What is the role of a neurologist?
What are neuromuscular standards of care?

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The neurologist is the “quarterback” of the multidisciplinary team.
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
• Use of braces/orthotics
• Exercise—aquatherapy, hypnotherapy (horseback), modified sports
• Physical/occupational therapy