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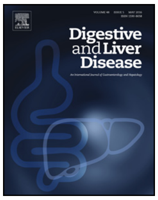

Alimentary Tract

Treatment satisfaction, preferences and perception gaps between patients and physicians in the ulcerative colitis CARES study: A real world-based study

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ABSTRACT

Background: Ulcerative colitis (UC) is a life time disease and issues with therapy may impact on patient satisfaction and treatment preferences.
Aims: To assess disease and treatment perception gaps from patients' and physicians' perspectives in UC patients.
Methods: Adult patients with moderate-to-severe UC (Mayo score $\geq$6) naïve to biologic therapy were enrolled in a European, observational, cross-sectional, retrospective study. Treatment satisfaction was assessed by the TSQM questionnaire and treatment preferences and patient's knowledge with pre-defined questions. Physicians' and patients' perceptions were compared through the level of agreement.
Results: 256 patients from 11 European countries were included. 48.0% of patients were dissatisfied with their current treatment. Effectiveness, long lasting action, rapid start of action, and fewer side effects were the attributes more frequently considered *important* or *very important* by patients (96.9%, 89.1%, 83.8%, and 81.8%, respectively). 26.2% patients rated their overall disease knowledge as *very knowledgeable.* The agreement between patients' and physicians on disease severity was good (kappa = 0.62).
Conclusion: Half patients with moderate-to-severe UC managed with conventional therapy, are dissatisfied with their treatments. Effectiveness, long lasting action and rapidity of action were the most frequently rated items in treatment preferences. There are major gaps between physicians and patients when evaluating disease burden.

1. Introduction

Ulcerative colitis (UC) is a chronic and disabling condition. The advent of biologics has changed the way to treat inflammatory bowel disease (IBD) refractory to standard medications. However, therapies used in clinical practice often result in failure or in unacceptable side effects. Approximately 50% of all patients with UC have ongoing disease activity and up to 10% of patients need to undergo a surgical procedure such a colectomy [1,2]. While enriching our therapeutic armamentarium with new biologics such as vedolizumab or golimumab, the choice between different medications has emerged as a major issue in clinical practice. Route of

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administration, rapidity of action and safety profile, are key issues when discussing treatment preferences [3]. Patients report that efficacy and durable control of symptoms as well as safety are more important to them than the nature of the treatment they receive [4].

However there may be some disease and treatment perception gaps between patients and physicians, possibly resulting in under treatment of UC patients [5].

In spite of the clearly demonstrated negative impact of UC, there are few studies addressing patients' experiences and opinions of their disease, treatment satisfaction, or the concordance between patients' and physicians' perspectives, awareness and knowledge of UC [6,7]. The UC-CARES study was designed to characterize the unmet needs of disease control and limitations of available therapies in UC considering both patients' and physicians' perspectives and including evaluation of patients' treatment preferences and satisfaction.

2. Materials and methods

2.1. Study design

The UC-CARES study was an observational, multinational, multi-centre, retrospective chart review including cross-sectional collection of patient reported outcomes (PRO). The day of patient data inclusion was defined as index date with a maximum retrospective review period of 24 months.

The study was carried out in 11 European countries: Belgium, France, Germany, Greece, Italy, Spain, Sweden, Switzerland, The Netherlands, Turkey, and United Kingdom, under the International Conference of Harmonization (ICH) ethical standards, and was notified to or approved by the Ethics Committees of participating centres in accordance with regional and national regulations.

This paper has focused on PROs related to disease knowledge, patient treatment satisfaction, patient attitudes, sources of information, severity as perceived by patients and information on available treatments. Symptoms and disease severity as assessed by the physician are also described. Agreement between patient and physician assessments of severity has been evaluated. Moreover, patients were asked to indicate their level of agreement with statements related to UC and their physician. The protocol was amended after data collection had started to include specially designed questions on patient treatment preferences.

Investigators were physicians involved in the management and treatment of UC patients (gastroenterologists, internists or GPs). Patients that attended a routine office visit and met the study selection criteria were included consecutively. It was not a requirement that UC was the primary reason for the patients visit on the index date.

After giving written informed consent, patients completed a set of questionnaires. Patients' demographic and clinical data were collected at the index date and retrospectively for a period of 12 months from the patient's medical charts.

2.2. Study population

Enrolled patients were $\geq$18 years and were diagnosed with moderate to severe UC (Mayo score $\geq$6) at index date or within the 12 months prior to the index date. Core study data from medical charts had to be available at least for the previous 12 months and patients had to be able to self-complete study questionnaires. Patients were excluded if they had ever received biologic therapies, if their UC-treatment medications had been changed within the 2 weeks prior to index date, and if they had a previous colectomy with or without ileo-anal J-pouch reconstruction. Pregnant or breast-feeding patients and those who had participated in any clinical trial during or 3 months before the retrospective observational period were excluded.

2.3. Study measures

2.3.1. Disease remission and control

Disease remission was defined as presence of total Mayo score $\leq$2, with no individual sub-score >1 [8]. For patients with no endoscopy available, definition of remission considered the presence of partial Mayo score $\leq$2, with no individual sub-score >1.

Those patients in remission that had not received corticosteroids during the previous two months were defined as controlled.

2.3.2. Patient's reported treatment satisfaction and unmet needs

Treatment satisfaction was assessed using the Treatment Satisfaction Questionnaire for Medication (TSQM) [9]. Only effectiveness-related items were included in this study for two reasons: because effectiveness of treatment was perceived as the key one for the study objectives and to avoid overloading patients with too many questionnaires.

Patients with unmet needs were defined as those who met all three of the following criteria: uncontrolled disease, self-perception of 'moderate' or 'severe' disease activity, and dissatisfaction with current treatments (dissatisfaction was defined as 'TSQM global satisfaction score' lower than the median values obtained for study patients).

In addition, a Kruskal–Wallis test and a Chi-square test were conducted to compare the global scores and the categories (i.e., no-dissatisfied versus dissatisfied), respectively, from the TSQM between countries.

2.3.3. Patient's reported treatment preferences

Treatment preferences were measured by asking the patients to score the following treatment-related attributes on a six point scale that ranged from 'very important' to 'don't know/no opinion': effectiveness, fewer side effects, financial cost to you, mode of administration, dosing frequency (every two weeks, monthly, every two months), rapid start of action, long lasting action and setting (hospital or home treatment). Patients were also asked about their preferences with respect to colectomy versus three possible treatment options for biological therapy. The biological treatment options used for induction treatment, stated to have comparable effectiveness and safety, were as follows:

- Intravenous, three administrations, hospital/specialist IV clinic, every eight weeks.
- Subcutaneous, three administrations, hospital by nurse/home by patient, every two weeks.
- Subcutaneous, two administrations, hospital by nurse/home by patient, every four weeks.

2.3.4. Disease awareness and knowledge

Patients were asked to complete a set of specially prepared questions to measure their disease knowledge. They could select from four possible responses, ranging from "very knowledgeable" to "no knowledge", and the parameters evaluated were: overall knowledge; knowledge regarding possible progression of UC; and knowledge regarding possible complications of UC. Patients were also asked if they had or not participated in a patient support programme.

2.3.5. Patients' perception of disease disruption

Patients were asked to complete a series of questions related to living with UC; there were four possible responses, ranging from