Comprehensive Care for Duchenne Muscular Dystrophy

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Comprehensive Care for DMD

"Are we there yet?"
Historical Perspective

- 1999: single specialty, NM/MDA clinic
- 2000: multi-disciplinary with Rehab in clinic, dedicated NM pulmonologist
- 2001: dedicated NM cardiologists
- 2002: + genetic counselor in clinic
- 2003: CCHMC Clinical Expansion Opportunity support for interdisciplinary program (Nm nurse practitioner, 0.2 FTE admin asst)
- 2007: NM Matrix – merging clinical program with basic science program
## CCHMC Neuromuscular Network

- Anesthesia
- Cardiology
- Concierge services
- Critical Care Medicine
- Dev Med and Behav Peds
- Endocrinology
- Genetics
- Guest services
- Infectious Diseases
- Nephrology
- Neurology
- Neuropsychology
- Nutrition
- Orthopedics
- Palliative Care Services
- Pulmonary Medicine
- Pediatrics
- Pediatric Surgery
- PT/Orthotics/ST
- Psychology
- Rehab Medicine
- Research coordinator
- Social services
Rationale for Interdisciplinary care: DMD – a multi-systemic disorder

**Muscle strength and function**
- Improve strength - corticosteroids
- Maintain motor function – physical therapy, braces, mobility aids, weight control
- Prevent and manage scoliosis and skeletal deformities

Pediatric neurologists, physiatrists, physical therapists, orthotists, pediatric orthopedic surgeons, dieticians
Rationale for Interdisciplinary care: DMD – a multi-systemic disorder

**Pulmonary function**
- Prevent and treat chest infections
- Monitor and manage respiratory failure
  Pediatric pulmonologists, respiratory therapists

**Cardiac function**
- Monitor and manage cardiomyopathy
  Pediatric cardiologists
Rationale for Interdisciplinary care: DMD – a multi-systemic disorder

**Endocrine/metabolic function**

- Growth failure
- Insulin resistance, impaired glucose tolerance
- Obesity
- Osteoporosis and fractures
- Hypothyroidism
- Hypercalciuria, renal stones

Pediatric endocrinologists, pediatric nephrologists, dieticians
Rationale for Interdisciplinary care: DMD – a multi-systemic disorder

Neuropsychological function/mental health

- Learning difficulties
- Behavioural problems
- Social isolation and difficulties

Neuropsychologists, psychologists, social workers
Rationale for Interdisciplinary care: DMD – a multi-systemic disorder

**Genetics**

- Genetic counseling for family members

Genetic counselors
Rationale for Interdisciplinary care: DMD – a multi-systemic disorder

Inpatient care

- Anesthesia for surgical procedures
- Critical care for severe cardiopulmonary complications
- Inpatient rehabilitation
  Pediatric anesthesiologists, critical care specialists, physiatrists, physical therapists, respiratory therapists
Rationale for Interdisciplinary care: DMD – a multi-systemic disorder

Single specialty clinics with parallel care practice

- Poor communication
- Fragmentation of care

Multi-disciplinary care vs Interdisciplinary care approach
Rationale for Interdisciplinary care: DMD – a multi-systemic disorder

Collaborative evaluation and joint development of a personalized/individualized care plan (ICP) by a team of specialists in an interdependent manner

- Face-to-face meetings/phone conferences/email communication by members of team to formulate decisions re patient care

- DMD specialist to integrate and formulate medical care decisions; nursing support to co-ordinate team efforts for effective communication and clinical decision making and care planning
Duchenne Muscular Dystrophy Key Driver Analysis

OUTCOMES (Measure)

1. Patient Survival (Mean Age of Death)
2. Patient Ambulation (Mean Age at Loss of Ambulation)
3. Quality of Life (PedsQL)

KEY DRIVERS

Motor Function
Medication Complications
Cardiovascular Health
Pulmonary Health
Orthopedic Outcomes
Behavioral and Mental Health
Patient/Parent Satisfaction

STANDARDIZED INTERVENTIONS

History and Physical Exam
Testing and Evaluation:
- MRI
- carrier status
- gene mutation
- blood analysis
- muscle biopsy
- EKG
- echo
- PFT
- sleep study

Medications:
- steroids
- growth hormone
- Synthroid
- supplements
- antidepressants

Orthopedic:
- physical therapy and rehabilitation

Surgery:
- scoliosis
- contractures

Self-Management:
- nutrition/diet
- daily stretches
- night braces

Follow-Up Care:
- visits with a nutritionist
- coordination of care (nurse)
- integration of care (physician)
- genetic counseling
- reassessment of patient

Orthopedic:
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Outcomes: Steroids (n=54) vs. No Steroids (n=26)
### Outcomes – steroid Rx ambulating boys

<table>
<thead>
<tr>
<th>Age (years) n=37</th>
<th>Percent boys Ambulating</th>
<th>Avg Duration of Steroids (years)</th>
<th>Avg steroid Start age (years)</th>
</tr>
</thead>
<tbody>
<tr>
<td>12</td>
<td>83%</td>
<td>4.5</td>
<td>7.5</td>
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<tr>
<td>13</td>
<td>55%</td>
<td>5.8</td>
<td>7.3</td>
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<tr>
<td>14</td>
<td>27%</td>
<td>5.1</td>
<td>8.8</td>
</tr>
<tr>
<td>15</td>
<td>50%</td>
<td>4.8</td>
<td>10.3</td>
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<tr>
<td>16 &amp; 17</td>
<td>33%</td>
<td>7.0</td>
<td>9.5</td>
</tr>
<tr>
<td>18 - 24</td>
<td>0%</td>
<td></td>
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</tbody>
</table>
Maintaining Ambulatory Status in DMD with percutaneous myofascial lengthenings, ischial weight-bearing KAFOs and Intensive Therapy (US Pediatrics, Touch Briefings 2008)
Growth hormone may improve growth, neuromuscular and pulmonary function in Duchenne muscular dystrophy
Rutter, Rose, Wong, PAS 2008; WMS 2008
Growth hormone may improve growth, neuromuscular and pulmonary function in Duchenne muscular dystrophy
Rutter, Rose, Wong, PAS 2008; WMS 2008
Challenges and Limitations

- Administrative and financial support – crossing divisions and business budgets
- Integrated clinic – space and time constraints for optimal clinic flow and communication amongst providers
- Insurance coverage – need for cost-benefit analyses; to establish the clinical effectiveness of comprehensive, integrated care (clinical, QOL and financial outcomes)
- Patient and family adherence to care plans
Comprehensive Care for DMD

"Are we there yet?"
Conclusion

CCHMC Mission:
“Cincinnati Children’s will improve child health and transform delivery of care through fully integrated, globally recognized research, education and innovation.
For patients from our community, the nation and the world, the care we provide will achieve the best:
- Medical and quality of life **outcomes**
- Patient and family **experiences** and
- **Value**
today and in the future.