Policy Entrepreneurship for Regulation, Delivery, and Evaluation of Medical Products: Final Report

Why Did FAS Do This Study?

The U.S. Food and Drug Administration (FDA) reviews hundreds of medical products for regulatory approval each year and they use Federal Advisory Committees (FACs) to support their employees in making evidence-based decisions for these products. However, over the years, complaints have risen regarding the decline in the amount of Federal Advisory Committees and their convenings. This decline has been documented and supported by former FDA Advisory Committee member, Aaron Kesselheim, Professor of Medicine at the Harvard Medical School, and his team in a recent Journal of the American Medical Association Health Forum article. The consistent decline in the number of Committees and convenings has presented fewer opportunities for Committee members, who serve as experts and an independent party, to provide insights on medical product regulation to the FDA. These independent parties are needed for the regulatory decision-making process because they provide additional expertise and in-depth knowledge for the Agency on topics that contribute to evidence-based decision making.

FACs are often the single entry point external experts and the public have to comment on medical products in the approvals pipeline and hold the FDA accountable for their approval decisions. Nonetheless, many of the crucial decisions about the efficacy, safety, affordability, and equitable design of medical products are being made solely between the FDA and its sponsors, with no input from the communities who these products will affect. Because the public relies on the FDA to regulate products for safety and efficacy, regulatory decision-making should always be evidence-based. Evidence-based policy uses the best available research to identify what works and highlight any gaps in an effort to guide decisions at all stages of the policy process within the federal government. Ensuring evidence-based policy is at the center of all federal government decisions that affect the American public is responsible governance.

With the growing mistrust in institutions from the federal government to science, education, and more, FAS felt it was necessary to re-envision how the FDA and other federal agencies engage external scientific experts and the public to address critical challenges facing public health. FAS examined these engagement processes by researching the use of Advisory Committees. An article from the Pew Charitable Trusts mentions that historically respected institutions are losing the trust of Americans, stating that only 22% of participants in 2024 trust the federal government to do the right thing. This percentage has decreased from 35% public trust in the government in 2022. Most recently, a 2024 poll displayed that only 15% of

Americans have a "great deal of trust" in the FDA and their decisions. Public trust is important for the federal government overall, but specifically for the FDA due to their decision-making efforts that affect the public health and safety of all Americans.

As currently organized, FACs are not up to the task of providing the best evidence-based advice to assist with building trust through transparency and this is partially due to their decline in convenings, inadequate onboarding for incoming Advisory Committee members, controversial approvals, and lack of transparency with the conflict of interest process. For this reason, it is critical that FACs are reformed and equipped with the necessary tools to continue providing the government with the best evidence-based advice.

Federal Advisory Committee Reform

To drive this agenda forward of proposing actionable policy recommendations to reform the Federal Advisory Committee ecosystem, FAS successfully crowdsourced ideas from current and former FAC members, as well as other external experts through the use of interviews, focus groups, and public convenings. FAS was well positioned to broaden the scope of responsible federal actors and drive uptake of policy recommendations across the federal government toward more evidence-based regulation due to previously having:

- Developed +50 implementation-ready health policy memos for the federal government, on topics like generic drug repurposing, pharmacoeconomics, and bias in medical innovation.
- Developed +200 relationships with a wide range of health stakeholders — policymakers, public health, patient advocates, industry, researcher, and clinicians — building expertise on topics from regulatory policy to clinical trials to procurement innovation as a lever for incentivizing equitable and accessible medical products.
- Regularly convened diverse stakeholder groups, creating bridges between the science and technology community and federal policymakers

For this project, FAS deployed a multi-pronged approach to deliver policy recommendations through interviews and focus groups that were specifically held with current and former FAC members using a semi-structured guide to learn about their interest in Advisory Committee service, the application and invitation process, recruitment, conflict of interest audits, and more. Interviewees were selected by generating a list of all Advisory Committee members from the FDA's website and reaching out to equal numbers of individuals from academic, scientific, consumer, patient advocate, industry and medical backgrounds for diverse perspectives. Recruiting members from different backgrounds was critical to this project to establish a baseline for strategic thinking and increase the potential outcome of innovative policy ideas that will be instrumental in rebuilding the future of Advisory Committees.

Our interview guide included questions that were both open-ended and scaled to ensure we were able to document a comprehensive viewpoint of the process and areas of reform that members deemed to be the most important for the success of Advisory Committees. We grouped similar responses together and were able to extract five areas as key focal points of the discussions. These areas became our case study topics for recommendations which will be mentioned in the Looking Ahead section of this report. In addition to data from interview responses, the interview transcripts and current literature were used to develop policy recommendations for each of the topics.

After the development of preliminary recommendations, the second step was to stress test those recommendations and determine their feasibility for implementation for the FDA. We facilitated focus groups with Advisory Committee members to further refine the policy recommendations. The final recommendations received buy-in from all participants and can potentially assist with enhancing the FDA's ability to obtain valuable advice for evidence-based decision-making.

The final steps for the data gathering process included a series of workshops with external stakeholders where we shared our recommendations, solicited feedback for the development of an official public comment, held an informational session to discuss the purpose of AdComms and their role in the regulatory process, and briefed the Senate HELP Committee on the implications our recommendations hold for the FDA, as well as Congressional considerations. These after action and public comment workshops were a result of the FDA's public meeting to gather intel on the use of Advisory Committees. To prepare for these workshops, we summarized the public comments from the FDA's public meeting that aligned with our recommendations and documented potential best practices for the future. This analysis is summarized in the appendix of this report.

Lessons Learned

This project allowed FAS a direct line into the experiences of Advisory Committee members, what they see as the main issues with the AdComm system, and changes they feel are necessary to increase productivity, evidence-based advice, and encouragement of participatory regulatory science. Lessons learned have been synthesized and briefly summarized using the points below.

AdComms are critical to transparency in FDA decision making
AdComms act as independent, unbiased experts. They are the bridge between the public and the FDA and give a voice to those who the medical products will affect. AdComms and AdComm convenings provide a window for the public to see how the FDA makes regulatory decisions and showcases that the Agency is placing an emphasis on input from external parties.

AdComm members have invaluable expertise

AdComm members are experts who have years of experience in various fields. Many of them have stated they got involved in AdComms because they see the big picture of ensuring safe and effective medical products for the American public. They've also mentioned that they understand the FDA has assumed a huge risk when it comes to approving medical products, and they view this work as their responsibility to the public. However, even with the passion to help the FDA fulfill their mission of protecting public health, members felt as though the FDA does not value their time as experts or their expertise itself. These feelings are a result of issues such as 1) not feeling there's enough time for deliberation, 2) the inability to ask clarifying questions of the FDA, 3) not being provided with training as incoming members, and 4) not being provided with any justification when the Agency decides to go in a direction that is in opposition of the Committee vote. The FDA should consider revising some of their processes to ensure that AdComm members feel valued and supported as they are a great resource for agency decision-making.

Process adjustments and communication improvements are needed

Interviews with current and former AdComm members revealed that many of them enjoy providing expertise and guidance to the FDA. However, they prefer certain changes in some of the overall processes for AdComms as it pertains to 1) streamlining of the conflict of interest process 2) returning to in-person meetings, 3) training, and 4) communication between the Agency and the AdComm when there are disagreements. These improvements can be made to the system through process adjustments and stronger communication.

Robust investment in the AdComms system through annual appropriations

AdComms require extensive resources to operate depending on the frequency of meetings, the number of AdComm members, and supporting FDA staff. Therefore, the annual cost for every FDA Committee can vary. For example, for fiscal year 2024, the total operation cost for all AdComms focused on reviewing medical products was $10,638,771.00. Projected operating costs for fiscal year 2025 estimates a total of $11,711,814.00, which is an increase of $1,073,043. While these endeavors require significant resources, it is necessary for annual appropriations to continue in funding these Committee efforts because AdComms are needed to enhance federal decision-making, improve public policy, boost public credibility, and Agency morale. Operational AdComm costs for the current year and estimates for the next fiscal year can be found here.

Looking Ahead

While many stakeholders have called for changes to the AdComm system and meetings, we know this process will require significant external input to ensure the result of a transparent, participatory regulatory system. Effective, participatory regulatory science is critical to building

public trust in the FDA's AdComm system and the transformative medical products they review and regulate. Thereby, we encourage the following considerations for FDA, GSA, and Congress to aid in this establishment of an AdComm system that will benefit all interested parties.

Considerations for FDA

This section includes a high-level overview of considerations for the U.S. Food and Drug Administration (FDA). A full listing of all recommendations, best practices for implementation, and the appropriate stakeholders from the FDA can be found in our toolkit available for download here.

Voting

- Retain voting as an integral function of AdComms
- Revise AdComm voting guidance documents

Resolving Disagreements

- Make staff aware of Scientific Dispute Resolution at FDA guidance
- Develop guidance for resolving conflicts between the Agency and AdComms

Leveraging Advisory Committee Membership

- Include standing areas of expertise for all Committees
- Expand the role of the Chair
- Provide introductory trainings for AdComm members
- Streamline the Conflict of Interest (COI) process to prevent duplicative work

Patient Representative and Advocate Representation

- Include patient representatives on all AdComms reviewing medical products
- Develop an outlet for engagement to be ongoing, rather than annually or quarterly

Public Awareness and Understanding

- Leverage social media platforms for widespread dissemination of information
- Include disclaimers for communication materials indicating the role of AdComms and the purpose of an AdComm vote
- Partner with local/state public health agencies and advocacy organizations to educate patient advocate communities and the general public

Considerations for GSA

The General Services Administration (GSA) is responsible for any changes to Advisory Committees that require the creation of new Committees, the renewing of or altering of current Committees, oversight of conflict of interest protocol, and proper implementation of all Federal

Advisory Committee Act (FACA) requirements. Therefore, the following considerations have been provided for possible implementation:

- Review conflict of interest policies for all AdComms and work with the FDA to develop and/or revise current processes to alleviate administrative burden

Considerations for Congress

Improving the FDA Advisory Committee process and experience can be advanced through Congressional action in the legislative and communicative options as listed below.

Legislative Options

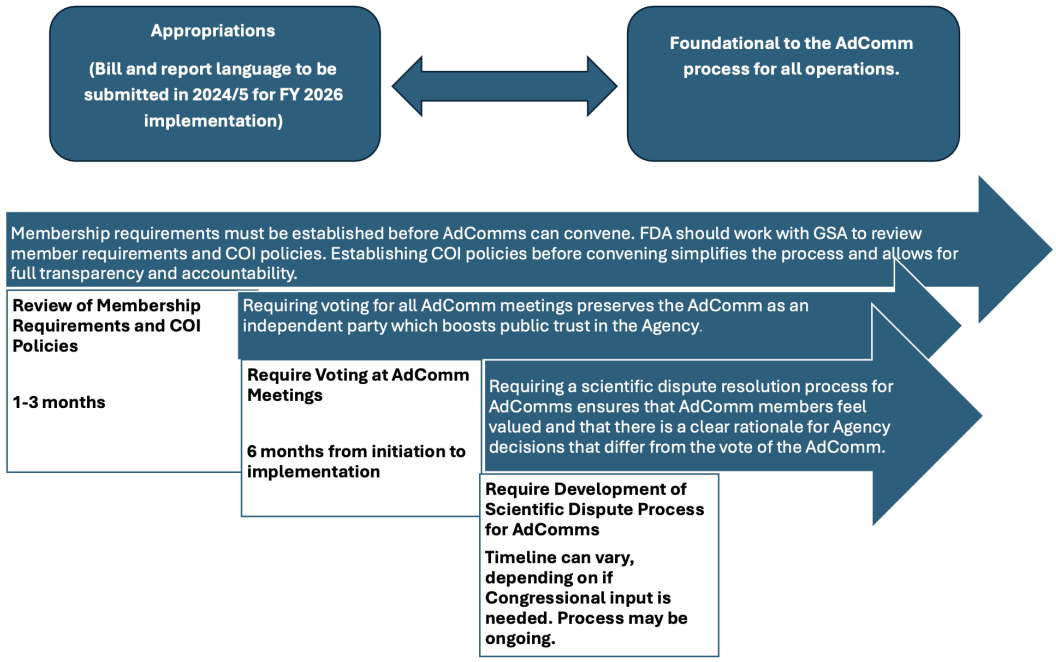
- Require voting for all AdComm meetings that review regulated medical products
- Require FDA to create a scientific dispute process between the Agency and its AdComms
- Review COI policies for SGEs and require agencies to establish a disclosure protocol for COI waivers that are administered
- Review membership on AdComms to ensure balance of perspectives
- Ensure adequate appropriations to run a robust AdComms system

Communication with the Agency

- Encourage the FDA to supply trainings to AdComm members
    - Where necessary, supply additional appropriations
- Encourage the FDA to develop a streamlined administrative COI process
    - For example, FDA can leverage CMS' Open Payments to streamline conflicts of interest
- Encourage transparency around the influence of AdComms on FDA decision-making and how FDA uses this expert and public input

Implementation Roadmap

The goal of AdComms is to help the FDA make better evidence-based decisions on the safety and efficacy of medical products for the public. Enforcement of the aforementioned considerations will ultimately build a more functional Advisory Committee system through 1) upholding agency transparency and accountability, 2) increasing Advisory Committee meeting productivity and ability to provide evidence-based advice, and 3) improving processes for AdComms and public communication.



In an effort to implement these considerations for FDA Advisory Committees, certain steps must be completed. Considering that AdComms take considerable resources to operate, it would be beneficial to have increased appropriations and user fees for their activities. Appropriations lay the groundwork for all Advisory Committees to function properly. Without proper appropriations and user fees, AdComms will not have the ability to recruit and retain top talent and convene to review drug applications in a timely manner. The FDA can develop and submit appropriations language and Congressional report language (to give the Agency more direction on how Congress wants them to spend their funding) to the appropriations Committee to 1) increase appropriations and user fees or 2) to use a certain amount of appropriations for various AdComm activities. The entire appropriations duration can potentially last up to 18 months. Currently, the House and Senate have issued preliminary marks for 2025, so future appropriations will most likely not be included until 2026. However, with the reauthorization of the Prescription Drug User Fee Act (PDUFA) scheduled for September 30, 2027, the FDA should also consider adding justification for increased user fees in the appropriations and report language to be submitted to Congress.

Upon appropriations approval, the FDA, with encouragement from Congress should consider reviewing AdComm membership requirements to ensure that they are inclusive of individuals who bring real-world experience to the process. These individuals include, but are not limited to patient and consumer representatives, statisticians, pharmacists, and pharmacologists. It is also recommended that GSA work with the FDA to review all COI policies for AdComms and require that the FDA develop a disclosure protocol for all members who receive waivers for increased transparency and accountability in the regulatory process. Additional Congressional

consideration should be given to encouraging the FDA to establish voting as a formal part of all Advisory Committee meetings to maintain integrity and developing a dispute resolution process for the Agency and AdComms. The review and enactment timeline of these requirements can vary, particularly with respect to increased user fees since the reauthorization will occur in 2027.

Implications for FDA Advisory Committees Under Incoming Administration

FDA AdComms are instrumental in facilitating transparent and collaborative deliberation between the federal government (executive branch), the advisory body, and the American public. Their recommendations are integral to strengthening public trust and reinforcing the credibility of federal agencies. The state and future of FDA AdComms are under question, and even more so as we transition to a new administration. Under the 2016 U.S. Presidential administration, Executive Order (EO) 13875 resulted in a significant decrease in the number of federal advisory meetings which limited agencies' ability to convene external advisors. It was discovered that since the government started tracking AdComm activities in 1997, federal science advisory committees met less during this administration than any prior administration, met less than what was required from their charter, disbanded long standing AdComms, and barred scientists who were receiving agency grants from serving on AdComms. Federal Advisory Committee membership also decreased by 14% which displayed an issue with recruiting and retaining top talent.

This EO required all federal agencies to slash their AdComms by one-third and set an overall federal government cap to only have 350 total AdComms when there were over 1,000 at the time. The disbandment of AdComms, exclusion of key scientific external experts from AdComms, and burdensome procedures can potentially present severe consequences that can affect the health and safety of Americans. A reinstatement of a similar EO and a continuation of practices from the 2016 administration could result in a glaring removal of robust science advice from federal decision making. Given current discussions of deregulation, downsizing the federal budget, and restructuring the FDA, it is imperative that Advisory Committees remain active and continue to assist the FDA with the evidence-based advice needed to make critical decisions that affect the American public. We encourage this incoming administration to uphold the principles of scientific integrity and continue to foster a decision-making environment that relies on evidence-based recommendations from AdComms.

Conclusion

The FDA is responsible for the public health and safety of all Americans. Through the use of Advisory Committees, the FDA can improve their ability to make sound decisions when approving products for market. Successful implementation of the mentioned considerations for Congress, GSA, and the FDA will ensure a more robust, well-defined AdComm process that allows participatory collaboration between the Agency, the AdComm, and the public.

Acknowledgements

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FAS FEDERATION OF AMERICAN SCIENTISTS

Appendix

This appendix features the most recent deliverables produced by this project. Not shown in this appendix are previously submitted AV deliverables such as the official public comment (hyperlinked below), interview transcripts, and focus group summaries.

Public Comment Analysis

The Food and Drug Administration received public comments from five private citizens, two academic research institutes, 7 associations representing regulated industries, 4 strategic consultancies that advise companies on the regulatory approvals process, 7 associations representing medical care providers and pharmacists, 7 patient advocacy organizations or coalitions, 4 regulated industries, and 5 comments from think tanks and advocacy groups. Our team analyzed the feedback to find alignments with our recommendation set developed through AdComm member engagement as well as areas for future exploration:

Alignment with FAS Recommendations

1. Ensure perspectives from patient representatives are representative of the patient population served. Incorporate concepts from patient-focused drug development.
2. Improve the representation of perspectives on AdComms, such as including biostatisticians with familiarity in the trial designs being discussed and pharmacologists that are familiar with prescribing drugs to patients.
3. Creating trainings for incoming AdComm members to orient them to their roles
4. Utilize the AC Chair as a resource; such as in recruiting new members
5. Retain voting but evaluate new systems that can better elicit feedback from the Committee (such as voting along a scale).
6. Address the lack of public awareness about the AdComms' role through targeted FDA-led communications after every meeting. This provides a consistent way for the FDA to message about the purpose of AdComms
7. Decide on how to incorporate conflicted members. If included, do so in a way that transparently acknowledges the conflicts held or removes possibility for biasing the results (i.e. making them a non-voting member)
8. Create a more transparent process for recruiting experts onto AdComms, including those with expertise in rare diseases and emerging technologies.

Areas for Future Exploration

1. Hire a professional meeting facilitator for AdComm meetings to allow members to focus on the topic at hand
2. Create analytical tools (like AI) that synthesize the public comments and make meaningful recommendations from the synthesis

3. Make the distinction between consumer representatives and patient representatives, as well as review the reasons behind the differences in their roles on committees (i.e. consumer reps are voting members and patient reps are not).
4. Create a prioritization matrix for the Open Public Hearing to prioritize hearing from perspectives relevant to the subject of the AdComm.
5. Give more advanced notice of AdComm meeting topics as well as provide the meeting materials to the public more than two days in advance.
6. Reduce redundancies between the sponsor presentation and the FDA presentation, and create more opportunities for pre-meeting communication between the applicant and the agency to decide on meeting roles and communication.
7. The FDA could delay voting to a few days after the meeting to allow for more consideration by AdComm members about their final decisions.
8. Include the perspectives of primary-care providers, OB-Gyns, and nurses.

Public Comment

- Public Comment Submitted to Docket FDA-2024-N-1809

Case Studies

- The Future Of Voting For FDA Advisory Committees
- Leveraging AdComm Membership
- FDA Staff And Leadership Disagreements And The Role Of The AdComm In The Regulatory Process
- The Role Of Patient Advocacy In The AdComm Process
- Improving Public Awareness And Understanding Of Advisory Committees

Toolkit

- Advisory Committees For The 21st Century Recommendations Toolkit

Op-Ed (published on October 1st for STAT)

- *Five Things FDA AdComm Members Want*