



**Testimony of Cartier Esham, Ph.D.
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Energy and Commerce Committee
Subcommittee on Health
Hearing on "Combating the Opioid Crisis: Prevention and Public Health
Solutions"
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Chairman Burgess, Ranking Member Green, and Members of the Committee:
BIO appreciates the opportunity to speak with you today about policy solutions put forward by this Committee to address America's opioid crisis.

I am Cartier Esham, Executive Vice President of the Emerging Companies Section and Senior Vice President of Science & Regulatory Affairs at BIO. BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers, and related organizations across the United States and in more than 30 other nations. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, to delay the onset of diseases, and to prevent them in the first place.

BIO's member companies are dedicated to the development of the next generation of biomedical breakthroughs for the millions of patients suffering from diseases for which there are no effective cures or treatments. It is this mission focused on innovation that guided the development of BIO's policy recommendations to change the paradigm of how we treat pain and addiction and eliminate prescription opioid drug addiction in the United States.

The development of innovative therapies for treating pain and addiction is a crucial component of mitigating the current opioid crisis and preventing future opioid addiction. The current state of innovation for next generation pain and addiction therapies holds promise but requires a more conducive policy environment focused on enabling access and incentivizing the investment needed to unleash the full potential innovation to change the paradigm of treatment and improve the lives of patients. Today, less than four percent of total venture investment in the biopharmaceutical sector is being directed into companies whose lead product is a novel pain therapy. Additionally, over the last decade, companies working on novel pain therapies have received 17 times less funding than companies working on oncology drugs, with even less investment for the development of novel therapies



for addiction. There are currently 220 clinical stage drug programs, with 125 testing novel chemical entities, 87% of which are for non-opioid receptors. While promising, when compared to the 2,617 clinical development programs in oncology it is clear the full potential of innovation in improving care for patients suffering from pain or addiction has not been realized.¹

BIO's policy recommendations are focused on the following three pillars, which if acted on, would serve to mitigate and work to eliminate prescription opioid drug abuse by encouraging investment into better and safer therapies to treat pain and addiction.

- (1) Advancing the understanding of the biology of pain and addiction to enable the development of innovative treatments, and ensuring appropriate and optimal use of existing therapies;
- (2) Ensuring that patients suffering from pain or addiction are able to receive the right treatment at the right time with the right support, without stigma; and
- (3) Stimulating research and development of innovative treatments that effectively treat pain and opioid addiction and prevent abuse.

BIO would like to discuss three bills under consideration today that if enacted would improve care for people suffering from pain or addiction and help prevent individuals from developing prescription opioid addictions.

BIO supports the discussion draft legislation on FDA Opioid Sparing. This legislation would enable Food and Drug Administration (FDA) and stakeholder collaborations to discuss issues relating to data collection on opioid sparing and inclusion of such information in product labeling to inform development of guidance. Specifically the legislation would direct FDA to develop guidance that would better enable utilization of innovative clinical trial designs to ethically and efficiently collect data on opioid sparing, improve the development and acceptance of endpoints that measure the reduction of chronic pain and opioid use, improve the ability to utilize real world evidence and enable information about opioid sparing data to be included in the label of a product. Enactment and implementation of this legislation would provide FDA, biopharmaceutical companies, medical researchers, and investors with an improved understanding about how data sources can be utilized to support demonstrations that a novel therapy reduces opioid use. In addition to encouraging collaborations and guidance development on opioid sparing, BIO believes the same approach to stakeholder engagement and guidance development on other critical issues such as benefit-risk assessment of novel and safer treatments, improved approval processes for chronic pain indications, modern approaches to non-opioid drug development and review processes, improved mechanisms for evaluating pain, and utilization of innovative clinical trial designs for novel pain and addiction therapies would serve to provide increased understanding and improve drug

¹ Thomas, D., & Wesel, C. (2018) [The State of Innovation in Highly Prevalent Chronic Diseases, Volume II: Pain and Addiction Therapeutics](#).



development and review processes. These actions would serve as critical signals to biopharmaceutical companies and their investors that there is a well-defined, efficient and effective path forward for developing pain and treatment therapies that are safer, improve quality of care and reduce the use of opioids.

BIO also supports draft discussion legislation that would better enable the utilization of Accelerated Approval and Breakthrough Therapy pathways for innovative and safer treatments of pain and addiction. Enactment and implementation of this legislation would provide FDA and the biopharmaceutical industry with a greater understanding of what is required to meet criteria for these expedited approval pathways and ensure processes intended to expedite development and approval meet the unique needs of pain and addiction medicines. This too would serve as a powerful signal to stakeholders and investors that treatments and therapies that improve and protect the lives of patients suffering from pain and addiction is a top public health priority.

Lastly, BIO supports H.R.5002, the Advancing Cutting Edge Research Act. This draft legislation would provide the National Institutes of Health (NIH) with transactional authority that would enable them to more efficiently distribute funds to conduct or support research required to respond to a public health threat, such as the current opioid crisis. This new authority could also be used to design and implement innovative business models within the government and engage with nontraditional research partners in ways that would otherwise not be feasible. In addition to these new authorities BIO has developed recommendations that call for the development and implementation of a research strategy that is transparent and accountable and focused on advancing our understanding of the biology of pain and addiction, developing tools that would improve diagnosis and treatment of these diseases, and enabling utilization of data to better ensure appropriate and optimal use of existing and future therapies. BIO believes the legislation would be strengthened if these ideas on collaboration and data analytics were added to HR 5002. Making research on the biology of pain and addiction a national priority will further strengthen investor confidence in this area.

BIO strongly believes that innovation is a key component of efforts to address America's opioid crisis. The three bills discussed above and under consideration by this Committee and BIO's complimentary policy proposals described below would serve to advance our scientific understanding of these diseases and enable improved drug development and review processes for the next generation of pain and addiction treatments. These actions would encourage R&D and investment in medicines that will change the paradigm of how we treat pain and addiction, improve patients' lives and help prevent prescription opioid drug abuse in the United States.

Additional Recommendations



In addition to the excellent work being conducted and discussed by this Committee today, BIO has developed additional complimentary recommendations that would further serve to stimulate investment and advance the development of novel and safer treatment options for pain and addiction and help eliminate opioid prescription drug abuse in the United States.

Stimulating research and development of innovative treatments that effectively treat pain and opioid addiction and prevent abuse: Stimulation of research and development of novel treatments for pain and addiction is critical to ensuring an America free of addiction in the future. BIO recommends that the FDA develop activities to better enable effective and efficient drug development and review for novel and safer treatments for pain and innovative treatments for addiction. In addition to the Opioid Sparing legislation discussed above, BIO recommends that FDA engage stakeholders to discuss and take action to address the following critical issues identified as regulatory barriers for the development of new pain and addiction therapies:

- **Benefit-Risk Assessment of Novel and Safer Treatments:** Discuss issues and develop recommendations about how to most effectively evaluate the entire benefit-risk profile of a given product. For example, the benefits of abuse deterrent formulations of conventional opioids, non-opioid treatments, and innovative treatments that have, in general, lower or no abuse liability should be considered relative in the context of existing options.
- **Approvals for Chronic Pain Indications:** Discuss issues and develop recommendations about how to efficiently and effectively obtain approval for a broad chronic pain indication.
- **Non-Opioid Alternatives:** Discuss modern approaches to the development and review of non-opioid alternatives for pain and develop recommendations.
- **Mechanisms for Evaluating Pain:** Discuss issues and develop recommendations about how to improve upon current mechanisms for evaluating chronic and acute pain both in the clinic and the clinical trials setting, as well as, how to better enable the utilization of biomarkers and novel endpoints.
- **Innovative Clinical Trials:** Discuss and develop acceptable mechanisms to streamline clinical trial strategies to expedite the development of novel and safer non-opioid treatments for pain and addiction.

Advancing the understanding of the biology of pain and addiction to enable the development of innovative treatments for pain and addiction and ensure appropriate and optimal use of existing therapies: BIO recommends NIH develop a comprehensive plan that prioritizes and supports



research at the appropriate institutes and in academia focused on improving our understanding of the biology of pain and addiction, and advancing preclinical modeling and development of better measurements of pain and addiction in the clinical setting. We encourage such a plan to include collaboration with stakeholders and transparency about what activities are planned or completed and how those efforts are advancing the state of scientific understanding of pain and addiction diseases. Specific areas of research that are critical to these advancements include:

- *Developing a more comprehensive understanding of the preclinical pain and addiction environment*
 - Research focused on the following areas would serve to develop a more comprehensive understanding of mechanisms underlying different types of pain as well as biomarkers that allow for the differentiation of pain subsets, and identification of preclinical models that better translate to therapeutic outcomes for people and would help inform the development of novel and safer treatments for pain and addiction.
 - Research investigating potential therapeutic targets with increased receptor/intracellular signaling selectivity
 - Research investigating novel mechanisms for preventing and/or treating opioid addiction and overdose
 - Research on the identification and validation of biomarkers that identify subsets of pain and more accurately predict treatment response for both pain and addiction
 - Research on the identification and utilization of more effective drug screening models and assays that better translate to therapeutic outcomes for patients
 - Research focused on elucidating the underlying biological mechanisms of different types of pain and addiction, as well as differences in pain perception
- *Support a more comprehensive understanding of the clinical pain and addiction environment*
 - Research on and support for developing comprehensive methods for evaluating pain in the patient, tools that allow for better diagnosis of pain, as well as the establishment of clinical trial registries, clinical trial networks, and data sharing and analytics are critical to ensuring patients are able to receive the most appropriate treatment.
 - Research and encourage the development and use, in both the clinic and clinical trial setting, of more comprehensive and objective tools for assessing pain that take into account acute versus chronic pain, the possible neurobiological and psychosocial mechanisms underlying pain, individual differences in pain perception, and distinctions between somatic and psychic pain



- Research on the development of diagnostic tools that enable the identification of specific causes of pain
 - Research on and encouragement of multi-disciplinary approaches to treating pain and addiction by enabling approaches for determining risk or susceptibility for individual patients, and associate outcomes with the basic neurobiology and/or pharmacological alterations
 - Support the establishment of clinical trial registries and clinical trial networks for pain and addiction to help develop, validate, refine, and deliver new treatment options to patients
 - Support NIH's recommendations to the Drug Enforcement Agency (DEA) regarding research exemption for controlled substances, as this can help advance scientific discoveries in pain and addiction treatment.
- *Improve ability to use data to improve medical decision making*
 - BIO recommends that NIH collaborate with stakeholders, the Food and Drug Administration, and the Centers for Medicare and Medicaid Services and other agencies as appropriate to discuss how to best collect, analyze, and apply data to answer the following critical questions and better inform medical decision making. This collaboration should explore what data is available as well as any data gaps or quality issues that need to be addressed.
 - Which treatment(s) works best for a particular patient and what factors must be considered when making that determination? Is there a certain "type" of patient that typically sees more success with one treatment compared to another?
 - What factors (e.g., training, stigma, reimbursement, faculty size) prevent providers from offering, directly or by referral, all available addiction treatment options?
 - What is the optimal duration of specific treatments?
 - When should a patient's treatment regimen be changed, and how?
 - What gaps or barriers exist which impact the ability to collect, analyze and leverage data to answer the above questions?

Ensuring that patients suffering from pain or addiction are able to receive the right treatment at the right time with the right support, without stigma: BIO recommends that policy makers remove coverage and reimbursement barriers that prevent patient-centered decisions about and access to the most effective treatments for pain and addiction. Additionally, BIO supports successful implementation of laws and regulations enacted to improve patient access to appropriate treatments across pain and addiction.



Closing Remarks

BIO urges Congress to act swiftly to move legislation on FDA Opioid Sparing, FDA Accelerated Approval and Breakthrough Therapy Status, and H.R. 5002 Advancing Cutting Edge Research forward. We request the Committee consider BIO's additional and complimentary policy proposals outlined in this testimony. Enactment of these policies would change the paradigm of how we treat patients suffering from pain and addiction and achieve our shared goal of eliminating prescription opioid drug abuse in the United States.

Thank you for the opportunity to present our views today. I am happy to answer any questions you may have.