



PSC Forum 7

Tuesday, June 4, 2024

Starhotels Business Palace

Via Privata Pietro Gaggia, 3, 20139, Milan, Italy

PATIENT EXPERIENCE SUB-WORKING GROUP
ACUTE CHOLANGITIS PATIENT EXPERIENCE SURVEY

STEPHEN ROSSI PHARM.D.
CHIEF SCIENTIFIC OFFICER
PSC PARTNERS SEEKING A CURE



Acute Cholangitis Patient Experience Survey

Disclosures:

Consultancy and Grants to PSC Partners – ChemoMab, Escient, GSK, Mirum, Pliant, Rectify

Acute Cholangitis Patient Experience Survey

Goal of Survey

- Acute cholangitis as a potential endpoint
 - Clinical endpoint for potential full approval
 - Outcomes event in Confirmatory Trials for Accelerated Approval
- Potential Uses of Patient Symptom PROs in trials
 - Event frequency and adjudication
 - Monitoring change in patient symptoms
- Quantitative high-level survey is an important first step for development of a PRO as a clinical endpoint
- Significant qualitative, sensitivity and statistical analyses required for formal development and validation

Acute Cholangitis Patient Experience Survey

Survey Modules

- Patient and Disease Characteristics
- Acute cholangitis symptoms
 - Attack history
 - Symptom frequency and severity at start, during, and after most recent attack
 - Symptom outcomes
 - Abdominal pain locator
 - Relationship to IBD
- Access to care and treatments
 - Care providers
 - Antibiotic use
 - Procedures and other interventions

Acute Cholangitis Patient Experience Survey

Patient Disposition



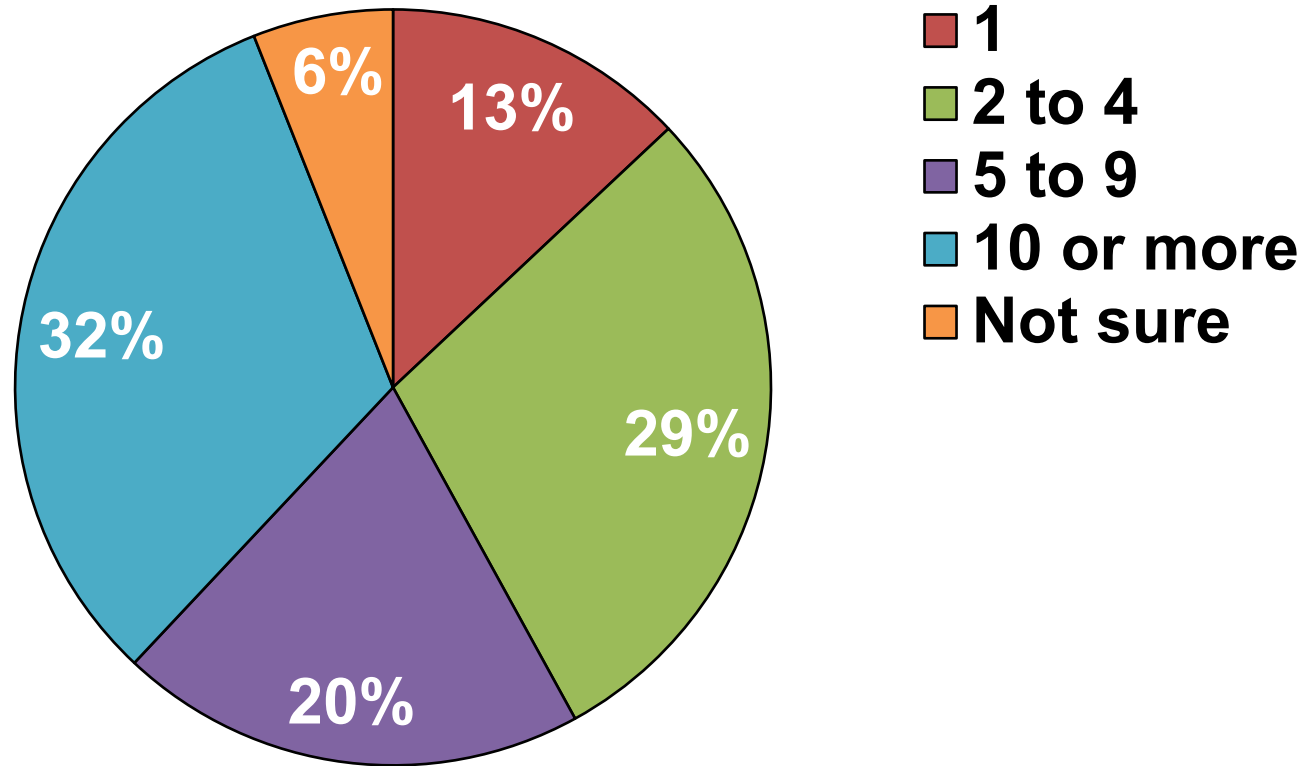
Acute Cholangitis Patient Experience Survey

Key Patient Characteristics

Female/Male	64% Female / 36% Male
Age	50.2 (18-80)
IBD	217 (61%)
<i>UC</i>	156 (44%)
<i>Crohn's</i>	46 (13%)
<i>Indeterminate/Other/Not Sure</i>	15 (4%)
Other Medical Conditions	
<i>Cirrhosis</i>	120 (34%)
<i>Listed for OLT</i>	24 (7%)
<i>Post-OLT</i>	78 (22%)
<i>CCA</i>	8 (2%)
<i>Gallbladder Removed</i>	177 (50%)
<i>Gallbladder Cancer</i>	2 (1%)

Acute Cholangitis Patient Experience Survey

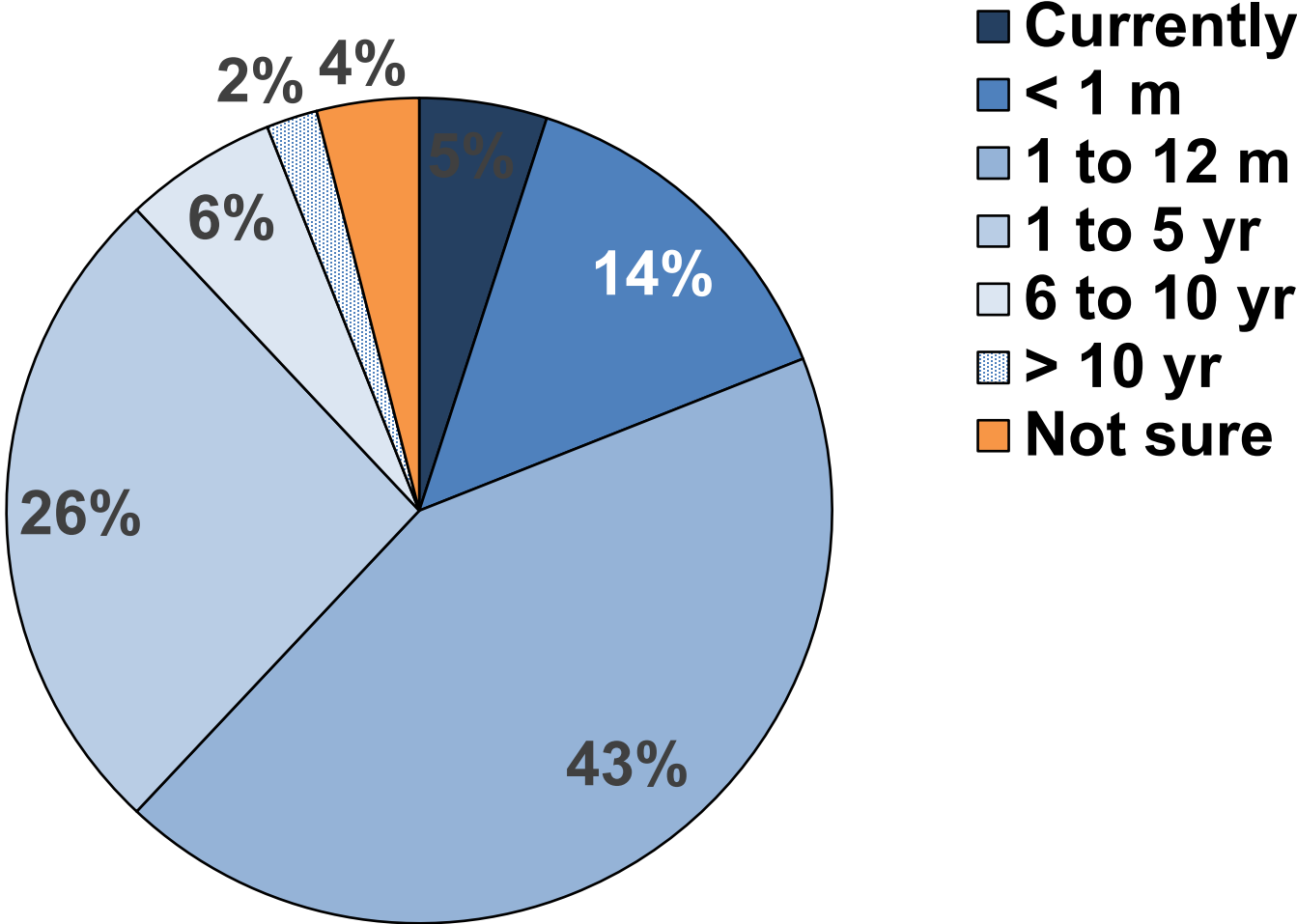
Total Attacks Since Diagnosis



Timing of first AC attack relative to PSC diagnosis ranged from before (38%), concurrent (14%) and after (48%)

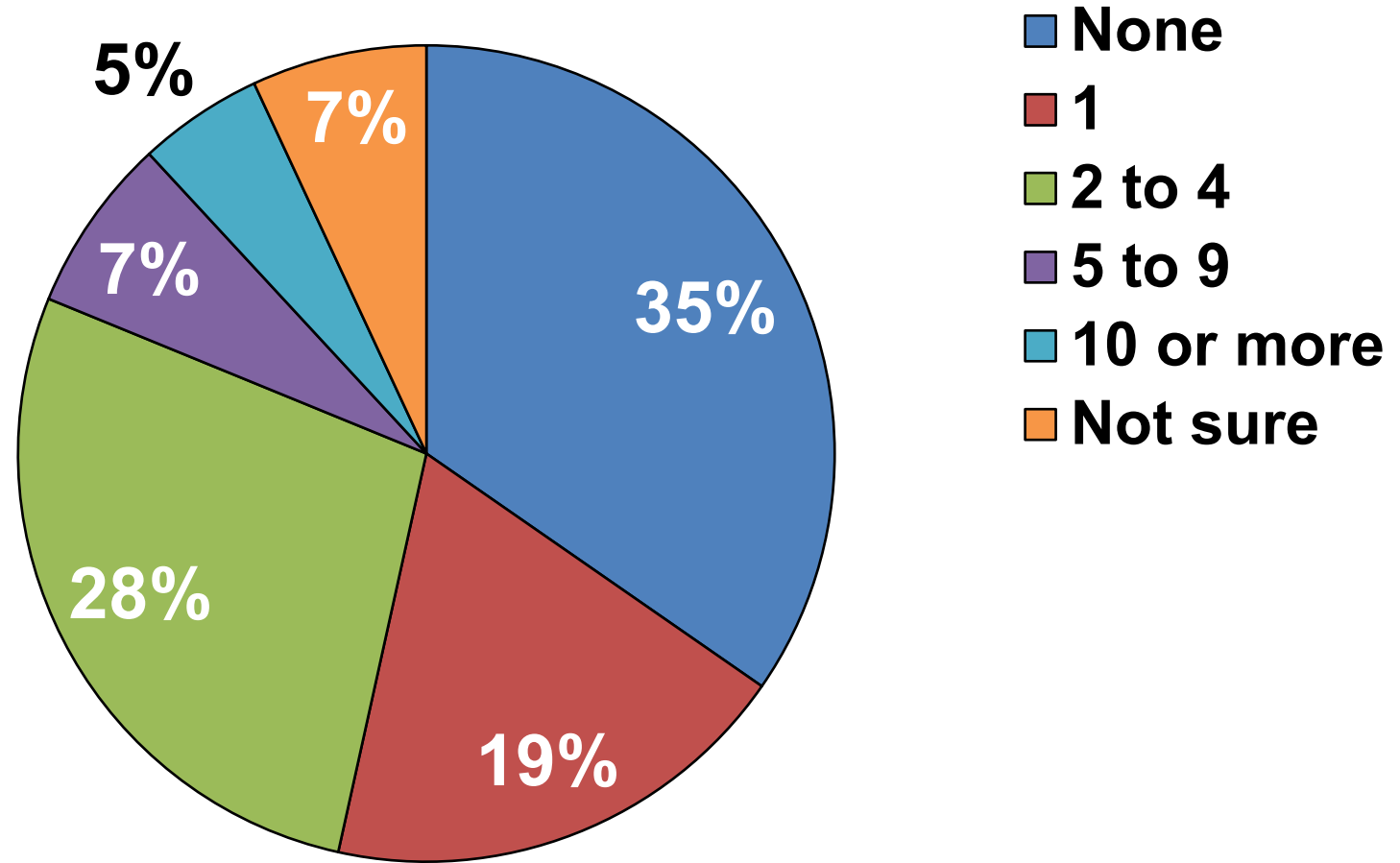
Acute Cholangitis Patient Experience Survey

Timing of Most Recent Attack



Acute Cholangitis Patient Experience Survey

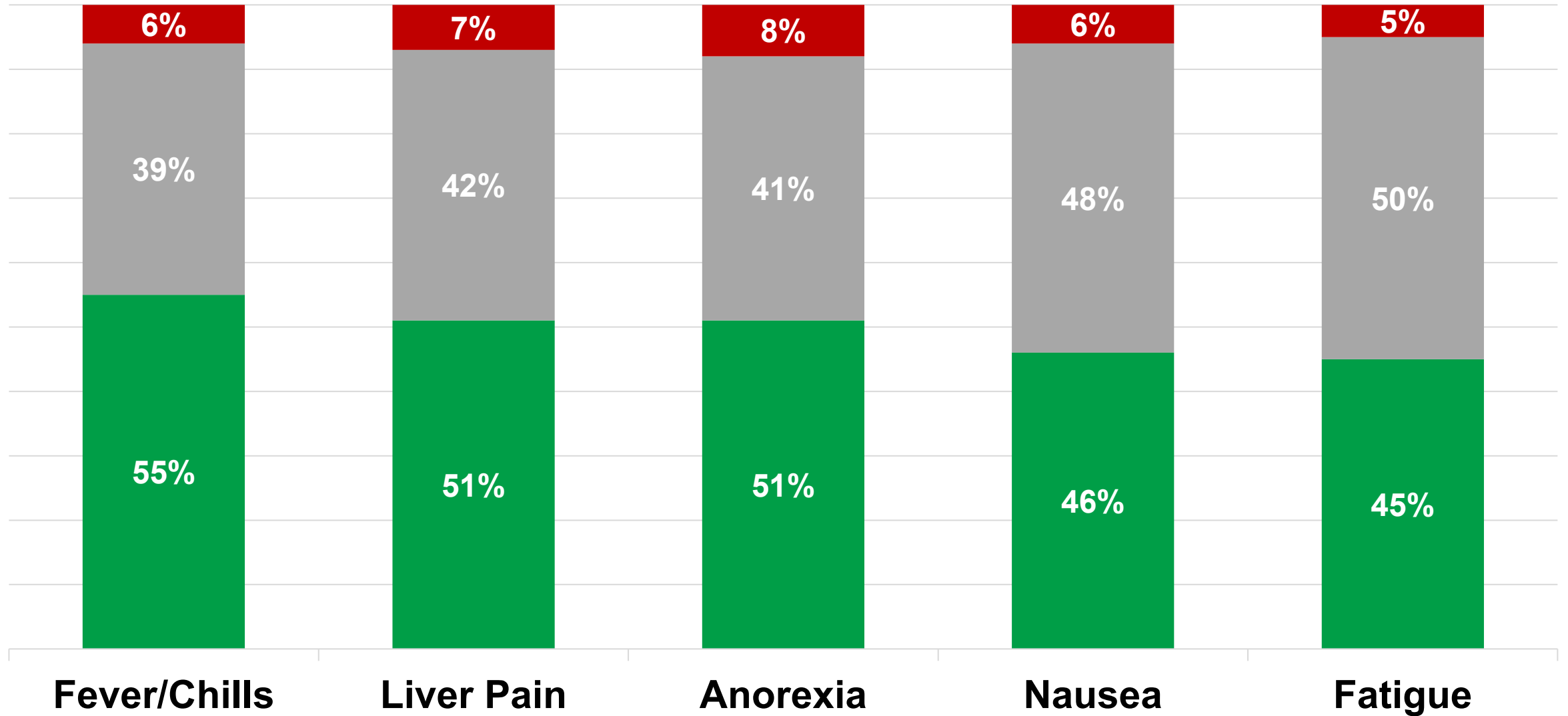
Attacks in the Past Year



Symptom	Start of Attack	During Attack (At Worst)	After Attack (At Worst)
Fatigue	83%	92%	89%
Liver pain	77%	86%	76%
Anorexia	72%	81%	65%
Fever/Chills	69%	76%	43%
Nausea	63%	70%	53%
Bodily aches	60%	74%	60%
Choluria	56%	68%	50%
Night sweats	54%	66%	47%
Pruritus	52%	66%	60%
Insomnia	50%	77%	69%
Other Abd. Pain	43%	61%	53%
Brain fog	39%	60%	55%
Jaundice	35%	47%	35%
Anxiety	28%	54%	57%
Vomiting	27%	40%	24%

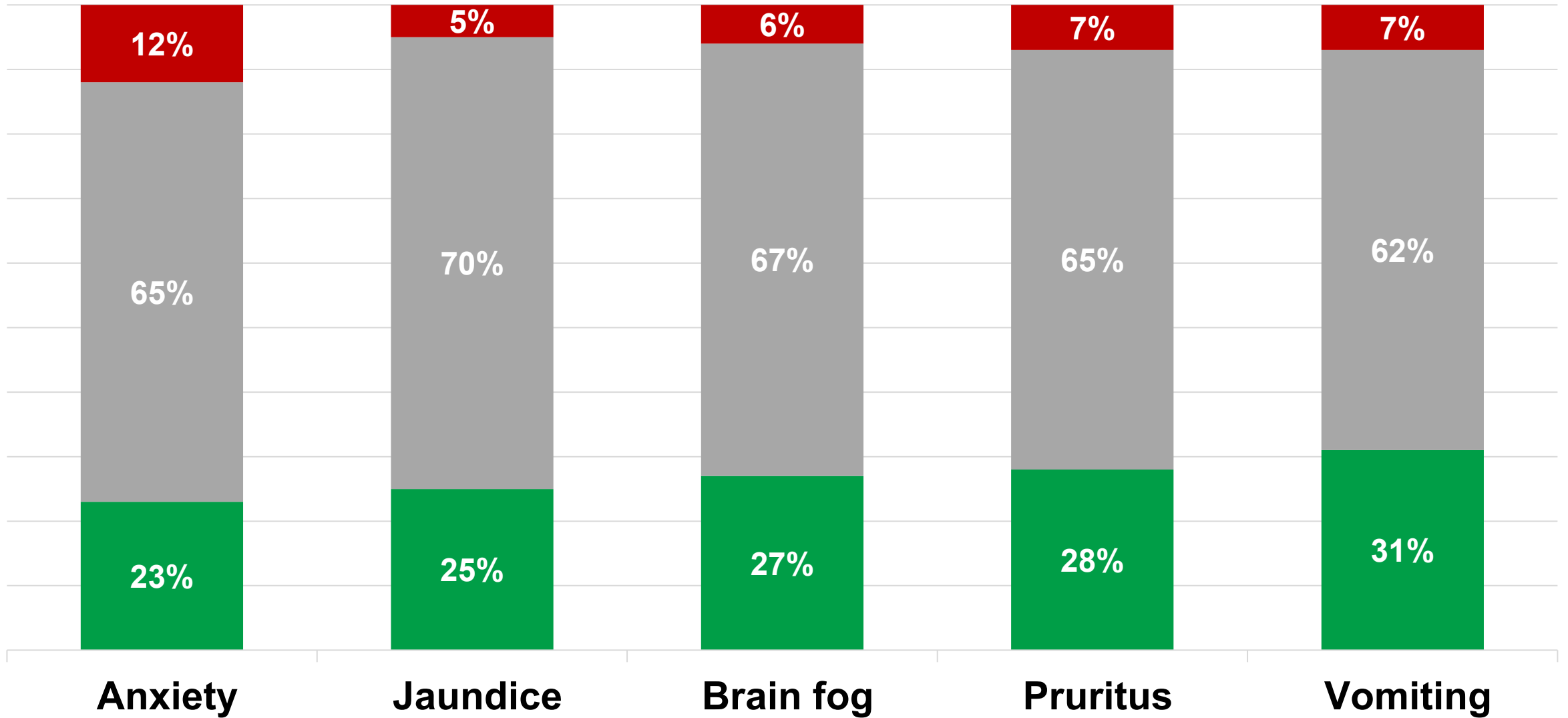
Change in Symptom Severity – Most Improved

■ Improve ■ No Change ■ Worsen



Change in Symptom Severity – Least Improved

■ Improve ■ No Change ■ Worsen



Acute Cholangitis Patient Experience Survey

Symptom Outcomes – Δ from During to After Attack

	All Severity	Severe
Fever/Chills	-34%	-17%
Night Sweats	-19%	-15%
Nausea	-17%	-18%
Choluria	-17%	-17%
Anorexia	-16%	-12%
Vomiting	-16%	-16%
Bodily Aches	-14%	-11%
Jaundice	-12%	-9%
Liver Pain	-10%	-27%
Other Abd. Pain	-8%	-20%
Insomnia	-8%	-16%
Pruritus	-6%	-14%
Brain fog	-5%	-8%
Fatigue	-2%	-24%
Anxiety	3%	-7%

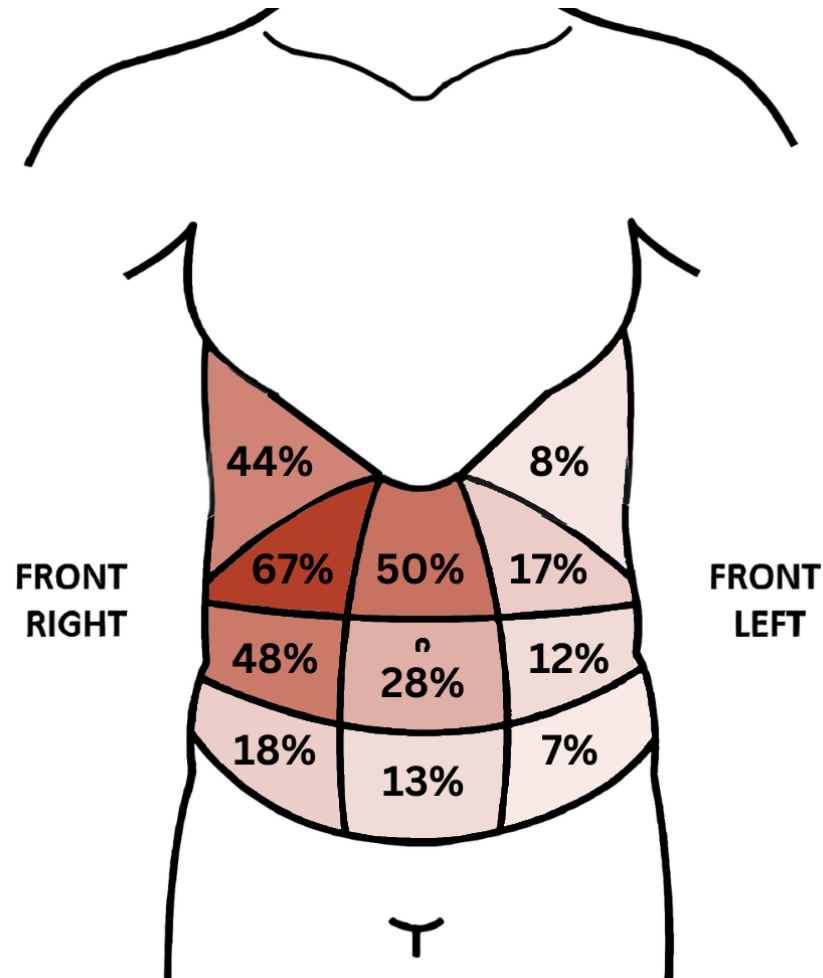
Acute Cholangitis Patient Experience Survey

Symptom Outcomes – Resolved vs New Onset

	Resolved	New Onset
Vomiting	50%	6%
Fever/Chills	46%	9%
Night sweats	31%	6%
Nausea	29%	9%
Jaundice	27%	5%
Anorexia	25%	18%
Choluria	25%	5%
Other Abd. Pain	24%	16%
Bodily Aches	22%	12%
Brain fog	17%	7%
Difficulty sleeping	15%	16%
Liver pain/discomfort	14%	16%
Pruritus	11%	7%
Anxiety	10%	15%
Fatigue	6%	33%

Acute Cholangitis Patient Experience Survey

Abdominal Pain Locator



- Patients asked to pick all the locations of pain during attack (multiple locations allowed)
- Primary pain located in RUQ area
- Independent of IBD, advanced disease or post-transplant
- 70% of patients could clearly differentiate pain from AC attack from IBD flare
- Other reported pain locations ($\geq 10\%$)
 - Bodily aches or pains 74%
 - Back 17%
 - Right Shoulder 10%

Acute Cholangitis Patient Experience Survey

Interventions/Treatments for Most Recent Attack

Sought Medical Care	76%
Any Antibiotics	86%
Oral	29%
IV	11%
Both Oral & IV	60%
ERCP	44%
Stent/Balloon Dilation	26%
MRI	68%
Ultrasound	57%

Acute Cholangitis Patient Experience Survey *Summary*

- Large cohort reporting patient experience and the broad range of symptom frequency and severity
- Initial key step in PRO development for symptoms
 - Inherent limitations of patient reported data
 - Large sample size is key
- No single symptom likely to provide adequate specificity or sensitivity for AC
- Significant need to develop a multi-symptom PROM for diagnosis and monitoring of acute cholangitis for clinical trials and patient care

Acute Cholangitis Patient Experience Survey

Acknowledgements

PSC Patient Community and Patient Organizations for Sharing Their Experiences

Sub-Working Group

Martine Walmsley - Lead

Donna Evon

Alastair Garfield

Joanne Hatchett

Bryce Reeve

Stephen Rossi

Pam Vig

Survey Development & Analysis

Kerrie Goldsmith

Rachel Gomel

Ricky Safer

Brian Thorsen

Mary Vyas