**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, follow-up, and encompassing 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**

- Emphasis on inpatient and outpatient care coordination and patient education.
- Core team includes a Patient Navigator, Neuromuscular Medicine, Genetic Counseling, and Psychosocial Services.
- Coordination with other specialties as needed.

**Care-Centralizing Strategy for Patients:** The clinic and core team serve as a central medical ‘home’ that partners with and integrates other efforts 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.

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

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

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

**Cardiopulmonary & Renal Function**
- Pulmonary function
  - Prevent and treat chest infections
  - Monitor and manage respiratory failure
  - Pediatric pulmonologists, respiratory therapists
  - Pediatric Cardiologist
  - Monitor and manage cardiomyopathy
  - Pediatric cardiologists
  - Renal function
    - Cardiorenal syndrome

**Endocrine/metabolic Function**
- Growth failure
- Insulin resistance, impaired glucose tolerance
- Obesity
  - Hypothyroidism
  - Hypercalciuria, renal stones
  - Adrenal insufficiency

**Gastrointestinal & Urological Function**
- Constipation
- Secondary lower urinary tract symptoms – urine frequency, secondary enuresis
- Fatty liver (NASH)
- Echocardiography
  - GI Specialists, Nephrologists
  - Otolaryngologists, Nephrologists

**Eye care**
- Ophthalmology
  - Dissociative compulsions of long-term glucocorticoids
  - Cataract surgery
  - Idiopathic intracranial hypertension
  - (Pseudotumor cerebi)

**Family Centric Care - Carriers**
- Genetic counseling needs for XL recessive
- Carriers for myopathy, fibrosis and left ventricular systolic dysfunction
- Genetic counseling needs for XL recessive disorder

**Clinical Outcomes**
- Registry with real-world EMR data to track care over time and across various 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)
- 2. Patient and family surveys

**Financial Outcomes**
- Revenues and costs assembled across and in the environment to model financial realization.

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**References:**