SPF Global Regulatory & Scientific Forum on HSP and PLS: From Data to Approval

June 15, 2026 | 1:00 – 5:45 PM CDT | St. Louis, Missouri + Virtual

MEETING OVERVIEW

This forum is a focused, action-oriented meeting designed to align the HSP and PLS community on concrete next steps toward regulatory readiness and successful clinical trials. The agenda emphasizes brief framing remarks from global experts followed by structured, solution-focused discussion rather than extended scientific presentations. Sessions are designed to advance coordination, consensus building, and alignment around shared priorities, including priority translational research, validated endpoints, regulatory-ready data and systems, and global trial readiness. The overarching objective is to translate scientific progress into coordinated action that accelerates the development of urgently needed treatments. The forum will also directly inform SPF's future funding and investment strategy to advance their vision for a robust clinical development ecosystem for HSP and PLS.

AGENDA AT A GLANCE (times in U.S. CDT)

1:00 pm	Opening Remarks
1:15 pm	Accelerating Therapeutic Development for HSP & PLS
2:40 pm	Break
3:00 pm	Measurement That Matters: Fit-for-Use Endpoints
4:45 pm	Global Convergence on a Regulatory-Ready Data System
5:30 pm	Closing

DETAILED AGENDA

1:00 – 1:15 PM CDT | Session I: Opening Remarks

- Greg Pruitt, SPF
- Gerald Fischer, EuroHSP
- Collin Hovinga, C-Path

1:15 – 2:40 PM CDT | Session II: Accelerating Therapeutic Development for HSP & PLS

The purpose of this session is to collaboratively identify and prioritize key preclinical and translational opportunities in HSP and PLS, with an emphasis on shared biological pathways and disease convergence, and to align on the evidence required to support future regulatory development.

- Framing remarks (5 min): Srikanth Ranganathan, NINDS: disease pathways & convergence, preclinical models, target identification, translation to human studies.
- Primary Discussant Presentations: Each primary discussant will provide 4 min of prepared remarks on the indicated topic, focusing on existing evidence, key gaps, and top priorities moving forward.
    - John Fink – A pathway approach to therapeutic development in HSP and PLS
    - P. Hande Ozdinler – Disease convergence, preclinical models & translational research
    - Darius Fakhari – Gene therapy readiness & priorities in translational research
    - Keith Gottlieb – Moving from preclinical discovery to clinical development
- Moderated Discussion: Collin Hovinga & Srikanth Ranganathan will co-facilitate a discussion with all primary discussants and other speakers and participants.

2:40 – 3:00 PM CDT | BREAK

3:00 – 4:45 PM CDT | Session III: Measurement That Matters: Fit for Use Endpoints

The purpose of this session is to build alignment on priority "fit-for-use" endpoints for HSP and PLS and the evidence required to support regulatory acceptance and clinical trial use.

- Framing remarks: (4 min) Collin Hovinga, C-Path – Patient Focused Drug Development & the path to regulatory acceptance.
- Primary Discussant Presentations: Each primary discussant will provide 4 min of prepared remarks on the indicated topic, focusing on existing evidence, key gaps, and top priorities moving forward.
    - Rebecca Schüle, Jonas Saute, & Darius Fakhari – COA validation, with a focus on the Modified SPRS and PROs
    - Craig Blackstone – Developing digital endpoints & remote monitoring
    - Michael Benatar & Jeffrey Statland – Progress toward fluid biomarkers for HSP and PLS
    - Peter Bede – Progress toward imaging biomarkers for HSP and PLS
    - Sabrina Paganoni — Implications for patient-centered clinical trial design & innovation
- Moderated Discussion: Collin Hovinga will facilitate a discussion with all primary discussants as well as other speakers and participants.

4:45 – 4:50 PM CDT | BRIEF BIO BREAK

4:50 – 5:30 PM CDT | Session IV: Global Convergence on a Regulatory-Ready Data System

The purpose of this session is to build alignment on evidentiary standards and data infrastructure required for regulatory use and identify priority actions to coordinate global efforts.

- Framing remarks (8 min): Collin Hovinga – data standards, regulatory expectations, and the role of registries and natural history data in supporting endpoints & trial design.
- Primary Discussant Presentations: Each primary discussant will provide brief prepared remarks on the indicated topic, focusing on existing evidence, key gaps, and top priorities moving forward.
    - Ikjae Lee (4 min prepared remarks) – PLS Natural History Study
    - Jonas Saute, Marcondes França, Darius Fakhari, & Rebecca Schüle (10 min prepared remarks, shared) – global data standardization; key priorities following the Brazil meeting; regulatory engagement
- Moderated Discussion: Collin Hovinga will facilitate a discussion with all primary discussants as well as other speakers and participants.

5:30 – 5:45 PM CDT | Session V: Closing

- Collin Hovinga – reflections from the day
- Greg Pruitt – closing remarks & call to action

DISCUSSION QUESTIONS BY SESSION

Session II: Accelerating Therapeutic Development for HSP & PLS (1:15 – 2:40 CT)

- What pathways or targets are most ready for therapeutic development in HSP and PLS?
- What criteria should be used to justify prioritization of targets going forward?
- Where can areas of convergence with other diseases be leveraged to advance therapeutic development in HSP and PLS? (i.e. what can we learn from progress in other rare neurodegenerative diseases, like ALS?)
- What gene therapy or ASO approaches are most advanced or promising for HSP? What evidence is still needed to support further progression toward clinical testing?
- How should preclinical evidence be used to inform clinical development decisions in HSP/PLS?
- What level and type of evidence is needed to support regulatory engagement? What gaps exist?
- Where are the most critical evidence gaps across models and mechanisms?
- If we had to prioritize 2-3 actions in the next 1 to 2 years to accelerate therapeutic development in HSP/PLS, what would they be?

Session III: Measurement That Matters: Fit for Use Endpoints (3:00 – 4:45 CT)

- What endpoints across COAs, biomarkers, digital measures, and imaging are closest to being fit-for-use today in HSP or PLS?
- What evidence supports their use? How have they been validated?
    - What concepts do current COAs capture, and how were patients involved in defining them?
    - For biomarkers, what evidence supports prognostic, progression, or pharmacodynamic use?
    - How has imaging been linked to clinical outcomes and/or genetic subtypes?
- What gaps must be addressed for candidate endpoints to be used in trials and the regulatory context?
- As we move toward validated endpoints, what are the important considerations for trial design?
    - How can digital tools improve sensitivity and/or patient burden in trials?
    - How does natural history data inform trial design & what should the field consider now?
    - What key steps should the field take to get to a master protocol in HSP and PLS?
- What are the top 2-3 actions needed to advance endpoint readiness in the next 12-18 months?

Session IV: Global Convergence on a Regulatory-Ready Data System (4:50-5:30)

- What natural history and registry data are currently being generated across global networks in HSP and PLS?
- What types of data are available, and what common data elements exist across studies/networks?
- Where are the biggest gaps in data standardization across global efforts?
- What resources would be needed to create more interoperability?
- How can the field better coordinate data sharing while respecting governance and access considerations?
- What are the top priorities coming out of the Brazil meeting to advance toward a regulatory ready data system?
- What are the top 2-3 actions needed to move toward a globally aligned, regulatory ready data system?