

Disease experience and care expectations in European patients with immune-mediated liver disease



Contact information
pierre.soret@aphp.fr

Pierre-Antoine Soret¹, Bert Tomsin², José Willemse³, Martine Walmsley⁴, Victorio Gnutti⁵, Wiebke Papenthin⁶, Natalie Uhlenbusch⁷, Tom JG Gevers^{8,9}, Nora Cazzagon¹⁰, Christoph Schramm¹¹, Pierre-Antoine Corret¹², Sara Lemoine¹ and Angela Leburgue¹² on behalf of ERN RARE-LIVER

¹ Sorbonne University, Reference Center for Inflammatory Biliary Diseases and Autoimmune Hepatitis, Saint-Antoine Hospital, Assistance Publique - Hôpitaux de Paris; Sorbonne University, INSERM, Saint-Antoine Research Center (CRSA), Paris, France ; ² Mijnlever patient association rare liver disease, Belgium ; ³ Nederlandse Leverpatiënten Vereniging, The Netherlands ; ⁴ PSC Support, Oxford, UK ; ⁵ AMAF Monza Onlus, Italy ; ⁶ Morbus Wilson e.V., Germany ; ⁷ Department of Psychosomatic Medicine and Psychotherapy, University Medical Center Hamburg-Eppendorf, Germany ; ⁸ Department of Gastroenterology and Hepatology, Maastricht University Medical Centre, Maastricht, The Netherlands ; ⁹ Nutrim School for Nutrition and Translational Research in Metabolism, Maastricht University, Maastricht, The Netherlands ; ¹⁰ Unit of Gastroenterology, Department of Surgery, Oncology and Gastroenterology, University Hospital of Padova, Padova, Italy ; ¹¹ Martin Zeitz Center for Rare Diseases and 1st Department of Medicine, University Medical Center Hamburg-Eppendorf ; ¹² Association ALBI, ERN patient representative, Paris, France

Introduction

Immune-mediated liver diseases (IMLD) are rare diseases that include autoimmune hepatitis (AIH), primary sclerosing cholangitis (PSC) and primary biliary cholangitis (PBC).

The prognosis of IMLD is variable but IMLD can lead to **cirrhosis** and the necessity of **liver transplantation**.

Symptoms as **fatigue**, **pruritus**, **depression** are frequent and can severely impair quality of life.

In context of IMLD, **gaps between physician's perception and patient's experience** of their disease can **mask patients' unmet needs**

Aim

The aims of our study were:

- 1/ to **assess patients' disease experience**, including impairment in quality of life, in context of IMLD
- 2/ to **characterize patients' expectation** regarding care and knowledge of their disease

Method

Observational international study, conducted in 2023 on behalf of ERN RARE-LIVER.

Study based on **online self-response survey (EU platform)**

Conception and revision of the survey by a **multidisciplinary group**: 5 physicians, 1 psychologist and 6 patient organization representatives (from Belgium, France, Italy, Netherlands, and United-Kingdom)

2 different sections in the questionnaire:

- Section 1: **Open-ended questions** about disease feeling and impairment in quality of life
- Section 2: **Focus questions** about patients' expectations in care, disease information and patient-physician relationship

Inclusion criteria:

- Adult patient with a confirmed diagnosis of AIH, PSC or PBC in Belgium, France, Italy, Netherlands and UK
- Overlap syndrome AIH-PBC or AIH-PSC were accepted
- Transplanted or cirrhotic patients were excluded

Results

Table 1. Population of the study

	PBC	PSC	AIH	Overlap	Total
Participants	294	153	202	81	730
< 18 years (%)	0	0	4 (2,0)	1 (1,2)	5 (0,7)
Transplanted (%)	3 (1,0)	19 (12,4)	3 (1,5)	3 (3,7)	28 (3,8)
Cirrhotic (%)	35 (11,9)	36 (23,5)	45 (22,3)	21 (25,9)	137 (18,8)
Included	256	98	150	56	560

Figure 1. Patients' self declaration of 3 main uncontrolled symptoms (A) and impact in quality of life (B)

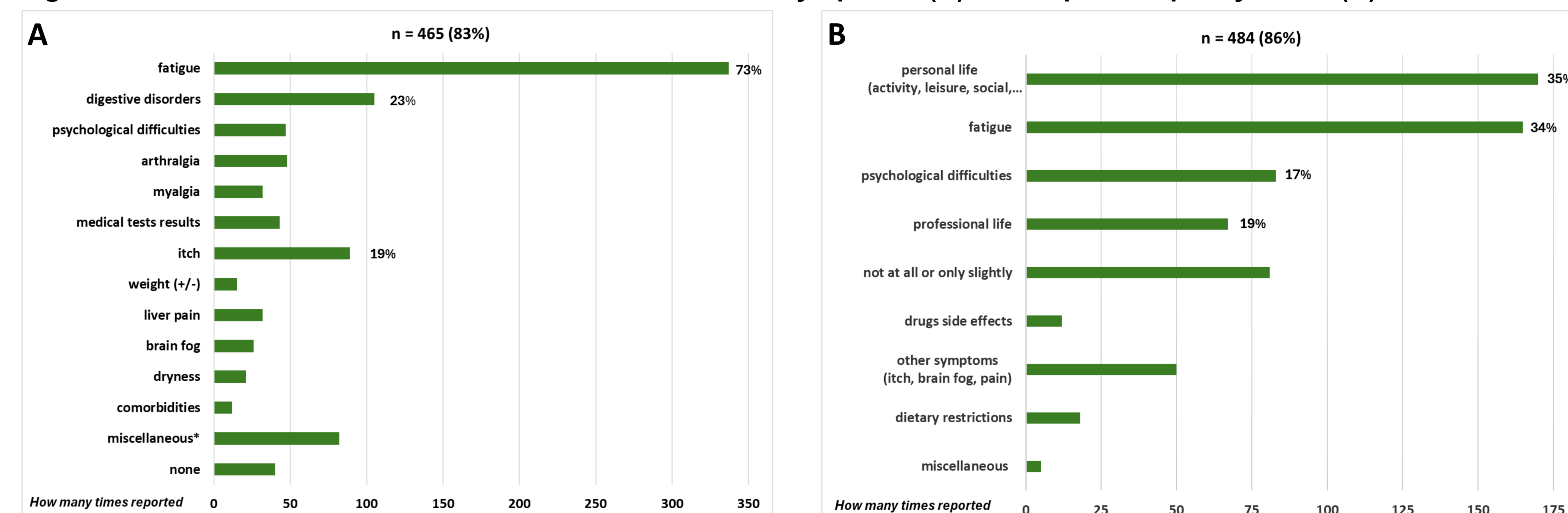
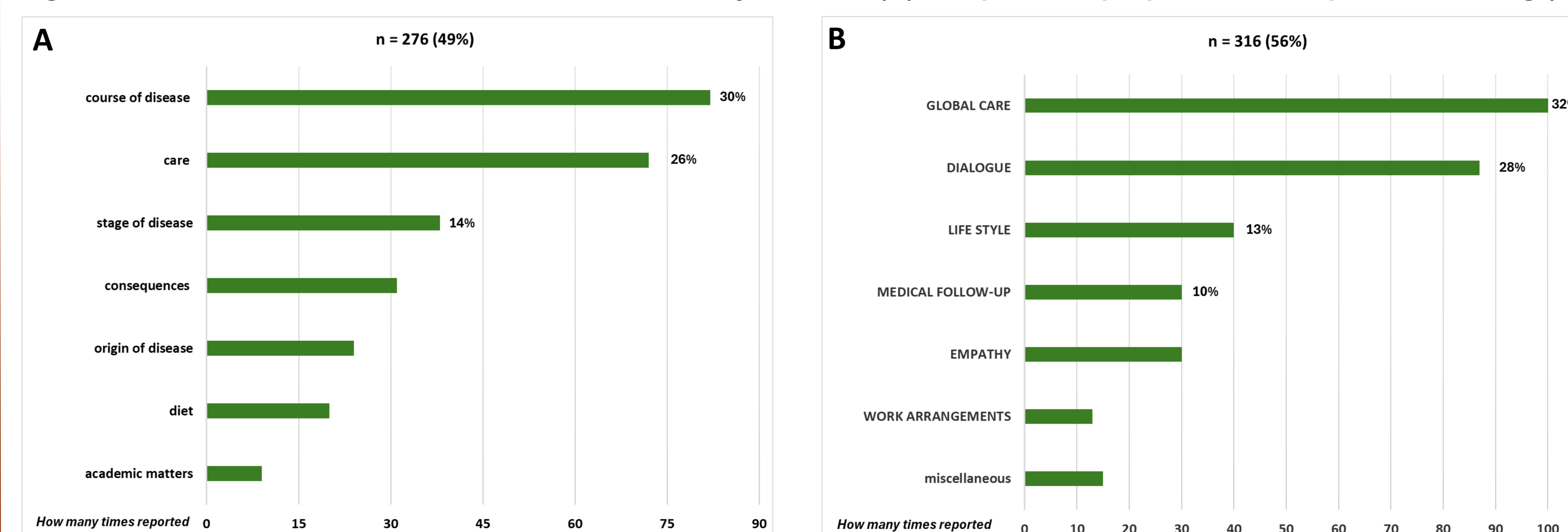


Figure 2. Questions never asked or without satisfactory answers (A) and patients' proposition to improve wellbeing (B)



Conclusions

The 3 main declared uncontrolled symptoms were: **fatigue** (73%), **digestive issues** (23%) and **pruritus** (19%)

Impact of the disease in quality of life was reported in 72% of patients, with **impairment in personal life** (35%) and **professional life** (19%). 17% of patients **declared psychological** relating to their disease

The main patients' questions that had never been asked or that had not had satisfactory answers related to the **course and the stage of the disease**, the **global care** and **consequences to their health or life**

Patients asked for a **better global care** including **symptom management and psychological support**, a **more open and empathic dialogue** with their physicians and **advice on adapting their lifestyle**.