



North Carolina Pediatric Society

Chapter President

W. Scott St. Clair, MD, FAAP
Blue Ridge Pediatric & Adolescent
Medicine

579 Greenway Road, #200
Boone, NC 28607
Phone: (828) 262-0100
wscottstclair@gmail.com

Chapter Vice President

Susan Mims, MD, MPH, FAAP
Mission Children's Hospital
Missions Hospital, 509 Biltmore Ave
Asheville, NC 28801
Phone: (828) 213-1747
Susan.Mims@msj.org

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Executive Director

Elizabeth Hudgins, MPP
1100 Wake Forest Road
Suite 200
Raleigh, NC 27604
Phone: (919) 839-1156
Fax: (919) 839-1158
Elizabeth@ncpeds.org
www.ncpeds.org

March 13, 2018

Kelly Baxley, RN, BSN
NC Department of Health and Human Services
Raleigh NC

Dear Ms. Baxley:

Thank you for the opportunity to comment on the Division of Medical Assistance, Clinical Policy Section proposal to add KYMRIA (tisagenlecleucel) for pediatric beneficiaries with refractory or relapsed B-cell acute lymphoblastic leukemia (ALL) and YESCARTA (axicabtagene ciloleucel) for adult beneficiaries with relapsed or refractory large B-cell lymphoma, as a covered benefit for NC Medicaid and NC Health Choice beneficiaries.

(1) What is important to our colleagues in regard to the potential coverage benefit:

This is a critical therapy that can help save lives. Tisagenlecleucel, in particular, is an FDA approved therapy for pre-B acute lymphoblastic leukemia (ALL), one of the most common forms of childhood cancer. This therapy is typically reserved for refractory or relapsed disease, and has resulted in improved outcomes for these difficult to treat cancers. This immune based therapy now offers greater of hope of cure for children with high risk pre-B acute lymphoblastic leukemia. Tisagenlecleucel offers different toxicity profiles from standard chemotherapy or bone marrow transplant, which makes this an important addition to those therapies, especially for patients who may no longer be able to tolerate chemotherapy or bone marrow transplant.

2) Any limitations to the service:

The infusions may be given inpatient or outpatient but if the patient develops cytokine releasesyndrome (CRS) they must be admitted to the hospital until it starts to resolve. It should be limited to patients with CD19 positive acute lymphoblastic leukemia who have not yet had their 26th birthday. They should have either persistent (resistant) disease or have suffered at least one relapse. Specifically as regards tisagenlecleucel, this service should not have any units or limitations, as the administration of this drug is solely through Novartis and its Risk Evaluation and Mitigation Strategy (REMS) program is establishing a network of certified treatment centers. Depending on the longevity of the patient's response, more than one infusion of tisangenlecleucel may be needed to maintain remission.

3) If the service should be limited to certain diagnoses:

Kymriah is only FDA approved at this time for childhood acute lymphoblastic leukemia that expresses CD19. The patients must be 25 years old or younger. Tisagenlecleucel is specifically indicated for treatment of refractory or relapsed pre-B acute lymphocytic leukemia (lymphoblastic leukemia)

4) Is there any additional evidence in medical literature on the medication that we would like to present:

Maude, et al. [Tisagenlecleucel in Children and Young Adults with B-Cell Lymphoblastic Leukemia](#). N Engl J Med. 2018 Feb 1;378(5):439-448.

This group of authors include some of the nation's foremost experts in chimeric antigen receptor T- cell therapy which has become standard option for patients with high risk, relapsed, or refractory pre-B acute lymphoblastic leukemia.

5. What criteria would you include in the policy to define the service and identify community standards of practice?

Please consider verifying to include coverage of supportive care including tocilizumab which may be required for safe administration of tisagenlecleucel. The definition of refractory or relapsed pre-B ALL may not be indicated solely by the presence of measurable disease at the time of tisagenlecleucel infusion. Refractory disease may include those patients who did not achieve complete remission with standard chemotherapy and/or who have indications to proceed to bone marrow transplant but may be unable to do so due to the lack of potential donors or the excessive risks of toxicities.

Thank you again for reaching out. If you any questions please contact our Executive Director, Elizabeth Hudgins (elizabeth@ncpeds.org) and she will help connect you with the appropriate experts.

Sincerely,



Scott St. Clair, MD, FAAP, Chapter President
North Carolina Pediatric Society (NCPeds)

Cc: Elizabeth Hudgins, NC Pediatric Society