Patients' role in using PED/PROs in drug development | An industry perspective

Novo Nordisk is progressively integrating Patient-Reported Outcome (PRO) Measures and Patient Experience Data (PED) in a systematic manner during product development, recognizing it is essential to include the perspectives of patients. However, there is a need for greater clarity on the utilization of qualitative PED data in regulatory decision-making.

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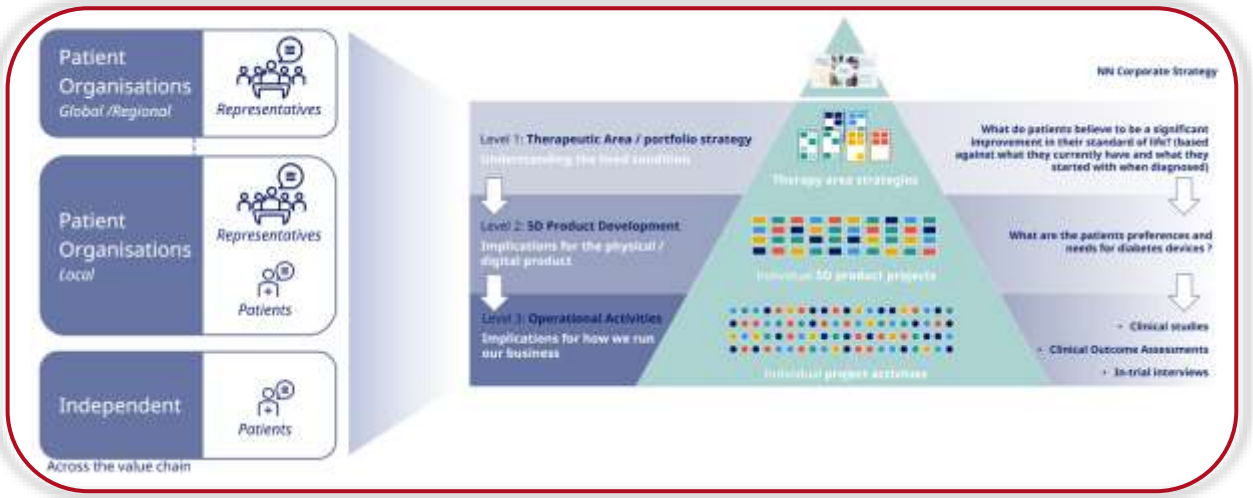
ESC
European Society of Cardiology

Insights from patients and patient organisations are key across product development activities

A patient voice strategy ensures a systematic approach to integrate patient experiences into the development of Novo Nordisk's treatment solutions

Co-create

Dialogue and co-creation lead to improved healthcare outcomes



Insights

NN uses PED to inform internal and regulatory decision-making to align with patients' unmet needs

Process overview

- Patient voice strategy development
- Insight collection from PED methods

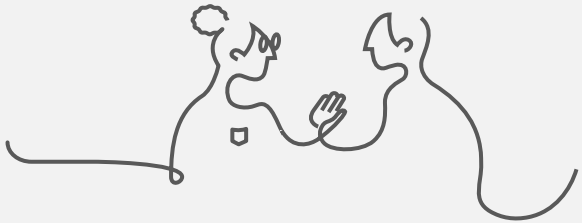
Use of foundational patient insights to inform product development activities

Questions used to collect foundation patient insights

How do patients understand their disease?

Which aspects of the patients' life are affected by the disease?

How do patients and their relatives manage everyday life with the disease?



How do patients experience the journey towards diagnosis?

How do patients talk about and perceive their treatment (or lack of treatment) and their interactions with the health care system?

Examples of use of the insights

Update of PRO strategy for Phase 3

Input to meaningful change assessment

Input to topics for in-trial interviews

In-trial interviews differ from PROs/questionnaires and add additional value due to their specific characteristics

Cardiovascular Round Table

In-trial interviews

Characteristics

… are **systematically collected PED** from trial participants via e.g. face-to-face or telephone interviews

… have **open-ended questions and** are **semi structured**

… are analysed by trained experts on **qualitative data analysis** methods

PROs/questionnaires

Characteristics

… can be measured via **diaries and questionnaires**

… have **close-ended questions and include scales**

… provide quantitative data for statistical analyses

Qualitative data from clinical trial allow for deeper understanding of the quantitative data

PROs and other assessments in clinical trials

In-trial interview in clinical trials

Quantitative data answers “How many/much”

Qualitative data answers “Why/What”

Insights from in-trial interviews provide value for internal and external stakeholders' decision-making

Utility of in-trial interview data for *internal* stakeholders

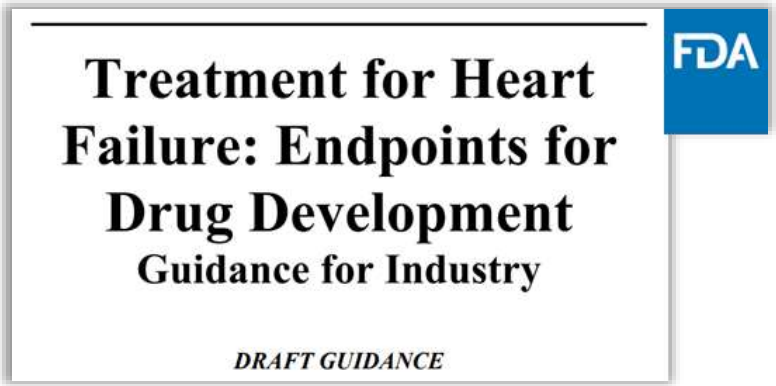
- **Clinical Drug Development**
  - Provide insight to **burden and enhance safety and optimise drug/solution** development
- **Medical Affairs**
  - Improve disease **education efforts** using patient and caregiver insights
- **Regulatory**
  - Provide additional patient experience data to **support submission** for a label claim
- **Market Access**
  - Contextualise the clinical trial data for the **global value dossier**

Utility of in-trial interview data for *external* stakeholders

- **Patients**
  - Inform how a **treatment solution has impacted a patient's disease** experience
- **Clinicians**
  - Inform patient-clinician **communication**
- **Regulators**
  - Help **interpretation of trial data** for decisions on label claims
- **Payers**
  - **Inform HTA** decision-making on reimbursement

EMA and FDA provide guidance on the use of PROs in drug development for Chronic Heart Failure

EMA's guidance states that PROs, capturing patients' perspectives on symptoms and quality of life, are usually included as secondary endpoints but can also be included as primary endpoints in case the patients are unable to undergo exercise testing



FDA's guideline makes it clear that an effect on symptoms or physical function, without a favourable effect on survival or risk of hospitalisation, can be a basis for approving drugs to treat heart failure

LET'S DISCUSS

To which degree should qualitative data from trial participants play a role* in regulatory decision making in CVD?

Currently

Hard endpoints **PROs endpoints** **Qualitative data**

In the future

* Perhaps especially for situations where regulatory qualified PROs are missing or in rare diseases

LET'S DISCUSS

Comprehensive guidance documents are available on the collection of quantitative data, including PRO data

However, there is a critical need for detailed guidelines on collecting qualitative PED data in clinical trials. It is complex due to e.g. GCP and GDPR

What would be required from academia, patient organizations, regulators and industry to accelerate the development of global guidelines e.g. ICH patient focused drug development guideline?

The International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH)

THANK YOU