

# Title: Preventing Acute Chest Syndrome by Transfusion Trial (PROACTIVE): Feasibility Study

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## Synopsis:

The Feasibility Study has two primary objectives.

### OBJECTIVE 1

To assess the feasibility of recruiting sufficient eligible subjects into the trial such that:(a) consent to the trial and randomization occur within 8 hours of eligibility determination; and b) initiation of the experimental transfusion occurs within 6 hours of randomization, a span of 14 hours between determination of trial eligibility and initiation of transfusion.

### OBJECTIVE 2

To assess the predictive utility of sPLA2 in screening for imminent ACS, characterize the distribution of sPLA2 values in sickle cell patients hospitalized with pain, and define a cut-off point for predicting ACS to optimize its reliability, sensitivity and specificity in predicting ACS.

### *Study design*

This feasibility study for the randomized controlled trial (RCT) PROACTIVE, includes two design components:

Component 1: The PROACTIVE RCT protocol, completed on N=40 subjects (20 in each treatment group, including at least two from each site); and

Component 2: An observational cohort consisting of an estimated 300 subjects ineligible for the PROACTIVE trial, either because they test negative on all precursors of ACS or because they are already diagnosed with ACS within 72 hours of admission but not yet randomized.

The trial duration is expected to be 13 months, determined by expected recruitment of 40 trial participants in 12 months. As of randomization of the 40th trial participant, recruitment of ineligible subjects (Component 2) will cease. Follow up of all participants is 28 days.

### *Study population*

Sickle cell disease subjects > 2 years of age who are hospitalized with acute pain are potentially eligible for participation in the Trial component of the feasibility pilot study. Screened subjects ineligible for trial participation will be enrolled in an observational study.

### *Intervention or Treatment:*

Twenty subjects randomized to the transfusion arm will be given a single transfusion of 7-13cc/kg packed red blood cells (RBCs). The other 20 randomized subjects, as well as the estimated 300 ineligible subjects in the observational cohort, will receive standard care.