



Dear Senator Cassidy,

The Society for Immunotherapy of Cancer (SITC) appreciates the opportunity to comment to the U.S. Senate Committee on Health, Education, Labor, and Pensions regarding rare diseases.

SITC is the world's leading member-driven organization specifically dedicated to improving cancer patient outcomes by advancing the science, development and application of cancer immunology and immunotherapy. For decades, SITC members have been at the forefront of cancer immunotherapy research working to develop and advance novel therapeutics into the clinic for the treatment of various cancers including those considered as rare disease.

Immunotherapy has served as a prudent example of a class of therapies that has a broad range of application against potentially all cancers. Innovations in therapeutically harnessing one's own immune system to create effective, durable responses has created a paradigm shift throughout the field of oncology and has saved numerous patient lives. While many of the immunotherapeutic breakthroughs have occurred in more common disease settings, including melanoma and lung cancer, the lessons learned are now being applied for developing drugs to treat patients with rare disease. As such, the topics presented within this RFI are highly relevant to our members actively working to advance novel treatments into the clinic for patients, both adult and pediatric, with rare cancers. Please find below SITC's comments on a few of the proposed questions from within the RFI:

**How should lawmakers define an ultra-rare disease or disorder, and which cell and gene therapies should be eligible? What definitions should be considered?**

SITC generally agrees with the standard definitions of "rare" is one in 1,500 and "ultra-rare" as one in 50,000 patients, respectively. In terms of cancer types, several pediatric and adult cancers qualify as rare or ultra-rare diseases and significantly lack effective treatment options. Thankfully, many immunotherapeutic development programs are focusing on treatment of rare and ultra-rare disease, including pediatric brain cancers and sarcomas, among other examples, as the underlying principles of these treatments are the same as those used for treatment of more common cancers. SITC volunteers are among the leading experts on cell and gene therapies being actively developed for rare/ultra-rare cancers and believe that any FDA approved agent should ultimately be deemed eligible for financial coverage and patient access. Of note, one advantage of further developing cell and gene therapies for treatment of cancers is that the foundational knowledge acquired from these studies is often applicable to future iterations of the treatment itself or translation of the drug into other disease settings. In turn, these studies subsequently reduce the financial burden of treating rare/ultra-rare disease in the long-term as our knowledge base grows through current studies.

**Should the federal government mandate coverage of these therapies?**

SITC is in support of mandating coverage of all FDA approved cancer treatments regardless of whether they are considered common or rare/ultra-rare. Immunotherapies in particular have demonstrated long-term, durable response for many patients with variety of cancers and thus should be readily available for all patients when treatments are deemed safe and effective by regulators. SITC encourages continued discussions with private payers and CMS to ensure that coverage and reimbursement are appropriate and timely when novel drugs become FDA approved.

**How do physician provide access to these therapies? How quickly should these covered therapies be made available to patients?**

SITC recognizes that one of the fundamental problems in treating rare/ultra-rare diseases is the difficulty in conducting and recruiting for clinical trials. The overall cost of activating a study combined with small patient populations due to the rarity of the diseases limit investigator's and sponsor's ability/willingness to provide these therapies within a research setting. Unfortunately, due to the limited number of safe and effective FDA approved treatment options for patients with rare/ultra-rare cancers, clinical trials are often the best option available. As such, SITC supports regulatory reform that allows for "point-of-care" clinical trials that can be rapidly activated when a patient with a rare/ultra-rare cancer is interested in participating in a clinical trial. Such reforms would greatly enlarge the patient population for clinical trials investigating drugs for treatment of these diseases while simultaneously enhancing patient access. These aspects would support the long-term sustainability of future treatment development.

SITC is excited to further assist or collaborate on this effort by discussing the above or additional topics presented within the RFI. SITC leaders have vast expertise in immunotherapeutic drug development and have diverse perspectives on the future of rare/ultra-rare disease treatments.

We thank the committee for providing an opportunity to provide comment on this important topic. Should you have any questions, please do not hesitate to contact me at [mdean@sitcancer.org](mailto:mdean@sitcancer.org). We look forward to collaborating in the future on all efforts focused on providing cancer patients with safe and effective treatment options.

Sincerely,

A handwritten signature in black ink, appearing to read "Mary Dean". The signature is fluid and cursive, with the first name "Mary" and last name "Dean" clearly distinguishable.

Mary Dean, JD, CAE  
Executive Director  
Society for Immunotherapy of Cancer