Pilot Program for eSCaPe: electronic Sickle Cell application for Pain evaluation for patients enrolled in NKT 120-SCD1

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ABSTRACT

Background: The determination of symptomatic endpoints and safety signals such as pain, respiratory compromise in the development of new therapeutics depend on timely and accurate communication between patients and clinicians. Historically, documentation of patients’ symptoms has relied on memory recall, a method reported to be inaccurate. Here we describe an electronic symptom diary (eDiary) for adults with non-acute sickle cell disease to use before, during and after the depletion of their iNKT cells (an immune system cell that is implicated in SCD crises) by the investigational monoclonal antibody, NKTT120.

Objective: In designing QoL and pain assessment endpoints in any therapeutic trial for patients with SCD, the ASCQ-Me and PROMIS tools are essential components; however these have at least a 7 day look-back. In order to obtain real-time daily assessments of pain, fatigue, shortness of breath, and overall well-being, an eDiary was designed to accompany the ASCQ-Me and PROMIS tools for non-acute SCD adults. This tool, eSCaPe, will be used by patients enrolled on a phase I trial of treatment with monoclonal antibody, NKTT120 that depletes iNKT cells and may reduce the inflammation associated with the sequelae of SCD. The hypothesis is that the eDiary data, collected in real-time on Apple and android smart-phones, may have higher adherence rates and provide more accurate symptom data collection relative to the infrequently administered standard tools.

Methods: An eDiary was assembled, based on features of other tools used to collect pain and QoL assessments. This eDiary included daily ratings of pain, fatigue, shortness of breath and general well being. This diary was provided to the patients, and their data was entered and uploaded daily using a smartphone app. Patients on the trial will have a 2-week run-in period to assess their baseline, prior to administration of the treatment. Patients enrolled in the NKTT120 trial were to continue their eDiary reporting until their completion of the study endpoint (the recovery of their iNKT cells).

Results: Overall adherence of daily e-reporting for 32 patients enrolled to date on the NKTT120-SCD1 trial will be reported. Any differences between their baseline 2 week run-in period and their post treatment symptoms will be described. Technical difficulties encountered in the administration of a mobile phone eDiary will be reported.

Conclusions: An eDiary tool for adult SCD patients undergoing investigational treatments has been developed. This data may confirm previous e-diary experience of improved reporting of symptoms/side-effects which may reflect more accurate beneficial or adverse effects of the novel treatment.

INTRODUCTION & STUDY DESIGN

Handheld Electronic Assessment System via Wireless Technology:

We utilize a handheld electronic multidimensional pain monitoring, assessment, and real time treatment program (eSCaPe) downloaded to an Android/Phone platform smart-phone/personal digital assistant to remotely assess the pain, fatigue, breathing and general well being in adults with SCD who are receiving NKTT120.

The eSCaPe is an electronic multidimensional pain diary that uses real time data capture (Stinson et al., 2006, 2008). The eSCaPe software manages the screen displays, tabulates, and stores the user responses to the displayed pain scales. It was developed to obtain pain ratings: 1) on auto-inquiry that can be programmed from 0 n times, 2) initiated by the patient, initiated by the nurse or doctor. It uses a signal contingent approach (audible alarms at fixed times with 30-minute compliance windows). The screens on the FDA were designed to appear similar to the paper forms: 1) the visual analog scale (VAS) interference scales that were adapted from Brief Pain Inventory-Short Form (Daut, Cleeland, & Flanery, 1983) and 2) the 32 pain word descriptors from the Pediatric Pain Tool (Abu-Saad et al., 1990, 1994).

The eSCaPe was programmed to follow a specific sequence of questions with a built-in response loop. The patient is instructed to fill out the eSCaPe eDiary on a daily basis when prompted by audible alarm. When the patient logs in, they will be queried about any past missing daily entries. They will be asked about 4 general areas, pain, fatigue, breathing and general well being, and then asked to upload the entries when completed. If the patient completes just the pain questions for the day, however, the entry will be considered complete. If the patient indicates having no pain, the diary skips the pain related questions.

In broad view, if the patient has pain on inquiry an analog scale allows grading of pain and amount of pain medication required.

MATERIALS & METHODS

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1. GoMed Solutions Inc. was asked by NKT Therapeutics (NKT) to develop a mobile application that could track pain levels in the Phase 1b clinical testing of a new drug for the treatment of Sickle Cell disease (SCD).

2. NKT has previously developed a QoL (Quality of Life) study using standard tools to evaluate pain, fatigue, shortness of breath, and general well-being in patients with SCD. The hypothesis is that the eDiary data, collected in real-time on Apple and Android smartphones, may have higher adherence rates and provide more accurate symptom data collection relative to the infrequently administered standard tools. This will confirm previous GoMed data.

3. Working closely with the investigators and data managers, GoMed Solutions used research validated measurement tools to create an electronic diary that patients could easily download and install on their iOS and Android smartphones to collect pain and QoL assessments in non-acute SCD adults.

4. This novel method to gather critical data directly from patients in real-time represents an advance in clinical trial methodology. It provides more accurate, more timely data, and it is more cost effective for the company as well.