



Patient and Family Centric Care for Duchenne and Becker Muscular Dystrophy: Towards the IPU (Integrated Practice Unit) Model

Brenda Wong¹ MD; Tracy Seckler²; Laura Dalle Pazzi²; Robert Brown³ MD; Lawrence Rhein¹ MD; Terrence Flotte⁴

¹Department of Pediatrics, University of Massachusetts Medical School, Worcester, MA; ²Charley's Fund, New York, NY; ³Department of Neurology, University of Massachusetts Medical School, Worcester, MA; ⁴University of Massachusetts Medical School, Dean's Office, Worcester, MA



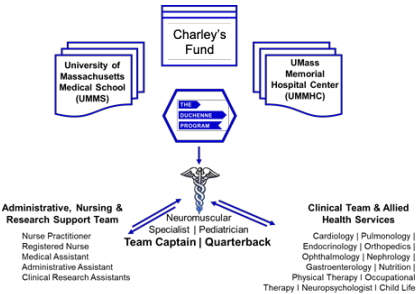
Background

- Duchenne and Becker muscular dystrophy (DBMD) patients have complex, multisystem care needs including motor, cardiac, pulmonary, and neurobehavioral health problems as well as endocrine co-morbidities from long-term glucocorticoid therapy. Carrier mothers and sisters are at risk for cardiomyopathy.
- Near- and long-term outcomes for DBMD patients can be improved by employing a care model known as an Integrated Practice Unit (IPU).
- IPUs are defined as care models “organized around the patient and providing the full cycle of care for a medical condition, including patient education, engagement and follow-up, and encompass inpatient, outpatient, and rehabilitative care as well as supporting services”. Rather than function-based and specialist-group silos, they promote work in multidisciplinary patient-oriented teams led by a single provider who coordinates care.^{1,2}
- The Duchenne Program at UMass Medical School applies an IPU care model for a DBMD patient base of 300+ families with a three-fold goal: 1) improve health of both individual patient and overall disease community; 2) improve the family experience in care; and 3) decrease financial cost, both to patients and the system overall.

Objective

- To describe the IPU model of care at The Duchenne Program at UMass Medical School in Worcester, MA, USA.

Program Overview



Core Structure + Administration: Neuromuscular specialist ‘team captain’ coordinates a core team that works with key specialists; hospital departments and founding patient advocacy group provide input and oversight.

Patient Centered Specialty Practice Medical Home



Care-Centralizing Strategy for Patients: The clinic and core team serve as a central medical ‘home’ that partners with and integrates other parties to ensure optimized, coordinate care across routine and emergency needs.

Summary

The Duchenne Program is a patient and family centric care program that aims to improve near- and long-term health, experience, and financial outcomes for DBMD patients – both individually and as a community. It delivers team-based, coordinated, collaborative support across the full cycle of care for 350 patients and families, including carrier mothers and sisters. Program electronic medical records system (EPIC My Chart), a website, and a patient portal enable not only optimal point-of-care delivery but also ongoing patient education, engagement, and follow-up. A health IT-based clinic registry tracks care over time for this progressive chronic disorder and across settings of multisystem environment to inform clinical outcomes and care decisions and provide guidance for providers and patients for the participation of clinical trials in DBMD. A key challenge to establishing the IPU care model more broadly is cost of staff efforts, due to current insurance reimbursement. Cost effectiveness and budget impact studies are needed to assess the potential for value or outcome-based financing for IPU models of care.

Multi-disciplinary Elements of Team-based Integrated Care in DBMD

The Duchenne Program integrates all specialty care disciplines into one seamless, coordinated, personalized experience for patients

Neuromotor Function

Muscle strength and function

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

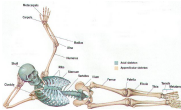


Pediatric Neurologist, Physical Therapist, Occupational Therapist, Pediatric Orthopedic Surgeon, Dietician

Fracture Management

Fractures

- Fat embolism syndrome
- PT and rehab needs



Dental needs

- Bisphosphonates and osteonecrosis
- Orthopedists, PT, Rehabilitation Doctors, ED and PICU staff, Dental Colleagues

Neuropsychological Function/Mental Health

- Intellectual disability 26%
- ASD 21%
- Hyperactivity 24%
- Inattention 44%
- Internalizing behaviors 24%
- Externalizing behaviors 15%



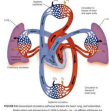
Neuropsychologists, Psychologists, Psychiatrists, Social Workers, School Teachers

Cardiopulmonary & Renal Function

Pulmonary function

- Prevent and treat chest infections
- Monitor and manage respiratory failure

Pediatric pulmonologists, respiratory therapists



Cardiac function

- Monitor and manage cardiomyopathy

Pediatric cardiologists

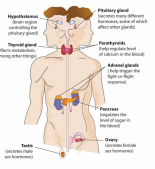


Renal function

- Cardio-renal syndrome

Endocrine/metabolic Function

- Growth failure
- Insulin resistance, impaired glucose tolerance
- Obesity
- Pubertal delays/testosterone insufficiency/deficiency
- Bone health – Osteoporosis, fractures
- Hypothyroidism
- Hypercalciuria, renal stones
- Adrenal insufficiency

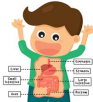


Pediatric Endocrinologists, Pediatric Nephrologists, Dieticians

Gastrointestinal & Urological Function

- Constipation
- Secondary lower urinary tract symptoms – urine frequency, secondary enuresis
- Fatty liver (NASH)
- Kidney stones

GI Specialists, Urologists, Nephrologist

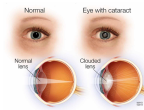


Eye care

Ophthalmology

- Ocular complications of long-term glucocorticoids
- Cataract surgeries
- Idiopathic intracranial hypertension (Pseudotumor cerebri)

Ophthalmologists, Pediatric Neurologists



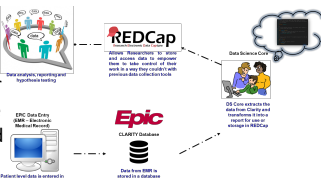
Family Centric Care - Carriers

- Cardiac surveillance for Myocardial Fibrosis and left ventricular systolic dysfunction
- Genetic counseling needs for XL recessive disorder

Resources to Track + Enable Outcomes-based Care across 3 Key Aims

Clinical Outcomes:

Registry with real-world EMR data to track care over time and across multiple settings
IRB-approved protocol: Characterization of Duchenne Muscular Dystrophy Outcomes –Electronic clinical registry for prospective studies in DMD, to obtain a more complete characterization and comprehensive overview of the disease course of DMD and investigate the efficacy of the care provided



Patient and Family Experience:

- PROM – Patient-reported outcome measures (PROMIS, PHQ, Anxiety, Strength and Difficulties)
- Patient and family surveys

Financial Outcomes:

Revenues and costs assembled across and in collaboration with multiple parties in hospital system.

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References:

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