

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