EMA stakeholder interaction on the development of medicinal products for chronic non-infectious liver diseases (PBC, PSC, NASH)

PSC Patient Perspective

Martine Walmsley

On behalf of the **PSC Patient Expert Pool**

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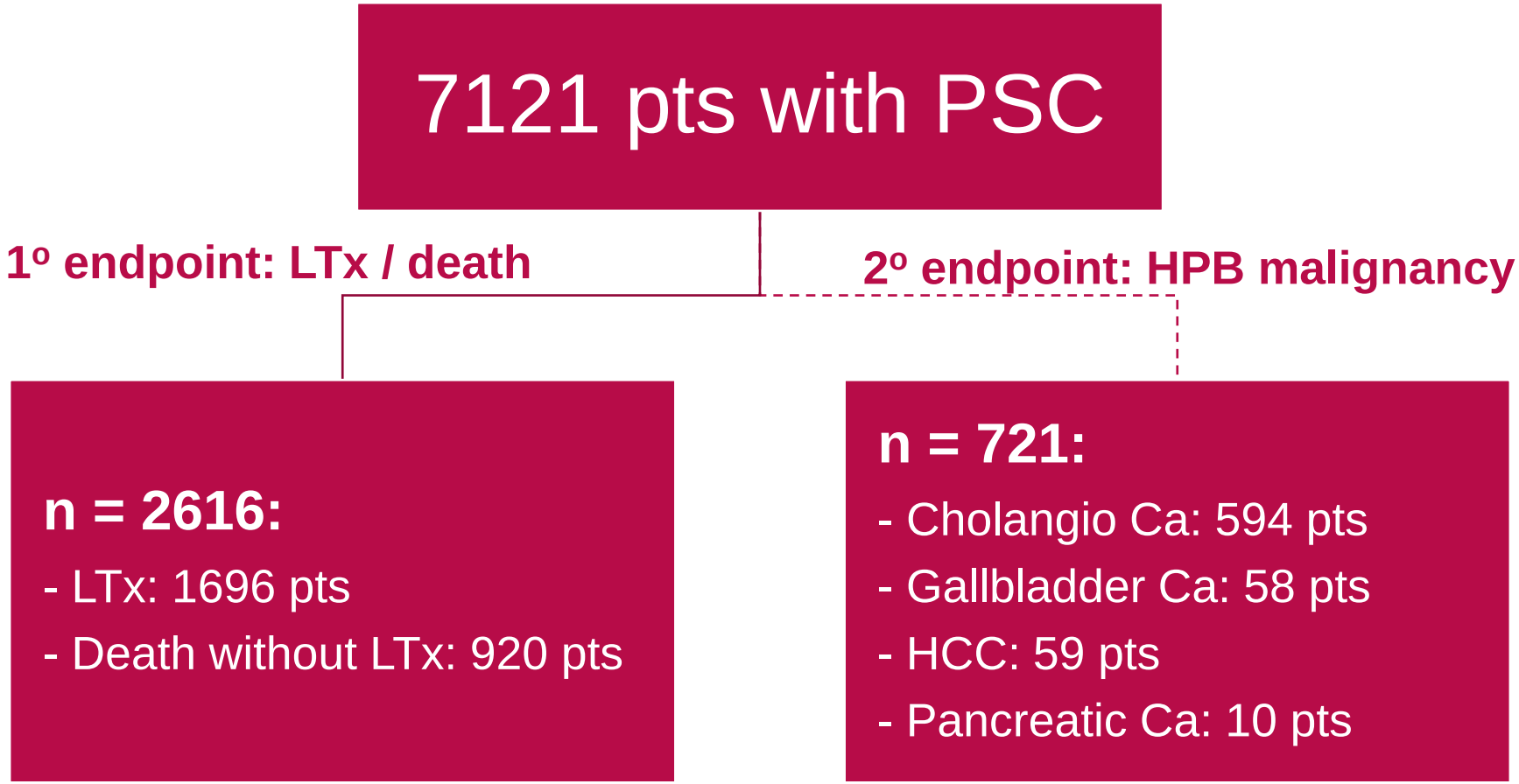
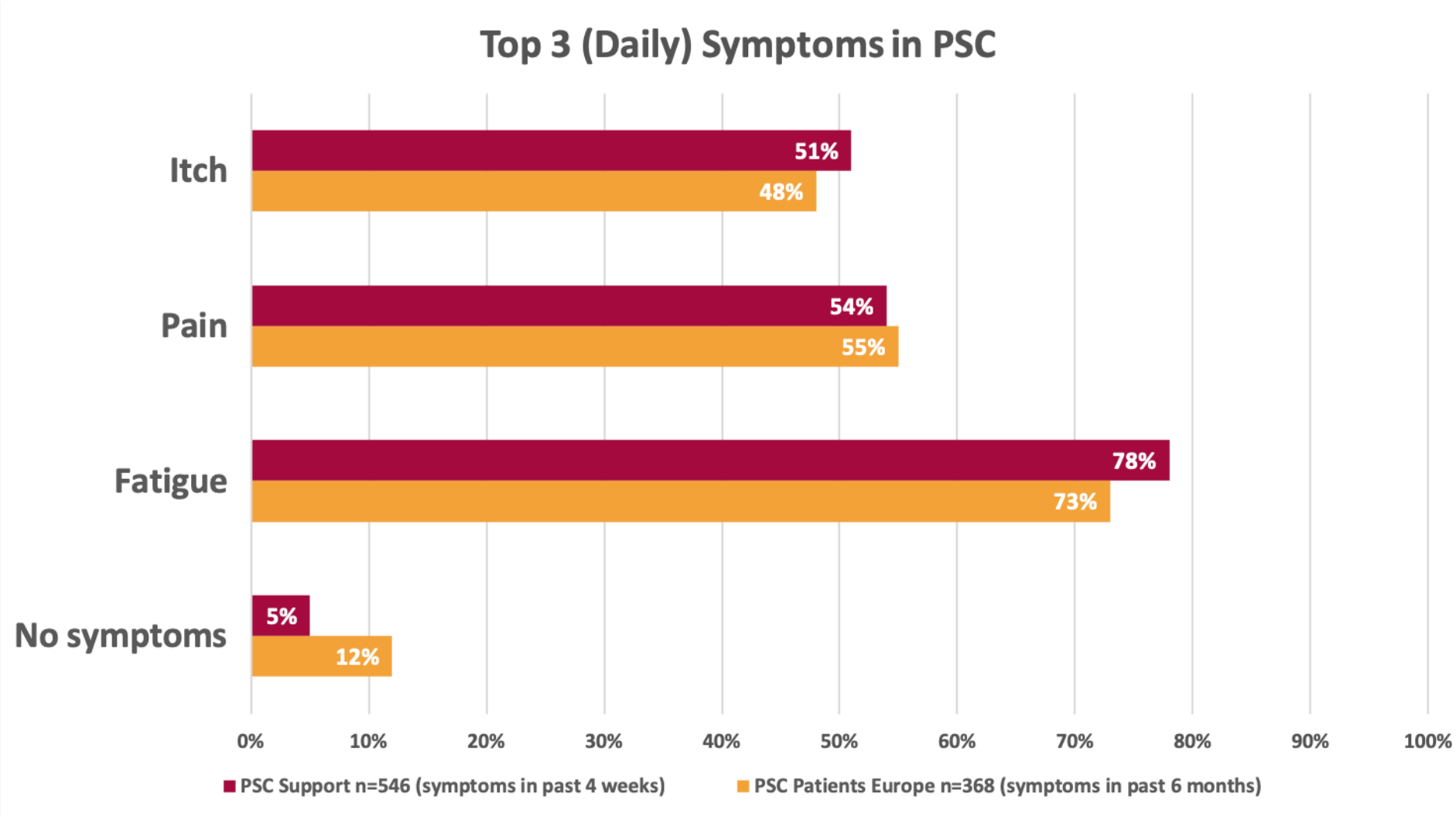
The Impact of PSC

‘Patient age, sex, and inflammatory bowel disease phenotype associate with course of primary sclerosing cholangitis’



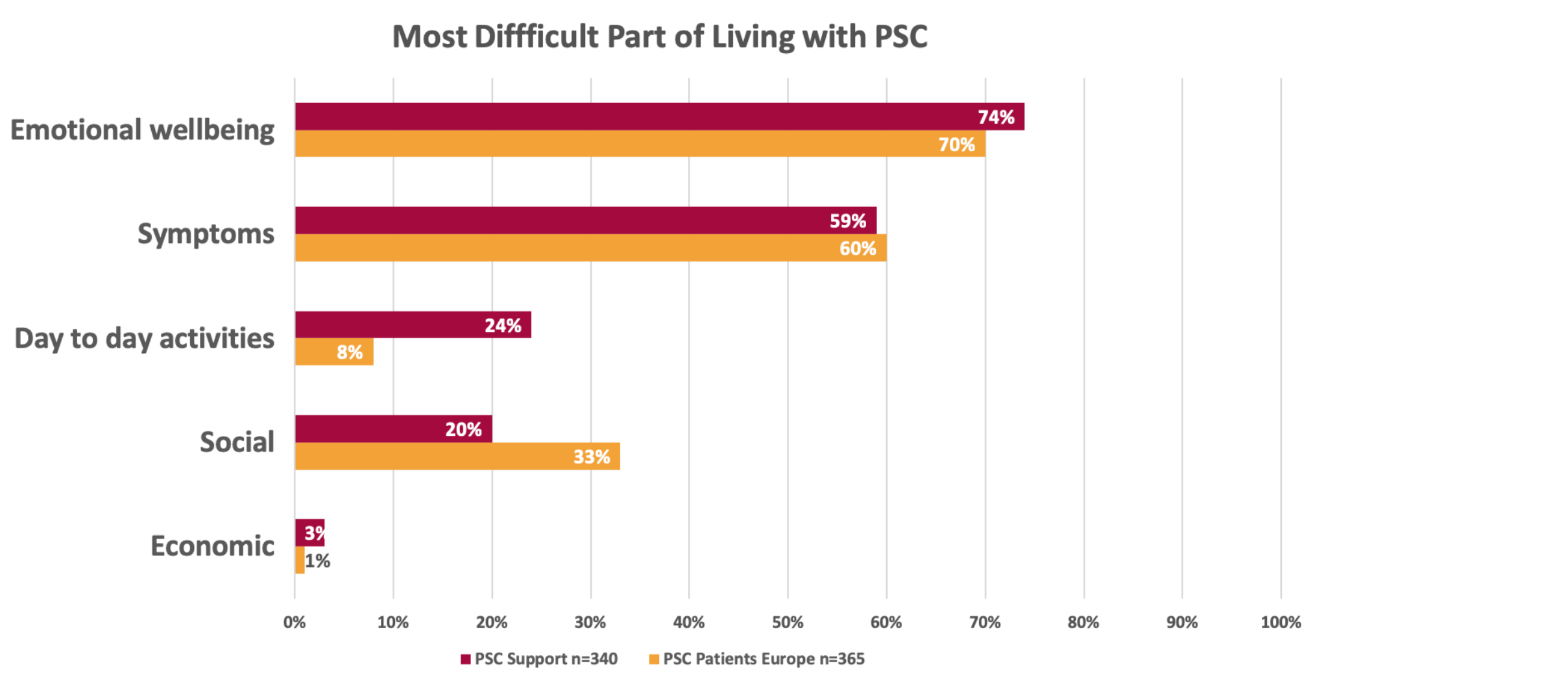
Weismüller*, Trivedi* et al. *Gastroenterology*, 2017

PSC: Daily Symptoms



Sources: pscsupport.org.uk/unmetneeds and pscpatientseurope.org/psc-patients-survey

PSC: Living with PSC



Sources: pscsupport.org.uk/unmetneeds and pscpatientseurope.org/psc-patients-survey

PSC: Living with PSC

- **Uncertainty and helplessness**
  - Long term
  - Day to day
- **Physical and emotional burden**

“Trying to lead a normal life while suffering from symptoms that most people don’t understand or can't relate to.”

Sources: pscsupport.org.uk/unmetneeds

Living **better** with PSC

- **Lived experience** of PSC is a huge area of unmet need
- Improvement includes a **better quality of life**
- What patients want:

  “give me more peace of mind”

  “allow me to work”

  “allow me to lead an active life”

Sources: pscpatientseurope.org/psc-patients-survey

Burden of Symptoms - Patient Reported Outcomes (PROs)

- Use **Patient Reported Outcomes** as endpoints, especially:
    - Fatigue
    - Itch
    - Pain
    - Quality of life
- Develop and validate/qualify **Patient Reported Outcome Measures** for use in clinical trials
    - Patients engaged in PROM-related activities
    - PSC PRO, UK-PSC Quality of Life Measure
- Value of PROs post-authorisation (HTA)

Living longer with PSC

- Patients don’t want an **early death**
- Develop **new medicines** that show meaningful and convincing results to modify the disease in order to:
    - **prolong life** with PSC
    - reduce PSC **complications**
    - reduce bile duct **infections**
    - prevent or reduce **risk of cancer**
    - prevent or reduce occurrence of **rPSC**
    - **predict progression** of PSC

Validate Surrogate Endpoints

- Co-ordinated development programmes towards validating **novel non-invasive** endpoints:
    - Use **common exploratory** endpoints
    - Use new and emerging **technologies**
    - **Replace biopsies**
- Longer-term outcome observation
- Share (placebo) data
- Patients want to **live longer**, even if that's with PSC

Real World PSC

- Creative trial design to incorporate **different phenotypes** and medications:
    - co-existing diseases, ages, disease stage and severity, fibrosis, inflammation, colon/bile duct damage…
- Biochemistry fluctuations and cholangitis
    - **Screening windows** and **re-screening** opportunity
- Study impact of treatment on **co-existing diseases**
    - especially IBD
- UDCA

Eligibility Criteria

- **Broader eligibility criteria**

Approve treatments for the majority, not the minority

Summary

- Engage the PSC patient community
    - well-educated, well-organised and expertise within our networks
- Act with urgency
- Coordinate development programmes to validate/qualify endpoints
    - *include* **non-invasive** surrogates
    - *include* **Patient Reported Outcomes** always

We want to live as well as possible, as long as possible

Thank you

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