August 12, 2018

The Honorable Mike Kelly
U.S. House of Representatives
Washington, D.C. 20515

The Honorable Markwayne Mullin
U.S. House of Representatives
Washington, D.C. 20515

The Honorable Ron Kind
U.S. House of Representatives
Washington, D.C. 20515

The Honorable Ami Bera, M.D.
U.S. House of Representatives
Washington, D.C. 20515

Dear Representatives Kelly, Kind, Mullin, and Bera, M.D.:

The Alliance for Regenerative Medicine (ARM) appreciates the opportunity to comment on the Health Care Innovation Caucus solicitation for feedback on value-based payment reform and value-based arrangements. The United States health care system's transition to value-based care holds important implications for medical innovation. We applaud the launch of the bipartisan caucus and believe the caucus will bring much-needed leadership to the evolution of value-based models that encourage innovation. As the healthcare industry continues to transition to value-based models, the caucus' leadership will be paramount in exploring and advancing federal policies that promote patient access to life-saving regenerative medicine therapies.

The ARM is a multi-stakeholder advocacy organization that promotes legislative, regulatory, and reimbursement initiatives necessary to facilitate access to life-giving advances in regenerative medicine globally. The ARM comprises more than 290 leading life sciences companies, research institutions, investors, and patient groups that represent the regenerative medicine and advanced therapies community. ARM takes the lead on the sector's most pressing and significant issues, fostering research, development, investment, and commercialization of transformational treatments and cures for patients globally.

The regenerative medicine and advanced therapies sector are the next frontier in the fight against some of humankind's most devastating diseases and disorders. ARM estimates that as of year-end 2017, more than 850 regenerative medicine and advanced therapies developers are sponsoring 946 clinical trials across dozens of indications, including but not limited to oncology, cardiovascular, central nervous system, musculoskeletal, bleeding and blood disorders, connective tissue disorders, metabolic disorders, ophthalmological disorders.

New value-based payment models allow manufacturers to share financial risk with payers and providers to hold all stakeholders accountable for treatment outcomes. These models, critically, can help bridge the gap following Food and Drug Administration (FDA) approval when limited data on the durability of clinical effect is available. They also can help defray the cost density of multiple one-time administered, potentially curative therapies coming to market in a short timeframe, or for such treatments that are indicated for large patient populations. In these ways, value-based payment models can help increase timely patient access to paradigm-shifting technologies.

Accordingly, it is important the Congress consider how value-based arrangements can be used to foster innovation and maximize patient access. Regenerative medicine therapies have the potential to add value to patients' lives and offset significant direct and indirect costs to our nation's healthcare system. In some cases, these therapies can eliminate the need for long-term therapy and further avert downstream costs associated with complications of disease progression (e.g., hospitalizations, physician office visits, surgical interventions). However, to achieve these savings, innovative payment models must be developed to support patient access to transformational regenerative medicine treatments.

ARM encourages Congress to explore and expand opportunities to test value-based payment models for new therapies. To do so will require addressing some barriers to value-based payment arrangements for regenerative medicine therapies. Both legislation and regulation are needed to define voluntary, value-based contracting arrangements properly. The ARM provides more details on the needed regulatory flexibilities below.

1) Ensure that Medicaid Best Price and other drug price reporting requirements do not impede the exploration of value-based contracts between health plans and drug companies.

The Medicaid Best Price law (P.L. 101-508), enacted in 1990, may inadvertently limit the degree to which manufacturers can share risk under value-based arrangements. Consider the following example: under a specific value-based payment model, a manufacturer may agree to provide a 50 percent rebate to the payer if a patient does not achieve a prespecified outcome following administration of regenerative medicine therapy. Under existing regulations, it is possible that the therapy's Best Price would be considered to be 50 percent of the actual cost of the drug if the value-based payment model were not in effect. In turn, this interpretation would require a manufacturer to offer the therapy at a 50 percent to all Medicaid Agencies regardless of whether the value-based payment model was in place. This would likely dissuade a manufacturer from participating in such an arrangement. Similarly, value-based arrangements can distort the reporting of the average sales price for Medicare Part B reimbursements purposes.

ARM encourages Congress to direct CMS to work with stakeholders to provide more flexibility in how Best Price is calculated under a value-based payment model and establish a demonstration that carves-out therapies subject to a value-based payment model from government price reporting requirements.

2) Clarifying safe harbors allowed under anti-kickback rules.

Federal anti-fraud and abuse policies, such as the Stark Law and Anti-Kickback Statute (AKS), complicate the ability of manufacturers to enter into value-based contracts with insurers and providers because such agreements may be viewed as inducing providers to prescribe a particular medication or as incentivizing payers to cover certain drug therapies. Congress and the Office of Inspector General (OIG) of the U.S. Department of Health and Human Services (HHS) should clarify and update these laws' safe harbor provisions to reflect the needs of the changing payment landscape.

3) Support the creation of new value-based payment approaches for regenerative medicine.

Enabling pricing and reimbursement approaches that allow payers, providers, and manufacturers to arrive at value-based arrangements is essential to ensure patients have access to these life-saving therapies. The FDA's new regenerative medicine advanced therapy (RMAT) designation means that more therapies are moving quickly through the approval process and into the market. As a result, payers, including Medicare and Medicaid, should expect to see several new regenerative medicines

available every year. Accordingly, ARM encourages Congress to call upon the Centers for Medicare & Medicaid Services (CMS) and the Center for Medicare & Medicaid Innovation (the Innovation Center) to quickly advance models that allow these regenerative medicine therapies to provide value to patients.

It is also important that multiple payment models are advanced to reflect unique disease and product characteristics. Product characteristics, payer needs, current reimbursement mechanisms and innovator needs may vary case-by-case. There is no "one size fits all" approach. The variety of technologies and approaches will require regulatory flexibility regarding the development and testing of value-based payment models.

Given all the considerations noted above, value-based payment solutions will require significant collaboration between product developers, government agencies, public and private payers, and other stakeholders. There are several potentially viable models for addressing the unique challenges of payment and financing for innovative therapies. For example, one model may include a payment system that enables payment over time based on results or allows multiyear contracts that can travel with the patient between payers. It is critical that multiple models are studied and piloted to accommodate different diseases, patient populations, sites of care, and product characteristics.

Conclusion

The pathway to advancing these remarkable therapies are full of potential challenges including policy limitations. As such, we call on Congress to advance efforts that maximize patient access to medicines that can transform lives. The ARM looks forward to working with the caucus to ensure that the value of regenerative medicine therapies is recognized in payment models. Should you have any questions regarding this response, please contact Robert Falb at rfalb@alliancerm.org or Miranda Franco at miranda.franco@hklaw.com.