

THE FORUM
For Collaborative ResearchSM

Session III

Collaborations Across Datasets

June 20, 2023

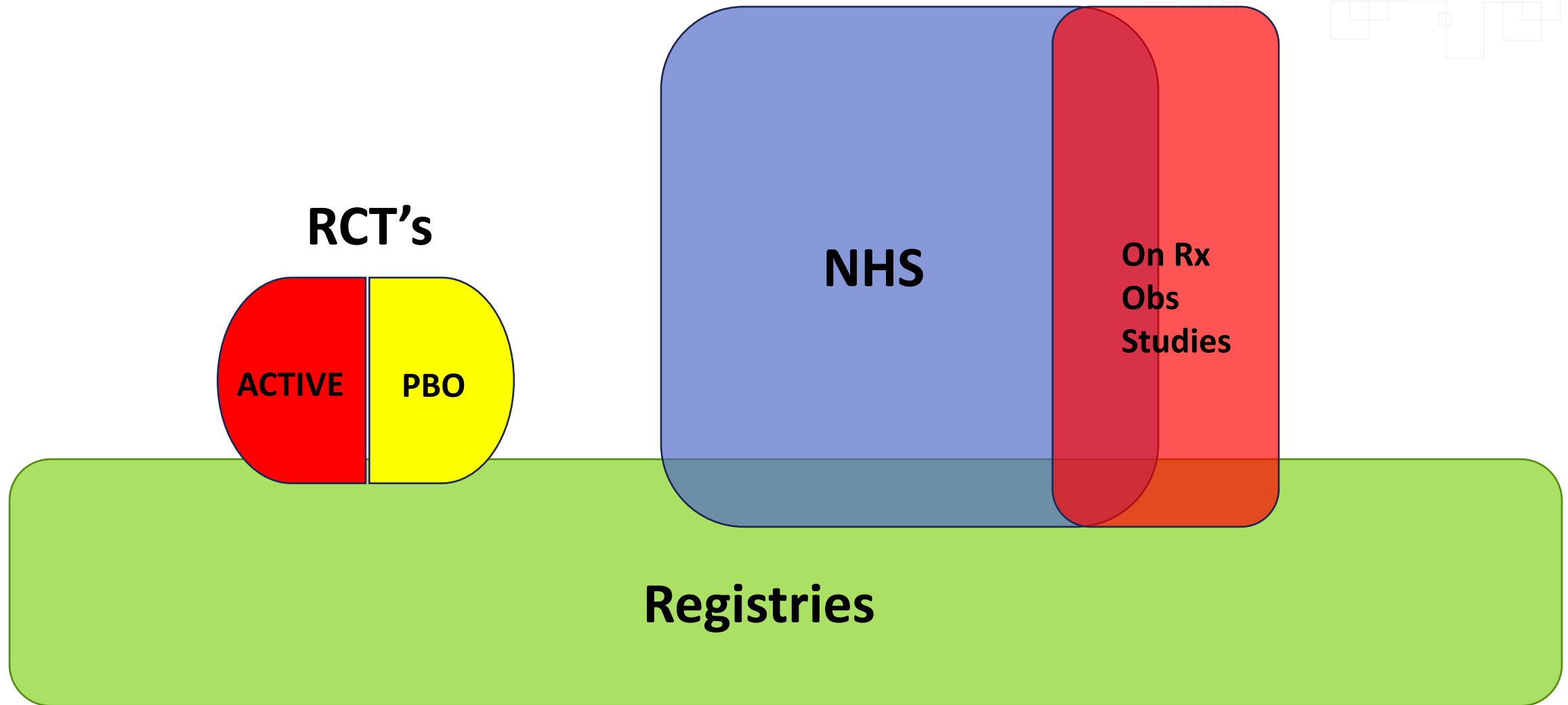
Vienna, Austria

Berkeley Public
Health

Need natural history/observational studies



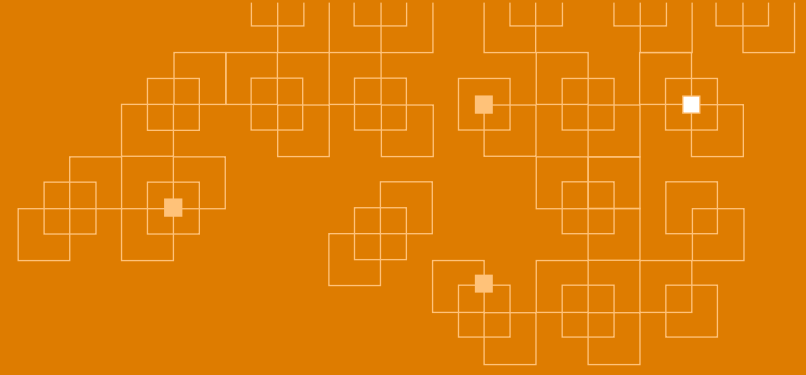
- Better understand natural history of PSC patients
 - Improve clinical care for all (heterogenous) PSC patients
- Potential support for drug development
 - RWD/RWE in untreated patients
 - Long-term follow-up
- RWD/E for treated patients
 - Long-term effectiveness
 - Safety



Data Sources for PSC Drug Dev



	PROS	CONS
Randomized Clinical Trials	<ul style="list-style-type: none"> *Curated patient selection *Standardized baseline *Randomization (↓bias) *More intense follow-up *Novel biomarkers 	<ul style="list-style-type: none"> *Short(er) follow-up *Restricted patient selection *Need for extrapolation
Natural History Cohorts (full clinic-based data) On-Treatment Observ. Studies	<ul style="list-style-type: none"> *Patient heterogeneity *Longer follow-up 	<ul style="list-style-type: none"> *No entry restriction *Patient heterogeneity *No standardized baseline *No novel biomarker info
Patient Registries (minimal data) (patient entered) (claims data)	<ul style="list-style-type: none"> *Broader access to patients *General prevalence of symptoms *Patient preferences *Inclusion of patient voice in drug dev 	<ul style="list-style-type: none"> *More limited information



Placebo Arms = Select NHS

Placebo and NHS are complementary

Potential PSC Clinical Trial Tracker Data



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Count of Clinical Trials	Sum of Participant Enrollment	Sum of Placebo Arm Participants
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35

3899

1726

PSC Clinical Trials by Country



Country Location	Count of Rank
Asia	3
Iran	1
China	2
Canada	2
Europe	21
France	2
Germany	2
Italy	2
Norway	1
Spain	2
Sweden	2
UK	10
USA	9
Grand Total	35

PSC Clinical Trials by Phases



Clinical Trial Phases	Count	
Behavioral/Dietary/Procedure	5	
Phase 1 Phase 2	1	
Phase 2	17	
Phase 2 Phase 3		2
Phase 3		7
Phase 4	3	
Grand Total	35	

≈70%

PSC Clinical Trial Tracker Data by Intervention

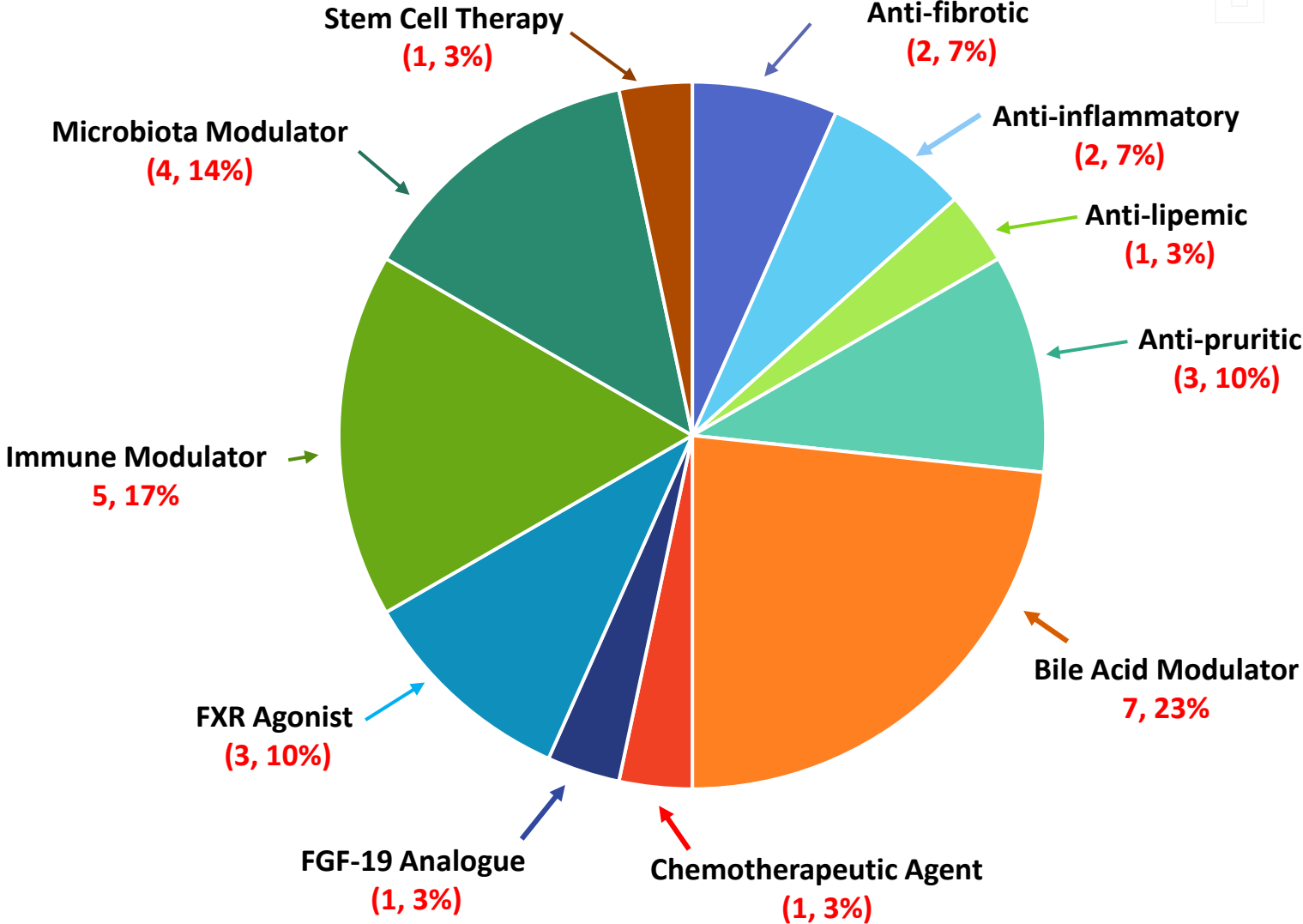


Intervention	Count of CT
Drugs	30
Biologics	5
Others	5
Dietary	1
QoL	1
Procedure	3
Grand Total	35

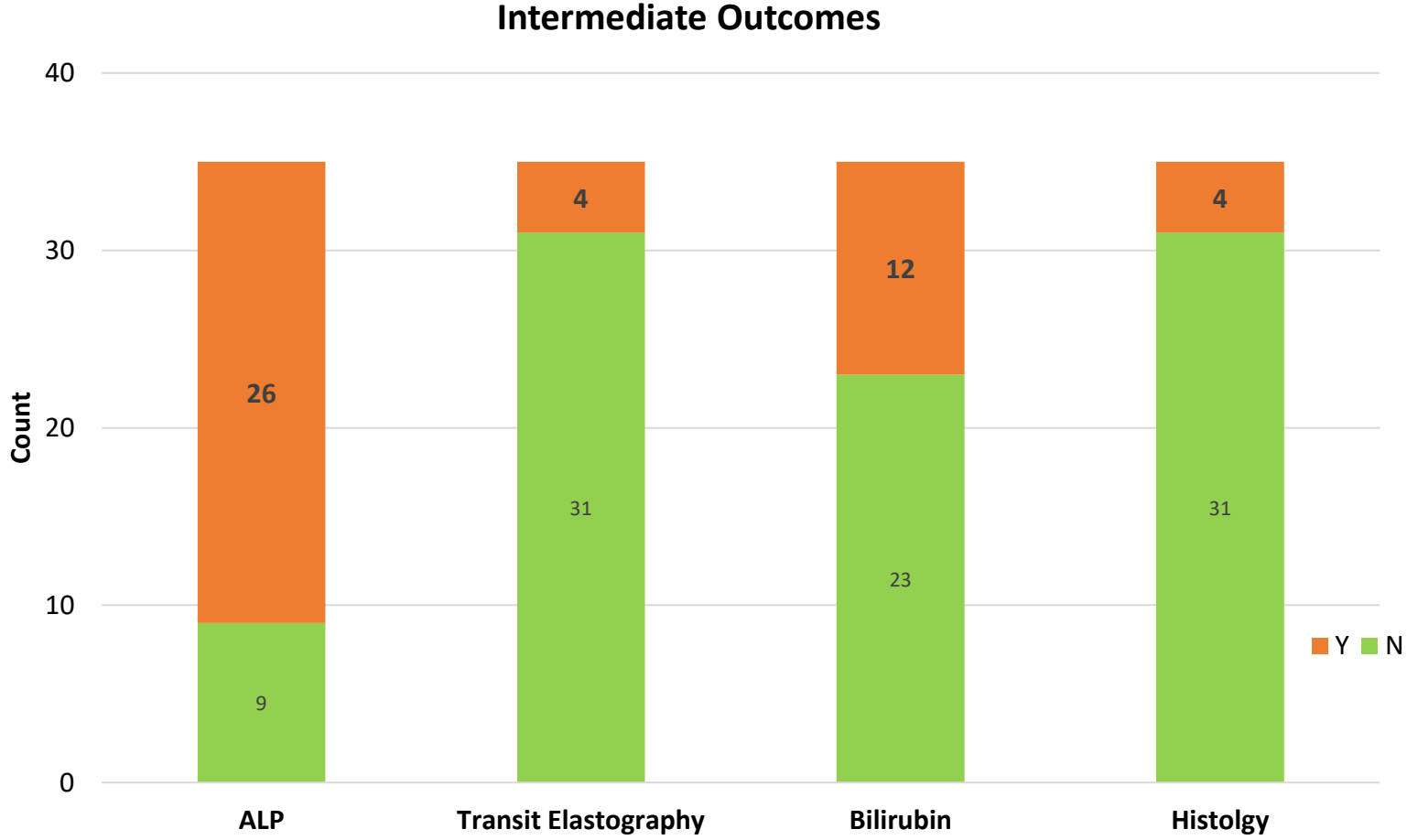
Clinical Trials by Treatment Targets



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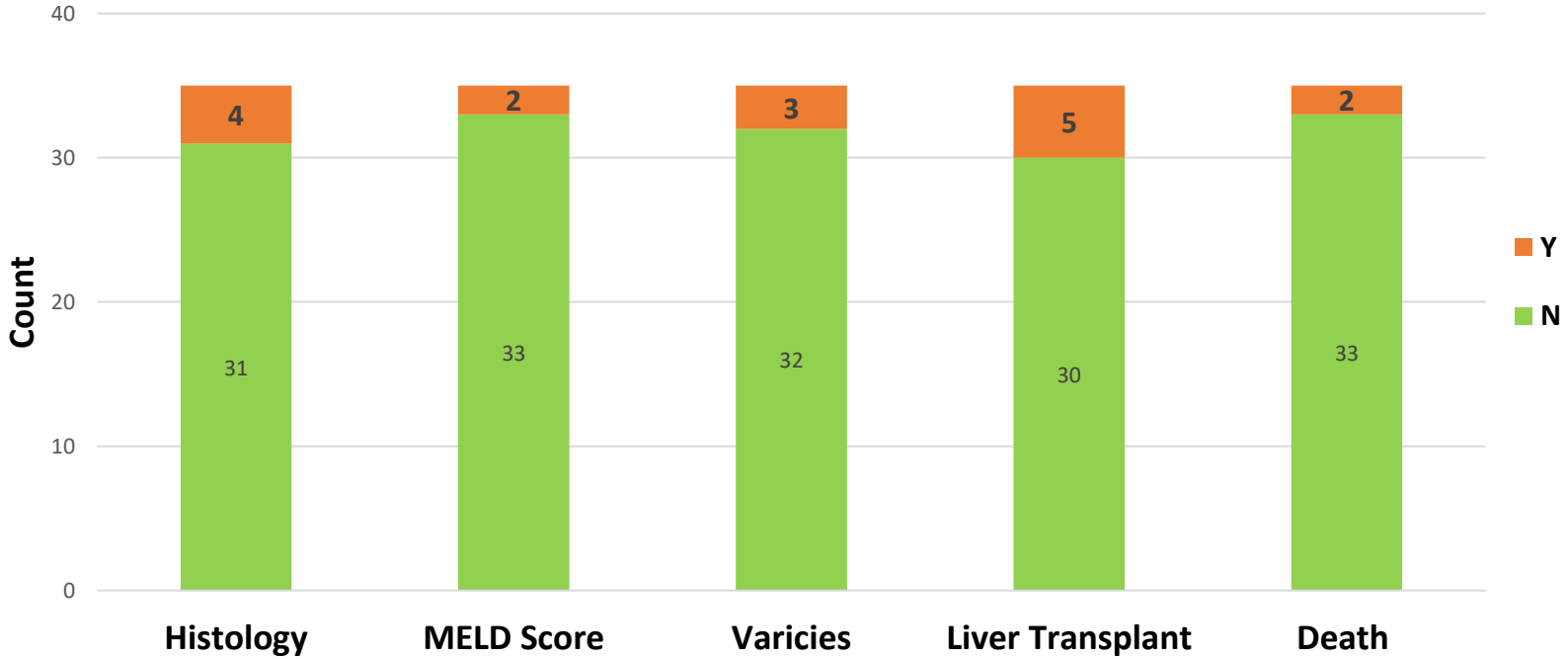
PSC Clinical Trial Tracker Data by **Intermediate Outcomes**



PSC Clinical Trials with Long Term Outcomes (Composite Endpoints)



Long Term Outcomes (Composite Endpoints)



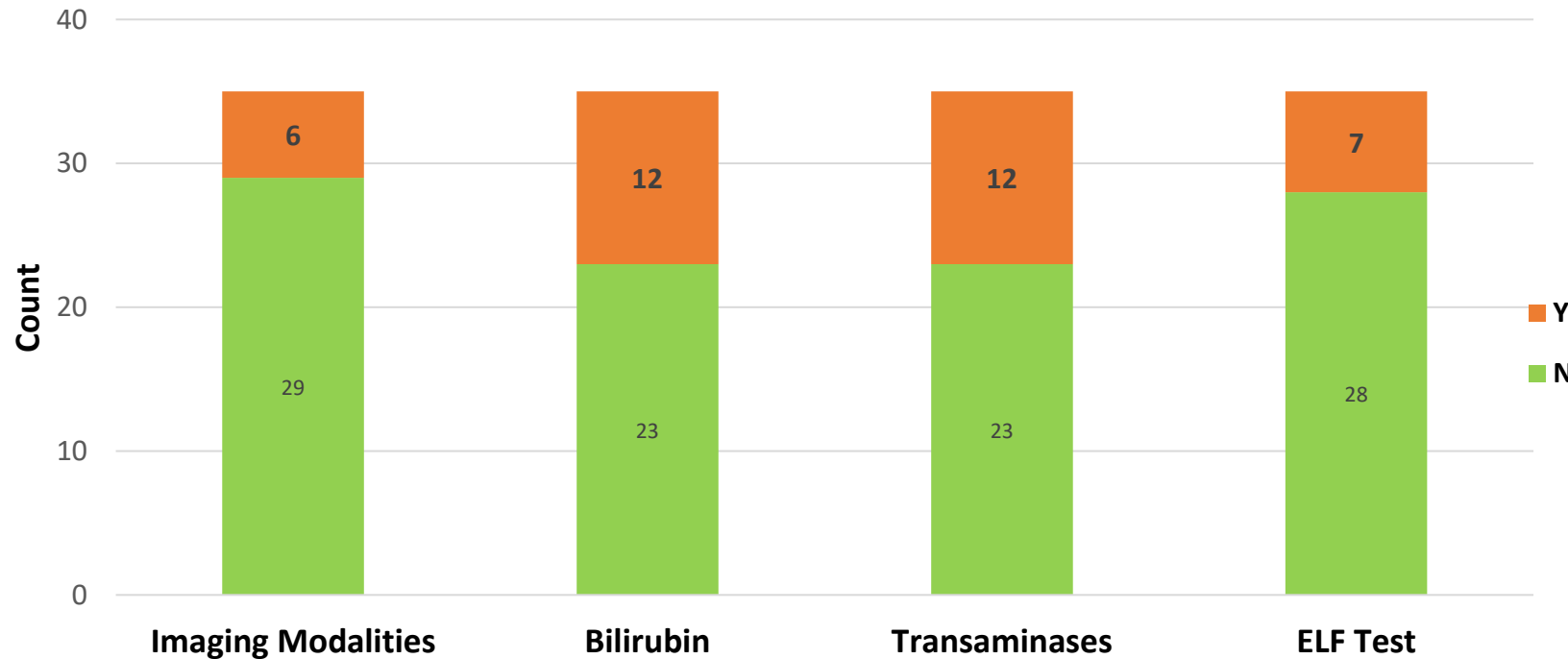
PSC Clinical Trial Tracker Data

by **Supportive Evidence for Long Term Efficacy**



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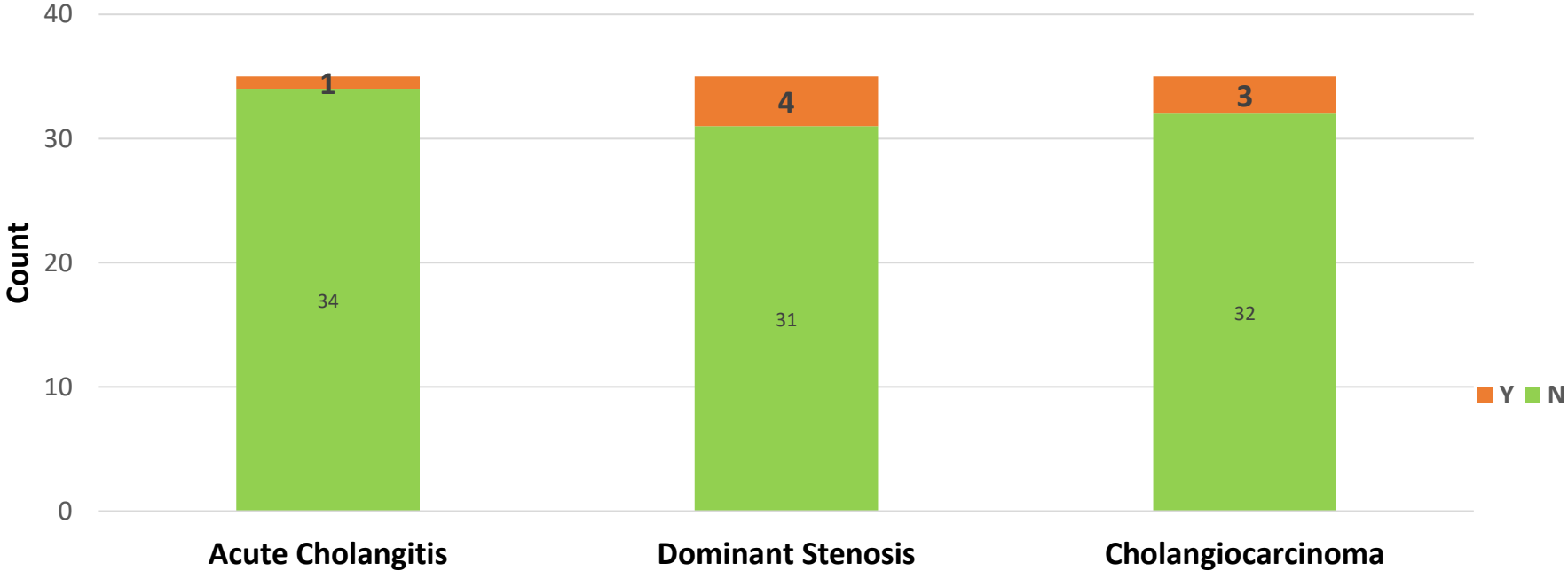
Supportive Evidence for Long Term Efficacy



PSC Clinical Trial with Important Clinical Events During The Course of PSC



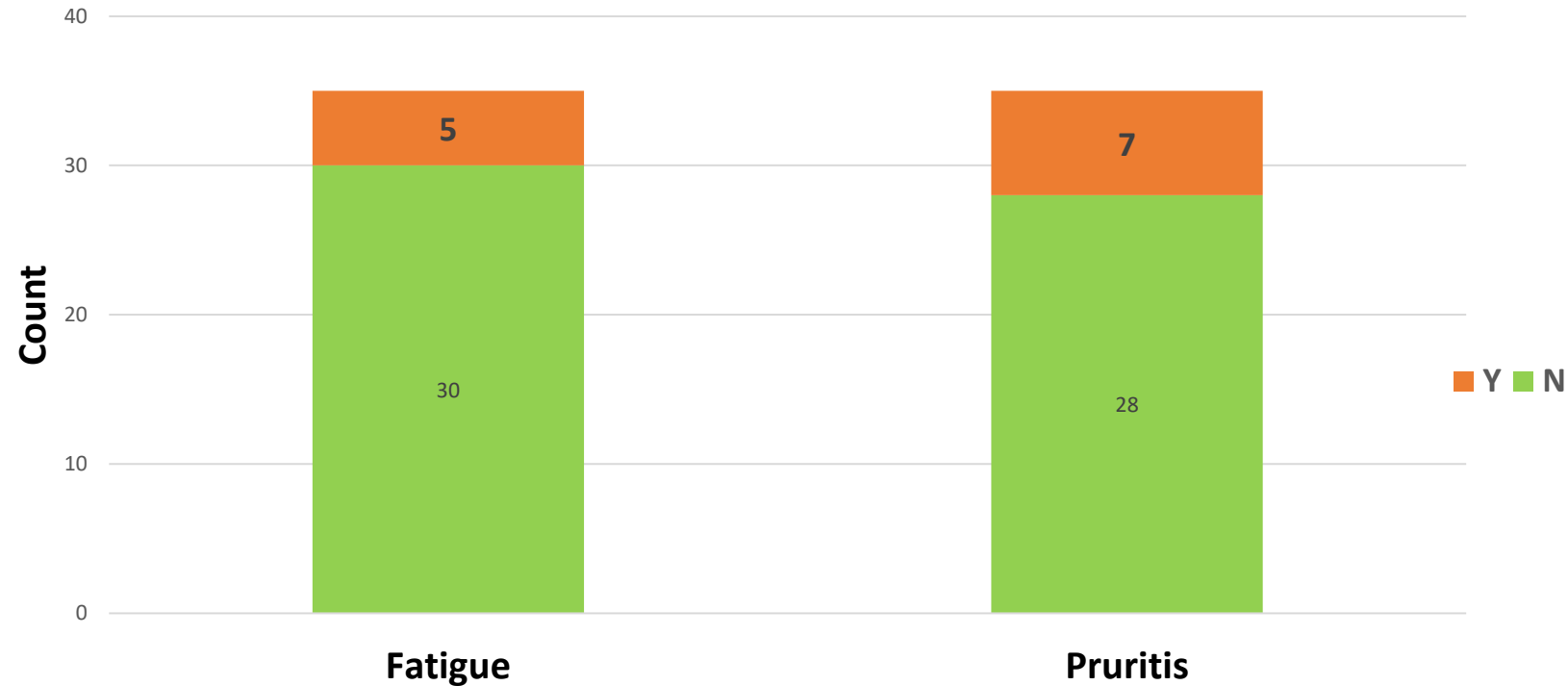
Important Clinical Events



PSC Clinical Trial for Symptomatic Treatment



Symptomatic Treatment



PSC Databases

Database Name	Number of patients	Characteristics	Country
PSC Partners Patient Registry	~2507	Retrospective Prospective;	USA
Pediatric PSC	~781	Retrospective; Multi-center	USA/Canada
Canadian PSC Registry		Prospective, new	Canada
IPSCSG	~3000	Retrospective; International;	International
Dutch PSC		Prospective	Netherland
PROGRESS (Samples from UK PSC Consortium and IPSCSG)	~3402	Prospective; Multicenter; Extensive Biobanking	UK
CALID/ North American PSC Registry	~2241	Prospective; Multicenter; Broad-based Clinical Data	US, Canada
UK-PSC	~2000	Prospective	UK
European Reference Network	~300	Prospective	Europe and worldwide
STOPSC	~1240(2001-2006) +annually expected 265	Retrospective/ Prospective	US, Canada
NoPSC	~200~300	Retrospective/ Prospective Transplanted patients	Scandinavia

Questions to be addressed



PSC Challenges and Topics for Discussion

