Inclusion of Children Under 3 Years in Sickle Cell Clinical Trials: Implications for Nurse Scientists

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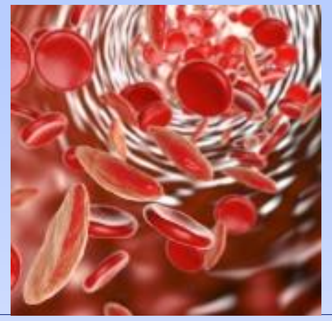
BACKGROUND

- Sickle cell disease affects upwards of 100,000 Americans and is most commonly diagnosed in populations of African or Mediterranean ancestry
- Disparities in participation in sickle cell disease research and clinical trials exist in part due to mistrust of research among racial/ethnic minorities, further limited by an overall lack of funding and research opportunities
- Obstacles in conducting pediatric clinical trials include concerns about safety and lack of enrollment. This can be particularly challenging for a disease that often presents in the first few months of life
- Children under 3 are excluded from clinical trials even more often than other pediatric populations
- Knowledge of factors influencing enrollment, and the rate of early termination and scientific success among sickle cell disease clinical trials is limited



PURPOSE

Analyze the relationship between inclusion of very young children (under 3) in sickle cell disease research and study completion, early termination of the study, and publication of study results.

METHODS

- A retrospective analysis of the ClinicalTrials.gov database was conducted over a 4 week period in June 2016
- The search keywords “sickle cell” identified all therapeutic, behavioral, and observational clinical trials that were registered in the database ranging from 1997 to present
- All trials related to sickle cell with a “status” (completed, terminated, recruiting) outcome were included
- The main independent predictor variable was inclusion criteria of the clinical trial related to pediatric age ranges

ANALYSIS

- Descriptive statistics on clinical trial characteristics
- Logistic regressions were calculated to determine the relationship of key predictor variables on our outcomes of interest (study completion, early termination and publication of study findings).

RESULTS

526 clinical trials were included in the initial analysis, including both adult and pediatric populations. 338 of those studies were pediatric only. The univariate analysis revealed no statistically significant relationship between inclusion of children under 3 in eligibility criteria and the outcomes of study completion, early termination, or publication of study findings.

Pediatric Only Sickle Cell Clinical Trial Statistics (n=338)	
Percent including children under 3	41.7%
Percent of studies completed	40.8%
Percent of studies terminated early	11.5%
Presented publications linked to clinical trial	32.5%
Average minimum age range for inclusion	5.8

CONCLUSIONS

- Knowledge of sickle cell disease in pediatric patients from birth to 3 years of age is limited due to disproportionate exclusion of this population in clinical trial eligibility, even though there is no statistically significant relationship between inclusion of this age group and study completion, early termination or publication of findings
- Unequal participation in clinical trials could limit individual patient access and benefits from advances in drugs, devices, behavioral interventions
- Nurses can be advocates in clinical trial design and recruitment to promote the inclusion and enrollment of the youngest pediatric patients to advance sickle cell disease understanding without hindering the success of the research process

Disclosures: None