

# Patients Involved – Clinical trial design

## Introduction

An example of patients involved through patients and patient organisations providing input into a Cushing's disease clinical trial design. The consultation took place between Novartis and nurses from different teams (USA, Canada, and Brazil), Cushing's disease patients (USA) and caregivers and patient organisation representatives.



When does it happen? – Phase III

## Description of the case

The objectives for this case were to:

- Obtain specific feedback on sections of a draft protocol from patients, caregivers, advocates, and research study coordinators on Cushing's disease clinical trial development.
- Identify potential areas of concern from the patient and research community that may impact the Cushing's disease trial accrual process in the future.
- Identify ways that Novartis can help support new product development with related tools and educational materials

for patients with Cushing's disease.

## **Type(s) of patient (advocates) involved**

- Patients with personal disease experience.
- Expert patients / patient advocates with good expertise on disease, but little R&D experience.
- Expert patient / patient advocate with good expertise on disease and good R&D experience.

## **Benefits of patient involvement**

The following topics were discussed and then given to the development team:

- Multiple issues to consider when designing a rare disease trial including patient-investigator communication, enrolment, education, psycho-social support, access, tracking, reporting and follow-up.
- Study length, transportation and visit schedule were named among the main barriers from the patient perspective.
- Working with patient advocate groups in clinical trials is crucial. Patient advocate groups should be used to announce and disseminate information about trials to boost enrolment.
- Consideration on the proof of concept and possible importance of the medicine being studied for the patients.

## **Results**

- Trial design was better tailored to patient's needs.
- Different perspectives and insights allowed to better inform a study design.

# Challenges and barriers

No specific barriers were experienced.

## Learnings

The need provide better preparation for the patient advocates to participate in the discussion, including topics such as:

- What a clinical trial is.
- Why a clinical trial is important.
- What to expect in a clinical trial, along with the time commitment it requires.
- Why certain tests in a clinical trial are essential, potentially providing tips to facilitate management of these tests.
- What a trial crossover means, and why it is needed.

## Attachments

- Patients Involved Case Report – Cushing’s clinical trial

Size: 450,012 bytes, Format: .pdf

An infographic describing a patients involved case report of patient input into a clinical trial design for a Cushing’s clinical trial.