Non-Interventional Study (NIS) Primary Report Synopsis

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CAPRELSA® REGISTRY: a Belgian Registry to evaluate the use of Vandetanib (Caprelsa®) in current clinical practice

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NIS REPORT SYNOPSIS

CAPRELSA® REGISTRY: a Belgian Registry to evaluate the use of Vandetanib (Caprelsa®) in current clinical practice

Study sites and Investigators

The selection of the study sites in this multicentre study conducted in Belgium was based on previous participation in vandetanib studies, on the participation in the medical need program for vandetanib and was based on the medical expertise of the Investigators in this oncology field. Eight out of the 10 approached study sites participated in the study.

Total planned study period	
Date of first patient in (first data entered in Case Report Form [CRF])	29 April 2014
Date of last patient last visit (last data entered in CRF)	10 June 2015
Date of data base lock	22 July 2015
Date of final study report	29 September 2015

Medicinal Products (type, dose, mode of administration) and concomitant medication

Caprelsa® 100 mg or 300 mg film-coated oral tablets. Each film-coated tablet contains 100 mg or 300 mg of vandetanib.

As per SmPC, the recommended dose is 300 mg once a day, taken with or without food at about the same time each day. If a dose is missed, it should be taken as soon as the patient remembers. If it is less than 12 hours to the next dose, the patient should not take the missed dose. Patients should not take a double dose (two doses at the same time) to make up for a forgotten dose.

Publication

None at the time of this report.

Objectives of this Non-Interventional Study

Primary objective

The primary objective of this registry was to describe the characteristics of patients who receive Caprelsa® and who were fulfilling the reimbursement criteria.

Secondary objectives

N/A

Study design

This was a multi-centre, prospective and retrospective, registry using paper case report forms (CRFs). The assignment to treatment fell within current practice. Patients, for which vandetanib had been prescribed according to the marketing authorisation and fulfilling the criteria for reimbursement, were enrolled in the registry at a first visit (baseline) after reading and signing the Informed Consent Form (ICF). For each patient, the Investigator recorded data of visits occurring according to his/her current clinical practice. It must be noted that patients, who took vandetanib (Caprelsa®), and who had died before they could participate in this registry could also be included. There were no restrictions of a maximum number per site as this registry captured all patients for which vandetanib had been prescribed according to the marketing authorisation and fulfilling the criteria for reimbursement.

At the first visit (baseline), the Investigator recorded the following data: patient visit and Informed Consent date, demographics, inclusion criteria, medullary thyroid cancer (MTC) information, rearranged during transfection (RET) mutation status, previous MTC treatment and treatment with vandetanib as from 1st of May 2013.

Every time a patient attended the hospital for a visit, the Investigator reported follow-up data in the CRF (not applicable for deceased patients). During the follow-up visits, the Investigator recorded the date of the visit, treatment with vandetanib and evolution of the disease. At the end of the registry, taking place at treatment discontinuation or at the closure of the registry, the Investigator recorded the following data for each patient, except for the patients who had died before inclusion in the registry: date of final evaluation and treatment with vandetanib.

Target population

The target population included all Belgian patients diagnosed with aggressive and symptomatic unresectable locally advanced or metastatic MTC who have received a prescription for vandetanib. Patients who had received vandetanib within the framework of a study and who fulfil the reimbursement criteria of vandetanib in Belgium were also captured in this registry.

Study variables

The following variables were collected in the medical records:

- Patient demographics: age, sex
- Medical history: diagnosis and evolution of the disease prior to vandetanib intake
- Latest RET mutation status
- Previous treatment prior to vandetanib intake
- Treatment with vandetanib
- Evolution of the disease after start vandetanib

Statistical methods

All analyses were performed according to the Statistical Analysis Plan (SAP) dated 25 June 2015.

All statistical analyses were essentially descriptive (no causal analysis), therefore no clinical interpretation can be made based on the results obtained in this study.

The study population was analysed as a whole. In view of the limited number of patients, no

subpopulation analysis was planned.

Descriptive analyses included the following:

- For continuous variables: number of observations, number of missing observations, mean, standard deviation, minimum, median and maximum.
- For categorical variables: number of observations, number of missing observations, and absolute and relative frequencies of each category.

Descriptive analyses were performed on the following variables: demographics, MTC history, RET mutation, previous treatment, and treatment with vandetanib.

Summary

Ten patients with a median age of 60 (range: 29-80) years were included in the registry, most of them (80%) were men. Of the 6 patients tested for the RET mutation status, 1 patient had RET mutation. Eight patients had previous surgery after a mean period of 126.1 days since initial MTC diagnosis. Three patients had previous radiotherapy after a mean period of 2514.7 days since the initial MTC diagnosis, while 1 patient had previous chemotherapy 1826 days after the initial MTC diagnosis; 4 patients had other previous treatment. Patients were exposed to vandetanib for a mean number of 540.8 days (i.e., 17.78 months). Treatment with vandetanib started after a mean number of 1416.6 (SD=2299.22) days since diagnosis (data available for 9 patients), while the median number of days between diagnosis and treatment start was 629 (range: 44-7420) days. Vandetanib was used as first line treatment for 70% of the patients, second line treatment for 10% and third line treatment for 20% of the patients. Of the 6 patients with available results, 1 had no follow-up visit, 1 had 1 follow-up visit, 2 had 4 follow-up visits, 1 had 5 follow-up visits, and 1 patient had 14 follow-up visits. The dose was changed for 5 out of 10 patients, temporarily discontinued for 4 out of 10 patients and permanently discontinued for 4 out the 10 patients. An average period of 229 days elapsed from treatment initiation until the first dose change, 188 days from treatment initiation until the first temporary treatment discontinuation and 278.5 days from treatment initiation until permanent treatment discontinuation. Out of the 9 dose changes, 6 occurred due to an adverse event. Temporary discontinuation occurred due to an adverse event (1 patient) or surgery (1 patient), while temporary discontinuation followed by dose change occurred due to an adverse event for 3 patients. At the time of the database lock, 4 out of the 6 alive patients continued treatment with vandetanib while one of the other 2 patients was prescribed another treatment.