Alliance for Regenerative Medicine

January 8, 2021

The Honorable Ami Bera
172 Cannon House Office Building
Washington, DC 20515

The Honorable Robin Kelly
2416 Rayburn House Office Building
Washington, DC 20515

The Honorable Ron Kind
1502 Longworth House Office Building
Washington, D.C. 20515

The Honorable Markwayne Mullin
2421 Rayburn House Office Building
Washington, D.C. 20515

Dear Representatives Bera, Kelly, Kind and Mullin:

We appreciate you reaching out to the Alliance for Regenerative Medicine (ARM) seeking input on addressing the legislative and regulatory hurdles impeding the adoption of innovative payment models for regenerative medicines -- including cell therapies, gene therapies and tissue-based therapies. As the global voice of the sector, ARM is the leading advocacy organization focused on promoting legislative, regulatory and reimbursement initiatives to further the advancement of these transformative therapies and the benefits they offer patients. We commend you for your leadership and welcome your commitment to helping eliminate barriers to further innovation.

Early regenerative medicine products to market have demonstrated profound benefits that are already helping thousands of patients worldwide, many of whom have no other viable treatment options. These life-changing treatments are intended to repair or replace damaged cells, genes, tissues, or organs. They are often delivered in a single administration, have long-term durability and are potentially curative. Hundreds of additional product candidates are contributing to a robust pipeline including more than 1100 clinical trials that are currently underway. Unfortunately, our current fee-for-service healthcare system is outdated when it comes to regenerative medicine. That is why ARM strongly advocates for the adoption of innovative payment models that recognize the unique characteristics of these therapies and the benefits they deliver to patients.

<u>ARM's Innovative Payment Model Principles</u>

ARM has done considerable work to identify and address the legislative and regulatory hurdles to the adoption of innovative payment models for regenerative medicines which are outlined below. This includes a set of principles we urge you to consider as you develop your bill:

- *Overall, the legislative initiative should facilitate the development of innovative contracts between payers, manufacturers, and other stakeholders in the healthcare system.*

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- *The legislation must improve timely patient access to regenerative medicine therapies.*

- *The legislation should be inclusive of all regenerative medicines at a minimum given that the access barriers posed by the current reimbursement paradigm can affect all such therapies (albeit to varying degrees).*

- *The legislation should not unduly restrict adoption of a variety of value-based and installment payment models as "one size" will not fit all.*

- *The legislation must effectively address barriers to government price reporting requirements and federal fraud and abuse prohibitions, at a minimum including, but not limited to, hospital inpatient and outpatient payment methodologies, Medicaid Best Price, Anti-Kickback Statute, Average Manufacturer Price (AMP), and the Stark Law.*

- *The legislative effort takes a targeted approach, making only those statutory changes necessary to accomplish the aforementioned goals and preserving regulatory flexibility for manufacturers and payers.*

Recent Legislative and Regulatory Initiatives

In the last Congress, the Senate Finance Committee approved legislation with language allowing Medicaid to enter into alternative payment arrangements with manufacturers of gene therapies for rare diseases. In addition, last year, several members of Congress initiated separate efforts to craft legislation to address the legislative and regulatory hurdles to the establishment of innovative payment models. Representatives DeGette and Upton announced that they were working on a "Cures 2.0" bill and Representatives Mullin and Guthrie began working on an "outcomes-based agreements" bill. More recently, the Centers for Medicare & Medicaid Services (CMS) finalized a rule that addressed how it would account for these types of arrangements.

CURES 2.0: In their Cures 2.0 concept paper released in the Spring of 2020, Representatives DeGette and Upton included a specific request for feedback from interested stakeholders on the "barriers that impede or otherwise slow coverage for new cell and gene therapy products." In response to this request, ARM provided recommended legislative language (enclosed) to address Best Price, AMP, 340B, the Anti-Kickback Statute and the Stark Law.

"Outcomes-Based Agreements" Draft Bill: At the end of October, Representatives Mullin and Guthrie unveiled their "Outcomes-Based Agreements draft bill. Overall, ARM was supportive of their approach and recommended several minor modifications. A copy of our letter is enclosed.

CMS Final Rule: Last summer, CMS published a proposed rule that among other things, addressed how the agency would consider "value-based purchasing"(VBP) arrangements in the context of state Medicaid programs. Enclosed is a copy of ARM's comments on the proposal.

On December 21, 2020, the agency released the final rule. Overall, ARM is pleased that it provides for significant flexibility for manufacturers, state Medicaid programs and other payers to develop and design these agreements. We also support the broad definition of innovative payment models and concur with the agency that "it allows manufacturers and payers to develop, structure and implement these contracts in an ever-evolving health care environment, as well as allow manufacturers and payers to consider future changes in the scope and nature of such arrangements."

Regarding best price reporting, under the new rule, manufacturers that enter into an innovative payment agreement may report a "best price" that includes varying price points for a single dosage form and the strength. ARM was supportive of this approach. While CMS acknowledged the challenges presented by the Anti-Kickback Statute and the Stark Law, it deferred from taking any action at this time but wrote that it is considering whether additional guidance may be necessary in the future. ARM would support appropriate legislation to address the Anti-Kickback Statue and Stark Law impediments.

In conclusion, the Food and Drug Administration has indicated that it expects to be approving 10-20 cell and gene therapies on an annual basis starting as soon as 2025. It is vital that the current statutory and regulatory obstacles that hinder widespread patient access to these transformative therapies be remedied. ARM looks forward to working with you to advance legislation that will achieve this goal.

Sincerely,

Robert J. Falb
Director, U.S. Policy and Advocacy

Enclosures