Collaboration Drives ALS Advances in 2025

For amyotrophic lateral sclerosis (ALS), 2025 may not have been a year of headline-grabbing treatment approvals, but it saw significant developments.

“2025 was a strong year in terms of progress around new biomarkers, new drug development programs — especially those focused on targeted treatments — and global collaborative efforts,” says Sabrina Paganoni, MD, PhD, co-director of the Neurological Clinical Research Institute at Mass General Brigham (MGB) and associate professor at Harvard Medical School. “Momentum is building around different clinical development programs that I hope will give results in 2026.”

Biomarker moves from research to routine care

One meaningful shift was the transition of neurofilament light chain (NfL) testing into routine clinical practice. Clinicians can now measure NfL levels at diagnosis, offering an early prognostic signal of how quickly a person’s ALS may progress.

“It gives us one more tool to be more personalized in our treatments, helping to guide supportive interventions and treatments,” Dr. Paganoni says.

Focus on genes

Another important development is the availability of rapid genetic testing, which is allowing clinicians to more quickly identify ALS associated with an SOD1 gene mutation and begin targeted treatment with tofersen (Qalsody).

“One of the factors that predicts treatment success is giving it early,” Dr. Paganoni explains. The ALS clinic at MGB now operates a dedicated SOD1 clinic to diagnose patients and begin treatment as rapidly as possible.

This testing approach also creates new opportunities for at-risk family members and may lead to prevention strategies in the future as more targeted therapies emerge.

Momentum has also grown around treatments designed to “turn off” specific genetic problems or disease pathways. In 2025, trials moved forward for other rare genetic forms of ALS, and researchers also began using the same techniques to target key pathways involved in sporadic ALS, the most common form of the disease.

Collaborative research and clinical trials

ALS research is accelerating thanks to collaborative models that unite academic centers, industry, regulators, and people living with ALS. There are many examples of collaborative research in the field, including:

- **The HEALEY ALS Platform Trial**, a collaboration between MGB and the Network of Excellence for ALS (NEALS), has been testing multiple therapies simultaneously on a shared infrastructure to reduce timelines, lower costs, and expand access.
- **The ALL in ALS Consortium** brings together study sites across the nation to gather standardized clinical and biomarker data, creating a strong, trial-ready foundation for future treatments.

Together, these and many other efforts show how shared data, coordinated networks, and unified trial design can bring discoveries to patients faster.

MDA's role in driving progress

As progress continues, MDA is helping to push discoveries forward. "MDA has funded several ALS projects, including important efforts to enable the analysis of biomarkers and digital health data, which is allowing us to use trial data to generate new insights," Dr. Paganoni notes.

This support strengthens the research pipeline and helps ensure that advances in 2026 are built on a foundation that benefits people living with ALS today and in the future.

MDA Resources

- **Grand Rounds Webinar**: How Are Registries Informing Neuromuscular Disease?
- The **MDA Clinical Research Awareness Program** provides a service to promote clinical trials and observational studies that are actively enrolling participants. Learn more and submit a study at mda.org/meded.