Expanded access of Blincyto® in patients with acute lymphoblastic leukaemia: a retrospective observational study (Neuf Study)

SUMMARY OF THE PROTOCOL

This study will aim to characterise the ALL patient population and the specific subgroups receiving Blincyto in the expanded access setting and describe selected outcomes and Blincyto utilisation.

1. Research Question and Objectives

Primary Objective:

- To describe the clinical characteristics and treatment patterns of patients with B precursor ALL, having received Blincyto in the expanded access setting and identify clinically relevant subgroups.

Secondary Objectives:

- To describe the effectiveness of Blincyto within identified subgroups as outlined in the protocol
- To describe Blincyto utilization within identified subgroups as outlined in the protocol

Hypothesis/Estimation:

- This study is descriptive and no formal hypothesis will be tested.

2. Study Design/Type

This is a retrospective, observational, multi-centre study involving medical record review of patients who initiated Blincyto that was provided via an expanded access program.

3. Study Population

This study is planned to be conducted in 6 countries: France, Germany, Italy, Spain, UK and Russia. Eligible patients will include ALL patients who have initiated Blincyto in the expanded access setting from 1st January 2014 up until 31st December 2016.

4. Summary of Patient Eligibility Criteria

Inclusion:

- B-precursor ALL patients who have initiated Blincyto in an expanded access setting from 1st January 2014 up until 31st December 2016

Exclusion:

- Patients enrolled in Amgen expanded access protocol 20130320
- Patients who do not provide informed consent, where required per country regulations
- Patient's medical chart is not available for data extraction

5. Variables

Clinical and treatment characteristics
Effectiveness
Blincyto utilisation

6. Study Sample Size

The actual number of patients that will be included in the study will depend on the results of site feasibility, willingness of sites to participate in the study and availability of the medical records. It is not relevant to evaluate the precision of estimates for the primary objective. The secondary objectives would describe effectiveness, utilisation and safety endpoints in clinically relevant subgroups.

7. Data Analysis

All analyses will be descriptive. Continuous variables will be summarised by mean, median, standard deviation, lower and upper quartiles, and minimum and maximum values. Categorical variables will be summarised by number and percentage of patients in each category. For categorical outcomes, 95% confidence intervals (CIs) will also be presented where appropriate. For time-to-event endpoints, Kaplan-Meier (KM) curves and KM proportions at select time points, the number of subjects with events and the number of subjects censored will be used to summarise the data.