Workshop on the development of new measurement tools in primary Sjögren's syndrome

Several members of the NECESSITY Patient Advisory Group participated to a workshop organized on October 30, 2019 in France by the NECESSITY consortium. This is a summary of this one-day event.

Objectives

This workshop was organized in the context of Work Package 8 which aims at reaching consensus with key stakeholders regarding the use of previous and new measurement tools in clinical trials with the goal to obtain approval and reimbursement of drugs for the management of primary Sjögren's syndrome.

The objectives of the workshop were to:

1. Establish the specific inputs needed from patients
2. Gain initial feedback on the approach to the development and validation of a new scoring system for clinical trials
3. Identify the optimal approach to gain feedback on the identification and validation of new measurement tools from authorities responsible for the marketing authorization and reimbursement of drugs

Participation

This workshop brought together 25 participants representing NECESSITY consortium (academia and industry), Health Technology Assessment experts (approving new drugs for market), payers (setting the level of reimbursement of drugs), and patient representatives.

Discussions

The workshop was structured to facilitate discussion between participants by allocating time for presentation by each stakeholder group followed by full group discussions and small group debates.

<u>Building a novel scoring system in primary Sjögren's syndrome</u>

To date, there is a high unmet need for treatments for the management of primary Sjögren's syndrome. Recent clinical trials were disappointing and current outcome measures used in clinical trials to assess the efficacy of a new drug appear to be inadequate. Important features of the disease such as swallowing difficulties or mental health challenges are not properly considered. The NECESSITY consortium has developed an innovative methodology for building a new scoring system called STAR (Sjögren's Tool for Assessing Response). The validity of this scoring system as a tool evaluating a new drug's ability to improve patient's symptoms and well-being will be tested in the NECESSITY clinical trial.

<u>Stakeholder's feedback</u>

- Patients

Patient's testimonies were given to highlight the elements of importance for patients in their daily life with this disease. The patients need treatments to help better functioning and limit debilitating symptoms (such as fatigue, dryness, joint pain) and limit episodes of flare. Sleep

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disturbances and isolation from social life are frequent. Furthermore, the financial burden of the disease in some countries is important. Finally, it may take up to 15 years to establish the diagnosis so raising awareness of the disease is essential, and medical education on primary Sjögren's syndrome could be emphasized to help health care professionals recognize the signs of the disease.

- Health Technology Assessment and Payer experts

Feedback on the approach to endpoint development and validation

STAR will be used to determine if a drug improves the patient's symptoms and well-being. The items assessed as part of STAR should be items that clinicians find relevant in terms of clinical and biological improvement, and, as importantly, that patients deem essential to improve their quality of life.

Optimal approach to gain regulatory and payer feedback

Several procedures are available to interact with the European Medicines Agency (a regulatory agency approving drug for market) and obtain advice on the methodology and validation of the new scoring system. Important aspects to consider for obtaining a positive evaluation of a new drug by regulatory experts are the benefits to the patients, the robustness of the methodology, the cost effectiveness and the feasibility of its use in standard care.

Outcomes and next steps

This workshop served as a platform for sharing experiences of clinical practice and living with primary Sjögren's syndrome. The best route to obtain advice from regulatory experts was defined. Patients' testimonies were carefully listened to by the other participants. The feedback provided by patients on items to be included in the new scoring system was recorded in the meeting report which was communicated to every member of the NECESSITY Consortium.

The WP8 team will now focus on engaging with the European Medicines Agency.

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