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From awareness to action: How patient organizations are driving DRPLA research forward

Dentatorubral-pallidoluysian atrophy (DRPLA) is an ultra-rare neurodegenerative disorder characterized by ataxia, cognitive decline, myoclonus, chorea, epilepsy, and psychiatric manifestations. With no disease-modifying therapies approved, coordinated efforts from patients, clinicians, and researchers are essential to accelerate translational progress. CureDRPLA, a family-led non-profit foundation, was established in 2019 to address this unmet need by driving research and enabling global collaboration. Since its inception, the organization has launched the DRPLA Research Program in partnership with Ataxia UK and supported a broad portfolio of studies, including development of human cellular and mouse models, natural history and biomarker investigations, and the creation of an international patient registry.

Collecting the lived experience from people with DRPLA and their caregivers has been a priority. We conducted qualitative interviews with caregivers to capture symptom progression, impact on daily activities and views on treatments. We also hosted, in collaboration with the National Ataxia Foundation, an Externally-Led Patient Focused Drug Development meeting to share lived experiences directly with the FDA, treatment developers, and researchers.

Another major focus of CureDRPLA is the advancement of therapeutic strategies aimed at reducing expression of ATN1, the causal gene underlying DRPLA, with the goal of targeting disease mechanisms at their origin. To this end, CureDRPLA actively supports and coordinates preclinical evaluation of multiple therapeutic modalities across international research teams. However, the rarity of DRPLA presents persistent challenges, particularly in harmonizing basic and clinical research activities across geographically dispersed sites.

Keywords: DRPLA, rare diseases, patient advocacy group

Biography

Dr Silvia Prades is a neurobiologist with over six years' experience coordinating international research and clinical programs in the rare disease space. She has established collaborations across academia, industry, and patient organizations. Combining scientific rigor with strategic leadership, she has built global research networks, designed patient registries, and strengthened the bridge between scientific innovation and real-world patient impact. In 2025 Silvia joined the Ataxia Global Initiative Steering Committee. Silvia holds a PhD in Neurobiology from University College London and is completing an MBA from Imperial.

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