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March 22, 2018

The Honorable Alex Azar  
Secretary, U.S. Department of Health and Human Services  
200 Independence Avenue, SW  
Washington, DC 20201

The Honorable Seema Verma  
Administrator, Centers for Medicare & Medicaid Services  
U.S. Department of Health and Human Services  
7500 Security Boulevard  
Baltimore, MD 21244

Re: Medicare Beneficiary Access to Innovations in Cancer Treatment

Dear Secretary Azar and Administrator Verma:

Surgery, chemotherapy, and radiation therapy have historically been the foundation of cancer treatment. However, advances in the fields of precision medicine and immunology have drastically changed the way we treat patients. The Leukemia & Lymphoma Society (LLS) is the world's largest voluntary health agency dedicated to blood cancer. Our research investment has played a critical role in advancing the field of immunotherapy, as well as bringing novel cell-based therapies to patients. In particular, LLS has taken a leading role in advancing new precision medicine-based treatments for acute myeloid leukemia (AML) and has been instrumental in the development of Chimeric Antigen Receptor T Cell (CAR-T) Therapy.

However, our efforts on behalf of blood cancer patients do not stop at the scientist's bench. We are also committed to ensuring that groundbreaking innovations in cancer treatment are available to the patients who need them most, in a timely manner. Given the complexity of many innovative therapies, as well as the significant costs to the healthcare system as a whole and Medicare in particular, LLS understands the challenges inherent in incorporating these new technologies into the current coverage and payment system.

LLS is very concerned that existing procedures and timing for incorporating new treatments and technologies into the Medicare inpatient coverage and payment systems are outdated, resulting in unacceptable delays for patient access to these life-saving treatments. We urge the agency to modernize the current inpatient payment system so that these potentially life-saving treatments are available to those who need them most.

#### **LLS Master Protocol for Biomarker-Based Treatment of AML**

As mentioned above, LLS has been involved in funding research for decades. Over the past 40 years, there have been few advances in the treatment of AML – with today's therapies, only one in four AML patients survives five years after diagnosis. In the United States, AML takes the lives of more than 10,000 people each year. To address this unmet urgent medical need, LLS is leading the offensive against AML through our Master Protocol for Biomarker-Based Treatment of AML, a collaborative precision medicine approach. This is the first clinical trial

of its kind in a blood cancer and involves multiple medical institutions, pharmaceutical companies and the FDA, all of whom have committed to working together to drive this master clinical trial forward. As with any research project, the goal is to develop and accelerate treatments and cures for patients. In this case, our hope is that one or more innovative treatments will emerge from this trial – products that will advance cures for AML patients. The structure of the Master Protocol is such that new study arms are opened on an ongoing basis and any one of these study arms could include a treatment that may require extended inpatient stays. Furthermore, since AML is a disease that mainly afflicts the elderly who often require lengthy hospitalizations, an updated, modernized Medicare coverage and payment system will benefit current and future Medicare beneficiaries.

### **Chimeric Antigen Receptor T Cell (CAR-T) Therapy**

LLS recognized early the promise of CAR-T, a treatment in which a patient's T-cells are genetically reprogrammed to find and kill cancer cells. Over the past two decades, LLS has provided \$40 million in funding for more than 15 researchers and companies around the world to develop this revolutionary approach to treating cancer. Two types of CAR T-cell immunotherapy were approved by the FDA in 2017, and early outcomes from CAR-T trials have generated impressive results in patients with blood cancers. LLS is continuing to fund the next generation of CAR-T therapy, as we anticipate that this method will be useful in many different blood cancers, and perhaps even other major tumors that are difficult to treat, such as brain, lung, and colon cancer.

Most patients receiving CAR-T are critically ill and have received multiple prior therapies. Due to the extensive facility and provider capabilities that must be immediately available during and after treatment, CAR-T is most safely administered in the inpatient setting. The very high cost of personalized CAR-T, as well as the additive cost of hospital services needed to provide the therapy, are not supported by current Medicare inpatient coverage and payment structures. This has led to payment delays and a lack of access to this potentially life-saving treatment.

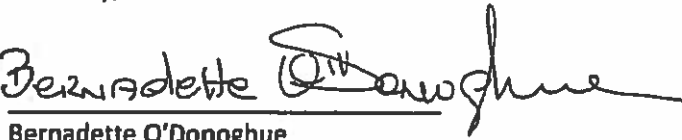
The LLS Information Resource Center (IRC), a call center staffed with healthcare professionals equipped to respond to patient questions regarding blood cancers, has heard from Medicare beneficiaries who have been denied access to CAR-T due to coverage and payment hurdles. In one case, a patient was placed on the waiting list for CAR-T, yet the hospital was unwilling to move forward with treatment because they did not receive a response from the Centers for Medicare and Medicaid Services (CMS) with respect to the amount of reimbursement. Another Medicare beneficiary was medically cleared to receive the therapy, but was then told by the hospital that he would have to pay \$850,000 for the treatment – a prohibitive cost for the patients' family. In another case, a patient who had failed three prior therapies was accepted on a CAR-T trial. The hospital approved her enrollment but CMS refused reimbursement, leaving the patient with no further options.

While LLS is very proud of its involvement in advancing treatments for patients and excited about the medical promise of CAR-T, finding cures is only one aspect of our mission. Cures without access does not benefit patients; we remain committed to ensuring that CAR-T is accessible to the patients who need it most in a timely manner.

In summary, novel therapies and disease-altering developments, such as CAR-T, hold significant promise for changing the way that cancer is treated. LLS believes that the Medicare coverage and payment system should be modernized to facilitate access and to keep pace with scientific innovation. Accordingly, LLS is eager to work with the administration to modernize the current system of coverage and payment to ensure that Medicare beneficiaries can access innovative therapies.

If you have any questions please reach out to Liza Holder, Director, Policy at [liza.holder@lls.org](mailto:liza.holder@lls.org) or me at [bernadette.odonoghue@lls.org](mailto:bernadette.odonoghue@lls.org).

Sincerely,

A handwritten signature in black ink that reads "Bernadette O'Donoghue". The signature is written in a cursive style with a large, stylized initial "B".

**Bernadette O'Donoghue**  
Vice President, Office of Public Policy  
The Leukemia & Lymphoma Society