IMACS FORM 00: CLINICAL TRIAL DESIGN FEATURES

To be completed for all trials in the registry

GENERAL INFORMATION

Name/number of trial:_____

Principle investigator: (name, affiliation, contacts):_____

Agent(s) under investigation: _____

Phase of trial (check all that apply):

_____Phase 1
_____Phase 2
_____Phase 3
_____Phase 4
Other:_____

Number of subjects enrolled in the trial _____
Number of subjects who met primary improvement criteria _____
Number of subjects withdrawn from the trial during treatment phase _____
Number of sites which enrolled subjects _____

INCLUSION/EXCLUSION CRITERIA FOR TRIAL ENTRY:

Myositis Primary Clinical Groups included in trial:
⓪ Adult OR ⓪ Juvenile
⓪ Polymyositis
⓪ Dermatomyositis
⓪ Inclusion body myositis
⓪ Other: please clarify_____

Classification Criteria used for Trial entry:
___Bohan and Peter criteria for IIM
___Griggs criteria for IBM
___Other classification criteria used: Specify
_____
_____

Was a muscle biopsy at baseline required for trial entry?
____Yes
____No

Inclusion Criteria for Trial Entry (check all that apply):

___Muscle strength less than a certain strength: _____
___Disease activity > certain amount: _____
___Specified level of functional disability:_____
___Refractory disease with inadequate response to first- line agents such as corticosteroids and methotrexate
___New onset disease: _____
___Inadequate response to other therapeutic agents _____
___Unacceptable corticosteroid toxicity_____
___Cutaneous or other extra-muscular manifestations: _____

Definition of Inadequate Response to First Line Agents:

___Adequate corticosteroid treatment trial to define treatment failure was agreed to be 60 mg/day for at least 2 months in adult patients, and 2.0 mg/kg/day prednisone for at least 2.5 months in pediatric patients
___ Methotrexate treatment failure in pediatric patients was agreed to be 25 mg/m^2/week parenterally for at least 3 months duration.
___Other definitions used: _____

Exclusion criteria for trial entry:

____Myositis associated with malignancy
____Myositis associated with another connective tissue disease
____Myositis associated with an environmental risk factor (penicillamine, collagen implants, etc.)
____Significant organ system involvement: _____
____ Significant myositis damage _____
____ Hepatic disease
____ Other_____

Allowable Concomitant Therapy:

____Prednisone: Dose_____
____Methotrexate: dose_____
____Other medications- list and dose_____
____Physical therapy- continued, stable regimen
____Other:_____

Was a standard dose reduction regimen used for corticosteroid tapering? ____Yes _____No
If so, please include:

_____

Trial Design:

___Double-blinded
___Placebo controlled: Duration placebo phase: _____
___Cross over
___Direct comparison to active agent
___Open label
___Other: _____

Trial Duration:
____Months for active treatment phase
____Months for open-label follow-up after active treatment phase

Assessment Intervals for Efficacy and Safety:

Every ___ months during active treatment phase
Every ___ months during open label follow-up phase after completion of active treatment

Safety Assessment:

____NCI Common Toxicity Criteria
____Other_____

Trial outcome measures (specify primary or secondary):
___IMACS Preliminary Definitions of Improvement _____
___IMACS Core set activity measures_____
___PRINTO Preliminary definitions of Improvement_____
___PRINTO Core set activity measures_____
___Corticosteroid dose reduction
___Time to complete clinical response
___Other_____

Trial dropout criteria:
___Physician global worsening of ≥ 2cm on a 10cm VAS and a worsening of the manual muscle testing by ≥ 20%, or

____Extramuscular organ disease activity worsening by ≥ 2cm on a 10cm VAS,

____Any 3 of 6 IMACS core set activity measures worse by ≥ 30%

____Other_____

Trial Flare Criteria: did your trial use a definition of flare? ____Yes ____No

If yes, as a trial endpoint? ____ As withdrawal criteria? _____

If yes, specify definition of flare used_____

If yes, % of subjects who met flare criteria in the study_____

COMPLETE CLINICAL RESPONSE/REMISSION:

Complete clinical response:

Was complete clinical response assessed in the trial? ___Yes ____ No (if no skip to Remission)

If yes, did your trial use IMACS complete clinical response criteria (6-month continuous period of no evidence of disease activity while still on myositis therapy) as a trial endpoint?____Yes ____No

Did you use a different definition than the one specified above? If yes, please specify:
_____

What % of subjects vs. controls achieved a complete clinical response in your trial?

What was the mean duration and range of complete clinical response (in months) in your trial?

_____

Remission:

Was remission assessed in the trial? ___Yes ____ No (if no skip to Analyses)

Did your trial use IMACS remission criteria (6-month continuous period of no evidence of disease activity while off myositis therapy) as a trial endpoint? ____Yes ____No

Did you use a different definition of remission than the one specified above? If yes, please specify:_____

What % of patients vs. controls achieved remission in your trial?

What was the mean duration and range of remission (in months) in your trial?

_____

TRIAL ANALYSES:

Primary outcome analyses performed:

____Intention to treat

____Last observation carried forward

____Other analyses performed

Post-hoc stratification:

Did you perform any post-hoc stratification? ____Yes ___No

If yes, please specify the post-hoc stratification variables assessed:

____Clinical group,

____Duration of disease,

____Degree of muscle weakness/dysfunction at enrollment,

____Extramuscular organ involvement: _____

____Autoantibodies: _____

____ Muscle histopathology: _____

____Cutaneous or gastrointestinal ulceration

____Calcinosis

____Other:_____