Huntington's Disease Society of America

September 11, 2026

The Honorable Jake Auchincloss
U.S. House of Representatives
1524 Longworth House Office Building
Washington, DC 20515

The Honorable Morgan Griffith
U.S. House of Representatives
2110 Rayburn House Office Building
Washington, DC 20515

The Honorable John Hickenlooper
U.S. Senate
316 Hart Senate Office Building
Washington, DC 20510

The Honorable Mike Rounds
U.S. Senate
716 Hart Seante Office Building
Washington, DC 20510

Dear Rep. Auchincloss, Rep. Griffith, Sen. Hickenlooper and Sen. Rounds,

The Huntington's Disease Society of America (HDSA) strongly supports the *Next-Generation U.S. Clinical Development to Accelerate Cures* legislation and its goal of modernizing the clinical development system to make clinical trials faster, more efficient, accessible, and responsive to patients with serious and rare diseases. The draft legislation's emphasis on embedding clinical research into routine care, developing point-of-care trial platforms, improving FDA transparency, and strengthening rare disease infrastructure represents an important step toward ensuring that patients are not left behind as scientific innovation accelerates.

We encourage several additional provisions to strengthen the legislation and ensure it delivers meaningful benefits for rare disease communities.

1. The legislation should explicitly recognize the critical role of natural history data in clinical development and regulatory decision-making. Robust, longitudinal natural history datasets can provide essential context for understanding disease progression, identifying meaningful endpoints, designing efficient trials, and evaluating treatment effects—particularly in rare diseases where traditional randomized trial designs can be difficult to implement. Federal policy should continue to encourage the use of natural history studies, and this legislation should take additional steps to support development, maintenance, interoperability, and appropriate regulatory use of high-quality natural history data, including data generated through patient registries and other longitudinal studies.

2. The legislation should recognize the important role played by major academic medical centers that support disease focused centers of excellence in delivering care to patients with rare diseases and build upon the model to ensure that clinical trial modernization expands access beyond urban centers. Patients with rare and progressive diseases frequently live far from specialized research centers and may face substantial travel, financial, caregiving, and accessibility barriers to participation. Point-of-care and platform trials should therefore include incentives and infrastructure to bring the expertise of the providers within centers of excellence

to rural and underserved communities, including through partnerships with community providers, telehealth, mobile research capabilities, decentralized trial approaches, and partnerships with local health care organizations. This would build on the draft legislation's recognition of the need to support health care organizations with less research capacity and to enable implementation by non-academic providers.

Many rare diseases such as Huntington's disease (HD) are particularly complex, requiring multidisciplinary expertise across neurology, psychiatry, behavioral health, rehabilitation, genetics, social work, and other areas of care. An effective approach should therefore support a model that can anchor a broader network of care and research at centers of excellence and other expert research centers with community-based providers, enabling patients to receive appropriate care and participate in clinical research closer to home while maintaining access to specialized expertise.

HDSA can help Congress identify practical opportunities to expand access. We would be pleased to arrange a meeting with HDSA Centers of Excellence staff that are already taking steps to bring clinical research closer to patients and communities. These experts can provide specific guidance on both the opportunities and challenges involved in implementing decentralized, community-based, and other innovative approaches to clinical trial participation.

3. HDSA recommends that the legislation establish stronger regulatory consistency and continuity protections. Patients, researchers, and sponsors must be able to rely on clinical trial designs and regulatory decisions that have been reviewed and accepted by the FDA. A trial design that has received FDA agreement should not subsequently be deemed unacceptable solely because of a change in FDA leadership, administration, or regulatory personnel. The legislation should promote mechanisms that document and preserve agency commitments, require clear scientific justification when previously agreed-upon trial designs or methodologies are reconsidered, and provide meaningful opportunities for sponsors and patient stakeholders to engage before major changes are made. Regulatory consistency is essential to maintaining patient confidence, encouraging investment in rare disease research, and ensuring that patients do not lose years of potential treatment opportunities because of shifting regulatory expectations.

4. The legislation should establish a pathway for greater FDA collaboration with rare disease centers of excellence. Rare diseases often require highly specialized clinical expertise to understand disease progression, identify meaningful outcomes, evaluate clinical trial designs, and interpret emerging evidence. Congress should create mechanisms that encourage the FDA to routinely engage clinical and research experts from qualified rare disease centers of excellence, such as HDSA Centers of Excellence. These centers can provide critical, real-world insight into the natural history of disease, patient experience, trial feasibility, endpoint selection, and interpretation of treatment effects. Formalizing opportunities for FDA engagement with disease-specific centers of excellence would strengthen the scientific and clinical expertise informing regulatory decisions while helping ensure that the most knowledgeable providers and researchers are meaningfully incorporated into the development and evaluation of therapies for rare and progressive diseases.

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HDSA appreciates the sponsors' efforts to modernize clinical development and believes these additions would further advance the legislation's central objective: creating a clinical research system that is more efficient, equitable, predictable, and capable of delivering new treatments to patients with unmet needs.

Sincerely,

Amy Gray
President and CEO
Huntington's Disease Society of America