IMACS Outcomes Repository

IMACS/JDM Working Group Meeting
Washington DC; November 12, 2012
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Outcomes Repository

- Purposes:
  - To prospectively validate and improve outcome measures for adult and juvenile myositis trials (including definitions of improvement)
  - Further develop clinical trial design consensus
  - Develop predictors of treatment responses
  - Serve as repository of data for other studies

- Collection of prospective clinical trials and natural history studies that are utilizing common core set measures, demographic/clinical features and outcomes
Outcomes Repository – Composition

- **Required data (minimum 2 time points):**
  - Core demographic, clinical features and response to therapy
  - IMACS Core Set Activity Measures
  - Assessment of Study Outcome
  - Medication received in study

- **Optional data:**
  - PRINTO Activity Measures: CMAS, DAS, CHQ
  - MD and Patient/Parent Global Damage, Myositis Damage Index
  - Patient-Reported Outcomes: SF-36
  - Ancillary data specific to a study
Outcomes Repository – Studies Deposited

- 2 randomized controlled trials
  - Gourley: Infliximab in PM/DM (12 patients)
  - Amato/Muscle Study Group: Enbrel in DM (16 patients)

- 7 open label trials
  - Fiorentino: Rituximab in DM (8 patients)
  - Lundberg: Infliximab, Anakinra, Exercise (13, 10, 8 patients)
  - Isenberg/Sultan: Rituximab (5 patients)
  - Pilkington: Infliximab, Cyclophosphamide (12 patients)

- 3 natural history studies
  - Garcia de la Torre: adult DM/PM (21 patients)
  - Song: DM/PM/IBM (7 patients)
  - Rider/Miller: Adult and Juvenile DM/PM (80 patients)
Outcomes Repository – Data Deposited

- What is deposited:
  - IMACS Core Set Measures, Clinical trial design form (all)
  - Demographic data, background therapy (most)
  - Assessment of outcomes (most)
  - Clinical data, Myositis Damage Index (few)
  - Ancillary data: 2 studies
  - PRINTO core set – 1 study

- All study visits: 6 trials; Key visits: 3 trials;
  Natural history studies: 2 - 4 visits

- Detailed data: 6 studies; Summary data: 6 studies (most committed to providing detailed data if needed)
<table>
<thead>
<tr>
<th><strong>Principal Investigator</strong></th>
<th><strong>Trial/Population</strong></th>
<th><strong>Detailed vs. Summary Data</strong></th>
<th><strong>Comments</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>Gourley, Plotz</td>
<td>Infliximab (RCT); 12 PM, DM</td>
<td>Detailed data, All study visits</td>
<td>Core set activity measures, Myositis Damage Index, clinical and demographic data, therapy, ancillary data</td>
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<tr>
<td>Amato (Muscle Study Group)</td>
<td>Etanercept (RCT); 16 DM</td>
<td>Detailed data, All study visits</td>
<td>Core set activity measures, SF-36, background therapy</td>
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<tr>
<td>Fiorentino, Chung</td>
<td>Rituximab; 8 DM</td>
<td>Detailed data, Key visits</td>
<td>Core set activity measures, MD assessment outcome; clinical and demographic data, therapy</td>
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<td>Lundberg</td>
<td>Infliximab; 13 DM, PM, IBM</td>
<td>Summary data, All study visits</td>
<td>Core set activity measures; MD assessment outcome; therapy and autoabs</td>
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<tr>
<td>Lundberg</td>
<td>Anakinra; 10 DM, PM</td>
<td>Summary data, All study visits</td>
<td>Core set activity measures; MD assessment outcome; demographics, therapy, autoabs</td>
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<tr>
<td>Lundberg</td>
<td>Exercise training; 8 DM, PM</td>
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# Clinical Trials in IMACS Outcomes Repository

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<td>Isenberg, Sultan</td>
<td>Rituximab; 5 DM, PM</td>
<td>Summary data, All study visits</td>
<td>Core set activity measures; myometry</td>
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<tr>
<td>Pilkington</td>
<td>Cyclophosphamide; 8 JDM</td>
<td>Summary data, Key visits</td>
<td>Core set activity measures, CMAS, CHQ; MD assessment outcome</td>
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<td>Pilkington</td>
<td>Infliximab; 5 JDM</td>
<td>Summary data, Key visits</td>
<td>Core set activity measures, CMAS, CHQ; MD assessment outcome</td>
</tr>
<tr>
<td>Garcia de la Torre</td>
<td>Natural History; 21 DM/PM</td>
<td>Detailed data, 2 visits</td>
<td>Core set activity measures, Myositis Damage Index, Global Damage, MD Assessment Outcomes, Demographic and clinical data, therapy</td>
</tr>
<tr>
<td>Song</td>
<td>Natural History; 7 PM/DM/IBM</td>
<td>Detailed data; 2 visits</td>
<td>Core Set Activity Measures, MD Assessment Outcomes, Demographic and clinical data, therapy</td>
</tr>
<tr>
<td>Rider, Miller</td>
<td>Natural History; 53 JDM, 27 DM/PM</td>
<td>Detailed data; 2-4 visits</td>
<td>Core Set Activity Measures, CMAS, DAS, CHQ-PF50, Myositis Damage Index, Global Damage, MD Assessment Outcomes, Demographic and clinical data, therapy</td>
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Research Study Proposals for Use of IMACS Repository

- Open to IMACS members for use
- Submit research proposal to IMACS Repository Research Review Committee
  - Composed of contributors to Repository
- Proposal approved by local IRB (copy to NIH), Data use agreement completed
- Investigator receives de-identified data for their study
- Include IMACS repository contributors as co-authors
- Procedures on IMACS website
  
  http://www.niehs.nih.gov/research/resources/collab/imacs/researchguidelines/index.cfm
Acknowledgements –
Sponsors

National Institute of Environmental Health Sciences

American College of Rheumatology