Mycology Clinical Trial Response Criteria
LISTENING SESSION (virtual)
Monday, January 8, 2024, 09:00-10:30 EST

Listening Session # 1 Comments	Category
Define reliable and validated outcomes	Biostatistical, Design
Reconsider binary response (success or failure) consider interval or continuous response	Biostatistical, Design
Lack of criteria for salvage therapy designs	Biostatistical, Design
Why is stable disease always a failure	Biostatistical, Design
Considerations for combination therapy	Biostatistical, Design
Discordant results are difficulty- use adjudication of casese	Biostatistical, Design
Objective clinical assessments	Clinical
Prioritize clinical response	Clinical
Diagnostics and radiology lag behind clinical response	Clinical
Reconsider timepoints for evaluation (e.g., Day 42, 84) , too early for rare molds	Clinical
Clinical co-infections , example: 70% of candida infections also have gram neg microbes	Clinical
Consider host	Clinical
Consider relapse-free survival in oncology or specific immunocompromised hosts	Clinical
Radiology and culture- can't trump clinical	Clinical
Clinical must take precidence	Clinical
All cause mortality vs attributable mortality- consider underlying disease	Clinical
Relapsed AML patients live much longer today - difficult to use attributable mortality, many die of Aspergillus, but not due to aspergillus	Clinical
GM in aspergillus- FDA says not a viable outcome - clinical stage more important	Clinical
Consider other underlying disease factors- such as Diabetes - DKA and surgery complications in mucroales	Clinical
Mortality can be organism dependent- mortality in Crypto occurs in first 10-12 weeks generally	Clinical
Non geologic timeline- Day 42, 84 - still alive -	Clinical
Length of treatment- do they need 14 days of therapy?	Clinical
Perfect is the enemy of good	Consensus Process

Timeline too aggressive (Sept 2024)	Consensus Process
Include EMA	Consensus Process
Historical papers may no longer be relevant- so consider which evidence to include	Consensus Process
Bring in other specialties like ICU doctors, not just ID	Consensus Process
Include a statistician - such as Chiung-Yu Huang (UCSF) in design of CT and in this process	Consensus Process
Include EMA	Consensus Process
Disease Specific	Disease category
Separate Moulds from Aspergillus	Disease category
Cocci- serologic response = no cure- need disease specific outcomes	Disease category
Don't discard the criteria that work- in Pulmonary IA, EORTC/MSG definitions may still be fine	Disease category
Cryptococcal antigen can fluctuate- though the patient improves	Disease category
Use of non-culture diagnostic methodology	Non-Culture dx
Develop novel testing methods	Non-Culture dx
Identify limitations of diagnostic testing - GM not as sensitive, BDG not very specific	Non-Culture dx
Consider host biology- release of antigen may not be related to fungal burden but response to fungal therapy- which confounds what we are measuring	Non-Culture dx
GM, BDG, PCR, LFT, PET-CT- what else?	Non-Culture dx
Diagnostics as an endpoint- GM can't trump clinical	Non-Culture dx
Cost effectiveness of diagnostics like T2- limitation for global trials = availability	Non-Culture dx
Bundle of diagnostics and catheters (candidemia) can fungal outcomes be a bundled?	Non-Culture dx
PET-CT- not readily available, affordable, how do you get a patient at Day 42 back for CT	Non-Culture dx
Central labs- difficult, costly, and delay in data, so doesn't trump clinical	Non-Culture dx
DOOR (AE, treatment failure, infection complications)	PRO/DOOR
Importance of Validated PRO tools	PRO/DOOR
Patient important outcomes	PRO/DOOR
How does the patient feel?	PRO/DOOR
Patient reported outcomes (PRO)- process is lengthy and harder to do (validate)- example Cocci	PRO/DOOR
Cultural differences can confound PRO and QOL instrument results	PRO/DOOR