

The Patient Perspective

Insights on Clinical Trial Participation and Retention from the PSC Partners Community



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PSC Patient and CEO of PSC Partners Seeking a Cure

PSC Partners Seeking a Cure

The mission of PSC Partners Seeking a Cure is to provide education and support to PSC patients, families and caregivers and to raise funds to research causes, treatments and a cure for primary sclerosing cholangitis.



Organizational Overview

- Established in 2005
- \$2.6 million awarded in research grants
- Patient reported registry: 1194 participants
- 13 annual patient & caregiver conferences
- Active online community: 4000+ participants
- Average of 120 website visitors per day
- Canadian affiliate launched in 2017



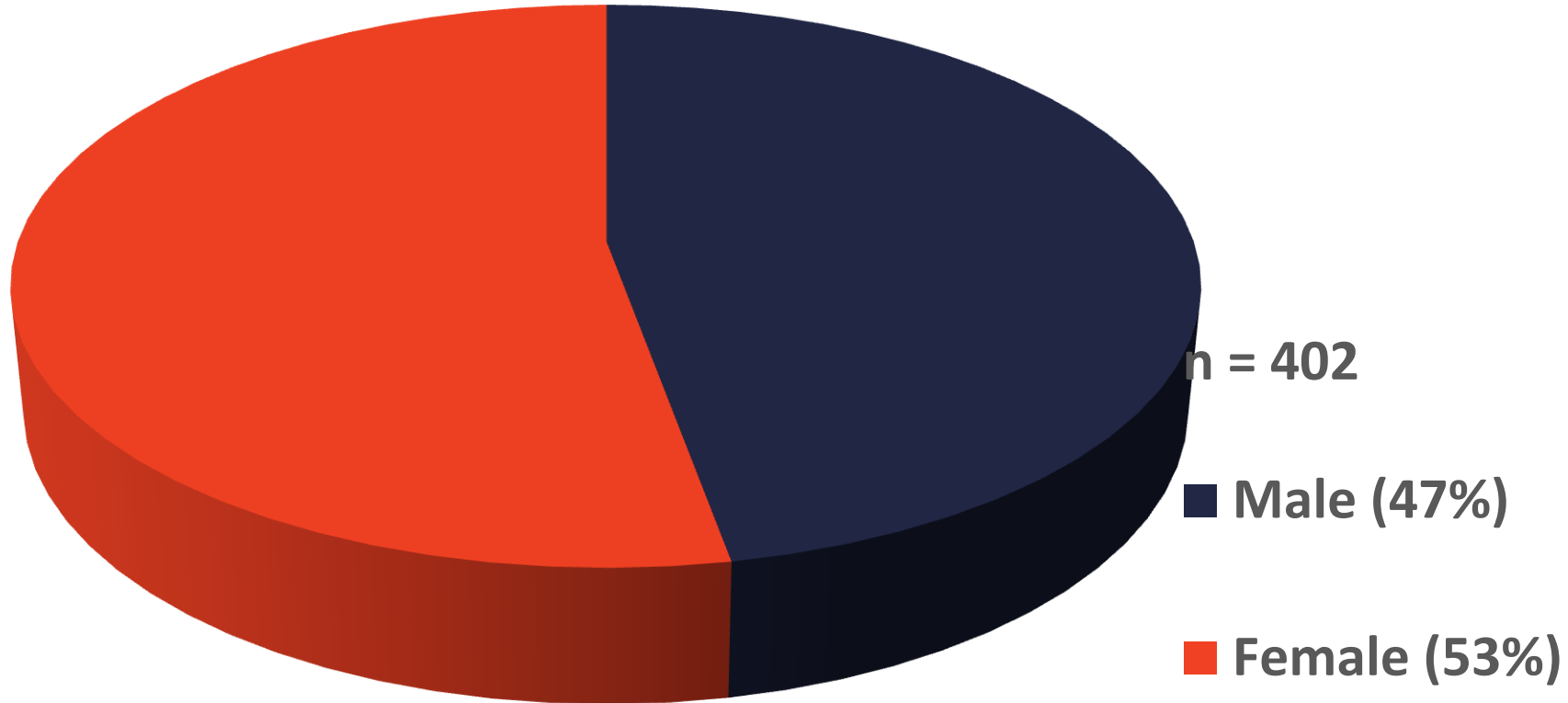
PSC Partners Patient Registry

- 1178 patients & growing
- Annual IRB review
- HIPAA and FISMA compliant
- Medical proof of diagnosis required
- Proposal review by medical committee and PSC Partners
- Patient data includes:
 - Diagnosis
 - General health
 - Surgeries
 - Family history
 - Symptoms
 - Medications
 - Demographics
- Complements academic and clinical research



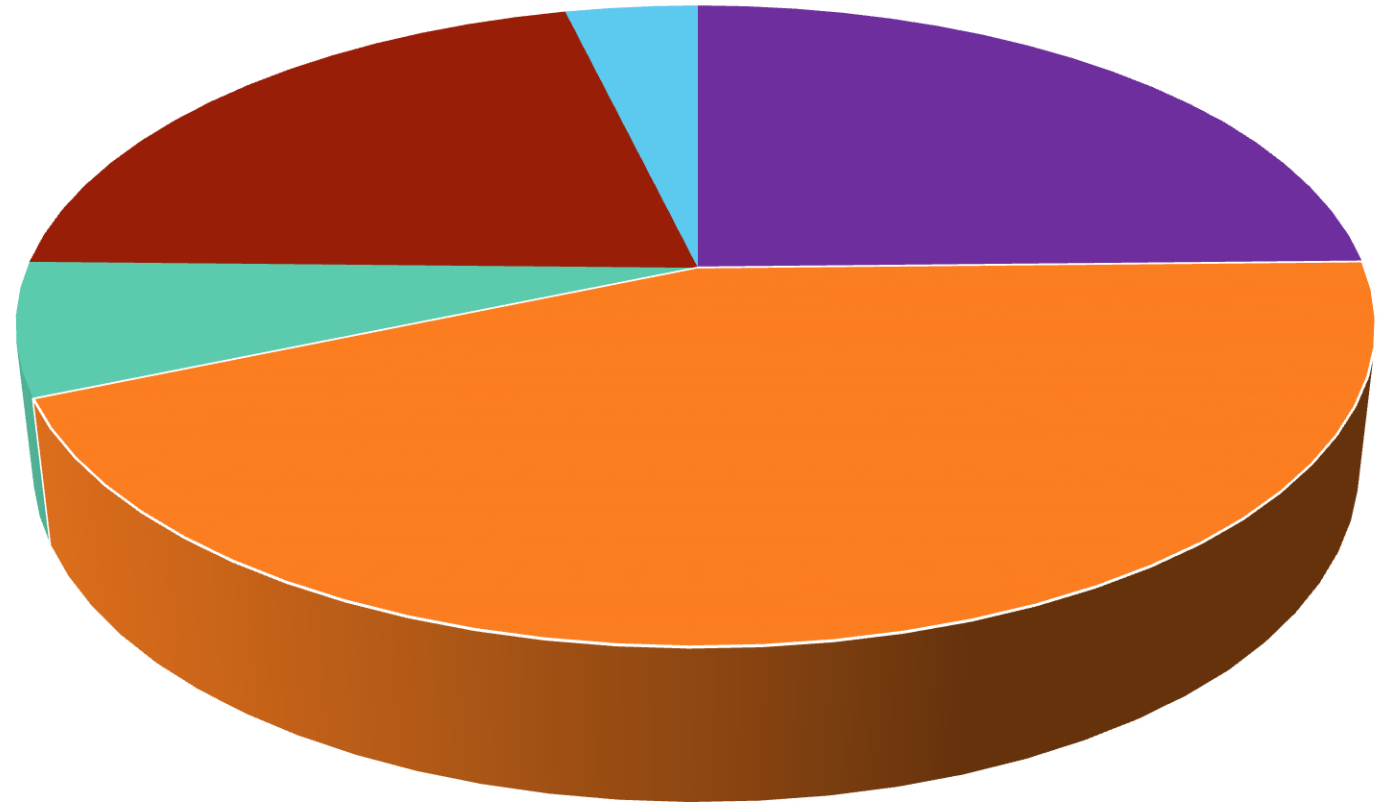
<http://pscpartners.org/patient-registry/>

Gender of Survey Participants

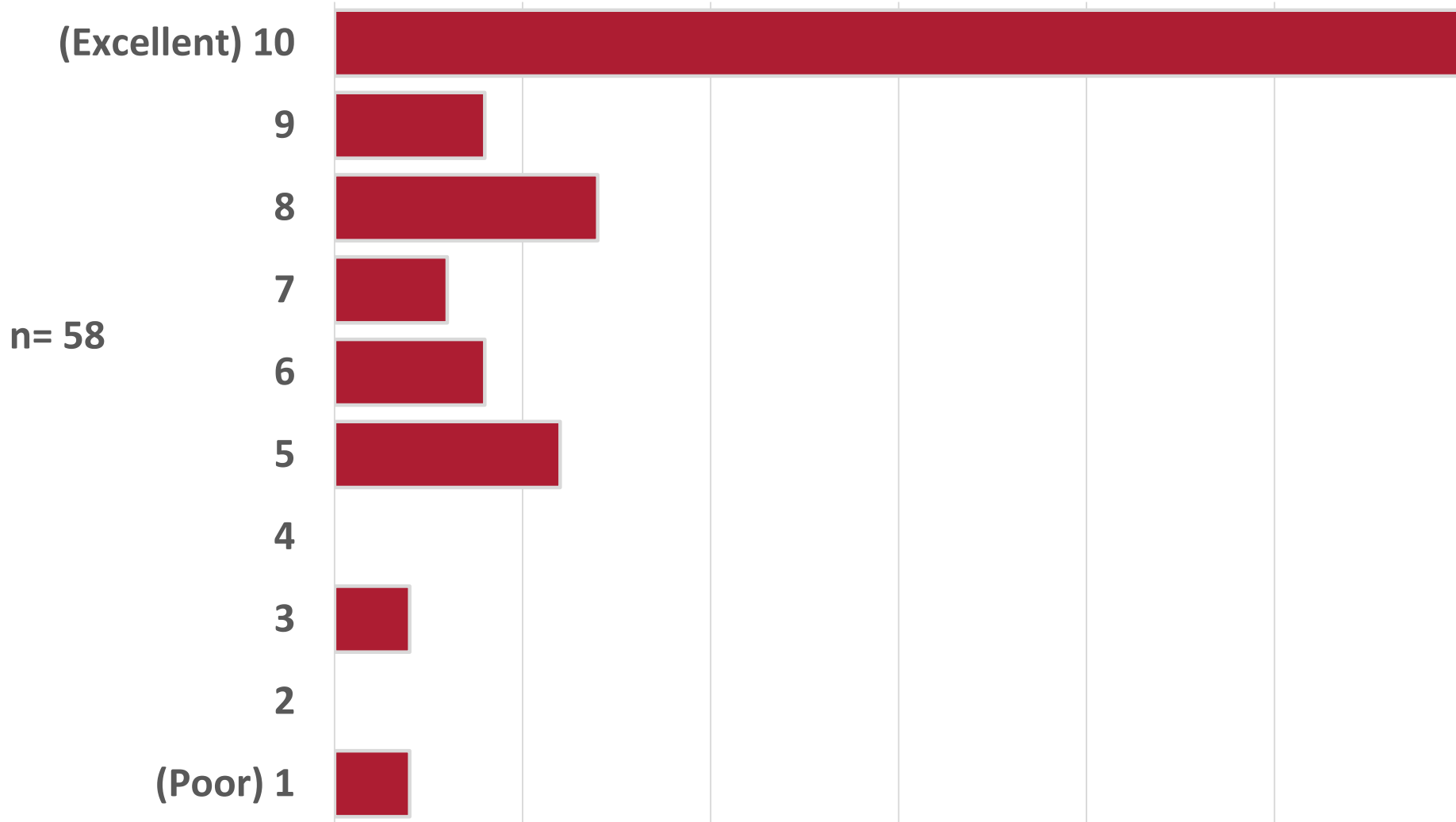


Participant Disease Stage

- Asymptomatic (24.6%)
- Mildly Symptomatic (43.7%)
- Severely Symptomatic (6.7%)
- Post-Transplant (20.8%)
- Other (3.7%)



Clinical Trial Experience Rating



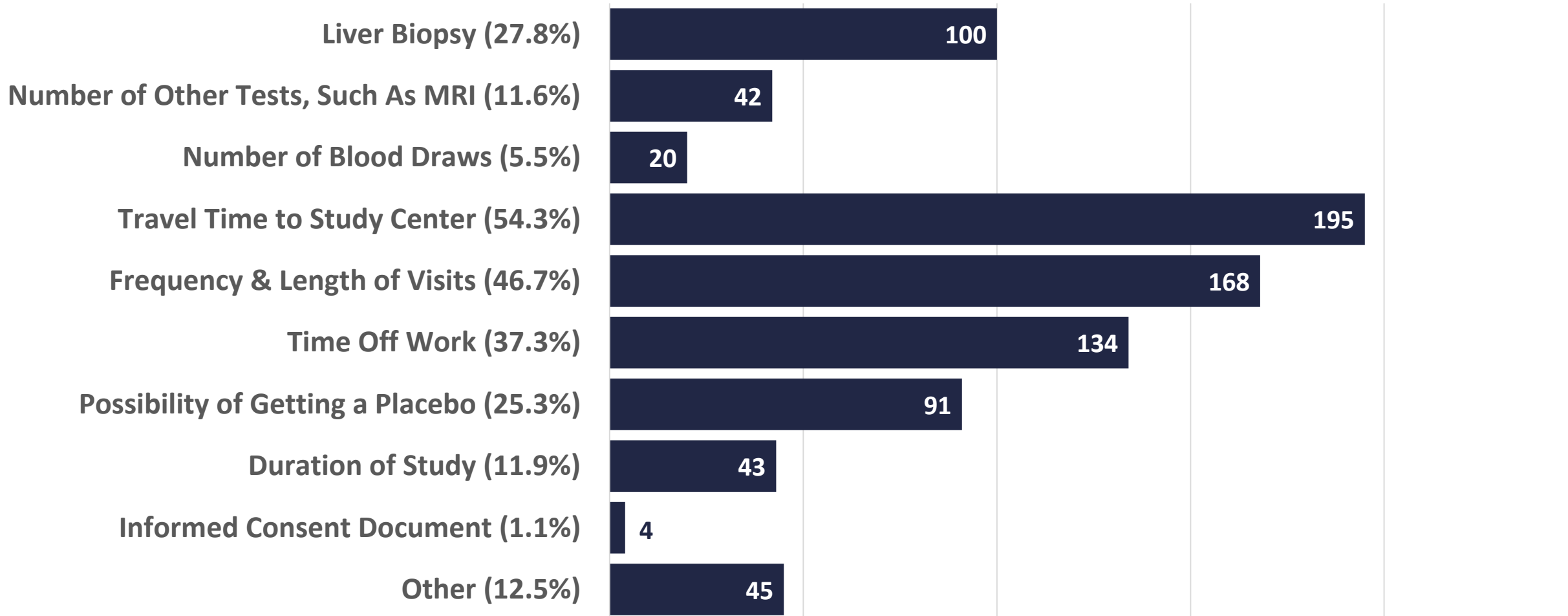
General Concerns About Enrolling in a Clinical Trial



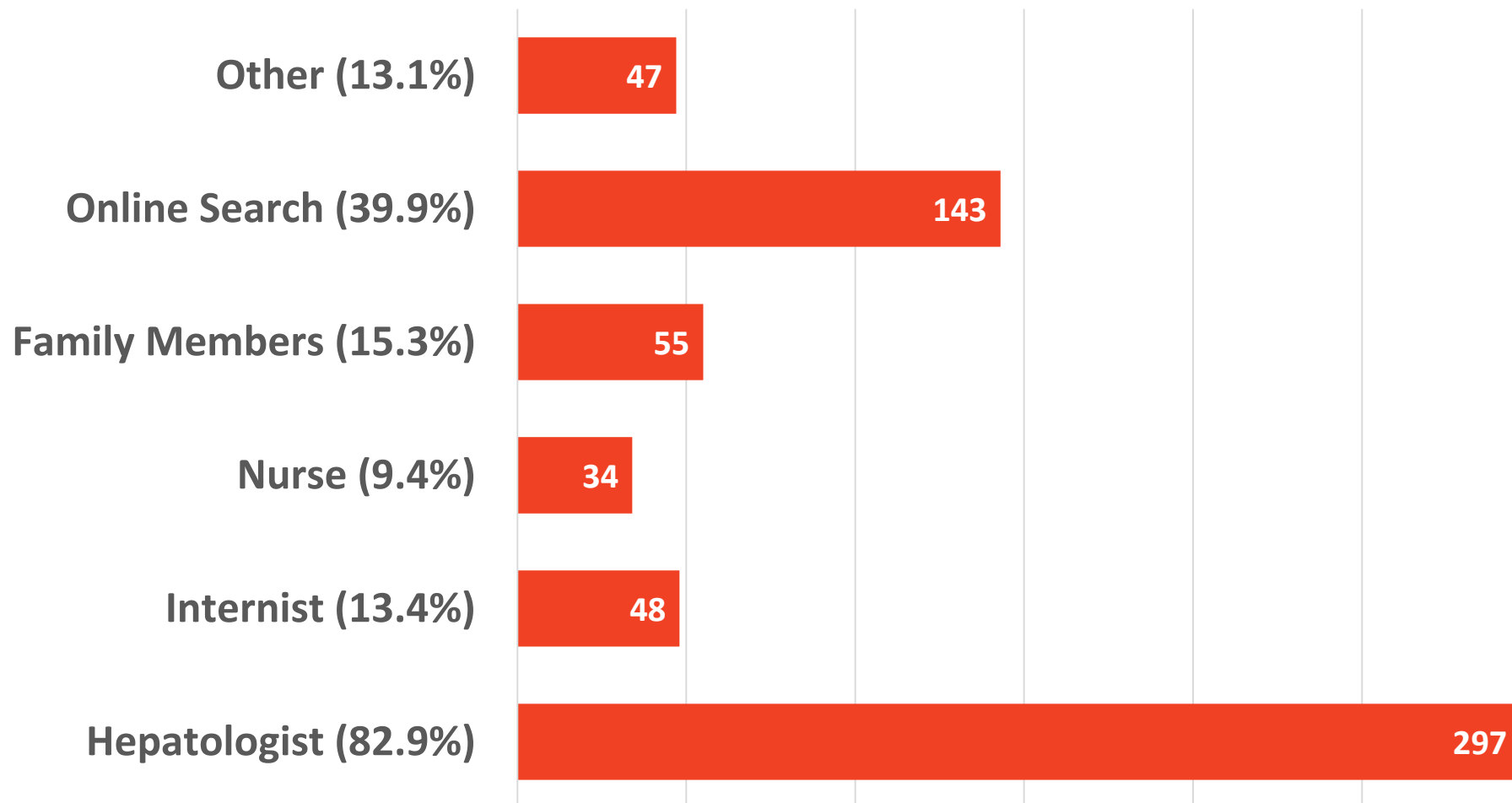
- General fear of the unknown
- Unknown side effects or long-term effects
- Possibility that the drug might affect current treatment for concurrent diseases
- Being in the placebo group
- Decreasing chance for transplant
- Disruption to daily family life
- Fear of jeopardizing current quality of life



Specific Trial Requirements as Obstacles



Where Patients Turn for Advice



The Hepatologist in the Clinical Trial Process

“I would participate in a clinical trial if my hepatologist told me that the pharmaceutical company was responsible, that the drug wouldn’t hurt me, that he would go to bat for me to prescribe the drug after the trial was over. I would want my hepatologist to be part of the trial process. The trial would have to be part of my care, as if my hepatologist were the one telling me ‘let’s try this new treatment and see how you do with it.’”

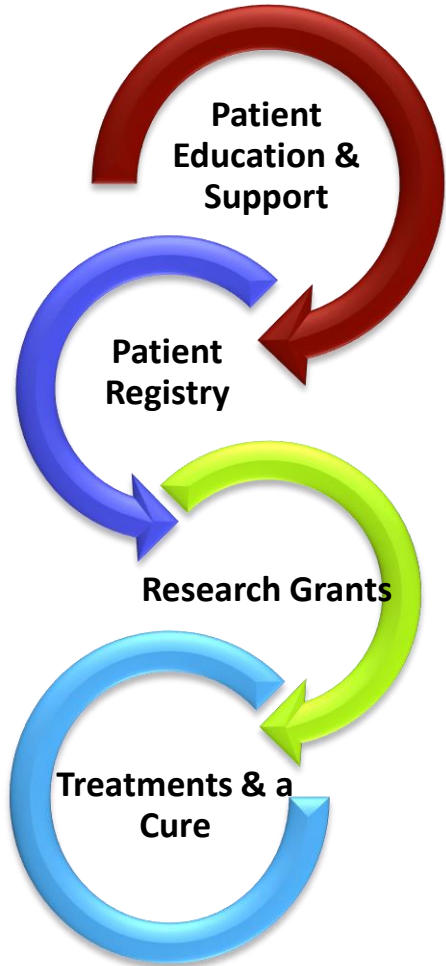
Patient Suggestions to Improve the Clinical Trial Experience



- Enable an honest dialogue with the team of researchers
 - Pre-enrollment
 - During the trial
 - Following trial completion
- Offer partial reimbursement
- Expand inclusion/exclusion criteria
- Optimize length of each trial visit



Most important is the need for multi-stakeholder education and meaningful collaboration!



Thank You!

Together in the fight, whatever it takes...

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