



ADVOCACY FOR NEUROMUSCULAR DISEASE

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Amato Disclosures

- Medical advisory boards /Consultant for Argenx, Ra Pharmaceuticals, Alexion, Johnson & Johnson (COVID-19 vaccination program)

Learning Objectives



- Advocate for better and proper treatments for our patients with neuromuscular disorders)



Key Messages

- Need for bench and translational research
- Clinical trials with aims of improving function and quality of life
- Better quality of care for our patients in practice



Major Obstacles

- We did with rare diseases that are often slowly progressive
 - Difficultly randomizing enough patients with study long enough to demonstrate a clinically significant benefit
 - What primary outcome measures should we use?
- Cost of Drugs
- Access to participation in clinical trials and available therapies once approved
 - Inequality of access



Gene Therapies and other Recently Approved Drugs

- Eteplirsen (EXONDYS 51®), golodirsen (VYONDYS 53™), and viltolarsen (VILTEPSO®) have been approved to treat specific types of DMD
- Onasemnogene abeparvovec-xioi (ZOLGENSMA®), nusinersen (SPINRAZA®), and an oral-solution, risdiplam (EVERYSDI™), are medications that have been approved for the treatment of SMA
- S-glucosidase replacement for Pompe
- Edaravone for ALS
- Many in trial and development



American Academy of Neurology

- The AAN is advocating for the following permanent federal policy changes:
 - Removing restrictions on the site of service of the patient to ensure that all patients can access care at home and other appropriate locations
 - Ensuring patients maintain access to virtual care without restrictions based on geography
 - Continuing coverage of audio-only services, which are essential to provide health equity for patients lacking high speed internet access or are otherwise unable to operate the necessary technology
 - Ensuring equitable access to telehealth services through the development of universal access to broadband and resources to increase digital literacy to reduce disparities for patients from underrepresented racial, ethnic, and socioeconomic populations



AAN continued

- Supporting sustainable and reasonable reimbursement for audio-visual and audio-only telehealth services
- Funding data collection and more research of telehealth to better understand impact on neurologic patient care
- Maintaining and enhancing federal authority to determine appropriate providers and services for telehealth
- Making federal temporary waiver authority permanent for future emergencies