

# Patients Involved | Case Report

## Input of Patient Organisations into Cushing's Disease Clinical Trial Design

An example of input from patient organisations into a Cushing's Disease clinical trial from Novartis.

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.



### Benefits

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, psychosocial 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

No specific barriers were detected.

### Learnings

The need for a better preparation for the patient advocates to 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.

### Who was involved?

Nurses from USA, Canada and Brazil; USA Cushing patients, caregivers and patient organisations representatives, Novartis.

### Level of patient expertise

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

