IMACS FORM 00: CLINICAL TRIAL DESIGN FEATURES

To be completed for all trials in the registry

GENERAL INFORMATION

Name/number of trial:_____

Principal investigator (name, affiliation, email, phone):
_____
_____

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Number of subjects withdrawn from the trial during treatment phase _____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 _____

Date enrollment started for this study/trial: (mm/dd/yyyy) ___/____/_____
Date enrollment concluded for this study/trial: (mm/dd/yyyy) ___/____/_____

INCLUSION/EXCLUSION CRITERIA FOR TRIAL ENTRY:

Myositis Primary Clinical Groups included in trial: (check all that apply):

- ◯ Adult OR ◉ Juvenile
- ☐ Polymyositis
- ☐ Dermatomyositis
- ☐ Inclusion body myositis
- ☐ Other: please clarify_____

Classification Criteria used for Trial entry: (check all that apply):

- ☐ 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: (check all that apply):

☐ 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: (check all that apply):

☐ 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: (complete all that apply):

☐ 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 (check all that apply and specify primary or secondary endpoint):
☐ 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: (check all that apply):
☐ 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?

Patients: _____ % Controls: _____%

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

Mean duration complete clinical response:_____ months

Minimum duration complete clinical response:_____ months

Maximum duration complete clinical response_____months

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?

Patients: _____ % Controls: _____%

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

Mean duration remission:_____ months

Minimum duration remission:_____ months

Maximum duration remission: _____months

TRIAL ANALYSES:

Primary outcome analyses performed: (check all that apply)

☐ 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: (check all that apply)

☐ Clinical group,

☐ Duration of disease,

☐ Degree of muscle weakness/dysfunction at enrollment,

☐ Extramuscular organ involvement: _____

☐ Autoantibodies: _____

☐ Muscle histopathology: _____

☐ Cutaneous or gastrointestinal ulceration

☐ Calcinosis

☐ Other:_____