

What is the role of a
neurologist?

What are neuromuscular
standards of care?

Yaacov Anziska, MD, MPH

Director, SUNY-Downstate

MDA Neuromuscular Care Center

The neurologist
is the
“quarterback”
of the multi-
disciplinary
team

Pulmonary
Cardiology
Rehabilitation Medicine
Physical Therapy
Occupational Therapy
Orthopedic Surgery
Vocational Educational Counselling
Neuropsychology/Psychiatry
Social Work
Nutrition
Respiratory therapy
Ophthalmology
Orthotics



What are the various roles of the neurologist in DMD?

- 1) Treat DMD medically—decide on which medication (steroids, Exondys, etc.) is most suitable for the patient..
- 2) Assist in psychosocial needs—schooling, equipment, housing
- 3) Treat pain, depression—not just weakness..
- 4) Coordinate with other health care providers (not just doctors--physical therapists, occupational therapists, respiratory therapists)
- 5) Evaluate and discuss clinical trials/research in DMD, making recommendations for the patient (and his family)
- 6) *Be a good listener...*
- 7) *Be an advocate for patient and family..*
- 8) *Be a resource for patients!*

What are the standards of care in DMD?

- **Treatment**--Steroids
- **Treatment**--Exon skipping technology
- **?Future treatment**--Gene therapy
- (Investigational drugs/clinical trials)
- **Treatment**--Following with the multi-disciplinary team
- **Goal 1**—maintain ambulation for as long as possible
- **Goal 2**—if non-ambulatory, preserve hand function as long as possible
- **Goal 3**—Avoid complications—bedsores, contractures, aspiration pneumonia

Assessing response to treatment

- **Maintain Function!**
- 6-Minute Walk test
- PUL (Performance of Upper Limb)
- Timed measures (climb 4 steps, rise from supine, walk/run 10 meters)
- MRC scores/dynamometer—quantitative muscle testing
- North Star Ambulatory Assessment/MFM
- Pulmonary function testing
- Cardiac monitoring (echocardiogram, MRI)
- ?MRI, Ultrasound, Electrical impedance myography

Neuromuscular: Rehabilitation

- Use of braces/orthotics
- Exercise—aquatherapy, hypnotherapy (horseback), modified sports
- Physical/occupational therapy