Building a Stronger Path to Therapies for Hereditary Spastic Paraplegia and Primary Lateral Sclerosis

For more than 20 years, the Spastic Paraplegia Foundation (SPF) has worked toward a future with better treatments and improved quality of life for individuals and families affected by hereditary spastic paraplegia (HSP) and primary lateral sclerosis (PLS). Central to that mission has been engaging the patient, caregiver, research and clinical communities to better understand their needs and establish priorities that can move the field forward.

In 2025, SPF partnered with Critical Path Institute to build on that work and bring an additional regulatory science perspective to HSP and PLS research. The partnership is focused on evaluating the current research landscape and defining gaps that must be addressed to support successful clinical trials and regulatory decision-making. **The goal: create a more coordinated path from scientific discovery to meaningful treatments for people living with HSP and PLS.**

Advancing a promising scientific discovery into a therapy requires more than identifying a potential treatment. Researchers and drug developers need reliable data, meaningful outcome measures, biomarkers, well-designed studies, and evidence that can ultimately support regulatory decision-making.

C-Path brings expertise in these areas, including data standards, clinical outcome assessments, translational research, biomarkers, and regulatory science. SPF's deep understanding of and connection to the lived experience community ensures the work is founded in the needs and priorities of individuals and families affected by HSP and PLS.

"C-Path is proud to collaborate with SPF to develop a neutral, collaborative environment where patients, researchers, clinicians, regulators, industry, and other stakeholders can align around shared challenges and opportunities," said C-Path's Megan Miller, Director of Outreach for C-Path's Rare/Orphan and Pediatric Disease Programs.

A major milestone in the partnership came this June, when SPF and C-Path convened the Global Regulatory & Scientific Forum on HSP and PLS in St. Louis, Missouri.

The Forum brought together internationally recognized experts from academia, clinical research networks, patient advocacy organizations, government, regulatory science and industry. It was also open to attendees of the SPF Annual Conference, creating an important opportunity for patients, caregivers, researchers, clinicians and advocates to participate in conversations about the future of HSP and PLS research.

Another important component of the C-Path/ SPF partnership is improving how existing clinical research data can be used. C-Path has identified available clinical research datasets relevant to HSP and PLS and is working to compile data into its Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®). Bringing research data together in a centralized environment can help researchers better understand disease progression, identify meaningful measures and potential biomarkers, inform clinical trial design and uncover insights that may be difficult to see when datasets remain isolated.

For rare diseases, where patient populations and available data are inherently limited, maximizing the value of existing information is particularly important. A more coordinated data ecosystem can help the community learn from research that has already been conducted while laying out a stronger foundation for future studies.

C-Path's work in HSP also extends to supporting therapeutic innovation directly. C-Path's Translational Therapeutics Accelerator provided $150,000 in funding to support translational research on a novel small-molecule treatment for HSP. In addition to financial support, researchers receive scientific and regulatory guidance from the Translational Therapeutics Accelerator team intended to help bridge the gap between early discovery and therapeutic development.

Over the next year, C-Path will continue supporting SPF in developing a new scientific strategy that translates the insights generated through the forum, data initiatives, community engagement and ongoing research into clear and measurable priorities. The strategy will help identify where research investments can have the greatest impact, what tools and infrastructure are still needed, and where collaboration can help address challenges that no single organization can solve alone.

Through the partnership, SPF and C-Path are helping build that foundation. By connecting patient priorities with scientific and regulatory expertise, strengthening the use of data and creating greater alignment across the field, the two nonprofits are working to accelerate the journey from research and discovery to clinical trials and ultimately, to new treatments for people living with HSP and PLS.