ALLIANCE for Regenerative Medicine

September 20, 2017

The Honorable Lindsey Graham
United States Senate
Washington, DC 20510

The Honorable Bill Cassidy
United States Senate
Washington, DC 20510

The Honorable Dean Heller
United States Senate
Washington, DC 20510

The Honorable Ron Johnson
United States Senate
Washington, DC 20510

Dear Senators Graham, Cassidy, Heller, and Johnson:

As CEO of the Alliance for Regenerative Medicine (ARM), a multi-stakeholder advocacy organization comprising more than 270 leading life sciences companies, research institutions, medical centers, investors, and patient groups that make up the broader regenerative medicine community, I am writing to express ARM's concerns regarding the *American Health Care Act*, (H.R. 1628).

Specifically, as the leading organization representing companies developing cell and gene therapies and other regenerative medicine products with the intention to treat and potentially cure currently unmet medical needs, ARM is concerned that the Act will limit Medicaid beneficiaries' access to the potentially life-changing and curative therapies that they need.

Regenerative medicine products, including cell and gene therapies and tissue engineering products (broadly termed "advanced therapies"), represent innovative approaches to potentially curative medical treatments and are likely to address many of the most burdensome, difficult to treat, and costly diseases in the United States. Many of these treatments are expected to deliver a profound and durable therapeutic benefit, in some cases following only a single administration. Unfortunately, the structural changes to the Medicaid program in your current legislation pose a risk to patients seeking access to these therapies. In addition, reductions in the Medicaid program budget could impede innovation of new therapies in the regenerative medicine field, especially for rare diseases, which is already highly complex, costly, and high risk for innovator companies.

For instance, a shift to a per-capita-cap or block grant system in Medicaid would severely limit the resources necessary for states and the providers within them to properly care for the sickest patients in their communities. This would disproportionately impact medically complex patients, including adults and children with rare genetic diseases, as well as those suffering cancer and other life-threatening conditions.

The block grant and per-capita-cap programs proposed under the current version of H.R. 1628 would not adequately account for the increased costs needed to care for these medically complex patients. This, in turn, would force state Medicaid programs into the untenable position of limiting or delaying patient access to the best treatments available based on short-term cost concerns. Not only would this situation risk patients' health but it also could lead to higher, long-term, overall healthcare costs (e.g., from increased hospitalizations, emergency department visits, surgical interventions).

Ultimately, establishing systematic barriers to patient access to new curative therapies through a transition to block grant and/or per-capita-cap programs will negatively impact investment in research and development of these transformative treatments in the future.

Further, provisions in the bill that allow states to waive coverage for essential health benefits, could also have significant, negative implications for patient access to gene and cell therapies. Implementation of such waivers could have far-ranging consequences for those with complex medical conditions and would adversely impact access to needed treatments for serious and costly diseases by altering what categories of benefits insurers must cover, including the prescription drugs covered under these categories.

Given budget constraints imposed by per-capita-caps and block grants, state Medicaid agencies may be forced to limit or deny access to the lifesaving and life-improving rare disease therapies for the very patients that Congress intended Medicaid to help. These restrictions, especially when combined with a Medicaid system that struggles to adapt to providing access to these therapies, will restrict patient access and impede the development of new innovative regenerative medicine products.

Although H.R. 1628 as written would not retain the protections needed to ensure these patients have access to the therapies they need, including high-value, curative therapies, ARM is working with key stakeholders to define and propose new payment and financing models that could better address payer, provider, patient, and innovator needs. For example, previous legislation on this topic established separate eligibility categories for certain high cost beneficiaries, and in that context, ARM had suggested a category for beneficiaries with medically complex conditions.

As Congress continues to deliberate changes to the American healthcare system, our organization looks forward to serving as a resource to you.

Sincerely,

Janet Lynch Lambert
CEO, Alliance for Regenerative Medicine