Alliance for Regenerative Medicine

The Alliance for Regenerative Medicine ("ARM") appreciates the opportunity to provide comments to the Mullin-Schrader-Guthrie Amendment, as offered in the Energy and Commerce Committee markup of HR 3 on October 17, 2019 (the "MSG") as you continue to address barriers to the adoption of value-based payment arrangements both for public and commercial payers.

About the Alliance for Regenerative Medicine

ARM is an international multi-stakeholder advocacy organization that promotes legislative, regulatory, and reimbursement initiatives necessary to facilitate access to life-giving advances in regenerative medicine worldwide. ARM comprises more than 350 leading life sciences companies, research institutions, investors, and patient groups that represent the regenerative medicine and advanced therapies community. The regenerative medicine and advanced therapies sector is the next frontier in the fight against some of our most devastating diseases and disorders.

The Importance of Value-Based Arrangements

As you know, value-based arrangements serve to link payments to performance in ways that account for both the cost and impact on patient outcomes. The most basic value-based purchasing model may function essentially as a "money-back guarantee," where the cost of the treatment would be refunded if the treatment does not meet certain committed levels of efficacy for a particular patient or group of patients. A variation on this approach is a model in which there is an initial or discounted payment upfront when the therapy is first administered, with continued evaluation of clinical outcomes and other measures to determine future payments for the remaining cost of the treatment. Both of these options present significant benefits when such treatments otherwise require a higher upfront investment for a one-time treatment, when in fact the eventual cost savings for a curative therapy accumulates over time. It is important that legislation helping to define value-based payment arrangements provide appropriate flexibility to reflect the variety of potential value-based agreements between innovators and payers.

Recommendations for Further Refinement of the Mullin-Schrader-Guthrie Amendment

Below please find the major provisions included in the MSG along with the identification of certain elements that we believe need to be modified in order to ensure that value-based payment arrangements are crafted in a way to help provide patients with greater access to these groundbreaking therapies. These recommendations are provided in the order they appear within the current version of the MSG. We also note certain recommended revisions to existing language in blue for ease of reference.

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1. Actuarial Certification of Net Federal Spending

Value-based payment arrangements enable and facilitate access to new, potentially curative therapies while solving for healthcare system sustainability concerns surrounding the ability to bear upfront costs for such treatments. It is thus critical that the approval process for Medicaid value-based payment arrangements does not impair or penalize beneficiary access. In particular, the proposed actuarial certification of "net Federal spending" does not address the possibility of an increase in net Federal spending resulting from an increase in a drug's utilization due to improved beneficiary access, even if the price of that drug has actually decreased due to value-based payment arrangements. In addition, ARM remains concerned that the actuarial certification process to evaluate net federal spending is likely to unduly delay patient access to life-saving drug therapies. ARM respectfully recommends deleting **Line 17 on Page 2 to Line 4 on Page 4** accordingly.

2. Launch and List Price Justification

ARM recommends that this provision requiring documentation in support of drug launch and list prices be struck from the MSG. This requirement would pose a substantial administrative burden on both manufacturers asked to present such data as well as to CMS, which would be compelled to develop and maintain a standardized method for reporting such information when each manufacturer considers such a wide range of financial and economic factors often unique to that particular developer. Launch and list prices also do not take into account applicable discounts, rebates or price concessions that decrease the actual costs that a patient may be charged, and as such, may unintentionally inhibit patient access to drugs by reporting only the non-discounted price. We respectfully suggest that the provisions set forth in **Lines 5 to 15 of Page 4** be deleted.

3. Consultation with the Commissioner of Food and Drugs

The Secretary, when considering whether to approve a risk-sharing value-based payment agreement, is required to consult with the FDA Commissioner "to determine whether the relevant clinical parameters" in the agreement are appropriate. This provision should be struck as it is overly bureaucratic and would not only unnecessarily delay approval if every parameter for every contract executed with each state Medicaid program would need to be reviewed but it also restricts state flexibility. We respectfully recommend deletion of **Lines 14 to 21 on Page 6** in its entirety, and to strike reference to the Commissioner of Food and Drugs on **Lines 1 to 2 on Page 11**.

4. Early Mortality

ARM believes that "early mortality," as referenced in the MSG as a factor in defining a "potentially curative treatment intended for one-time use" is vague and needs to be refined. For some diseases, early mortality is not necessarily an expected outcome, as current chronic care therapies may help patients manage one illness or disease while other, sometimes multiple, comorbidities continue to impact the patient's well-being and quality of life. Moreover, the use

of the term "early mortality" suggests that these therapies will ensure that patients suffering these diseases will have the same life expectancy as the general population. Unfortunately, this is not a realistic expectation, as a therapy may extend a patient's life by many years but still not result in a patient reaching 79 years, which is the average life expectancy in the U.S. as of 2018. We respectfully suggest modification to the definition of the term "potentially curative treatment intended for one-time use" at **Lines 13-17 of Page 24** as follows to include therapies that prevent, eliminate or halt the progression of comorbidities or of the disease itself:

> "(II) a reduction in the symptoms of such disease or condition, or any of the patient's comorbidities to such disease or condition, to the extent that such drug is expected to extend the patient's life expectancy or prevent, eliminate or halt progression of the disease, condition or comorbidity; and"

5. Achievement of Result After Not More than 3 Administrations

The current definition of a "potentially curative treatment intended for one-time use" includes a clause stating that a desired result for the treatment "may be achieved over an extended period of time, after not more than 3 administrations." The inclusion of a "cap" of not more than 3 administrations is arbitrary and may not reflect the treatment plans required across a wide range of therapies. For example, if a potentially curative therapy requires treatment in both eyes, the use of this specific threshold calls into question whether each administration for each eye counts towards this limit. In addition, at this point in the development of new technologies, it is difficult to estimate whether a given curative therapy requires less than 3 administrations, versus 4 or 5. We understand that there is a benefit to including a specific threshold to help define what constitutes a "one-time use," and that any follow-up or "booster" therapies that may be required some time after the initial treatment should not be included in this definition. We respectfully suggest instead that the definition at **Line 21 of Page 24** be revised to read that the result of the treatment "may be achieved over time, after not more than 3 administrations or as otherwise administered for non-chronic use."

6. Relevant Clinical Parameter

The "relevant clinical parameter" definition should be modified to allow for additional determinants beyond those stated in the provision, *i.e.* clinical endpoints in the label (or compendia) and surrogate endpoints. Specifically, it should be expanded to include clinical parameters agreed upon by the developer and state Medicaid agencies as part of the negotiated, value-based agreement. This change is needed to ensure that state Medicaid programs have the maximum amount of flexibility to negotiate and tailor agreements with developers to the specifics of the curative therapy in question, their patient population, and their budgetary constraints. Traditionally utilized endpoints may not be the most appropriate measure given the revolutionary nature of these therapies, especially for those in which there is currently no approved treatment. In addition, these arrangements may contemplate certain patient-reported outcomes or measures beyond what may be set forth in clinical or surrogate endpoints, and the statute should accommodate such flexibilities.

We respectfully suggest that a subclause be added after **Line 2 of Page 26** of the MSG as follows:

> "(iii) any other clinical and/or non-clinical, value-based metric, measure or outcome set forth in the agreement where the manufacturer is required to provide a rebate or other price concession based on the occurrence or non-occurrence of such clinical metric, measure or outcome, or where full payment for the drug is conditioned on the occurrence or non-occurrence of such clinical metric, measure or outcome."

7. Use of Relevant Clinical Parameters in Risk-Sharing Value-Based Payment Agreement

ARM asks that the requirement that a risk-sharing value-based payment agreement be conditioned on "at least 2 relevant clinical parameters" be struck from the section given the practical reality that not all eligible conditions or diseases (or the eligible regenerative medicines that treat them) will have 2 associated meaningfully relevant clinical parameters. Through this legislation, Congress should focus on creating the flexibility state Medicaid programs and developers need to implement a myriad of alternative payment models that are fit-for-purpose to the specifics of a patient population (or sub-population) and eligible therapy. This provision, as written, artificially establishes a threshold (*i.e.,* 2 relevant clinical outcomes) that appears arbitrary and may be at odds with the scientific and clinical evidence available at launch, undermining the intention to provide flexibility to the detriment of payer autonomy and, ultimately, patient access. As such, we respectfully suggest deletion of the phrase "at least 2" from **Line 17 of Page 26** and **Line 23 of Page 33**.

8. Flexibility for States to Construct the Best Possible VBA

The definition of "risk-sharing value-based payment agreement" under Medicaid should be modified to allow for the needed flexibility for States and manufacturers to design the best possible agreement able to address the issue at stake for a specific product, patient population and/or to fit their specific financial and resourcing needs. The proposed change should clarify that not all payments under such an "risk-sharing value-based payment agreement" need to be "installment" payments which are currently defined as payments tied to outcomes. In particular, it should be allowed for some payments under a risk-sharing value-based payment agreement not to be linked to outcomes (e.g. it should be possible to have an initial payment made at time of therapy administration with only subsequent payments tied to outcomes). Other risk-sharing options, such as payment for on-going health care costs tied to outcomes not achieved, should also be permitted if designed and agreed upon by the parties.

We respectfully suggest modification of subsection (E)(iii) of the MSG from **Lines 12-19 on Page 26** as follows:

> ''(ii) under which payment for such drug shall be made pursuant to an installment-based payment structure that meets the requirements of paragraph (3), or otherwise pursuant to a risk-sharing design agreed upon by the State plan and a manufacturer;

"(iii) which conditions some or all payments on the achievement of relevant clinical parameters (as defined in subparagraph 19(D));"

We also recommend further conforming changes to reflect that (1) not all payments under a risk-sharing value-based payment agreement need to be installment payments, and (2) not all installment payments need to be tied to patient outcomes. These changes include the following:

- **Line 4 on Page 7** should be modified to strike the word "equal" in "equal installments."
- **Lines 15-16 on Page 23** should be modified to read "during which a covered outpatient drug is subject to the agreement."
- **Lines 13-14 on Page 27** should be modified to read "the unit price of the drug charged under the agreement multiplied by the percentage of such price that is subject to installment payments."
- **Lines 15-16 on Page 27** should be modified to read "the number of units of such drug subject to the agreement during such installment year."

9. Best Price ("BP") and Average Manufacturer Price ("AMP") Reporting Requirements

ARM recognizes that BP and AMP are important tools to ensure that Medicaid and other federal health programs, such as the 340B Drug Pricing Program, are able to take advantage of discounts and price concessions available to other payers. However, the regulations governing the calculation and reporting of AMP and BP were written in an era that did not contemplate the scientific advances that are now possible as a result of regenerative medicines, nor the economic considerations that come with these developments. Thus, these regulations pose an inadvertent but significant hurdle that is forestalling the adoption of alternative payment models, both in the commercial sector and in government programs.

We appreciate the effort made to address this problem in the MSG, however, we believe certain elements to address these barriers can be simplified. Regarding BP, we support a methodology that disregards the "adjusted price" calculation as currently drafted in MSG in favor of a more straightforward approach. BP should instead exclude discounts and price concessions provided exclusively as a result of the failure of measured patient outcomes negotiated by developers and payers as part of a value-based payment arrangement, whether based on the outcomes of an individual patient's therapy and/or the results of a patient population or sub-population. Similarly, AMP calculations should utilize this same direct approach. It is important to note that addressing BP in this manner would ensure that there are no anomalies in Medicare Part B reimbursement without further amendment to Title XVIII of the Social Security Act, as current regulations require any exclusions from BP to also be excluded from the calculation of average sales price (ASP), the payment methodology for drugs administered under the Medicare Part B benefit.

We respectfully suggest that the proposed revisions as set forth in **Line 21 of Page 28 to Line 16 of Page 32** of the MSG be replaced with the following:

(a) DEFINITION OF BEST PRICE.—Section 1927(c)(1)(C) of the Social Security Act (42 U.S.C. 1396r-8(c)(1)(C)) is amended—

(1) in clause (i)—

(A) in subclause (V), by striking "and";

(B) in subclause (VI), by striking ".", and inserting "; and";

(C) by adding at the end the following new subclause:

"(VII) in the case of a covered outpatient drug that is a potentially curative treatment intended for one-time use (as defined in subsection (l)(11)(C) and is sold under a value-based payment arrangement (as defined in clause (iv)) during a rebate period, any prices resulting from a discount, rebate, refund, or other price concession from a manufacturer or a third party on behalf of a manufacturer due to the failure of a patient or a patient population to achieve the outcomes specified in such agreement as a condition of payment."; and

(2) in clause (ii)—

(A) in subclause (III), by striking "and";

(B) in subclause (IV), by striking ".", and inserting "; and";

(C) by adding at the end the following new subclause:

"(V) in the case of a value-based payment arrangement (as defined in clause (iv)), shall be determined as if the aggregate price per the terms of such arrangement was paid in full at the time of the first payment under the arrangement, and excluding any value-based discount, rebate, refund or other price concession that may be provided under the arrangement."; and

(3) by adding at the end the following new clause:

"(iv) VALUE-BASED PAYMENT ARRANGEMENT. — The term 'value-based payment arrangement' means an agreement between a manufacturer of a covered outpatient drug that is a potentially curative treatment intended for one-time use (as defined in subsection (l)(11)(C)) and a purchaser of such drug under which—

"(I) the manufacturer is required to provide a discount, rebate, refund or other price concession to the purchaser based on the occurrence or nonoccurrence of 1 or more outcomes specified in the agreement; or

"(II) full payment for the drug is conditioned on the occurrence or non-occurrence of 1 or more outcomes specified in the agreement.".

(b) DEFINITION OF AVERAGE MANUFACTURER PRICE.—Section 1927(k)(1) of the Social Security Act (42 U.S.C. 1396r–8(k)(1)) is amended—

(1) in subclause (B)(i)(IV), by striking "; and" and inserting a semicolon;

(2) in subclause (B)(i)(V), by striking the period at the end and inserting "; and";

(3) by adding at the end the following new subclause:

"(VI) payments made to, or discounts, rebates, refunds, or other price concessions provided by, manufacturers or third-parties on behalf of manufacturers for covered outpatient drugs that are potentially curative treatments intended for one-time use (as defined in sub-section (l)(11)(C)) under a value-based payment arrangement (as defined in subsection (c)(1)(C)(iv))."; and

(4) by adding at the end the following new subsection (D):

"(D) VALUE-BASED PAYMENT ARRANGEMENTS. In the case of a value-based payment arrangement (as defined in subsection (c)(1)(C)(iv)), the average manufacturer price for a covered outpatient drug shall be determined as if the aggregate price per the terms of such arrangement was paid in full at the time of the first payment under the arrangement, and excluding any value-based discount, rebate, refund or other price concession that may be provided under the arrangement.".

Finally, ARM recommends that all necessary statutory cross-references be made throughout this section to explicitly ensure that best price reporting provisions and average manufacturer price provisions addressed in these sections only apply to those value-based payment arrangements that meet the requirements detailed in the new subsection 1927(l).

10. Consideration for Curative Therapies Administered on an Inpatient Basis

We ask that due consideration be afforded for covered inpatient drug therapies that are curative in nature by at least installing similar protections under the federal Anti-Kickback Statute and the Stark Law for inpatient drugs subject to value-based purchasing agreements.

We respectfully suggest an additional provision to be included after **Line 10 of Page 34** as follows:

"(M) an agreement between a State plan and a manufacturer for the purchase of a covered drug of the manufacturer that is administered on an inpatient basis and that otherwise meets the definition for a risk-sharing value-based payment arrangement set forth in section 1128(b)(3)(L), and that otherwise satisfies the conditions set forth in subsection (L)."

We also suggest that the provision set forth in **Lines 15 to 17 of Page 34** of the MSG be revised as follows:

"(iv) Any amounts determined under a risk-sharing value-based payment arrangement described in section 1128(b)(3)(L), or other agreement between a State plan and a manufacturer for the purchase of a covered drug of the manufacturer that is administered

on an inpatient basis and that otherwise meets the definition set forth in that section 1128(b)(3)(L)."

10. Effective Date

The regenerative medicine pipeline is robust. There is the potential for several FDA marketing approvals this year alone. FDA stated last year that that they have over 800 cell and gene therapies INDs and that they expect to have more than 200 INDs filed per year. Many patients cannot afford to wait for these therapies given the progression of their disease and prognosis. In addition, we believe that the recommendations made herein to streamline the use of value-based purchasing arrangements in Medicaid while allowing maximum flexibility for the states will help ensure that legislation may be implemented more quickly following its passage. Therefore, ARM recommends that the effective date should be upon enactment and not delayed several years after it becomes the law of the land. We respectfully suggest deletion of Section (d) of the MSG from **Lines 18-23 on Page 34**, and deletion of any other references to January 1, 2022 and March 31, 2022 as incorporated within the MSG.

Thank you for your consideration and please do not hesitate to contact Robert Falb, Director of U.S. Policy and Advocacy rfalb@alliancerm.org if you have any questions.