



March 4, 2016

Senator Ron Wyden
Ranking Member
U.S. Senate Committee on Finance
221 Dirksen Senate Office Building
Washington, D.C., 20510

Senator Charles Grassley
U.S. Senate Committee on Finance
135 Hart Senate Office Building
Washington, D.C. 20510

BY ELECTRONIC DELIVERY

RE: January 21 Letter to the Health Care and Patient Community

Ranking Member Wyden and Senator Grassley:

The Biotechnology Innovation Organization (BIO) is pleased to submit the following comments regarding Ranking Member Wyden's and Senator Grassley's January 21 Letter to the Health Care and Patient Community (the "January 21 Letter").¹ 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 these diseases, or to prevent them in the first place. In that way, our members' novel therapeutics, vaccines, and diagnostics not only have improved health outcomes, but also have reduced healthcare expenditures due to fewer physician office visits, hospitalizations, and surgical interventions.

BIO represents an industry that is devoted to discovering, and ensuring patient access to, innovative treatments. Recognizing the ongoing dialogue around the value of such innovative therapies, in February 2016, BIO released *Principles on the Value of Biopharmaceuticals*.² These *Principles* champion the commitment on the part of industry to call upon and work collaboratively with all stakeholders to address issues around value, access, and sustainability of the innovative ecosystem. The *Principles* represent the first-ever systemic, industry-endorsed set of commitments by research-based biopharmaceutical companies to support comprehensive and sustainable solutions to improve patient access to, and affordability of, innovative medicines. We have provided BIO's *Principles* as an Appendix to these comments given their role in framing our response.

In the January 21 Letter, five questions are posited to the stakeholder community that identify and ask for feedback around policy questions that impact access to innovative therapies. In considering these questions closely, BIO notes that the questions reflect two broader themes: (1) the value of innovative therapies; and (2) collaborative approaches to

¹ Senators Wyden and Grassley. 2016 (January 21). *Letter to Health Care and Patient Community*, available at: <http://www.finance.senate.gov/imo/media/doc/012116%20Wyden%20Grassley%20Sovaldi%20Report%20Feedback%20FINAL4.pdf>.

² BIO. 2016 (February). *Principles on the Value of Biopharmaceuticals*, available at: https://www.bio.org/sites/default/files/BIO_PRINCIPLES_ON_THE_VALUE_OF_BIOPHARMACEUTICALS.pdf.

improving patient access to innovative therapies. BIO provides detailed feedback on each of these themes that underlie the Letter's questions in the balance of these comments.

I. The Value of an Innovative Therapy—including Its Benefits and Costs—Must Be Assessed Holistically.

The need to assess the value of an innovative therapy underlies several of the questions included in the January 21 Letter. BIO continues to be concerned that the dialogue around the value of innovative therapies is increasingly focused on too-short a timeframe and/or on a single element of value only. Instead, value must be assessed across the entire time course over which the therapy's benefits and costs accrue, and the assessment must be holistic. Innovative therapies have the unique potential to significantly improve the standard of care, mitigating or curing, chronic diseases that would have otherwise required a patient to receive treatment over years or decades. For example, new medicines to treat HIV/AIDS resulted in a 23 percent decline in hospitalization rates between 2002 and 2007.³ As another example, recent estimates suggest that the U.S. would save \$367 billion on health services by 2050 if we develop a new medicine that delays the onset of Alzheimer's disease by just 5 years.⁴

Given the potential long-term effects of innovative therapies, BIO is very concerned that the value of these therapies will be undermined by any assessment of their impact that fails to take into account the entire timeframe over which the benefits/costs of utilization accrue. Consider, for example, treatment for oncology. Innovative treatments for chronic myeloid leukemia, in particular, have helped to increase the ten-year survival rate from less than 20 percent in 1980 to more than 80 percent today. In turn, the benefits of increased survival have accrued not just to the individual patient: it is estimated that increased survival has generated more than \$140 billion in societal benefits since 2001, of which more than 90 percent is retained by patients and society.⁵ In fact, estimates suggest that reducing cancer death rates by 10 percent—saving thousands of lives—would also save current and future generations approximately \$4.4 trillion.⁶ Relatedly, assessing value based on a single metric alone—for example, short-term cost to an individual stakeholder—would have a similar impact as it would ignore the benefits of innovation to patients, the healthcare system, and society as a whole.

It is critical that all stakeholders take a holistic approach to assessing the value of innovative therapies to ensure that any proposed changes to the existing healthcare system aimed at improving its overall efficiency and effectiveness result in fostering, rather than impeding, future innovation. Proposed policies that focus on only one element of value—for example, short-term cost—risk unbalancing the ecosystem of the discovery, development, and delivery of innovative biopharmaceuticals. For example, research demonstrates that policies that negatively impact adequate reimbursement for innovative therapies can delay the delivery of these therapies to patients.⁷

³ Yehia, B. R., J. A. Fleishman, P. L. Hicks, M. Ridore, R. D. Moore, K. A. Gebo, and HIV Research Network. 2010. Inpatient health services utilization among HIV-infected adult patients in care 2002-2007. *Journal of Acquired Immune Deficiency Syndromes* 53(3):397-404.

⁴ Alzheimer's Association. Changing the Trajectory of Alzheimer's Disease: How a Treatment by 2025 Saves Lives and Dollars. Section: *Cost of Care*, p. 10, available at: https://www.alz.org/documents_custom/trajectory.pdf.

⁵ Yin, W., J.R. Penrod, J.R. Maclean, D.N. Lakdawalla, and T. Philipson. 2012. Value of Survival Gains in Chronic Myeloid Leukemia. *American Journal of Managed Care* 18(11 Suppl):S257-S264.

⁶ Lakdawala D. N., E. C. Sun, A. B. Jena, C. M. Reyes, D. P. Goldman, and T. J. Philipson. 2010. An economic evaluation of the war on cancer. *Journal of Health Economics* 29(3):333-346.

⁷ Danzon, P.M., Y.R. Wang, and L. Wang. 2005. The impact of price regulation on the launch delay of new drugs—evidence from twenty-five major markets in the 1990s. *Health Economics* 14(3):269-292.

Moreover, the impact of such policies can be exacerbated for smaller biopharmaceutical companies, the vast majority of which are unprofitable.⁸ The ongoing work at these companies represents more than half of the industry's clinical pipeline, including therapies targeting cancer, neurodegenerative diseases like Alzheimer's disease, autoimmune conditions, diabetes, and rare diseases.⁹ These innovators depend on sustained investor funding over the course of many years to deliver technologies to waiting patients, a prospect that can be challenged by potential policies that undermine medical innovation, and thus inject uncertainty into the marketplace. Additionally, any policies that may require these innovators to devote limited resources away from drug discovery and development could similarly result in delaying patient access to improvements in the standard of care or could prevent such advancements from coming to the market at all.

Therefore, the dialogue with regard to maximizing the efficiency and effectiveness of the healthcare system—a goal which BIO broadly supports—must balance this goal with sustaining the innovation ecosystem that has successfully improved the lives of millions of patients. The holistic assessment of the value of innovative therapies is a critical component of such a balance, and BIO looks forward to a continued inclusive dialogue among a broad group of stakeholders, including policymakers, with regard to assessing the value of innovative therapies.

II. Twenty First Century Treatments Need To Be Met by a Twenty First Century Healthcare System.

The foundation of several of the questions posed in the January 21 Letter is the acknowledgement that access to health insurance does not always translate to access to care, especially for some of the sickest, most vulnerable patients. These patients are often subjected to onerously high cost-sharing requirements that delay or effectively deny access to appropriate therapies. This can be the case even after the provider, patient, and insurer agree that a specific therapy offers the highest potential for improving health outcomes. Thus, we urge policymakers to work with a diversity of stakeholders to consider mechanisms to improve patient access to innovative therapies, including, but not limited to, ensuring that existing federal prohibitions on non-discrimination are enforced such that patients are not penalized for characteristics beyond their control.

The reality that access to insurance does not always translate to access to care also is symptomatic of the need for innovative approaches to insuring patients such that they have meaningful access to the treatments most appropriate for them. BIO's *Principles* (see Appendix) reflect this need as well. The *Principles* affirm BIO and our members' commitment to work collaboratively with policymakers and other stakeholders to advocate for the removal of current legal barriers that can stifle innovative approaches to ensuring patient access. For example:

- Manufacturers should be able to communicate product information—including pharmacoeconomic, and other truthful and non-misleading, information—to payors and providers to meaningful inform coverage and reimbursement determinations.
- Policymakers should collaborate with stakeholders to facilitate engagement between manufacturers and payors with respect to novel arrangements that

⁸ Cite pending: BIO internal analysis (2015).

⁹ Cite pending: BIO internal analysis (2015).

may help to ensure patient access to innovative therapies. In particular, stakeholders should consider how to address potential impediments to value-based arrangements, including the uncertainty created by current government price reporting requirements and the federal Anti-Kickback Statute.

Additionally, we note that patients' frequent transitions between plans in certain segments of the health insurance market can prove to be a challenge to a holistic assessment of the value of innovative therapies, insofar as this incentivizes insurers to prioritize short-term, rather than longer-term, benefits and costs. Overall, it is crucial that the healthcare system is able to adapt to ensure that patients are the ultimate beneficiaries of medical innovation.

III. Conclusion

BIO reiterates our appreciation for the opportunity to provide this feedback in relation to the January 21 Letter. We look forward to additional opportunities to work with your offices to strengthen patient access to innovative therapies for them and sustain a healthcare ecosystem that fosters future medical advances. Thank you for your attention to this very important matter.

Sincerely,

/s/

James C. Greenwood
President & CEO