# Do all children have equal access to Phase I cancer trials?

Approximately 60% of pediatric cancer patients participate in clinical trials compared to only 5% of adult cancer patients. These pediatric clinical trials have been instrumental in improving the 5-year survival rate of childhood and adolescent cancer patients from 63% in 1975-1979 to 83% in 2003-2009. There are four phases for clinical trials with Phase I attempting to establish the safety of the treatment while also having therapeutic intent for the patients in the trial. For patients who do not respond to conventional therapies, Phase I trials offer new options to patients, though untested, while collecting important data for cancer research. While previous researchers have analyzed the availability of all stages of clinical trials to patients across demographic categories, this study is the first to analyze the equitable enrollment in Phase I trials.


This retrospective study utilized four databases: the Children's Oncology Group (COG) database, the Pediatric Brain Tumor Consortium (PBTC) database, the Surveillance, Epidemiology and End Results (SEER) database, and the US Census database. The COG and PBTC databases contain the demographics of patients enrolled in pediatric Phase I trials while the SEER database contains the incidence rates and corresponding demographic characteristics for all cancer patients across approximately 28% of the US population. We used the US Census data to transform the crude incidence rates from the SEER database into the expected incidence count for each demographic category. We compared the demographics of the pediatric cancer patients in Phase I trials (using the COG and PBTC databases), to the expected enrollment we calculated using the SEER and US Census databases. Our goal was to determine whether demographics affect the access to Phase I trials. While enrollment is not a perfect measure of access, it would still be concerning if one group had significantly higher enrollment than another.

We analyzed 1,348 children with 128 different diagnoses who participated in COG and PBTC Phase I clinical trials between 2/28/2000 and 12/29/2008. While all children were underrepresented (27,766 expected vs. 1,348 enrolled), Hispanic children were highly underrepresented (27% expected vs. 11.4% enrolled) compared to non-Hispanic whites (54% expected vs. 61.6% enrolled) and non-Hispanic blacks (10.5% expected vs. 11.2% enrolled). Particularly concerning was the

significant underrepresentation of Hispanic females(18% expected vs. 6% enrolled). As it is unlikely that Hispanics have a disproportionately better prognosis or lower relapse rate to explain the lower enrollment, Hispanic children (especially Hispanic females) may lack proper access to Phase I trials. Non-Hispanic black males aged 5-9 years old with lymphohematopoietic (LH) tumors were also underrepresented (11% expected vs. 0% enrolled).

Children 0-4 years old were also underrepresented (36.5% expected vs. 11.7% observed) while patients with solid tumors (63% expected vs. 90.6% observed) were overrepresented compared to patients with LH tumors (36.7% expected vs. 9.3% observed). The underrepresentation of children ages 0-4 can be explained by: a high incidence of highly curable CNS tumors with conventional therapies, natural aging into the 5-9 age group, or the ineligibility of infants to participate in trials with oral medications. The underrepresentation of LH tumors may be due to the higher overall survival rate for LH malignancy (90%(LH) and 83%(solid), respectively over 2004-2010), thus indicating a higher efficacy for conventional LH malignancy therapies and a lower demand for Phase I trial participation.

Overall our results indicate that each demographic group is well represented in Phase I trials, with the exception of patients aged 0-4 years old, Hispanic children (especially Hispanic females) and non-Hispanic black males that are 5-9 years old with LH tumors. Focused accrual of these groups may be necessary to ensure equal access to Phase I trials for all pediatric patients.

***Samuel Casey Allen*** *and* ***Rebecca Pentz***
*Emory School of Medicine, Hematology/Oncology*
*Emory University Winship Cancer Institute, USA*

## Publication

Access to Children's Oncology Group and Pediatric Brain Tumor Consortium phase 1 clinical trials: Racial/ethnic dissimilarities in participation.
Nooka AK, Behera M, Lonial S, Dixon MD, Ramalingam SS, Pentz RD
*Cancer. 2016 Oct 15*