Factors influencing the outcome of nilotinib treatment in patients with chronic myeloid leukemia in daily practice

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Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Leukaemias
Study type	Observational invasive

Summary

ID

NL-OMON41699

Source
ToetsingOnline

Brief title
Response and Adherence to Nilotinib in Daily practice (RAND-study)

Condition

- Leukaemias
- Leukaemias

Synonym
Chronic Myelogenous Leukemia; Cancer of white blood cells

Research involving
Human

Sponsors and support

Primary sponsor: Vrije Universiteit Medisch Centrum
Source(s) of monetary or material Support: Ministerie van OC&W,Novartis

Intervention

Keyword: Medication Adherence, Nilotinib, Patients experiences, Treatment outcome

Outcome measures

Primary outcome

The primary efficacy endpoint is the MMR within 12 months after the start of first study medication. A MMR is defined as <=0.1% BCR-ABL/ABL in the international scale.

The primary means of assessing adherence is the total intake of nilotinib capsules as counted by means of a medication event monitoring system, taken as percentage of the number of capsules prescribed over the 12 months follow-up period.

Secondary outcome

Secondary parameters include:

- complete hematological response (CHR; the normalization of the blood cell count, particularly of white blood cells)

- complete cytogenetic response (CCyR, complete absence of Ph+ cells in blood and bone marrow)

- complete molecular response (CMR)(using RQ-PCR Ph+ DNA cannot be detected)

- trough plasma level of nilotinib

- potential drug-drug interactions based on patient-reported data from the questionnaire, data from the patient's medical file and pharmacy dispension

records of the past 6 months

- patient-reported side effects data obtained by means of questionnaires

- adherence by means of the conventional PPP method (Patient*s files-Pharmacy records-Pill count method).

- adherence behaviour data obtained by means of the Medication Adherence Rating Scale (MARS) questionnaire.

- quality of life data obtained by means of the SF-12 Health Survey

- attitude towards disease data obtained by means of the Brief Illness Perception Questionnaire (Brief IPQ)

- beliefs about medicines data obtained by means of the Beliefs about Medicines Questionnaire (BMQ)

- patients* appreciation of information given about the medication obtained by means of the Satisfaction with Information about Medicines Scale (SIMS) questionnaire

- percentage of dose adjustment

- patient-reported discontinuation by means of questionnaire.

Study description

Background summary

Nilotinib is indicated for the treatment of chronic myeloid leukemia (CML). The antitumor drug nilotinib has a large inter- and intra individual variability of pharmacokinetics. Adherence to treatment may substantially influence plasma levels. A better understanding of the various factors contributing to the adherence to nilotinib treatment is essential for the development of interventions to optimize the treatment of CML with a protein kinase inhibitor like nilotinib.

Study objective

The primary study objective is to assess whether CML patients who respond to treatment with nilotinib (MMR within 12 months of treatment) differ from the non-responders in terms of adherence. Secondary objective is to evaluate whether responders and non-responders to nilotinib differ with respect to their plasma levels of nilotinib. Furthermore, the study aims to identify possible predictors for adherence to nilotinib treatment among a variety of hypothesized predictors.

Study design

Multicenter prospective observational cohort study in which 70 patients with CML on treatment with nilotinib will be followed up for at least 12 months.

Study burden and risks

Response to treatment will be evaluated at 3, 6 and 12 months of treatment, and, for patients already on treatment at recruitment, also at study entry and at the end of the 12 months follow-up period. Adherence is primarily assessed by counting the total intake of nilotinib capsules by means of a medication event monitoring system, taken as percentage of the number of capsules prescribed. At baseline and after 3, 6 and 12 months, patients will be asked to fill in a comprehensive questionnaire. After 3, 6 and 12 months and, for patients already on treatment at recruitment, also at baseline, patients will be asked to provide a blood spot sample.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

- Male or female patients >= 18 years of age
- ECOG 0, 1, or 2.
- Diagnosis of chronic myelogenous leukemia with cytogenetic confirmation of Philadelphia chromosome of (9;22) translocations.
- Treatment with nilotinib.

Exclusion criteria

- Inability to grant consent.

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Health services research

Recruitment

NL

Recruitment status:	Recruitment stopped

Start date (anticipated):	22-08-2013
Enrollment:	70
Type:	Actual

Ethics review

Approved WMO Date:	28-05-2013
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	09-07-2013
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	05-05-2014
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	23-09-2014
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	28-07-2015
Application type:	Amendment
Review commission:	METC Amsterdam UMC

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

ID: 23279
Source: Nationaal Trial Register
Title:

In other registers

Register	ID
CCMO	NL41762.029.13
OMON	NL-OMON23279