



LONDC

Late-Onset Neuromuscular Disease Consortium

Inaugural Meeting Report

September 20, 2022

Nashville, Tenn.

INTRODUCTION

The Late-Onset Neuromuscular Disease Consortium (LONDC) held its inaugural meeting on September 20, 2022 in Nashville, Tenn., in conjunction with the annual meeting of the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM). The main purpose of the inaugural meeting was for the American Neuromuscular Foundation (ANF) to introduce patient advocacy organizations (PAOs), clinicians, industry, and other stakeholders to the concept of the LONDC; gauge levels of interest in the LONDC; and begin to identify and prioritize projects for the LONDC to pursue.

Purpose of the LONDC

The LONDC aims to address the needs of individuals with any neuromuscular condition, or any sub-type, that typically manifests in adulthood. It was created to drive progress by:

- Identifying common needs and opportunities across conditions
- Establishing community-wide goals and strategies to improve care, education, and quality of life
- Facilitating collaboration among multiple stakeholders
- Supplementing work underway in the community
- Providing a forum to share updates and progress

Pre-Meeting Listening Sessions

One of the first activities of the LONDC was to conduct a series of 30-minute Zoom listening sessions with the leaders of PAOs, most of whom were unable to attend the inaugural meeting. These sessions were designed to gather input on common needs among LOND patient communities, and to inform the agenda for the inaugural meeting.

“ These people are in the middle of their careers... ”
living a regular life, and then all of a sudden, it’s
brought to a halt when diagnosed with a late-
onset neuromuscular condition. It’s a life-altering
moment; completely different compared to
someone who has had it their entire life.

-PAO Leader

INAUGURAL MEETING HIGHLIGHTS

Defining Mechanisms for Effective Collaboration

While many organizations seek the benefits of collaboration, several barriers hinder its effectiveness. The meeting participants identified and discussed the following barriers:

- Organizational inertia and lack of resources, bandwidth
- Insufficient or inconsistent communication
- Misalignment on priority goals and needs
- Reluctance of individual organizations to cede control
- Distrust and lack of understanding of other organizations
- Uncertainty and short-term costs

Meeting participants then engaged in an activity in which each table was asked to identify mechanisms for effective collaboration, and to write each mechanism on a blank puzzle piece. They then assembled the pieces into a complete collaboration puzzle that highlighted the following:

- › Listening, trust, equity, agreeing on goals, passion, compromise, prioritization, intentionality
- › Communication, follow-up, give and take, leveraging strengths, curiosity
- › Teamwork, clarity, lack of judgment, ownership, trust, acceptance, commitment, diversity
- › Action, listening, sharing, compassion, results, inclusion, money, selflessness, synthesis

The group also outlined the following practical steps, which the LONDC will apply in its project management and execution practices:

- Acknowledge limitations as individual organizations
- Identify common goals
- Identify an independent manager for the collaborative process
- Use division of labor to leverage each organization's strengths
- Share the small victories to build confidence
- Formalize processes for sharing, communication, and governance

Identifying Common Needs

A major focus of the inaugural meeting and the pre-meeting listening sessions was on identifying important needs that are common to each stakeholder group in the LONDC.



Expanded pool of specialists in adult conditions

A limited number of practitioners leads to longer diagnosis journeys and inadequate care



Increased clinician and public awareness of LONDCs

Needs include a more clearly defined burden of neuromuscular conditions on people, caregivers, families, and society



Improved accessibility and awareness of current resources

Focused on educational materials and support programs



Additional resources for clinicians

Create a “one-stop shop” for advocacy resources to increase referrals to PAOs for information and assistance



Better utilize electronic medical record (EMR) systems

Generate referrals and initiate appropriate education, diagnostic pathways, and therapeutic interventions



More clinical trials for adults

Including improved access to clinical trial education and information

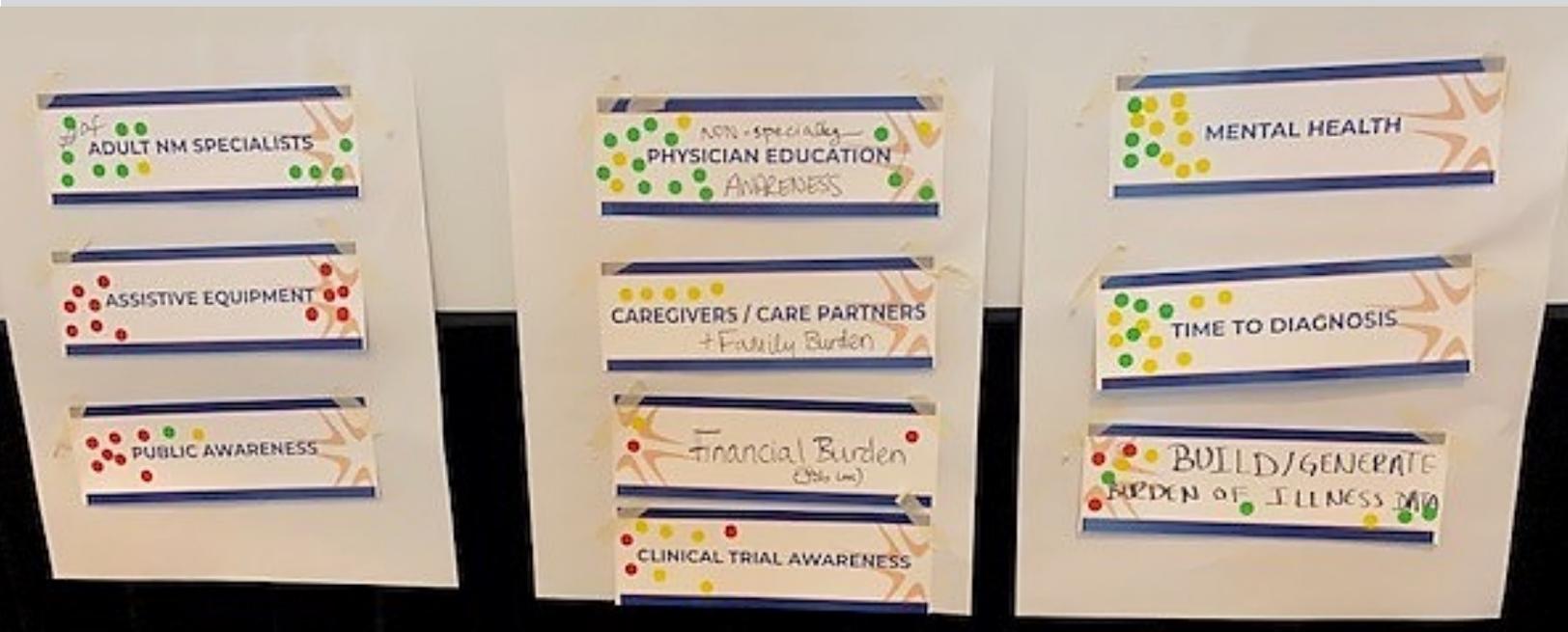


Additional funding

Increase coverage for durable medical equipment, devices, and home modifications

Prioritization of Needs/Proposed Projects

The preceding activities set the stage for a separate activity in which participants were asked to prioritize several potential projects based on identified needs. Each participant was given a limited number of colored dots to place next to specific projects displayed around the meeting room; green dots signified “Must do,” yellow dots signified “Should do,” and red dots represented “Shouldn’t do.”



The following potential projects emerged as priorities for the LONDC:

Common Experience Awareness Campaign

This initiative would identify similarities across all LONDCs (e.g., fall risk, foot drop, general weakness, swallowing difficulty, aspiration risk) as a prerequisite for creating a “warning bell” – a simple, clear mnemonic or acronym – to help clinicians and patients recognize symptoms that may indicate a neuromuscular condition. Those similarities, reinforced by the warning bell, would inform a symptom-based campaign with the aim of directing physicians to start a treatment pathway as a means to speed time to diagnosis and get people to treatment sooner.

Initial steps of the campaign would include soliciting input from groups that have conducted similar programs in other diseases (e.g., diabetes, stroke, autism, Parkinson’s); that input would be used to establish campaign timelines and to highlight lessons learned (successes as well as failures) from other programs. Other key steps – possibly conducted in parallel with the input-gathering activities – would include a literature review of similar awareness initiatives; a summit of key opinion leaders tasked with developing a consensus about common experiences among individuals with LONDCs; and a series of focus groups with patients to gain a better understanding of symptoms (i.e., to facilitate earlier diagnosis), as well as with physicians to help them understand what they have been missing.

“ We need to look at the burden of illness — this would be high-impact and the data can be used to help raise awareness. ”

-PAO Leader

Burden of Illness Study

This comprehensive study would collect psychometrics and financial data across LONDCs to measure the collective impact of LONDCs on individuals, caregivers, family members, and society. There was consensus among the meeting participants that the burden of illness study would be a high-impact/low-effort activity. The consensus was largely due to what would be a built-in population of patients and caregivers, and to the collection and analysis of data that could be used to raise awareness, presenting a relatively quick “win” for the LONDC. The data would also presumably generate a “domino effect,” whereby education and enhanced awareness would support funding for activities aimed to shorten time to diagnosis, which would allow people to start treatment sooner, and possibly prevent disease development and/or progression.

Electronic Medical Record (EMR) Triggers

This project would entail the creation of “disease state artificial intelligence” by which a presenting symptom or combination of symptoms would prompt an entry into a EMR and trigger referral to an appropriate specialist, who could make a proper diagnosis and implement an appropriate intervention. The streamlined EMR process would also be a useful teaching tool for physicians in that it would remind them of what they have learned about LONDCs. In addition to disease-specific symptoms, the EMR mechanism would incorporate quality and/or safety metrics (e.g., fall prevention), and identify best practices based on data to enable changes in physician practice patterns. Incorporation into existing electronic health record (EHR) systems could also shorten the time to implementation of best practices.

As an initial step, the LONDC would convene a panel of medical experts to identify precursor symptoms, and to evaluate project costs and feasibility. The panel would include multiple specialists, as well as one or more EMR and/or information technology (IT) experts.

The meeting participants also discussed potential mechanisms to expedite connectivity between patients/families and PAOs, utilizing patient education pathways for EMR channels. Primary care physicians also need connections to educational materials, such as those available through AANEM, to help them better understand LONDCs and how to diagnose them. One recommendation included establishing a clearinghouse website that would link to other rare diseases, with sub-links to disease-specific PAOs and physician education.

Enhancing Access to Specialty Care

The group identified a clear need to increase access to chronic health specialists, particularly psychologists and other mental health professionals who are aware of and understand chronic or neuromuscular conditions. An enhanced specialty access program would facilitate referrals for patients presenting with emotional symptoms pre-diagnosis, while helping them address and cope with the stigma of mental illness. The program would also highlight indicators for mental health professionals to facilitate appropriate referrals to clinical services. BetterHelp, an online therapy platform with a tab to help individuals locate chronic illness specialists, was mentioned as a potential model for this initiative.

Benefits Landscape Analysis

This project would aim to identify caregiver benefits that are available through state and federal programs. It would specifically query for programs that provide financial relief for job loss, as well as other programs designed to ease the burden of LONDS, while also noting state-by-state differences. Outputs would include creation of a “report card” of benefits by category. In particular, the benefits landscape analysis would evaluate how such benefits would work across the shared LONDS space and explore the reasons why such benefits have not been extended to caregivers of people with LONDS up to now.



LONDC Steering Committee

The participants discussed the establishment of the LONDC Steering Committee, the goal of which is to ensure a fair, community-driven, effective approach to execution of LONDC activities and programs. The Steering Committee's proposed role is to advise, guide, and provide input on program content to maximize credibility, value, and utility across organizations. LONDC-SC membership will include leaders of advocacy organizations in the neuromuscular space (approximately 10 representative organizations) with external input from industry and medical advisors on an ad-hoc basis.

The consensus among the participants was that everyone is interested in such an initiative but are strapped for time and resources, and therefore want to ensure the LONDC provides value and merits participation. To that end, the participants proposed the following next steps for the Steering Committee:

- Identify goals/objectives and topics in advance
- Secure administrative support to ensure timely action and follow-through
- Establish criteria for measuring/meeting milestones
- Establish a charter that outlines expectations for each project
- Consider the formation of one or more subcommittees as the Steering Committee grows
- Provide an honorarium for participation

NEXT STEPS

The inaugural meeting of the LONDC identified several unmet physician, patient, and caregiver needs, and generated numerous program ideas designed to meet those needs.

The next steps for the consortium are to:

- Establish a Steering Committee to define and develop a comprehensive program scope and plan
- Host a webinar to review a summary of the post-meeting report
- Share potential projects and priorities with all stakeholders as a means to solicit input and comments
- Secure funding to support the formation of a governance structure and priority projects
- Present project(s) to the ANF board for approval

CONTRIBUTORS

The following PAO leaders participated in the listening sessions:

- **Rachel Alvarez**, *Executive Director, Cure CMD*
- **Aditi Narayan, MSSW**, *Director, Community Support, I AM ALS*
- **Gina Olsen**, *Vice President, Healthcare Partnerships, Muscular Dystrophy Association*
- **Marissa Lozano, MEd.**, *National Director, Community Education, Muscular Dystrophy Association*
- **Susan Woolner, CPXP**, *President, Myasthenia Gravis Foundation of Michigan*
- **Jim Joyce**, *Executive Director, Myasthenia Gravis Foundation of Western Pennsylvania*
- **Kathryn Bryant**, *Founder & President, The SPEAK Foundation*

The following individuals attended the inaugural meeting:

Advocacy Organization Representatives

- **Rich Brennan, MA**, *Vice President, Federal Affairs, The ALS Association*
- **Neil Thakur, PhD**, *Chief Mission Officer, The ALS Association*
- **Lisa Butler**, *Executive Director, GBS | CIDP Foundation International*
- **Kristina Patafio**, *Board of Directors, The Lambert-Eaton LEMS Family Association*
- **Dawn Warner**, *Chief Executive Officer, MG Holistic Society*
- **Allison Foss**, *Executive Director, Myasthenia Gravis Association of Kansas City*
- **Susan Woolner, CPXP**, *President, Myasthenia Gravis Association of Michigan*
- **Rachel Bromley**, *Senior Manager, Patient Education, Support and Advocacy, The Myositis Association*

Industry Representatives

- **Christine Rowe, MBA**, *Global Director, Patient Advocacy, Alexion Pharmaceuticals, Inc.*
- **Nikita Lyons Murry, MBA**, *Patient Education Manager, Alexion Pharmaceuticals, Inc.*
- **Megan Grosso**, *Head, Global Field Medical Affairs, Amylyx Pharmaceuticals, Inc.*
- **Janelle Schafer, PhD**, *Medical Director, Amylyx Pharmaceuticals, Inc.*
- **Kathy Perez**, *Vice President, Head of Global Patient Advocacy, argenx SE*
- **Cliff Bizzell, RPh, MBA**, *Senior Medical Science Liaison, Biogen Inc.*
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- **Amy Grover**, *Senior Director, Patient Advocacy, Catalyst Pharmaceuticals, Inc.*
- **Susan Lehner**, *Senior Director, KOL Strategy, Catalyst Pharmaceuticals, Inc.*
- **BongYoung Lee**, *Director, Global Customer Engagement in Neuroscience, The Janssen Pharmaceutical Companies of Johnson & Johnson*
- **Michael McGee**, *Senior Brand Manager, Neuromuscular Disorders, Takeda*

ANF Representatives

- **Shirlyn Adkins, JD**, *Executive Director*
- **Shelly Jones, CAE, CPPM**, *Meetings, Corporate & Foundation Director*
- **Holli Horak, MD**, *2021-2022 ANF President, Professor of Neurology, University of Arizona-Tucson*
- **Robert Irwin, MD**, *2021-2022 ANF President-Elect, Professor and Vice Chair of Operations, University of Miami*
- **Dianna Quan, MD**, *2022-2023 ANF President-Elect, Professor of Neurology, University of Colorado, Anschutz*
- **Faye Tan, MD**, *2022-2023 ANF Secretary/Treasurer, Professor and Vice Chair, Faculty Affairs, Baylor College of Medicine*
- **Barry McLeish**, *ANF Public Board Member*
- **Millie Suk, JD, MPP**, *Director, Health Policy & Standards*

The inaugural meeting was facilitated and moderated by SmithSolve, a healthcare communications firm specializing in patient advocacy and rare disease-based communications.



If you would like to get involved in, or learn more about the LONDC, please contact us at LONDC@neuromuscularfoundation.org.

Thank you to our founding sponsors for supporting the inaugural meeting!

CHAMPIONS



CONTRIBUTORS

