# Patients Involved – Patient feedback on a paediatric CML study

## Introduction

A collaboration of the CML Advocates Network / Leukaemia Patient Advocates Foundation with a major pharmaceutical company regarding the use of a medicine in Phase III trials (adult use), aiming for its use in paediatric population.

Where in the process? – Phase II

When does it happen? – Phase II

## Description of the case

A major pharmaceutical company prepared a combined Phase I/II study of the pharmacokinetics, safety and efficacy of a new targeted medicine in paediatric patients with a chronic myeloid leukaemia (CML) with resistance or intolerance to other medicines. By the time of this protocol design, the medicine was in Phase III trials aiming for approval in adult use.

Paediatric CML is an ultra-rare condition which affects only about 20 children a year in a population of 80 million. Barely any larger paediatric centre has more than 1 to 2 paediatric CML patients. Hence, recruitment into trials is difficult. By the time of the protocol review, two other medicines were approved for paediatric use.

The patient relations department set up a two hours meeting between:

- An experienced patient / patient advocate with personal disease experience as well as advocacy experience in medicines R&D in CML, and
- Clinical development staff involved in the protocol design.

The trial synopsis (16 pages) was shared with the patient advocate 14 days prior to the meeting, subject to non-disclosure agreements. The advocates' written comments were returned by the patient advocate to the clinical development two days prior to the meeting, and were then discussed face to face.

Feedback provided by the patient advocate focused on in/exclusion criteria (e.g. unnecessary exclusion of children <10 years, required ability to swallow pills despite dissolvability of the medicine), access to the medicine after the study conclusion, diagnostics (e.g. necessity of quite invasive bone marrow biopsies), dosing (e.g. number of pills given in paediatric use vs. difficulties in paediatric admission), involvement of parents in creation of informed consent / assent documents.

# Type(s) of patient (advocates) involved

- Patients with personal disease experience.
- Expert patient / patient advocate with good expertise on disease and good R&D experience.

# Benefits of patient involvement

According to direct feedback of the clinical development team at the conclusion of the meeting, as well as feedback received

by the patient relations department days later, the input received was perceived as ‘invaluable’ and has led to significant modification of the trial protocol.

The development process was not delayed by involving patients, as the consultation was incorporated into the process of protocol development. Serious issues that might have threatened recruitment, trial retention or ethics were uncovered at design stage, and resolved before submission of the protocol to authorities.

Given there has been little prior exposure of the clinical development team to real (adult) CML patients and no prior experience with paediatric CML patients or their parents, a number of issues had surfaced that, according to our assessment, would have prevented parents from enrolling their children into those trials, or might have caused serious rates of trial drop-outs. According to clinical development, much of the feedback ‘was covering issues that we should have really thought about, but have not surfaced in discussions both within the team and with investigators prior to the meeting’.

# Challenges and barriers

- Perceived legal barriers for disclosure of the trial synopsis and protocol (solved by persistence of the patient relations department to agree on NDA).
- Resistance of the clinical development team to involve patients and agree on a face-to-face meeting with patient advocates, mainly due to the lack of perceived value (these perceptions completely changed as a result of this meeting)

# Learnings

This is a good example of a mind-set change induced by a short, concise and well-prepared meeting between the clinical

development team and an experienced patient advocate, initiated, enforced and facilitated by the responsible patient relations person.

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# Attachments

- Patients Involved Case Report – Patient feedback on a pediatric CML study
  Size: 489,432 bytes, Format: .pdf
  Patients involved case report on a collaboration of the CML Advocates Network/Leukaemia Patient Advocates Foundation with a pharmaceutical company regarding the use of a medicine in Phase III trials (adult use), aiming for its use in paediatric population