conflicted, and with needs that must be understood and incorporated to make equitable and effective decisions. Increased patient and patient advocate involvement can only lead to more informed decisions and better understanding of disease impact.

There are multiple ways that this could be incorporated into the current AdComm structure, including an increase in the number of patient representatives to reflect a deeper understanding of diverse stakeholders. This change could lead to a more comprehensive scope of the disease and its impact. It is our experience that not all people with ALS are the same, with various backgrounds, experiences, and beliefs. By having multiple patient and caregiver representatives, the Agency can better capture a more realistic sample of impacted American families. Furthermore, we strongly recommend that a patient and patient representative should always be included as voting members, specifically in hearings that directly impact critical topics in access and treatment approval.

Finally, as it relates to patient voice, we recognize the current use of the lottery system for speakers has served the Agency well in preventing biases; however, we find that this method does not guarantee diversity of perspective. Instead, we recommend a possible shift to having categories and performing a lottery within these categories. For example, individuals wishing to speak would classify themselves into a category and then be in the pool for that specific group. This ensures fairness and that diverse voices throughout the field are being represented.

Inclusivity

We have found that when the Agency holds AdComms that impact our community, there is often a strong desire to participate by sharing experiences; however, as ALS progresses, many individuals are limited in their ability to travel and be physically present at hearings. We are grateful that since the COVID-19 pandemic, the Agency has utilized Zoom to facilitate virtual participation; however, we think that AdComms would benefit from modifying the existing options. For example, not allowing individuals to be on camera does not allow for full participation and makes video chat feel secondary.

In diseases such as ALS, individuals already experience challenges, and it should be the goal of the Agency to remove any barriers that might introduce more complications. The communities of people with disease must be allowed to be seen and valued as significant contributors to this necessary proceeding. We believe that shutting out the faces of those impacted by ALS not only



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hinders open and intentional conversation but also diminishes the integral role of the ALS community in decision-making processes.

By keeping participant cameras off, the Agency is unintentionally denying the visibility of the daily challenges of ALS patients, thereby diminishing and devaluing their testimony and preventing authentic conversations about the realities of ALS. We are incredibly concerned about how this limitation significantly reduces the critical patient perspective and voice. By not allowing video testimony from the ALS community, we find that it substantially dehumanizes any patient contributor. For many within the disease communities, this choice is upsetting, but for a person living with ALS who may have already lost their voice due to the progression of the disease, this decision is cruel.

Transparency

With their long history, Advisory Committees have played a crucial role in the decision-making process for multiple agencies. However, as time passes, we must modernize AdComms to better serve our communities. The existing AdComm process can be relatively confusing to many, and we have found that the Agency has failed to improve transparency. For many, the AdComm process can feel overwhelming and challenging to navigate. Many individuals become advocates out of necessity, and we firmly believe that making this an accessible process is critical. By improving the AdComms' announcement process and providing ample time for individuals to participate, AdComms will receive more meaningful and enhanced engagement.

Another critical issue we have identified with the AdComms process is the current selection of voting members and the lack of information shared with stakeholders. For example, the Agency does not provide meaningful information such as the number of individuals who applied to be voting members, the process by which applicants are vetted, applicant qualifications, and who conducts the selection process. Withholding this information makes the selection decisions seem underhanded. This lack of transparency can lead to mistrust and a feeling of exclusion among the community.

Further, following the Agency's decision, we strongly encourage rationale to be provided and include how the AdComm's recommendations were weighed into the decision-making process. This type of information is critical to communities as it allows for full transparency, but it is especially important in situations where the Agency's decision is not in line with the recommendations of the committee.

Thank you again for this opportunity to share our comments, and for the Agency's commitment to improving the AdComm process so that it may better serve the community. We hope to continue to be a resource for the Agency moving forward, and should you have any further questions please reach out to our Chief Government Affairs Officer, Sonya Sotak Elling.

Sincerely,

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