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FORUM
for Collaborative
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Placebo Arm Data Working Group

Update

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Meeting Recap

- General interest in exploring the possibility of pooling placebo data from completed Ph 2/3 RCTs
- Pros
 - Further characterize natural history of NASH by collecting available RCT data
 - Harmonize data collection to aid in identification of predictors of disease progression
 - Foster drug development, inform future study design, reduce need for PBO patients
 - Provide clinical safety context for interpreting rare events
- Cons
 - Observation period in Ph 2/3 RCTs relatively short, less reflective of natural history
 - Main outcomes likely to be progression to cirrhosis, with few hard outcomes
 - Targeted patient population with a “therapeutic intent” bias, not real-life setting
 - May be duplicative of similar ongoing efforts in other cohorts



Takeaways and Next Steps

- Unique opportunity at hand to learn from available PBO data in completed RCTs
- Need to better define study aims, advantages and limitations, analyses plans and resources required
- Assess data gaps and complementary of PBO data with ongoing NASH registries and “real life” setting