AMERICAN SOCIETY of GENE & CELL THERAPY

July 13, 2018

The Honorable Alex M. Azar II
United States Secretary of Health and Human Services
U.S. Department of Health & Human Services
200 Independence Avenue, S.W.
Washington, D.C. 20201

Dear Secretary Azar:

The American Society of Gene & Cell Therapy (ASGCT) appreciates the opportunity to respond to the request for information from the Department of Health and Human Services (HHS or the Agency) on the issue of lowering drug prices. ASGCT is the premier membership organization consisting of scientists, physicians, and other professionals involved in gene and cell therapy. The Society's mission is to advance knowledge, awareness, and education leading to the discovery and clinical application of genetic and cellular therapies to alleviate human disease.

Considerations for Gene Therapies – Patient Access to Valuable, Cost-Effective Therapies

The Society urges the Agency to consider the high value and cost-effectiveness of gene therapies (including gene-modified cell therapies) when discussing pricing. Gene therapy is a radical shift in our approach to disease treatment. By modifying the expression of a patient's genes or repairing abnormal genes, gene therapy often addresses the root cause of diseases. While several gene and cell therapies have received FDA approvals over the past 20 years, the field has recently experienced a turning point. Three gene therapies were approved for human medical use in the U.S. in 2017,[1-3] including the first in the country for an inherited condition, and many more are likely on the way.[4]

These recently approved gene therapies offer substantial benefits to patients who otherwise have little to no hope of cure or even meaningful improvement. They treat certain instances of non-Hodgkin lymphoma, acute lymphoblastic leukemia (ALL), and a hereditary genetic defect that nearly always leads to blindness. Each is a potentially one-time treatment—just a single infusion—that may provide long-term, durable efficacy.

While assigning a specific financial value to these treatments is challenging, their life-altering and sometimes lifesaving benefits are exceptional. In addition, the Institute for Clinical and Economic Review (ICER) has deemed CAR T-cell therapies to be cost-effective[5] for certain types of ALL and lymphoma and has indicated that if a societal perspective is used, for a younger population, Luxturna is also likely to be cost effective compared to standard of care.[6]

Another factor to consider in addressing the costs of gene therapies is that they are not uniquely costly for high-value treatments. For example, the average cost of a heart transplant is $1.4 million, while the three gene therapies approved in 2017 range in price from $373,000 to $850,000.

Moreover, the potential for gene therapy to have a durable effect from a one-time treatment could have substantial cost savings in some instances. For example, the average lifetime cost for treatment of a person with hemophilia is nearly $12 million (not accounting for inflation).[7] If the current clinical trials for gene therapy for hemophilia proceed to an FDA approval, the cost savings could be immense. When addressing the effects of these therapies on the health care system, ASGCT respectfully requests the consideration of both added costs and the potential for cost savings.

The accessibility of gene therapies to patients is of paramount importance to ASGCT. Therefore, the Society is willing to explore a variety of potential solutions to barriers to patient access, including pricing, upfront costs, reimbursement, regulations, and payment models. Further discussion will be necessary about the appropriate balance of fair pricing determinations and continued stimulation of innovation. Because FDA-approved gene therapies are already in use, provide high value to patients, are cost effective in many instances, are not uniquely costly, and have the potential to create immense cost savings for certain future applications, initial ASGCT priorities at this time include support of, and further exploration of, reimbursement policies and novel payment models that encourage patient access to these treatments, outlined below.

Response to Solicitation of Comments

Additional Feedback on Accessibility of Prescription Drugs. Gene therapies typically involve a single administration of treatment, so a potential challenge to payers is the one-time upfront cost for treatment, rather than treatments that are administered multiple times over the course of time. Many payment models have been proposed to enable patient access while addressing payer ability to cover upfront costs.[8,9]

Payer-preferred solutions include value-based contracts that share risk, in which the payer's exposure is reduced or eliminated if a patient does not respond to treatment; contracts that offer installment payments spread out over time; and risk pools, to provide insurers with a resource to which they all contribute and that serves to support them when a patient's medicine costs exceed a certain threshold.[9-11] In risk pooling, private payers, budget holders, employers and/or state governments would put a certain percent of their premiums or health care budget into a dedicated fund for specified high value medicines. If a patient's medicine costs exceed a certain predetermined threshold, monies would be paid out from this fund.[9]

ASGCT supports further exploration of the following in terms of feasibility of implementation, enhanced patient access, and risk reduction for a variety of stakeholders:

- Value-based payment models, including their ability to produce cost savings for gene therapies.
- Payment over time, with payments spread out over a defined number of years.
- A high-risk pool to limit exposure by any one payer or budget holder.

Addressing Medicare and Medicaid Reimbursement Challenges. The request for information states under section IIIB, "regulations governing how Medicare and Medicaid pay for prescription drugs have

not kept pace with the availability of new types of drugs, particularly higher-cost curative therapies intended for use by fewer patients."

Limitations specific to current Medicare and Medicaid reimbursement policies have become apparent with newly approved gene therapies. Medicaid is the single largest health insurer of U.S. children, especially those with special health needs,[12] which is relevant for both Kymriah and Luxturna patient populations, and approximately 56 percent of new cases of non-Hodgkin lymphoma are in patients of Medicare age (65+).[13]

For CAR T-cell therapies, existing claims submission and reimbursement processes are leaving hospitals facing vast financial losses for direct expenses. ASGCT appreciates the recent CMS invitation of public comment on payment options and alternatives to enhance Medicare reimbursement of CAR T-cell therapy. In its comments to the IPPS proposed rule for FY 2019, ASGCT recommended assignment to MS-DRG 016 (an existing higher weighted DRG, currently used for Autologous Bone Marrow Transplant with CC/MCC), with the acquisition cost (average sales price) paid as a pass-through payment.

Regarding Medicaid program determinations, the New York State Medicaid fee-for-service (FFS) program provides an example of how to sufficiently reimburse for the three new gene therapies, by reimbursing facilities for the drug in addition to the bundled payment for services.[14-16] ASGCT encourages the adoption of similar payment policies by state Medicaid programs, when possible, to sufficiently cover the costs of both the gene therapy and the cost of services to administer the therapy. The Society would also welcome the exploration of additional ways to sufficiently reimburse gene therapy for Medicaid beneficiaries.

In addition, some of the potential solutions mentioned above to address reimbursement of upfront costs are limited by the Medicaid Best Price program, which requires drug manufacturers to give Medicaid the best price given to any other purchaser (by providing it with a mandatory rebate of 23.1 percent of the average manufacturers' price or, if another purchaser is offered a greater rebate, that greater rebate amount.[17]) Value-based payment agreements and long-term financing models may be prevented by Medicaid Best Price requirements because, if a manufacturer accepts a reduced or installment payment that is lower than the price it gave a Medicaid program, a new best price could be established at just a fraction of the actual price set by the manufacturer. ASGCT supports exploration of modifications to Medicaid Best Price requirements that could facilitate the development of value-based payment arrangements, to assess the ability of such arrangements to affect costs. ASGCT supports tying payment to treatment outcomes—with lower costs being incurred for less effective individual patient results—as a voluntary option for manufacturers.

Continuing the Conversation

On September 24, 2018 in Washington, DC, the Society is hosting the ASGCT Value Summit: *Advancing Patient Access to the Benefits of Gene Therapy,* to continue discussions of these issues. The goals of the summit are to discuss current efforts and proposals to enhance patient access to the value of approved therapies; and to identify shared priorities of multiple stakeholders for solutions to barriers to patient access that may benefit from further action, support, exploration, and/or expansion. Groups to be invited include health policy experts, academics, pharmaceutical companies,

governmental representatives, payers, patient advocacy and other nonprofit organizations, and clinical providers. ASGCT welcomes you and your staff to attend.

The Society appreciates this opportunity to comment on the topic of accessibility of prescription drug treatment. Patient access to FDA-approved transformative, and sometimes lifesaving, gene and cell therapies is a priority for ASGCT. Please let us know if you have questions.

Sincerely,

Michele Calos, PhD
President

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